# Vertex and Mammoth Biosciences Announce Collaboration to Develop In Vivo Gene-Editing Therapies for Serious Diseases

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*Vertex to use Mammoth's proprietary ultra-small CRISPR systems to discover and develop novel* in vivo *gene-editing therapies*

BRISBANE, Calif.--(BUSINESS WIRE)--Oct. 26, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Mammoth Biosciences, a biotech company building the next generation of CRISPR products to cure and detect disease, today announced a new partnership to develop *in vivo* gene-editing therapies for two genetic diseases using Mammoth's next-generation CRISPR systems.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20211026005322/en/

"We believe our novel ultra-small CRISPR systems have the potential to be game changers when it comes to systemic and targeted delivery of *in vivo* gene-editing therapies," said Peter Nell, Chief Business Officer and Head of Therapeutic Strategy at Mammoth Biosciences. "The combination of Mammoth's unique technology with Vertex's unmatched experience in serious disease research and development will only accelerate programs with the goal of reaching patients with high unmet medical need."

Driven by its unique protein discovery engine, Mammoth's CRISPR platform consists of a proprietary toolbox of novel, ultracompact Cas enzymes, including Cas14 and Casϕ. The small size of these Mammoth systems, together with further optimized parameters, have the potential to facilitate advanced delivery, which may increase the scope of *in vivo* gene-editing for genetic diseases.

"Vertex and Mammoth share the same commitment to developing therapies that have the potential to be transformative for people with serious diseases," said David Altshuler, M.D., Ph.D., Chief Scientific Officer of Vertex Pharmaceuticals. "We look forward to expanding our cell and genetic therapies capabilities with the addition of Mammoth's ultra-small CRISPR systems for *in vivo* genome editing, which will provide us with another set of tools to tackle many of the diseases we're interested in."

***About the Collaboration***

Under the terms of the agreement, Mammoth Biosciences will receive upfront payments of $41 million, including an investment in the form of a convertible note, and is eligible to receive up to $650 million in potential future payments based upon the successful achievement of prespecified research, development and commercial milestones across two potential programs. In addition, Vertex will pay tiered royalties on future net sales on any products that may result from this collaboration.

**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 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 cell and genetic 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.

**About Mammoth Biosciences**
Mammoth Biosciences is harnessing the diversity of life to power the next generation of CRISPR products. Through the discovery and engineering of novel CRISPR systems, the company is enabling the full potential of its platform to read and write the code of life. Mammoth aims to develop permanent genetic cures through best-in-class *in vivo* and *ex vivo* therapies and to democratize disease detection with on-demand diagnostics. By leveraging its internal research and development and exclusive licensing to Cas12, Cas13, Cas14 and Casϕ, Mammoth can provide enhanced diagnostics and genome editing for life science research,

healthcare, agriculture, biodefense and more. Based in the San Francisco Bay Area, Mammoth Biosciences is co-founded by CRISPR pioneer and Nobel Laureate Jennifer Doudna and Trevor Martin, Janice Chen and Lucas Harrington. The firm is backed by top institutional investors including Redmile Group, Foresite Capital, Senator Investment Group, Sixth Street, Decheng, Mayfield, NFX and 8VC, along with leading individual investors including Brook Byers, Tim Cook and Jeff Huber.

**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, M.D., Ph.D. and Peter Nell in this press release, statements about the potential benefits and results that may be achieved through Vertex's collaboration with Mammoth, statements regarding the future activities of the parties pursuant to the collaboration, statements regarding upfront and milestone payments, and potential royalties on future sales. 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 Mammoth may not be achieved, 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 most recent annual report and subsequent quarterly reports 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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