



Oncternal Awarded Exclusive Worldwide License to ROR1 Antibody and Related Programs from UCSD

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Pioneering Research at UC San Diego Provides Foundation for New Cancer Focused Start-Up

SAN DIEGO, April 12, 2016 – Oncternal Therapeutics, Inc, a new oncology-focused biotechnology company, today announced that it has received an exclusive worldwide license to develop and commercialize antibodies and antibody-related binding agents recognizing Receptor-tyrosine kinase-like orphan receptor 1 (ROR1) from University of California San Diego. The licensing agreement also encompasses rights for all therapeutic indications to [cirmtuzumab](#), an anti-ROR1 monoclonal antibody that is currently in a clinical trial for patients with chronic lymphocytic leukemia (CLL), as well as rights to develop antibody-drug conjugates (ADCs), genetically modified effector immune cells, such as chimeric antigen receptor T-cells (CAR-T), and bispecific antibodies.

The formation of Oncternal Therapeutics is based upon the pioneering research of UC San Diego scientist and clinician, [Thomas J Kipps, MD, PhD](#), Evelyn and Edwin Tasch Chair in Cancer Research, Distinguished Professor UC San Diego, and Deputy Director, Research Operations, Moores Cancer Center. As one of the most wide-ranging licensing transactions executed by UC San Diego's Office of Innovation and Commercialization, the agreement includes upfront and milestone payments, research funding for the Kipps laboratory, clinical support, product royalties, and an equity position in Oncternal. Specific financial terms of the agreement were not disclosed.

"We are delighted to complete this agreement with Oncternal Therapeutics, as it furthers our goal of developing effective therapies for patients with any one of a number of types of cancer, including leukemia and lymphoma, as well as solid tumors, such as ovarian or breast cancer," said Dr. Kipps. "We are very pleased to see our discovery, research and early clinical development program trigger the formation of a new biotechnology company in San Diego led by an experienced leadership team."

"The scientific work done by Dr. Kipps and his colleagues has been extraordinary and holds great promise to generate a new wave of first-in-class, targeted cancer therapies," said [David F Hale](#), Chairman of Oncternal Therapeutics. "We are excited to build a world-class company focused on the development of these promising ROR1 programs, which feature novel science and a strong intellectual property portfolio that we plan to enhance in the future. We look forward to rapidly advancing cirmtuzumab into additional clinical trials and bringing forward novel therapeutics for the treatment of patients with these devastating diseases."

The anti-ROR1 monoclonal antibody, cirmtuzumab, was developed at UC San Diego by Dr. Kipps, with funding from the US National Institutes of Health, the California Institute for Regenerative Medicine (CIRM), and the Blood Cancer Research Fund. Cirmtuzumab is currently being tested in a phase 1 clinical trial for patients with relapsed/refractory CLL. Studies have shown that ROR1 is expressed by a number of hematologic malignancies as well as by a number of solid tumors.

"Creating a treatment that will help patients is not just a matter of good scientific work, it's also a matter of good business planning and leadership. This agreement brings those two elements together," says C. Randal Mills, President and CEO of CIRM. "We are hopeful this alliance will help accelerate the progress of Dr. Kipps research, and we congratulate him and his team at UC San Diego."

Oncternal will also continue development of several preclinical development programs initiated by UC San Diego, including potential ROR1-directed ADCs, with early evidence of promising anti-tumor efficacy and specificity, and several potential CAR-T vectors, which can be introduced into a cancer patient's T-cells, enabling the cells to recognize and kill tumor cells that express ROR1. Oncternal Therapeutics intends to continue the development of the CAR-T program in collaboration with the Moores Cancer Center.

"One of our missions as a public university is to translate our research into public benefit," said Paul Roben, Associate Vice Chancellor for Innovation at UC San Diego. "This successful collaboration exemplifies that commitment to social responsibility."

Cirmtuzumab

Cirmtuzumab is a humanized IgG1 monoclonal antibody that was designed and developed to bind with high affinity to a biologically important epitope on the extracellular domain of ROR1. Binding of cirmtuzumab to ROR1 on tumor cells inhibits Wnt5a signalling, a pathway that is important for tumor-cell proliferation, migration, and survival. Blockade of this Wnt5a signaling leads to tumor cell death by apoptosis.

About ROR1

Receptor-tyrosine kinase-like orphan receptor 1 — also known as ROR1 — is a type 1 transmembrane protein that is expressed on the plasma membrane with an extracellular domain that is essential for ligand binding and signal transduction. High-level expression of ROR1 has been found in many cancers. The highest and most consistent expression of the ROR1 protein is in mantle cell lymphoma (MCL) where virtually all patient samples express ROR1, and CLL, where 95 percent express ROR1 on the cell surface. ROR1 is also expressed at high frequency in many solid tumors, including lung and ovarian cancers, as well as triple-negative breast cancer.

About Oncternal Therapeutics

Oncternal Therapeutics has been formed around the groundbreaking research at UC San Diego of Dr. Thomas Kipps on the ROR1 signaling system, and the development of antibodies and related binding agents to treat ROR1-expressing cancers. The Oncternal leadership team has extensive

experience in the formation and successful development of biotechnology companies and innovative pharmaceutical products. The founders and initial board members include [David F Hale, Cam Garner, Scott Glenn, and James B Breitmeyer](#), who have been involved in founding and/or development of a number of life sciences companies in San Diego. Hale and Garner were members of the senior management team of Hybritech with its subsequent sale to Eli Lilly and Company. One or more of the founders have had key roles with Gensia, Dura, CancerVax, Santarus, SkinMedica, Quidel, Dexcom, Neurelis, Cadence, Evoke Pharma, Zogenix, and a number of other life sciences companies in San Diego. For more information, visit www.oncternal.com.