



Vertex and Obsidian Therapeutics Establish Collaboration to Discover Novel Therapies That Regulate Gene Editing

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- Multi-year strategic research collaboration leverages Obsidian's proprietary cytoDRiVE® platform to discover controllable genetic therapies to treat serious diseases

- Vertex will pay Obsidian up to \$75 million in equity, upfront payments and potential research milestones

BOSTON & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 22, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX) and Obsidian Therapeutics announced today that the two companies have entered into a strategic research collaboration and licensing agreement focused on the discovery of novel therapies that regulate gene editing for the treatment of serious diseases. The collaboration leverages Obsidian's cytoDRiVE® platform technology to discover gene-editing medicines whose therapeutic activity can be precisely controlled using small molecules and Vertex's established scientific and clinical capabilities in small molecule, cell and genetic therapies to more rapidly bring these approaches to patients.

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"This collaboration with Obsidian builds upon and will expand Vertex's leadership in small molecule and genetic therapies, and we're excited to partner with the team at Obsidian to explore the capabilities of their technology," said Vertex's David Altshuler, Executive Vice President, Global Research and Chief Scientific Officer. "The ability to tune gene-editing activity to a specific level is an important innovation that has the potential to address several serious diseases."

"At Obsidian, we're using our cytoDRiVE technology to pioneer a new generation of engineered cell and gene therapies for patients with serious diseases," said Paul Wotton, Chief Executive Officer of Obsidian Therapeutics. "Partnering with Vertex, an established leader at the forefront of genetic therapies, will further advance our technology into the gene therapy field and accelerate development of controllable gene editing therapies for patients."

About the Collaboration

Under the terms of the agreement, Obsidian will use its cytoDRiVE technology to develop novel regulated gene editing therapy candidates for multiple serious diseases. Obsidian grants Vertex the exclusive option to license worldwide rights to candidates discovered and developed under the collaboration. Following Vertex's exercise of its options, Vertex will be responsible for further preclinical and clinical development and commercialization.

Vertex will pay Obsidian up to \$75 million in upfront payments and research milestones that may be paid during the research term, including an equity investment in Obsidian. Obsidian is eligible to receive up to \$1.3 billion in potential payments based upon the successful achievement of specified research, development, regulatory, and commercial milestones across up to five potential programs. In addition, Vertex will pay tiered royalties on future net sales on any products that may result from this collaboration. Specific diseases that are the subject of this collaboration are not disclosed.

About Obsidian Therapeutics

Obsidian Therapeutics, Inc. is a biotechnology company pioneering engineered cell and gene therapies to deliver transformative outcomes for patients with intractable diseases. The Company expects to submit an IND in mid-2022 for its lead cytoTIL™ program, a novel engineered tumor infiltrating lymphocyte therapy armored with regulated membrane-bound IL15 that does not require patients to receive toxic IL2. Obsidian's proprietary cytoDRiVE® technology provides a way to control protein degradation using FDA-approved small molecules, permitting precise control of the timing and level of protein expression. Obsidian is headquartered in Cambridge, Mass. For more information, please visit www.obsidiantx.com.

About Vertex Pharmaceuticals

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple 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 small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy

and type 1 diabetes mellitus.

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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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.

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, statements by David Altshuler and Paul Wotton in this press release, statements about the potential benefits and results that may be achieved through Vertex's collaboration with Obsidian, including Obsidian's cytoDRIVE® platform technology, statements regarding upfront and milestone payments and potential royalties on future sales, and expectations about the ability to rapidly bring therapies to patients. 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 Obsidian 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.

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