



2seventy bio Announces Expanded Collaboration Agreement With Novo Nordisk to Continue Development of *in vivo* Gene Editing Approach

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Collaboration further validates 2seventy bio's megaTAL™ gene editing platform with potential applicability across company's oncology pipeline

2seventy bio is eligible to receive \$40M in near-term payments, in addition to future milestone payments and sales royalties

2seventy bio and Genevant Sciences announce collaboration for lipid nanoparticle delivery of megaTAL mRNA

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 6, 2022-- [2seventy bio, Inc.](https://www.2seventybio.com) (NASDAQ:TSVT) today announced that it has entered into an option and license agreement with Novo Nordisk for joint research and development of an *in vivo* gene editing treatment for hemophilia A. This agreement builds upon a successful existing multi-year research collaboration between the two companies. Under the terms of this agreement, Novo Nordisk will have the option to license 2seventy bio's proprietary mRNA-based megaTAL™ technology for the development of a new treatment approach for hemophilia A patients with the goal of offering a sustained therapeutic effect.

"This collaboration aims to develop the first direct *in vivo* application of our megaTAL technology. We are excited to explore this technology with Novo Nordisk, with the goal of developing a new potential therapeutic approach for patients with hemophilia A. Moreover, we view this work as further validation and a natural extension of our technology platform. Based on what we continue to learn, this technology may play a potential role in expanding our gene editing platform toward future *in vivo* and *ex vivo* applications that can enhance our oncology pipeline," said Philip Gregory, D. Phil., chief scientific officer, 2seventy bio. "We are also excited to announce a partnership between 2seventy bio and Genevant Sciences for the use of Genevant's lipid nanoparticle (LNP) platform in our collaboration with Novo Nordisk."

The collaboration agreement with Novo Nordisk builds upon the original research collaboration signed between bluebird bio and Novo Nordisk in 2019, focused on identifying a development gene therapy candidate for people with hemophilia A. The collaboration utilizes 2seventy bio's megaTAL technology that has the potential to provide a highly specific and efficient way to silence, edit, or insert genetic components. Hemophilia A is a genetic bleeding disorder resulting from defective Factor VIII.

"We are excited to continue our partnership with 2seventy bio to jointly develop a next-generation *in vivo* genome editing treatment, with the ultimate ambition of offering people with hemophilia A lifetime free of factor replacement therapy," said Karin Conde-Knape, senior vice president, Global Drug Discovery, Novo Nordisk. "This partnership reflects Novo Nordisk's commitment to utilizing novel technology platforms to advance truly disease-modifying therapies for people with serious chronic diseases."

Under the terms of the agreement, Novo Nordisk will obtain the option to exclusively license 2seventy bio's *in vivo* mRNA platform and gene editing technology for use in the treatment of patients with hemophilia A. 2seventy bio will receive an upfront payment of \$5 million and is eligible for near-term milestone and option exercise payments of up to \$35 million, in addition to development, regulatory, and commercial milestones, as well as a royalty on net sales. Novo Nordisk will be responsible for funding all research and development activities.

Related to this collaboration, 2seventy bio has also entered into an agreement with Genevant Sciences for access to Genevant's industry-leading LNP technology platform for use in 2seventy bio's collaboration with Novo Nordisk for the treatment of patients with hemophilia A. 2seventy bio plans to use the Genevant LNP platform for efficient delivery of megaTAL mRNA to hepatocyte cells within the liver.

"We are very pleased that 2seventy bio has entrusted Genevant and our LNP platform with delivery for its important gene editing program in hemophilia A," said Pete Lutwyche, Ph.D., president and chief executive officer, Genevant Sciences Corporation. "Our scientists have been at the forefront of the LNP field for more than 20 years, and we are excited for our innovative technology to be used for this important application with great unmet need."

Under the terms of the agreement between 2seventy bio and Genevant, 2seventy bio obtained rights to license Genevant's LNP technology for use with megaTAL mRNA products in the treatment of patients with hemophilia A. Genevant is eligible for upfront and near-term option exercise payments totaling \$10 million, as well as development and commercialization milestones, and royalties in the mid-single digits on future product sales.

About megaTALS

MegaTALS are single-chain enzymes that combine the natural DNA recognition and cleavage processes of Homing Endonucleases (HEs) with the modular DNA binding properties of transcription activator-like (TAL) effectors. This protein fusion architecture allows the generation of highly specific and active nucleases in a compact format compatible with all current viral and non-viral cell delivery methods.

About Novo Nordisk A/S

Novo Nordisk is a leading global healthcare company, founded in 1923 and headquartered in Denmark. Our purpose is to drive change to defeat diabetes and other serious chronic diseases such as obesity and rare blood and endocrine disorders. We do so by pioneering scientific breakthroughs, expanding access to our medicines, and working to prevent and ultimately cure disease. Novo Nordisk employs about 47,000 people in 80 countries and markets its products in around 170 countries. For more information, visit [novonordisk.com](https://www.novonordisk.com), [Facebook](https://www.facebook.com/novonordisk), [Twitter](https://twitter.com/novonordisk), [LinkedIn](https://www.linkedin.com/company/novonordisk) and [YouTube](https://www.youtube.com/novonordisk).

About Genevant Sciences

Genevant Sciences is a leading nucleic acid delivery company with world-class platforms, the industry's most robust and expansive lipid nanoparticle (LNP) patent estate, and decades of experience and expertise in nucleic acid drug delivery and development. The Company's scientists have pioneered LNP delivery of nucleic acids for over 20 years, and the Company's LNP platform, which has been studied across more than a dozen discrete product candidates and is the delivery technology behind the first and only approved RNAi-LNP (patisiran), enables a wide array of RNA-based applications, including vaccines, therapeutic protein production, and gene editing. For more information, please visit www.genevant.com.

About 2seventy bio

Our name, 2seventy bio, reflects why we do what we do - TIME. Cancer rips time away and our goal is to work at the maximum speed of translating human thought into action – 270 miles per hour — to give the people we serve more time. We are building the leading immuno-oncology cell therapy company, focused on discovering new therapies and leveraging platform technologies that truly disrupt the cancer treatment landscape.

With a deep understanding of the human body's immune response to tumor cells and how to translate cell therapies into practice, we're applying this knowledge to deliver next generation cellular therapies that focus on a broad range of hematologic malignancies, including the first FDA-approved CAR T cell therapy for multiple myeloma, as well as solid tumors. Our research and development is focused on delivering therapies that are designed with the goal to "think" smarter and faster than the disease. Importantly, we remain focused on accomplishing these goals by staying genuine and authentic to our "why" and keeping our people and culture top of mind every day.

For more information, visit www.2seventybio.com.

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2seventy bio Cautionary Statement Regarding Forward-Looking Statements

This press release contains "forward-looking statements". All statements that are not statements of historical facts are, or may be deemed to be, forward-looking statements, including statements regarding: plans for the expansion of our oncology pipeline and the development, regulatory approval, manufacture or sale of our current or future product candidates; expectations regarding use of our megaTAL technology for the treatment of patients with hemophilia A and other genetic conditions; expectations regarding use of Genevant's LNP technology for the treatment of patients with hemophilia A and other genetic conditions; and expectations regarding the benefits we may receive from our agreements with Novo Nordisk and Genevant. Such forward-looking statements are subject to risks, assumptions and uncertainties that could cause actual results to differ materially from those express or implied by such statements, including: the risk that our megaTAL technology is not effective in addressing hemophilia A and other genetic diseases; the risk that Genevant's LNP technology will not be able to provide safe and efficient delivery of megaTAL mRNA to hepatocyte cells within the liver; the risk that we will not realize anticipated benefits from our agreements with Novo Nordisk and Genevant; and other internal or external factors that could delay, divert or change any of them in the next several years, that are difficult to predict, may be beyond our control and could cause our future financial results, goals, plans and objectives to differ materially from those expressed in, or implied by, the statements. No forward-looking statement can be guaranteed. We caution investors not to place considerable reliance on forward-looking statements contained in this press release. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Quarterly Report filed by 2seventy bio with the Securities and Exchange Commission on December 1, 2021, as well as discussions of potential risks and uncertainties in 2seventy bio's subsequent filings with the Securities and Exchange Commission. The forward-looking statements included in this document are made only as of the date of this document and except as otherwise required by applicable law, 2seventy bio undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events, changed circumstances or otherwise.

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