



Vertex and TreeFrog Therapeutics Announce Licensing Agreement and Collaboration to Optimize Production of Vertex's Cell Therapies for Type 1 Diabetes

April 23, 2024

- Vertex obtains an exclusive license to TreeFrog's C-Stem™ manufacturing technology in type 1 diabetes -

- TreeFrog and Vertex to collaborate on scale-up of fully differentiated, insulin-producing pancreatic islet cells -

BOSTON and BORDEAUX, France – April 23, 2024 – [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) and TreeFrog Therapeutics today announced that Vertex has obtained an exclusive license to TreeFrog's proprietary cell manufacturing technology, C-Stem™, to optimize production of Vertex's cell therapies for type 1 diabetes (T1D). TreeFrog and Vertex will collaborate to scale-up TreeFrog's process to produce and amplify cells for Vertex's T1D therapies.

TreeFrog's proprietary technology platform, C-Stem™, is designed to mimic the natural microenvironment, allowing cells to grow exponentially in 3D. The technology will enhance Vertex's ability to generate large amounts of fully differentiated cells for its portfolio of T1D cell therapies.

"Our goal is to transform the treatment of T1D, and our stem cell-derived, fully differentiated islet cell VX-880 Ph 1/2 program has demonstrated this potential," said Morrey Atkinson Ph.D., Executive Vice President, Chief Technical Operations Officer, Head of Biopharmaceutical Sciences and Manufacturing Operations, at Vertex. "We're excited to explore TreeFrog's C-Stem™ to scale up stem cell production and deliver for the large number of people living with T1D."

"We are delighted to partner with Vertex Pharmaceuticals, the clear leader in cell therapy for T1D. This is a fantastic opportunity to apply and further develop our C-Stem™ technology for a first-in-class cell therapy treatment which could meet a huge unmet need," said Frédéric Desdouits, Ph.D., CEO of TreeFrog Therapeutics. "This collaboration with Vertex advances our business strategy of helping bring best-in-class cell therapies to millions of people — both through our own therapeutic programs, and with best-in-class partners like Vertex. We look forward to combining TreeFrog's extensive experience in cell therapy production and biology with Vertex's renowned expertise in T1D and cell therapy to bring these transformative therapies to patients."

About the Transaction

Under the agreement, TreeFrog will receive a \$25M upfront payment, an equity investment from Vertex and up to \$215M in milestones paid in connection with the development of a scaled-up process for fully differentiated islet cells. TreeFrog is also eligible to receive an additional \$540M in clinical, regulatory and commercial milestones on up to two future products and tiered single digit royalties. Vertex will fund all research and development costs related to the collaboration. Vertex will be responsible for all development and commercialization of its cell therapies.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including acute and neuropathic pain, APOL1-mediated kidney disease, autosomal dominant polycystic kidney disease, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on [LinkedIn](#), [Facebook](#), [Instagram](#), [YouTube](#) and [Twitter/X](#).

About TreeFrog Therapeutics

TreeFrog Therapeutics is a French-based biotech company set to unlock access to cell therapies for millions of patients. TreeFrog is unique in its approach to cell therapy development bringing together biophysicists, cell biologists and bioproduction engineers to address the challenges of the industry — producing and differentiating cells of quality at unprecedented scale, cost-effectively. To realize their mission of **Cell Therapy for all**, TreeFrog operates a hybrid business model that includes its own therapeutic programs and partnerships with leading biotech and industry players in other areas. In the last 3 years, the company has raised \$82 million to advance a pipeline of stem cell-based therapies in regenerative medicine. In 2022, the company opened technological hubs in Boston, USA, and Kobe, Japan, to drive the adoption of the C-Stem™ platform and establish strategic alliances with leading academic, biotech and industry players.

www.treefrog.fr

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements by Morrey Atkinson, Ph.D., and Frédéric Desdouits, Ph.D., in this press release, statements about the terms of and expectations for Vertex's collaboration with TreeFrog, potential benefits and results that may be achieved through the collaboration to scale-up TreeFrog's process to produce and amplify cells for Vertex's T1D therapies, including the expectation that the technology will enhance Vertex's ability to generate large amounts of fully differentiated cells, statements regarding the future activities of the parties pursuant to the collaboration, and statements regarding upfront and milestone payments, Vertex's expected equity investment in TreeFrog and potential royalties on future products.

While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the anticipated benefits and potential of Vertex's collaboration with TreeFrog may not be achieved on the anticipated timeline, or at all, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy, or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

Vertex Pharmaceuticals Incorporated

Investors:

InvestorInfo@vrtx.com

or

617-961-7163

Media:

mediainfo@vrtx.com

or

U.S.: 617-341-6992

or

Heather Nichols: +1 617-839-360

or

International: +44 20 3204 5275

TreeFrog Therapeutics

Rachel Mooney

Chief Communications Officer

Rachel.mooney@treefrog.fr

Tel: +33 674063461