



Vertex to Acquire Semma Therapeutics With a Goal of Developing Curative Cell-Based Treatments for Type 1 Diabetes

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-Semma's unique investigational approach combines robust production process of pancreatic islet cells with proprietary delivery system to restore insulin secretion in type 1 diabetes patients-

-Semma to be acquired for \$950 million in cash-

BOSTON & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 3, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced that the company has entered into a definitive agreement under which Vertex will acquire Semma Therapeutics, a privately held biotechnology company pioneering the use of stem cell-derived human islets as a potentially curative treatment for type 1 diabetes, for \$950 million in cash. Semma has demonstrated a differentiated approach to treat type 1 diabetes, a serious disease affecting over one million people in the United States alone. Semma has made two major scientific advances: the ability to produce large quantities of functional human pancreatic beta cells that restore insulin secretion and ameliorate hypoglycemia in animal models and a novel device that encapsulates and protects these cells from the immune system, enabling durable implantation without the need for ongoing immunosuppressive therapy.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20190903005227/en/>

"This acquisition aligns perfectly with our strategy of investing in scientific innovation to create transformative medicines for people with serious diseases in specialty markets," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We are excited to work with the talented scientists at Semma to build on their significant progress toward providing effective and potentially curative cell therapy options for people living with type 1 diabetes. We see a substantial opportunity to transform the treatment paradigm for type 1 diabetes, a specialty disease cared for by endocrinologists, both by advancing the development and manufacturing of the cells themselves, as well as through the highly innovative cell/device combination."

"The therapeutic approach pioneered by Semma has the potential to address the causal human biology of type 1 diabetes, a serious disease inadequately controlled by existing therapies. Unlike insulin injections and insulin pumps, islet cell transplantation can provide physiologic regulation of blood glucose thereby potentially ameliorating or preventing both the hyperglycemic and hypoglycemic episodes associated with the current standards of care," said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research and Chief Scientific Officer of Vertex. "Their compelling proof-of-concept data in animals demonstrates the opportunity to develop transformative and potentially curative therapies to treat people with type 1 diabetes. In addition, the acquisition of Semma continues to expand the Vertex toolbox of cutting edge technologies and capabilities, and bolsters our team of leading scientists."

"Type 1 diabetes is a disease that afflicts millions of people worldwide and has no curative therapies available," said Bastiano Sanna, Ph.D., President and Chief Executive Officer of Semma. "Vertex has a proven track record of serial innovation and a deep commitment to developing transformative therapies for patients in need. Being a part of Vertex will allow the Semma team to rapidly and effectively advance our cell therapy and delivery approaches to patients who need them."

"Semma was founded to dramatically improve the lives of patients with type 1 diabetes," said Douglas Melton, Ph.D., Scientific Founder of Semma. "Vertex is ideally suited to accelerate the achievement of this goal."

About Semma Therapeutics

Semma was founded by Douglas Melton, Ph.D. and others to develop transformative therapies for patients who currently depend on insulin injections. The company is focused on advancing Dr. Melton's method of generating billions of functional, insulin-producing beta cells grown from stem cells in the laboratory, which develop in islet-like clusters. Initial preclinical work in animal models of diabetes has shown that transplantation of these cells by infusion into the liver is sufficient to control blood glucose levels. This breakthrough technology has been exclusively licensed to Semma for the development of a cell-based therapy for diabetes.

In addition to pursuing direct intra-hepatic transplantation of these islet cells, ongoing research at Semma is focused on combining these proprietary cells with a state-of-the-art cell device and immune protection strategy that can protect these cells from the patient's immune system and allow the beta cells to function as they do in non-diabetic individuals. Implantation of the islet cell-filled device has the potential to replace the missing beta cells in a diabetic patient without requiring patient immunosuppression. Semma is working to bring this new therapeutic option to the clinic and improve the lives of patients with

diabetes.

Semma recently announced the achievement of preclinical proof-of-concept for these two lead programs based on data in which they tested human stem cell-derived islets (SC-islets) in both non-human primates and pigs. These data were presented in a plenary session at the International Society for Stem Cell Research (ISSCR) in June 2019. To date, Semma's cell therapy approach is the only islet cell transplantation program to have demonstrated both positive c-peptide release (a marker of insulin secretion), as well as positive glycemic control of experimentally induced diabetes.

Transaction Terms

Under the terms of the acquisition, Vertex will acquire all outstanding shares of Semma for \$950 million in cash, and Semma will become a separate operating subsidiary of Vertex. Dr. Sanna will join Vertex as President of Semma. Also, Dr. Melton will continue in his role as Chair of Semma's Scientific Advisory Board and provide oversight and guidance on the research and development of the programs.

The companies anticipate the acquisition will close in the fourth quarter of 2019, subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and other customary conditions. Vertex will provide any appropriate updates to its current 2019 financial guidance in conjunction with its third quarter 2019 earnings release or upon closing the transaction, whichever occurs later.

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 three approved medicines that treat the underlying cause of cystic fibrosis (CF) - a rare, life-threatening genetic disease - and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including nine consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Vertex Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Leiden's statements in the second paragraph of the press release, Dr. Altschuler's statements in the third paragraph of the press release, Dr. Sanna's statements in the fourth paragraph of the press release, Dr. Melton's statements in the fifth paragraph of the press release, and statements regarding the timing of the potential closing of the transaction and the potential benefits of the acquisition. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent Vertex's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, the transaction is subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, Vertex may not realize the potential benefits of the acquisition, and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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