UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K/A

(Amendment No. 1)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(D) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2020

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(D) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission File Number 000-50549

Oncternal Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**62-1715807**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**

12230 El Camino Real, Suite 300
San Diego, CA 92130
(858) 434-1113
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Trading Symbol (s)	Name of Each Exchange on Which Registered
Common Stock, par value $0.001 per share	**ONCT**	**The Nasdaq Capital Market**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒.

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒.

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company", and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Smaller reporting company	☒
Accelerated filer	☐	Emerging growth company	☐
Non-accelerated filer	☒		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

Indicate by check mark whether the registrant is a shell company (as defined by Rule 12b of the Exchange Act). Yes ☐ No ☒

As of June 30, 2020, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock held by non-affiliates of the registrant was approximately $27.2 million, based on the closing price of the registrant's common stock on the Nasdaq Capital Market on June 30, 2020 of $2.84 per share.

The number of outstanding shares of the registrant's common stock as of March 3, 2021 was 49,281,327.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Registrant's proxy statement to be filed with the Securities and Exchange Commission pursuant to Regulation 14A in connection with the Registrant's 2021 Annual Meeting of Stockholders, which will be filed subsequent to the date hereof, are incorporated by reference into Part III of this Form 10-K. Such proxy statement will be filed with the Securities and Exchange Commission not later than 120 days following the end of the Registrant's fiscal year ended December 31, 2020.

EXPLANATORY NOTE

Oncternal Therapeutics, Inc. (the "Company") is filing this Amendment No. 1 on Form 10-K/A (the "Amendment") to its annual report on Form 10-K for the fiscal year ended December 31, 2020, which was originally filed with the Securities and Exchange Commission on March 11, 2021 (the "Original Filing").

The purpose of the Amendment is to correct a typographical error in the BDO USA, LLP, Report of Independent Registered Public Accounting Firm ("Auditor's Report"), included in Item 8, "Financial Statements and Supplementary Data." The typographical error that was corrected concerns the date of the Auditor's Report, which referenced 2020 instead of 2021 due to an inadvertent oversight in the EDGAR preparation process. No other changes were made to the Auditor's Report other than the date included in the EDGAR filing.

This Amendment amends the Original Filing with respect to Item 8, "Financial Statements and Supplementary Data." As required by Rule 12b-15 under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), new certifications of our principal executive officer and principal financial officer are also being filed as exhibits 31.1, 31.2 and 32.1 to this Amendment. Similarly, revised XBRL exhibits are being filed as exhibits to this Amendment. As a result, Item 15, "Exhibits and Financial Statement Schedules", has also been modified. In addition, the consent filed as Exhibit 23.1 to this Amendment is dated as of the filing date of this Amendment.

This Amendment does not reflect events occurring after the filing of the Original Filing or modify or update any related other disclosures and should be read in conjunction with the Original Filing made with the Securities and Exchange Commission.

Oncternal Therapeutics, Inc.

FORM 10-K — ANNUAL REPORT
For the Fiscal Year Ended December 31, 2020

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PART I

Cautionary Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K, or this Annual Report, including the sections entitled “Summary,” “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Business,” contains forward-looking statements. We may, in some cases, use words such as “anticipate,” “believe,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes, to identify these forward-looking statements. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. Forward-looking statements in this Annual Report include, but are not limited to, statements about:

- our ability to obtain and maintain regulatory approvals for our product candidates, including cirmtuzumab and TK216;
- our ability to identify and advance into the clinic product candidates from our ROR1-targeted CAR-T therapy program;
- the expected timing for achieving key milestones, including commencing, completing and announcing clinical trial results of our product candidates;
- the timing or likelihood of regulatory filings and approvals;
- the estimated size of the patient population and anticipated market potential for our product candidates;
- the impact of products that compete with our product candidates that are or may become available;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- our ability to obtain and maintain favorable regulatory designations for our product candidates and preclinical programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and our ability to operate our business without infringing upon the intellectual property rights of others;
- our commercialization, marketing and reliance on third-party manufacturing capabilities and strategy;
- the impact the COVID-19 pandemic has had on our business and the U.S and global economies;
- the plans and objectives of management for future operations and future results of anticipated products; and
- our estimates regarding the sufficiency of our cash resources and our expenses, capital requirements and need for additional financing, and our ability to obtain additional financing.

These forward-looking statements reflect our management’s beliefs and views with respect to future events and are based on estimates and assumptions as of the date of this Annual Report and are subject to risks and uncertainties. We discuss many of these risks in greater detail under “Risk Factors.” Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

We qualify all of the forward-looking statements in this Annual Report by these cautionary statements. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

SUMMARY OF RISK FACTORS

Investing in our common stock is subject to numerous risks and uncertainties, including those described in Part I, Item 1A, "Risk Factors" of this Annual Report on Form 10-K. The principal risks and uncertainties affecting our business include the following:

- We have a limited operating history, have incurred significant operating losses since our inception and expect to incur significant losses for the foreseeable future. We may never generate any revenue or become profitable or, if we achieve profitability, we may not be able to sustain it.
- We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed and on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations.
- The COVID-19 outbreak may adversely impact our business.
- We depend heavily on the success of cirmtuzumab and TK216, which are in Phase 1 or Phase 1/2 clinical trials, as well as our ROR1 CAR-T program, which is in preclinical development. If we are unable to advance our product candidates in clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.
- Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and the results of preclinical studies and early clinical trials are not necessarily predictive of future results. Our product candidates may not have favorable results in clinical trials or receive regulatory approval on a timely basis, if at all.
- We may find it difficult to enroll patients in our clinical trials. If we encounter difficulties enrolling subjects in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- We rely on third parties for the manufacture of our product candidates for clinical and preclinical development and expect to continue to do so for the foreseeable future.
- We may not be able to maintain orphan drug designations for certain of our product candidates, and may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.
- Fast Track designation by the FDA for TK216 or our other product candidates may not actually lead to a faster development or regulatory review or approval process.
- Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.
- We rely on third parties to conduct many of our preclinical studies and clinical trials. Any failure by a third-party to conduct the clinical trials according to good laboratory practices, GCPs and other requirements and in a timely manner may delay or prevent our ability to seek or obtain regulatory approval for or commercialize our product candidates.
- If the market opportunities for our products are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.

- Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or any guidance we may provide.

- Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Item 1. Business.

Overview

Oncternal Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies for cancers with critical unmet medical need. Our development efforts are focused on promising, yet untapped, biological pathways implicated in cancer generation or progression. Our pipeline includes cirmtuzumab, an investigational monoclonal antibody that is designed to inhibit Receptor tyrosine kinase-like Orphan Receptor 1, or ROR1, a growth factor receptor that is widely expressed on many tumors and that activates pathways leading to increased tumor proliferation, invasiveness and drug resistance. Cirmtuzumab is being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib (Imbruvica®) (Cirmtuzumab and Ibrutinib targeting ROR1 for Leukemia and Lymphoma, or CIRLL) for the treatment of patients with B-cell lymphoid malignancies, including mantle cell lymphoma, or MCL, and chronic lymphocytic leukemia, or CLL, and in an investigator-sponsored, Phase 1b clinical trial in combination with paclitaxel for the treatment of women with HER2-negative metastatic or locally advanced, unresectable breast cancer. We are also developing a chimeric antigen receptor T cell, or CAR-T, therapy that targets ROR1, which is currently in preclinical development as a potential treatment for hematologic cancers and solid tumors. In addition, we are developing TK216, an investigational small molecule that inhibits the ETS, or E26 Transformation Specific, family of oncoproteins. ETS alterations have been shown in preclinical studies to alter gene transcription and RNA processing and lead to increased cell proliferation and invasion. TK216 is being evaluated in a Phase 1 clinical trial as a single agent and in combination with vincristine in patients with relapsed or refractory Ewing sarcoma, a rare pediatric cancer.

The U.S. Food and Drug Administration, or FDA, has granted orphan drug designations for cirmtuzumab for the treatment of MCL and for the treatment of CLL/small lymphocytic lymphoma, and has granted rare pediatric disease designation, as well as orphan drug and fast track designations for TK216 for the treatment of Ewing sarcoma.

Cirmtuzumab is an investigational, humanized, potentially first-in-class, ROR1 monoclonal antibody that is designed to bind to a specific functionally important epitope of ROR1. ROR1 is a protein expressed on many tumors, and is a potentially attractive target for cancer therapy because it is an onco-embryonic antigen, a protein typically expressed during embryogenesis that may confer a survival and fitness advantage when reactivated and expressed by tumor cells. ROR1 overexpression in multiple tumor types, including MCL, CLL and breast cancer, has been associated with more aggressive disease, resistance to therapy and shorter progression-free survival, or PFS, and overall survival, or OS. In preclinical models, inhibition of ROR1 has shown anti-tumor activity, and we believe may have additive or synergistic effects when combined with either targeted therapy or chemotherapy. Preclinical data indicated that when cirmtuzumab bound to ROR1, it blocked growth factor Wnt5a signaling, inhibited tumor cell proliferation, migration and survival, and induced differentiation of CLL tumor cells. Cirmtuzumab was developed in the laboratory of one of our scientific advisors, Professor Thomas Kipps, M.D., Ph.D., Professor of Medicine and Evelyn and Edwin Tasch Chair in Cancer Research at the University of California San Diego, or UC San Diego, with support from the California Institute for Regenerative Medicine, or CIRM. We have an exclusive, worldwide license to develop cirmtuzumab for certain therapeutic uses from UC San Diego.

In December 2020, we presented updated interim data from the CIRLL trial in patients with MCL and CLL at the American Society for Hematology 2020 Virtual Meeting, or ASH 2020 annual meeting. The combination of cirmtuzumab plus ibrutinib has been well tolerated, with adverse events consistent with those reported for ibrutinib alone. To date, there have been no dose-limiting toxicities and no serious adverse events attributed to cirmtuzumab alone. As of the data cut-off date of October 30, 2020, 15 patients with relapsed/refractory MCL enrolled in the dose-finding and dose-expansion cohorts of this clinical trial were evaluable for efficacy. The objective response rate, or ORR was 87% (13 of 15 evaluable patients), improved over the 83% ORR reported at the ASCO Annual Meeting in May 2020, or ASCO 2020 annual meeting. The complete response rate, or CR, determined by Cheson

criteria, remained 57% (7 of 12 evaluable patients) for Part 1 of the study, and was 47% (7 of 15 evaluable patients) for Part 1 + Part 2, including three patients from Part 2 who had been on the study for a relatively brief time as compared to other patients. One of the seven patients with CR had a complete metabolic response as assessed by PET scan, with an indeterminate bone marrow biopsy on blinded review. All complete responses were durable, ranging from 5 to 25 months as of the cutoff date, with no progressions reported after achieving a CR. Six patients (40%) achieved a partial response, or PR. In addition, two patients had stable disease, or SD, for a total best clinical benefit rate (CR, PR and SD) of 100%. Median PFS was not reached, with the 95% confidence interval above 17.5 months, after a median follow-up of 12.1 months. Patients had received a median of two prior therapies (range 1-5) before participating in this clinical trial, with 73% of patients having received two or more prior lines of therapy. Four patients had received prior treatment with ibrutinib and all four achieved clinical responses in this clinical trial, with two CRs and two PRs. Fourteen of the 15 evaluable patients (93%) had high or intermediate MIPI-b risk score at study entry. Historical data for 370 patients with relapsed/refractory MCL from three clinical trials who had also received a median of two prior therapies (Rule et al., 2017, British Journal of Haematology), showed an ORR of 66%, CR rate of 20%, and median PFS of 12.8 months (95% confidence interval 8.5 – 16.6 months) for single agent ibrutinib in patients with MCL.

Additionally, as of the data cut-off date of October 30, 2020, 56 evaluable patients with CLL were enrolled in the dose-finding, dose-confirming and randomized cohorts of this clinical trial, 49 of whom were treated with the combination of cirmtuzumab and ibrutinib. Forty-five of the 49 patients achieved a clinical response, for an overall best ORR of 92%, including one patient who achieved a CR. In addition, four patients had SD, for a total clinical benefit rate (CR, PR, and SD) of 100%. The median PFS was not reached for patients with treatment-naïve CLL (n=19) after a median follow-up of 16.6 months, and median PFS was 29.5 months for patients with relapsed/refractory CLL (n=30) after a median follow-up of 17.1 months.

We expect to announce a data update from this Phase 1/2 clinical trial in the second quarter of 2021.

Cirmtuzumab is also being evaluated in an investigator-sponsored Phase 1b clinical trial in combination with paclitaxel in patients with HER2 negative breast cancer. We expect to announce interim data from this Phase 1b clinical trial in the second quarter of 2021. We are also supporting a new investigator-sponsored Phase 2 clinical trial of cirmtuzumab in combination with venetoclax, a Bcl-2 inhibitor, in patients with relapsed/refractory CLL in collaboration with UC San Diego, which is open and enrolling patients. Additionally, we plan to further explore clinical combination strategies for cirmtuzumab for patients with hematologic malignancies. We also continue to evaluate the use of cirmtuzumab in additional ROR1-expressing tumors and expect to announce data from additional preclinical studies in the second quarter of 2021.

We are also developing a ROR1-targeted CAR-T cell therapy candidate utilizing the binding domain of cirmtuzumab as a single-chain variable region fragment, or scFv, as a potential treatment for patients with aggressive hematological malignancies or solid tumors. Because cirmtuzumab has been shown to bind to multiple cancers but not to normal adult tissues in preclinical studies, we believe that a cirmtuzumab-based CAR-T may be selective in distinguishing cancer from normal tissues. Our preclinical ROR1-targeted CAR-T cell therapy candidate was developed in collaboration with UC San Diego, with funding support from CIRM, and is being further developed in collaboration with the Karolinska Institutet in Stockholm, Sweden and under a manufacturing agreement with Lentigen Technology, Inc., or Lentigen, to produce clinical-grade lentivirus.

Shanghai Pharmaceuticals Holding Co., Ltd., or SPH, has the right to develop a ROR-1 targeted CAR-T therapy in greater China, and we are collaborating with them in this effort. SPH recently announced breaking ground on a 3.2 million square foot biotech park in Shanghai that will include R&D facilities and manufacturing facilities for CAR-T. We expect the first-in-human dosing of our ROR1-targeted CAR-T cell therapy in China in the second half of 2021 in collaboration with SPH. In parallel, we are continuing preclinical development activities in the U.S. and Europe.

TK216 is an investigational, potentially first-in-class, targeted small molecule that is designed to specifically inhibit the biological activity of the ETS family of oncoproteins. Tumorigenic gene fusions involving ETS factors are frequently found in tumors such as Ewing sarcoma and prostate cancer, and ETS proteins are often overexpressed in other tumors, such as acute myeloid leukemia, or AML, and diffuse large B-cell lymphoma, or DLBCL. Researchers in the laboratory of Professor Jeffrey Toretsky, M.D., at

Georgetown Lombardi Comprehensive Cancer Center, identified the precursor to TK216 using a novel chemical screening assay they developed based on a deep understanding of the underlying biological mechanism of ETS factors. Following this early work, TK216, which is designed to be a specific inhibitor of ETS factors, was created by us through the rational design and screening of novel small molecule inhibitors of a critical protein-protein interaction. In preclinical models, TK216 inhibited the interaction between ETS family members and RNA helicase A, or RHA, and by doing so, inhibited excessive cell proliferation. We own intellectual property related to TK216 and have an exclusive license to product candidates targeting ETS oncoproteins for therapeutic, diagnostic or research tool purposes from Georgetown University.

We are evaluating TK216 as a single agent and in combination with vincristine in an open-label, multicenter Phase 1/2 clinical trial in patients with relapsed or refractory Ewing sarcoma. Ewing sarcoma is a rare pediatric cancer that has historically been very challenging to treat effectively, particularly for recurrent and metastatic disease. ETS fusion proteins have been shown to be present in over 90% of Ewing sarcoma cases. The dose-finding portion of the study was completed in 2019, and we continue to enroll patients in the Phase 2 expansion cohort to evaluate the clinical response of treatment with TK216 in combination with vincristine using the recommended Phase 2 dose, or RP2D, regimen. The RP2D has been established to be 200 mg/m2/day of TK216 for 14 days, with vincristine 0.75 mg/m2 on the first day of each 28 day treatment cycle. In November 2020, we announced updated interim clinical data from our ongoing open-label, multicenter Phase 1/2 clinical trial evaluating TK216 in patients with relapsed or refractory Ewing sarcoma. Patients entering the trial had previously been treated with a median of three, and as many as eight prior lines of systemic therapy. The presentation included interim data for 50 evaluable patients, including 23 evaluable patients treated at the RP2D as of the October 16, 2020 efficacy cut-off date. Two of the 23 patients treated at the RP2D (9%) achieved a CR, including one surgical CR. Both patients achieving CRs remain on treatment, with no evidence of disease, at 1.5 years and 8 months on study as of the cutoff date. The best ORR was 9%. Eight additional patients treated at the RP2D had SD, for a disease control rate (CR, PR or SD) of 43%. The median progression-free survival for patients treated at the RP2D was 1.8 months.

TK216 has been generally well tolerated in this trial. As of the October 2, 2020 safety cut-off date, the most common drug-related adverse events included myelosuppression, fatigue, alopecia, nausea, pyrexia and decreased appetite. Dose limiting toxicities consisted of transient and manageable myelosuppression, primarily neutropenia. No unexpected off-target toxicities have been observed. We expect to announce interim data from the Phase 1/2 expansion cohort as well as preclinical data in additional ETS-driven tumors in the second quarter of 2021.

Pharmacokinetic data from the clinical trial showed that TK216 drug levels at the RP2D exceeded plasma levels associated with anti-tumor activity in preclinical models.

We have assembled a management team, board of directors and scientific founders who have significant experience in successfully developing and commercializing therapeutics in oncology and orphan diseases, having worked or served on the Board of companies such as Amgen, Inc., Bavarian Nordic, Inc. (lead cancer asset acquired by Bristol Meyers Squibb Company), Cadence Pharmaceuticals, Inc. (acquired by Mallinckrodt plc), Dynavax Technologies Corporation, Elan Corporation (acquired by Perrigo), Eli Lilly and Co., Gilead Sciences, Inc., Innocrin Precision Therapeutics, Inc., Janssen Pharmaceuticals, Inc., Merck, Micromet, Inc. (acquired by Amgen, Inc.), Pfizer, Inc., Roche Holding AG, Sorrento Therapeutics, Inc., Tracon Pharmaceuticals, Inc., and Zavante Therapeutics, Inc. (acquired by Nabriva Therapeutics plc).

Our strategy

Our mission is to build a leading oncology company that creates novel and transformative treatments for a wide range of oncology indications for which there are significant unmet medical needs. We believe our investigational agents target novel cancer pathways and have unique mechanisms of action. Our current pipeline is derived from our ability to identify therapeutic candidates that have generated promising, late-stage preclinical results or early clinical data, and in-license them for further development. We are particularly focused on therapeutic approaches for which there is a genetic or protein biomarker that can be used to identify populations of patients most likely to respond. We prioritize targets that we believe have the potential to transform the treatment paradigm of difficult-to-treat cancers with either single agent or combination therapy. As is the case for many oncology products, we believe that potential efficacy in one indication suggests the potential for application in other indications that carry the same target.

Key elements of our strategy are as follows:

- advance cirmtuzumab through clinical development in multiple indications, with a primary focus in MCL;
- advance our ROR1-targeting CAR-T cell therapy candidate into clinical development, initially in hematological cancers and then in solid tumors;
- evaluate cirmtuzumab preclinically in additional ROR1-positive solid tumors such as lung, ovarian and prostate cancers, as well as in additional hematological malignancies;
- advance TK216 in combination with vincristine for the treatment of relapsed/refractory Ewing sarcoma through clinical development, including completion of the ongoing Phase 1/2 clinical trial; and
- evaluate TK216 preclinically in additional tumors with ETS fusion proteins or overexpression, such as prostate cancer, lymphoma and AML.

Pipeline

The following figure summarizes our current programs:

Business Update Regarding COVID-19

The COVID-19 worldwide pandemic has presented substantial public health and economic challenges and is affecting economies, financial markets and business operations around the world. International and U.S. governmental authorities in impacted regions have taken action in an effort to slow the spread of COVID-19, including issuing and modifying varying forms of “stay-at-home” orders, and restricting business functions outside of one’s home. In response, we have put restrictions on employee travel and working from our executive offices, with many employees continuing their work remotely. To date, we have been able to continue to supply cirmtuzumab and TK216 clinical trial sites for patients enrolled in our ongoing clinical trials and do not currently anticipate any interruptions in the supply of cirmtuzumab or TK216. While we are continuing the clinical trials we have underway in sites across the U.S., we expect that COVID-19 precautions may continue to directly or indirectly impact the timeline for some of our clinical trials. For example, some of our clinical trial sites, including those located in areas severely impacted by the pandemic, have placed new patient enrollment into clinical trials on hold or, for patients travelling from out-of-state, have implemented a 14-day self-quarantine before appointments. Patients with MCL or CLL may be at increased risk of severe disease if they develop COVID-19 because of advanced age and/or immunosuppression, and so may be unwilling to travel to our study centers to enroll in our clinical trials. For our existing patients, we are actively working with all of our clinical trial sites to minimize disruptions and address concerns on an individual site or patient basis in order to allow participating patients to

continue to receive treatment at home or in alternative healthcare settings while minimizing their potential exposure to the virus that causes COVID-19. If restrictions related to the COVID-19 pandemic continue for a prolonged period of time or if additional clinical trial sites pause patient enrollment or treatments, our clinical trial milestones would be negatively impacted. Any delays in the completion of our clinical trials and any disruption in our supply chain could have a material adverse effect on our business, results of operations and financial condition. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain or treat it, , the success or failure of vaccination programs, the emergence of new variants of COVID-19, as well as the economic impact on local, regional, national and international markets.

Cirmtuzumab - monoclonal antibody targeting ROR1

Cirmtuzumab scientific background: inhibition of ROR1 as a therapeutic strategy in cancer

ROR1 is an onco-embryonic protein essential for normal fetal development whose expression is suppressed in adults unless reactivated as a survival factor by many different cancers. The switching-on of ROR1 is consistent with a pattern in cancer, in which normal cells lose their highly differentiated functions and return to a more primitive state, where they exhibit a greatly increased capacity for invasion, metastasis and resistance to treatment. This de-differentiation is associated with expression of a number of genes normally restricted to fetal development, one of which is ROR1. Cancer cells with the highest potential for self-renewal are sometimes referred to as tumor-initiating cells or cancer stem cells and are capable of invading other tissues or metastasizing to form tumors in distant sites in the body. These tumor-initiating cells are also the cells that have been found to be the most resistant to standard cancer therapies including chemotherapy and radiation therapy. Cancer cells that overexpress ROR1 have been shown to have increased survival, migration and resistance to chemotherapy.

Over-expression of ROR1 has been reported in multiple hematological and solid tumor types. Histological staining of over 350 human tumor samples identified that a majority expressed ROR1, including 90% or more of uterine cancers, lymphomas and prostate cancers.

Cancer type	ROR1 Expressed (%)	Cancer type	ROR1 Expressed (%)
Uterus	96%	Adrenal	83%
MCL	>95%	Lung	77%
CLL	95%	Breast	75%
Lymphoma	90%	Testicular	73%
Prostate	90%	Colon	57%
Skin	89%	Ovarian	54%
Pancreas	83%	Bladder	43%

High ROR1 expression on patients' tumor cells in a variety of cancers is associated with the development of metastases, and early relapse after therapy. ROR1 expression levels on patients' tumor cells is higher in cancers that are more advanced or poorly differentiated. For example, whereas Grade 1 or 2 ovarian tumors were found to be 21% positive for ROR1, Grade 3 or 4 tumors were found to be 62% positive for ROR1. Similar patterns in the percentage of ROR1-positive tumors were seen in pancreatic cancers, with 54% of Grade 1 or 2 tumors and 100% of Grade 3 or 4 tumors testing positive for ROR1 by immunohistochemistry. High expression of ROR1 has been associated with more aggressive disease and shorter patient survival in multiple tumor types, including CLL, breast cancer and ovarian cancer.

Inhibition of ROR1 signaling or silencing ROR1 in multiple preclinical cancer models, including breast cancer, ovarian cancer and glioblastoma, was associated with suppressing the expression of genes characteristic of tumor-initiating cells, and with repression of cancer migration and metastasis. Preclinical models have also demonstrated that inhibition of ROR1, or blocking of Wnt5a-induced signaling, inhibited tumor cell proliferation, migration and survival, and induced differentiation of the tumor cells – resulting in fewer metastases and improved survival.

Inhibition of ROR1 has been demonstrated in preclinical models to be additive to, or synergistic with, chemotherapy agents such as paclitaxel, and with targeted therapy agents such as ibrutinib and venetoclax. In addition, inhibition of ROR1 has been shown to enhance sensitivity of cancer cells to targeted therapy with agents such as erlotinib and may increase apoptosis and decrease proliferation.

In summary, we believe that ROR1 is an attractive therapeutic target in oncology for several reasons:

- ROR1 is widely expressed on many tumors, including hematological malignancies and solid tumors;
- Expression of high levels of ROR1 on patients' tumors is associated with more rapid disease progression, resistance to therapy and shorter patient survival;
- Blocking of ROR1 in preclinical models inhibited tumor cell proliferation, migration and survival, and induced differentiation of the tumor cells;
- Inhibition of ROR1 has been observed in preclinical models to be synergistic with chemotherapy and targeted therapy; and
- Clinical data presented for a ROR1-targeting antibody-drug conjugate (ADC) presented at the ASH 2020 annual meeting did not reveal any unusual off-tumor organ toxicity.

Two notable acquisitions in 2020 involved companies developing product candidates targeting ROR1: Merck & Co. acquired VelosBio, Inc. and its ROR1-targeting ADC, and Boehringer-Ingelheim acquired NBE Therapeutics and its ROR1-targeting ADC.

Cirmtuzumab development in Mantle Cell Lymphoma and Chronic Lymphocytic Leukemia

MCL disease overview

MCL is an aggressive form of non-Hodgkin's lymphoma. There are approximately 4,200 new cases of MCL each year in the U.S., with the average age at diagnosis in the mid-60s. MCL is an aggressive lymphoma and carries a poor prognosis, with a median survival of about two to five years. The 10-year survival rate is only approximately 5-10%.

While there are several therapeutic options available to treat patients with relapsed or refractory MCL, none of these options offers long-term benefit, with most patients relapsing in less than 20 months. Inhibitors of Bruton's Tyrosine kinase, or BTK, such as ibrutinib (Imbruvica®) are emerging as a standard of care in patients who have failed other therapies. These therapies are given continuously for prolonged periods of time, and their use can be associated with significant toxicity. The majority of patients with MCL are older, and remissions are not durable for most patients treated with continuous ibrutinib therapy. As a result, we believe that more effective and better tolerated therapies with shorter treatment periods represent a significant unmet need.

CLL disease overview

CLL is the most common form of leukemia in adults, accounting for 25-30% of all leukemias in the U.S. An estimated 20,720 new cases of CLL were expected to occur in the U.S. in 2019, and in 2016 the prevalence of CLL in the U.S. was estimated to be 178,000 patients. CLL is primarily a disease of older adults. The median age at diagnosis is 71 years of age. Most patients are diagnosed as a result of routine blood work when elevated levels of lymphocytes are detected.

BTK inhibitor therapy has also emerged as a standard of care for CLL, and is increasingly used as first-line therapy. Patients with CLL can enjoy a substantial period of disease control, but the disease eventually recurs, and is more likely to do so for patients with previous CLL therapy, and if certain risk factors such as high simplified MCL international prognostic index (sMIPI), presence of bulky disease, and blastoid histology. The trade-off of these newer targeted therapies is the paradigm of continuous treatment required for BTK inhibitors, resulting in accumulating costs and toxicities. Adverse events have been shown in a real-world analysis to limit ibrutinib treatment duration for almost half of all patients. An acceptable safety profile may be particularly important for patients with CLL who are older (the median age at diagnosis is 71) and have multiple co-morbidities.

The market for CLL therapies in the U.S., France, Germany, Italy, Spain, the UK, and Canada is estimated to be approximately $8 billion, largely driven by recently approved therapies, including ibrutinib, venetoclax, and idelalisib. We believe that CLL represents an attractive clinical and commercial opportunity for cirmtuzumab.

Cirmtuzumab preclinical summary in MCL and CLL

ROR1 is a potentially attractive target for cancer therapy because it is an onco-embryonic antigen, which is a protein typically expressed during embryogenesis that may confer a survival and fitness advantage when reactivated and expressed by tumor cells. ROR1 is over-expressed in many different cancers, including MCL, CLL and breast cancer, and has been reported to be associated with more aggressive disease, resistance to therapy and shorter PFS and OS. In preclinical models, inhibition of ROR1 has shown anti-tumor activity and we believe may have additive or synergistic effects when combined with other agents.

Cirmtuzumab is an investigational, humanized monoclonal antibody, or mAb, designed to bind to a specific functionally important epitope of ROR1. The ligand for ROR1 in hematologic malignancies is Wnt5a, a secreted glycoprotein that has a critical role in embryonic and fetal development. Researchers at the UC San Diego School of Medicine discovered that targeting a critical epitope on ROR1 was key to inhibiting Wnt5a activation, specifically targeting ROR1 expressing tumors. This led to the development of cirmtuzumab, which binds this critical epitope of ROR1. Cirmtuzumab was developed in the laboratory of one of our scientific advisors, Thomas Kipps, M.D., Ph.D., Professor of Medicine and Evelyn and Edwin Tasch Chair in Cancer Research at UC San Diego with support from CIRM. We have an exclusive, worldwide license to develop cirmtuzumab for certain therapeutic uses from UC San Diego. Unlike antibodies that bind to other epitopes of ROR1, cirmtuzumab was not observed to bind to normal adult tissues in a Good Laboratory Practice, or GLP, tissue cross-reactivity study.

Studies in mice have shown that ROR1 expression on tumor cells accelerated the development and progression of leukemia in models of CLL, and that Wnt5a enhanced CLL cell viability, migration and proliferation in a ROR1-dependent manner. Patients with high levels of ROR1 on their CLL cells have more aggressive disease and have a significant reduction in survival. An analysis of MCL and CLL patient samples has shown that ROR1 surface expression, as well as secreted Wnt5a levels, were comparable between patients with MCL and CLL.

Preclinical studies showed that when cirmtuzumab bound to ROR1, it blocked growth factor Wnt5a signaling, inhibited tumor cell proliferation, migration and survival, and induced differentiation of CLL tumor cells. Preclinical studies with cirmtuzumab showed that treating MCL or CLL patient's tumor cells with a combination of cirmtuzumab and ibrutinib led to reduced proliferation. *In vivo* studies conducted in mouse CLL models have shown that ibrutinib and cirmtuzumab exerted antitumor activities through independent pathways; that is, inhibition of BTK by ibrutinib did not alter ROR1 signaling nor did it impair the rate at which cirmtuzumab blocked ROR1 signaling. The combination of both drugs reduced the size of the spleen, the primary site of leukemic disease in these mice, as well as the number of CLL cells in these spleens. Further preclinical studies suggested that cirmtuzumab was synergistic with venetoclax *in vitro*.

Cirmtuzumab clinical development in MCL and CLL

Cirmtuzumab Phase 1 clinical trial in patients with CLL

A Phase 1 dose escalation clinical trial of cirmtuzumab, which was funded jointly by us, CIRM, and others, was conducted in 26 patients with actively progressing CLL who had relapsed or refractory disease. Patients received four doses of cirmtuzumab administered every two weeks in cohorts of three, with each patient receiving escalating doses from 0.15 to 20 mg/kg/dose. Cirmtuzumab infusions were generally well tolerated. There were no dose-limiting toxicities, no serious adverse events, and no discontinuations related to adverse events. The most common adverse events included anemia, thrombocytopenia, and neutropenia, which were primarily attributed to the underlying CLL. Pharmacokinetic data showed a plasma half-life of approximately 32 days following four doses of cirmtuzumab at 16 mg/kg. In this clinical trial, 22 patients were evaluable for response assessment; four patients who discontinued cirmtuzumab early without meeting criteria for progressive disease were not considered evaluable. No patients met criteria for complete or partial

remission following this brief treatment. Seventeen of 22 evaluable patients had SD. Five patients had progressive disease. Most patients experienced reductions in their leukemic lymphocyte counts and were able to delay initiation of further treatments for an average of 262 days, at which point plasma levels of cirmtuzumab were undetectable. Although cirmtuzumab therapy was limited to four doses, one patient who received cirmtuzumab at 20 mg/kg had a greater than 50% reduction in lymphadenopathy. Analysis of blood samples from these patients prior to treatment showed significantly higher plasma levels of Wnt5a compared to healthy matched controls. Patients also had high levels of expression of ROR1 on their CLL cells. In addition, when compared to baseline, cells from cirmtuzumab treated patients showed a reduction in expression of a panel of genes identified as being highly correlated with stem cells and oncogenic dedifferentiation. These results were consistent with other preclinical observations that cirmtuzumab-induced ROR1 inhibition may drive cells away from a stem-cell-like profile.

Cirmtuzumab Phase 1/2 clinical trial in combination with ibrutinib in patients with MCL and CLL

We and UC San Diego, with major funding from the California Institute for Regenerative Medicine, or CIRM and a donation of ibrutinib product from Pharmacyclics LLC, are conducting a Phase 1/2 trial of cirmtuzumab in combination with ibrutinib in patients with relapsed/refractory MCL, or patients with CLL who are either relapsed/refractory or treatment-naïve (the CIRLL Study). This clinical trial was designed to evaluate the safety, pharmacokinetics, pharmacodynamics, immunogenicity, and antitumor activity of cirmtuzumab in combination with ibrutinib in adult subjects with adequate performance status and organ function. The study has three parts:

Part 1 is a Phase 1b, open-label, sequential allocation, dose-finding evaluation of the administration of cirmtuzumab monotherapy followed by cirmtuzumab/ibrutinib combination therapy in subjects with CLL or relapsed/refractory MCL;

Part 2 is an open-label dose-confirming or expansion evaluation of the concurrent administration of cirmtuzumab and ibrutinib in CLL or MCL using the recommended cirmtuzumab dose regimen derived from Part 1; and

Part 3 is a Phase 2 open-label, randomized, controlled evaluation of the clinical activity and safety of cirmtuzumab plus ibrutinib versus ibrutinib alone in patients with CLL.

We have completed enrollment of patients with CLL in Parts 1 and 2, and those patients are completing therapy or are in long-term follow-up. Following an evaluation of safety and PK data from Part 1, the recommended dose regimen, or RDR, of cirmtuzumab for Part 2 was determined to be 600 mg of cirmtuzumab administered intravenously every two weeks for three doses, followed by dosing every four weeks until disease progression or intolerance develop. This cirmtuzumab regimen was designed and chosen to be used in combination with 420 mg of ibrutinib administered once daily for patients with CLL, or 560 mg of ibrutinib once daily for patients with MCL, which are the FDA-approved doses of ibrutinib in these indications.

Cirmtuzumab CIRLL clinical trial Part 1 interim data in MCL

In June 2020, we announced an updated clinical strategy for cirmtuzumab that prioritizes development in MCL, based on encouraging interim clinical results from the CIRLL Phase 1/2 clinical trial that were presented at the ASCO 2020 annual meeting. As a result, we amended the CIRLL study to increase the number of patients with relapsed/refractory MCL to be enrolled in the Phase 2 expansion cohort to at least 20 patients and to allow the enrollment of patients with a broader range of prior Bruton’s tyrosine kinase inhibitor treatments. In addition, we limited the total enrollment of CLL patients in the randomized Part 3 of the CIRLL study to 28 patients, in order to focus resources on the MCL portion of the study. In September 2020, we met with the FDA to discuss potential accelerated approval pathways for cirmtuzumab plus ibrutinib in patients with relapsed/refractory MCL, and are in an ongoing dialogue with them.

In December 2020, we presented updated interim data from the CIRLL trial in patients with MCL and CLL at the ASH 2020 annual meeting. The combination of cirmtuzumab plus ibrutinib has been well tolerated, with adverse

events consistent with those reported for ibrutinib alone. There have been no dose-limiting toxicities and no serious adverse events attributed to cirmtuzumab alone. As of the data cut-off date of October 30, 2020, 15 patients with relapsed/refractory MCL enrolled in the dose-finding and dose-expansion cohorts of this clinical trial were evaluable for efficacy. The ORR was 87% (13 of 15 evaluable patients), improved over the 83% ORR reported at the ASCO 2020 annual meeting. The CR rate, determined by Cheson criteria, remained 57% (7 of 12 evaluable patients) for Part 1 of the study, and was 47% (7 of 15 evaluable patients) for Part 1 + Part 2, including three patients from Part 2 who had been on the study for a relatively brief time as compared to the other patients. One of the seven patients with a CR had a complete metabolic response as assessed by PET scan, with an indeterminate bone marrow biopsy on blinded review. All complete responses remained durable, ranging from 5 to 25 months as of the cutoff date, with no progressions reported after achieving a CR. Six patients (40%) achieved a PR. In addition, two patients had SD, for a total best clinical benefit rate (CR, PR and SD) of 100%. Median PFS was not reached, with the 95% confidence interval above 17.5 months, after a median follow-up of 12.1 months. Patients had received a median of two prior therapies (range 1-5) before participating in this clinical trial, with 73% of patients having received two or more prior lines of therapy. Four patients had received prior treatment with ibrutinib and all four achieved clinical responses in this clinical trial, with two CRs and two PRs. Fourteen of the 15 evaluable patients (93%) had high or intermediate MIPI-b risk score at study entry. Historical data for 370 patients with relapsed/refractory MCL from three clinical trials who had also received a median of two prior therapies (Rule et al., 2017, British Journal of Haematology), showed an ORR of 66%, CR rate of 20%, and median PFS of 12.8 months (95% confidence interval 8.5 – 16.6 months) for single agent ibrutinib in patients with MCL. Results from the ASH 2020 annual meeting poster presentation for patients with MCL treated with cirmtuzumab plus ibrutinib are shown in the two figures below.

Resp to Cirm	# Prior Tx	Prior Treatment Regimens	MCL Variant Ki67	MIPIb Score at SCR	Bulky >5cm LD	Cirm Dose	Change Tumor Size
PR	2	R-CHOP/ Lenalidomide; Rituximab/High dose Cytarabine/Lenalidomide	Ki67 20%	Int. 6.2	Yes	600 mg	-97%
PR	1	R-HyperCVAD and R-Methotrexate/Cytarabine	Blastoid Ki67 95%	High 8.1	Yes	600 mg	-64%
PR	1	Rituximab	Ki67 50%	High 6.6		600 mg	-66%
SD*	2	RCHOP/ITMTX, R-hyperCVAD	Pleo. Ki67 90%	High 7.1	(4.8cm)	8 mg/kg	-79%
SD	2	RCHOP, R-DHAP	Ki67 10%	High 6.7		16 mg/kg	-11%
CMR	2	RCHOP; R-BEAM/Auto-SCT	Ki67 10%	High 6.8		2 mg/kg	-100%
CR	2	R/Ibrutinib (10mths); R-HyperCVAD	Pleo. Ki67 70%	High 7	Yes	8 mg/kg	-97%
PR	2	R/Ibrutinib (10mths); R-HyperCVAD	Ki67 40%	High 6.6		4 mg/kg	-54%
CR	2	R/Ibrutinib (7mths); R-HyperCVAD	Nodular Ki67 30%	Int. 5.8		16 mg/kg	-100%
PR	1	R/Ibrutinib	NA	Int. 5.7 No KI-67	Yes	8 mg/kg	-91%
CR	4	R/hyperCVAD, RCHOP, RICE, BEAM/Auto-SCT	Ki67 ~30%	Int. 5.8		16 mg/kg	-95%
PR	3	Benda/R, R/AraC/Cisplt/Dex, BEAM/ Auto-SCT	Ki67 60%	High 7.8		4 mg/kg	-78%
CR	4	RCHOP/HDMTX/R, R/velcade/ITMTX, Auto-SCT, CAR-T	Nodular Ki67 ~15%	Low5.5		4 mg/kg	-100%
CR	1	RCHOP (Plus RT to orbits)	Blastoid Ki67 75%	High 7.6		2 mg/kg	-77%
CR	5	RCHOP, BEAMR/ Auto-SCT, Benda/R/Allo-SCT, ICE-R, R	Diffuse Ki67 20%	High 7	Yes	2 mg/kg	-100%

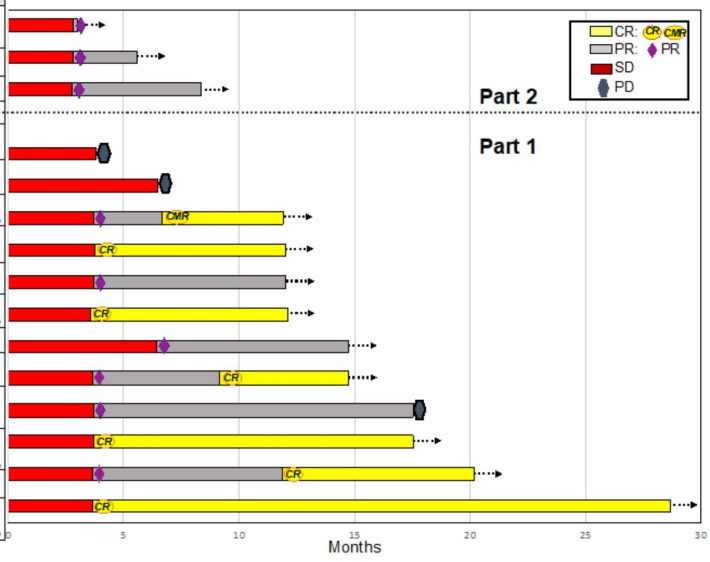

Figure 1. Individual patient response over time in the MCL cohort, based on investigator assessments, in the CIRLL clinical trial of cirmtuzumab in combination with ibrutinib as of October 30, 2020.

Note: Bars represent response status of patients on treatment; arrows indicate that a patient continues on treatment.

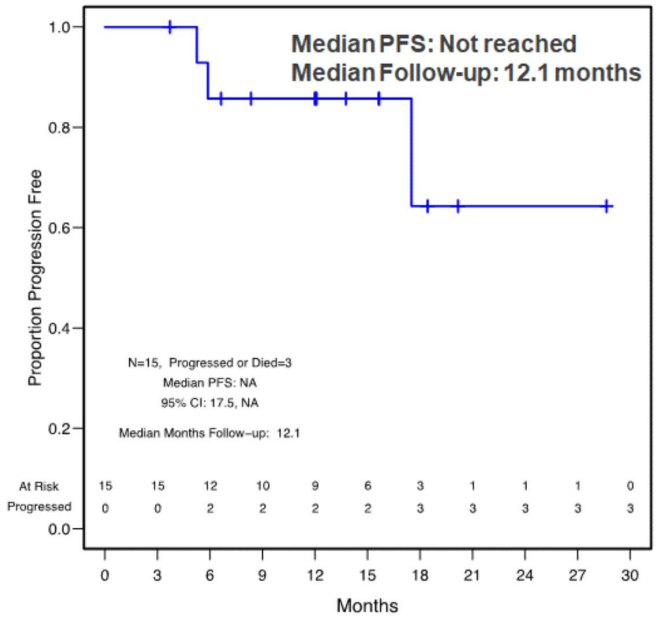

Figure 2. Progression-free survival in the MCL cohort in the CIRLL clinical trial of cirmtuzumab in combination with ibrutinib, as of October 30, 2020.

Cirmtuzumab CIRLL clinical trial interim data in CLL

The combination of cirmtuzumab plus ibrutinib has been well tolerated, with adverse events consistent with those reported for ibrutinib alone. There have been no dose-limiting toxicities and no serious adverse events attributed to cirmtuzumab alone. As of the data cut-off date of October 30, 2020, 56 evaluable patients with CLL were enrolled in the dose-finding, dose-confirming and randomized cohorts of this clinical trial, 49 of whom were treated with the combination of cirmtuzumab and ibrutinib. Forty-five of the 49 patients achieved a clinical response, for an overall best objective response rate of 92%, including one patient who achieved a CR. In addition, four patients had SD, for a total clinical benefit rate (CR, PR, and SD) of 100%. The median PFS was not reached for patients with treatment-naïve CLL (n=19) after a median follow-up of 16.6 months, and median PFS was 29.5 months for patients with relapsed/refractory CLL (n=30) after a median follow-up of 17.1 months.

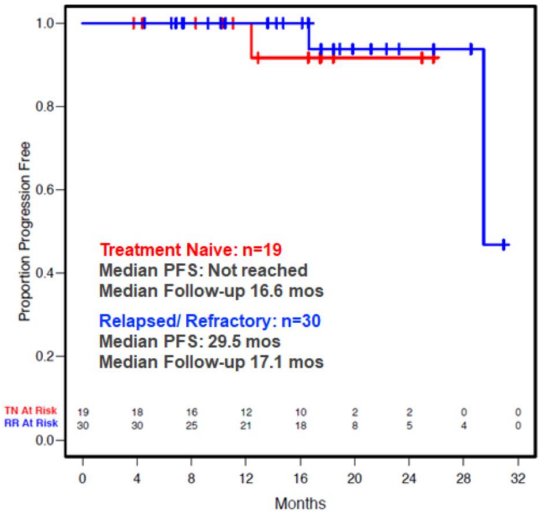

Figure 3. Progression-free survival in the CLL cohort in Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib, as of October 30, 2020.

We expect to announce a data update from this Phase 1/2 clinical trial in the second quarter of 2021.

Cirmtuzumab development in breast cancer

Breast cancer disease overview

Breast cancer is the most common type of invasive cancer among women and the second leading cause of cancer deaths among women. There are approximately 266,000 new diagnoses and 41,000 breast cancer deaths in the U.S. each year, and 12.4% of women will develop breast cancer in their lifetime. The Centers for Disease Control and Prevention, or CDC, estimates that there are approximately one million women in the U.S. living with breast cancer that has been diagnosed within the past five years.

Breast cancers can be segregated into subtypes based upon the presence of three protein receptors: estrogen receptor, or ER, progesterone receptor and human epidermal growth factor receptor 2, or HER2. Therapies have been developed that target tumors containing one or more of these receptors. Approximately 10% to 15% of breast cancers, however, do not express any of these three receptors and are referred to as triple-negative breast cancers, or TNBC. These tumors have a more aggressive phenotype and a poorer prognosis due to the high propensity for metastatic progression and absence of specific targeted treatments. The five-year survival rate for patients with breast cancer other than TNBC has been reported to be 80.8%, but only 62.1% for patients with TNBC. One hypothesis for the high rate of metastasis and poor response to chemotherapy for patients with TNBC is that these tumors contain a high number of tumor-initiating cells, or cancer stem cells, that are highly migratory and insensitive to standard chemotherapy. Treatment options for TNBC are limited and include chemotherapy, targeted therapy (such as PARP inhibitors), surgery, radiation, and immunotherapy. For Her2-negative aggressive breast cancers, an unmet medical need exists for new breast cancer therapies that can target rapidly proliferating cancer cells and target cancer stem cells, with minimal damage to normal tissues.

ROR1 Expression and Historical Clinical Outcomes in Patients with Breast Cancer

Approximately 75% of breast tumors have been shown to express ROR1. In a retrospective analysis, patients with TNBC with high levels of ROR1 were found to have a significantly reduced disease-free survival (p< 0.00015) as well as OS (p<0.026) compared to patients with low ROR1 levels.

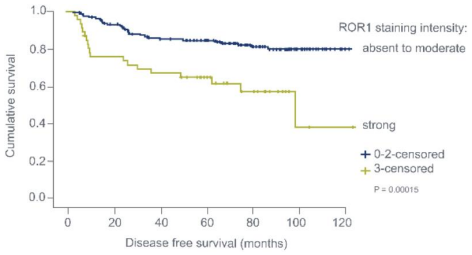

Figure 4. TNBC patients with high levels of ROR1 expression had lower disease-free survival.

Another retrospective, long-term analysis that included all breast cancer types showed that patients with tumors expressing high levels of ROR1 were at a statistically significantly higher risk of developing metastases within the first several years. Over 60% of patients with high ROR1 developed metastases, compared to only 35% of patients with the lowest levels of ROR1.

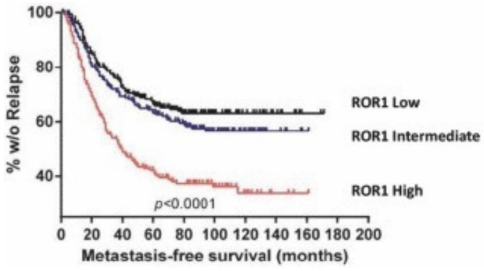

Figure 5. High levels of ROR1 in breast cancer were associated with shorter metastasis-free survival.

Preclinical experiments have shown that treatment of breast tumors with paclitaxel increased the percentage of cells with high levels of ROR1. In these experiments, immunodeficient mice were implanted with patient-derived xenografts, or PDX, then were treated with paclitaxel. While paclitaxel either slowed tumor growth or reduced the size of tumors in these mice, the surviving cells were enriched for expression of ROR1. This increased expression of ROR1 was also associated with a shift in the properties of cells from these

tumors towards a more metastatic and more tumorigenic phenotype. Cells from tumors that had been treated with paclitaxel were more likely to form spheroids in tissue culture and were enriched for cells with the ability to form new tumors when transplanted, both properties that are correlated with tumor aggressiveness.

In a preclinical model, cirmtuzumab reduced the growth rate of primary human breast cancers in immunodeficient mice and led to complete suppression of tumor growth for twenty days when used in combination with paclitaxel. Even after tumors did eventually grow, they lacked the ability to form new tumors. All tumor samples isolated from control mice, most of the tumor samples from paclitaxel-treated mice, and some of the cirmtuzumab-treated mice were able to establish new tumors when transplanted into other mice. No tumors, however, were formed when equal numbers of tumor cells from mice treated with the combination of cirmtuzumab and paclitaxel were introduced into other mice.

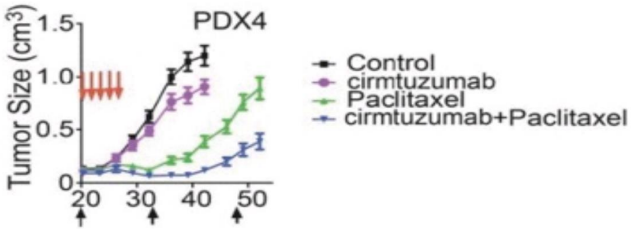

Figure 6. Combination of cirmtuzumab and paclitaxel suppressed growth of primary human breast tumors in a mouse model.

Together, these clinical and preclinical data are consistent with a model of the natural disease progression in breast cancer centered on the critical role played by tumor-initiating cells or stem-like cancer cells that express high levels of ROR1:

- TNBC is initially responsive to chemotherapy such as paclitaxel, because chemotherapy kills the majority of cancer cells, leaving cells with stem-like properties that express ROR1;
- TNBC recurs more often than other types of breast cancer in part because the initial chemotherapy enriches for cells with a higher propensity to form tumors;
- The site of recurrence is often at site in the body remote from the original tumor because cells with stem cell-like properties are able to metastasize; and
- The recurring tumor may be resistant to therapy because it contains a high percentage of cells with stem cell-like properties.

Cirmtuzumab clinical development in breast cancer

An investigator-sponsored single-arm, open-label, Phase 1b trial of cirmtuzumab in combination with paclitaxel in patients with locally advanced, unresectable or metastatic HER2-negative breast cancer is underway at UC San Diego. The objectives of the trial include the evaluation of safety, tolerability, pharmacokinetics, and clinical activity of cirmtuzumab plus paclitaxel. The treatment regimen is cirmtuzumab at a dose of 600 mg on days 1 and 15 of cycle 1, and then on day 1 of each subsequent 28-day cycle, and paclitaxel weekly at a dose of 80 mg/m. In December 2019, an interim clinical data update for this clinical trial was presented by the investigator at the 2019 San Antonio Breast Cancer Symposium. As of the data cut-off date of November 27, 2019, a total of eight patients with HER2-negative, metastatic or locally advanced unresectable breast cancer was enrolled in the study. Seven of the eight patients were evaluable for safety and efficacy. Four of the patients had TNBC at study enrollment. Four of the seven evaluable patients achieved a PR, for an ORR of 57%, including one patient who had a PR that continued on cirmtuzumab alone for 30 weeks after discontinuing paclitaxel. It was reported that the combination of cirmtuzumab and paclitaxel was well tolerated in this trial, with no study discontinuations for toxicity and no dose-limiting toxicities observed

as of the cutoff date. Adverse events were consistent with the known safety profile of paclitaxel alone. Pharmacokinetic analysis of serial plasma samples for free unbound antibody from two patients provided results similar to those observed in previous studies of patients with CLL, consistent with a projected half-life of 30 days. No abrupt decline in antibody concentration over time was observed, consistent with the absence of anti-drug or neutralizing antibodies. In the second quarter of 2021, we expect to announce additional interim data from this study of patients with HER2-negative, metastatic or locally advanced unresectable breast cancer.

Potential additional clinical opportunities for cirmtuzumab in solid tumors

Lung cancer. ROR1 is expressed by approximately 77% to 93% of lung cancers. In adenocarcinoma of the lung, higher levels of ROR1 expression were correlated with advanced stages of disease and with positive lymph node metastases. In addition, Kaplan-Meier survival analysis indicated an association of high ROR1 expression with worse OS in lung adenocarcinoma patients. ROR1 expression has been shown to be correlated with the presence of other negative prognostic factors such as phosphorylated AKT, or p-AKT, or phosphorylated CREB, or p-CREB. Inhibition of ROR1 in lung cancer cell lines induced apoptosis and cell cycle arrest and led to a reduction in levels of p-CREB and p-AKT. Notably, a recent preclinical study has shown that downregulating ROR1 expression re-sensitizes erlotinib-resistant lung cancer cells to an EGFR inhibitor drug.

Ovarian cancer. ROR1 is expressed by approximately 54% of ovarian cancers, which is the most lethal gynecologic malignancy among women worldwide. Analysis of ROR1 expression on ovarian cancer patient samples revealed that disease-free survival and OS rate in patients with high ROR1 expression were significantly lower than in patients with low or no ROR1 expression. In a preclinical study, it was shown that a ROR1 antibody inhibited growth of ovarian cancer cell lines in vitro and slowed tumor growth in a mouse model.

Prostate cancer. ROR1 is expressed by approximately 90% of prostate cancers, and the Wnt5a signaling pathway is activated in patients with advanced prostate cancer that is progressing while on treatment with an androgen receptor, or AR, inhibitor. Treatment of prostate cancer cell lines with an AR inhibitor was found to increase the expression of Wnt5a, and the addition of Wnt5a attenuated the antiproliferative effect of AR inhibition. The expression of Wnt5a in patients with metastatic castrate resistant prostate cancer, or mCRPC, has been associated with poor OS. We are collaborating with academic investigators to investigate the potential effects of cirmtuzumab on this disease.

Pancreatic cancer. ROR1 is expressed by approximately 83% of pancreatic cancers. A recent preclinical study has shown that blocking ROR1 led to apoptotic cell death, which was further enhanced in combination with chemotherapeutic drugs such as erlotinib and ibrutinib, when tested against a panel of pancreatic cancer cell lines.

We are undertaking further preclinical studies with cirmtuzumab in several cancer types, and expect to identify one or more indication for potential clinical development in the second quarter of 2021.

ROR1 CAR-T Cell Therapy Program

We are developing CAR-T cell therapy candidates based on the ROR1 binding domain of cirmtuzumab to treat patients with aggressive hematological malignancies or solid tumors. We believe that the selective expression of ROR1 on tumor cells and its absence on normal cells make it an ideal target for a CAR-T cell therapy approach. In addition, we believe that ROR1-negative relapses might be less likely to develop after ROR1 CAR-T therapy, because the survival benefit imparted on cancer cells by expressing ROR1 may limit the development of ROR1-negative tumors, such that any tumor cells that lose or mutate ROR1 to escape CAR-T treatment may be less aggressive than the original cells. Our ROR1 targeted CAR-T cell therapy candidate is in preclinical development.

We are pursuing a two-pronged development strategy for our ROR1 CAR-T cell therapy program. The first part of the strategy is to demonstrate evidence of safety and activity of a ROR1 CAR-T cell therapy in humans - while seeking to reduce the development risks by using an autologous CAR-T approach and selecting a hematological indication that is known to be susceptible to CAR-T cell therapy. The second part of the strategy would be to develop next-generation cell therapies targeting ROR1 by introducing more advanced cell therapy technologies, which could include CAR-T bearing additional features to overcome the solid tumor microenvironment, or “off-the-shelf” allogeneic CAR-T and/or CAR-NK (Natural Killer) therapies.

We expect partnerships and collaborations to be essential for implementing our strategy. In January 2021, we announced a research and development collaboration with Karolinska Institutet to advance novel ROR1-targeting cell therapies focused on CAR-T cells and CAR-NK cells from the laboratory into the clinic. In addition, in January 2021, we announced an agreement with Lentigen, a wholly-owned subsidiary of Miltenyi Biotec B.V. & Co. KG, to manufacture lentiviral vectors for our investigational ROR1-targeting CAR-T cell therapy program.

We are also collaborating with SPH for our CAR-T cell therapy program, through its U.S. subsidiary Shanghai Pharmaceutical (USA) Inc., or SPH USA. SPH USA has entered into the SPH USA License Agreement with us to develop ROR1 targeted CAR-T cell therapy product candidates in greater China. We and SPH USA also intend to collaborate by conducting one or more initial clinical trials of our CAR-T cell therapy candidate at hospitals in China that have experience with processing cellular immunotherapy materials and conducting CAR-T clinical trials. In the second half of 2021, we expect to announce first-in-human dosing of our ROR1 CAR-T therapy candidate in China by our partner SPH USA. In parallel, we are continuing preclinical development activities in the U.S. and Europe.

Scientific background: CAR-T cell therapy overview

Immuno-oncology approaches to treating cancer involve redirecting one of the pillars of the immune system, the adaptive immune system, so that it specifically and efficaciously recognizes cancerous cells that might previously have escaped immune recognition. A key element in the adaptive immune response is the T cell. T cells are white blood cells that can recognize and kill infected and abnormal cells. T cells also act to signal other immune cells to respond to threats. T cells recognize their targets because they are created in a way that allows them to specifically recognize foreign antigens on the surface of other cells.

T cells are ideally suited for immuno-oncology applications based on several characteristics. They are created to be exquisitely specific and avid killers. One T cell can eliminate numerous target cells. T cells are extremely specific, able to recognize a cancer cell and kill it, while ignoring an almost identical healthy cell. T cells are thought to be active all the time, eliminating cancer cells from the body before they can form tumors. However, tumor cells sometimes evolve to escape killing by T cells by activating a number of pathways that suppress T cell function. Adoptive T cell therapies, and specifically CAR-T cells, were developed to provide a method to generate large quantities of T cells capable of specifically recognizing and killing tumor cells despite tumor suppressive mechanisms.

CAR-T cell therapeutics are created by isolating T cells from patients and modifying them to recognize specific antigens on tumors. T cells have potent cell killing activity that is directed to target cells that are recognized by specific T cell receptors, or TCRs, that are expressed on the surface of these T cells. While some T cells have TCRs that can recognize cancer cells leading to their killing, potent T cells do not develop to all targets. In some cases, the potential cancer cell target is also a protein that has an essential role in other tissues or at other stages of development, and TCRs that recognize these targets are eliminated during normal T cell development.

CAR-T cell therapy has emerged as a way to engineer T cells to recognize specific targets, such as those that are selectively expressed on cancer cells. A gene encoding a chimeric protein is constructed that contains a single antigen-binding domain of an antibody that recognizes the target, which is coupled to a T cell costimulatory domain and a portion of the T cell receptor.

CAR-T cell therapies are typically produced from a patient's own T cells which are isolated by leukapheresis. These cells are then genetically modified with the chimeric antigen gene construct which can be delivered by various mechanisms such as lentiviral gene delivery vectors. Transduced cells are then expanded and undergo quality testing before being reintroduced into the same patient.

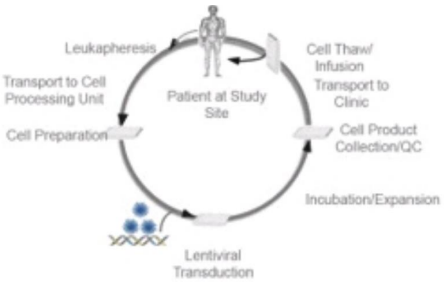

Figure 7. CAR-T production and patient treatment.

TK216 - ETS oncoprotein inhibitor

TK216 is an investigational, potentially first-in-class, targeted small molecule that was designed to specifically inhibit the biological activity of the ETS family of oncoproteins. Tumorigenic gene fusions involving ETS factors are frequently found in tumors such as Ewing sarcoma and prostate cancer, and ETS factors are often overexpressed in other tumors, such as AML and DLBCL. Researchers in the laboratory of one of our scientific advisors, Jeffrey Toretsky, M.D. of Georgetown Lombardi Comprehensive Cancer Center, identified the precursor to TK216 by using a novel chemical screening assay that they developed based on a deep understanding of the underlying biological mechanism of ETS factors. Following this early work, TK216, which is designed to be a specific, high-affinity inhibitor of ETS factors, was created by us through the rational design and screening of novel small molecule inhibitors of a critical protein-protein interaction. In preclinical models, TK216 has inhibited the interaction between ETS family members and RNA helicase A, or RHA, and by doing so, shut down excessive cell proliferation.

We are evaluating TK216 as a single agent and in combination with vincristine in a Phase 1 clinical trial in patients with relapsed or refractory Ewing sarcoma. The dose-finding portion of the study was completed in 2019, and we are currently enrolling patients in an expansion cohort to evaluate the clinical response of treatment with TK216 in combination with vincristine using the RP2D regimen. Ewing sarcoma is a rare pediatric cancer that has historically been very challenging to treat effectively, particularly for recurrent and metastatic disease. ETS fusion proteins have been shown to be present in over 90% of Ewing sarcoma cases. TK216 has received an Orphan Drug Designation and Fast Track Designation from the FDA for the treatment of patients with relapsed or refractory Ewing sarcoma.

TK216 scientific background: ETS transcription factors and oncogenesis

TK216 targets the ETS family of oncoproteins known to be associated with both solid tumors and hematological malignancies. In normal development and physiology, ETS transcription factors govern processes such as cell cycle control, differentiation, proliferation, apoptosis, tissue remodeling and angiogenesis. However, when alterations in the functions of ETS factors develop, through overexpression, gene fusion or modulation, they have been shown to lead to tumor initiation, progression, and metastasis. Fusion proteins are a well-known category of targets for small molecule cancer therapy that have been cited in the scientific literature as providing a number of diagnostic and therapeutic advantages because of their tumor-specific expression. ETS overexpression or fusion proteins incorporating an ETS factor have been observed in multiple tumor types:

Ewing sarcoma*	98%
Prostate cancer*	55%
Diffuse Large B Cell-Lymphoma	52%
Head & Neck cancer	33%
Acute Myeloid Leukemia*	30%
Breast cancer*	25%
Melanoma	25%
Ovarian cancer	23%
Lung cancer	21%
Glioblastoma multiforme	15%

* Fusion identified

Fusion proteins involving ETS factors have been implicated in various solid tumors, including Ewing sarcoma and prostate cancer. For example, approximately 85% of Ewing sarcomas contain a genomic rearrangement between chromosomes 11 and 22. DNA is exchanged between these chromosomes in a pathological manner, and this exchange results in a fusion of two genes: the *FLI1* gene, an ETS family member, and the *EWSR1* gene, an unrelated transcription factor. This gene fusion, known as *EWS/FLI1*, functions as a transcription activator that is no longer controlled by the relevant regulatory machinery in the cell. In addition to escaping regulation, the dysregulated function of the resultant EWS/FLI1 fusion protein causes a series of abnormalities in RNA processing including aberrant mRNA expression and splicing, where it leads to defects in the synthesis of proteins such as BRCA1, a DNA repair protein. EWS/FLI1 fusion proteins also cause the formation of abnormal and potentially deleterious DNA and RNA structures known as R-loops that are associated with replication and transcriptional blocks as well as being prone to increased DNA damage.

Multiple other tumors contain gene fusions of other ETS factors. For example, over 50% of metastatic prostate cancers carry a *TMPRSS2-ETS* gene fusion. Other tumors have genetic changes that result in overexpression of ETS factors.

ETS Fusions	ETS Overexpression
• Ewing sarcoma	• AML
◦ EWS-FLI1	◦ FLI1, ERG, ETV5, ETS2
• Prostate cancer	• DLBCL
◦ TMPRSS2-ERG	◦ ETV1, FLI1, ETV4, SPIB
• AML	• Prostate cancer
◦ ETV6-various (20+)	◦ ERG, ETV1, ETV4, ETV6
• ALL	• Lung cancer
◦ ETV6-RUNX1	◦ ETV5, ETV1, FLI1, ETS1
• Secretory breast cancer	• Breast cancer
◦ ETV6-NTRK3	◦ ETV6, ETV4, SPIB, ETV5

Despite the genetic associations between ETS factors and tumorigenesis and the reported correlation between high levels of ETS factor expression and survival, there are currently no approved therapeutics available that target these factors. It had been widely considered that transcription factors are difficult to target due to their non-enzymatic mechanism of action. Researchers in the laboratory of Jeffrey Toretsky, M.D., Professor at Georgetown University, identified the precursor to TK216 by using a chemical screening assay that they developed based on a deep understanding of the underlying biological mechanism of ETS factors. TK216 has been observed to inhibit the interaction between ETS family members and RNA helicase A, or RHA, a critical component of the human transcriptional complex, and by doing so, shuts down excessive cell proliferation in preclinical tumor models. We believe that our approach of inhibiting protein-protein interactions is novel and that our product candidate TK216 targeting ETS factors could fill an important gap in the treatment landscape for both solid tumors and hematological malignancies.

TK216 development in Ewing sarcoma

Ewing sarcoma disease overview

Ewing sarcoma is the second most common bone tumor of children, and it occurs most often in adolescents, accounting for approximately 2% of all childhood cancer diagnoses. The incidence of Ewing sarcoma for all ages is approximately 1.3 cases per 1 million people in the U.S., corresponding to approximately 430 new patients diagnosed per year in the U.S. The median age at diagnosis of patients with Ewing sarcoma is 15.

Nearly all Ewing sarcoma cases are driven by translocations of *ETS* family oncogenes, including 85-90% of cases driven by the *EWS-FLI1* fusion, and approximately 10% by *EWS-ERG*.

Ewing sarcoma typically develops in the pelvis, femur, and bones of the head and trunk, but its diagnosis often takes months as other causes for non-specific symptoms such as localized pain, fever, fatigue, weight loss, or anemia are ruled out. The five-year survival of patients who are diagnosed with non-metastatic disease is between 50% and 70%. Patients diagnosed with metastatic disease have five-year survival between 18% and 30%. The prognosis for patients with recurrent Ewing sarcoma is particularly poor, and five-year survival after recurrence is approximately 10 to 15%.

Ewing sarcoma is usually treated systemically due to the fact that local treatments, even in patients without overt metastases, have an 80% to 90% relapse rate. The current standard therapy for patients with localized Ewing sarcoma in the U.S. is a combination of chemotherapy agents, including vincristine, doxorubicin and cyclophosphamide, with alternating cycles of ifosfamide and etoposide – a therapy known as VDC/IE. Patients that respond to this therapy may be candidates for tumor resection and continued treatment for a total of 14 to 17 cycles. This therapeutic regimen, however, is associated with significant toxicities. Patients with metastatic disease are often treated with VDC/IE or variations of this therapy with higher or more compressed dosing. This may also be supplemented by local radiation therapy or systemic radiation followed by autologous hematopoietic stem cell transplant. We believe that more effective therapies are needed for this rare and severe pediatric disease.

TK216 preclinical data in Ewing sarcoma

TK216 was the product of a novel approach based on developing small molecule inhibitors of a critical protein-protein interaction linked to the ETS family of transcription factors. Researchers at Georgetown University identified YK-4-279, the precursor to TK216, by using a novel chemical screening assay. Following this early work, TK216, a specific inhibitor of ETS factors, was then created by Oncternal through the rational design and screening of novel small molecule inhibitors of a critical protein-protein interaction linked to the ETS family of transcription factors. TK216 is a structural analog of YK-4-279 that has shown increased potency in biochemical, cellular and xenograft tumor models.

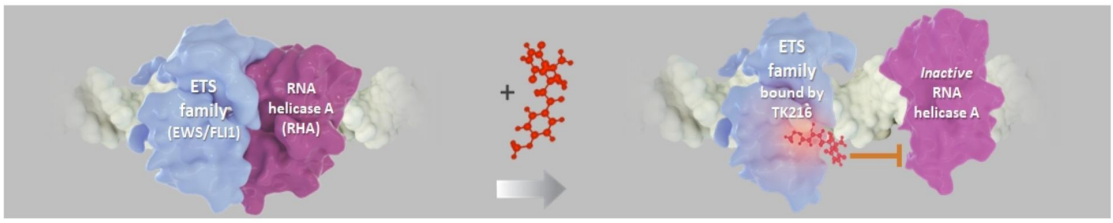

Figure 8. TK216 inhibits interaction of ETS fusion protein EWS/FLI1 with RNA helicase A.

In Ewing sarcoma, a key heterodimer between EWS/FLI1 and RHA forms the core of a transcriptome complex causing activated oncogenes, inhibited tumor suppressors, abnormal RNA transcription and abnormal RNA splicing. TK216 was developed to disrupt that heterodimer, thereby potentially preventing transcription and leading to inhibition of the oncogenic activity of EWS/FLI1, by decreasing oncogene expression, increasing tumor suppressor function, and apoptotic cell death. In preclinical models, TK216 inhibited the interaction between ETS family members and RNA helicase A or RHA, and by doing so, shut down excessive cell proliferation and caused apoptotic cell death.

TK216 clinical development in Ewing sarcoma

We are evaluating TK216, as a single agent and in combination with vincristine, in an open-label, multicenter Phase 1/2 clinical trial in patients with relapsed or refractory Ewing sarcoma. The dose-finding portion of the study was completed in 2019, and we continue to enroll patients in the Phase 2 expansion cohort to evaluate the clinical response of treatment with TK216 in combination with vincristine using the recommended Phase 2 dosing, or RP2D, regimen. The RP2D has been established to be 200 mg/m2/day of TK216 for 14 days, with vincristine dosed at 0.75 mg/m2 on the first day of each treatment cycle. In November 2020 at the Connective Tissue Oncology, or CTOS meeting, we announced updated interim clinical data from our ongoing open-label, multicenter Phase 1 clinical trial evaluating TK216 in patients with relapsed or refractory Ewing sarcoma. The objectives of the clinical trial include the evaluation of safety, tolerability, pharmacokinetics, and tumor response. Patients entering the trial had previously been treated with a median of three, and as many as eight prior lines of systemic therapy. The presentation included interim data for 50 evaluable patients, including 23 evaluable patients treated at the RP2D as of the October 16, 2020 efficacy cut-off date. Two of the 23 patients treated at the RP2D (9%) achieved a CR, including one surgical CR. Both patients achieving CRs remain on treatment, with no evidence of disease. The first patient with a CR was on treatment in this clinical trial for over 1.5 years and the second patient with a CR was on study for over 8 months as of the cutoff date. The best ORR was 9%. Eight additional patients treated at the RP2D had SD, for a disease control rate (CR, PR or SD) of 43%. The median progression-free survival for patients treated at the RP2D was 1.8 months, with some patients enjoying extended PFS.

	Evaluable Patients N= 50	ORR	CR	PR	SD	Disease Control Rate CR+PR+SD
Dose Escalation Cohorts 1-6	21	0	0	0	1	4.8%
Schedule Escalation Cohorts 7-8	6	0	0	0	0	0
RP2D Cohort 9 & Expansion	**23**	**2 (9%)**	**2 (9%)**	**0**	**8 (35%)**	**43%**

Figure 9. Overall best clinical response in Phase 1/2 clinical trial of TK216 in patients with relapsed/refractory Ewing sarcoma, as of October 16, 2020.

Note: Patients were considered evaluable for efficacy if they completed 2 planned cycles of treatment and follow-up tumor assessment studies or had documented or clinical PD following a complete first cycle of therapy.

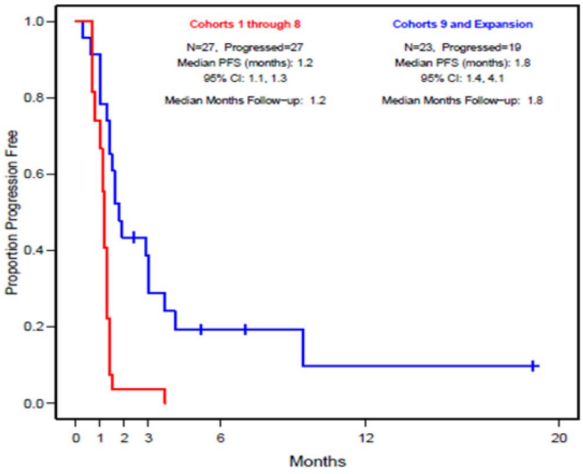

Figure 10. Progression-free survival in Phase 1/2 clinical trial of TK216 in patients with relapsed/refractory Ewing sarcoma, as of October 16, 2020.

TK216 was generally well tolerated. As of the October 2, 2020 safety cutoff date, the most common drug-related adverse events included myelosuppression, fatigue, alopecia, nausea, pyrexia, and decreased appetite. Dose limiting toxicities consisted of transient and manageable myelosuppression, primarily neutropenia. No unexpected off-target toxicities were observed.

Pharmacokinetic data from the clinical trial showed that TK216 drug levels at the RP2D exceeded plasma levels associated with anti-tumor activity in preclinical models.

In the second quarter of 2021, we expect to announce additional interim clinical data from this Phase 1/2 clinical trial in patients with Ewing sarcoma.

Potential additional clinical opportunities for TK216

Acute myeloid leukemia (AML). AML is a hematologic malignancy characterized by dysregulated maturation of myeloid or blood stem cells and failure of the bone marrow to properly function, leaving patients with anemia and immune deficiency, and at high risk of infections and bleeding. AML is the most common type of acute leukemia in adults. Approximately 21,450 new AML cases and 10,920 AML associated deaths occur annually in the U.S. The average age of an AML patient is 68 years. The National Cancer Institute estimated in 2018 that the five-year survival rate for adult patients with AML was approximately 27%. We believe that there is a need for more effective and less toxic therapies for AML.

ETS overexpression or fusion proteins incorporating ETS family member have been observed in about 30% of AML cases. The ETS family member ERG is overexpressed in many cancers, such as AML. In a retrospective analysis of patients with AML, the quartile of patients with the highest levels of ERG expression had a significantly higher rate of relapse and poorer OS than patients with lower levels of ERG expression. Those with the highest levels of ERG had a five-year survival rate of 20%, while those with lower levels of ERG had a survival rate of approximately 50%. ERG overexpression was an independent negative prognostic factor. Similarly, AML patients with high levels of ETS2, another ETS family member, had a significantly lower five-year survival rate of approximately 15% compared to 40% for patients with lower levels of ETS2. ETS2 overexpression was an independent negative prognostic factor.

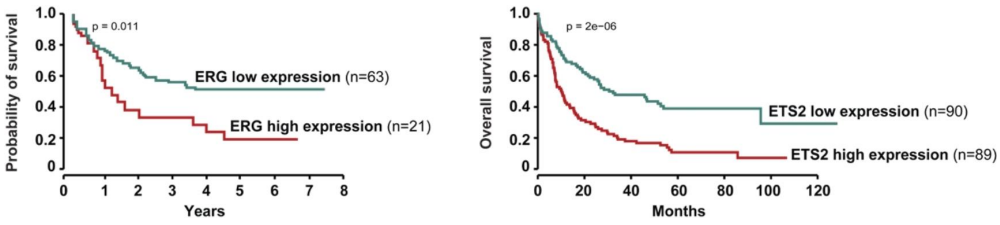

Figure 11. Survival of the quartile of AML patients with the highest ERG (left) or ETS2 (right) expression was significantly lower than those with lower expression.

Multiple AML cell lines have been shown to be sensitive to being killed by TK216, with sensitivity proportional to ETS expression. TK216 may provide a novel therapeutic strategy for the treatment of patients with relapsed and refractory AML, a patient population known to express, in certain cases, fusion proteins involving ETV6, and to have overexpression of ETS family members including FLI1, ERG, ETS2, and ETV5.

Prostate cancer. Approximately 174,650 new cases of prostate cancer are diagnosed annually in the U.S. The incidence of metastatic prostate cancer is increasing, causing an estimated 31,620 deaths per year in the U.S. New therapeutic options are needed after failure of androgen antagonism and prior to chemotherapy. Approximately 55% of men with advanced prostate cancer carry the ETS family fusion gene *TMPRSS2-ERG* that is related to androgen resistance.

We believe TK216 may provide a novel therapeutic strategy for the treatment of patients with advanced prostate cancer, in particular those who carry the ETS family fusion gene *TMPRSS2-ERG*. In a preclinical in vivo study, YK-4-279, which is an analog of TK216, showed anti-tumor activity against a prostate cancer cell line harboring the ETS-family translocation, while growth of a prostate cancer cell without the translocation was not inhibited.

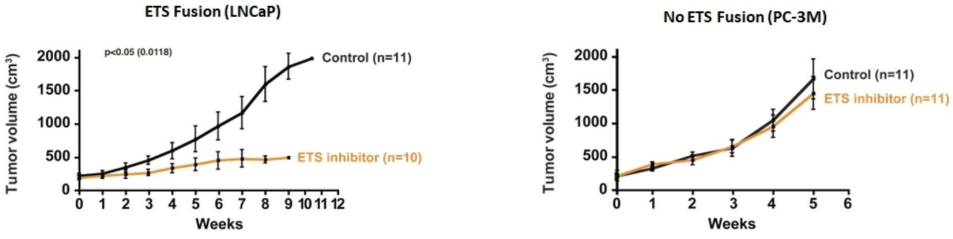

Figure 12. Prostate cancer sensitivity was associated with an ETS-family fusion protein in human prostate cancer xenograft models.

In the second half of 2021, we expect to announce additional preclinical data for TK216 in ETS-driven tumors.

SARD and SARM Technologies

Prior to the Merger as defined below, GTx had been developing selective androgen receptor modulators, or SARMs, under an exclusive, worldwide license agreement with the University of Tennessee Research Foundation, or UTRF. GTx's SARM product candidate, enobosarm (GTx-024), was evaluated in post-menopausal women with stress urinary incontinence, or SUI in a Phase 2 clinical study. During 2018, GTx announced that the study failed to achieve statistical significance on its primary endpoint and determined that there was not a sufficient path forward and discontinued the further development of enobosarm. Effective in March 2020, we terminated the SARM License Agreement with UTRF.

Also, under an exclusive worldwide license agreement with UTRF, GTx had been developing UTRF's proprietary selective androgen receptor degrader, or SARD, technology, to provide compounds to degrade or antagonize multiple forms of androgen receptor, thereby potentially inhibiting tumor growth in patients with progressive castration-resistant prostate cancer, including those patients who do not respond to or are resistant to current androgen targeted therapies. We are performing additional mechanistic preclinical studies in order to determine if one or more of these SARD compounds should be advanced into the additional preclinical studies required to submit an investigational new drug application, and whether we should advance one of the SARD compounds into a first-in-human clinical trial.

Competition

The biotechnology and pharmaceutical industries are intensely competitive and characterized by rapid technology evolution. Our potential competitors include large pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as government, academic and other research institutions. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, as well as in acquiring technologies complementary to our programs. Our commercial opportunities may be reduced or eliminated if our competitors develop and commercialize similar products that are safer, more effective, have fewer side effects or are less expensive than any products that we or our collaborators may develop.

In particular, we compete with other companies that are developing and commercializing treatments for patients with cancer. Competing therapies include chemotherapies, targeted therapies and immunotherapies and may represent various therapeutic modalities including small molecules, antibodies, cell therapies, gene therapies, and cancer vaccines. These companies may compete with us for clinical trial sites and eligible patient populations, scientific and management talent, outsourced manufacturing capacity and healthcare budgets for commercial-stage products.

Cirmtuzumab competition

While there are several therapeutic options available to treat patients with relapsed or refractory MCL, none of these options offers long-term benefit, with most patients relapsing in less than 20 months. In an open-label Phase 2 clinical trial, ibrutinib (Imbruvica®), a BTK inhibitor that is approved by the FDA for the treatment of patients with relapsed MCL, demonstrated an ORR of 67% and a CR rate of 23%, with a median duration of response, or DoR, of 17.5 months, median PFS of 13 months and median OS of 22.5 months. In an open-label Phase 2 clinical trial, acalabrutinib (Calquence®), another BTK inhibitor approved by the FDA for the treatment of patients with relapsed MCL, demonstrated an ORR of 80% and CR rate of 40%. Another BTK inhibitor approved in 2019, zanubrutinib (Brukinsa®), demonstrated an ORR of 84% and CR rate of 59%, with a median DoR of 19.5 months in an open-label Phase 2 clinical trial. These therapies are given continuously for prolonged periods of time, and their use can be associated with significant toxicity. The majority of patients

with MCL are older, and remissions are not durable for most patients treated with continuous ibrutinib therapy. As a result, we believe that more effective and better tolerated therapies with shorter treatment periods represent a significant unmet need.

Significant progress has been made in the treatment of CLL since the advent of targeted therapies and FDA approval of ibrutinib for CLL in 2014. A treatment paradigm shift has taken place, from chemotherapies to targeted therapies. Three classes of targeted therapies have been approved for the treatment of patients with CLL: inhibitors of BTK, a key component of cell signaling in B-cells, such as ibrutinib, which is marketed as Imbruvica® by AbbVie, Inc., and Johnson & Johnson, and acalabrutinib, which is marketed as Calquence® by AstraZeneca PLC; inhibitors of the protein B-cell lymphoma-2, or Bcl-2, such as venetoclax, which is marketed as Venclexta® and Venclyxto® by AbbVie, Inc., and Roche/Genentech; and inhibitors of Phosphoinositide 3-kinase, or PI3K, which include idelalisib, which is marketed as Zydelig® by Gilead Sciences, Inc., and duvelisib, which is marketed as Copiktra® by Verastem, Inc. These targeted therapies are now the core of the recommended treatment regimens for patients with both first-line and relapsed or refractory CLL, and have achieved objective response rates of 85-90%, two-year PFS of 65-90%, and two-year overall survival of 75-95%. The outcomes are worse for patients with certain prognostic factors, such as 17p or 11q chromosome deletions; for such patients with relapsed or refractory CLL treated with ibrutinib, the reported PFS is 50-75%.

While there are currently no approved products targeting the ROR1 receptor, we are aware of therapeutics in clinical development that target ROR1, including an ADC being developed by VelosBio, Inc. (acquired by Merck in 2020), an ADC being developed by NBE-Therapeutics (acquired by Boehringer Ingelheim in 2020), and a ROR1 CAR-T therapy being developed by Juno Therapeutics, Inc., a subsidiary of the Bristol-Myers Squibb Company. VLS-101, the ADC being tested clinically by VelosBio was originally designed and had its early development at Oncternal, it binds to the same epitope on ROR1, and it utilizes cirmtuzumab to target ROR1.

There are numerous companies developing or marketing treatments for the same oncology indications that we are targeting with our cirmtuzumab program. Therapies approved or in clinical development for the treatment of patients with treatment-naïve or relapsed/refractory CLL and relapsed/refractory MCL include BTK inhibitors, Bcl-2 inhibitors, PI3K inhibitors, anti-CD20 antibodies, and cell therapies that are being marketed or developed by companies including AbbVie, Inc., AstraZeneca PLC, BeiGene, Ltd., Eli Lilly and Company, Gilead Sciences, Inc., Johnson & Johnson, MEI Pharma, Merck, Novartis Pharmaceuticals Corporation, Roche Holding AG's Genentech subsidiary, TG Therapeutics, Inc., and Verastem, Inc.

ROR1 CAR-T competition

While there are currently no approved cell therapy products targeting the ROR1 receptor, we are aware of an autologous CAR-T cell therapy that targets ROR1 in clinical development for solid and liquid tumors by the Fred Hutchinson Cancer Research Center and Bristol-Myers Squibb.

There are numerous companies developing or marketing cell therapy treatments for the same oncology indications that we may target with our ROR1 CAR-T program including AbbVie, Inc., Adicet, Allogene Therapeutics, Atara Biotherapeutics, Inc., Bluebird Bio, Inc., Bristol-Myers Squibb, Caribou Therapeutics, Fate Therapeutics, Gilead Sciences, Inc., Johnson & Johnson, Legend Biotech, Merck, NantKwest, Nkarta Therapeutics, Novartis Pharmaceuticals Corporation, Poseida Therapeutics, Roche Holding AG, and others. Four CAR-T cell therapies, Yescarta® and Tecartus®, developed by Kite Pharma, Inc., a wholly-owned subsidiary of Gilead Sciences, Inc., and Kymriah®, developed by Novartis Pharmaceuticals Corporation, have been approved by the FDA. All of these therapies target the CD19 protein, a protein expressed on the surface of the majority of B cells, including B cell tumorigenic cells. Yescarta has been approved for the treatment of relapsed or refractory large B-cell lymphoma, Tecartus for the treatment of mantle cell lymphoma and Kymriah for the treatment of relapsed or refractory B-cell precursor acute lymphoblastic leukemia. These therapies have shown high response rates with prolonged treatment effects for a subset of patients. No CAR-T therapies have been approved for use in patients with solid tumors. Despite the high response rates and prolonged treatment effects observed for a subset of patients, we believe that novel CAR-T cell therapy approaches have the potential to improve efficacy, duration of response as well as safety.

TK216 competition

While there are currently no approved drugs targeting ETS oncoproteins, there are numerous companies developing or marketing treatments for the same oncology indications that we are targeting with our TK216 program. Investigational therapies in clinical development for the treatment of patients with relapsed/refractory Ewing sarcoma include kinase inhibitors, LSD1 inhibitor and other targeted therapies, therapeutic antibodies and cell therapies that are being developed by companies including Bayer AG, Bristol-Meyers Squibb Company, Eisai Co., Ltd., Epizyme, Inc., Gradalis, Inc., Eli Lilly and Company, Johnson & Johnson, Exelixis, Inc., NantCell, Inc., Pharmamar S.A., Pfizer, Inc., Salarius Pharmaceuticals, Inc., Takeda Pharmaceutical Company Limited, and others.

Licenses and Collaborative Relationships

UC San Diego

In March 2016, we entered into a license agreement with the Regents of the University of California, or the Regents, represented by UC San Diego, which was amended and restated in August 2018, and amended on March 25, 2019, May 15, 2019 and February 5, 2021 (the "UC San Diego License Agreement"), for the development, manufacturing and distribution rights to naked antibodies, including cirmtuzumab and genetically engineered cellular therapy products, including CAR-T products that are covered by licensed patents for all human therapeutic, diagnostic and preventive applications in all indications. Under the UC San Diego License Agreement, we paid an upfront license fee of $0.5 million and issued 107,108 shares of common stock. Commencing in 2017, we also pay UC San Diego an annual license maintenance fee and reimburses to UC San Diego its annual patent costs for the licensed patents. The UC San Diego License Agreement also requires the payment of certain development and regulatory milestones, aggregating from $10.0 million to $12.5 million, on a per product basis, certain worldwide sales milestones based on achievement of tiered revenue levels aggregating $75.0 million, low single-digit royalties including potential future minimum annual royalties on net sales of each product, reimbursement of certain annual patent costs, and requires certain minimum diligence efforts to advance the licensed assets, including spending at least $1.0 million in development annually through 2021. Unless terminated earlier, the UC San Diego License Agreement will expire upon the later of the expiration date of the longest-lived patent rights or the 15th anniversary of the first commercial sale of a licensed product. UC San Diego may terminate the UC San Diego License Agreement if a material breach by us is not cured within a reasonable time, we file a claim asserting the licensed patent rights are invalid or unenforceable, or we file for bankruptcy. We may terminate the agreement at any time upon at least 90 days' written notice. In July 2016, we entered into a research agreement with UC San Diego (the "UC San Diego Research Agreement"), for further research on the ROR1 therapeutic development program. Under this five-year agreement, UC San Diego will have an aggregate budget of $3.6 million, with $125,000 payable quarterly. The costs paid to UC San Diego under the UC San Diego Research Agreement are included as part of our annual diligence obligations under the UC San Diego License Agreement. As of December 31, 2020, we believe we have met our obligations under the UC San Diego License Agreement.

CIRM

In August 2017, CIRM awarded an $18.3 million grant to researchers at UC San Diego to advance our Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib for the treatment of patients with B-cell lymphoid malignancies, including MCL and CLL. We are conducting the trial in collaboration with UC San Diego, and we are responsible for study conduct and data management. We estimate we will receive approximately $14.0 million in development milestones under research subaward agreements throughout the award project period, estimated to be from October 1, 2017 to March 31, 2022. We are required to provide UC San Diego progress and financial update reports throughout the award period. The subaward does not bear a royalty payment commitment, nor is the subaward otherwise refundable. As of December 31, 2020, we believe we have met our obligations under the CIRM award and UC San Diego subawards.

In October 2017, CIRM awarded a $5.8 million grant to the researchers at UC San Diego to develop a novel anti-cancer stem cell targeted therapy for patients with advanced solid and hematological malignancies. In connection with such CIRM award, we agreed to provide up to $1.0 million in contingency funds if required during the grant period.

CIRM may suspend or permanently cease disbursements of funds under the research subaward agreements, or pursue other remedies as allowed by law, if CIRM determines that UC San Diego has not complied with the terms and conditions of the award, or if there are unexpected, substantial manufacturing failure leading to delayed enrollment in the clinical trial, failure to enroll the trial, or if FDA issues a clinical hold order with respect to the clinical trial.

Georgetown University

In March 2014, we entered into an exclusive license agreement (the "Georgetown License Agreement"), with Georgetown University, or Georgetown, pursuant to which we licensed the exclusive worldwide right to patents and technologies for the development and commercialization of certain product candidates targeting EWS-FLI1 as an anti-tumor therapy for therapeutic, diagnostics, or research tool purposes. Under the Georgetown License Agreement, we are solely responsible for all development and commercialization activities and costs in our respective territories, and are also responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights. Commencing in 2015, we are obligated to pay Georgetown an annual license maintenance fee until the first commercial sale occurs, make up to $0.2 million in aggregate milestone payments upon the achievement of certain regulatory milestones, and will be required to pay low single digit royalties based on annual net product sales. The term of the Georgetown License Agreement continues until the expiration of the last valid claim within the patent rights covering the product, but may be terminated by either party upon material breach, or by us as to one or more countries with 90 days written notice of termination. Additionally, Georgetown may terminate the agreement in the event we fail to pay any amount and fails to cure such failure within 30 days after receipt of notice, defaults in our obligation to obtain and maintain insurance and fails to remedy such breach within 60 days after receipt of notice, or declares insolvency or bankruptcy. We may terminate the agreement at any time upon at least 60 days' written notice.

Shanghai Pharmaceutical (USA) Inc. ("SPH USA")

In November 2018, we entered into the SPH USA License Agreement, with SPH USA under which we granted exclusive rights to SPH USA to manufacture, develop, market, distribute and sell in the People's Republic of China, Hong Kong, Macau, and Taiwan (the "SPH USA Territory" or "Greater China"), our product candidates under the Georgetown License Agreement and the UC San Diego License Agreement. Under the SPH USA License Agreement, SPH USA is solely responsible for all pre-clinical and clinical development activities specific to obtaining regulatory approval for such product candidates in the SPH USA Territory, any third-party license milestone or royalty payments owed under the Georgetown License Agreement and the UC San Diego License Agreement, and paying us a low single digit royalty on net sales of licensed products in the SPH USA Territory. The SPH USA License Agreement will expire on a licensed product-by-licensed product and country/region-by-country/region basis on the later of ten years from the date of first commercial sale or when there is no longer a valid patent claim covering such licensed product in such country/region. The SPH USA License Agreement may be terminated by SPH USA, on a country/region-by-country/region or product-by-product basis with 180 days written notice following the first anniversary of the effective date of the agreement or at any time on a product-by-product basis for a safety concern with respect to such product. Either party may terminate the SPH USA License Agreement in its entirety or on a licensed product-by-licensed product basis upon material breach that is not cured within 90 days, or in its entirety the event the other party becomes insolvent or enters into bankruptcy proceedings. We may terminate the agreement with 60 days written notice if SPH USA or its affiliates or sublicensees commence an action challenging the validity or enforceability of any licensed patent, or with 10 days written notice if SPH USA fails to own at least 20% of the voting securities of any assignee of the SPH USA License Agreement. Upon termination of the agreement for any reason all rights and licenses granted to SPH USA under the agreement will terminate, and in the event of termination for reasons other than our material breach, SPH USA would grant us non- exclusive, royalty-free, worldwide license to any intellectual property rights controlled by SPH USA or its affiliates to exploit the terminated program in the SPH USA Territory.

Selexis S.A.

In May 2014, ROAR Therapeutics, Inc., our predecessor company, entered into a commercial license agreement (the "Selexis License Agreement"), with Selexis, S.A., a Swiss company, as amended in February 2020, pursuant to which we obtained a world-wide, non-exclusive license under certain of Selexis' patents and technology rights to use a recombinant cell line produced using the Selexis technology to produce cirmtuzumab. Under the terms of the Selexis License Agreement, we will pay Selexis milestone payments totaling, in the aggregate, CHF 1,235,000, and a royalty in the low single digits on net sales of cirmtuzumab to third parties. The Selexis License Agreement remains in effect until the last to expire of the licensed Selexis patents, but may be terminated by either party if the other party materially breaches the agreement and fails to cure the breach within sixty days after receipt of a notice of default from the other party, or in the event the other party becomes insolvent or enters into bankruptcy proceedings. Additionally, we may terminate the Selexis License Agreement and the license granted therein at any time upon sixty days prior written notice to Selexis. In May 2015, Selexis' rights to receive future milestone payments and royalties under the Selexis License Agreement were assigned to Ligand Pharmaceuticals, Incorporated.

University of Tennessee Research Foundation ("UTRF")

In March 2015, we entered into a license agreement with UTRF (the "SARD License Agreement"), pursuant to which we were granted exclusive worldwide rights in all proprietary selective androgen receptor degrader, or SARD, technologies owned or controlled by UTRF, including all improvements thereto. Under the SARD License Agreement, we are obligated to employ active, diligent efforts to conduct preclinical research and development activities for the SARD program to advance one or more lead compounds into clinical development. We are also obligated to pay UTRF annual license maintenance fees, low single-digit royalties on net sales of products and additional royalties on sublicense revenues, depending on the state of development of a clinical product candidate at the time it is sublicensed. Unless terminated earlier, the term of the SARD License Agreement will continue, on a country-by-country basis, until the expiration of the last valid claim of any licensed patent in the particular country in which a licensed patent is granted. UTRF may terminate the SARD License Agreement for our uncured breach or upon its bankruptcy. On January 30, 2020, the SARD License Agreement was amended to extend certain diligence milestones.

In July 2007, we entered into a consolidated, amended and restated license agreement with UTRF (the "SARM License Agreement") which granted us exclusive worldwide rights in all existing selective androgen receptor modulator, or SARM, technologies owned or controlled by UTRF. In 2018, we ceased the development of the SARM technology following the failure of a Phase 2 clinical study of enobosarm to achieve statistical significance with respect to the primary endpoint of the study. Effective on March 31, 2020, we terminated the SARM License Agreement, and we no longer have any obligation to make further payments under the SARM License Agreement, including payments for patent prosecution and maintenance. We no longer have any rights to develop or sublicense the SARM technology.

Manufacturing

We have adopted a manufacturing strategy of contracting with third parties to manufacture drug substance and product in accordance with current Good Manufacturing Practices, or cGMPs, and additional manufacturers are used to label, package and distribute investigational drug products. This strategy allows us to maintain a more flexible infrastructure while focusing our expertise on developing our products.

We expect to continue to rely on third parties for the production of clinical and commercial quantities of any product candidates. There are no unusually complicated biochemistries or unusual equipment required in the manufacturing process for either cirmtuzumab or TK216.

We have established a quality control and quality assurance program, which includes a set of standard operating procedures and specifications designed to ensure that our products are manufactured in accordance with cGMPs, and other applicable domestic and foreign regulations.

Intellectual Property

We strive to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to our business, including seeking, maintaining, and defending patent rights, whether developed internally or acquired or licensed from third parties. Our policy is to seek to protect our proprietary position by, among other methods, filing patent applications in the U.S and in jurisdictions outside of the U.S. related to our proprietary technology, inventions, and improvements that are important to the development and implementation of our business. We also rely on trade secrets and know-how relating to our proprietary technology, continuing innovation, and acquisition and in-licensing opportunities to develop, strengthen, and maintain our proprietary position in the field of cancer therapeutics.

Our commercial success may depend in part on our ability to (i) obtain and maintain patent and other proprietary protection for our technology, inventions, and improvements; (ii) preserve the confidentiality of our trade secrets; (iii) defend and enforce our proprietary rights, including our patents; and (iv) operate without infringing the valid and enforceable patents and other proprietary rights of third parties.

We have developed, licensed and acquired numerous patents and patent applications and possess substantial know-how and trade secrets relating to the development and commercialization of healthcare products and services. As of February 19, 2021, our owned and in-licensed patent portfolio consisted of approximately 39 issued U.S. patents and 18 pending U.S. patent applications related to certain of our proprietary technology, inventions, and improvements, and 50 issued patents and 72 pending patent applications in jurisdictions outside of the U.S.

ROR1 Program

We have an exclusive, commercial, worldwide, transferrable license to a portfolio of patents and patent applications directed to ROR1 antibodies and CAR-T therapies for all therapeutic indications. This portfolio is licensed from the Regents of the University of California. We have know-how and trade secrets related to compositions of matter for treating cancers, methods for treating cancer, and methods of screening for additional compositions of matter used for treating cancer, as well as to additional antibodies and molecules that modulate ROR1 signaling. We have also developed certain patents and patent applications directed to ROR1 based therapies, which are owned by Oncternal.

As of February 22, 2021, our licensed patent portfolio included patents related to our clinical candidate currently in phase 1/2 clinical trials, cirmtuzumab. Cirmtuzumab is a humanized monoclonal antibody that specifically binds to the ROR1 receptor. We have two issued U.S. patents directed to the cirmtuzumab composition of matter: U.S. Pat. No. 9,217,040, with a patent term not due to expire before 2032; and U.S. Patent No. 9,758,591, with a patent term not due to expire before March 2033. We have one patent issued in the U.S. directed to methods of using cirmtuzumab to treat cancer, U.S. Pat, No. 10,344,096, with a patent term not due to expire before March 2033. We have one patent application pending in the U.S. related to single chain variable region fragments derived from cirmtuzumab which, if issued, would have a patent term not due to expire before 2033. We also have patents issued in Australia, China, Europe, Israel, Japan, Korea, Macao, Canada and Mexico directed to cirmtuzumab compositions of matter. In Europe patents directed to cirmtuzumab compositions of matter have been validated in jurisdictions including France, Germany, Italy, UK, Spain, Turkey, Belgium, Poland, Netherlands, Greece, Switzerland, Sweden, Austria, Denmark, and Ireland. We have approximately 13 pending applications in foreign jurisdictions related to cirmtuzumab compositions of matter and methods of use in treating cancer, including Australia, China, Europe, Japan, Korea, Malaysia, Mexico, Philippines, and Thailand. Patents, if issued from these pending foreign applications, would not be due to expire before 2033.

As of February 22, 2021, we have licensed patent applications pending in the U.S. and in approximately 22 jurisdictions outside the U.S. related to methods of treating cancer using a combination of cirmtuzumab and small-molecule chemotherapeutics. Patents, if issued from these pending non-provisional applications, would not be due to expire before dates ranging from 2037 to 2041.

As of February 22, 2021, we have licensed patents and patent applications related to additional ROR1 binding antibodies, polypeptides, chimeric antigen receptors, and nucleic acids encoding such non-cirmtuzumab ROR1 binding antibodies, polypeptides, and chimeric antigen receptors. We have six issued U.S. patents directed to non-cirmtuzumab ROR1 binding antibodies, polypeptides, chimeric antigen receptors, and nucleic acids encoding such non-cirmtuzumab ROR1 binding antibodies, polypeptides, and chimeric antigen receptors: U.S. Pat. No. 8,212,009, with a patent term not due to expire before November 2026; U.S. Patent No. 9,242,014, with a patent term not due to expire before June 2031; U.S. Patent No. 9,938,350, with a patent term not due to expire before June 2031; U.S. Patent No. 9,217,040, with a patent term not due to expire before January 2032; U.S. Patent No. 10,627,409 with a patent term not due to expire before January 2032; U.S. Patent No. 10,900,973 with a patent term not due to expire before January 2032. We have two patent applications pending in the U.S. related to additional non-cirmtuzumab ROR1 binding antibodies, polypeptides, chimeric antigen receptors, and nucleic acids encoding such non-cirmtuzumab ROR1 binding antibodies, polypeptides and chimeric antigen receptors, which, if issued, would have a patent term not due to expire before dates ranging from 2031 to 2032. We also have patents issued in Europe and Canada directed to additional ROR1 binding antibodies. We have one patent application pending in Europe related to additional ROR1 binding antibodies specific for ROR1. Any patent issued from this pending foreign application, would not be due to expire before 2032.

As of February 22, 2021, we have licensed patents and patent applications related to methods of screening for antibodies that specifically bind to ROR1. We have two issued U.S. patents, U.S. Pat. Nos. 9,523,695, and 9,933,434, with patent terms not due to expire before January 2032, directed to methods of screening for antibodies that specifically bind to ROR1. We additionally have one issued U.S. patent and one patent application issued in Japan directed to methods of screening for modulators of ROR1 signaling; additionally, we have applications pending in the U.S., Australia, Canada, China, Hong Kong, and Europe directed to methods of screening for modulators of ROR1 signaling.

As of February 22, 2021, we also own one patent application filed under the Patent Cooperation Treaty directed to methods of treating cancer using a combination of cirmtuzumab and small molecule cancer chemotherapeutics. We also own two U.S. provisional applications directed to ROR1 targeted cell-based therapies. Patents, if issued from these pending applications would not be due to expire before 2041.

TK216 Program

We have exclusive worldwide rights to a portfolio of patents and patent applications related to small molecules, including TK216, targeting EWS-FLI1 for use in therapeutics and companion diagnostics. We hold a portfolio of patents and patent applications, the Oncternal Portfolio, related to TK216, analogs thereof, and uses thereof, as well as the Georgetown Licensed Portfolio, which is licensed from Georgetown University.

As of February 19, 2021, the Oncternal Portfolio consisted of approximately eight U.S. issued patents and two pending applications in the U.S., as well as approximately nine patents and approximately 24 pending patent applications in jurisdictions outside of the U.S. As of February 19, 2021, we had two U.S. patents directed to TK216: U.S. Pat. No. 9,604,927, with a patent term not due to expire before October 2035, and U.S. Pat. No. 9,987,251, with a patent term not due to expire before October 2035. We also had a patent with claims directed to methods of inhibiting proliferation of a cell that overexpresses an ETS gene or comprises an ETS fusion gene, or inhibiting growth of or killing neoplastic cells: U.S. Pat. No. 9,895,352, with a patent term not due to expire before October 2035. We had approximately one pending U.S. application and approximately 19 patents or pending applications in jurisdictions outside the U.S., including Australia, Canada, China, Eurasia, Europe, Hong Kong, India, Israel, Japan, Korea, Macao, Mexico, New Zealand, and Taiwan. These patents have a patent term not due to expire before October 2035, and patents, if issued from these applications, would not be due to expire before October 2035. We also had a patent with claims covering compositions of TK216 in combination with venetoclax

and associated methods of inducing apoptosis in cells in AML and DLBCL: U.S. Pat. No. 10,159,660, with a patent term not due to expire before July 2037, and a patent covering TK216 in combination with lenalidomide and associated methods for inducing apoptosis in a lymphocyte produced in mantle cell lymphoma: U.S. Pat. No. 10,646,470, with a patent term not due to expire before July 2037. We had approximately one pending U.S. application and approximately 10 pending applications filed in jurisdictions outside the U.S., including Canada, China, Europe, Hong Kong, Japan, Korea, Mexico, Singapore, and Taiwan. Patents, if issued from these applications, would not be due to expire before July 2037. The Oncternal Portfolio further contained additional patents and pending applications related to indoline derivative compounds, which are analogs of TK216. We had two issued U.S. patents directed to compounds and methods of inhibiting proliferation of a cell expressing an ETS gene or comprising an ETS fusion gene: U.S. Pat. No. 9,822,122, with a patent term not due to expire before March 2037, and U.S. Pat. No. 10,351,569, with a patent term not due to expire before March 2037. We also had an issued U.S. patent with claims directed to killing or inhibiting the growth of a neoplastic cell and methods of treating specific cancers by administering an analogue of TK216: U.S. Pat. No. 10,711,008, with a patent term not due to expire before March 2037. There was also approximately six patents or applications pending outside the U.S. in China, Europe (including a European patent validated in Austria, Belgium, Denmark, France, Germany, Great Britain, Ireland, Italy, Spain, Sweden, and Switzerland), Japan, Korea, and Taiwan. Patents, if issued from these applications, would not be due to expire before March 2037.

As of February 19, 2021, the Georgetown Licensed Portfolio contained patents directed to other EWS-FLI1 inhibitor compounds. We had three U.S. patents directed to compounds and methods for treating Ewing sarcoma or pancreatic cancer: U.S. Pat. No. 8,232,310, with a patent term not due to expire before November 2028, U.S. Pat. No. 9,045,415, with a patent term not due to expire before August 2028, and U.S. Pat. No. 9,758,481, with a patent term not due to expire before December 2027. We had four issued patents in jurisdictions outside the U.S., including Australia, Canada, Europe (validated in Germany, France and Great Britain), and Hong Kong. These patents are not due to expire before December 2027. We had two issued U.S. patents directed to compounds and methods for treating pancreatic cancer or Ewing sarcoma: U.S. Pat. No. 9,290,449, with a patent term not due to expire before April 2033, and U.S. Pat. No. 9,714,222, with a patent term not due to expire before April 2033. There are approximately 18 patents or pending applications outside the U.S. in Australia, Canada, China, Europe, Hong Kong, India, Israel, Japan, Korea, Macao, Mexico, and New Zealand. These patents have a patent term not due to expire before April 2033, and patents, if issued from these applications, would not be due to expire before April 2033. The Georgetown Licensed Portfolio contained additional patents and pending applications related to methods of treating cancers. We had one issued U.S. patent directed to methods of treating lung cancer or glioblastoma multiforme: U.S. Pat. No. 9,511,050, with a patent term not due to expire before October 2034. There were approximately two patents issued outside the U.S. in China and Japan. These patents have a patent term not due to expire before October 2034.

SARD Program

We have exclusive worldwide rights to a portfolio of patents and patent applications related to Selective Androgen Receptor Degrader (SARD) compounds for use in therapeutics. We hold a portfolio of patents and patent applications related to SARDs and jointly owned with UTRF, including 10 issued U.S. patents directed to SARD ligands and methods of use thereof: U.S. Pat. No. 9,814,698, U.S. Pat. No. 10,017,471, U.S. Pat. No. 10,035,763, U.S. Pat. No. 10,441,570, U.S. Pat. No. 10,865,184, U.S. Pat. No. 9,815,776, U.S. Pat. No. 9,834,507, U.S. Pat. No. 10,093,613, U.S. Pat. No. 10,597,354, and U.S. Pat. No. 10,806,720, as well as four issued patents in Australia, Japan, and Russia, with a patent term not due to expire before April 2036. We also have a portfolio of patents and patent applications licensed from UTRF including three issued U.S. patent directed to SARD ligands and methods of use thereof: U.S. Pat. No. 10,314,797, U.S. Pat. No. 10,654,809, and U.S. Pat. No. 10,806,719 with a patent term not due to expire before June 2037. A third portfolio for the SARD program includes 10 patent applications licensed from UTRF.

The patents if issued from these applications will have a patent term not due to expire before May 2039. Individual patents extend for varying periods of time, depending upon the date of filing of the patent application, the date of patent issuance, and the legal term of patents in the countries in which they are obtained. Generally, patents issued for applications filed in the U.S. are effective for 20 years from the earliest

effective and non-provisional filing date. The patent term may be adjusted to compensate for delayed patent issuance, when such delays are caused by the patent office or successful appeals against patent office actions. There is no limit on this patent term adjustment. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of the FDA regulatory review period. The extended restoration period cannot be longer than five years and the total patent term, including the restoration period, must not exceed 14 years following the date of FDA approval of the applicable drug product. The duration of patents outside of the U.S. varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective non-provisional filing date. Our issued patents are due to expire on dates ranging from 2026-2037. If patents are issued on our pending patent applications, the resulting patents would be due to expire on dates ranging from 2026-2040. However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country, and the validity and enforceability of the patent. Most countries require a patent owner to pay maintenance fees or annuities in order to extend the patent to the full length of its term. If these fees and annuities are not paid timely, our patents will expire prior to the expiration date.

Government Regulation

Government authorities in the U.S., at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, marketing and export and import of products such as those we are developing. A new drug must be approved by the FDA through the new drug application, or NDA, process and a new biologic must be approved by the FDA through the biologics license application, or BLA, process before it may be legally marketed in the U.S.

United States Drug Development Process

In the U.S., the FDA regulates drugs under the federal Food, Drug, and Cosmetic Act, or FDCA, and in the case of biologics, also under the Public Health Service Act, or PHSA, and their implementing regulations. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

The process required by the FDA before a drug or biologic may be marketed in the U.S. generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in accordance with GLP requirements and other applicable regulations;
- submission to the FDA of an Investigational New Drug Application, or IND, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practice, or GCP, requirements to establish the safety and efficacy of the proposed drug, or safety, purity and potency of the proposed biologic, for its intended use;
- submission to the FDA of an NDA or BLA after completion of all pivotal clinical trials;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA whether to file the application for review;
- potential review of the product application by an FDA advisory committee, where appropriate and if applicable;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug or biologic's identity, strength, quality and purity, and audits of selected clinical trial sites to ensure compliance with GCP; and

- FDA review and approval of the NDA or BLA.

An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls, or CMC, information, and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA.

In addition to the submission of an IND to the FDA, supervision of certain human gene transfer trials may also require evaluation and assessment by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such assessment may result in some delay before initiation of a clinical trial.

Clinical trials involve the administration of a product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, patient selection and exclusion criteria, and the parameters to be used to monitor patient safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations including GCP requirements, including the requirement that all research patients provide informed consent. Further, each clinical study must be reviewed and approved by an independent IRB at or servicing each institution at which the clinical study will be conducted. The FDA, the IRBs, or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk or if a trial is unlikely to meet its stated objectives. In addition, some clinical trials are overseen by an independent group of qualified experts organized by the sponsor, known as a data safety monitoring board or committee. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- **Phase 1**: The product candidate is initially administered to healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, such as cancer, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

- **Phase 2**: The product candidate is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine the appropriate dosage for further clinical trials.

- **Phase 3**: The product candidate is administered to an expanded patient population at geographically dispersed clinical study sites. These clinical trials are intended to establish the safety and efficacy of the product and the overall risk-benefit ratio of the product candidate and provide, if appropriate, an adequate basis for product labeling and commercial use of the product.

Post-approval trials, sometimes referred to as Phase 4 studies, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA or BLA.

During the development of a new drug or biologic, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA or BLA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice, and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the meetings at the end of the Phase 2 trial to discuss Phase 2 clinical results and present plans for the pivotal Phase 3 clinical trial that they believe will support approval of the new drug or biologic.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. In addition, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life. While the IND is active and before approval, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or *in vitro* testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator brochure.

There are also requirements governing the reporting of ongoing clinical trials and completed trial results to public registries. Sponsors of certain clinical trials of FDA-regulated products are required to register and disclose specified clinical trial information, which is publicly available at www.clinicaltrials.gov. Information related to the product, patient population, phase of investigation, trial sites and investigators and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved.

United States Review and Approval Process

The results of product development, preclinical and other non-clinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA or BLA requesting approval to market the product. The NDA or BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's CMC and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of the product, or from a number of alternative sources, including studies initiated and sponsored by investigators. The submission of an NDA or BLA is subject to the payment of user fees; a waiver of such fees may be obtained under certain limited circumstances.

Within 60 days following submission of the application, the FDA reviews all NDAs and BLAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. The FDA may request additional information rather than accept an NDA or BLA for filing. In this event, the NDA or BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing.

Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA's goal is to review standard applications within ten months after the filing date, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process may also be extended by FDA requests for additional information or clarification. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality and purity. The FDA reviews a BLA to determine, among other things whether the product is safe, pure and potent and the facility in which it is manufactured, processed, packed or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may refer the NDA or BLA to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

Before approving an NDA or BLA, the FDA will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates an NDA or BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be produced, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes the specific deficiencies in the NDA or BLA identified by the FDA and may require additional clinical data, such as an additional clinical trial or other significant and time-consuming requirements related to clinical trials, nonclinical studies or manufacturing. If a Complete Response Letter is issued, the sponsor must resubmit the NDA or BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may decide that the NDA or BLA does not satisfy the criteria for approval.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. In addition, the FDA may require a sponsor to conduct Phase 4 testing, which involves clinical trials designed to further assess a drug's safety and effectiveness after NDA or BLA approval, and may require testing and surveillance programs to monitor the safety of approved products which have been commercialized. The FDA may also place other conditions on approval including the requirement for a risk evaluation and mitigation strategy, or REMS, to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS. The FDA will not approve the NDA without an approved REMS, if required. A REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Marketing approval may be withdrawn for non-compliance with regulatory requirements or if problems occur following initial marketing.

The Food and Drug Administration Safety and Innovation Act, or FDASIA, made permanent the Pediatric Research Equity Act, or PREA, which requires a sponsor to conduct pediatric clinical trials for most drugs and biologics, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, original NDAs, BLAs and supplements thereto must contain a pediatric assessment unless the sponsor has received a deferral or waiver. The required assessment must evaluate the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The sponsor or FDA may request a deferral of pediatric clinical trials for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug or biologic is ready for approval for use in adults before pediatric clinical trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric clinical trials begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the U.S. or, if it affects more than 200,000 individuals in the U.S., there is no reasonable expectation that the cost of developing

and making a drug or biologic product available in the U.S. for this type of disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA or BLA. After the FDA grants orphan designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or inability to manufacture the product in sufficient quantities. The designation of such drug or biologic also entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. However, competitors, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Expedited Development and Review Programs

The FDA offers a number of expedited development and review programs for qualifying product candidates. For example, the FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drug products that meet certain criteria. Specifically, new drugs or biologics are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the review team during product development and, once an NDA or BLA is submitted, the product may be eligible for priority review. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. With regard to a Fast Track product candidate, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA or BLA, the FDA agrees to accept sections of the NDA or BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA or BLA.

A product candidate intended to treat a serious or life-threatening disease or condition may also be eligible for Breakthrough Therapy designation to expedite its development and review. A product candidate can receive Breakthrough Therapy designation if preliminary clinical evidence indicates that the product candidate, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product candidate, including involvement of senior managers.

Any product candidate submitted to the FDA for approval, including a product with a Fast Track designation or Breakthrough Therapy designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A BLA or NDA for a product candidate is eligible for priority review if the product candidate has the potential to provide a significant improvement in the treatment, diagnosis or prevention of a serious disease or condition compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of a BLA or NDA designated for priority review in an effort to facilitate the review. The FDA endeavors to review applications with priority review designations within six months of the filing date as compared to ten months for review of original BLAs and new molecular entity NDAs under its standard review goals.

In addition, a product candidate may be eligible for accelerated approval. Drugs and biologics intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. Products receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to conduct the required post-marketing studies or if such studies fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

In 2017, the FDA established a new regenerative medicine advanced therapy, or RMAT, designation as part of its implementation of the 21st Century Cures Act. The RMAT designation program is intended to fulfill the 21st Century Cures Act requirement that the FDA facilitate an efficient development program for, and expedite review of, any drug or biologic that meets the following criteria: (i) the drug or biologic qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (ii) the drug or biologic is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (iii) preliminary clinical evidence indicates that the drug or biologic has the potential to address unmet medical needs for such a disease or condition. RMAT designation provides all the benefits of Breakthrough Therapy designation, including more frequent meetings with the FDA to discuss the development plan for the product candidate and eligibility for rolling review and priority review. Product candidates granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of clinical trial sites, including through expansion of trials to additional sites.

Fast Track designation, Breakthrough Therapy designation, RMAT designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process. Even if a product candidate qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Rare Pediatric Disease Priority Review Voucher Program

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

For purposes of this program, a “rare pediatric disease” is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare diseases or conditions within the meaning of the Orphan Drug Act. On December 27, 2020, the Rare Pediatric Disease Priority Review Voucher Program was extended. Under the current statutory sunset provisions, after September 30, 2024, FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug, and that designation was granted by September 30, 2024. After September 30, 2026, FDA may not award any Rare Pediatric Disease Priority Review Voucher.

Post-approval requirements

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. After approval, some types of changes to the approved product, such as adding new indications, certain manufacturing changes and additional labeling claims, are subject to further FDA review and approval. Drug and biologics manufacturers and other entities involved in the manufacture and distribution of approved drugs and biologics are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP regulations and other laws and regulations. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

Any drug products we or our partners manufacture or distribute pursuant to FDA approvals will be subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the drug, providing the FDA with updated safety and efficacy information, drug sampling and distribution requirements, complying with certain electronic records and signature requirements, and complying with FDA promotion and advertising requirements. The FDA strictly regulates labeling, advertising, promotion and other types of information on products that are placed on the market and imposes requirements and restrictions on drug and biologics manufacturers, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and consistent with the provisions of the approved label. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications or supplements to approved applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties.

Drug Product Marketing Exclusivity

Market exclusivity provisions authorized under the FDCA can delay the submission or the approval of certain marketing applications. For example, the FDCA provides a five-year period of non-patent marketing exclusivity within the U.S. to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an abbreviated new drug application, or ANDA, or an NDA submitted under Section 505(b)(2), or 505(b)(2) NDA, submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder.

The FDCA alternatively provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to any preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of marketing exclusivity available in the U.S. Pediatric exclusivity provides for an additional six months of marketing exclusivity attached to another period of exclusivity if a sponsor conducts clinical trials in children in response to a written request from the FDA. The issuance of a written request does not require the sponsor to undertake the described clinical trials. In addition, orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity, except in certain circumstances.

Biosimilars and Exclusivity

The Affordable Care Act includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being addressed by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor’s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed “interchangeable” by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

A biological product can also obtain pediatric market exclusivity in the U.S. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

FDA Regulation of Companion Diagnostics

Our product candidates may require use of an *in vitro* diagnostic to identify appropriate patient populations. These diagnostics, often referred to as companion diagnostics, are regulated as medical devices. In the U.S., the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, pre-clinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Unless an exemption applies, companion diagnostic tests require marketing clearance or approval from the FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and premarket approval, or PMA, approval.

If use of companion diagnostic is essential to safe and effective use of a drug or biologic product, then the FDA generally will require approval or clearance of the diagnostic contemporaneously with the approval of the therapeutic product. On August 6, 2014, the FDA issued a final guidance document addressing the development and approval process for "*In Vitro* Companion Diagnostic Devices." According to the guidance, for novel candidates such as our product candidates, a companion diagnostic device and its corresponding drug or biologic candidate should be approved or cleared contemporaneously by FDA for the use indicated in the therapeutic product labeling. The guidance also explains that a companion diagnostic device used to make treatment decisions in clinical trials of a drug generally will be considered an investigational device, unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under the FDA's Investigational Device Exemption, or IDE, regulations. Thus, the sponsor of the diagnostic device will be required to comply with the IDE regulations. According to the guidance, if a diagnostic device and a drug are to be studied together to support their respective approvals, both products can be studied in the same investigational study, if the study meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the study plan and subjects, a sponsor may seek to submit an IND alone, or both an IND and an IDE. In July 2016, the FDA issued a draft guidance document intended to further assist sponsors of therapeutic products and sponsors of *in vitro* companion diagnostic devices on issues related to co-development of these products.

The FDA generally requires companion diagnostics intended to select the patients who will respond to cancer treatment to obtain approval of a PMA for that diagnostic contemporaneously with approval of the therapeutic. The review of these *in vitro* companion diagnostics in conjunction with the review of therapeutic candidates such as those we are developing involves coordination of review by the FDA's Center for Drug Evaluation and Research and by the FDA's Center for Devices and Radiological Health. The PMA process, including the gathering of clinical and pre-clinical data and the submission to and review by the FDA, can take several years or longer. It involves a rigorous premarket review during which the applicant must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA applications are also subject to an application fee. In addition, PMAs for certain devices must generally include the results from extensive pre-clinical and adequate and well-controlled clinical trials to establish the safety and effectiveness of the device for each indication for which FDA approval is sought. In particular, for a diagnostic, the applicant must demonstrate that the diagnostic produces reproducible results when the same sample is tested multiple times by multiple users at multiple laboratories. In addition, as part of the PMA review, the FDA will typically inspect the manufacturer's facilities for compliance with the Quality System Regulation, or QSR, which imposes elaborate testing, control, documentation and other quality assurance requirements.

If the FDA evaluations of both the PMA application and the manufacturing facilities are favorable, the FDA will either issue an approval letter or an approvable letter, which usually contains a number of conditions that must be met in order to secure the final approval of the PMA, such as changes in labeling, or specific additional information, such as submission of final labeling, in order to secure final approval of the PMA. If the FDA concludes that the applicable criteria have been met, the FDA will issue a PMA for the approved indications, which

can be more limited than those originally sought by the applicant. The PMA can include post-approval conditions that the FDA believes necessary to ensure the safety and effectiveness of the device, including, among other things, restrictions on labeling, promotion, sale and distribution.

If the FDA's evaluation of the PMA or manufacturing facilities is not favorable, the FDA will deny approval of the PMA or issue a not approvable letter. A not approvable letter will outline the deficiencies in the application and, where practical, will identify what is necessary to make the PMA approvable. The FDA may also determine that additional clinical trials are necessary, in which case the PMA approval may be delayed for several months or years while the trials are conducted and then the data submitted in an amendment to the PMA. Once granted, PMA approval may be withdrawn by the FDA if compliance with post approval requirements, conditions of approval or other regulatory standards is not maintained or problems are identified following initial marketing. PMA approval is not guaranteed, and the FDA may ultimately respond to a PMA submission with a not approvable determination based on deficiencies in the application and require additional clinical trial or other data that may be expensive and time-consuming to generate and that can substantially delay approval.

After a device is placed on the market, it remains subject to significant regulatory requirements. Medical devices may be marketed only for the uses and indications for which they are cleared or approved. Device manufacturers must also establish registration and device listings with the FDA. A medical device manufacturer's manufacturing processes and those of its suppliers are required to comply with the applicable portions of the QSR, which cover the methods and documentation of the design, testing, production, processes, controls, quality assurance, labeling, packaging and shipping of medical devices. Domestic facility records and manufacturing processes are subject to periodic unscheduled inspections by the FDA. The FDA also may inspect foreign facilities that export products to the U.S.

Approval Process Outside of the United States

In addition to regulations in the U.S., we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our product candidates. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries. In addition, ethical, social and legal concerns about gene-editing technology, gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use.

Whether or not we obtain FDA approval for a product candidate, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product candidates in those countries. The requirements and process governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. Failure to comply with applicable foreign regulatory requirements, may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Clinical Trials

Certain countries outside of the U.S. have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, or EU, for example, a clinical trial authorization, or CTA, must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and the IRB, respectively. Once the CTA is approved by the national health authority and the ethics committee has granted a positive opinion in relation to the conduct of the trial in the relevant member state(s), in accordance with a country's requirements, clinical study development may proceed.

Clinical trials of medicinal products in the European Union must be conducted in accordance with EU and national regulations and the International Conference on Harmonization, or ICH, guidelines on Good Clinical Practices, or GCP, as well as the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki. Additional GCP guidelines from the European Commission, focusing in particular on traceability, apply to clinical trials of advanced therapy medicinal products, or ATMPs. If the sponsor of the clinical trial is not established within the EU, it must appoint an EU entity to act as its legal representative. The sponsor must take out a clinical trial insurance policy, and in most EU countries, the sponsor is liable to provide 'no fault' compensation to any study subject injured in the clinical trial.

Prior to commencing a clinical trial, the sponsor must obtain a CTA from the competent authority, and a positive opinion from an independent ethics committee. The CTA must include, among other things, a copy of the trial protocol and an investigational medicinal product dossier containing information about the manufacture and quality of the medicinal product under investigation. Currently, CTAs must be submitted to the competent authority in each EU member state in which the trial will be conducted. Under the new Regulation on Clinical Trials, which is currently expected to take effect by early 2022, there will be a centralized application procedure where one national authority takes the lead in reviewing the application and the other national authorities have only limited involvement. Any substantial changes to the trial protocol or other information submitted with the CTA must be notified to or approved by the relevant competent authorities and ethics committees. Medicines used in clinical trials must be manufactured in accordance with GMP. Other national and European Union-wide regulatory requirements may also apply.

Marketing Authorizations

In the European Union, medicinal products can only be placed on the market after obtaining a Marketing Authorization, or MA. To obtain regulatory approval of an investigational biological product under European Union regulatory systems, we must submit a marketing authorization application, or MAA. The application used to file the BLA in the U.S. is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements. The process for doing this depends, among other things, on the nature of the medicinal product. The centralized procedure results in a single MA, issued by the European Commission, based on the opinion of the EMA's CHMP, which is valid across the entire territory of the EU. The centralized procedure is compulsory for human medicines that are: (i) derived from biotechnology processes, such as genetic engineering, (ii) contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative diseases, autoimmune and other immune dysfunctions and viral diseases, (iii) designated orphan medicines and (iv) advanced therapy medicinal products, or ATMPs, such as gene therapy, somatic cell therapy or tissue-engineered medicines. The centralized procedure may at the request of the applicant also be used in certain other cases. It is very likely that the centralized procedure would apply to the products we are developing.

Data and Marketing Exclusivity

The European Union also provides opportunities for market exclusivity. For example, in the European Union, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic or biosimilar application. During the additional two-year period of market exclusivity, a generic/biosimilar marketing authorization can be submitted, and the innovator's data may be referenced, but no generic/biosimilar product can be marketed until the expiration of the market exclusivity. The overall ten-year market exclusivity period may be extended to a maximum of eleven years if, during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity.

Orphan Medicinal Products

The criteria for designating an "orphan medicinal product" in the European Union are similar in principle to those in the U.S. A medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition. The application for orphan drug designation must be submitted before the application for marketing authorization. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a MA, entitled to ten years of market exclusivity for the approved therapeutic indication. During the ten-year market exclusivity period, the EMA cannot accept a MAA, or grant a MA, or accept an application to extend a MA, for the same indication, in respect of a similar medicinal product. The applicant will receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the MAA is submitted. An orphan product can also obtain an additional two years of market exclusivity in the European Union for pediatric studies. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. In addition, MA may be granted to a similar product for the same indication at any time if (1) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (2) the applicant consents to a second orphan medicinal product application; or (3) the applicant cannot supply enough orphan medicinal product.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Other Healthcare Laws and Compliance Requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the U.S. federal and state governments and by authorities in the foreign jurisdictions in which they conduct their business. At the federal level, such laws include, without limitation: the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program; federal civil and criminal false claims laws, including the civil False Claims Act, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent and civil monetary penalty laws; the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which prohibits, among other things, executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters; and the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to the federal government, information related to payments or other transfers of value made to physicians, certain other health care professionals beginning in 2022, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

Pharmaceutical companies are also subject to U.S. state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or that apply regardless of payor; laws which require pharmaceutical companies to comply with the

pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, laws which require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and information related to drug pricing, and laws requiring the registration of pharmaceutical sales and medical representatives. Violation of these laws or other governmental regulations may result in penalties, including, without limitation, significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of operations.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement for such product by third-party payors. Decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the U.S., and coverage and reimbursement can differ significantly from payor to payor. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that may require companies to provide scientific and clinical support for the use of a product to each payor separately. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. Lastly, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product. Decreases in third-party reimbursement for any product or a decision by a third-party payor not to cover a product could reduce physician usage and patient demand for the product.

Healthcare Reform

The U.S. and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the U.S., the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

In March 2010, the Affordable Care Act, or ACA, was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the U.S., and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular importance to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal health care programs.

Since its enactment, there have been judicial, Congressional, and executive branch challenges to certain aspects of the ACA. For example, the Tax Act, was enacted on December 22, 2017, which includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health insurance for all or part of a year that is commonly referred to as the "individual mandate". On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. The U.S. Supreme Court is currently reviewing the case, although it is unclear how the Supreme Court will rule. It is also unclear how other efforts, if any, to challenge, repeal or replace the ACA will impact the law.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year, which was temporarily suspended from May 1, 2020 through March 31, 2021, and reduced payments to several types of Medicare providers. Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. The likelihood of these and other proposals initiated by the former Trump administration is uncertain, particularly in light of the new Biden administration. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Data Privacy and Security Laws

Pharmaceutical companies may be subject to U.S. federal and state health information privacy, security and data breach notification laws, which may govern the collection, use, disclosure and protection of health-related and other personal information. HIPAA imposes privacy, security and breach reporting obligations with respect to individually identifiable health information upon "covered entities" (health plans, health care clearinghouses and certain health care providers), and their respective business associates, individuals or entities that create, receive, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity. HIPAA mandates the reporting of certain breaches of health information to the U.S. Department of Health and Human Services, or HHS, affected individuals and if the breach is large enough, the media. Entities that are found to be in violation of HIPAA as the result of a breach of unsecured PHI, a complaint about privacy practices or an audit by HHS, may be subject to significant civil, criminal and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance. Even when HIPAA does not apply, according to the Federal Trade Commission or the FTC, failing to take appropriate steps to keep consumers' personal information secure constitutes unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act. The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards.

In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which may be more stringent, broader in scope or offer greater individual rights with respect to protected health information, or PHI, than HIPAA, and many of which differ from each other, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. For example, California enacted the California Consumer Privacy Act, or the CCPA, on June 28, 2018, which took effect on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for

violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability. Further, the California Privacy Rights Act, or CPRA, recently passed in California. The CPRA will impose additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It will also create a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. The majority of the provisions will go into effect on January 1, 2023, and additional compliance investment and potential business process changes may be required.

In the European Union, the General Data Protection Regulation, or GDPR, went into effect in May 2018 and imposes stringent data protection requirements for controllers and processors of personal data of individuals within the European Economic Area, or EEA. The GDPR applies to any company established in the EEA as well as to those outside the EEA if they collect and use personal data in connection with the offering of goods or services to individuals in the EEA or the monitoring of their behavior. The GDPR allows EU and EEA member states to make additional laws and regulations further limiting the processing of genetic, biometric or health data. Failure to comply with the requirements of GDPR and the applicable national data protection laws of the EU and EEA member states may result in fines of up to €20 million or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the U.S., and the efficacy and longevity of current transfer mechanisms between the European Union and the U.S. remains uncertain. For example, in 2016, the European Union and U.S. agreed to a transfer framework for data transferred from the European Union to the U.S., called the Privacy Shield, but the Privacy Shield was invalidated in July 2020 by the Court of Justice of the European Union. Further, from January 1, 2021, companies have to comply with the GDPR and also the United Kingdom GDPR, or UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, e.g. fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. The relationship between the United Kingdom and the European Union in relation to certain aspects of data protection law remains unclear, and it is unclear how United Kingdom.

Merger

On March 6, 2019, we, then operating as GTx, Inc., or GTx, entered into an Agreement and Plan of Merger and Reorganization, as amended, or the Merger Agreement, with privately-held Oncternal Therapeutics, Inc., or Private Oncternal, and Grizzly Merger Sub, Inc., our wholly-owned subsidiary, or Merger Sub. Under the Merger Agreement, Merger Sub merged with and into Private Oncternal, with Private Oncternal surviving as our wholly-owned subsidiary (the "Merger"). On June 7, 2019, the Merger was completed. GTx changed its name to Oncternal Therapeutics, Inc., and Private Oncternal, which remains as our wholly-owned subsidiary, changed its name to Oncternal Oncology, Inc. On June 10, 2019, the combined company's common stock began trading on The Nasdaq Capital Market under the ticker symbol "ONCT."

Pursuant to the terms of the Merger Agreement, each outstanding share of Private Oncternal common stock outstanding immediately prior to the closing of the Merger was converted into approximately 0.073386 shares of our common stock (the "Exchange Ratio"), after taking into account a one-for-seven reverse stock split of our then-outstanding common stock (the "Reverse Stock Split"). Immediately prior to the closing of the Merger, all shares of Private Oncternal preferred stock then outstanding were exchanged into shares of common stock of Private Oncternal. In addition, all outstanding options exercisable for common stock of Private Oncternal and warrants exercisable for convertible preferred stock of Private Oncternal became options and warrants exercisable for the same number of shares of common stock of the Company multiplied by the Exchange Ratio. Immediately following the Merger, stockholders of Private Oncternal owned approximately 77.5% of the outstanding common stock of the combined company. The par value and the authorized shares of our common stock were not adjusted as a result of the Reverse Stock Split.

The transaction was accounted for as a reverse asset acquisition in accordance with generally accepted accounting principles in the United States of America, or GAAP. Under this method of accounting, Private Oncternal was deemed to be the accounting acquirer for financial reporting purposes. This determination was primarily based on the facts that, immediately following the Merger: (i) Private Oncternal's stockholders owned a substantial majority of the voting rights in the combined company, (ii) Private Oncternal designated a majority of the members of the initial board of directors of the combined company, and (iii) Private Oncternal's senior management holds all key positions in the senior management of the combined company. As a result, as of the

closing date of the Merger, our net assets were recorded at their acquisition-date relative fair values in our consolidated financial statements and the reported operating results prior to the Merger are those of Private Oncternal.

Human Capital

As of March 5, 2021, we had twelve full-time employees, two part-time employees, and a number of consultants, most of whom are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, and incentivizing our management team and our clinical, scientific and other employees and consultants. The principal purposes of our equity and cash incentive plans are to attract, retain and motivate personnel through the granting of stock-based and cash-based compensation awards, in order to align our interests and the interests of our stockholders with those of our employees and consultants.

Facilities

Our corporate headquarters are located in San Diego, California, where we currently sublease 4,677 square feet of office space used primarily for corporate, research, development, clinical, regulatory, manufacturing and quality functions.

Corporate Information

We were incorporated under the name Genotherapeutics, Inc. in Tennessee in September 1997. We changed our name to GTx, Inc. in 2001 and reincorporated in Delaware in 2003. On March 6, 2019, we, then operating as GTx, Inc., entered into the Merger Agreement with Private Oncternal and Merger Sub. Under the Merger Agreement, Merger Sub merged with and into Private Oncternal, with Private Oncternal surviving as our wholly-owned subsidiary. On June 7, 2019, the Merger was completed and GTx, Inc. changed its name to Oncternal Therapeutics, Inc. Private Oncternal, which remains as our wholly-owned subsidiary, changed its name to Oncternal Oncology, Inc. On June 10, 2019, the combined company's common stock began trading on The Nasdaq Capital Market under the ticker symbol "ONCT."

Our principal executive offices are located at 12230 El Camino Real, Suite 300, San Diego, CA 92130, and our telephone number is (858) 434-1113. Our website address is www.oncternal.com.

We file electronically with the Securities and Exchange Commission, or SEC, our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended. We make available on our website at www.oncternal.com, free of charge, copies of these reports, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The SEC maintains a website that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. The address of that website is www.sec.gov. The information in or accessible through the SEC and our website are not incorporated into, and are not considered part of, this filing. Further, our references to the URLs for these websites are intended to be inactive textual references only.

Item 1A. Risk Factors.

You should consider carefully the following risk factors, together with the other information contained in this annual report on Form 10-K, including our financial statements and the related notes and "Management Discussion and Analysis of Financial Condition and Results of Operations," before making a decision to purchase or sell shares of our common stock. We cannot assure you that any of the events discussed in the risk factors below will not occur. If any of the following events actually occur, our business, operating results, prospects or financial condition could be materially and adversely affected. This could cause the trading price of our common stock to decline and you may lose all or part of your investment. The risks described below are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also affect our business operations or financial condition.

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We have a limited operating history, have incurred significant operating losses since our inception and expect to incur significant losses for the foreseeable future. We may never generate any revenue or become profitable or, if we achieve profitability, we may not be able to sustain it.

Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We are a clinical-stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. To date, we have focused primarily on organizing and staffing our company, business planning, raising capital, identifying, acquiring and in-licensing our product candidates and conducting preclinical studies and early-stage clinical trials. Cirmtuzumab and TK216 are in clinical development, while our ROR1 CAR-T program is in the preclinical stage. We have not yet demonstrated an ability to successfully obtain regulatory approvals, manufacture a commercial scale product, or arrange for a third-party to do so on our behalf, or embark on sales and marketing activities necessary for successful post regulatory approval product commercialization, and have not developed any companion diagnostic test for our product candidates. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a history of successfully developing and commercializing biopharmaceutical products.

We have incurred significant operating losses since our inception. If our product candidates are not successfully developed and approved, we may never generate any revenue. Our net losses were $17.2 million and $34.2 million ($18.1 million related to nonrecurring Merger costs) for the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, we had an accumulated deficit of $82.8 million. Substantially all of our losses have resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations. All of our product candidates will require substantial additional development time and resources before we would be able to apply for or receive regulatory approvals and begin generating revenue from product sales. We expect to continue to incur losses for the foreseeable future, and anticipate these losses will increase substantially as we continue to develop, seek regulatory approval for and potentially commercialize any of our product candidates, and seek to identify, assess, acquire, in-license or develop additional product candidates.

To become and remain profitable, we must succeed in developing and eventually commercializing products that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing clinical trials and preclinical studies of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling any products for which we may obtain regulatory approval. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. In addition, we have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical industry. Because of the numerous risks and uncertainties associated with biopharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress our value and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product candidates or even continue our operations. A decline in our company's value could also cause stockholders to lose all or part of their investment.

We will require substantial additional financing to achieve our goals, and a failure to obtain this necessary capital when needed and on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations.

The development of biopharmaceutical product candidates is capital-intensive. We expect our expenses to increase in connection with our ongoing activities, particularly as we conduct our ongoing and planned clinical trials of cirmtuzumab and TK216, continue research and development and initiate clinical trials of our other development programs, including our preclinical ROR1 CAR-T program, and seek regulatory approval for our current product candidates and any future product candidates we may develop. In addition, as our product candidates progress through development and toward commercialization, we will need to make milestone payments to the licensors and other third parties from whom we have in-licensed or acquired our product candidates, including cirmtuzumab, TK216 and ROR1 CAR-T. If we obtain regulatory approval for any of our product candidates, we also expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution and we will need to make royalty payments to the licensors and / or other third parties from whom we have in-licensed or acquired our product candidates.

Because the outcome of any clinical trial or preclinical study is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates. Furthermore, following the completion of the Merger, we have incurred additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We have based our estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through a combination of equity financings, debt financings, government funding or other capital sources, including potentially collaborations, licenses and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Attempting to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop our product candidates.

Our future capital requirements will depend on many factors, including:

- the costs incurred as a result of the COVID-19 pandemic, including clinical trial delays;
- the type, number, scope, progress, expansions, results, costs and timing of, our clinical trials of cirmtuzumab and TK216, and preclinical studies or clinical trials of our ROR1 CAR-T program or additional indications of cirmtuzumab or TK216 as well as other product candidates that we may choose to pursue in the future;
- the costs and timing of manufacturing for our product candidates, including commercial manufacturing if any product candidate is approved;
- the costs of obtaining ibrutinib, for which we currently obtain supply at no cost under our clinical supply agreement with Pharmacyclics LLC, and vincristine to conduct our clinical trials of cirmtuzumab and TK216, respectively;
- the costs and capacity for CAR-T development and lentivirus manufacturing;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of obtaining, maintaining and enforcing our patents and other intellectual property rights;
- our efforts to evaluate, develop or partner the SARD assets; our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company, including enhanced internal controls over financial reporting;

- the costs associated with hiring additional personnel and consultants as our clinical and other development activities increase;
- the timing and amount of the milestone or other payments we must make to the licensors and other third parties from whom we have in-licensed or acquired our product candidates or technology;
- the costs and timing of establishing or securing sales and marketing capabilities if any of our product candidates are approved;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- the terms and timing of establishing and maintaining collaborations, licenses and other similar arrangements; and
- costs associated with any products or technologies that we may in-license or acquire.

Conducting clinical trials and preclinical studies is a time consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all.

Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

In January 2021, our Form S-3 registration statement expired and we do not currently have an effective shelf registration statement on Form S-3. Future sales under a Form S-3, if any, will depend on a variety of factors including, but not limited to, the effectiveness of a Form S-3, prevailing market conditions, the trading price of our common stock and our capital needs. If we are successful in filing a Form S-3 in the future, there can be no assurance that we will be successful in consummating future sales based on prevailing market conditions or in the quantities or at the prices that we deem appropriate.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through equity offerings, debt financings, government funding or other capital sources, including potentially collaborations, licenses and other similar arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, existing stockholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect stockholders' rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through future collaborations, licenses and other similar arrangements, we may have to relinquish valuable rights to our future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or that may reduce the value of our common stock.

Risks Related to the Discovery, Development and Regulatory Approval of Our Product Candidates

The COVID-19 outbreak may adversely impact our business.

The current COVID-19 worldwide pandemic has presented substantial public health and economic challenges and is affecting economies, financial markets and business operations around the world. International and U.S.

governmental authorities in impacted regions are taking action in an effort to slow the spread of COVID-19, including issuing varying forms of "stay-at-home" orders, and restricting business functions outside of one's home. In response, we have put restrictions on employee travel and working from our executive offices with many employees continuing their work remotely. In addition, while we are currently continuing the clinical trials we have underway in sites across the U.S., we expect that COVID-19 precautions may directly or indirectly impact the timeline for some of our clinical trials. For example, some of our clinical trial sites, including those located in areas severely impacted by the pandemic, have placed new patient enrollment into clinical trials on hold or, for patients traveling from out of state, have implemented a 14-day self-quarantine before appointments. Patients with MCL or CLL may be at increased risk of severe disease if they develop COVID-19 because of advanced age and/or immunosuppression, and so may be unwilling to travel to our study centers to enroll in our clinical trials. For our existing patients, we are actively working with all of our clinical trial sites to minimize disruptions and address concerns on an individual site or patient basis in order to allow participating patients to continue to receive treatment at home or in alternate healthcare settings while minimizing their potential exposure to the virus that causes COVID-19. If restrictions related to the COVID-19 outbreak continue for a prolonged period of time or if additional clinical trial sites pause patient enrollment or treatments, our clinical trial milestones would be negatively impacted. Additionally, our expectations for the timing of first-in-human dosing of our ROR1 CAR-T therapy in China has been delayed to the second half of 2021.

At the present time, we believe we have sufficient quantities of our cirmtuzumab and TK216 clinical trial materials to continue to treat patients in our clinical trials through at least the end of 2021. However, if our third-party manufacturers, including those located in China, experience additional manufacturing difficulties due to the COVID-19 outbreak or as a result of natural disasters, labor disputes, unstable political environments, or other public health emergencies, our ability to provide our product candidates to patients in clinical trials, or to provide product for treatment of patients if approved, would be jeopardized.

As the COVID-19 pandemic continues to spread around the globe, we may experience disruptions that could severely impact our business, clinical trials and manufacturing and supply chains, including:

- interruptions or delays in the operations of the FDA or other regulatory authorities, which may delay receiving feedback or approvals from the FDA or other regulatory authorities with respect to future clinical trials or regulatory submissions;
- further delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures, which may impact the integrity of subject data and clinical study endpoints;
- limiting our ability to interact with our clinical trial investigators, present our data in person at scientific and investor conferences, develop and renew contracts due to travel limitations or cancellations of scientific or investor conferences;
- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems, including interruption of supply cirmtuzumab or TK216;

- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials and interruption in global shipping that may affect the transport of clinical trial materials;

- limitations on employee resources that would otherwise be focused on the conduct of our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;

- changes in local regulations as part of a response to COVID-19 which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;

- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees; and

- difficulties launching or commercializing products, including due to reduced access to doctors as a result of social distancing protocols.

In addition, the spread of COVID-19 may have impacted, and may continue to impact, the trading price of shares of our common stock and could further severely impact our ability to raise additional capital on a timely basis, or at all, or enter into partnerships with pharmaceutical companies.

The COVID-19 pandemic continues to rapidly evolve. The extent to which the COVID-19 may impact our business, including our clinical trials, manufacturing and supply chains and financial condition will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the geographic spread of the disease, the duration of the pandemic, travel restrictions and social distancing in the U.S. and other countries, business closures or business disruptions and the effectiveness of actions taken in the U.S. and other countries to contain and treat the disease.

To the extent the COVID-19 pandemic adversely affects our business and financial results, it may also have the effect of heightening many of the other risks described in this section.

We depend heavily on the success of cirmtuzumab and TK216, which are in Phase 1 or Phase 1/2 clinical trials, as well as our ROR1 CAR-T program, which is in preclinical development. If we are unable to advance our product candidates in clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

Our two clinical-stage product candidates, cirmtuzumab and TK216, are in Phase 1 or Phase 1/2 clinical development. Cirmtuzumab is being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib (Imbruvica®) for the treatment of patients with B-cell lymphoid malignancies, including MCL and CLL and in an investigator-sponsored Phase 1b clinical trial in combination with paclitaxel for the treatment of women with HER2-negative metastatic or locally advanced, unresectable breast cancer. We are also developing TK216, an investigational small molecule that is designed to inhibit the ETS, or E26 Transformation Specific, family of oncoproteins, which have been shown in preclinical studies to alter gene transcription and RNA processing and lead to increased cell proliferation and invasion. TK216 is being evaluated in a Phase 1 clinical trial as a single agent and in combination with vincristine in patients with relapsed or refractory Ewing sarcoma, a rare pediatric cancer. In addition, we are developing a chimeric antigen receptor T cell, or CAR-T, therapy candidate that targets ROR1, which is currently in preclinical development as a potential treatment for hematologic cancers and solid tumors. None of our product candidates have advanced into a pivotal or registrational study for the indications for which we are studying them. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates. The success of our product candidates will depend on various factors, including the following:

- successful completion of preclinical and clinical studies with favorable results;

- acceptance of investigation new drug applications, or INDs, by the FDA, or similar regulatory filing by comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates and our proposed designs for future clinical trials;
- demonstrating safety and efficacy of our product candidates to the satisfaction of applicable regulatory authorities;
- receiving marketing approvals from applicable regulatory authorities, including Biologics License Applications, or BLAs, or new drug applications, or NDAs, from the FDA, and maintaining such approvals;
- making arrangements with our third-party manufacturers for commercial manufacturing capabilities and manufacturing process optimization for our product candidates;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- establishing and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates;
- the demonstration of an acceptable safety profile of our products following approval, if any;
- developing, in-licensing or acquiring companion diagnostics to our product candidates; and
- maintaining and growing an organization for people who can develop our product candidates and technology.

The success of our business, including our ability to finance the company and generate any revenue in the future, will primarily depend on the successful development, regulatory approval and commercialization of our product candidates, which may never occur. We have not yet succeeded and may not succeed in demonstrating efficacy and safety for any of our product candidates in clinical trials or in obtaining marketing approval thereafter. Given our early-stage of development, it may be several years, if at all, before we have demonstrated the safety and efficacy of a product candidate sufficient to warrant approval for commercialization. If we are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize our product candidates, we may not be able to generate sufficient revenue to continue our business.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and the results of preclinical studies and early clinical trials are not necessarily predictive of future results. Our product candidates may not have favorable results in clinical trials or receive regulatory approval on a timely basis, if at all.

Clinical drug development is expensive and can take many years to complete, and its outcome is inherently uncertain. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all, and failure can occur at any time during the preclinical study or clinical trial process. Despite promising preclinical or clinical results, any product candidate can unexpectedly fail at any stage of preclinical or clinical development. The historical failure rate for product candidates in our industry is high.

The results from preclinical studies or clinical trials of a product candidate may not predict the results of later clinical trials of the product candidate, and interim results of a clinical trial are not necessarily indicative of final results. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy characteristics despite having progressed through preclinical studies and initial clinical trials. In particular, while cirmtuzumab was well tolerated and showed favorable results in the Phase 1 portion of our ongoing Phase 1/2 clinical trial as well as the inhibition of ROR1 signaling in patients with CLL in early clinical trials, we do not know how cirmtuzumab will perform in the Phase 2 portion of the clinical trial and one or more of the reported clinical outcomes may materially change as patient enrollment continues in such trial, and such results may not be replicated in any other future clinical trials, including as a result of any differences in the target population, drug interactions or other differences in our trial design. It is not uncommon to observe results in clinical trials that are unexpected based on preclinical studies and early clinical trials, and many product candidates fail in clinical trials despite very promising early results. Moreover, this and any future preclinical and clinical data may be susceptible to varying

interpretations and analyses. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies. Furthermore, we cannot assure you that we will be able to successfully progress our preclinical programs from candidate identification to Phase 1 clinical development.

For the foregoing reasons, we cannot be certain that our ongoing and planned clinical trials and preclinical studies will be successful. Any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operations.

Any difficulties or delays in the commencement or completion, or termination or suspension, of our current or planned clinical trials could result in increased costs to us, delay or limit our ability to generate revenue, and adversely affect our commercial prospects.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of the product candidates in humans. We are conducting a Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib in patients with treatment-naïve or relapsed or refractory CLL and previously treated patients with MCL. Additionally, we are evaluating TK216 as a single agent and in combination with vincristine in a Phase 1 clinical trial in patients with relapsed or refractory Ewing sarcoma. We will have to follow the same procedure for our other preclinical product candidates that we plan to advance to clinical development, and would also be required to submit regulatory filings to foreign regulatory authorities if we decide to initiate clinical trials outside of the U.S.

We do not know whether our planned trials will begin on time or be completed on schedule, if at all. The commencement and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical studies;
- difficulties in obtaining regulatory authorizations to commence a trial or reaching a consensus with regulatory authorities on trial design;
- difficulties in recruiting clinical trial investigators with the appropriate competencies and experience;
- failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delays in obtaining approval from one or more institutional review boards, or IRBs;
- IRBs refusing to approve, suspending or terminating the trial at an investigational site, precluding enrollment of additional subjects, or withdrawing their approval of the trial;
- changes to clinical trial protocols;
- clinical sites deviating from trial protocols or dropping out of a trial;
- challenges in manufacturing sufficient quantities of product candidates or obtaining sufficient quantities of combination therapies for use in clinical trials;
- subjects failing to enroll or remain in our trial at the rate we expect, or failing to return for post-treatment follow-up;
- subjects choosing an alternative treatment for the indication for which we are developing our product candidates, or participating in competing clinical trials;
- lack of adequate funding to continue clinical trials;
- subjects experiencing severe or unexpected drug-related adverse effects;

- occurrence of serious adverse events in clinical trials of the same class of agents conducted by other companies;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current Good Manufacturing Practices, or cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials in a timely manner or consistent with applicable clinical trial protocols, good clinical practices, or GCP, or other regulatory requirements; third-party contractors not performing data collection or analysis in a timely or accurate manner; or
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

In addition, disruptions caused by the COVID-19 pandemic may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting or completing our planned and ongoing clinical trials. We could also encounter delays if our clinical trials are suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by a Data Safety Monitoring Board for such trial, or by the FDA or comparable foreign regulatory authorities. Regulatory authorities may suspend or terminate clinical trials due to a number of factors, including failure to conduct clinical trials in accordance with regulatory requirements or the applicable clinical protocols, inspection of the clinical trial operations or trial site by the FDA or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, changes in regulatory requirements and policies may occur, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing or successful completion of a clinical trial.

Further, if we decide to conduct clinical trials of our product candidates in foreign countries additional risks may arise that may delay completion of those clinical trials. These risks include the failure of enrolled patients in other countries to adhere to clinical protocol as a result of differences in healthcare practices or cultural customs, managing additional administrative burdens associated with the regulatory schemes of other countries, as well as political and economic risks relevant to other countries. Under our license and development agreement with SPH USA, SPH USA has the right to manufacture, develop, market, distribute and sell our cirmtuzumab, ROR1 CAR-T, and TK216 product candidates in the People's Republic of China, Hong Kong, Macau and Taiwan, or Greater China, and the obligation to perform all preclinical and clinical development activities required to obtain regulatory approvals for such product candidates in Greater China. In the event that SPH USA's preclinical studies or clinical trials of our product candidates raise new safety or efficacy concerns, the prospects for obtaining regulatory approval of our product candidates in the U.S. and other countries, and our business, could be adversely impacted.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval,

or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we experience delays in the completion of, or termination of, clinical trials of our product candidates, the commercial prospects of such product candidates may be harmed, and our ability to generate product revenues from such product candidates may be delayed. Moreover, delays in completing our clinical trials may increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues.

In addition, many of the factors that cause, or lead to, the termination, suspension or delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate. If we make formulation or manufacturing changes to our product candidates or revise the route of administration or dosing regimen for our product candidates, we may be required to conduct additional preclinical or clinical studies to bridge our modified product candidates to earlier versions or to bridge the new dosing regimens to dosing regimens used in our clinical trials. The need to conduct additional preclinical or clinical studies could result in delays in the approval or commercialization of our product candidates, which could shorten any period during which we may have the exclusive right to commercialize our product candidates and enable our competitors to bring products to market before we do. In such an event, the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition and prospects significantly.

We may find it difficult to enroll patients in our clinical trials. If we encounter difficulties enrolling subjects in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the U.S. Subject enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility and exclusion criteria for the trial, the design of the clinical trial, the availability of competing clinical trials and clinicians' and patients' perceptions as to the potential advantages and risks of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating as well as any drugs under development. We will be required to identify and enroll a sufficient number of subjects for each of our clinical trials. Potential subjects for any planned clinical trials may not be adequately diagnosed or identified with the diseases which we are targeting or may not meet the entry criteria for such trials. For example, a limited number of patients are affected by CLL, MCL and particularly Ewing sarcoma, which are our initial target indications for cirmtuzumab and TK216. We also may encounter difficulties in identifying and enrolling subjects with a stage of disease appropriate for our planned clinical trials. We may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible subjects to participate in the clinical trials required by the FDA or comparable foreign regulatory authorities. In addition, the process of finding and diagnosing subjects may prove costly.

The timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials, as well as completion of required follow-up periods. For certain of our product candidates, including cirmtuzumab and TK216, the conditions which we currently plan to evaluate are orphan or rare diseases with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants. If patients are unwilling to participate in our clinical trials for any reason, including the existence of concurrent clinical trials for similar patient populations or the availability of approved therapies, or if we otherwise have difficulty enrolling a sufficient number of patients, the timeline for recruiting subjects, conducting studies and obtaining regulatory approval of our product candidates may be delayed. Our inability to enroll a sufficient number of subjects for any of our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will have limited influence over their actual performance.

We cannot assure stockholders that our assumptions used in determining expected clinical trial timelines are correct or that we will not experience delays in enrollment, which would result in the delay of completion of such trials beyond our expected timelines.

Use of our product candidates could be associated with side effects, adverse events or other properties or safety risks, which could delay or preclude approval, cause us to suspend or discontinue clinical trials, abandon a product candidate, limit the commercial profile of the label for an approved product candidate, or result in other significant negative consequences that could severely harm our business, prospects, operating results and financial condition.

As is the case with oncology drugs generally, it is likely that there may be side effects and adverse events associated with the use of our product candidates. Results of our clinical trials could reveal a high and unacceptable severity and prevalence, or unexpected characteristics of side effects. For example, our ongoing clinical trials of cirmtuzumab in combination with ibrutinib, and TK216 in combination with vincristine, and the ongoing investigator-initiated clinical trial of cirmtuzumab in combination with paclitaxel, may reveal adverse events based on the combination therapy under evaluation. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials, result in a more restrictive label for the product candidate, or delay or cause the denial of regulatory approval of the product candidate by the FDA or comparable foreign regulatory authorities. The drug-related side effects could also affect patient recruitment for our clinical trials, or the ability of enrolled patients to complete the trials, or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Moreover, if our product candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may elect to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which may limit the commercial prospects for the product candidate if approved. We may also be required to modify our plans for future studies based on findings in our ongoing clinical trials. Many compounds that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the compound. In addition, regulatory authorities may draw different conclusions or require additional testing to confirm these determinations.

It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as the use of our product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by subjects. If such side effects become known later in development or upon approval, if any, such findings may harm our business, financial condition and prospects significantly.

In addition, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw, suspend or limit approvals of such product;
- we may be required to recall a product or change the way such product is administered to patients;
- regulatory authorities may require additional warnings on the label, such as a “black box” warning or a contraindication;
- we may be required to implement a Risk Evaluation and Mitigation Strategy, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product is distributed or administered, conduct additional clinical trials or change the labeling of a product or be required to conduct additional post-marketing studies or surveillance;
- we could be sued and held liable for harm caused to patients;
- sales of the product may decrease significantly or the product could become less competitive; and
- our reputation could suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

We may not be able to maintain orphan drug designations for certain of our product candidates, and may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

Regulatory authorities in some jurisdictions, including the U.S. and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan product if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the U.S., or a patient population of greater than 200,000 individuals in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the European Union, the EMA's, Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. In June 2020, we announced that we had obtained orphan drug designations in the U.S. for cirmtuzumab for treatment of MCL and for treatment of CLL/small lymphocytic lymphoma. We have also received orphan drug designation in the U.S. for TK216 for patients with Ewing sarcoma. We may seek additional orphan drug designations for cirmtuzumab or TK216 or for certain of our other product candidates. There can be no assurance that the FDA or the EMA's Committee for Orphan Medicinal Products will grant orphan designation for any indication for which we apply, or that we will be able to maintain such designation.

In the U.S., orphan designation entitles a party to financial incentives such as opportunities for grant funding for clinical trial costs, tax advantages and user-fee waivers. In addition, if a product candidate that has orphan designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including a NDA or BLA, to market the same drug for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity. The applicable exclusivity period is ten years in Europe, but such exclusivity period can be reduced to six years if a product no longer meets the criteria for orphan designation or if the product is sufficiently profitable so that market exclusivity is no longer justified.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same condition if such regulatory authority concludes that the later drug is clinically superior if it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

The regulatory landscape that will apply to development of gene therapy or cell-based therapeutic product candidates by us or by our collaborators is rigorous, complex, uncertain and subject to change, which could result in delays or termination of development of such product candidates or unexpected costs in obtaining regulatory approvals.

Regulatory requirements governing products involving gene therapy treatment have changed frequently and will likely continue to change in the future. Approvals by one regulatory agency may not be indicative of what any other regulatory agency may require for approval, and there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of gene therapy products, cell therapy products and other products created with genome editing technology. For example, in addition to the submission of an IND to the FDA, before initiation of a clinical trial in the U.S., certain human clinical trials for cell therapy products and gene therapy are subject to the National Institutes of Health Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. The NIH Guidelines call for the supervision of human gene transfer trials including an evaluation and assessment by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some

delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. We will therefore be subject to significant regulatory oversight by the FDA, and in addition to the government regulators, the applicable IBC and IRB of each institution at which we or our collaborators conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review and approve the proposed clinical trial.

Similar requirements apply in the European Union. The European Medicines Agency, or the EMA, has a Committee for Advanced Therapies, or CAT, that is responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products. Advanced-therapy medical products include gene therapy medicine, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. Similarly complex regulatory environments exist in other jurisdictions in which we might consider seeking regulatory approvals for our product candidates, further complicating the regulatory landscape. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any of our gene therapy product candidates such as CAR-T, but that remains uncertain at this point.

The clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to evaluate the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for product candidates involving gene therapy can be more lengthy, rigorous and expensive than the process for other better known or more extensively studied product candidates and technologies. Since we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the EMA or comparable regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. This may be a particularly significant risk for many of the genetically defined diseases for which we may develop product candidates alone or with collaborators due to small patient populations for those diseases, and designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene therapy in a timely manner or under technically or commercially feasible conditions. Even if our product candidates obtain required regulatory approvals, such approvals may later be withdrawn as a result of changes in regulations or the interpretation of regulations by applicable regulatory agencies.

Additionally, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA, the EMA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene therapy, either of which could materially harm our business. Furthermore, regulatory action or private litigation could result in increased expenses, delays or other impediments to our research programs or the development or commercialization of current or future product candidates.

Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a product candidate to market could decrease our ability to generate sufficient product revenue to maintain our business.

As an organization, we have limited experience in the process of enrolling patients in our clinical trials, have never conducted later-stage clinical trials or submitted a BLA or an NDA, and may be unable to do so for any of our product candidates.

We are early in our development efforts for our product candidates, and will need to successfully complete later-stage and pivotal clinical trials in order to obtain FDA or comparable foreign regulatory approval to market cirmtuzumab, TK216, ROR1 CAR-T, or any future product candidates. Carrying out later-stage clinical trials and submitting a successful BLA or NDA is a complicated process. As an organization, we are in the process of conducting a Phase 1/2 clinical trial for cirmtuzumab in combination with ibrutinib and a Phase 1 clinical trial for TK216, alone and in combination with vincristine. We have not yet conducted any clinical trials for our other product candidates. We have not previously conducted any later stage or pivotal clinical trials, have limited experience as a company in preparing, submitting and prosecuting regulatory filings and have not previously

submitted a BLA, an NDA or other comparable foreign regulatory submission for any product candidate. In addition, we have had limited interactions with the FDA and cannot be certain how many additional clinical trials of cirmtuzumab, TK216 or any other product candidates will be required or how such trials should be designed. We may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to regulatory submission and approval of any of our product candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in our planned clinical trials could delay or prevent us from submitting BLAs or NDAs for, and commercializing, our product candidates.

Our product candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA in the U.S. and by comparable foreign regulatory authorities in foreign markets. In the U.S., we are not permitted to market our product candidates until we receive regulatory approval from the FDA. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and the FDA has substantial discretion in the drug approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed. We are not permitted to market any of our product candidates in the U.S. until we receive approval of a BLA or an NDA from the FDA.

Prior to obtaining approval to commercialize a product candidate in the U.S. or abroad, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses, and in the case of biological products, that such product candidates are safe, pure and potent. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or may object to elements of our clinical development program.

The FDA or comparable foreign regulatory authorities can delay, limit or deny approval of a product candidate for many reasons, including:

- such authorities may disagree with the design or execution of our clinical trials;
- negative or ambiguous results from our clinical trials or results may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- such authorities may not accept clinical data from trials that are conducted at clinical facilities or in countries where the standard of care is potentially different from that of their own country;
- we may be unable to demonstrate that a product candidate’s clinical and other benefits outweigh its safety risks;
- such authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- such authorities may not agree that the data collected from clinical trials of our product candidates are acceptable or sufficient to support the submission of a BLA, NDA or other submission or to obtain regulatory approval in the U.S. or elsewhere, and such authorities may impose requirements for additional preclinical studies or clinical trials;

- such authorities may disagree with us regarding the formulation, labeling and/or the product specifications of our product candidates;
- approval may be granted only for indications that are significantly more limited than those sought by us, and/or may include significant restrictions on distribution and use;
- such authorities may find deficiencies in the manufacturing processes or facilities of the third-party manufacturers with which we contract for clinical and commercial supplies; or
- such authorities may not accept a submission due to, among other reasons, the content or formatting of the submission.

With respect to foreign markets, approval procedures vary among countries and, in addition to the foregoing risks, may involve additional product testing, administrative review periods and agreements with pricing authorities. In addition, events raising questions about the safety of certain marketed pharmaceuticals may result in increased cautiousness by the FDA and comparable foreign regulatory authorities in reviewing new drugs based on safety, efficacy or other regulatory considerations and may result in significant delays in obtaining regulatory approvals. Any delay in obtaining, or inability to obtain, applicable regulatory approvals would prevent us or any of our potential future collaborators from commercializing our product candidates.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failure to obtain regulatory approval to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete clinical trials and receive approval of a BLA, NDA or comparable foreign marketing application for our product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a REMS, which may be required because the FDA believes it is necessary to ensure safe use of the drug after approval. The FDA or the comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or comparable foreign regulatory authority may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

We may expend our limited resources to pursue a particular product candidate and fail to capitalize on product candidates or indications that may be more profitable or for which there are a greater likelihood of success.

Because we have limited financial and managerial resources, we are focused on specific product candidates, indications and development programs. As a result, we may forgo or delay the pursuit of opportunities with other indications or other product candidates that could have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we could relinquish valuable rights to that product candidate through collaborations, licenses and other similar arrangements, when it might be more advantageous for us to retain sole development and commercialization rights to such product candidate.

Fast Track designation by the FDA for TK216 or our other product candidates may not actually lead to a faster development or regulatory review or approval process.

We have been granted a Fast Track designation for TK216 in the U.S. for the treatment of Ewing sarcoma and may seek Fast Track designation for cirmtuzumab or our other product candidates. The Fast Track program is intended to expedite or facilitate the process for reviewing new product candidates that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended, alone or in combination with one or more drugs, to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. With a Fast Track product candidate, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA or BLA, the FDA

agrees to accept sections of the NDA or BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA or BLA.

Obtaining a Fast Track designation does not change the standards for product approval, but may expedite the development or approval process. Even though the FDA has granted such designation for TK216, it may not actually result in faster clinical development or regulatory review or approval. Furthermore, such a designation does not increase the likelihood that TK216 or any other product candidate that may be granted Fast Track designation will receive marketing approval in the U.S.

We may conduct certain of or portions of our clinical trials for our product candidates outside of the U.S. and the FDA may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We may in the future choose to conduct one or more of our clinical trials or a portion of our clinical trials for our product candidates outside the U.S. Although the FDA may accept data from clinical trials conducted outside the U.S., acceptance of this data is subject to certain conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with GCP requirements, and, and FDA must be able to validate the data from the study through an onsite inspection, if required. In general, the patient population for any clinical studies conducted outside of the U.S. must be representative of the population for whom we intend to label the product in the U.S. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will be dependent upon its determination that the studies also complied with all applicable U.S. laws and regulations. There can be no assurance the FDA will accept data from trial conducted outside of the U.S. If the FDA does not accept the data from our clinical trials of our product candidates, it would likely result in the need for additional trials, which would be costly and time consuming and delay or permanently halt our development of our product candidates.

Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary or topline data from our clinical studies, which are based on preliminary analyses of then-available data. Such preliminary results and related findings and conclusions are subject to change following more comprehensive reviews of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our clinical studies, such as the positive interim data we announced from our Phase 1/2 clinical trial of cirmtuzumab in combination with ibrutinib in May and December 2020. Interim data from this clinical trial and future clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues, following more comprehensive reviews of the data, and as more patient data become available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses of data from preclinical studies or clinical trials of its product candidates, or may interpret or weigh the importance of data differently, which could impact the value of the particular product candidate, the approvability or prospects for commercialization of the product candidate, or our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and stockholders and others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure. Information that we decide not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, product candidate or our business. If the interim, topline or preliminary data that we disclose differ from actual results, or if others, including regulatory authorities, disagree with the conclusions we reach based on our analyses of such data, our ability to obtain approval for, and commercialize our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Any Breakthrough Therapy designation that we may receive from the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek Breakthrough Therapy designation for some of our product candidates, including cirmtuzumab and TK216. A Breakthrough Therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biologic may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For drugs or biologics that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. The designation also includes the same program features as Fast Track designation, including eligibility for priority review. Designation as a Breakthrough Therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as Breakthrough Therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification and rescind the designation.

We may seek a rare pediatric disease designation for TK216, however, there is no guarantee that we will obtain such designation, and even if we do, there is no guarantee that FDA approval of TK216 will result in a priority review voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

We may seek a rare pediatric disease designation for TK216 for the treatment of Ewing’s sarcoma, however, we may not be able to obtain such designation. If we are able to obtain rare pediatric disease designation, there is no guarantee that we will be able to obtain a priority review voucher, even if TK216 is approved by the FDA. Moreover, Congress included a sunset provision in the statute authorizing the rare pediatric disease priority review voucher program. On December 27, 2020, the Rare Pediatric Disease Priority Review Voucher Program was extended. Under the current statutory sunset provisions, after September 30, 2024, FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug, and that designation was granted by September 30, 2024. After September 30, 2026, FDA may not award any rare pediatric disease priority review vouchers.

Risks Related to Our Reliance on Third Parties

We rely on third parties to conduct many of our preclinical studies and clinical trials. Any failure by a third-party to conduct the clinical trials according to good laboratory practices, GCPs and other requirements and in a timely manner may delay or prevent our ability to seek or obtain regulatory approval for or commercialize our product candidates.

We are dependent on third parties to conduct our clinical trials and preclinical studies, including our ongoing clinical trials for cirmtuzumab and TK216 and preclinical studies for ROR1 CAR-T and our other development programs. Specifically, we have used and relied on, and intend to continue to use and rely on, medical institutions, clinical investigators, CROs and consultants to conduct our clinical trials in accordance with our clinical protocols and applicable regulatory requirements. These CROs, investigators and other third parties play a significant role in the conduct and timing of these trials and subsequent collection and analysis of data. While we have agreements governing the activities of our third-party contractors, we have limited influence over their actual performance. Nevertheless, we are responsible for ensuring that each of its clinical trials is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs or trial sites fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

There is no guarantee that any such CROs, investigators or other third parties will devote adequate time and resources to such trials or perform as contractually required. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, or otherwise performs in a substandard manner, our clinical trials may be extended, delayed or terminated. In addition, many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA concludes that the financial relationship may have affected the interpretation of the study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any BLA or NDA we submit to the FDA. Any such delay or rejection could prevent us from commercializing our product candidates.

If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties or do so on commercially reasonable terms. Switching or adding additional CROs, investigators and other third parties involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, investigators and other third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We rely on third parties for the manufacture of our product candidates for clinical and preclinical development and expect to continue to do so for the foreseeable future. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate manufacturing facilities and have no plans to build our own clinical or commercial scale manufacturing capabilities. We rely, and expect to continue to rely, on third parties for the manufacture of our

product candidates and related raw materials for clinical and preclinical development, as well as for commercial manufacture if any of our product candidates receive marketing approval. The facilities used by third-party manufacturers to manufacture our product candidates must be approved by the FDA or other regulatory agencies pursuant to inspections that will be conducted after we submit a BLA or an NDA to the FDA or their equivalent to other regulatory agencies. We do not control the manufacturing process of, and are completely dependent on, third-party manufacturers for compliance with cGMP requirements for manufacture of our drug products. If these third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, including requirements related to the manufacturing of high potency and pure compounds or other products, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of third-party manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates, or if regulatory authorities withdraw any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Our or a third-party's failure to execute on our manufacturing requirements, to do so on commercially reasonable terms, or to comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials of cirmtuzumab, TK216 or any future product candidates under development;
- delay in submitting regulatory applications, or receiving marketing approvals, for our product candidates;
- subjecting third-party manufacturing facilities to additional inspections by regulatory authorities;
- requirements to cease development or to recall batches of our product candidates; and
- in the event of approval to market and commercialize our product candidates, an inability to meet commercial demands for our product candidates.

In addition, we may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third-party;
- failure to manufacture our product according to our specifications;
- failure to manufacture our product according to our schedule, or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third-party at a time that is costly or inconvenient for us.

Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval, and any related remedial measures may be costly or time consuming to implement. We do not currently have arrangements in place for redundant supply or a second source for all required raw materials used in the manufacture of our product candidates. If our current third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all.

Our current and anticipated future dependence upon others for the manufacture of our product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed.

Because we currently rely on third parties to manufacture our product candidates and to perform quality testing, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements, consulting agreements or other similar agreements with our advisors, employees, consultants and contractors prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are intentionally or inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets and despite our efforts to protect our trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

We have entered into and may seek to enter into additional collaborations, licenses and other similar arrangements, and we may not be successful in doing so, and we may not realize the benefits of such relationships.

We may seek to enter into collaborations, joint ventures, licenses and other similar arrangements for the development or commercialization of our product candidates, due to capital costs required to develop or commercialize the product candidate or manufacturing constraints, in addition to our collaboration with Shanghai Pharmaceutical Holding Co., Ltd. and SPH USA. We may not be successful in our efforts to establish such collaborations for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy or significant commercial opportunity. In addition, we face significant competition in seeking appropriate strategic partners, and the negotiation process can be time consuming and complex. Further, any future collaboration agreements may restrict us from entering into additional agreements with potential collaborators. We cannot be certain that, following a strategic transaction or license, we will achieve an economic benefit that justifies such transaction.

Even if we are successful in our efforts to establish such collaborations, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such collaborations if, for example, development or approval of a product candidate is delayed, the safety of a product candidate is questioned or sales of an approved product candidate are unsatisfactory.

In April 2018, we entered into a clinical trial and supply agreement with Pharmacyclics, LLC, an AbbVie Company, in support of our clinical trial to evaluate the combination of cirmtuzumab with ibrutinib. Ibrutinib is an inhibitor of Bruton's tyrosine kinase, a key component of cell signaling in B-cells, and is marketed by Pharmacyclics for treatment in patients with CLL and MCL. We initiated a Phase 1/2 clinical trial in May 2018 to assess cirmtuzumab in combination with ibrutinib in patients with CLL and MCL. Pursuant to the agreement, Pharmacyclics has supplied ibrutinib up to a maximum aggregate amount at no cost to us for part 1 (a dose-finding arm) and part 2 (dose expansion arm) of the ongoing Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib. Under the clinical trial and supply agreement with Pharmacyclics, we are required to provide

periodic reports to Pharmacyclics, including safety data reports, and to collaborate with Pharmacyclics in relation to any interactions with regulatory authorities regarding ibrutinib. The agreement includes no upfront costs, milestone or royalty payment commitments. In August 2019, Pharmacyclics agreed to provide additional quantities of ibrutinib at no cost to us for part 3 of the clinical trial, and so that patients who participated in parts 1 and 2 of the study may continue to receive ibrutinib in combination with cirmtuzumab for as long as their disease is responding. In the event the clinical supply agreement is terminated, we would likely incur substantial additional costs in order to obtain and purchase ibrutinib from a source other than Pharmacyclics and the Phase 2 part 3 of the Phase 1/2 clinical trial may be delayed.

In addition, any potential future collaborations may be terminable by our strategic partners, and we may not be able to adequately protect our rights under these agreements. Furthermore, strategic partners may negotiate for certain rights to control decisions regarding the development and commercialization of our product candidates, if approved, and may not conduct those activities in the same manner as we would. Any termination of collaborations we enter into in the future, or any delay in entering into collaborations related to our product candidates, could delay the development and commercialization of our product candidates and reduce their competitiveness if they reach the market, which could have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Commercialization of Our Product Candidates

Even if we receive regulatory approval for any product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions on marketing or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Following potential approval of any of our product candidates, the FDA may impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly and time-consuming post-approval studies, post-market surveillance or clinical trials to monitor the safety and efficacy of the product. The FDA may also require a REMS as a condition of approval of our product candidates, which could include requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our products will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and GCP requirements for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with our products, including adverse events of unanticipated type, severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of our products, withdrawal of the product from the market or voluntary or mandatory product recalls;
- restrictions on product distribution or use, or requirements to conduct post-marketing studies or clinical trials;
- fines, restitutions, disgorgement of profits or revenues, warning letters, untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications we filed or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of our products; and
- injunctions or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

In addition, if any of our product candidates are approved, our product labeling, advertising and promotion will be subject to regulatory requirements and continuing regulatory review. The FDA strictly regulates the

promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. If we receive marketing approval for a product candidate, physicians may nevertheless prescribe it to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant sanctions. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

The FDA and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the U.S. or abroad. For example, the results of the 2020 U.S. Presidential Election may impact our business and industry. Namely, the Trump administration took several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict whether or how these orders will be implemented, or whether they will be rescinded and replaced under the Biden administration. The policies and priorities of the new administration are unknown and could materially impact the regulations governing our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs and biologics, or modifications to approved drugs and biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the COVID-19 pandemic, on March 10, 2020 the FDA announced its intention to postpone most inspections of foreign manufacturing facilities and products, and on March 18, 2020 the FDA temporarily postponed routine surveillance inspections of domestic manufacturing facilities. Subsequently, on July 10, 2020, the FDA announced its intention to resume certain on-site inspections of domestic manufacturing facilities subject to a risk-based prioritization system. The FDA intends to use this risk-based assessment system to identify the categories of regulatory activity that can occur within a given geographic area, ranging from mission critical inspections to resumption of all regulatory activities. Regulatory authorities outside the U.S. may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

The commercial success of our product candidates will depend upon the degree of market acceptance of such product candidates by physicians, patients, healthcare payors and others in the medical community.

Our product candidates may not be commercially successful. Even if any of our product candidates receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors or the medical community. The commercial success of any of our current or future product candidates will depend

significantly on the broad adoption and use of the resulting product by physicians and patients for approved indications. The degree of market acceptance of our products will depend on a number of factors, including:

- demonstration of clinical efficacy and safety compared to other more-established products;
- the indications for which our product candidates are approved;
- the limitation of our targeted patient population and other limitations or warnings contained in any FDA-approved labeling;
- acceptance of a new drug for the relevant indication by healthcare providers and their patients;
- the pricing and cost-effectiveness of our products, as well as the cost of treatment with our products in relation to alternative treatments and therapies;
- our ability to obtain and maintain sufficient third-party coverage and adequate reimbursement from government healthcare programs, including Medicare and Medicaid, private health insurers and other third-party payors;
- the willingness of patients to pay all, or a portion of, out-of-pocket costs associated with our products in the absence of sufficient third-party coverage and adequate reimbursement;
- any restrictions on the use of our products, and the prevalence and severity of any adverse effects;
- potential product liability claims;
- the timing of market introduction of our products as well as competitive drugs;
- the effectiveness of our or any of our potential future collaborators' sales and marketing strategies; and
- unfavorable publicity relating to the product.

If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors or patients, we may not generate sufficient revenue from that product and may not become or remain profitable. Our efforts to educate the medical community and third-party payors regarding the benefits of our products may require significant resources and may never be successful.

The market opportunities for our product candidates may be limited to patients who are ineligible for or have failed prior treatments and may be small or different from our estimates.

Cancer therapies are sometimes characterized as first line, second line or third line, and the FDA often approves new therapies initially only for third line use. When cancer is detected early enough, first line therapy is sometimes adequate to cure the cancer or prolong life without a cure. Whenever first line therapy, including targeted therapy, immunotherapy, chemotherapy, hormone therapy, surgery or a combination of these, proves unsuccessful, second line therapy may be administered. Second line therapies often consist of more chemotherapy, radiation, antibody drugs, tumor targeted small molecules or a combination of these. Third line therapies can include bone marrow transplantation, antibody and small molecule targeted therapies, more invasive forms of surgery and new technologies. In markets with approved therapies, there is no guarantee that our product candidates, even if approved, would be approved for second line or first line therapy. This could limit our potential market opportunity. In addition, we may have to conduct additional clinical trials prior to gaining approval for second line or first line therapy.

Our projections of both the number of people who have the cancers we are targeting, as well as the subset of people with these cancers in a position to receive later stage therapy and who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations or market research and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these cancers. The number of patients may turn out to be lower than expected. In addition, the potentially addressable

patient population for our product candidates may be limited or may not be amenable to treatment with our product candidates. Even if we obtain significant market share for our product candidates, we may never achieve profitability without obtaining regulatory approval for additional indications, including use as a first- or second-line therapy.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty, and any processes adopted by the FDA to implement the BPCIA could have a material adverse effect on the future commercial prospects for our biological products.

We believe that any of our future product candidates approved as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to Congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, could be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found or alleged to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, as our product candidates would be, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion and avoid off-label promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

The successful commercialization of our product candidates, if approved, will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and favorable pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our products could limit our ability to market those products and decrease our ability to generate revenue.

The availability of coverage and the adequacy of reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford prescription medications such as our product candidates, if approved. Our ability to achieve coverage and acceptable levels of reimbursement for our products by third-party payors will have an effect on our ability to successfully commercialize those products. Even if we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and reimbursement in the U.S., the European Union or elsewhere will be available for any product that we may develop, and any reimbursement that may become available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs when an equivalent generic drug or a less expensive therapy is available. It is possible that a third-party payor may consider our products as substitutable and only offer to reimburse patients for the less expensive product. Even if we are successful in demonstrating improved efficacy or improved convenience of administration with our products, pricing of existing drugs may limit the amount we will be able to charge for our products. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our products and may not be able to obtain a satisfactory financial return on products that we may develop.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products. In the U.S., third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs will be covered. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our products.

Obtaining and maintaining reimbursement status is time consuming, costly and uncertain. The Medicare and Medicaid programs increasingly are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs. However, no uniform policy for coverage and reimbursement for products exists among third-party payors in the U.S. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases at short notice, and we believe that changes in these rules and regulations are likely.

Additionally, we or our collaborators may develop companion diagnostic tests for use with our product candidates as we are targeting certain defined populations for our treatments. We, or our collaborators, will be required to obtain coverage and reimbursement for these tests separate and apart from the coverage and reimbursement sought for our product candidates, once approved. While we, or our collaborators, have not yet developed any companion diagnostic test for our product candidates, if we do, there is significant uncertainty regarding our ability to obtain approval, coverage and adequate reimbursement for the same reasons applicable to our product candidates.

Outside the U.S., international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries has and will continue to put pressure on the pricing and usage of our products. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products. Accordingly, in markets outside the U.S, the reimbursement for our products may be reduced compared with the U.S. and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our products. We expect to experience pricing pressures in connection with the sale of any of our products due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

We face significant competition, and if our competitors develop technologies or product candidates more rapidly than we do, or their technologies are more effective, our ability to develop and successfully commercialize products may be adversely affected.

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates. Our competitors have developed, are developing or may develop products, product candidates and processes competitive with our product candidates. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may attempt to develop product candidates. In particular, there is intense competition in the fields of immunology, inflammation and oncology. Our competitors include larger and better funded pharmaceutical, biopharmaceutical, biotechnological and therapeutics companies. Moreover, we may also compete with universities and other research institutions who may be active in the indications we are targeting and could be in direct competition with us. We also compete with these organizations to recruit management, scientists and clinical development personnel, which could negatively affect our level of expertise and our ability to execute our business plan. We will also face competition in establishing clinical trial sites, enrolling subjects for clinical trials and in identifying and in-licensing new product candidates. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

If any of our product candidates are approved in oncology indications such as CLL or MCL, they will compete with small molecule therapies, biologics, cell-based therapies and vaccines, either approved or under development, that are intended to treat the same cancers that we are targeting, including through approaches that may prove to be more effective, have fewer side effects, be less costly to manufacture, be more convenient to administer or have other advantages over any product candidates we develop. In addition to competing with other therapies targeting similar indications, there are numerous other companies and academic institutions focused on similar targets as our product candidates and/or different scientific approaches to treating the same indications. We face competition from such companies in seeking any future potential collaborations to partner our product candidates, as well as potentially competing commercially for any approved products.

Significant progress has been made in the treatment of CLL since the advent of targeted therapies and FDA approval of ibrutinib for CLL in 2014. Three classes of targeted therapies have now been approved for the treatment of patients with CLL: inhibitors of BTK a key component of cell signaling in B-cells, such as ibrutinib, which is marketed as Imbruvica® by AbbVie, Inc., and Johnson & Johnson, and acalabrutinib, which is marketed as Calquence® by AstraZeneca PLC; inhibitors of the protein B-cell lymphoma-2, or Bcl-2, such as venetoclax, which is marketed as Venclexta® and Venclyxto® by AbbVie, Inc., and Roche/Genentech; and inhibitors of Phosphoinositide 3-kinase, or PI3K, which include idelalisib, which is marketed as Zydelig® by Gilead Sciences, Inc., and duvelisib, which is marketed as Copiktra® by Verastem, Inc. These targeted therapies are now the core of the recommended treatment regimens for patients with both first-line and relapsed or refractory CLL, and have achieved objective response rates of 85-90%, two-year PFS of 65-90%, and two-year overall survival of 75-95%. The outcomes are worse for patients with certain prognostic factors, such as 17p or 11q chromosome deletions; for such patients with relapsed or refractory CLL treated with ibrutinib, the reported PFS is 50-75%. While CLL is treatable, it generally remains incurable, and patients with CLL will generally experience a recurrence of their cancer. Additionally, clinicians are investigating their potential in earlier stage disease in multiple clinical trials.

There are several therapeutic options available to treat MCL. Newly diagnosed patients are typically treated with rituximab combined with a chemotherapy regimen known as CHOP, comprised of cyclophosphamide, doxorubicin, vincristine, and prednisone. Alternative chemotherapy regimens include bortezomib or bendamustine. Patients with clinical responses to chemotherapy may become candidates for another therapeutic approach, autologous stem cell transplantation, a procedure in which radiation and/or chemotherapy is used to eliminate the patient's immune cells, including residual MCL cells. Recently, ibrutinib was granted accelerated approval by the FDA for the treatment of relapsed MCL. Additionally, two other BTK inhibitors, acalabrutinib (Calquence®) and zanubrutinib (Brukinsa®) have been approved by the FDA for the treatment of patients with relapsed MCL. These therapies are given continuously for prolonged periods of time, and their use can be associated with significant toxicity.

The current standard therapy for patients with localized Ewing sarcoma in the U.S. is a combination of chemotherapy agents, including vincristine, doxorubicin and cyclophosphamide, with alternating cycles of ifosfamide and etoposide – a therapy known as VDC/IE. Patients that respond to this therapy may be candidates for tumor resection and continued treatment for a total of 14 to 17 cycles. This therapeutic regimen, however, is associated with significant toxicities. Patients with metastatic disease are often treated with VDC/IE or variations of this therapy with higher or more compressed dosing. This may also be supplemented by local radiation therapy or systemic radiation followed by autologous hematopoietic stem cell transplant.

Many of our competitors have significantly greater financial, technical, manufacturing, marketing, sales and supply resources or experience than we do. If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products, the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing products could present superior treatment alternatives, including by being more effective, safer, more convenient, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. If we are unable to compete effectively, our opportunity to generate revenue from the sale of products we may develop, if approved, could be adversely affected.

If the market opportunities for our products are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.

The precise incidence and prevalence for all the conditions we aim to address with our product candidates are unknown. Our projections of both the number of people who have these diseases, the number who have the specific indicated stage or treatment history we believe will be the approved indication, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research, and may prove to be incorrect. Further, new trials may change the estimated incidence or prevalence of these diseases. The total addressable market across all of our product candidates will ultimately depend upon, among other things, the indication approved by regulatory agencies and the diagnostic criteria included in the final label for each of our product candidates approved for sale for these indications, the availability of alternative treatments and the safety, convenience, cost and efficacy of our product candidates relative to such alternative treatments, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients in the U.S. and other major markets and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because some of our potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

We currently have no marketing and sales organization and have no experience as a company in commercializing products, and we may have to invest significant resources to develop these capabilities. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our products, we may not be able to generate product revenue.

We have no internal sales, marketing or distribution capabilities, nor have we commercialized a product. If any of our product candidates ultimately receives regulatory approval, we must build a marketing and sales organization with technical expertise and supporting distribution capabilities to commercialize each such product in major markets, which will be expensive and time consuming, or collaborate with third parties that have sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. We have no prior experience as a company in the marketing, sale and distribution of biopharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may not be able to enter into collaborations or hire consultants or external service providers to assist us in sales, marketing and distribution functions on acceptable financial terms, or at all. In addition, our product revenues and our profitability, if any, may be lower if we rely on third parties for these functions than if we were to market, sell and distribute any products that we develop. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we are not successful in commercializing our products, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses.

Our future growth may depend, in part, on our ability to operate in foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future growth may depend, in part, on our ability to develop and commercialize our product candidates in foreign markets. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from applicable regulatory authorities in foreign markets, and we may never receive such regulatory approvals for any of our product candidates. To obtain separate regulatory approval in most other countries, we must comply with numerous and varying regulatory requirements regarding safety and efficacy and governing, among other things, clinical trials, commercial sales, manufacturing, pricing and distribution of our product candidates. If we receive regulatory approval of our product candidates and ultimately commercialize our products in foreign markets, we would be subject to additional risks and uncertainties, including:

- different regulatory requirements for approval of drugs in foreign countries;
- reduced protection for intellectual property rights;
- the existence of additional third-party patent rights of potential relevance to our business;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, public health emergencies, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is common;

- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, public health emergencies, such as the outbreak of a novel strain of coronavirus affecting the People's Republic of China and elsewhere or natural disasters including earthquakes, typhoons, floods and fires.

Risks Related to Our Business Operations and Industry

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or any guidance we may provide.

Our quarterly and annual operating results may fluctuate significantly, which makes it difficult for us to predict our future operating results. These fluctuations may occur due to a variety of factors, many of which are outside of our control, including, but not limited to:

- the timing and cost of, and level of investment in, research, development, regulatory approval and commercialization activities relating to our product candidates, which may change from time to time;
- coverage and reimbursement policies with respect to our product candidates, if approved, and potential future drugs that compete with our products;
- the cost of manufacturing our product candidates, which may vary depending on the quantity of production and any manufacturing issues or challenges requiring additional manufacturing activities, and the terms of our agreements with third-party manufacturers;
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters such as earthquakes, typhoons, floods and fires or public health emergencies or pandemics such as the recent coronavirus (COVID-19) outbreak;
- the timing and amount of any milestone or other payments we must make to the licensors and other third parties from whom we have in-licensed or acquired our product candidates;
- expenditures that we may incur to acquire, develop or commercialize additional product candidates and technologies;
- the level of demand for any approved products, which may vary significantly;
- future accounting pronouncements or changes in our accounting policies; and
- the timing and success or failure of preclinical studies or clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

We are dependent on the services of our management and if we are not able to retain these individuals or recruit additional management or other key personnel, our business will suffer.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management, particularly our Chief Executive Officer, as well as other members of our senior management team. The loss of services of any of these individuals could delay or prevent the successful development of our product pipeline, initiation or completion of our planned operations, planned clinical trials or the commercialization of our product candidates. Although we have executed employment agreements or offer letters with each member of our senior management team, these agreements are terminable at will with or without notice and, therefore, we may not be able to retain their services as expected. We do not currently maintain "key person" life insurance on the lives of any of our employees. This lack of insurance means that we may not have adequate compensation for the loss of the services of these individuals.

We will need to expand and effectively manage our managerial, operational, financial and other resources in order to successfully pursue our clinical development and commercialization efforts. We may not be successful in maintaining our unique company culture and continuing to attract or retain qualified management and scientific and clinical personnel in the future due to the intense competition for qualified personnel among pharmaceutical, biotechnology and other businesses, particularly in the San Diego area. Our industry has experienced a high rate of turnover of management personnel in recent years. If we are not able to attract, integrate, retain and motivate necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As of March 5, 2021, we had twelve full-time employees and two part-time employees. As we continue research and development activities and pursue the potential commercialization of our product candidates, as well as function as a public company, we will need to expand our financial, research, development, regulatory, manufacturing, marketing and sales capabilities or contract with third parties to provide these capabilities for the company. As our operations expand, we expect that we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to develop and commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively.

We are subject to various foreign, federal, and state healthcare laws and regulations, and our failure to comply with these laws and regulations could harm our results of operations and financial condition.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors and customers expose us to broadly applicable foreign, federal and state fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any products for which we obtain marketing approval. Such laws include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe or certain rebates), directly or indirectly, overtly or covertly, in cash or in kind, in return for, either the referral of an individual or the purchase, lease, or order, or arranging for or recommending the purchase, lease, or order of any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal Anti- Kickback Statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;

- the federal false claims and civil monetary penalties laws, including the civil False Claims Act, which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making or causing to be made a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- HIPAA, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the Centers for Medicare and Medicaid Services, CMS, information related to payments and other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members. Beginning in 2022, such reporting obligations will also extend to payments and other transfer of value made during the prior calendar year to physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists, anesthesiology assistants and certified nurse midwives;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

- analogous U.S. state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by non- governmental third-party payors, including private insurers, or by the patients themselves; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information or which require tracking gifts and other remuneration and items of value provided to physicians, other healthcare providers and entities; and state and local laws that require the registration of pharmaceutical sales representatives.

Ensuring that our internal operations and business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including our consulting arrangements with physicians and other healthcare providers, some of whom received stock options as compensation for services provided, do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government funded healthcare programs, such as Medicare and Medicaid, or similar programs in other countries or jurisdictions, disgorgement, individual imprisonment, contractual damages, reputational harm, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, diminished profits and the curtailment or restructuring of our operations. Further, defending against any such actions can be costly, time consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil

or administrative sanctions, including exclusion from government funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to obtain marketing approval for and commercialize our product candidates and may affect the prices we may set.

In the U.S. and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably sell any product candidates for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, referred to collectively as the ACA, was enacted in the U.S. Among the provisions of the ACA of importance to our potential product candidates, the Affordable Care Act: established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; extended manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations; expands eligibility criteria for Medicaid programs; expanded the entities eligible for discounts under the Public Health program; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; created a new Medicare Part D coverage gap discount program; establishes a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

Since its enactment, there have been judicial, executive and Congressional challenges to certain aspects of the ACA. For example, the Tax Cuts and Jobs Act, or the Tax Act, was signed into law, which included a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or Texas District Court Judge, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court's decision that the individual mandate was unconstitutional but remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. The U.S. Supreme Court is currently reviewing the case, although it is unclear how the Supreme Court will rule. It is also unclear how other efforts, if any, to challenge, repeal or replace the ACA will impact the ACA or our business.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, resulted in reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030, with the exception of a temporary suspension from May 1, 2020 through March 31, 2021, unless additional Congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further, there has been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. The likelihood of success of these and other proposals initiated by the former Trump administration is uncertain, particularly in light of the new Biden administration.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates, if approved, or put pressure on our product pricing, which could negatively affect our business, results of operations, financial condition and prospects.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients with life-threatening diseases or conditions to access certain investigational new drug products that have completed a Phase 1 clinical trial. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA approval under the FDA expanded access program. There is no obligation for a drug manufacturer to make our drug products available to eligible patients as a result of the Right to Try Act.

We expect that the ACA, these new laws and other healthcare reform measures that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our product candidates, if approved.

Actual or perceived failures to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the U.S. and abroad. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties and damage to our reputation, any of which could have a material adverse effect on our operations, financial performance and business.

As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities. In the U.S., HIPAA imposes, among other things, certain standards relating to the privacy, security, transmission and breach reporting of individually identifiable health information. Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA, as amended by HITECH. Any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information. In addition, we may maintain sensitive personally identifiable information, including health information, that we receive throughout the clinical trial process, in the

course of our research collaborations, and directly from individuals (or their healthcare providers) who enroll in our patient assistance programs. As such, we may be subject to state laws requiring notification of affected individuals and state regulators in the event of a breach of personal information, which is a broader class of information than the health information protected by HIPAA.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. In addition, the CCPA went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability, and many similar laws have been proposed at the federal level and in other states. Further, the CPRA, was recently voted into law by California residents. The CPRA significantly amends the CCPA, and imposes additional data protection obligations on covered companies doing business in California, including additional consumer rights processes and opt outs for certain uses of sensitive data. It also creates a new California data protection agency specifically tasked to enforce the law, which would likely result in increased regulatory scrutiny of California businesses in the areas of data protection and security. The substantive requirements for businesses subject to the CPRA will go into effect on January 1, 2023, and become enforceable on July 1, 2023.

In the European Union, or EU, the GDPR went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the U.S., and the efficacy and longevity of current transfer mechanisms between the EU and the U.S. remains uncertain. For example, in 2016, the EU and U.S. agreed to a transfer framework for data transferred from the EU to the U.S., called the Privacy Shield, but the Privacy Shield was invalidated in July 2020 by the Court of Justice of the EU. Further, from January 1, 2021, companies have to comply with the GDPR and also the UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, i.e., fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. The relationship between the United Kingdom and the EU in relation to certain aspects of data protection law remains unclear, and it is unclear how United Kingdom data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the United Kingdom will be regulated in the long term. Currently there is a four to six-month grace period agreed in the EU and United Kingdom Trade and Cooperation Agreement, ending June 30, 2021 at the latest, whilst the parties discuss an adequacy decision. However, it is not clear whether (and when) an adequacy decision may be granted by the European Commission enabling data transfers from EU member states to the United Kingdom long term without additional measures. These changes may lead to additional costs and increase our overall risk exposure.

Although we work to comply with applicable laws, regulations and standards, our contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. If we or our third-party CROs or other contractors or consultants fail to comply with applicable federal, state or local regulatory requirements, we could be subject to a range of regulatory actions that could affect our or our contractors' ability to develop and commercialize our product candidates and could harm or prevent sales of any affected products that we are able to commercialize, or could substantially increase the costs and expenses of developing, commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Increasing use of social media could give rise to liability, breaches of data security or reputational damage.

We and any of our third-party manufacturers or suppliers may use potent chemical agents and hazardous materials, and any claims relating to improper handling, storage or disposal of these materials could be time consuming or costly.

We and any of our third-party manufacturers or suppliers will use biological materials, potent chemical agents and may use hazardous materials, including chemicals and biological agents and compounds that could be dangerous to human health and safety of the environment. Our historical operations and the operations of our third-party manufacturers and suppliers also produce hazardous waste products. Federal, state and local laws and regulations govern the use, generation, manufacture, storage, handling and disposal of these materials and wastes. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development efforts. In addition, we cannot eliminate the risk of accidental injury or contamination from these materials or wastes. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. In the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

Although we maintain workers' compensation insurance for certain costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially adversely affect our business, financial condition, results of operations and prospects.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

We face an inherent risk of product liability as a result of the clinical trials of our product candidates and will face an even greater risk if we commercialize our product candidates. For example, we may be sued if our product candidates allegedly cause injury or are found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product candidate, negligence, strict liability and a breach of warranties. Claims may be brought against us by clinical trial participants, patients or others using, administering or selling products that may be approved in the future. Claims could also be asserted under state consumer protection acts.

If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit or cease the commercialization of our products. Even a successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our products;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;

- significant negative financial impact;
- the inability to commercialize our product candidates; and
- a decline in our stock price.

We currently hold $10.0 million in product liability insurance coverage in the aggregate. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of our product candidates. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies will also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

We and any of our potential future collaborators will be required to report to regulatory authorities if any of our approved products cause or contribute to adverse medical events, and any failure to do so would result in sanctions that would materially harm our business.

If we and any of our potential future collaborators are successful in commercializing our products, the FDA and foreign regulatory authorities would require that we and any of our potential future collaborators report certain information about adverse medical events if those products may have caused or contributed to those adverse events. The timing of our obligation to report would be triggered by the date we become aware of the adverse event as well as the nature of the event. We and any of our potential future collaborators or CROs may fail to report adverse events within the prescribed timeframe. If we or any of our potential future collaborators or CROs fail to comply with such reporting obligations, the FDA or a foreign regulatory authority could take action, including criminal prosecution, the imposition of civil monetary penalties, seizure of our products or delay in approval or clearance of future products.

Our internal computer systems, or those of any of our CROs, manufacturers, other contractors or consultants or potential future collaborators, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

U.S. federal and various state and foreign governments have adopted or proposed requirements regarding the collection, distribution, use, security, and storage of personally identifiable information and other data relating to individuals, and federal and state consumer protection laws are being applied to enforce regulations related to the online collection, use, and dissemination of data. Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors, consultants and collaborators are vulnerable to damage from computer viruses, cybersecurity threats, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Attacks upon information technology systems are increasing in their frequency, levels of persistence, sophistication and intensity, and are being conducted by sophisticated and organized groups and individuals with a wide range of motives and expertise. As a result of the COVID-19 pandemic, we may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period. If such an event were to occur and cause interruptions in our operations or result in the unauthorized disclosure of or access to personally identifiable information or individually identifiable health information (violating certain privacy laws such as GDPR or the CCPA), it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other similar disruptions. Some of the federal, state and foreign government requirements include obligations of companies to notify individuals of security breaches involving particular personally identifiable information, which could result from breaches experienced by us or by our vendors, contractors, or organizations with which we have formed strategic relationships. Even though we may have contractual protections with such vendors, contractors, or other organizations, notifications and follow-up actions related to a security breach could impact our reputation, cause us to incur significant costs, including legal expenses, harm customer confidence, hurt our expansion into new markets, cause us to incur remediation costs, or cause us to lose existing customers. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the

data. We also rely on third parties to manufacture our product candidates, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, the further development and commercialization of our product candidates could be delayed, and we could be subject to significant fines, penalties or liabilities for any noncompliance to certain privacy and security laws.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. We rely on third- party manufacturers to produce our product candidates. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers were affected by a man-made or natural disaster or other business interruption. In addition, our corporate headquarters is located in San Diego, California near major earthquake faults and fire zones, and the ultimate impact on us of being located near major earthquake faults and fire zones and being consolidated in a certain geographical area is unknown. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

Our employees and independent contractors, including principal investigators, CROs, consultants and vendors, may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that our employees and independent contractors, including principal investigators, CROs, consultants and vendors may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate: (1) the laws and regulations of the FDA and other similar regulatory requirements, including those laws that require the reporting of true, complete and accurate information to such authorities, (2) manufacturing standards, including cGMP requirements, (3) federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the U.S. and abroad or (4) laws that require the true, complete and accurate reporting of financial information or data. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials, or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, individual imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We could face criminal liability and other serious consequences for violations, which could harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, clinical research organizations, contractors

and other collaborators and partners from authorizing, promising, offering, providing, soliciting or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the U.S., to sell our products abroad once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, clinical research organizations, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases and out-licensing or in-licensing of intellectual property, products or technologies, similar to our approach in in-licensing and acquiring our current product candidates. Any future transactions could increase our near and long-term expenditures, result in potentially dilutive issuances of our equity securities, including our common stock, or the incurrence of debt, contingent liabilities, amortization expenses or acquired in-process research and development expenses, any of which could affect our financial condition, liquidity and results of operations. Additional potential transactions that we may consider in the future include a variety of business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Future acquisitions may also require us to obtain additional financing, which may not be available on favorable terms or at all. These transactions may never be successful and may require significant time and attention of management. In addition, the integration of any business that we may acquire in the future may disrupt our existing business and may be a complex, risky and costly endeavor for which we may never realize the full benefits of the acquisition. Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above, any additional transactions that we do complete could have a material adverse effect on our business, results of operations, financial condition and prospects.

Risks Related to Our Intellectual Property

Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Our commercial success depends in part on our ability to obtain and maintain patent protection and trade secret protection for our product candidates, proprietary technologies and their uses as well as our ability to operate without infringing upon the proprietary rights of others. If we are unable to protect our intellectual property rights or if our intellectual property rights are inadequate for our technology or our product candidates, our competitive position could be harmed. We generally seek to protect our proprietary position by licensing or filing patent applications in the U.S. and abroad related to our product candidates, proprietary technologies and their uses that are important to our business. Our or our licensor's patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issue from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our or our licensor's patent applications will result in patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents if issued will not be infringed, designed around or invalidated by third parties. Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. The degree of future protection for our proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. These uncertainties and/or limitations in our ability to properly protect the intellectual property rights relating to our product candidates could have a material adverse effect on our financial condition and results of operations.

Although we own and license issued patents in the U.S. and foreign countries, we cannot be certain that the claims in our or our licensor's other U.S. pending patent applications, corresponding international patent applications and patent applications in certain foreign countries will be considered patentable by the United States

Patent and Trademark Office, or USPTO, courts in the U.S. or by the patent offices and courts in foreign countries, nor can we be certain that the claims in our or our licensor's issued patents will not be found invalid or unenforceable if challenged.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we, our licensors or any of our potential future collaborators will be successful in protecting our product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or block our ability to make, use and sell our product candidates;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing products.

The patent prosecution process is also expensive and time consuming, and we and our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner or in all jurisdictions where protection may be commercially advantageous. It is also possible that we or our licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, directed to technology that we license from third parties. We may also require the cooperation of our licensor in order to enforce the licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such applications. If they fail to do so, this could cause us to lose rights in any applicable intellectual property that we in-license, and as a result our ability to develop and commercialize products or product candidates may be adversely affected and we may be unable to prevent competitors from making, using and selling competing products.

In addition, although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, licensees, collaboration partners, and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties, including with respect to cirmtuzumab and TK216, or otherwise experiences disruptions in our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to several license agreements under which we are granted rights to intellectual property that are important to our business and we may enter into additional license agreements in the future. For example, in March 2014, we entered into an exclusive license agreement with Georgetown University, or Georgetown, to obtain an exclusive license to certain intellectual property rights to develop and commercialize compounds targeting EWS-

FLI1. In March 2016, we entered into an exclusive license agreement with the Regents of the University of California to obtain an exclusive license to certain intellectual property rights to develop and commercialize cirmtuzumab and other ROR1 related naked antibodies.

These license agreements impose, and we expect that any future license agreements where we in-license intellectual property, will impose on us, various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to bankruptcy-related proceedings, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist which might be enforced against our product candidates in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- our right to transfer or assign the license; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may not be able to successfully develop and commercialize the affected product candidates, which would have a material adverse effect on our business.

If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical product candidates would be adversely affected.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensor's patent rights are highly uncertain. Our and our licensor's pending and future patent applications may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive product candidates.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own or license currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we own or license may be challenged or circumvented by third parties or may be narrowed or invalidated as a result of challenges by third parties. Consequently, we do not know whether our product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may

be able to circumvent our or our licensor's patents by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our and our licensor's patents may not cover our product candidates or may be challenged in the courts or patent offices in the U.S. and abroad. Our and our licensor's patents may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation, reexamination, post-grant review, or PGR, and inter partes review, or IPR, or other similar proceedings in the USPTO or foreign patent offices challenging our or our licensor's patent rights. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we or our predecessors or our licensor and the patent examiner were unaware during prosecution. There is no assurance that all potentially relevant prior art relating to our patents and patent applications or those of our licensors has been found. There is also no assurance that there is not prior art of which we, our predecessors or licensors are aware, but which we do not believe affects the validity or enforceability of a claim in our patents and patent applications or those of our licensors, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our or our licensor's patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us. Such loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our or our licensor's patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The patent protection and patent prosecution for some of our product candidates may be dependent on third parties.

We or our licensors may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we or our licensors may miss potential opportunities to strengthen our patent position. It is possible that defects of form in the preparation or filing of our or our licensor's patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If there are material defects in the form, preparation, prosecution, or enforcement of our or our licensor's patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. If we or our licensors, whether current or future, fail to establish, maintain or protect our patents and other intellectual property rights, such rights may be reduced or eliminated. If our licensors are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

As a licensee of third parties, we rely on third parties to file and prosecute patent applications and maintain patents and otherwise protect the licensed intellectual property under some of our license agreements. We have not had and do not have primary control over these activities for certain of our patents or patent applications and other intellectual property rights. We cannot be certain that such activities by third parties have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. Pursuant to the terms of the license agreements with some of our licensors, the licensors may have the right to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents and even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. Even if we are not a party to these legal actions, an adverse outcome could harm our business because it might prevent us from continuing to license intellectual property that we may need to operate our business. If any of our licensors or any of our future licensors or future collaborators fail to appropriately prosecute and maintain patent protection for patents

covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products.

In addition, even where we have the right to control patent prosecution of patents and patent applications we have acquired or licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our predecessors or licensors and their counsel that took place prior to our assuming control over patent prosecution.

Our technology acquired or licensed from various third parties may be subject to retained rights. Our predecessors or licensors often retain certain rights under their agreements with us, including the right to use the underlying technology for noncommercial academic and research use, to publish general scientific findings from research related to the technology, and to make customary scientific and scholarly disclosures of information relating to the technology. It is difficult to monitor whether our predecessors or licensors limit their use of the technology to these uses, and we could incur substantial expenses to enforce our rights to our licensed technology in the event of misuse.

If we are limited in our ability to utilize acquired or licensed technologies, or if we lose our rights to critical in-licensed technology, we may be unable to successfully develop, out-license, market and sell our products, which could prevent or delay new product introductions. Our business strategy depends on the successful development of licensed and acquired technologies into commercial products. Therefore, any limitations on our ability to utilize these technologies may impair our ability to develop, out-license or market and sell our product candidates.

Some of our intellectual property has been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Some of the intellectual property rights we have acquired or licensed or may acquire or license in the future may have been generated through the use of U.S. government funding and may therefore be subject to certain federal regulations. For example, some of the research and development work on cirmtuzumab and TK216 was funded by government research grants. As a result, the U.S. government may have certain rights to intellectual property embodied in our product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right, under certain limited circumstances, to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third-party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if the grant recipient fails to disclose the invention to the government or fails to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the U.S. government requires that any products embodying any of these inventions or produced through the use of any of these inventions be manufactured substantially in the U.S. This preference for U.S. industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the U.S. or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. industry may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our future intellectual property is also generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply. With respect to state funding, specifically funding via the California Institute of Regenerative Medicine, or CIRM which has granted funds for the study of cirmtuzumab in combination with ibrutinib and a novel anti-cancer stem cell targeted therapy, the grantee has certain obligations and the state or CIRM has certain rights. For example, the grantee has an obligation to share intellectual property, including research results, generated by CIRM-funded research, for research use in California. In addition, the California government can exercise march-in rights if it determines that action is necessary because we or the

grantee failed to achieve practical application of the CIRM-funded technology, because we failed to comply with agreed to access and pricing requirements, or because action is necessary to address a public health emergency declared by the governor of California.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to develop products that are similar to our product candidates but that are not covered by the claims of the patents that we own or license;
- we or our licensors or predecessors might not have been the first to make the inventions covered by the issued patents or patent applications that we own or license;
- we or our licensors or predecessors might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our or our licensor's pending patent applications will not lead to issued patents;
- issued patents that we own or license may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, it could significantly harm our business, results of operations and prospects.

We rely on licensee relationships, and any disputes or litigation with our partners or termination or breach of any of the related agreements could reduce the financial resources available to us, including milestone payments and future royalty revenues.

Our existing collaborations may not continue or be successful, and we may be unable to enter into future collaborative arrangements to develop and commercialize our unpartnered assets. If any of our collaborative partners breach or terminate their agreements with us or otherwise fail to conduct their collaborative activities successfully, our product development under these agreements will be delayed or terminated. Disputes or litigation may also arise with our collaborators (with us and/or with one or more third parties), including those over ownership rights to intellectual property, know-how or technologies developed with our collaborators. Such disputes or litigation could adversely affect our rights to one or more of our product candidates and could delay, interrupt or terminate the collaborative research, development and commercialization of certain potential products, create uncertainty as to ownership rights of intellectual property, or could result in litigation or arbitration. In addition, a significant downturn or deterioration in the business or financial condition of our collaborators or partners could result in a loss of expected revenue and our expected returns on investment. The occurrence of any of these problems could be time-consuming and expensive and could adversely affect our business.

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. However, our or our licensee's research, development and commercialization activities may be subject to claims that we or our licensee infringes or otherwise violates patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our or our licensee's ability to make, use, sell, offer for sale or import our product candidates and products that may be approved in the future, or impair our competitive position. There is a substantial amount of litigation, both within and outside the U.S., involving patent and other intellectual property rights in the biopharmaceutical industry, including patent infringement lawsuits, oppositions, reexaminations, IPR proceedings and PGR proceedings before the USPTO and/or foreign patent offices. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates.

As the biopharmaceutical industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. Because patent applications are maintained as confidential for a certain period of time, until the relevant application is published we may be unaware of third-party patents that may be infringed by commercialization of any of our product candidates, and we cannot be certain that we were the first to file a patent application related to a product candidate or technology. Moreover, because patent applications can take many years to issue, there may be currently-pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation that may cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- subject us to an injunction preventing us from making, using, selling, offering for sale, or importing our products;
- prevent us from commercializing any of our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- subject us to significant liability to third parties; or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

Although no third-party has asserted a claim of patent infringement against us as of December 31, 2020, others may hold proprietary rights that could prevent our product candidates from being marketed. Any patent-related legal action against us claiming damages and seeking to enjoin activities relating to our product candidates or processes could subject us to potential liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or develop our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially reasonable

terms, if at all. Moreover, even if we or our future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. In addition, we cannot be certain that we could redesign our product candidates or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates, which could harm our business, financial condition and operating results.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court.

Competitors may infringe our intellectual property rights or those of our licensors. To prevent infringement or unauthorized use, we and/or our licensors may be required to file infringement claims, which can be expensive and time consuming. In addition, in a patent infringement proceeding, a court may decide that a patent we own or license is not valid, is unenforceable and/or is not infringed. If we or any of our licensors or potential future collaborators were to initiate legal proceedings against a third-party to enforce a patent directed at one of our product candidates, the defendant could counterclaim that our or our licensor's patent is invalid and/or unenforceable in whole or in part. In patent litigation, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution.

If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on such product candidate. In addition, if the breadth or strength of protection provided by our patents and patent applications or those of our licensors is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. Such a loss of patent protection would have a material adverse impact on our business.

Even if resolved in our favor, litigation or other legal proceedings relating to our or our licensor's intellectual property rights may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We or our licensor may not have sufficient financial or other resources to conduct or participate in such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we or our licensor can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings.

Intellectual property litigation may lead to unfavorable publicity that harms our reputation and causes the market price of our common shares to decline.

During the course of any intellectual property litigation, there could be public announcements of the initiation of the litigation as well as results of hearings, rulings on motions, and other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our existing products, programs or intellectual property could be diminished. Accordingly, the market price of shares of our

common stock may decline. Such announcements could also harm our reputation or the market for our future products, which could have a material adverse effect on our business.

Derivation or interference proceedings may be necessary to determine priority of inventions, and an unfavorable outcome may require us to cease using the related technology or to attempt to license rights from the prevailing party.

Derivation or interference proceedings provoked by third parties or brought by us or our licensors or declared by the USPTO or similar proceedings in foreign patent offices may be necessary to determine the priority of inventions with respect to our or our licensor's patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our or our licensor's defense of such proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with such proceedings could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the U.S. transitioned in March 2013 to a "first inventor to file" system in which, assuming that other requirements of patentability are met, the first inventor to file a patent application will be entitled to the patent regardless of whether a third-party was first to invent the claimed invention. A third-party that files a patent application in the USPTO after March 2013 but before we could therefore be awarded a patent covering an invention of our even if we had made the invention before it was made by such third-party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the U.S. and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensor was the first to either (1) file any patent application related to our product candidates or (2) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including PGR, IPR, and derivation proceedings. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position.

Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third-party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third-party may attempt to use the USPTO procedures to invalidate our or our licensor's patent claims that would not have been invalidated if first challenged by the third-party as a defendant in a district court action. Thus, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensor's patent applications and the enforcement or defense of our or our licensor's issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in U.S. patent law, or laws in other countries, could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves a high degree of technological and legal complexity. Therefore, obtaining and enforcing biopharmaceutical patents is

costly, time consuming and inherently uncertain. Changes in either the patent laws or in the interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property rights and may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. We cannot predict the breadth of claims that may be allowed or enforced in our or our licensor's patents or in third-party patents. In addition, Congress or other foreign legislative bodies may pass patent reform legislation that is unfavorable to us.

For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our and our licensor's ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the U.S. federal courts, the USPTO, or similar authorities in foreign jurisdictions, the laws and regulations governing patents could change in unpredictable ways that would weaken our or our licensor's ability to obtain new patents or to enforce our existing patents and patents we might obtain in the future.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distraction to management and other employees.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products. Given the amount of time required for the development, testing and regulatory review of product candidates, patents protecting our product candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our product candidates.

If we do not obtain patent term extension for our product candidates, our business may be harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, one or more of our or our licensor's U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. A maximum of one patent may be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension may also be available in certain foreign countries upon regulatory approval of our product candidates. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our or our licensor's patent expiration, and our revenue could be reduced, possibly materially. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

We may not be able to protect our intellectual property rights throughout the world.

Although we and our licensors have issued patents and pending patent applications in the U.S. and certain other countries, filing, prosecuting and defending patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the U.S. or from selling or importing products made using our inventions in and into the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we or our licensor has not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but enforcement is not as strong as that in the U.S. These products may compete with our product candidates, and our and our licensor's patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensor may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our or our licensor's efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or our licensor is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment and other requirements imposed by regulations and governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to the USPTO and various foreign patent offices at various points over the lifetime of our and our licensors' patents and/or applications. We have systems in place to remind us to pay these fees, and we rely on third parties to pay these fees when due. Additionally, the USPTO and various foreign patent office's require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with rules applicable to the particular jurisdiction. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If such an event were to occur, it could have a material adverse effect on our business.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition, we rely on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information to maintain our competitive position. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with employees, consultants and advisors, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, some courts inside and outside the U.S. are less willing or unwilling to protect trade secrets.

Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete

with us. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is common in the biopharmaceutical industry, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other biopharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Risks Related to Our Common Stock

An active, liquid and orderly market for our common stock may not be maintained.

Although our common stock is listed on the Nasdaq Capital Market, or Nasdaq, an active trading market for our common stock may never develop or, if it develops, it may not be sustained. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. An inactive market may also impair our ability to raise capital by selling shares and may impair our ability to acquire other businesses or technologies using our shares as consideration, which, in turn, could materially adversely affect our business.

The trading price of the shares of our common stock may be highly volatile, and purchasers of our common stock may incur substantial losses.

Our stock price is likely to be volatile. The stock market in general and the market for stock of biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above their purchase price. The market price for our common stock may be influenced by those factors discussed in this “Risk Factors” section and many others, including:

- our or our collaborators ability to enroll subjects in our ongoing and planned clinical trials;
- results of our clinical trials and preclinical studies, and the results of the trials of our competitors or those of other companies in our market sector;
- regulatory approval of our product candidates, or limitations to specific label indications or patient populations for its use, or changes or delays in the regulatory review process;
- regulatory developments in the U.S. and foreign countries;
- changes in the structure of healthcare payment systems, especially in light of current reforms to the U.S. healthcare system;
- the success or failure of our efforts to acquire, license or develop additional product candidates;
- innovations or new products developed by us or our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;

- announcements regarding the results of discovery efforts and preclinical, clinical and commercial activities by us, or those of our competitors;
- manufacturing, supply or distribution delays or shortages;
- any changes to our relationship with any manufacturers, suppliers, licensors, future collaborators or other strategic partners;
- achievement of expected product sales and profitability;
- variations in our financial results or those of companies that are perceived to be similar to us;
- market conditions in the biopharmaceutical sector and issuance of securities analysts' reports or recommendations;
- trading volume of our common stock;
- establishment of short positions by holders or non-holders of our common stock;
- an inability to obtain additional funding;
- sales of our stock by insiders and stockholders;
- general economic, industry and market conditions or other events or factors, many of which are beyond our control;
- additions or departures of key personnel; and
- intellectual property, product liability or other litigation against us.

In addition, in the past, stockholders have initiated class action lawsuits against biopharmaceutical companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition and results of operations.

Our executive officers, directors and principal stockholders, if they choose to act together, will continue to control or significantly influence all matters submitted to stockholders for approval. Furthermore, two of our directors have been appointed by one of our principal stockholders.

As of December 31, 2020, our executive officers, directors and greater than 5% stockholders, in the aggregate, owned approximately 9.70% of our outstanding common stock. Furthermore, two of our directors have been appointed by our largest stockholder, SPH USA. As a result, such persons or their appointees to our board of directors, acting together, will have the ability to control or significantly influence all matters submitted to our board of directors or stockholders for approval, including the appointment of our management, the election and removal of directors and approval of any significant transaction, as well as our management and business affairs. This concentration of ownership may have the effect of delaying, deferring or preventing a change in control, impeding a merger, consolidation, takeover or other business combination involving us, or discouraging a potential acquiror from making a tender offer or otherwise attempting to obtain control of our business, even if such a transaction would benefit other stockholders.

We do not currently intend to pay dividends on our common stock, and, consequently, your ability to achieve a return on your investment will depend on appreciation, if any, in the price of our common stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, the terms of any future debt agreements may preclude us from paying dividends. Any return to stockholders will therefore be limited to the appreciation of their stock. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

Sales of a substantial number of shares of our common stock by our stockholders in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity securities. As of December 31, 2020, 44,077,016 shares of our outstanding common stock are freely tradable, without restriction, in the public market, unless they are purchased by one of our affiliates.

As of December 31, 2020, up to 8,195,327 shares of common stock that are either subject to outstanding warrants, options or reserved for future issuance under our equity incentive plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended, or the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Provisions in our charter documents and under Delaware law could discourage a takeover that stockholders may consider favorable and may lead to entrenchment of management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could significantly reduce the value of our shares to a potential acquiror or delay or prevent changes in control or changes in our management without the consent of our board of directors. The provisions in our charter documents include the following:

- a classified board of directors with three-year staggered terms, which may delay the ability of stockholders to change the membership of a majority of our board of directors;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- the exclusive right of our board of directors, unless the board of directors' grants such right to the stockholders, to elect a director to fill a vacancy created by the expansion of the board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;
- the prohibition on removal of directors without cause due to the classified board of directors;
- the ability of our board of directors to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquiror;
- the ability of our board of directors to alter our amended and restated bylaws without obtaining stockholder approval;
- the required approval of at least 66-2/3% of the shares entitled to vote to adopt, amend or repeal our amended and restated bylaws or repeal certain provisions of our amended and restated certificate of incorporation;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- an exclusive forum provision providing that the Court of Chancery of the State of Delaware will be the exclusive forum for certain actions and proceedings;

- the requirement that a special meeting of stockholders may be called only by the chairman of the board of directors, the chief executive officer or the board of directors, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors; and

- advance notice procedures that stockholders must comply with in order to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquiror from conducting a solicitation of proxies to elect the acquiror's own slate of directors or otherwise attempting to obtain control of us.

We are also subject to the anti-takeover provisions contained in Section 203 of the Delaware General Corporation Law. Under Section 203, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty, any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. These choice of forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. By agreeing to this provision, however, stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. If a court were to find the choice of forum provisions in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

If the Merger does not qualify as a "reorganization" for U.S. federal income tax purposes, U.S. Holders of our common stock will be required to recognize gain or loss for U.S. federal income tax purposes upon the exchange of their Private Oncternal common stock for our common stock in the Merger.

The U.S. federal income tax consequences of the Merger to U.S. Holders will depend on whether the merger qualifies as a "reorganization" for U.S. federal income tax purposes. Our and Private Oncternal's obligations to effect the Merger were subject to the satisfaction, or waiver, at or prior to the effective time of the Merger, of the condition that each company receive an opinion of counsel, dated as of the closing date of the Merger, to the effect that the Merger will qualify as a "reorganization" within the meaning of Section 368(a) of the Code. If, contrary to the opinions from counsel, the Merger fails to qualify as a reorganization within the meaning of Section 368(a) of the Code, a U.S. Holder of Private Oncternal common stock would recognize gain or loss for U.S. federal income tax purposes on each share of Private Oncternal common stock surrendered in the Merger for our common stock and any cash received in lieu of a fractional share. For purposes of this discussion, a U.S. Holder is a beneficial owner of Oncternal common stock that, for U.S. federal income tax purposes, is or is treated as: an individual who is a citizen or resident of the U.S.; a corporation created or organized under the laws of the U.S., any state thereof, or the District of Columbia; an estate, the income of which is subject to U.S. federal income tax regardless of its source; or a trust that (i) is subject to the primary supervision of a U.S. court and the control of one or more "United States persons" (within the meaning of Section 7701(a)(30) of the Code) over all of its substantial decisions or (ii) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

Our ability to use net operating loss ("NOL") carryforwards and other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire (if at all). As of

December 31, 2020, we had federal and state NOL carryforwards of approximately $69.1 million and $47.7 million, respectively. Approximately $43.4 million of NOLs do not expire and the remaining federal and state NOL carryforward will begin to expire in 2033 and 2032, respectively, unless previously utilized. As of December 31, 2020, we had federal and state research and development credit carryforwards of approximately $1.3 million and $0.9 million, respectively. The federal research and development credit carryforwards will begin expiring in 2034, unless previously utilized. The state research and development credits do not expire.

Under the Tax Act, federal NOLs generated in taxable years ending after December 31, 2017, may be carried forward indefinitely but federal NOLs generated in taxable years beginning after December 31, 2017 may only be used to offset 80% of our taxable income annually. Under Sections 382 and 383 of the Code, our NOL and research and development tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50 percentage points. Our ability to utilize our NOL carryforwards and other tax attributes to offset future taxable income or tax liabilities may be limited as a result of ownership changes, including changes resulting from our Merger. We have not yet determined the amount of the cumulative change in our ownership resulting from the Merger or other transactions, or any resulting limitations on our ability to utilize our NOL carryforwards and other tax attributes. If we earn taxable income, such limitations could result in increased future tax liability to us and our future cash flows could be adversely affected. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

U.S. tax legislation may materially adversely affect our financial condition, results of operations and cash flows.

The Tax Act has significantly changed the U.S. federal income taxation of U.S. corporations, including by reducing the U.S. corporate income tax rate and revising the rules governing NOLs. Many of these changes became effective beginning in 2018, without any transition periods or grandfathering for existing transactions. The legislation is unclear in many respects and could be subject to potential amendments and technical corrections, as well as interpretations and implementing regulations by the U.S. Treasury Department and the IRS, any of which could lessen or increase certain adverse impacts of the legislation. In addition, it is unclear how these U.S. federal income tax changes will affect state and local taxation, which often uses federal taxable income as a starting point for computing state and local tax liabilities. There may be other material adverse effects resulting from the legislation that we have not yet identified. While some of the changes made by the tax legislation may adversely affect us in one or more reporting periods and prospectively, other changes may be beneficial on a going forward basis. We continue to work with our tax advisors to determine the full impact that the recent tax legislation as a whole will have on us. We urge our investors to consult with their legal and tax advisors with respect to such legislation.

Our stockholders prior to the Merger who hold CVRs may not receive any payment on the CVRs and the CVRs may otherwise expire valueless.

We and certain other parties have entered into a CVR Agreement, dated as of June 7, 2019, or the CVR Agreement, pursuant to which, for each share of GTx common stock held, stockholders of record as of immediately prior to the Merger received one contingent value right, or CVR, entitling such holders to receive in the aggregate 75% of any net proceeds received during the 15-year period after the closing of the Merger from the grant, sale or transfer of rights to our SARD or SARM technology that occurs during the 10-year period after the closing of the Merger (or in the 11th year if based on a term sheet approved during the initial 10-year period) and, if applicable, to receive royalties on the sale of any SARD products or SARM products by us during the 15-year period after the closing of the Merger.

In 2018, we announced that we had ceased development of the SARM technology following the failure of a Phase 2 clinical study of enobosarm to achieve statistical significance with respect to the primary endpoint of the study. Effective March 31, 2020, we terminated the amended and restated license agreement with UTRF for the development and production of the SARM technology, and we no longer have: (i) the obligation to make further payments to UTRF under the SARM license agreement, including payments for patent prosecution and maintenance, and (ii) any rights to develop or sublicense the SARM technology.

The CVRs are not transferable, will not have any voting or dividend rights, and interest will not accrue on any amounts potentially payable on the CVRs. Accordingly, the right of any stockholder of record as of immediately prior to the Merger to receive any future payment on or derive any value from the CVRs will be contingent solely upon the achievement of the foregoing events within the time periods specified in the CVR Agreement and if these events are not achieved for any reason within the time periods specified in the CVR Agreement, no payments will be made under the CVRs, and the CVRs will expire valueless. In addition, we (as successor in interest to GTx) have agreed only to use commercially reasonable efforts to develop SARD products, subject to certain limitations, which allows for the consideration of a variety of factors in determining the efforts that we are required to use to develop SARD products, and we are not required to take all possible actions to continue efforts to develop SARD products. Accordingly, under certain circumstances we may not be required to continue efforts to develop SARD products, or may allocate resources to other projects, which would have an adverse effect on the value, if any, of the CVRs. Furthermore, the CVRs will be unsecured obligations of our company and all payments under the CVRs, all other obligations under the CVR Agreement and the CVRs and any rights or claims relating thereto will be subordinated in right of payment to the prior payment in full of all of our current or future senior obligations. Finally, the U.S. federal income tax treatment of the CVRs is unclear. There is no legal authority directly addressing the U.S. federal income tax treatment of the receipt of, and payments on, the CVRs, and there can be no assurance that the IRS, would not assert, or that a court would not sustain, a position that could result in adverse U.S. federal income tax consequences to holders of the CVRs.

General Risk Factors

Our failure to meet the continued listing requirements of Nasdaq could result in a delisting of our common stock.

If we fail to satisfy the continued listing requirements of Nasdaq, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action we take to restore compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the Nasdaq minimum bid price requirement or prevent future non-compliance with Nasdaq's listing requirements.

We incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. We are subject to the reporting requirements of the Exchange Act, which will require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, Sarbanes-Oxley, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of Sarbanes-Oxley, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, the SEC has adopted additional rules and regulations in these areas, such as mandatory "say on pay" voting requirements that apply to us. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time consuming and costly. If these requirements

divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will increase our net loss and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If securities or industry analysts do not publish research or reports or publish unfavorable research or reports about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us, our business, our market or our competitors. We do not currently have and may never obtain research coverage by securities and industry analysts. If no securities or industry analysts commence coverage of our company, the trading price for our common stock would be negatively impacted. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our stock, our stock price would likely decline. If one or more of these analysts ceases to cover us or fails to regularly publish reports on us, interest in our stock could decrease, which could cause our stock price or trading volume to decline.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, investors may lose confidence in our financial reporting and the trading price of our common stock may decline.

Pursuant to Section 404 of Sarbanes-Oxley, we are required to report upon the effectiveness of our internal control over financial reporting. Additionally, our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we have been required to upgrade our information technology systems; implement additional financial and management controls, reporting systems and procedures; and hire additional accounting and finance staff. If we or, if required, our auditors are unable to conclude that our internal control over financial reporting is effective, investors may lose confidence in our financial reporting and the trading price of our common stock may decline.

We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

We may become involved in the future, in securities class action litigation that could divert management's attention, adversely affect our business and subject us to significant liabilities.

In the past, securities class action litigation has often been brought against a company following volatility in the market price of its securities. This risk is especially relevant for us, because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years.

Any future lawsuits to which we may become a party are subject to inherent uncertainties and will likely be expensive and time-consuming to investigate, defend and resolve, and will divert our management's attention and financial and other resources. The outcome of litigation is necessarily uncertain, and we could be forced to expend significant resources in the defense of future lawsuits, and we may not prevail. Any litigation to which we may become a party may result in an onerous or unfavorable judgment that may not be reversed upon appeal or in

payments of substantial monetary damages or fines, or we may decide to lawsuits on similarly unfavorable terms, which could adversely affect our business, financial condition, results of operations or stock price.

Item 1B. Unresolved Staff Comments.

Not applicable.

Item 2. Properties.

Our principal executive offices are located in San Diego, California, where we currently sublease 4,677 square feet of office space used primarily for corporate, research, development, clinical, regulatory, manufacturing and quality functions. Our sublease for this facility expires on March 31, 2021, and we are actively negotiating an extended lease arrangement for the same office space.

Item 3. Legal Proceedings.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5.Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information.

Our common stock is listed on The Nasdaq Capital Market under the ticker symbol "ONCT". As of March 3, 2021, there were approximately 130 holders of record of our common stock. This number was derived from our stockholder records and does not include beneficial owners of our common stock whose shares are held in the name of various dealers, clearing agencies, banks, brokers and other fiduciaries.

Dividend Policy.

We have never declared or paid any dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future.

Recent Sales of Unregistered Securities.

None.

Item 6.Selected Financial Data.

Reserved.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes and other financial information included elsewhere in this Annual Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business and future financial performance, includes forward-looking statements that are based upon current beliefs, plans and expectations and involve risks, uncertainties and assumptions. You should review the "Risk Factors" section of this Annual Report for a discussion of important factors that could cause our actual results and the timing of selected events to differ materially from those described in or implied by the forward-looking statements contained in the following discussion and analysis. Please also see the section within Part I of this Annual Report entitled "Forward-Looking Statements."

Unless otherwise indicated or as the context otherwise requires, the historical financial information included in this Management's Discussion and Analysis of Financial Condition and Results of Operations is that of Private Oncternal prior to the Merger because Private Oncternal was deemed to be the accounting acquirer in the Merger for financial reporting purposes.

Overview

We are a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies for cancers with critical unmet medical need. Our development efforts are focused on promising, yet untapped, biological pathways implicated in cancer generation or progression. Our pipeline includes cirmtuzumab, an investigational monoclonal antibody that is designed to inhibit Receptor tyrosine kinase-like Orphan Receptor 1, or ROR1, a growth factor receptor that is widely expressed on many tumors and that activates pathways leading to increased tumor proliferation, invasiveness and drug resistance. Cirmtuzumab is being evaluated in a Phase 1/2 clinical trial in combination with ibrutinib (Imbruvica®) (Cirmtuzumab and Ibrutinib targeting ROR1 for Leukemia and Lymphoma, or CIRLL), for the treatment of patients with B-cell lymphoid malignancies, including mantle cell lymphoma, or MCL, and chronic lymphocytic leukemia, or CLL, and in an investigator-sponsored, Phase 1b clinical trial in combination with paclitaxel for the treatment of women with HER2-negative metastatic or locally advanced, unresectable breast cancer. We are developing a chimeric antigen receptor T cell, or CAR-T, therapy candidate that targets ROR1, which is currently in preclinical development as a potential treatment for hematologic cancers and solid tumors. In addition, we are developing TK216, an investigational small molecule that is designed to inhibit the ETS, or E26 Transformation Specific, family of oncoproteins, which have been shown in preclinical studies to alter gene transcription and RNA processing and lead to increased cell proliferation and invasion. TK216 is being evaluated in a Phase 1 clinical trial as a single agent and in combination with vincristine in patients with relapsed or refractory Ewing sarcoma, a rare pediatric cancer.

The U.S. Food and Drug Administration, or FDA, has granted orphan drug designations for cirmtuzumab for the treatment of MCL and for the treatment of CLL/small lymphocytic lymphoma, and has granted rare pediatric disease designation, as well as orphan drug and fast track designations for TK216 for the treatment of Ewing Sarcoma.

In June 2020, we announced an updated clinical strategy for cirmtuzumab that prioritizes development in MCL, based on encouraging interim clinical results from the CIRLL Phase 1/2 clinical trial that were presented at the ASCO 2020 annual meeting. As a result, we amended the CIRLL study to increase the number of patients with relapsed/refractory MCL to be enrolled in the Phase 2 expansion cohort to at least 20 patients and to allow the enrollment of patients with a broader range of prior BTK inhibitor treatments. In addition, we limited the total enrollment of CLL patients in the randomized Phase 2 CLL cohort of the CIRLL study to 28 patients, in order to focus resources on the MCL portion of the study. In September 2020, we met with the FDA and are in dialogue with the FDA regarding potential accelerated approval pathways for cirmtuzumab plus ibrutinib in patients with relapsed/refractory MCL. Additionally, we are supporting a new, investigator-sponsored Phase 2 clinical trial of cirmtuzumab in combination with venetoclax, a Bcl-2 inhibitor, in patients with relapsed/refractory CLL in collaboration with the UC San Diego School of Medicine, or UC San Diego.

Since Private Oncternal's inception in 2013, we have devoted most of our resources to organizing and staffing, business planning, raising capital, acquiring product candidates and securing related intellectual property rights and advancing our cirmtuzumab and TK216 clinical development programs. Under research subaward agreements between us and UC San Diego, we are eligible to receive approximately $14.0 million in development milestones throughout the award project period, estimated to be from October 1, 2017 to March 31, 2022. Through December 31, 2020, we have funded our operations primarily through: (i) gross proceeds of $125.0 million from the issuance of common stock, (ii) gross proceeds of $49.0 million from the issuance of convertible preferred stock, (iii) receipt of $11.6 million in subaward grant payments received from UC San Diego, and (iv) cash proceeds of $18.3 million received in connection with the closing of the Merger described in Part I, Item 1, "Merger" of this Annual Report. As of December 31, 2020, we had cash and cash equivalents of $116.7 million.

We have incurred net losses in each year since inception. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our current or future product candidates. Our net losses were $17.2 million and $34.2 million (including $18.1 million related to nonrecurring merger costs) for the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, we had an accumulated deficit of $82.8 million. Substantially all of our net losses have resulted from costs incurred in connection with: (i) advancing our research and development programs, (ii) general and administrative costs associated with our operations, including the costs associated with operating as a public company, and (iii) in-process research and development costs associated with the Merger. We expect to continue to incur significant and increasing operating losses for at least the next several years. We expect that our expenses and capital funding requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- advance cirmtuzumab through clinical development in multiple indications, with a primary focus in MCL;
- generate clinical proof-of-concept data with TK216 in Ewing sarcoma, an orphan pediatric cancer indication;
- advance our ROR1-targeting CAR-T therapy candidate to clinical development, initially in hematological cancers and then in solid tumors;
- respond to the impacts of the COVID-19 pandemic, which has slowed enrollment into our clinical trials;
- evaluate cirmtuzumab in additional ROR1-positive solid tumors;
- evaluate TK216 in additional tumors with ETS fusion proteins or overexpression;
- continue to develop additional product candidates;
- acquire or in-license other product candidates and technologies;
- maintain, expand and protect our intellectual property portfolio;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval; and
- add operational, financial and management information systems and personnel, including personnel to support our planned product development and future commercialization efforts, as well as to support our transition to a public reporting company.

We will not generate product sales revenue unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. If we obtain regulatory approval for any of our product candidates and do not enter into a commercialization partnership, we expect to incur significant expenses related to developing our internal commercialization capability to support product sales, marketing and distribution. In addition, we expect to incur additional costs associated with operating as a public company.

As a result, we believe we will need substantial additional funding to support our continuing operations and pursue our business strategy. Until such time as we can generate significant product sales revenue, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, government funding, or other sources, including potentially collaborations, licenses and other similar arrangements. We may not be able to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly delay, reduce or eliminate the development and commercialization of one or more of our product candidates or delay our pursuit of potential in licenses or acquisitions.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

We expect that our existing cash and cash equivalents will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. Beyond that point, we will need to raise additional capital to finance our operations, which cannot be assured.

Business Update Regarding COVID-19

The COVID-19 worldwide pandemic has presented substantial public health and economic challenges and is affecting economies, financial markets and business operations around the world. International and U.S. governmental authorities in impacted regions have taken actions in an effort to slow the spread of COVID-19, including issuing and modifying varying forms of “stay-at-home” orders, and restricting business functions outside of one’s home. In response, we have put restrictions on employee travel and working from our executive offices, with many employees continuing their work remotely. To date, we have been able to continue to supply cirmtuzumab and TK216 clinical trial sites for patients enrolled in our ongoing clinical trials and do not currently anticipate any interruptions in the supply of cirmtuzumab or TK216. While we are continuing the clinical trials we have underway in sites across the U.S., we expect that COVID-19 precautions may directly or indirectly impact the timeline for some of our clinical trials. For example, some of our clinical trial sites, including those located in areas severely impacted by the pandemic, have placed new patient enrollment into clinical trials on hold or, for patients travelling from out-of-state, have implemented a 14-day self-quarantine before appointments. For our existing patients, we are actively working with all of our clinical trial sites to minimize disruptions and address concerns on an individual site or patient basis in order to allow participating patients to continue to receive treatment at home or in alternative healthcare settings while minimizing their potential exposure to the virus that causes COVID-19. If restrictions related to the COVID-19 outbreak continue for a prolonged period of time or if additional clinical trial sites pause patient enrollment or treatments, our clinical trial milestones would be negatively impacted. Additionally, our expectations for the timing of first-in-human dosing of our ROR1 CAR-T therapy in China has been delayed. Any delays in the completion of our clinical trials and any disruption in our supply chain could have a material adverse effect on our business, results of operations and financial condition. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain or treat it, the success or failure of vaccination programs, the emergence of new variants of COVID-19, as well as the economic impact on local, regional, national and international markets.

Components of Results of Operations

Grant Revenue

We have not and do not expect to generate any product sales revenue in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate product sales revenue in the future. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates. Our grant revenue has been derived from a California Institute for Regenerative Medicine, or CIRM, grant subaward with UC San Diego.

In August 2017, CIRM awarded an $18.3 million grant to researchers at UC San Diego to advance our Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib for the treatment of patients with B-cell lymphoid malignancies, including MCL and CLL. Oncternal is conducting this study in collaboration with UC San Diego and estimates it will receive approximately $14.0 million in development milestones under research subaward agreements throughout the award project period, estimated to be from October 1, 2017 to March 31, 2022. In addition, we are committed to certain co-funding requirements and are required to provide UC San Diego progress and financial update reports throughout the award project period. We received subaward payments of $1.4 million and $6.2 million in the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, we believe we have met our obligations under the CIRM award and UC San Diego subawards.

Operating Expenses

Research and Development

Research and development expenses consist primarily of costs incurred for the preclinical and clinical development of our product candidates, cirmtuzumab, TK216 and our ROR1-targeting CAR-T therapy candidate, which include:

- expenses under agreements with third-party contract organizations, investigative clinical trial sites that conduct research and development activities on our behalf;
- costs related to develop and manufacture preclinical study and clinical trial material;
- salaries and employee-related costs, including stock-based compensation;
- costs incurred under our collaboration and third-party licensing agreements; and
- laboratory and vendor expenses related to the execution of preclinical and clinical trials.

We accrue all research and development costs in the period for which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors, collaborators and third-party service providers. Advance payments for goods or services to be received in future periods for use in research and development activities are deferred and then expensed as the related goods are delivered and as services are performed. Any unearned advances would be refunded when known.

We expect our research and development expenses to increase substantially for the foreseeable future as we: (i) invest in additional operational personnel to support our planned product development efforts, and (ii) continue to invest in developing our product candidates preclinically, advance them into later stages of clinical development, and as we begin to conduct larger clinical trials. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

Our direct research and development expenses are tracked by product candidate and consist primarily of external costs, such as fees paid under third-party license agreements and to outside consultants, contract research organizations, or CROs, contract manufacturing organizations and research laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. We do not allocate employee costs and costs associated with our discovery efforts, laboratory supplies and facilities, including

other indirect costs, to specific product candidates because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to conduct our research as well as for managing our preclinical development, process development, manufacturing and clinical development activities. These employees work across multiple programs and, therefore, we do not track our costs by product candidate unless we can include them as subaward costs.

We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development, including any potential expanded dosing beyond the original protocols based in part on ongoing clinical success and the potential effects of the COVID-19 pandemic. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments of each product candidate's commercial potential. We will need to raise substantial additional capital in the future. In addition, we cannot forecast which product candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements.

General and Administrative

General and administrative expenses consist primarily of personnel-related costs, insurance costs, facility costs and professional fees for legal, patent, consulting, investor and public relations, accounting and audit services. Personnel-related costs consist of salaries, benefits and stock-based compensation. We expect our general and administrative expenses will increase substantially as we: (i) incur additional costs associated with being a public company, including audit, legal, regulatory, and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums, and investor relations costs, (ii) hire additional personnel, and (iii) protect our intellectual property.

Other Income (Expense)

Change in Fair Value of Preferred Stock Warrant Liability

In connection with Private Oncternal's Series B-2 preferred stock financing in 2017, Private Oncternal issued warrants to purchase shares of its Series B-2 preferred stock. We classified these warrants as a liability on our consolidated balance sheets and remeasured them to fair value at each reporting date, and we recognized changes in the fair value of the warrant liability as a component of other income (expense), net in our consolidated statements of operations.

Upon the closing of the Merger, all outstanding warrants to purchase Private Oncternal Series B-2 preferred stock were converted into warrants to purchase our common stock. As a result, such warrants no longer require liability accounting and the fair value of the warrant liability has been reclassified to stockholders' equity.

Paycheck Protection Program Loan

We received a loan under the Paycheck Protection Program, or PPP, a program implemented by the U.S. Small Business Administration, or SBA, under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act. In December 2020, the underlying principal and interest were fully forgiven by the SBA and the Company has no further obligations thereunder. The loan forgiveness was recorded as other income in the consolidated statement of operations.

Interest Income

Interest income consists of interest earned on our cash equivalents, which consist of money market funds. Our interest income has not been significant due to low interest earned on invested balances.

Results of Operations

Comparison of the Years Ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019 (in thousands):

	Years Ended December 31,		
	2020	2019	Change
Grant revenues	$ 3,375	$ 2,425	$ 950
Operating expenses:			
Research and development	12,544	10,159	2,385
In-process research and development	—	18,088	(18,088)
General and administrative	8,373	7,286	1,087
Total operating expenses	20,917	35,533	(14,616)
Loss from operations	(17,542)	(33,108)	15,566
Other income (expense):			
Change in fair value of warrant liability	—	(1,268)	1,268
Other income	301	—	301
Interest income	16	188	(172)
Total other income (expense)	317	(1,080)	1,397
Net loss	$ (17,225)	$ (34,188)	$ 16,963

Grant Revenue

Grant revenue for the year ended December 31, 2020 was $3.4 million, compared to $2.4 million for the year ended December 31, 2019. The increase of $1.0 million was primarily due to an increase in the revenue recognition rate.

Research and Development Expenses

The following table summarizes our research and development expenses for the periods indicated (in thousands):

	Years Ended December 31,		Increase/ (Decrease)
	2020	2019	
Cirmtuzumab	$ 6,359	$ 6,156	$ 203
TK216	2,340	1,101	1,239
Other	303	408	(105)
Unallocated research and development expenses	3,542	2,494	1,048
Total research and development expenses	$ 12,544	$ 10,159	$ 2,385

Research and development expenses for the years ended December 31, 2020 and 2019 were $12.5 million and $10.2 million, respectively, an increase of $2.4 million. The increase was primarily due to: (i) a $1.3 million net increase in direct product candidate costs, and (ii) a $1.0 million increase in unallocated research and development expenses.

Direct expenses for cirmtuzumab increased $0.2 million for the year ended December 31, 2020, compared to the year ended December 31, 2019, primarily due to the following partially offsetting factors: (i) a $0.2 million increase in preclinical study costs, (ii) a $0.7 million increase in manufacturing development costs, and (iii) a $0.7 million decrease in license and milestone fees under the Regents License Agreement, as defined below.

Direct expenses for TK216 increased $1.2 million for the year ended December 31, 2020, compared to the year ended December 31, 2019, primarily due to an increase in clinical trial costs related to our ongoing Phase 1 clinical trial of TK216 in refractory Ewing sarcoma.

Unallocated expenses increased $1.0 million for the year ended December 31, 2020, compared to the year ended December 31, 2019, primarily due to higher personnel costs, including higher share-based compensation expense of $0.3 million.

In-Process Research and Development Expenses

In-process research and development expenses decreased $18.1 million for year ended December 31, 2020, compared to the year ended December 31, 2019, due solely to the completion of the Merger in 2019.

General and Administrative Expenses

General and administrative expenses for the years ended December 31, 2020 and 2019 were $8.4 million and $7.3 million, respectively, an increase of $1.1 million. The increase is primarily due to the following partially offsetting factors: (i) higher personnel and professional related costs of $1.3 million, including higher share-based compensation expense of $0.7 million, (ii) higher director's and officer's insurance costs of $0.6 million, (iii) lower patent costs of $0.3 million, and (iv) lower public company and other expenses of $0.5 million.

Other Income (Expense)

Other income of $0.3 million in 2020 was primarily related to the forgiveness of the PPP loan. Other expense of $1.3 million in 2019 was primarily related to the change in fair value of warrant liability.

Liquidity and Capital Resources

We have incurred losses and negative cash flows from operations since inception. As of December 31, 2020, we had an accumulated deficit of $82.8 million and anticipate that we will continue to incur net losses for the foreseeable future. As of December 31, 2020, we had $116.7 million in cash and cash equivalents. We believe we have sufficient cash to fund our projected operating requirements into 2023.

Cash Flows

The following table summarizes our net cash flow activity for each of the periods presented (in thousands):

	Years Ended December 31,	
	2020	2019
Net cash provided by (used in):		
Operating activities	$ (17,495)	$ (16,746)
Investing activities	—	16,137
Financing activities	114,181	15
Increase in cash and cash equivalents	$ 96,686	$ (594)

Operating Activities

During the year ended December 31, 2020, operating activities used $17.5 million, resulting from our net loss of $17.2 million, which included net non-cash charges of $1.4 million related to stock-based compensation, lease expense, and forgiveness of the paycheck protection program loan, offset by a $1.7 million change in our operating assets and liabilities. The $1.7 million change in operating assets and liabilities primarily consisted of the following partially offsetting activities: (i) a $2.0 million decrease in deferred revenue, (ii) a $0.7 million increase in prepaid and other assets and operating lease liability, and (iii) a $1.0 million increase in accounts payable and accrued expenses.

During the year ended December 31, 2019, operating activities used $16.7 million of cash, resulting from our net loss of $34.2 million, which included non-cash charges of: (i) $18.1 million related to the acquisition of in-process research and development in connection with the Merger, (ii) $1.3 million related to the change in fair value of warrant liability, and (iii) $0.5 million related to stock-based compensation charges; partially offset by a $2.4 million change in operating assets and liabilities. The net $2.4 million change in operating assets and liabilities primarily consisted of a $6.0 million decrease in accounts payable and accrued liabilities and a $3.6 million increase in deferred revenue.

Investing Activities

Net cash provided by investing activities was none for the year ended December 31, 2020. Net cash provided by investing activities was $16.1 million for the year ended December 31, 2019, primarily resulting from cash received in connection with the Merger.

Financing Activities

Financing activities provided cash of $114.2 million for the year ended December 31, 2020, which primarily consisted of $113.9 million in net proceeds received from various public offerings. Financing activities provided cash of $15,000 for the year ended December 31, 2019, consisting of option and warrant exercises.

On May 21, 2020, we completed a registered direct offering pursuant to which we concurrently sold: (i) 1,943,636 shares of our common stock, and (ii) warrants to purchase up to an aggregate of 971,818 shares of common stock, at a combined price of $2.5725 per share. The net proceeds to us from this offering were $4.4 million, after deducting placement agent's cash commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the warrants.

On July 21, 2020, we completed a registered direct offering pursuant to which we concurrently sold: (i) 2,581,867 shares of our common stock, and (ii) warrants to purchase up to an aggregate of 1,290,933 shares of common stock, at a combined price of $2.3825 per share. The net proceeds to us from this offering were $5.7 million, after deducting the placement agent's cash commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the warrants.

On September 1, 2020, we completed an underwritten public offering pursuant to which we sold 2,428,886 shares of our common stock at a price to the public of $2.10 per share. The net proceeds to us from this offering were $4.4 million, after deducting the underwriter's discounts and commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the underwriter's warrants.

On November 20, 2020, we completed an underwritten public offering pursuant to which we sold 7,725,065 shares of our common stock at a price to the public of $3.10 per share. The net proceeds to us from this offering were $20.4 million, after deducting the underwriter's discounts and commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the underwriter's warrants.

On December 14, 2020, we completed an underwritten public offering pursuant to which we sold 19,161,667 shares of our common stock at a price to the public of $4.50 per share. The net proceeds to us from this offering were $79.0 million, after deducting the underwriter's discounts and commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the underwriter's warrants.

Operating Capital Requirements

We anticipate that we will continue to incur losses for the foreseeable future, and we expect the losses to increase as we continue the research and development of, and seek regulatory approvals for, our product candidates and conduct additional research and development activities. Our product candidates have not yet achieved regulatory approval and we may not be successful in achieving commercialization of our product candidates.

We believe our existing cash and cash equivalents as of March 11, 2021 will be sufficient to fund our projected operating requirements into 2023. However, our forecast of the period of time through which our financial resources will be adequate to support our planned operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. For example, the FDA or other regulatory authorities may require us to generate additional data or conduct additional preclinical studies or clinical trials, or may impose other requirements beyond those that we currently anticipate. Additionally, it is possible for a product candidate to show promising results in preclinical studies or in clinical trials, but fail to establish sufficient safety and efficacy data necessary to obtain regulatory approvals. As a result of these and other risks and uncertainties and the probability of success, the duration and the cost of our research and development activities required to advance a product candidate cannot be accurately estimated and are subject to considerable variation. We may encounter difficulties, complications, delays and other unknown factors and unforeseen expenses in the course of our research and development activities, any of which may significantly increase our capital requirements and could adversely affect our liquidity.

We will require additional capital for the research and development of our product candidates and to perform our research and development obligations under our collaboration agreement with UC San Diego, and we may be forced to seek additional funds sooner than expected to pursue our research and development activities. We expect to finance our capital requirements in the foreseeable future through a combination of the sale of public or private equity or debt securities, government funding, or other sources, including potentially collaborations, licenses and other similar arrangements. There can be no assurance that we will be able to obtain any sources of financing on acceptable terms, or at all. To the extent that we can raise additional funds by issuing equity securities, our stockholders may experience significant dilution. Any debt financing, if available, may involve restrictive covenants that may impact our ability to conduct our business. Any of these events could significantly harm our business, operations, financial condition and prospects.

Our forecast of the period of time through which our existing cash and cash equivalents and investments will be adequate to support our operations is a forward-looking statement and involves significant risks and uncertainties. We have based this forecast on assumptions that may prove to be wrong, and actual results could vary materially from our expectations, which may adversely affect our capital resources and liquidity. We could utilize our available capital resources sooner than we currently expect. The amount and timing of future funding requirements, both near- and long-term, will depend on many factors, including, but not limited to:

- the type, number, scope, progress, expansions, results, costs and timing of our preclinical studies and clinical trials of our product candidates which we are pursuing or may choose to pursue in the future;
- the costs incurred as a result of the COVID-19 pandemic, including clinical trial delays; the costs and timing of manufacturing for our product candidates, including commercial manufacturing if any product candidate is approved;
- the costs of obtaining ibrutinib, for which we currently obtain supply at no cost under our clinical supply agreement with Pharmacyclics LLC, and vincristine to conduct our clinical trials of cirmtuzumab and TK216, respectively;
- the costs, timing and outcome of seeking and obtaining regulatory approvals our product candidates;
- the costs of obtaining, maintaining and enforcing our patents and other intellectual property rights;
- the costs associated with hiring additional personnel and consultants as our preclinical and clinical activities increase;
- the costs and timing of establishing or securing sales and marketing capabilities if any product candidate is approved;

- our ability to achieve sufficient market acceptance, adequate coverage and reimbursement from third-party payors and adequate market share and revenue for any approved products;
- the terms and timing of establishing and maintaining collaborations, licenses and other similar arrangements, including milestone or other payments under our existing in-license agreements and any in-license agreements that we may enter into in the future;
- costs associated with any products or technologies that we may in-license or acquire; and
- the cost of establishing sales, marketing, manufacturing and distribution capabilities for, and the pricing and reimbursement of, any products for which we may receive regulatory approval.

If we cannot continue or expand our research and development operations, or otherwise capitalize on our business opportunities, because we lack sufficient capital, our business, operations, financial condition and prospects could be materially adversely affected.

In February 2018, we entered into the ATM Sales Agreement, pursuant to which we were able to offer and sell, from time to time, shares of our common stock having an aggregate offering price of up to $50.0 million. In August 2020, we terminated the ATM Sales Agreement and can no longer offer and sell shares under the ATM Sales Agreement. In connection with our sale of common stock and common stock warrants in July 2020, we agreed not to enter into other variable rate transactions prior to July 21, 2021, other than pursuant to an at-the-market offering facility with the placement agent. We do not currently have an active at-the-market facility available to us.

Under current SEC regulations, if at any time our public float is less than $75.0 million, and for so long as our public float remains less than $75.0 million, the amount we can raise through primary public offerings of securities in any twelve-month period using shelf registration statements is limited to an aggregate of one-third of our public float, which is referred to as the baby shelf rules. As of December 31, 2020, our calculated public float exceeded $75.0 million. In January 2021, our Form S-3 registration statement expired and we no longer have an effective Form S-3 shelf registration statement available. Future sales of our common stock, if any, will depend on a variety of factors including, but not limited to, the expected timing for achieving key milestones, including completing and announcing results of clinical trials of cirmtuzumab and TK216 and announcing the first-in-human dosing of our CAR-T product candidate targeting ROR-1, currently in preclinical development, prevailing market conditions, the trading price of our common stock and our capital needs. There can be no assurance that we will be successful in consummating future sales of our securities based on prevailing market conditions or in the quantities or at the prices that we deem appropriate or that we will enter into a new at-the-market offering facility.

Contractual Obligations and Commitments

We are party to a number of license agreements, pursuant to which we have payment obligations that are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones and are required to make royalty payments in connection with the sale of products developed under those agreements. As of December 31, 2020, we were unable to estimate the timing or likelihood of achieving the milestones or making future product sales. See Note 4 to our consolidated financial statements included elsewhere in this Annual Report for a description of these agreements.

We enter into contracts in the normal course of business with clinical trial sites and clinical supply manufacturers and with vendors for preclinical studies, research supplies and other services and products for operating purposes. These contracts generally provide for termination after a notice period, and, therefore, are cancelable contracts.

Critical Accounting Policies

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles ("U.S. GAAP"). The preparation of the financial statements requires us to make estimates and judgments that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods.

Our estimates are based on our historical trends and other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are described in more detail in Note 1, "*Description of Business, Basis of Presentation and Summary of Significant Accounting Policies*," in the notes to our consolidated financial statements as of December 31, 2020 and 2019 and for each of the years ended December 31, 2020 and 2019, appearing elsewhere in this Annual Report. However, we believe that the following accounting policies are the most critical for fully understanding and evaluating our financial condition and results of operations.

Research and Development Expenses and Accruals

Research and development expenses consist of costs incurred for our own and for sponsored and collaborative research and development activities. Research and development costs are expensed as incurred and include manufacturing process development costs, manufacturing costs, costs associated with preclinical studies and clinical trials, regulatory and medical affairs activities, quality assurance activities, salaries and benefits, including stock-based compensation, fees paid to third-party consultants, license fees and overhead.

We have entered into various research and development contracts with research institutions, clinical research organizations, clinical manufacturing organizations and other companies. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and payments made in advance of performance are reflected in the consolidated balance sheets as prepaid expenses and other assets or accrued liabilities. We record accruals for estimated costs incurred for ongoing research and development activities. When evaluating the adequacy of the accrued liabilities, we analyze progress of the services, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the prepaid or accrued balances at the end of any reporting period. Actual results could differ from our estimates.

Valuation of Warrants to Purchase Convertible Preferred Stock

Prior to the Merger, we classified outstanding warrants to purchase shares of our Series B-2 convertible preferred stock as a liability on our consolidated balance sheets as these warrants were free-standing financial instruments exercisable into contingently redeemable shares. The warrants were initially recorded at fair value on the date of grant, and were subsequently remeasured to fair value at each balance sheet date while the instrument was outstanding. Changes in the fair value of these warrants were recognized as a component of other income (expense) in our consolidated statements of operations. Upon the completion of the Merger, the Series B-2 warrants were amended such that they were converted into warrants to purchase our common stock. As amended, warrant liability accounting is no longer required and the fair value of the warrant liability has been reclassified into stockholders' equity.

Revenue Recognition

We currently generate revenue from a research subaward agreement with UC San Diego, which provides us with payments for certain types of expenditures in return for research and development activities over a contractually defined period. Revenue from such subaward is recognized in the period during which the related qualifying costs are incurred and services are rendered, provided that the applicable conditions under the subaward agreement have been met.

Stock-Based Compensation Expense

Stock-based compensation expense represents the estimated grant date fair value of stock option awards and employee stock purchase plan rights amortized over the requisite service period of the awards (usually the vesting period) primarily on a straight-line basis. We estimate the fair value of all stock option grants using the Black-Scholes option pricing model and recognize forfeitures as they occur.

Estimating the fair value of equity awards as of the grant date using valuation models, such as the Black-Scholes option pricing model, is affected by assumptions regarding a number of complex variables, including the expected stock price volatility, the risk-free interest rate, the expected term of stock options, the expected dividend yield and the fair value of the underlying common stock on the date of grant. Changes in the assumptions can materially affect the fair value and ultimately how much stock-based compensation expense is recognized. These inputs are subjective and generally require significant analysis and judgment to develop.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under SEC rules.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and are not required to provide the information required under this item.

Item 8. Financial Statement and Other Supplementary Information.

The Consolidated Financial Statements and supplementary data of Oncternal Therapeutics, Inc. required by this Item are described in Item 15 of this Annual Report on Form 10-K and are presented beginning on page F-1.

Report of Independent Registered Public Accounting Firm

Shareholders and Board of Directors
Oncternal Therapeutics, Inc.
San Diego, California

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Oncternal Therapeutics, Inc. and subsidiaries (the "Company") as of December 31, 2020 and 2019, the related consolidated statements of operations, convertible preferred stock and stockholders' equity (deficit), and cash flows for the years then ended, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2020 and 2019, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing separate opinions on the critical audit matter or on the accounts or disclosures to which it relates.

Clinical Trial Accruals

As described in Notes 1 and 2 to the consolidated financial statements, the Company has recorded $1.0 million for accrued clinical trial expenses for the estimated costs incurred but not yet billed or paid as of December 31, 2020. When payment terms under the related contracts do not align with the pattern of costs incurred, the Company is required to make estimates of the outstanding obligations as of period end. When evaluating the adequacy of the accrued clinical trial liabilities, the Company analyzes progress of the services, including the phase or completion of events, invoices received and contracted costs, which could involve significant judgments, estimates and specialized knowledge.

We identified clinical trial accruals as a critical audit matter due to the application of significant management judgement over the estimate of services provided as of year-end. Specifically, the amount of accrued clinical trial expenses recognized is sensitive to the availability of information to make the estimate, including the estimate of the progress of the clinical trial and the level of effort expended including patient enrollment and follow-up activity as of the balance sheet date and the associated cost of such services. Auditing these elements involved especially subjective auditor judgment due to the nature of the audit evidence available to address these matters.

The primary procedures we performed to address this critical audit matter included:

- Reviewing the Company’s contractual agreements with third parties and any change orders to assess the impact to the amounts recorded including changes in scope and timing.
- Testing clinical accrual cutoff and evaluating the completeness and valuation of clinical accruals by comparing invoices received by the Company subsequent to December 31, 2020 to the amounts recognized by the Company as of that date.
- Testing clinical accrual for completeness and accuracy by confirming amounts due at December 31, 2020 directly with clinical research organizations (CROs) including total expenses incurred for all services provided by the CRO during 2020.

/s/ BDO USA, LLP

We have served as the Company's auditor since 2016.

San Diego, California

March 11, 2021

Oncternal Therapeutics, Inc.
Consolidated Balance Sheets
(in thousands, except par value)

	December 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 116,737	$ 20,051
Prepaid and other	1,266	736
Total current assets	118,003	20,787
Right-of-use asset	40	190
Other assets	766	767
Total assets	$ 118,809	$ 21,744
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,143	$ 871
Accrued liabilities	3,042	2,731
Deferred grant revenue	1,633	3,640
Lease, current	40	99
Total current liabilities	5,858	7,341
Lease, net of current	—	91
Commitments and contingencies (Note 3)		
Stockholders' equity:		
Preferred stock, $0.001 par value, authorized shares – 5,000 and none at December 31, 2020 and 2019, respectively; issued and outstanding shares – none	—	—
Common stock, $0.001 par value; authorized shares – 60,000 at December 31, 2020 and 2019, respectively; issued and outstanding shares – 48,802 and 15,387 at December 31, 2020 and 2019, respectively	49	15
Additional paid-in capital	195,699	79,869
Accumulated deficit	(82,797)	(65,572)
Total stockholders' equity	112,951	14,312
Total liabilities and stockholders' equity	$ 118,809	$ 21,744

See accompanying notes.

Oncternal Therapeutics, Inc.
Consolidated Statements of Operations
(thousands, except per share data)

	Years Ended December 31,	
	2020	2019
Grant revenue	$ 3,375	$ 2,425
Operating expenses:		
Research and development	12,544	10,159
In-process research and development	—	18,088
General and administrative	8,373	7,286
Total operating expenses	20,917	35,533
Loss from operations	(17,542)	(33,108)
Other income (expense):		
Change in fair value of warrant liability	—	(1,268)
Other income	301	—
Interest income	16	188
Total other income (expense)	317	(1,080)
Net loss	$ (17,225)	$ (34,188)
Net loss per share, basic and diluted	$ (0.85)	$ (3.31)
Weighted-average shares outstanding, basic and diluted	20,305	10,329

See accompanying notes.

Oncternal Therapeutics, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	Years Ended December 31,	
	2020	**2019**
Cash flows from operating activities		
Net loss	$ (17,225)	$ (34,188)
Adjustments to reconcile net loss to net cash used in operating activities:		
In-process research and development	—	18,088
Gain on forgiveness of payroll protection program loan	(301)	—
Stock-based compensation	1,556	507
Change in fair value of preferred stock warrants liability	—	1,268
Noncash lease expense	150	92
Changes in operating assets and liabilities:		
Prepaid and other assets	(529)	(44)
Accounts payable	272	(4,762)
Accrued liabilities	739	(1,255)
Change in lease liability	(150)	(92)
Deferred grant revenue	(2,007)	3,640
Net cash used in operating activities	(17,495)	(16,746)
Cash flows from investing activities		
Cash acquired in connection with the Merger	—	18,292
Acquisition related costs paid	—	(2,155)
Net cash provided by investing activities	—	16,137
Cash flows from financing activities		
Proceeds from payroll protection loan	301	—
Proceeds from exercise of stock options and common stock warrants	4	15
Proceeds from the issuance of common stock and common stock warrants, net	113,876	—
Net cash provided by financing activities	114,181	15
Net increase (decrease) increase in cash and cash equivalents	96,686	(594)
Cash and cash equivalents at beginning of period	20,051	20,645
Cash and cash equivalents at end of period	$ 116,737	$ 20,051
Supplemental disclosure of non-cash investing and financing activities:		
Payment of 2019 bonus awards with stock options in lieu of cash	$ 415	$ —
Fair value of warrants issued to placement agent	$ 5,325	$ —
Gain on forgiveness of payroll protection program loan	$ 301	$ —
Conversion of convertible preferred stock into common stock	$ —	$ 46,588
Issuance of common stock to GTx stockholders	$ —	$ 29,049
Reclassification of preferred stock warrants liability to additional paid-in capital	$ —	$ 1,942
Net liabilities assumed in Merger	$ —	$ 5,177

See accompanying notes.

Oncternal Therapeutics, Inc.
Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(in thousands)

	Convertible Preferred Stock		Common Stock		Additional Paid-In	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Deficit	Equity
Balance at December 31, 2018	8,148	$ 46,588	3,762	$ 5	$ 1,748	$ (31,384)	$ (29,631)
Exercise of stock options for cash	—	—	19	—	14	—	14
Exercise of warrants for cash	—	—	—	—	1	—	1
Vesting related to repurchase liability	—	—	—	—	30	—	30
Issuance of common stock to former stockholders of GTx upon Merger	—	—	3,458	2	29,047	—	29,049
Conversion of convertible preferred stock into common stock upon Merger	(8,148)	(46,588)	8,148	8	46,580	—	46,588
Reclassification of convertible preferred stock warrant liability	—	—	—	—	1,942	—	1,942
Stock-based compensation	—	—	—	—	507	—	507
Net loss	—	—	—	—	—	(34,188)	(34,188)
Balance at December 31, 2019	—	—	15,387	15	79,869	(65,572)	14,312
Exercise of stock options for cash	—	—	5	—	4	—	4
Cashless exercise of warrants	—	—	36	—	—	—	—
Vesting related to repurchase liability	—	—	—	—	13	—	13
Issuance of common stock, net of issuance cost of $11,103	—	—	33,374	34	113,842	—	113,876
Issuance of 2019 bonus awards with stock option in lieu of cash	—	—	—	—	415	—	415
Stock-based compensation	—	—	—	—	1,556	—	1,556
Net loss	—	—	—	—	—	(17,225)	(17,225)
Balance at December 31, 2020	—	$ —	48,802	$ 49	$ 195,699	$ (82,797)	$ 112,951

See accompanying notes.

1. Description of Business, Basis of Presentation and Summary of Significant Accounting Policies

Description of Business

Oncternal Therapeutics, Inc. (the "Company," "Oncternal," or the "combined company"), formerly known as GTx, Inc., was incorporated in Tennessee in September 1997 and reincorporated in Delaware in 2003 and is based in San Diego, California. The Company is a clinical-stage biopharmaceutical company focused on the development of novel oncology therapies for the treatment of cancers with critical unmet medical need. The Company's clinical pipeline includes cirmtuzumab, a humanized monoclonal antibody that binds to ROR1 (Receptor-tyrosine kinase-like Orphan Receptor 1), and TK216, a small molecule inhibiting the biological activity of ETS-family transcription factor oncoproteins. The Company is also developing a CAR-T (chimeric antigen receptor T-cells) product candidate that targets ROR1.

Merger

On June 7, 2019, the Company, then operating as GTx, Inc. ("GTx"), completed its Agreement and Plan of Merger and Reorganization, as amended (the "Merger Agreement"), with privately-held Oncternal Therapeutics, Inc. ("Private Oncternal") and Grizzly Merger Sub, Inc., a wholly-owned subsidiary of the Company ("Merger Sub"), dated March 6, 2019. Under the Merger Agreement, Merger Sub merged with and into Private Oncternal, with Private Oncternal surviving as a wholly-owned subsidiary of the Company (the "Merger"). GTx changed its name to Oncternal Therapeutics, Inc., and Private Oncternal, which remains as a wholly-owned subsidiary of the Company, changed its name to Oncternal Oncology, Inc. On June 10, 2019, the combined company's common stock began trading on The Nasdaq Capital Market under the ticker symbol "ONCT."

Except as otherwise indicated, references herein to "Oncternal," "the Company," and the "combined company," refer to Oncternal Therapeutics, Inc. on a post-Merger basis, and the term "Private Oncternal" refers to the business of privately-held Oncternal Therapeutics, Inc., prior to completion of the Merger. References to GTx refer to GTx, Inc. prior to completion of the Merger.

Pursuant to the terms of the Merger Agreement, each outstanding share of Private Oncternal common stock outstanding immediately prior to the closing of the Merger was converted into approximately 0.073386 shares of Company common stock (the "Exchange Ratio"), after taking into account the Reverse Stock Split, as defined below. Immediately prior to the closing of the Merger, all shares of Private Oncternal preferred stock then outstanding were exchanged into shares of common stock of Private Oncternal. In addition, all outstanding options exercisable for common stock of Private Oncternal and warrants exercisable for convertible preferred stock of Private Oncternal became options and warrants exercisable for the same number of shares of common stock of the Company multiplied by the Exchange Ratio. Immediately following the Merger, stockholders of Private Oncternal owned approximately 77.5% of the outstanding common stock of the combined company.

The transaction was accounted for as a reverse asset acquisition in accordance with generally accepted accounting principles in the United States of America ("GAAP"). Under this method of accounting, Private Oncternal was deemed to be the accounting acquirer for financial reporting purposes. This determination was primarily based on the facts that, immediately following the Merger: (i) Private Oncternal's stockholders owned a substantial majority of the voting rights in the combined company, (ii) Private Oncternal designated a majority of the members of the initial board of directors of the combined company, and (iii) Private Oncternal's senior management holds all key positions in the senior management of the combined company. As a result, as of the closing date of the Merger, the net assets of the Company were recorded at their acquisition-date relative fair values in the consolidated financial statements of the Company and the reported operating results prior to the Merger are those of Private Oncternal.

Reverse Stock Split and Exchange Ratio

On June 7, 2019, in connection with, and prior to the completion of, the Merger, GTx effected a one-for-seven reverse stock split of its then outstanding common stock (the "Reverse Stock Split"). The par value and the authorized shares of the common stock were not adjusted as a result of the Reverse Stock Split. All of the Company's issued and outstanding common stock have been retroactively adjusted to reflect this Reverse Stock Split for all periods presented. All issued and outstanding Private Oncternal common stock, preferred stock, options and warrants prior to the effective date of the Merger have been retroactively adjusted to reflect the Exchange Ratio for all periods presented.

Principles of Consolidation

The consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, Oncternal Oncology, Inc. and Oncternal, Inc. All intercompany accounts and transactions have been eliminated in the preparation of the consolidated financial statements.

Liquidity and Going Concern

From inception, the Company has devoted substantially all of its efforts to drug discovery and development and conducting preclinical studies and clinical trials. The Company has a limited operating history and the sales and income potential of the Company's business and market are unproven. Successful transition to attaining profitable operations is dependent upon achieving a level of revenues adequate to support the Company's cost structure.

As of December 31, 2020, the Company had $116.7 million in cash and cash equivalents. The Company believes it has sufficient cash to fund its projected operating requirements for at least twelve months from the date of issuance. However, the Company has experienced net losses and negative cash flows from operating activities since its inception and has an accumulated deficit of $82.8 million as of December 31, 2020. The Company expects to continue to incur net losses for the foreseeable future and believes it will need to raise substantial additional capital to accomplish its business plan over the next several years. The Company plans to continue to fund its losses from operations and capital funding needs through a combination of equity offerings, debt financings or other sources, including potential collaborations, licenses and other similar arrangements. If the Company is unable to secure adequate additional funding, the Company may be forced to make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, or suspend or curtail planned programs. Any of these actions could materially harm the Company's business, results of operations and future prospects. There can be no assurance as to the availability or terms upon which such financing and capital might be available in the future.

Use of Estimates

The Company's consolidated financial statements are prepared in accordance with GAAP. The preparation of the Company's consolidated financial statements and accompanying notes requires it to make estimates and assumptions that impact the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities. Significant estimates consist of those used to determine the fair value of the Company's preferred stock, preferred stock warrant liability and stock-based awards, and those used to determine grant revenue and accruals for research and development costs. Although these estimates are based on the Company's knowledge of current events and actions it may undertake in the future, actual results may ultimately materially differ from these estimates and assumptions.

Cash and Cash Equivalents

The Company considers all highly liquid investments with maturities of three months or less when purchased to be cash equivalents. Cash and cash equivalents include cash in readily available checking accounts and money market accounts.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts and believes it is not exposed to significant risk on its cash balances due to the financial position of the depository institution in which those deposits are held. Additionally, the Company established guidelines regarding approved investments and maturities of investments, which are designed to maintain safety and liquidity.

Patent Costs

Costs related to filing and pursuing patent applications are recorded as general and administrative expense and expensed as incurred since recoverability of such expenditures is uncertain.

Research and Development Expenses and Accruals

Research and development expenses consist of costs incurred for the Company's own and for sponsored and collaborative research and development activities. Research and development costs are expensed as incurred and include manufacturing process development costs, manufacturing costs, costs associated with preclinical studies and clinical trials, regulatory and medical affairs activities, quality assurance activities, salaries and benefits, including stock-based compensation, fees paid to third-party consultants, license fees and overhead.

The Company has entered into various research and development contracts with research institutions, clinical research organizations, clinical manufacturing organizations and other companies. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and payments made in advance of performance are reflected in the accompanying consolidated balance sheets as prepaid and other assets or accrued liabilities. The Company records accruals for estimated costs incurred for ongoing research and development activities. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the services, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the prepaid or accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. As of December 31, 2020, the Company's clinical trial accrual balance of $1.0 million is included in accrued liabilities and other liabilities. The Company's related 2020 clinical trial expenses are included in research and development expense of $12.5 million.

Preferred Stock Warrant Liability

Prior to the Merger, Private Oncternal had outstanding freestanding warrants to purchase shares of its Series B-2 convertible preferred stock (the "Series B-2 warrants"). Private Oncternal adjusted the carrying value of such Series B-2 warrants to their estimated fair value at each reporting date, with any related increases or decreases in the fair value recorded as a change in fair value of warrant liability in the consolidated statements of operations. Upon the completion of the Merger, the Series B-2 warrants were amended such that they were converted into warrants to purchase the Company's common stock. As amended, warrant liability accounting is no longer required and the fair value of the warrant liability has been reclassified into stockholders' equity.

Fair Value Measurements

The accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or non-recurring basis. Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

Level 1: Observable inputs such as quoted prices in active markets.

Level 2: Inputs, other than the quoted prices in active markets that are observable either directly or indirectly.

Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

The carrying amounts of the Company's current financial assets and liabilities are considered to be representative of their respective fair values because of the short-term nature of those instruments. The Company has no financial assets or liabilities measured at fair value on a recurring basis. No transfers between levels have occurred during the periods presented.

Revenue Recognition

The Company currently generates revenue from the California Institute for Regenerative Medicine pursuant to a research subaward agreement (see Note 4), which provides the Company with payments in return for certain research and development activities over a contractually defined period. Revenue from such subaward is recognized in the period during which the related qualifying services are rendered and costs are incurred, provided that the applicable conditions under the subaward agreement have been met.

The subaward agreement is on a best-effort basis and does not require scientific achievement as a performance obligation. All fees received under the agreement are non-refundable. The costs associated with the agreement are expensed as incurred and reflected as a component of research and development expense in the accompanying consolidated statements of operations.

Funds received from the subaward agreement are recorded as revenue as the Company is the principal participant in the arrangement because the activities under the subaward are part of the Company's development programs. In those instances where the Company first receives consideration in advance of providing underlying services, the Company classifies such consideration as deferred revenue until (or as) the Company provides the underlying services. In those instances where the Company first provides the underlying services prior to its receipt of consideration, the Company records a grant receivable. At December 31, 2020 and 2019, the Company had deferred grant revenue of $1.6 million and $3.6 million, respectively.

Stock-Based Compensation

Stock-based compensation expense represents the fair value of equity awards, on the grant date, recognized in the period using the Black- Scholes option pricing model. The Company recognizes expense for awards with graded vesting schedules over the requisite service period of the awards (usually the vesting period) on a straight-line basis. For equity awards for which vesting is subject to performance-based milestones, the expense is recorded over the remaining service period after the point when the achievement of the milestone is probable. The Company recognizes forfeitures for all awards as such forfeitures occur.

Income Taxes

The Company accounts for income taxes under the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have

been included in the financial statements. Under this method, deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. The effect of a change in tax rates on deferred tax assets and liabilities is recognized in income in the period that includes the enactment date.

The Company recognizes net deferred tax assets to the extent that the Company believes these assets are more likely than not to be realized. In making such a determination, management considers all available positive and negative evidence, including future reversals of existing taxable temporary differences, projected future taxable income, tax-planning strategies, and results of recent operations. If management determines that the Company would be able to realize its deferred tax assets in the future in excess of their net recorded amount, management would make an adjustment to the deferred tax asset valuation allowance, which would reduce the provision for income taxes.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business in one operating segment in the United States.

Net Loss Per Share

Basic net loss per share is computed by dividing the net loss by the weighted-average number of common shares outstanding for the period, without consideration for potentially dilutive securities and adjusted for the weighted-average number of common shares outstanding that are subject to repurchase. The Company has excluded weighted-average shares subject to repurchase of 25,000 shares and 56,000 shares from the weighted-average number of common shares outstanding for the years ended December 31, 2020 and 2019, respectively. Diluted net loss per share is computed by dividing the net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for the period determined using the treasury-stock and if-converted methods. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding as inclusion of the potentially dilutive securities would be antidilutive.

Potentially dilutive securities not included in the calculation of diluted net loss per share, because to do so would be anti-dilutive, are as follows (in common stock equivalent shares; in thousands):

	December 31,	
	2020	2019
Warrants to purchase common stock	5,032	841
Common stock options	2,226	1,958
Common stock subject to repurchase	15	35
	7,273	2,834

Recently Issued Accounting Pronouncements

In August 2020, the FASB issued ASU 2020-06, Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity, which simplifies the accounting for certain financial instruments with characteristics of liabilities and equity, including convertible instruments and contracts on an entity's own equity. Specifically, ASU 2020-06 simplifies accounting for the issuance of convertible instruments by removing major separation models required under current GAAP. In addition, the ASU removes certain settlement conditions that are required for equity contracts to qualify for the derivative scope exception and simplifies the diluted earnings per share (EPS) calculation in certain areas. ASU 2020-06 will be effective for fiscal years beginning after December 15, 2023, including interim periods within those fiscal years. Early adoption is permitted, beginning in fiscal years which begin after December 15, 2020. The FASB has specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The Company is currently evaluating the impact of the pending adoption of this new standard on its consolidated financial statements.

Recently Adopted Accounting Pronouncements

In December 2019, the FASB issued ASU 2019-12, *Income Taxes: Simplifying the Accounting for Income Taxes*, which is intended to simplify the accounting for income taxes by eliminating certain exceptions related to the approach for intraperiod tax allocation, the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. The new standard also simplifies aspects of the accounting for franchise taxes and enacted changes in tax laws or rates and clarifies the accounting for transactions that result in a step-up in the tax basis of goodwill. The standard is effective for annual periods beginning after December 15, 2020 and interim periods within, with early adoption permitted. Adoption of the standard requires certain changes to be made prospectively, with some changes to be made retrospectively. The Company adopted this standard effective October 1, 2020, the adoption had no impact on the consolidated financial statements.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement: Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement*, which modifies the disclosure requirements for fair value measurements. The amendments relate to disclosures regarding unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and the narrative description of measurement uncertainty and are to be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption. All other amendments should be applied retrospectively to all periods presented upon their effective date. The amendments are effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years, and early adoption is permitted. The Company adopted this standard effective January 1, 2020, the adoption had no impact on the consolidated financial statements.

2. Balance Sheet Details

Accrued liabilities consist of the following (in thousands):

	December 31,	
	2020	2019
Research and development	$ 412	$ 582
Clinical trials	980	624
Legal fees	77	424
Unvested share liability	10	24
Compensation	1,528	825
Other	35	252
	$ 3,042	$ 2,731

3. Commitments, Contingencies and Related Party Transactions

Lease

Rent expense was $0.2 million and $0.1 million for the years ended December 31, 2020 and 2019, respectively. On May 22, 2019, the Company entered into an office sublease agreement for 4,677 square feet in San Diego, California (“San Diego Lease”) which expires on March 31, 2021. Base rent is approximately $166,000 annually and the monthly rent expense is being recognized on a straight-line basis over the lease term.

The San Diego Lease is included in the accompanying consolidated balance sheet at the present value of the lease payments. As the San Diego Lease does not have an implicit interest rate, the present value reflects a 10.0% discount rate which is the estimated rate of interest that the Company would have to pay in order to borrow an amount equal to the lease payments on a collateralized basis over a similar term and in a similar economic environment. The Company recognized a net operating lease right-of-use asset and a lease liability of $40,000 that matures in March 2021, as of December 31, 2020, in the accompanying consolidated balance sheet. The weighted average remaining lease term was 0.25 years.

Related Party Transactions

In January 2019, the Company engaged Newfront Insurance as its primary insurance broker. The son of Richard Vincent, the Company's Chief Financial Officer, acted as the Company's agent at Newfront Insurance. During the years ended December 31, 2020 and 2019, the Company paid total related policy premiums of $1.4 million and $1.2 million, respectively, for which Mr. Vincent's son received a commission of approximately $0.1 million in each respective period.

Effective in September 2019, the Company and Shanghai Pharmaceutical (USA) Inc. ("SPH USA") entered into a Materials Supply and Services Agreement ("SPH USA Services Agreement"), pursuant to which the Company and SPH USA will execute various statements of work for the transfer to SPH USA of key reagents and other materials, and for the supply of certain services by the Company to SPH USA, as contemplated under and in furtherance of the License and Development Agreement between the Company and SPH USA effective as of November 2018. During the year ended December 31, 2020, the Company recorded amounts receivable from SPH USA related to statements of work totaling $0.3 million (see Note 4).

In connection with the securities purchase agreements and underwritten offering, other investors included individuals or entities affiliated with David F. Hale, SPH USA, Daniel L. Kisner, Hazel M. Aker, and Michael G. Carter (see Note 7).

4. License, Collaboration and Research Subaward Agreements

Georgetown University ("Georgetown")

In March 2014, the Company entered into an Exclusive License Agreement (the "Georgetown License Agreement") with Georgetown, pursuant to which the Company: (i) licensed the exclusive worldwide right to patents and technologies for the development and commercialization of certain product candidates targeting EWS-FLI1 as an anti-tumor therapy for therapeutic, diagnostics, or research tool purposes, (ii) is solely responsible for all development and commercialization activities and costs, and (iii) is responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights.

Under the terms of the Georgetown License Agreement, commencing in 2015, the Company: (i) shall pay and has paid an annual license maintenance fee of $10,000 until the first commercial sale occurs, (ii) is required to make up to $0.2 million in aggregate milestone payments upon the achievement of certain regulatory milestones, and (iii) will be required to pay low single digit royalties based on annual net product sales. The Company accounted for the licensed technology as an asset acquisition because it did not meet the definition of a business. All milestone payments under the Georgetown License Agreement will be recognized as research and development expense upon completion of the required events, as the triggering events are not considered to be probable until they are achieved. As of December 31, 2020, the Company had not triggered or made any milestone payments under the Georgetown License Agreement.

The Georgetown License Agreement may be terminated by either party upon material breach or may be terminated by the Company as to one or more countries with 90 days written notice of termination. The term of the Georgetown License Agreement will continue until the expiration of the last valid claim within the patent rights covering the product. Georgetown may terminate the agreement in the event: (i) the Company fails to pay any amount and fails to cure such failure within 30 days after receipt of notice, (ii) the Company defaults in its obligation to obtain and maintain insurance and fails to remedy such breach within 60 days after receipt of notice, or (iii) the Company declares insolvency or bankruptcy. The Company may terminate the Georgetown License Agreement at any time upon at least 60 days' written notice.

The University of Texas MD Anderson Cancer Center ("MD Anderson")

In December 2014, the Company entered into a collaboration agreement (as amended, the "Collaboration") with MD Anderson, which provides for the conduct of preclinical and clinical research for TK216 in exchange for certain program payments. If MD Anderson successfully completes all the requirements of the Collaboration in full and the program is successfully commercialized, the Company will be required to pay aggregate milestone payments of $1.0 million based on net product sales. In July 2020, the

Company entered into a research agreement with MD Anderson for certain services up to an aggregate cost of $293,000. The amount recorded as research and development expense for the year ended December 31, 2020 was $122,000 and the amount was insignificant for the year ended December 31, 2019.

Agreements with the Regents of the University of California (the "Regents")

In March 2016, and as amended and restated in August 2018 in connection with the spin-off transactions described below, the Company entered into a license agreement (as amended, the "Regents License Agreement") for the development, manufacturing and distribution rights related to the development and commercialization of ROR1 related naked antibodies, antibody fragments or synthetic antibodies, and genetically engineered cellular therapy. The Regents License Agreement was amended on March 25, 2019 and May 15, 2019, to update the patents covered under the agreement. The Regents License Agreement provides for the following: (i) in May 2016, an upfront license fee of $0.5 million was paid and 107,108 shares of common stock were issued, (ii) $25,000 in annual license maintenance fees commencing in 2017, (iii) reimbursement of certain annual patent costs, (iv) certain development and regulatory milestones aggregating from $10.0 million to $12.5 million, on a per product basis, (v) certain worldwide sales milestones based on achievement of tiered revenue levels aggregating $75.0 million, (vi) low single-digit royalties, including potential future minimum annual royalties, on net sales of each target, and (vii) minimum diligence to advance licensed assets consisting of at least $1.0 million in development spend annually through 2021. Under the Regents License Agreement, the Company recorded: (i) $25,000 in license maintenance fees as research and development expense for each of the years ended December 31, 2020 and 2019, and (ii) approximately $0.2 million in patent costs as general and administrative expense for the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, the Company believes it has met its obligations under the Regents License Agreement.

In July 2016, and as modified by the amended and restated Regents License Agreement in August 2018, the Company entered into a Research Agreement (the "Research Agreement") with the Regents for further research on a ROR1 therapeutic development program. Under this five-year agreement, the Regents will have an aggregate budget of $3.6 million, with $125,000 payable quarterly. The Company recorded $0.5 million in research and development expense under this agreement for each of the years ended December 31, 2020 and 2019. Such costs are includable as part of the Company's annual diligence obligations under the Regents License Agreement. The Regents License Agreement will expire upon the later of the expiration date of the longest-lived patent rights or the fifteenth anniversary of the first commercial sale of a licensed product.

The Regents may terminate the Regents License Agreement if: (i) a material breach by the Company is not cured within a reasonable time, (ii) the Company files a claim asserting the Regents licensed patent rights are invalid or unenforceable and (iii) the Company files for bankruptcy. The Company may terminate the agreement at any time upon at least 60 days' written notice.

University of Tennessee Research Foundation ("UTRF")

In March 2015, the Company and UTRF entered into a license agreement (the "SARD License Agreement") pursuant to which the Company was granted exclusive worldwide rights in all existing selective androgen receptor degrader ("SARD") technologies owned or controlled by UTRF, including all improvements thereto. Under the SARD License Agreement, the Company is obligated to employ active, diligent efforts to conduct preclinical research and development activities for the SARD program to advance one or more lead compounds into clinical development. The Company is also obligated to pay UTRF annual license maintenance fees, low single-digit royalties on net sales of products and additional royalties on sublicense revenues, depending on the state of development of a clinical product candidate at the time it is sublicensed. The Company recorded research and development expense under this agreement of $0.2 million and $0.4 million for each of the years ended December 31, 2020 and 2019, respectively. As of December 31, 2020, the Company believes it has met its obligations under the SARD License Agreement.

As of December 31, 2020, the Company believes it has met its obligations under each of the UTRF agreements.

The California Institute for Regenerative Medicine ("CIRM") Award

In August 2017, and as amended and restated in December 2020, CIRM awarded an $18.3 million grant to researchers at UC San Diego to advance the Company's Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib for the treatment of patients with B-cell lymphoid malignancies, including chronic lymphocytic leukemia and mantle cell lymphoma. The Company: (i) is conducting this study in collaboration with UC San Diego, (ii) estimates it will receive approximately $14.0 million in development milestones under research subaward agreements throughout the award project period, estimated to be from October 1, 2017 to March 31, 2022, (iii) is committed to certain co-funding requirements, (iv) received subaward payments of $1.4 million and $6.2 million in the years ended December 31, 2020 and 2019, respectively, and (v) is required to provide UC San Diego progress and financial update reports throughout the award period. The subaward does not bear a royalty payment commitment, nor is the subaward otherwise refundable. For the years ended December 31, 2020 and 2019, the Company's grant revenue was $3.4 million and $2.4 million, respectively. Related qualifying subaward costs for the years ended December 31, 2020 and 2019 were $5.2 million and $5.4 million, respectively. As of December 31, 2020, the Company believes it has met its obligations under the CIRM award and UC San Diego subawards.

In October 2017, CIRM awarded a $5.8 million grant to the researchers at the University of California San Diego School of Medicine ("UC San Diego") to develop a novel anti-cancer stem cell targeted therapy for patients with advanced solid and hematological malignancies. In connection with such CIRM award, the Company agreed to provide up to $1.0 million in contingency funds if required during the grant period. The Company recorded no research and development expense, and no contingency funds have been provided under such CIRM award for the years ended December 31, 2020 and 2019.

Clinical Trial and Supply Agreement

In April 2018, the Company entered into a Clinical Trial and Supply Agreement with Pharmacyclics, LLC, an AbbVie Company ("Pharmacyclics") to supply ibrutinib for the Company's Phase 1/2 clinical trial evaluating cirmtuzumab in combination with ibrutinib, which agreement was amended in August 2019. Such agreement does not bear any upfront costs, inventory purchase costs, milestone or royalty payment commitments or other financial obligations.

SPH USA, a Related Party

License and Development Agreement ("LDA")

In November 2018, and as amended in August 2020, the Company entered into the LDA with SPH USA for: (i) the territory of the People's Republic of China, Hong Kong, Macau, and Taiwan ("Greater China"), and (ii) rights to manufacture, develop, market, distribute and sell all of the Company's product candidates under the Georgetown License Agreement and the Regents License Agreement (exclusive to Greater China only). Under the LDA, SPH USA is solely responsible for: (a) all preclinical and clinical development activities required in order to obtain regulatory approval in Greater China for such product candidates, (b) any third-party license milestone or royalty payments owed under the Georgetown License Agreement and the Regents License Agreement, and (c) paying the Company a low single digit royalty on net sales in the territory.

The LDA will expire upon the expiration of the last royalty term for the last licensed product. The LDA may be terminated by: (i) SPH USA on a country by country or product by product basis with 180 days written notice, (ii) either party upon material breach that is not cured within 90 days, and (iii) either party in the event the other party declares insolvency or bankruptcy.

5. Debt

Paycheck Protection Program Loan Payable

In May 2020, the Company received a $0.3 million unsecured loan, bearing interest at 1%, pursuant to the Paycheck Protection Program (the "PPP"), a program implemented by the U.S. Small Business Administration (the

"SBA") under the Coronavirus Aid, Relief, and Economic Security Act (the "CARES Act") (the "PPP Loan"). In December 2020, the underlying principal and interest were fully forgiven by the SBA and the Company has no further obligations thereunder. The loan forgiveness was recorded as other income of $301k in the statement of operations.

6. Merger

The Merger, which closed on June 7, 2019, was accounted for as a reverse asset acquisition, as substantially all of the fair value of the assets acquired were concentrated in a group of similar non-financial assets, and the acquired assets did not have outputs or employees. Because the assets had not yet received regulatory approval, the fair value attributable to these assets of $18.1 million was recorded as in-process research and development expenses in the Company's consolidated statement of operations in the year ended December 31, 2019.

Pursuant to the Merger Agreement on June 7, 2019, the Company, a representative of holders of the contingent value rights ("CVRs"), and Computershare, Inc. as rights agent entered into a Contingent Value Rights Agreement (the "CVR Agreement"). Pursuant to the CVR Agreement, the Company's stockholders of record as of immediately prior to the Merger received one CVR for each share of the Company's common stock held immediately prior to the Merger. CVR holders are entitled to receive 75% of the aggregate amount of any net proceeds received by the Company during the 15-year period after the closing of the Merger from the grant, sale or transfer of rights to the Company's SARD or SARM technology that occurs during the 10-year period after the closing (or in the 11th year if based on a term sheet approved during the initial 10-year period) and, if applicable, to receive royalties on the sale of any SARD or SARM products by the Company during the 15-year period after the closing. Effective in March 2020, the Company terminated the SARM license agreement with UTRF and no longer has any rights to the SARM technology. The CVR Agreement will continue in effect until the payment of all amounts payable thereunder. As of the years ended December 31, 2020 and 2019, no milestones had been accrued as there were no potential milestones yet considered probable. The total purchase price paid in the Merger has been allocated to the net assets acquired and liabilities assumed based on their fair values as of the completion of the Merger.

The following summarizes the purchase price paid in the Merger (in thousands, except share and per share amounts):

Number of shares of the combined organization owned by the Company's pre-Merger stockholders		3,458,170
Multiplied by the fair value per share of GTx common stock (1)	$	8.40
Fair value of consideration issued to effect the Merger	$	29,049
Transaction costs		2,154
Purchase price	$	31,203

(1) Based on the last reported sale price of the Company's common stock on the Nasdaq Capital Market on June 7, 2019, the closing date of the Merger, and gives effect to the Reverse Stock Split.

The allocation of the purchase price is as follows:

Cash acquired	$	18,292
Net liabilities assumed		(5,177)
IPR&D (2)		18,088
Purchase price	$	31,203

(2) Represents the research and development projects of GTx which were in-process, but not yet completed, and which the Company plans to advance, consisting primarily of GTx's preclinical SARD technology. Current accounting standards require that the fair value of IPR&D projects acquired in an asset acquisition with no

alternative future use be allocated a portion of the consideration transferred and charged to expense on the acquisition date. The acquired assets did not have outputs or employees.

7. Stockholders' Equity

Amended and Restated Articles of Incorporation

On June 7, 2019, the Company's certificate of incorporation was amended and restated to authorize 60,000,000 shares of common stock and 5,000,000 shares of undesignated preferred stock, each with a par value of $0.001 per share.

Convertible Preferred Stock

In connection with the Merger, all of the then outstanding shares of Private Oncternal's convertible preferred stock were converted into 8,148,268 shares of the Company's common stock. As of December 31, 2018, Private Oncternal's convertible preferred stock was classified as temporary equity on the accompanying consolidated statements of convertible preferred stock and stockholders' equity (deficit) in accordance with authoritative guidance for the classification and measurement of potentially redeemable securities whose redemption is based upon certain change in control events outside of Private Oncternal's control, including liquidation, sale or transfer of control of Private Oncternal. Private Oncternal did not adjust the carrying values of the convertible preferred stock to the liquidation preferences of such shares because the occurrence of any such change of control event was not deemed probable.

Securities Purchase Agreements and Underwritten Offering

In May 2020, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with several institutional and individual investors (including an entity affiliated with David F. Hale, the chairman of the Company's board of directors) for the concurrent sale of: (i) 1,943,636 shares of the Company's common stock in a registered direct offering, resulting in net proceeds of $4.4 million, after deducting the placement agent's cash commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the warrants, and (ii) unregistered warrants to purchase up to an aggregate of 971,818 shares of common stock. The combined purchase price for one share and one warrant to purchase half of a share of common stock was $2.5725. In addition, the Company issued warrants to purchase 116,618 shares of common stock at an exercise price of $3.2156 per share to the placement agent, H.C. Wainwright & Co., LLC ("Wainwright" or the "placement agent") as part of its compensation, which warrants were immediately exercisable and expire on May 21, 2025.

In July 2020, the Company entered into a Securities Purchase Agreement (the "July Purchase Agreement") with several institutional and individual investors for the concurrent sale of: (i) 2,581,867 shares of the Company's common stock in a registered direct offering, resulting in net proceeds of $5.7 million, after deducting the placement agent's cash commissions and other offering expenses, and excluding the proceeds, if any, from the exercise of the warrants, and (ii) unregistered warrants to purchase up to an aggregate of 1,290,933 shares of common stock. The combined purchase price for one share and one warrant to purchase half of a share of common stock was $2.3825. The warrants issued to investors were, subject to certain ownership limitations, immediately exercisable at an exercise price equal to $2.32 per share and expire on January 21, 2026. In addition, the Company issued warrants to purchase 154,912 shares of common stock at an exercise price of $2.9781 per share to the placement agent as part of its compensation, which warrants were immediately exercisable upon issuance and terminate on July 21, 2025. Other investors participating in the July Purchase Agreement included an entity affiliated with SPH USA, the Company's largest stockholder, Daniel L. Kisner, a member of the Company's board of directors, and Hazel M. Aker, the Company's then General Counsel.

In August 2020, the Company entered into an underwriting agreement (as amended and restated, the "August Underwriting Agreement") with Wainwright for the sale of 2,428,886 shares of the Company's common stock at a price to the public of $2.10 per share, resulting in net proceeds of $4.4 million, after deducting the underwriter's discounts, commissions and other offering expenses. In addition, the Company issued warrants to purchase 145,733 shares of common stock at an exercise price of $2.625 per share to Wainwright as part of its compensation, which warrants were immediately exercisable upon issuance and terminate on August 27, 2025. An investor participating in the transaction included Michael G. Carter, a member of the Company's board of directors.

In November 2020, the Company entered into an underwriting agreement (as amended and restated, the "November Underwriting Agreement") with Wainwright for the sale of 7,258,065 shares of the Company's common stock at a price to the public of $3.10 per share, resulting in net proceeds of $20.4 million, after deducting the underwriter's discounts, commissions and other offering expenses. In addition, the Company issued warrants to purchase 435,484 shares of common stock at an exercise price of $3.875 per share to Wainwright as part of its compensation, which warrants were immediately exercisable upon issuance and terminate on November 17, 2025.

In December 2020, the Company entered into an underwriting agreement (as amended and restated, the "December Underwriting Agreement") with Wainwright for the sale of 19,161,667 shares of the Company's common stock at a price to the public of $4.50 per share, resulting in net proceeds of $79.0 million, after deducting the underwriter's discounts, commissions and other offering expenses In addition, the Company issued warrants to purchase 1,149,700 shares of common stock at an exercise price of $5.625 per share to Wainwright as part of its compensation, which warrants were immediately exercisable upon issuance and terminate on December 9, 2025.

In connection with the May Purchase Agreement and July Purchase Agreement, the Company also agreed, on a best-efforts basis, to: (i) maintain its listing on The Nasdaq Capital Market to provide for the resale of the shares of common stock issuable upon the exercise of the warrants, and (ii) not enter into any agreement for the issuance of any shares of common stock involving a variable rate transaction before July 21, 2021, other than pursuant to a new at-the-market offering facility with the placement agent. Variable rate transaction means a transaction in which the Company issues or sells, or agrees to issue or sell, common stock or convertible securities in which the applicable sale, conversion, exercise or exchange price or rate may directly or indirectly effectively be reduced. The Company does not currently have an active at-the-market facility.

Common Stock Warrants

A summary of warrant activity and changes in warrants outstanding is presented below:

	Number of Shares Underlying Warrants	Weighted-Average Exercise Price per Share	Weighted-Average Remaining Contractual Term
Balance Outstanding - December 31, 2018	841,620	$ 37.97	3.75
Exercised	(196)	$ 6.13	—
Balance Outstanding - December 31, 2019	841,424	$ 37.97	2.75
Issued	4,265,198	$ 3.47	—
Exercised	(74,781)	$ 3.22	—
Balance Outstanding - December 31, 2020	5,031,841	$ 9.25	4.40

As of December 31, 2020 and 2019, all warrants met the criteria for classification in stockholders' equity.

Restricted Common Stock and Unvested Share Liability

Prior to the Merger, the Company issued restricted common stock subject to vesting and repurchase by the Company. For employee and non-employee awards, the issuance date fair value is recognized over the requisite service period of the award (usually the vesting period) on a straight-line basis. In addition, the Company has outstanding unvested shares related to the early exercise of stock options. The Company has the right, but not the obligation, to repurchase any unvested shares at the original purchase price upon any voluntary or involuntary termination. The consideration received in exchange for unvested shares is recorded as an unvested share liability on the accompanying consolidated balance sheets and is reclassified into common stock and additional paid-in capital as the shares vest. At December 31, 2020 and 2019, the unvested share liability was $10,000 and $24,000, respectively.

A summary of the Company's unvested shares is as follows (in thousands):

	Number of Shares
Balance at December 31, 2019	35
Vested shares	(20)
Balance at December 31, 2020	15

Equity Incentive Plans

Contemporaneous with the Merger closing: (i) Private Oncternal's 2015 Equity Incentive Plan, as amended (the "2015 Plan") was assumed by the Company, and (ii) the Company adopted the 2019 Incentive Award Plan ("2019 Plan") under which the sum of: (a) 1,678,571 shares of common stock, (b) up to 275,579 shares of common stock which were subject to outstanding awards under the GTx 2013 Equity Incentive Plan (the "2013 Plan") as of June 7, 2019, that are subsequently cancelled will become available for issuance under the 2019 Plan, and (c) an annual increase on the first day of each calendar year beginning January 1, 2020, and ending on and including January 1, 2029, equal to the lesser of (A) 5% of the aggregate number of shares of common stock outstanding on the final day of the immediately preceding calendar year and (B) such smaller number of shares of common stock as is determined by the Board, are reserved for issuance. At December 31, 2020, 937,837 shares remain available for future issuance under the 2019 Plan (see Note 10).

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
December 31, 2020:				
Options outstanding	2,107,625	$ 4.08	8.3	$ 2,509
Options vested and expected to vest	2,107,625	$ 4.08	8.3	$ 2,509
Options exercisable	965,129	$ 3.61	8.1	$ 1,525

As of December 31, 2020 under the 2013 Plan, there were: (i) 111,145 outstanding and fully vested options with a weighted average exercise price of $63.58 per share, and (ii) 145,652 cancelled options that were added back to the 2019 Plan as of December 31, 2020. As of December 31, 2020, the former GTx stock option plans had an aggregate of 118,024 outstanding and fully vested and exercisable options with a weighted average exercise price of $75.16 and a weighted average remaining contractual term of 0.4 years.

In July 2015, Private Oncternal adopted the 2015 Plan which provided for the issuance of up to 631,120 shares of common stock for incentive stock options, non-statutory stock options, restricted stock awards, restricted stock unit awards and other stock awards to its employees, members of its board of directors and consultants. In general, the options issued under the 2015 Plan expire ten years from the date of grant and vest over a four-year period. Certain grants vest based on the achievement of development or regulatory milestones. The 2015 Plan allowed for the early exercise of all stock option grants if authorized by the board of directors at the time of grant. The Company has the option to repurchase any unvested shares at the original purchase price upon any voluntary or involuntary termination.

No further awards will be made under the 2015 Plan, which was terminated as to new grants in June 2019.

A summary of the Company's stock option activity under the 2019 Plan and 2015 Plan is as follows:

	Number of Options	Weighted-Average Exercise Price
Balance at December 31, 2019	1,662,253	$ 4.17
Granted	624,260	$ 3.37
Cancelled	(173,753)	$ 2.57
Exercised	(5,135)	$ 0.77
Balance at December 31, 2020	2,107,625	$ 4.08

Information about the Company's outstanding stock options under the 2019 Plan and 2015 Plan is as follows (in thousands, except share and per share data and expected term):

The weighted average grant date fair value per share of option grants for the years ended December 31, 2020 and 2019 was $2.53 and $3.69 per share, respectively. The aggregate intrinsic value used in the above table of options at December 31, 2020 is based on the Company's closing market price per common share on December 31, 2020 of $4.90. The intrinsic value is calculated as the difference between the fair value of the Company's common stock at the time of the option exercise and the exercise price of that stock option. The aggregate intrinsic value of stock options exercised during the years ended December 31, 2020 and 2019 was not material.

Stock-Based Compensation Expense

The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of stock option grants were as follows:

	Years Ended December 31,	
	2020	2019
Risk-free interest rate	0.7%	1.6%
Expected volatility	91.6%	77.6%
Expected term (in years)	6.7	6.0
Expected dividend yield	—%	—%

Expected volatility. Prior to the Merger, Private Oncternal did not have a trading history for its common stock. Accordingly, the expected volatility assumption is based on volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the life sciences industry. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

Expected term. The expected term represents the period of time that options are expected to be outstanding. Because Private Oncternal did not have historical exercise behavior, it determined the expected life assumption using the simplified method for employees, which is an average of the contractual term of the option and its vesting period. The expected term for nonemployee options is generally the remaining contractual term.

Risk-free interest rate. The risk-free interest rate is based on the implied yield on the U.S. Treasury securities with a maturity date similar to the expected term of the associated stock option award.

Expected dividend yield. The Company bases the expected dividend yield assumption on the fact that it has never paid cash dividends and has no present intention to pay cash dividends and, therefore, used an expected dividend yield of zero.

Stock-based compensation expense recognized for all equity awards has been reported in the statements of operations as follows (in thousands):

	Years Ended December 31,	
	2020	2019
Research and development	$ 544	$ 237
General and administrative	1,012	270
	$ 1,556	$ 507

At December 31, 2020, the total compensation cost related to nonvested awards not yet recognized was $3.4 million and the weighted-average period over which it is expected to be recognized was 2.5 years.

Common Stock Reserved for Future Issuance

Common stock reserved for future issuance is as follows (in thousands):

	December 31,	
	2020	2019
Common stock warrants	5,032	841
Common stock options issued and outstanding	2,226	1,958
Common stock available for issuance under equity plans	938	495
	8,196	3,294

8. COVID-19 Pandemic and CARES Act

A novel strain of coronavirus (SAR-CoV-2) causing a severe respiratory disease (“COVID-19”), was declared a global pandemic by the World Health Organization in March 2020. COVID-19 has presented substantial public health and economic challenges and is affecting economies, financial markets and business operations around the world. International and U.S. governmental authorities in impacted regions have taken actions in an effort to slow the spread of COVID-19, including issuing varying forms of “stay-at-home” orders, and restricting business functions outside of one’s home. In response, the Company has put restrictions on employee travel and working from its executive offices with many employees continuing their work remotely. While the Company is currently continuing the clinical trials it has underway in sites across the U.S., the Company expects that COVID-19 precautions may directly or indirectly impact the timeline for some of its clinical trials. For example, some of its clinical trial sites, including those located in areas severely impacted by the pandemic, have placed new patient enrollment into clinical trials on hold or, for patients travelling from out-of-state, have implemented a 14-day self-quarantine before appointments. Additionally, the Company’s expectations for the timing of first-in-human dosing of its ROR1 CAR-T therapy in China has been delayed. The Company considered the impacts of COVID-19 on the assumptions and estimates used to prepare its consolidated financial statements and determined that there were no material adverse impacts on the Company’s results of operations and financial position at December 31, 2020. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company’s business results of operations and financial condition, will depend on future development that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain or treat it, the success or failure of vaccination programs, the emergence of new variants of COVID-19, as well as the economic impact on local, regional, national and international markets.

In response to the COVID-19 pandemic, the CARES Act was signed into law on March 27, 2020. The CARES Act, among other things, includes tax provisions relating to refundable payroll tax credits, deferment of employer’s social security payments, net operating loss utilization and carryback periods, modifications to the net interest deduction limitations and technical corrections to tax depreciation methods for qualified improvement property (QIP), and authorized the PPP (see Note 5). The CARES Act had no material impact on the Company’s income tax provision for the 12 months ended December 31, 2020.

9. Income Taxes

A reconciliation of the Company's effective tax rate and federal statutory tax rate is as follows (in thousands):

	Years Ended December 31,	
	2020	2019
Federal income taxes	$ (3,617)	$ (7,179)
State income taxes, net of federal benefit	(1,113)	(968)
Permanent items	(65)	873
In-process research and development	—	3,354
Research and development credit carryforwards	(505)	(464)
Other, net	226	265
Change in valuation allowance	5,074	4,119
Provision for income taxes	$ —	$ —

Significant components of the Company's net deferred tax assets are as follows (in thousands):

	December 31,	
	2020	2019
Deferred tax assets:		
Net operating loss carryforwards	$ 17,852	$ 18,108
Research and development credit carryforwards	1,952	1,446
Accrued expenses	367	214
Capitalized research and development costs	11,987	7,688
Other, net	393	143
Total deferred tax assets	32,551	27,599
Valuation allowance	(32,540)	(27,546)
	11	53
Deferred tax liabilities:		
Right of use asset	(11)	(53)
Total deferred tax liabilities	(11)	(53)
Net deferred tax assets	$ —	$ —

Based on the Company's history of operating losses, the Company is unable to conclude that it is more likely than not that the benefit of its deferred tax assets will be realized. Accordingly, the Company has provided a full valuation allowance for its deferred tax assets as of December 31, 2020 and 2019. As a result of the Merger in 2019, the Company recorded deferred tax assets of $13.1 million which are fully offset by a valuation allowance. The $13.1 million net deferred tax assets do not include federal and state net operating loss carryforwards and federal research and development credit carryforwards that are estimated to expire under Internal Revenue Code Sections 382 and 383 as a result of the Merger.

At December 31, 2020, the Company had federal and state net operating loss (NOL) carryforwards of approximately $69.1 million and $47.7 million, respectively. Of the federal net operating losses at December 31, 2020, $43.4 million do not expire, and the remaining federal and state net operating loss carryforwards will begin expiring in 2033 and 2029, respectively, unless previously utilized. At December 31, 2020, the Company also had federal and state research and development credit carryforwards of approximately $1.3 million and $0.9 million, respectively. The federal research and development credit carryforwards will begin expiring in 2034 unless previously utilized. The state research and development credits do not expire.

Pursuant to Internal Revenue Code Sections 382 and 383, annual use of the Company's net operating loss and research and development tax credit carryforwards may be limited in the event a cumulative change in ownership of more than 50% occurs within a three-year period. The Company has not completed a Section 382 study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since the Company's formation due to the complexity and cost associated with such a study and the

fact that there may be additional such ownership changes in the future. If eliminated, the related asset would be removed from the deferred tax asset schedule with a corresponding reduction in the valuation allowance. Due to the existence of the valuation allowance, limitations created by future ownership changes, if any, will not impact the Company's effective tax rate.

The Company recognizes a tax benefit from an uncertain tax position when it is more likely than not that the position will be sustained upon examination, including resolutions of any related appeals or litigation processes, based on the technical merits. Income tax positions must meet a more likely than not recognition at the effective date to be recognized. At December 31, 2020 and 2019, there were no unrecognized tax benefits recorded in the consolidated financial statements. The Company does not expect any material changes to unrecognized tax benefits within the next twelve months.

The Company is subject to taxation in the United States federal and state jurisdictions. The Company's 2013 through 2020 federal income tax and state income tax returns are subject to examination by federal and state tax authorities due to the carryforward of unutilized net operating losses and research and development credits. The Company is not currently under examination by any tax authority.

The Company's policy is to recognize interest and penalties related to income tax matters in income tax expense. The Company has not recognized interest or penalties in its consolidated statements of operations since inception.

10. Subsequent Event

Inducement Plan

In February 2021, the Company's board of directors adopted the 2021 Employment Inducement Equity Incentive Award Plan (Inducement Plan) and initially reserved 700,000 shares of common stock for issuance. The Inducement Plan is a non-shareholder approved stock plan adopted pursuant to the "inducement exception" provided under Nasdaq listing rules. The Inducement Plan will be used exclusively for the issuance of non-statutory stock options to certain new hires who satisfied the requirements to be granted inducement grants under Nasdaq rules as an inducement material to the individual's entry into employment with the Company. The terms of the Inducement Plan are substantially similar to the terms of our 2019 Plan.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Disclosure Controls and Procedures

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act")) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow for timely decisions regarding required disclosures.

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of December 31, 2020, the end of the period covered by this report. Based on the evaluation of these disclosure controls and procedures, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective as of December 31, 2020.

Management's Report on Internal Control Over Financial Reporting

We, as management of Oncternal Therapeutics, Inc., are responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Securities Exchange Act Rule 13a-15(f). Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with United States generally accepted accounting principles. Any system of internal control, no matter how well designed, has inherent limitations, including the possibility that a control can be circumvented or overridden and misstatements due to error or fraud may occur and not be detected. Also, because of changes in conditions, internal control effectiveness may vary over time. Accordingly, even an effective system of internal control will provide only reasonable assurance that the objectives of the internal control system are met.

Under the supervision and with the participation of management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2020 using the criteria for effective internal control over financial reporting as described in "Internal Control — Integrated Framework," issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). Based on this evaluation, we concluded that, as of December 31, 2020, our internal control over financial reporting was effective.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the fourth quarter of 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

The information set forth below is included herein, by our option, for the purpose of providing disclosure under "Item 8.01 – Other Events." of Form 8-K.

On February 26, 2021, Igor Bilinsky, Ph.D. resigned from his position as the Chief Business Officer of the Company effective as of March 12, 2021.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Information required by this item will be contained in our definitive proxy statement to be filed with the Securities and Exchange Commission on Schedule 14A in connection with our 2021 Annual Meeting of Stockholders or the Proxy Statement, which is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2020, under the headings “Executive Officers,” “Election of Directors,” “Information Regarding the Board of Directors and Corporate Governance,” and “Delinquent Section 16(a) Reports,” and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this item regarding executive compensation is incorporated by reference to the information set forth in the sections titled “Executive Compensation” in our Proxy Statement.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item regarding security ownership of certain beneficial owners and management is incorporated by reference to the information set forth in the section titled “Security Ownership of Certain Beneficial Owners and Management” in our Proxy Statement.

The information required by Item 201(d) of Regulation S-K is incorporated by reference to the information set forth in the section titled “Executive Compensation” in our Proxy Statement.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item regarding certain relationships and related transactions and director independence is incorporated by reference to the information set forth in the sections titled “Transactions with Related Parties” and “Election of Directors – Independence of the Board of Directors,” respectively, in our Proxy Statement.

Item 14. Principal Accountant Fees and Services.

The information required by this item regarding principal accountant fees and services is incorporated by reference to the information set forth in the section titled “Principal Accountant Fees and Services” in our Proxy Statement.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(a) Documents filed as part of this report.

1. Financial Statements

The consolidated financial statements of Oncternal Therapeutics, Inc. listed below are set forth in Item 8 of this Annual Report for the year ended December 31, 2020:

2. Financial Statement Schedules

These schedules have been omitted because the required information is included in the financial statements or notes thereto or because they are not applicable or not required.

3. Exhibits

A list of exhibits is set forth on the Exhibit Index immediately preceding the signature page of this annual report on Form 10-K and is incorporated herein by reference.

ITEM 16. FORM 10-K SUMMARY

None.

Exhibits

Exhibit Number	Description	Form	File No.	Exhibit No	Filing Date	Filed/ Furnished Herewith
2.1	Asset Purchase Agreement dated as of September 28, 2012 between the Registrant and Strakan International S.à r.l.	8-K	000-50549	2.1	October 3, 2012	
2.2*	Agreement and Plan of Merger and Reorganization, dated March 6, 2019, by and among the Registrant, Oncternal Therapeutics, Inc. and Grizzly Merger Sub, Inc.	8-K	000-50549	2.1	March 7, 2019	
2.3	Amendment No. 1 to Agreement and Plan of Merger, dated April 30, 2019, by and among the Registrant, Oncternal Therapeutics, Inc. and Grizzly Merger Sub, Inc.	8-K	000-50549	2.1	April 30, 2019	
2.4	CVR Agreement, dated as of June 7, 2019, by and between the Registrant, Marc S. Hanover, as the Holders' Representative, and Computershare Investor Services, as Rights Agent	8-K	000-50549	10.1	June 10, 2019	
3.1	Restated Certificate of Incorporation of the Registrant	S-3	333-127175	4.1	August 4, 2005	
3.2	Certificate of Amendment of Restated Certificate of Incorporation of the Registrant	8-K	000-50549	3.2	May 6, 2011	
3.3	Certificate of Amendment of Restated Certificate of Incorporation of the Registrant	8-K	000-50549	3.3	May 9, 2014	
3.4	Certificate of Amendment of Restated Certificate of Incorporation of the Registrant	10-Q	000-50549	3.4	May 11, 2015	
3.5	Certificate of Amendment of Restated Certificate of Incorporation of the Registrant	8-K	000-50549	3.1	December 5, 2016	
3.6	Certificate of Amendment of the Restated Certificate related to the Reverse Stock Split of the Registrant	8-K	000-50549	3.1	June 10, 2019	
3.7	Certificate of Amendment of the Restated Certificate related to the Name Change of the Registrant	8-K	000-50549	3.2	June 10, 2019	
3.8	Amended and Restated Bylaws of the Registrant	8-K	000-50549	3.3	June 10, 2019	
4.1	Specimen of Common Stock Certificate	10-Q	000-50549	4.2	August 9, 2019	
4.2	Form of Common Stock Warrant, issued by Registrant pursuant to the Purchase Agreement, dated September 25, 2017, between Registrant and the purchasers identified in Exhibit A therein	S-3	333-221040	4.9	October 20, 2017	

4.3	Form of Warrant to purchase shares of Series B-2 Preferred Stock of Oncternal Therapeutics, Inc.	S-4	333-230758	4.11	April 8, 2019
4.4	Form of Amendment to Warrant to Purchase shares of Series B-2 Preferred Stock of Oncternal Therapeutics, Inc.	10-Q	000-50549	4.1	August 9, 2019
4.5	Form of Common Stock Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated May 19, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	4.1	May 21, 2020
4.6	Form of Placement Agent Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated May 19, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	4.2	May 21, 2020
4.7	Form of Common Stock Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated July 17, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	4.1	July 21, 2020
4.8	Form of Placement Agent Warrant, issued by Registrant pursuant to the Securities Purchase Agreement, dated July 17, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	4.2	July 21, 2020
4.9	Form of Underwriter Warrant, issued by Registrant pursuant to the Amended and Restated Underwriting Agreement, dated August 27, 2020, between the Registrant and H.C. Wainwright & Co., LLC	8-K	000-50549	4.1	August 31, 2020
4.10	Form of Underwriter Warrant, issued by Registrant pursuant to the Amended and Restated Underwriting Agreement, dated November 17, 2020, between the Registrant and H.C. Wainwright & Co., LLC	8-K	000-50549	4.1	November 19, 2020
4.11	Form of Underwriter Warrant, issued by Registrant pursuant to the Amended and Restated Underwriting Agreement, dated December 9, 2020, between the Registrant and H.C. Wainwright & Co., LLC	8-K	000-50549	4.1	December 11, 2020
4.12	Description of Securities of Oncternal Therapeutics, Inc.	10-K	000-50549	4.1	March 16, 2020
10.1†	Commercial License Agreement between Selexis SA and ROAR Therapeutics, LLC (predecessor to Oncternal Therapeutics, Inc.), dated May 19, 2014	S-4	333-230758	10.46	April 8, 2019
10.2†	Amendment No. 1 to Commercial License Agreement between Selexis SA and Oncternal Therapeutics, Inc., dated February 7, 2020	10-K	000-50549	10.2	March 16, 2020

10.3†	Exclusive License Agreement between Georgetown University and Oncternal Therapeutics, Inc., dated March 26, 2014	S-4	333-230758	10.47	April 8, 2019	
10.4	Amendment to Exclusive License Agreement between Georgetown University and Oncternal Therapeutics, Inc., dated March 17, 2016	S-4	333-230758	10.48	April 8, 2019	
10.5†	Collaboration Agreement between Oncternal Therapeutics, Inc. and The University of Texas M.D. Anderson Cancer Center, dated December 15, 2014	S-4	333-230758	10.49	April 8, 2019	
10.6†	Amendment #1 to Collaboration Agreement between Oncternal Therapeutics, Inc. and The University of Texas M.D. Anderson Cancer Center, dated January 24, 2016	S-4	333-230758	10.50	April 8, 2019	
10.7†	Amendment #2 to Collaboration Agreement between Oncternal Therapeutics, Inc. and The University of Texas M.D. Anderson Cancer Center, dated May 1, 2016	S-4	333-230758	10.51	April 8, 2019	
10.8†	Amendment #3 to Collaboration Agreement between Oncternal Therapeutics, Inc. and The University of Texas M.D. Anderson Cancer Center, dated September 17, 2018	S-4	333-230758	10.52	April 8, 2019	
10.9†	Research agreement between Oncternal Therapeutics, Inc. and the Regents of the University of California, on behalf of its San Diego Campus, dated November 3, 2016	S-4	333-230758	10.53	April 8, 2019	
10.10†	License Agreement between Oncternal Therapeutics, Inc. and Velos Biopharma Holdings, LLC, dated February 6, 2018	S-4	333-230758	10.54	April 8, 2019	
10.11†	Amended and Restated License Agreement between Oncternal Therapeutics, Inc. and The Regents of the University of California, dated August 31, 2018	S-4	333-230758	10.55	April 8, 2019	
10.12†	Amendment #1 to Amended and Restated License Agreement between Oncternal Therapeutics, Inc. and the Regents of the University of California, dated March 25, 2019	S-4	333-230758	10.56	April 8, 2019	
10.13†	Amendment #2 to Amended and Restated License Agreement between Oncternal Therapeutics, Inc. and the Regents of the University of California, dated May 15, 2019	10-K	000-50549	10.13	March 16, 2020	
10.14	Amendment #3 to Amended and Restated License Agreement between Oncternal Therapeutics, Inc. and the Regents of the University of California, dated February 5, 2021					X
10.15#	Oncternal Therapeutics, Inc. 2015 Equity Incentive Plan, as amended	S-4	333-230758	10.57	April 8, 2019	

10.16#	Form of Stock Option Agreement under the Oncternal Therapeutics, Inc. 2015 Equity Incentive Plan, as amended	S-4	333-230758	10.58	April 8, 2019
10.17#	Form of Early Exercise Stock Option Agreement under the Oncternal Therapeutics, Inc. 2015 Equity Incentive Plan, as amended	S-4	333-230758	10.59	April 8, 2019
10.18#	Restricted Stock Purchase Agreement dated May 22, 2017, between Oncternal Therapeutics, Inc. and Richard G. Vincent	S-4	333-230758	10.60	April 8, 2019
10.19#	Restricted Stock Purchase Agreement dated December 14, 2017, between Oncternal Therapeutics, Inc. and Richard G. Vincent	S-4	333-230758	10.61	April 8, 2019
10.20#	Restricted Stock Purchase Agreement dated December 14, 2017, between Oncternal Therapeutics, Inc. and William R. LaRue	S-4	333-230758	10.62	April 8, 2019
10.21#	Restricted Stock Purchase Agreement dated May 9, 2018, between Oncternal Therapeutics, Inc. and Charles Theuer, M.D., Ph.D.	S-4	333-230758	10.63	April 8, 2019
10.22#	Employment Agreement dated August 26, 2019 between Oncternal Therapeutics, Inc. and Frank Hsu, M.D.	10-Q	000-50549	10.1	November 8, 2019
10.23#	Employment Agreement dated September 5, 2019 between Oncternal Therapeutics, Inc. and Gunnar F. Kaufmann, Ph.D.	10-Q	000-50549	10.2	November 8, 2019
10.24#	Employment Agreement dated September 9, 2019 between Oncternal Therapeutics, Inc. and Igor P. Bilinsky, Ph.D.	10-Q	000-50549	10.3	November 8, 2019
10.25#	Employment Agreement dated September 12, 2019 between Oncternal Therapeutics, Inc. and James B. Breitmeyer, M.D.	10-Q	000-50549	10.4	November 8, 2019
10.26#	Employment Agreement dated September 5, 2019 between Oncternal Therapeutics, Inc. and Richard G. Vincent	10-Q	000-50549	10.5	November 8, 2019
10.27#	Employment Agreement dated September 5, 2019 between Oncternal Therapeutics, Inc. and Hazel M. Aker	10-Q	000-50549	10.6	November 8, 2019
10.28#	Registrant's 2019 Incentive Award Plan effective June 7, 2019	8-K	000-50549	10.2	June 10, 2019
10.29	Amended Oncternal Therapeutics, Inc. Annual Incentive Plan	10-Q	000-50549	10.7	November 8, 2019
10.30	Sublease by and between Oncternal Therapeutics, Inc. and Host Hotels & Resorts, L.P., dated May 22, 2019	10-Q	000-50549	10.23	August 9, 2019
10.31	Form of Indemnification Agreement	10-K	000-50549	10.31	March 16, 2020

10.32#	Consulting Agreement dated September 20, 2019 between Robert J. Wills. Ph.D. and Oncternal Therapeutics, Inc.	10-K	000-50549	10.32	March 16, 2020	
10.34#	Oncternal Therapeutics, Inc. 2021 Employment Inducement Incentive Award Plan and form of stock option agreement thereunder	8-K	000-50549	10.1	February 17, 2021	
10.35	Form of Securities Purchase Agreement, dated May 19, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	10.1	May 21, 2020	
10.36	Form of Securities Purchase Agreement, dated July 17, 2020, between the Registrant and the purchasers signatory thereto	8-K	000-50549	10.1	July 21, 2020	
10.37	Engagement Letter between the Registrant and H.C. Wainwright & Co., LLC, dated as of December 9, 2020					X
10.38#	GTx, Inc. 2001 Stock Option Plan, as amended through November 3, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.6	March 24, 2017	
10.39#	GTx, Inc. 2002 Stock Option Plan, as amended through November 3, 2009, and Form of Stock Option Agreement	10-K	000-50549	10.7	March 24, 2017	
10.40#	GTx, Inc. 2004 Equity Incentive Plan, as originally adopted, and Form of Stock Option Agreement	S-1	333-109700	10.5	January 15, 2004	
10.41#	GTx, Inc. 2004 Equity Incentive Plan, as amended effective April 30, 2008	8-K	000-50549	10.6	May 6, 2008	
10.42#	GTx, Inc. 2004 Equity Incentive Plan, as amended effective November 4, 2008 and Form of Stock Option Agreement	10-K	000-50549	10.10	March 24, 2017	
10.43#	GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan and Form of Stock Option Agreement, as originally adopted	S-1	333-109700	10-6	January 15,2004	
10.44#	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, effective April 26, 2006	8-K	000-50549	10-1	April 27, 2006	
10.45#	Form of Stock Option Agreement under the Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan	10-Q	000-50549	10.35	August 9,2006	
10.46#	Amended and Restated GTx, Inc. 2004 Non-Employee Directors' Stock Option Plan, as amended effective November 4, 2008	10-K	000-50549	10.14	Mar 24, 2017	
10.47#	GTx, Inc. 2013 Equity Incentive Plan, as originally adopted	S-8	333-188377	99.1	May 6, 2013	
10.48#	GTx, Inc. 2013 Equity Incentive Plan, as amended effective May 6, 2015	10-K	000-50549	10.16	March 24, 2017	

10.49#	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan (Standard Form)	10-Q	000-50549	10.2	July 22, 2013	
10.50#	Form of Retention Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.3	November 12, 2013	
10.51#	Form of Retention Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.4	November 12, 2013	
10.52#	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under the GTx, Inc. 2013 Equity Incentive Plan	10-Q	000-50549	10.5	May 11, 2015	
10.53#	GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan, as originally adopted (refiled to reflect reverse stock split effected on December 5, 2016)	10-K	000-50549	10.21	March 24, 2017	
10.54#	Form of Stock Option Grant Notice and Option Agreement under the GTx, Inc. 2013 Non-Employee Director Equity Incentive Plan	10-Q	000-50549	10.4	Jul 22, 2013	
21.1	Subsidiaries	10-K	000-50549	21.1	March 16, 2020	
23.1	Consent of Independent Registered Public Accounting Firm					X
24.1	Power of Attorney (see Signature Page)					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-15(d) and 15d-15(e) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of Principal Financial Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002					X
32.1*	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X

101	The following financial statements from the Oncternal Therapeutics, Inc. Annual Report on Form 10-K for the year ended December 31, 2020, formatted in Inline Extensible Business Reporting Language (iXBRL): (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Operations, (iii) Consolidated Statements of Stockholders' Equity, (iv) Consolidated Statements of Cash Flows, and (v) Notes to Consolidated Financial Statements, tagged as blocks of text and including detailed tags	X
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)	X

* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934 and are not subject to the liability of that section. These certifications are not to be incorporated by reference into any filing of Oncternal Therapeutics, Inc., whether made before or after the date hereof, regardless of any general incorporation language in such filing.

\# Management compensatory plan or arrangement.

† Pursuant to Item 601(b)(10) of Regulation S-K, certain confidential portions of this exhibit were omitted by means of marking such portions with an asterisk because the identified confidential portions (i) are not material and (ii) would be competitively harmful if publicly disclosed.

Signatures

Pursuant to the requirements of the Section 13 or 15(d) of the Securities and Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Oncternal Therapeutics, Inc.

Date: March 12, 2021

By: /s/ James B. Breitmeyer, M.D., PH.D.
James B. Breitmeyer, M.D., Ph.D.
President and Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated

Signature	Title	Date
/s/ James B. Breitmeyer James B. Breitmeyer, M.D., Ph.D.	President, Chief Executive Officer and Member of the Board of Directors *(Principal Executive Officer)*	March 12, 2021
/s/ Richard G. Vincent Richard G. Vincent	Chief Financial Officer *(Principal Financial Officer)*	March 12, 2021
* David F. Hale	Chairman of the Board of Directors	March 12, 2021
* Michael G. Carter, M.D., ChB, FRCP	Director	March 12, 2021
* Man Cho	Director	March 12, 2021
* Daniel L. Kisner	Director	March 12, 2021
* William R. LaRue	Director	March 12, 2021
* Rosemary Mazanet, M.D., Ph.D.	Director	March 12, 2021
* Xin Nakanishi, Ph.D.	Director	March 12, 2021
* Robert Wills, Ph.D.	Director	March 12, 2021
* Charles P. Theuer, M.D., Ph.D.	Director	March 12, 2021

*By: /s/ Richard G. Vincent
Richard G. Vincent
Attorney-in-Fact

AMENDMENT NO. 3
TO THE AMENDED AND RESTATED LICENSE AGREEMENT
BETWEEN ONCTERNAL THERAPEUTICS, INC.
AND THE REGENTS OF THE UNIVERSITY OF CALIFORNIA
FOR UC CASE NUMBERS SD2005-212, SD2020-036, SD2011-178, SD2012-143, SD2012-403, SD2015-027, SD2015-200, SD2018-253 AND SD2019-278

This Amendment No. 3 (“Amendment No.3”) is made as of the Amendment No. 3 Effective Date by and between Oncternal Therapeutics, Inc. having an address at 12230 El Camino Real, Suite 300, San Diego, California 92130 ("LICENSEE") and The Regents of the University of California, a California public corporation having its statewide administrative offices at 1111 Franklin Street, Oakland, California 94607-5200 (“UNIVERSITY”), represented by its San Diego campus having an address at University of California San Diego, Office of Innovation and Commercialization (“OIC”), Mail Code 0910, 9500 Gilman Drive, La Jolla, California 92093-0910 (“UC San Diego”).

WHEREAS, LICENSEE and UNIVERSITY entered into an Amended and Restated License agreement, UC Control Number 2019-03-0137, effective August 31, 2018, which was amended by Amendment No. 1 (“Amendment No. 1”), UC Control Number 2019-03-0137(R501) effective March 25, 2019 and further amended by Amendment No.2 (“Amendment No. 2”), UC Control Number 2019-03-0137(R502) effective May 15, 2019 (collectively, “Agreement”); and

WHEREAS, LICENSEE and UNIVERSITY wish to further amend the Agreement to make certain changes.

NOW THEREFORE, in consideration of the foregoing premises and the mutual covenants set forth below, the parties amend the Agreement and otherwise agree as follows:

1. All capitalized terms used but not defined in this Amendment No,. 3 have the meaning ascribed to them in the Agreement.

2. Section 3.3(a)(A)(v) of the Agreement is hereby deleted and replaced with the following:

   “(v) dose the first patient in the first Phase I/Phase II clinical trial treating any solid tumor with Licensed Product within three (3) years from the Effective Date;”

3. Section 3.3(a)(A)(ix) of the Agreement is hereby deleted and replaced with the following:

   “(ix) dose the first patient in the first Phase III clinical trial for treating any solid tumor with Licensed Product within six (6) years from the Effective Date; and”

4. In consideration for this Amendment No. 3, LICENSEE shall pay an amendment fee in the amount of twenty-five thousand dollars (US $25,000). Payment shall be made within thirty days after the Amendment No. 3 Effective Date.

5. All other terms and conditions of the Agreement shall remain unchanged and in full force and effect. This Amendment No. 3 constitutes an integral part of the Agreement and sets forth, together with the Agreement, the entire agreement between the Parties in respect of the subject matter of this Amendment No. 3 and the Agreement.

6. This Amendment No. 3 shall be governed by, and construed in accordance with, the laws of the State of California, which govern the Agreement. This Amendment No. 3 is effective as of the date of the last signature below (the "Amendment No. 3 Effective Date").

7. The parties agree that this Amendment No. 3 may be executed electronically and in one or more counterparts each of which shall be deemed an original and all of which together shall constitute but one and the same document.

IN WITNESS WHEREOF, the Parties hereto have caused this Amendment No. 3 to be executed by their duly authorized representatives, as of the latest date written below.

ONCTERNAL THERAPEUTICS, INC.:

By: /s/ James Breitmeyer
(Signature)
James Breitmeyer, M.D., Ph.D.
Title: President & CEO

Date: 2/5/21

THE REGENTS OF THE UNIVERSITY OF CALIFORNIA:

By: /s/ Victoria Cajipe
(Signature)
Victoria Cajipe, Ph.D.
Associate Director

Date: 2/5/21

H.C.WAINWRIGHT&CO.

Execution Version

December 9, 2020

STRICTLY CONFIDENTIAL

Oncternal Therapeutics, Inc.
12230 El Camino Real
Suite 300
San Diego, California 92130

Attn: James B. Breitmeyer, M.D., Ph.D., President and Chief Executive Officer

Dear Dr. Breitmeyer:

This letter agreement (this "Agreement") constitutes the agreement between Oncternal Therapeutics, Inc. (the "Company") and H.C. Wainwright & Co., LLC ("Wainwright"), that Wainwright shall serve as the exclusive agent, advisor or underwriter in any offering (each, an "Offering") of securities of the Company (the "Securities") during the Term (as hereinafter defined) of this Agreement. The terms of each Offering and the Securities issued in connection therewith shall be mutually agreed upon by the Company and Wainwright and nothing herein implies that Wainwright would have the power or authority to bind the Company and nothing herein implies that the Company shall have an obligation to issue any Securities. It is understood that Wainwright's assistance in an Offering will be subject to the satisfactory completion of such investigation and inquiry into the affairs of the Company as Wainwright deems appropriate under the circumstances and to the receipt of all internal approvals of Wainwright in connection with an Offering. The Company expressly acknowledges and agrees that Wainwright's involvement in an Offering is strictly on a reasonable best efforts basis and that the consummation of an Offering will be subject to, among other things, market conditions. The execution of this Agreement does not constitute a commitment by Wainwright to purchase the Securities and does not ensure a successful Offering of the Securities or the success of Wainwright with respect to securing any other financing on behalf of the Company. Wainwright may retain other brokers, dealers, agents or underwriters on its behalf in connection with an Offering.

A. Compensation; Reimbursement. At the closing of each Offering (each, a "Closing"), the Company shall compensate Wainwright as follows:

1. *Cash Fee*. The Company shall pay to Wainwright a cash fee, or as to an underwritten Offering an underwriter discount (the "Cash Fee"), equal to 7.0% of the aggregate gross proceeds raised in each Offering, excluding any proceeds raised from any of the investors listed on Exhibit A hereto (the "Excluded Investors").

2. *Warrant Coverage*. The Company shall issue to Wainwright or its designees at each Closing, warrants (the "Wainwright Warrants") to purchase that number of shares of common stock of the Company equal to 6.0% of the aggregate number of shares of

common stock (or common stock equivalent, if applicable) placed in each Offering (and if an Offering includes a "greenshoe" or "additional investment" component, such number of shares of common stock underlying such "greenshoe" or "additional investment" component, with the Wainwright Warrants issuable only upon the exercise of such component). If the Securities included in an Offering are convertible, the Wainwright Warrants shall be determined by dividing the gross proceeds raised in such Offering by the Offering Price (as defined hereunder). The Wainwright Warrants shall be in a customary form reasonably acceptable to Wainwright, have a term of five (5) years and an exercise price equal to 125% of the offering price per share (or unit, if applicable) in the applicable Offering and if such offering price is not available, the market price of the common stock on the date an Offering is commenced (such price, the "Offering Price"). If warrants are issued to investors in an Offering, the Wainwright Warrants shall have the same terms as the warrants issued to investors in the applicable Offering, except that such Wainwright Warrants shall have an exercise price equal to 125% of the Offering Price.

3. *Expense Allowance*. Out of the proceeds of each Closing, the Company also agrees to pay Wainwright (a) a management fee equal to 1.0% of the gross proceeds raised in each Offering; (b) $35,000 for non-accountable expenses (to be increased to $40,000 in case of a public Offering); (c) up to $50,000 for reasonable and documented fees and expenses of legal counsel and other reasonable and documented out-of-pocket expenses (to be increased to $100,000 in case of a public Offering); plus the additional amount payable by the Company pursuant to Paragraph D.3 hereunder and, if applicable, the costs associated with the use of a third-party electronic road show service (such as NetRoadshow); provided, however, that such amount in no way limits or impairs the indemnification and contribution provisions of this Agreement.

4. *Tail*. Wainwright shall be entitled to compensation under clauses (1) and (2) hereunder, calculated in the manner set forth therein, with respect to any public, registered direct or private offering of Securities for capital raising purposes ("Tail Financing") to the extent that such financing or capital is provided to the Company by investors whom Wainwright had contacted during the Initial Term or Extension Term, as applicable, or introduced to the Company during the Initial Term or Extension Term, as applicable, other than any Excluded Investors, but only if (a) Wainwright had presented to the Company terms for a potential Offering during the Initial Term following receipt of investors' input (irrespective of whether the Company accepts such terms), and (b) such Tail Financing is consummated at any time within the 12-month period following the first Closing during the Term. Upon the Company's request, Wainwright shall as promptly as practicable provide a list of any such investors who are subject to this tail provision. Notwithstanding anything herein to the contrary, in the event that Wainwright is offered the right to participate in a transaction, any fees payable by the Company to Wainwright shall be governed by the definitive engagement or transaction agreement with respect to such transaction, and no Tail Financing fee shall be payable by the Company under this Paragraph A.4, irrespective of whether Wainwright accepts such engagement.

B. Term and Termination of Engagement; Exclusivity. The term of Wainwright's exclusive engagement will begin on the date hereof and end forty-five (45) days thereafter (the "Initial Term"); provided, however, that if an Offering is consummated within the Initial Term,

the term of this Agreement shall be extended by an additional forty-five (45) day period (the "Extension Term," and together with the Initial Term, the "Term"). Notwithstanding anything to the contrary contained herein, the Company agrees that the provisions relating to the payment of fees, reimbursement of expenses, tail, indemnification and contribution, confidentiality, conflicts, independent contractor and waiver of the right to trial by jury will survive any termination or expiration of this Agreement. Notwithstanding anything to the contrary contained herein, the Company has the right to terminate the Agreement for cause in compliance with FINRA Rule 5110(g)(5)(B)(i). The exercise of such right of termination for cause eliminates the Company's obligations with respect to the provisions relating to the tail fees. Notwithstanding anything to the contrary contained in this Agreement, in the event that an Offering pursuant to this Agreement shall not be carried out for any reason whatsoever during the Term, the Company shall be obligated to pay to Wainwright its actual and accountable out-of-pocket expenses related to an Offering (including the reasonable fees and disbursements of Wainwright's legal counsel not to exceed the limits set forth in Paragraph A.3 above) and, if applicable, for electronic road show service used in connection with an Offering. During Wainwright's engagement hereunder: (i) the Company will not, and will not permit its representatives to, other than in coordination with Wainwright, contact or solicit institutions, corporations or other entities or individuals as potential purchasers of the Securities (other than the Excluded Investors), provided that this shall not prohibit the Company from engaging in discussions with respect to any potential strategic transaction or merger or acquisition, and (ii) the Company will not pursue any equity or equity-linked financing transaction which would be in lieu of an Offering, other than a financing solely with the Excluded Investors. Furthermore, the Company agrees that during Wainwright's engagement hereunder, all inquiries from prospective investors (other than the Excluded Investors) will be referred to Wainwright. Additionally, except as set forth hereunder, the Company represents, warrants and covenants that no brokerage or finder's fees or commissions are or will be payable by the Company or any subsidiary of the Company to any broker, financial advisor or consultant, finder, placement agent, investment banker, bank or other third-party with respect to any Offering. Notwithstanding anything herein to the contrary, nothing shall prohibit the Company from filing one or more registration statements on Form S-8 or a shelf-registration statement on Form S-3 (but not any takedown therefrom).

C. Information; Reliance. The Company shall furnish, or cause to be furnished, to Wainwright all information reasonably requested by Wainwright for the purpose of rendering services hereunder and conducting due diligence (all such information being the "Information"). In addition, the Company agrees to make available to Wainwright upon request from time to time the officers, directors, accountants, counsel and other advisors of the Company. The Company recognizes and confirms that Wainwright (a) will use and rely on the Information, including any documents provided to investors in each Offering (the "Offering Documents") which shall include any Purchase Agreement (as defined hereunder), and on information available from generally recognized public sources in performing the services contemplated by this Agreement without having independently verified the same; provided that Wainwright shall keep in confidence and shall not provide to investors or potential investors any material non-public Offering Documents that have not been approved in advance by the Company for such use (b) does not assume responsibility for the accuracy or completeness of the Offering Documents or the Information and such other information; and (c) will not make an appraisal of any of the assets or liabilities of the Company. Upon reasonable request, the Company will

meet with Wainwright or its representatives to discuss all information relevant for disclosure in the Offering Documents and will cooperate in any investigation undertaken by Wainwright thereof, including any document included or incorporated by reference therein. At each Offering, at the request of Wainwright, the Company shall deliver such legal letters (including, without limitation, negative assurance letters), opinions, comfort letters, officers' and secretary certificates and good standing certificates, all in form and substance reasonably satisfactory to Wainwright and its counsel as is customary for such Offering. Wainwright shall be a third party beneficiary of any representations, warranties, covenants, closing conditions and closing deliverables made by the Company in any Offering Documents, including representations, warranties, covenants, closing conditions and closing deliverables made to any investor in an Offering.

D. Related Agreements. At each Offering, the Company shall enter into the following additional agreements:

1. *Underwritten Offering*. If an Offering is an underwritten Offering, the Company and Wainwright shall enter into a customary underwriting agreement in form and substance reasonably satisfactory to Wainwright and its counsel.

2. *Best Efforts Offering*. If an Offering is on a best efforts basis, the sale of Securities to the investors in the Offering will be evidenced by a purchase agreement ("Purchase Agreement") between the Company and such investors in a form reasonably satisfactory to the Company and Wainwright. Wainwright shall be a third party beneficiary with respect to the representations, warranties and covenants, closing conditions and closing deliverables included in the Purchase Agreement. Prior to the signing of any Purchase Agreement, officers of the Company with responsibility for financial affairs will be available to answer inquiries from prospective investors.

3. *Escrow, Settlement and Closing*. If each Offering is not settled via delivery versus payment ("DVP"), the Company and Wainwright shall enter into an escrow agreement with a third party escrow agent pursuant to which Wainwright's compensation and expenses shall be paid from the gross proceeds of the Securities sold. If the Offering is settled in whole or in part via DVP, Wainwright shall arrange for its clearing agent to provide the funds to facilitate such settlement. The Company shall pay Wainwright closing costs, which shall also include the reimbursement of the out-of-pocket cost of the escrow agent or clearing agent, as applicable, which closing costs shall not exceed $12,900.

4. *FINRA Amendments*. Notwithstanding anything herein to the contrary, in the event that Wainwright determines that any of the terms provided for hereunder shall not comply with a FINRA rule, including but not limited to FINRA Rule 5110, then the Company shall use commercially reasonable efforts to agree to amend this Agreement (or include such revisions in the final underwriting agreement) in writing upon the request of Wainwright to comply with any such rules; provided that any such amendments shall not provide for terms that are less favorable to the Company than are reflected in this Agreement.

E. Confidentiality. In the event of the consummation or public announcement of any Offering, Wainwright shall have the right to disclose its participation in such Offering, including, without limitation, the Offering at Wainwright's cost of "tombstone" advertisements in financial and other newspapers and journals.

F. Indemnity.

1. In connection with the Company's engagement of Wainwright hereunder, the Company hereby agrees to indemnify and hold harmless Wainwright and its affiliates, and the respective controlling persons, directors, officers, members, shareholders, agents and employees of any of the foregoing (collectively the "Indemnified Persons"), from and against any and all claims, actions, suits, proceedings (including those of shareholders), damages, liabilities and expenses incurred by any of them (including the reasonable fees and expenses of counsel), as incurred (collectively a "Claim"), that are (A) related to or arise out of (i) any actions taken or omitted to be taken (including any untrue statements made or any statements omitted to be made) by the Company, or (ii) any actions taken or omitted to be taken by any Indemnified Person in connection with the Company's engagement of Wainwright, or (B) otherwise relate to or arise out of Wainwright's activities on the Company's behalf under Wainwright's engagement, and the Company shall reimburse any Indemnified Person for all expenses (including the reasonable fees and expenses of counsel) as incurred by such Indemnified Person in connection with investigating, preparing or defending any such claim, action, suit or proceeding, whether or not in connection with pending or threatened litigation in which any Indemnified Person is a party. The Company will not, however, be responsible for any Claim (including any related expenses incurred by an Indemnified Person) that is finally judicially determined to have resulted from the gross negligence or willful misconduct of any such Indemnified Person for such Claim (and, to the extent that the Company has made any payments to an Indemnified Person under this Paragraph F with respect to any such Claim, such Indemnified Person shall, and Wainwright shall cause such Indemnified Person to, promptly return any such payments to the Company following such final judicial determination). The Company further agrees that no Indemnified Person shall have any liability to the Company for or in connection with the Company's engagement of Wainwright except for any Claim incurred by the Company as a result of such Indemnified Person's gross negligence or willful misconduct.

2. The Company further agrees that it will not, without the prior written consent of Wainwright, settle, compromise or consent to the entry of any judgment in any pending or threatened Claim in respect of which indemnification may be sought hereunder (whether or not any Indemnified Person is an actual or potential party to such Claim), unless such settlement, compromise or consent includes an unconditional, irrevocable release of each Indemnified Person from any and all liability arising out of such Claim. No Indemnified Person may, without the prior written consent of the Company, settle, compromise or

consent to the entry of any judgment in any pending or threatened Claim in respect of which indemnification may be sought hereunder (such consent shall not be unreasonably delayed, withheld or conditioned).

3. Promptly upon receipt by an Indemnified Person of notice of any complaint or the assertion or institution of any Claim with respect to which indemnification is being sought hereunder, such Indemnified Person shall notify the Company in writing of such complaint or of such assertion or institution but failure to so notify the Company shall not relieve the Company from any obligation it may have hereunder, except and only to the extent such failure results in the forfeiture by the Company of substantial rights and defenses. If the Company is requested by such Indemnified Person, the Company will assume the defense of such Claim, including the employment of counsel for such Indemnified Person and the payment of the fees and expenses of such counsel, provided, however, that such counsel shall be reasonably satisfactory to the Indemnified Person and provided further that if the legal counsel to such Indemnified Person reasonably determines that having common counsel would present such counsel with a conflict of interest or if the defendant in, or target of, any such Claim, includes an Indemnified Person and the Company, and legal counsel to such Indemnified Person reasonably concludes that there may be legal defenses available to it or other Indemnified Persons different from or in addition to those available to the Company, such Indemnified Person may employ its own separate counsel (including local counsel, if necessary) to represent or defend him, her or it in any such Claim and the Company shall pay the reasonable fees and expenses of such counsel. If such Indemnified Person does not request that the Company assume the defense of such Claim, such Indemnified Person may employ its own separate counsel (including local counsel, if necessary) to represent or defend him, her or it in any such Claim and the Company shall pay the reasonable fees and expenses of such counsel. Notwithstanding anything herein to the contrary, if the Company fails timely or diligently to defend, contest, or otherwise protect against any Claim, the relevant Indemnified Person shall have the right, but not the obligation, to defend, contest, compromise, settle, assert crossclaims, or counterclaims or otherwise protect against the same, and shall be fully indemnified by the Company therefor, including without limitation, for the reasonable fees and expenses of its counsel and all amounts paid as a result of such Claim or the compromise or settlement thereof. Notwithstanding anything herein to the contrary, it is agreed that the Company shall not, in connection with any proceeding or related proceedings, be liable for the fees and expenses of more than one separate firm for all Indemnified Persons (including local counsel, if necessary). In addition, with respect to any Claim in which the Company assumes the defense, the Indemnified Person shall have the right to participate in such Claim and to retain his, her or its own counsel therefor at his, her or its own expense.

4. The Company agrees that if any indemnity sought by an Indemnified Person hereunder is held by a court to be unavailable for any reason then (whether or not Wainwright is the Indemnified Person), the Company and Wainwright shall

contribute to the Claim for which such indemnity is held unavailable in such proportion as is appropriate to reflect the relative benefits to the Company, on the one hand, and Wainwright on the other, in connection with Wainwright's engagement referred to above, subject to the limitation that in no event shall the amount of Wainwright's contribution to such Claim exceed the amount of fees actually received by Wainwright from the Company pursuant to Wainwright's engagement. The Company hereby agrees that the relative benefits to the Company, on the one hand, and Wainwright on the other, with respect to Wainwright's engagement shall be deemed to be in the same proportion as (a) the total value paid or proposed to be paid or received by the Company pursuant to the applicable Offering (whether or not consummated) for which Wainwright is engaged to render services bears to (b) the fee paid or proposed to be paid to Wainwright in connection with such engagement.

5. The Company's indemnity, reimbursement and contribution obligations under this Agreement (a) shall be in addition to, and shall in no way limit or otherwise adversely affect any rights that any Indemnified Person may have at law or at equity and (b) shall be effective whether or not the Company is at fault in any way.

G. Limitation of Engagement to the Company. The Company acknowledges that Wainwright has been retained only by the Company, that Wainwright is providing services hereunder as an independent contractor (and not in any fiduciary or agency capacity) and that the Company's engagement of Wainwright is not deemed to be on behalf of, and is not intended to confer rights upon, any shareholder, owner or partner of the Company or any other person not a party hereto as against Wainwright or any of its affiliates, or any of its or their respective officers, directors, controlling persons (within the meaning of Section 15 of the Securities Act or Section 20 of the Securities Exchange Act of 1934, as amended (the "Exchange Act")), employees or agents. Unless otherwise expressly agreed in writing by Wainwright, no one other than the Company is authorized to rely upon this Agreement or any other statements or conduct of Wainwright, and no one other than the Company is intended to be a beneficiary of this Agreement. The Company acknowledges that any recommendation or advice, written or oral, given by Wainwright to the Company in connection with Wainwright's engagement is intended solely for the benefit and use of the Company's management and directors in considering a possible Offering, and any such recommendation or advice is not on behalf of, and shall not confer any rights or remedies upon, any other person or be used or relied upon for any other purpose. Wainwright shall not have the authority to make any commitment binding on the Company. The Company, in its sole discretion, shall have the right to reject any investor introduced to it by Wainwright.

H. Limitation of Wainwright's Liability to the Company. Wainwright and the Company further agree that neither Wainwright nor any of its affiliates or any of its or their respective officers, directors, controlling persons (within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act), employees or agents shall have any liability to the Company, its security holders or creditors, or any person asserting claims on behalf of or in the right of the Company (whether direct or indirect, in contract, tort, for an act of negligence or otherwise) for any losses, fees, damages, liabilities, costs, expenses or equitable relief arising

out of or relating to this Agreement or the services rendered hereunder, except for losses, fees, damages, liabilities, costs or expenses that arise out of or are based on any action of or failure to act by Wainwright and that are finally judicially determined to have resulted solely from the gross negligence or willful misconduct of Wainwright.

I. Representations Warranties and Covenants of Placement Agent. Wainwright hereby represents and warrants to the Company that the following representations and warranties are true and correct as of the date of this Agreement:

1. Wainwright is a member in good standing of FINRA and is registered as a broker-dealer under the Exchange Act. Wainwright is in compliance with all applicable rules and regulations of the SEC and FINRA, except to the extent that such noncompliance would not have a material adverse effect on the transactions contemplated hereby. None of Wainwright or its affiliates, or any person acting on behalf of the foregoing (other than the Company or its affiliates or any person acting on its or their behalf, in respect of which no representation is made) has or will engage in general advertising or general solicitation or has taken nor will it take any action that conflicts with the conditions and requirements of, or that would make unavailable with respect to the Offering, the exemption(s) from registration available pursuant to Rule 506 of Regulation D or Section 4(a)(2) of the Act.

2. Neither Wainwright nor any Wainwright Related Persons (as defined below) are subject to any Disqualification Event. Any prospectus related to the Offering will contain a true and complete description of the matters required to be disclosed with respect to Wainwright and Wainwright Related Persons pursuant to the disclosure requirements of Rule 506(e) of Regulation D, to the extent applicable. As used herein, "Wainwright Related Persons" means any predecessor of Wainwright, any affiliated company, any director, executive officer, other officer of Wainwright participating in the Offering, any general partner or managing member of Wainwright, any beneficial owner of 20% or more of Wainwright's outstanding voting equity securities, calculated on the basis of voting power, and any "promoter" (as defined in Rule 405 under the Act) connected with Wainwright in any capacity. Wainwright agrees to promptly notify the Company in writing of (i) any Disqualification Event relating to any Wainwright Related Person and (ii) any event that would, with the passage of time, become a Disqualification Event relating to any Wainwright Related Person.

J. Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the State of New York applicable to agreements made and to be fully performed therein. Any disputes that arise under this Agreement, even after the termination of this Agreement, will be heard only in the state or federal courts located in the City of New York, State of New York. The parties hereto expressly agree to submit themselves to the jurisdiction of the foregoing courts in the City of New York, State of New York. The parties hereto expressly waive any rights they may have to contest the jurisdiction, venue or authority of any court sitting in the City and State of New York. In the event Wainwright or any Indemnified Person is successful in any action, or suit against the Company, arising out of or relating to this Agreement, the final judgment or award entered shall be entitled to have and recover from the Company the costs and expenses incurred in connection therewith, including its reasonable attorneys' fees. Any rights to trial by jury with respect to any such action, proceeding or suit are hereby waived by Wainwright and the Company.

K. Notices. All notices hereunder will be in writing and sent by certified mail, hand delivery, overnight delivery or e-mail, if sent to Wainwright, at the address set forth on the first page hereof, e-mail: notices@hcwco.com, Attention: Head of Investment Banking, and if sent to the Company, to the address set forth on the first page hereof, e-mail: JBreitmeyer@oncternal.com, Attention: Chief Executive Officer. Notices sent by certified mail shall be deemed received five days thereafter, notices sent by hand delivery or overnight delivery shall be deemed received on the date of the relevant written record of receipt, notices sent by e-mail shall be deemed received as of the date and time they were sent.

L. Conflicts. The Company acknowledges that Wainwright and its affiliates may have and may continue to have investment banking and other relationships with parties other than the Company pursuant to which Wainwright may acquire information of interest to the Company. Wainwright shall have no obligation to disclose such information to the Company or to use such information in connection with any contemplated transaction.

M. Anti-Money Laundering. To help the United States government fight the funding of terrorism and money laundering, the federal laws of the United States require all financial institutions to obtain, verify and record information that identifies each person with whom they do business. This means Wainwright must ask the Company for certain identifying information, including a government-issued identification number (e.g., a U.S. taxpayer identification number) and such other information or documents that Wainwright considers appropriate to verify the Company's identity, such as certified articles of incorporation, a government-issued business license, a partnership agreement or a trust instrument.

N. Miscellaneous. The Company represents and warrants that it has all requisite power and authority to enter into and carry out the terms and provisions of this Agreement and the execution, delivery and performance of this Agreement does not breach or conflict with any agreement, document or instrument to which it is a party or bound. This Agreement shall not be modified or amended except in writing signed by Wainwright and the Company. This Agreement shall be binding upon and inure to the benefit of both Wainwright and the Company and their respective assigns, successors, and legal representatives. If any provision of this Agreement is determined to be invalid or unenforceable in any respect, such determination will not affect such provision in any other respect, and the remainder of the Agreement shall remain in full force and effect. This Agreement may be executed in counterparts (including facsimile or electronic counterparts), each of which shall be deemed an original but all of which together shall constitute one and the same instrument.

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In acknowledgment that the foregoing correctly sets forth the understanding reached by Wainwright and the Company, please sign in the space provided below, whereupon this letter shall constitute a binding Agreement as of the date indicated above.

Very truly yours,

H.C. WAINWRIGHT & CO., LLC

By: /s/ Mark W. Viklund
Name: Mark W. Viklund
Title: Chief Executive Officer
Date: December 9, 2020

Accepted and Agreed:

ONCTERNAL THERAPEUTICS, INC.

By: /s/ Richard G. Vincent
Name: Richard G. Vincent
Title: Chief Financial Offer

Consent of Independent Registered Public Accounting Firm

Oncternal Therapeutics, Inc.
San Diego, California

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (Nos. 333-221040, 333-204932, 333-201132 and 333-197911) and Forms S-8 (Nos. 333-233288, 333-223742, 333-210220, 333-208744, 333-188377, 333-165507, 333-149661, 333-136527, 333-118882, and 333-112576) of Oncternal Therapeutics, Inc., of our report dated March 11, 2021, relating to the consolidated financial statements, which appears in this Annual Report on Form 10-K/A.

/s/ BDO USA, LLP

San Diego, California
March 12, 2021

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, James B. Breitmeyer, certify that:

1. I have reviewed this Annual Report on Form 10-K of Oncternal Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the consolidated financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ James B. Breitmeyer
President and Chief Executive Officer
(Principal Executive Officer)

Dated: March 12, 2021

Exhibit 31.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Richard G. Vincent, certify that:

1. I have reviewed this Annual Report on Form 10-K of Oncternal Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the consolidated financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Richard G. Vincent
Chief Financial Officer
(Principal Financial Officer)

Dated: March 12, 2021

Exhibit 32.1

CERTIFICATION
Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
(Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code)

In connection with the Annual Report on Form 10-K of Oncternal Therapeutics, Inc. (the "Company") for the period ended December 31, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, James B. Breitmeyer, as Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ James B. Breitmeyer
President and Chief Executive Officer
(Principal Executive Officer)

Dated: March 12, 2021

The foregoing certification is being furnished solely to accompany the Report pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Company, whether made before or after the date hereof, regardless of any general incorporation language in such filing. A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Exhibit 32.2

CERTIFICATION
Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
(Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code)

In connection with the Annual Report on Form 10-K of Oncternal Therapeutics, Inc. (the "Company") for the period ended December 31, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Richard G. Vincent, as Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Richard G. Vincent
Chief Financial Officer
(Principal Financial Officer)

Dated: March 12, 2021

The foregoing certification is being furnished solely to accompany the Report pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Company, whether made before or after the date hereof, regardless of any general incorporation language in such filing. A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.