Ceptur Therapeutics Launches with $75M Series A Financing to Advance RNA Therapeutics Based on Proprietary U1 Adaptor Technology

- Financing co-led by venBio Partners & Qiming Venture Partners with participation from strong syndicate of new and existing investors
- Pioneering new wave of oligonucleotide-based genetic medicines through partnership of foundational leadership team with world-renowned Scientific Advisory Board

Philadelphia – (Business Wire) - Ceptur Therapeutics, Inc. ("Ceptur") - a biotechnology company - announces the completion of a $75M Series A financing round led by venBio Partners and Qiming Venture Partners; the round was further supported by new investors Perceptive Xontogeny Venture (PXV) Fund, Bristol Myers Squibb and Janus Henderson along with existing Seed investors Affinity Asset Advisors, Boxer Capital and LifeSci Venture Partners.

"We are extremely grateful for the support of our new and existing investors," says Ceptur Therapeutics co-founder, President and CEO P. Peter Ghoroghchian, MD, PhD. "In 2021, we in-licensed and internally expanded our foundational IP portfolio on U1 Adaptor technology; we further recruited a world-class scientific advisory board comprised of academic and industry leaders in oligonucleotide therapeutics. Moving forward, Ceptur will use the proceeds of this financing round to advance our broad discovery pipeline of differentiated genetic medicines."

U1 Adaptors are bivalent oligonucleotides that engage gene-specific mRNA and the U1 small nuclear ribonuclear protein (U1 snRNP), which is a ubiquitous intracellular machine that regulates transcription and splicing. U1 Adaptor therapeutics control gene-specific expression at the pre-mRNA level within the nucleus; they further afford advantageous properties for drugging difficult targets, in extrahepatic tissues, and in a non-genotoxic manner.

"Therapeutic approaches that target RNA have become an essential treatment modality for patients with genetic diseases and a priority for many biopharma companies; we believe that the U1 Adaptor technology is a differentiated approach to RNA regulation that has multiple potential advantages over current technologies", says Aaron Royston, M.D., M.B.A., Managing Partner at venBio Partners. "We are excited to further build out Ceptur's team and capabilities, to demonstrate these unique applications, and, ultimately, to advance novel therapeutics for patients with genetic diseases."

Colin Walsh, Ph.D., Partner at Qiming Venture Partners, adds, “RNA therapeutics are already revolutionizing medicine and there are multiple exciting developments underway. Ceptur’s use of synthetic oligonucleotides that engage U1 snRNP offers the ability to co-opt the natural function of this highly abundant and evolutionarily conserved master regulator of the transcriptome, modifying mRNA in a gene-specific fashion. We are delighted to support the further buildout of this disruptive approach to enable therapeutic applications that are not currently possible using existing methods.”

With this financing, Aaron Royston, M.D., M.B.A., and Colin Walsh, Ph.D., join Ceptur’s Board of Directors.

Daniel Heller, M.S., M.B.A., General Partner and Chief Investment Officer at Affinity Asset Advisors, continues, “In leading the Series Seed round, we identified early the potential of U1 Adaptor technology. Over the past year, we have worked closely with Peter and the Ceptur team and are delighted at the progress that has been made towards establishing the platform. In this financing round, we have significantly expanded upon our initial commitment and are inspired to partner with our new investor syndicate to advance U1 Adaptors for unmet patient needs.”

To realize the revolutionary potential of the U1 Adaptor technology, several new members join Samuel Gunderson, Ph.D., co-founder of Ceptur, Professor of Molecular Biology at Rutgers University, and expert on U1 snRNP biology, on Ceptur’s Scientific Advisory Board:

Thomas Andresen, Ph.D.
Dr. Thomas L. Andresen is the CEO of T-Cypher Bio and the former CSO of Torque Therapeutics, now Repertoire Immune Medicines. While at Torque, he led the company’s cellular immunotherapy programs from early-stage discovery to CMC scaling and through to clinical development. Dr. Andresen is a serial entrepreneur, having founded several US and EU life-science companies that further include Nanovi A/S and Monta Biosciences. His company creation track record spans early discovery to commercial and maps across multiple immunotherapy approaches for oncology. Dr. Andresen sits on several boards/advisory boards, including for Tidal Therapeutics (acquired by Sanofi), Monta Biosciences, and Nanovi; in academia, he’s further founded the Institute of Health Technology at the Technical University of Denmark, where he maintains a professorial position. Dr. Andresen has co-authored over >200 research articles, has been listed as an inventor on >45 patent applications, and has received multiple research prizes, including the Elite Research Price from the Danish Ministry of Science.

Dennis Benjamin, Ph.D.
Dr. Dennis Benjamin is the former SVP of Research at Seattle Genetics where he was a key developer of the company’s ADC technology and clinical pipeline. Prior, he worked at Praecis Pharmaceuticals and Genetics Institute, advancing DNA encoded libraries and working in protein and small molecule discovery. Over his career he has led teams that have discovered 25 biologics and small molecules that entered clinical trials and contributed to 4 drug approvals. He is currently an advisor and SAB member at several start-up biotechnology companies.

Steven Dowdy, Ph.D.
Dr. Steven F. Dowdy is a Professor of Cellular & Molecular Medicine at the UCSD School of Medicine and a cancer biologist, specializing in the development and delivery of RNA therapeutics as well as in G1 cell cycle control in cancer. The Dowdy lab is focused on the molecular details of delivery of RNA therapeutics across the endosomal lipid bilayer as well as the synthesis of endosomal escape domains to overcome this rate-limiting and billion year-old delivery challenge; its members were the first to synthesize bioreversible, charge neutralizing phosphotriester backbone RNAi prodrug triggers that increase metabolic stability, that augment pharmacokinetics and that enhance endosomal escape. Dr. Dowdy currently serves on five Science Advisory Boards for biotech companies and is an elected member of the Oligonucleotide Therapeutics Society (OTS) Board of Directors.

Sridhar Ganesan, M.D., Ph.D.
Dr. Shridar Ganesan is the Associate Director for Translational Science, Chief of the Section of Molecular Oncology, and the co-Leader of the Clinical Investigations and Precision Therapeutics Program at the Rutgers Cancer Institute of New Jersey; he is also the Omar Boraie Chair in Genomic Science and Professor of Medicine at the Rutgers Robert Wood Johnson Medical School. Dr. Ganesan is a medical oncologist with clinical expertise in triple-negative breast cancer, hereditary breast cancer and rare cancer. His research interests include the characterization of DNA repair abnormalities in cancer with a focus on the BRCA1 tumor suppressor gene, the multi-modal molecular characterizations of different cancers, and the identification of biomarkers of response and resistance in early phase clinical trials. He has authored or co-authored over 120 publications, serves on multiple national and international grant review committees and is an Associate Editor of JCO-Precision Oncology.

Adrian Krainer, Ph.D.
Dr. Adrian Krainer is the St Giles Professor at Cold Spring Harbor Laboratory (CSHL) and Deputy Director of Research of the CSHL Cancer Center. A world-renowned biochemist recognized for his basic work on RNA splicing and the development of its mechanism-based therapeutic applications, his seminal work with antisense oligonucleotides in mouse models of spinal muscular atrophy led to the development of nusinersen (Spinraza), which is the first FDA-approved drug for this disease; he is also a co-founder and a member of the Board of Directors at Stoke Therapeutics (NASDAQ: STOK). Dr. Krainer is the recipient of the Life Sciences Breakthrough Prize, the RNA Society's Lifetime Achievement Award, the Reemtsma Foundation International Prize in Translational Neuroscience, the Speiser Award in Pharmaceutical Sciences, and the Ross Prize in Molecular Medicine, amongst others. He previously served as the President of the RNA Society and is a member of the National Academy of Sciences, the National Academy of Medicine, the National Academy of Inventors, and the American Academy of Arts & Sciences.

Iain Mattaj, Ph.D.
Dr. Iain Mattaj is the founding Director of Fondazione Human Technopole in Milan, Italy. Dr. Mattaj has made seminal contributions to the fields of transcription, RNA metabolism, nucleocytoplasmic transport and cell division. His prominent standings in these fields are underlined by his election as the past President of the RNA Society, Fellow of the Royal Society

(London), Fellow of the Royal Society of Edinburgh, elected Member of the German Academy of Sciences Leopoldina, Member of Academia Europea, Foreign Honorary Member of the American Academy of Arts and Science, Fellow of the Academy of Medical Sciences (London) and Foreign Associate of the National Academy of Sciences (US). Dr. Mattaj was previously awarded the prestigious Louis-Jeantet Prize for Medicine in 2001. He is further a member of the European Molecular Biology Organization (EMBO) and helped to make The EMBO Journal a highly successful international publication, acting as Executive Editor from 1990 to 2004. Prior to his current appointment, Dr. Mattaj was a member of EMBL Heidelberg, Germany, serving as Group Leader (1985-1990), Coordinator of the Gene Expression Unit (1990-1999), and, subsequently, as the institute's Scientific Director (1999-2005) and Director General (2005-2018).

Henrik Oerum, Ph.D.
Dr. Henrik Oerum the co-founder and Chief Scientific Officer of Civi BioPharma and has previously founded 3 other oligonucleotide companies. Dr. Oerum has over 25 years of experience in the development and commercialization of oligonucleotide therapeutics, has authored >70 peer reviewed publications, and has been listed as an inventor on numerous patents in the field. In 1993, he founded PNA Diagnostics A/S, where he was also the Chief Scientific Officer until 1999. During his tenure at PNA, the company was sold to Boehringer Mannheim (1994) and later to Hoffman-La Roche (1997). In 1996, Dr. Oerum cofounded Exiqon A/S, a nucleic acid diagnostics company that was floated on the Copenhagen Stock Exchange in 2007 (CSE:EXQ). In 2000, he co-founded the LNA-oligotherapeutics company Santaris Pharma A/S, where he served as Chief Scientific Officer and Vice President of Business Development until its acquisition by Roche in 2014. Thereafter, he worked for Roche Pharma as Global Head of RNA therapeutics until March 2016, where he left to pursue new opportunities, leading to his founding of CiVi.

Thomas Tuschl, Ph.D.
Dr. Thomas Tuschl is a Professor of RNA Molecular Biology at Rockefeller University. Dr. Tuschl is world renown for his research into the regulation of RNA and has discovered small interfering RNAs (siRNAs), microRNAs (miRNAs) and piwi-interacting RNAs (piRNAs). He is a member of the German National Academy of Sciences and the recipient of numerous awards, including the NIH Director's Transformative Research Project Award, the Ernst Jung Prize, the Max Delbrück Medal, the Molecular Bioanalytics Prize, the Meyenburg Prize, the Wiley Prize and the AAAS Newcomb Cleveland Prize. He is also the co-founder and a former Director of Alnylam Pharmaceuticals (NASDAQ: ALNY).

About Ceptur Therapeutics, Inc.

Headquartered in Hillsborough New Jersey, Ceptur Therapeutics is a pre-clinical stage biotechnology company focused on developing targeted oligonucleotide therapeutics based on U1 Adaptor technology. For more information about Ceptur Therapeutics, please visit www.cepturtx.com or follow www.linkedin.com/company/ceptur-therapeutics/ on Linkedin.

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