



Regeneron and Mammoth Biosciences Collaborate to Pursue Next-Generation CRISPR-Based Gene Editing for Multiple Diseases

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Mammoth's proprietary ultracompact CRISPR-based gene editing platform and Regeneron's proprietary delivery technologies set to advance *in vivo* programs in multiple tissue and cell types

Mammoth to receive \$100 million total upfront payment and equity investment from Regeneron at signing

TARRYTOWN, N.Y., April 25, 2024 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ:REGN) and Mammoth Biosciences, Inc., today announced a collaboration to research, develop and commercialize *in vivo* CRISPR-based gene editing therapies for multiple tissues and cell types. Regeneron is developing adeno-associated viral vectors (AAVs) using antibody-based targeting to enhance delivery of genetic medicine payloads to specific tissues and cell types. Mammoth is developing novel ultracompact nucleases and associated gene editing systems, with a variety of editing functionalities at a significantly smaller size than other CRISPR-based systems, including first generation Cas9 nucleases. By leveraging Regeneron's expertise in AAV and antibody engineering and Mammoth's expertise in ultracompact gene editing systems, the teams will endeavor to create disease-modifying medicines that can be delivered to tissues beyond the liver, to which most gene editing treatments are currently limited.

"We believe in the incredible power of gene editing, which we are utilizing in our diverse preclinical and clinical genetic medicines pipeline. After years spent developing our next-generation delivery approaches, we are eager to combine them with Mammoth's gene editing systems to better match payload, delivery system and disease type," said Christos Kyratsous, Ph.D., Senior Vice President and co-Head of Regeneron Genetic Medicines at Regeneron. "Together, we have the potential to overcome significant delivery hurdles and effectively reach tissues around the body, impact multiple diseases and dramatically increase the number of patients who could benefit from gene editing treatments."

"Mammoth brings over a decade of scientific expertise in CRISPR, beginning with our co-founders' work in the Doudna Lab at University of California Berkeley. We believe we can further our mission to transform the lives of patients by accelerating the discovery and development of genetic medicines in collaboration with Regeneron. Mammoth's ultracompact CRISPR systems address the size constraints of viral delivery and complement Regeneron's targeted AAV technologies. We look forward to working with Regeneron to enable all-in-one AAV delivery and unlock the true potential of *in vivo* gene editing," said Trevor Martin, Ph.D., co-founder and Chief Executive Officer of Mammoth Biosciences.

"This exciting collaboration pairs two teams with a shared commitment to translating high science into ground-breaking *in vivo* genetic medicines that can potentially address the needs of more patients and more prevalent conditions," said Jennifer Doudna, Ph.D., co-founder and Chair of the Scientific Advisory Board at Mammoth Biosciences, CRISPR genome editing co-inventor and winner of the 2020 Nobel Prize in Chemistry.

Under the terms of the agreement, Mammoth will receive \$100 million inclusive of \$95 million in equity investment at signing, and an upfront payment, and is eligible to receive up to \$370 million per target in development, regulatory and commercial milestone payments, and royalty rates ranging from single digits to mid-teens on future net sales of all collaboration products. In addition, Mammoth has the right to opt-in to co-funding and sharing profits on a majority of collaboration programs in lieu of receiving milestones and royalties. In exchange, for a period of five and a half years, Regeneron is obtaining broad access to Mammoth's editing technologies, other than certain excluded targets, with the option to extend such access for an additional two years upon the payment of a research extension fee. The parties will jointly select and research collaboration targets, and then Regeneron will lead development and commercialization.

About Regeneron

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents, develops and commercializes life-transforming medicines for people with serious diseases. Founded and led by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to numerous approved treatments and product candidates in development, most of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, neurological diseases, hematologic conditions, infectious diseases and rare diseases.

Regeneron pushes the boundaries of scientific discovery and accelerates drug development using our proprietary technologies, including *VelociSuite*[®] which produces optimized fully human antibodies and new classes of bispecific antibodies. We are shaping the next frontier of medicine with data-powered insights from the Regeneron Genetics Center[®] and pioneering genetic medicine platforms, enabling us to identify innovative targets and complementary approaches to potentially treat or cure diseases.

For more information, please visit www.Regeneron.com or follow Regeneron on [LinkedIn](#), [Instagram](#), [Facebook](#) or [X](#).

About Mammoth Biosciences

Mammoth Biosciences is a biotechnology company focused on leveraging its proprietary ultracompact CRISPR systems to develop potential long-term curative therapies for patients with life-threatening and debilitating diseases. Founded by CRISPR pioneer and Nobel laureate Jennifer Doudna and Trevor Martin, Janice Chen, and Lucas Harrington, the company's ultracompact systems are designed to be more specific and enable *in vivo* gene editing in difficult to reach tissues utilizing both nuclease applications and new editing modalities beyond double stranded breaks, including base editing, reverse transcriptase editing, and epigenetic editing. The company is building out its wholly owned pipeline of potential *in vivo* gene editing therapeutics and capabilities and has partnerships with leading pharmaceutical and biotechnology companies to broaden the reach of its innovative and proprietary technology platform. Mammoth's deep science and industry experience, along with a robust and differentiated intellectual property portfolio, have enabled the company to further its mission to transform the lives of patients and deliver on the promise of CRISPR technologies.

For more information, please visit www.mammoth.bio or follow Mammoth on [LinkedIn](#) or [X](#).

Regeneron Forward-Looking Statements

This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. (“Regeneron” or the “Company”), and actual events or results may differ materially from these forward-looking statements. Words such as “anticipate,” “expect,” “intend,” “plan,” “believe,” “seek,” “estimate,” variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of products marketed or otherwise commercialized by Regeneron and/or its collaborators or licensees (collectively, “Regeneron’s Products”) and product candidates being developed by Regeneron and/or its collaborators or licensees (collectively, “Regeneron’s Product Candidates”) and research and clinical programs now underway or planned, such as the planned research programs with Mammoth Biosciences, Inc. to develop in vivo CRISPR-based gene editing therapies for multiple extrahepatic tissues and cell types as discussed in this press release; the potential for any license, collaboration, or supply agreement, including Regeneron’s agreements with Sanofi and Bayer (or their respective affiliated companies, as applicable), as well as Regeneron’s collaboration with Mammoth Biosciences discussed in this press release, to be cancelled or terminated; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees (including those to be conducted as part of the collaboration with Mammoth Biosciences discussed in this press release) may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; the potential of utilizing for therapeutic purposes Regeneron’s expertise in adeno-associated viral vectors and antibody engineering and Mammoth Biosciences’ expertise in ultracompact gene editing systems as discussed in this press release; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron’s Product Candidates and new indications for Regeneron’s Products; uncertainty of the utilization, market acceptance, and commercial success of Regeneron’s Products and Regeneron’s Product Candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary) on any of the foregoing; the ability of Regeneron’s collaborators, licensees, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron’s Products and Regeneron’s Product Candidates; the ability of Regeneron to manage supply chains for multiple products and product candidates; safety issues resulting from the administration of Regeneron’s Products and Regeneron’s Product Candidates in patients, including serious complications or side effects in connection with the use of Regeneron’s Products and Regeneron’s Product Candidates in clinical trials; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron’s ability to continue to develop or commercialize Regeneron’s Products and Regeneron’s Product Candidates; ongoing regulatory obligations and oversight impacting Regeneron’s Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement of Regeneron’s Products from third-party payers, including private payer healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payers and new policies and procedures adopted by such payers; competing drugs and product candidates that may be superior to, or more cost effective than, Regeneron’s Products and Regeneron’s Product Candidates; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; the impact of public health outbreaks, epidemics, or pandemics (such as the COVID-19 pandemic) on Regeneron’s business; and risks associated with intellectual property of other parties and pending or future litigation relating thereto (including without limitation the patent litigation and other related proceedings relating to EYLEA® (afibercept) Injection), other litigation and other proceedings and government investigations relating to the Company and/or its operations, the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron’s business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron’s filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2023. Any forward-looking statements are made based on management’s current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update (publicly or otherwise) any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron’s media and investor relations website (<https://investor.regeneron.com>) and its LinkedIn page (<https://www.linkedin.com/company/regeneron-pharmaceuticals>).

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