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Vertex expands into new disease areas and enhances gene editing capabilities

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Vertex will acquire privately held Exonics, a company focused on creating transformative gene editing therapies to repair mutations that cause DMD and other severe neuromuscular diseases



Vertex Pharmaceuticals Incorporated has announced that the company is enhancing its gene editing capabilities to develop novel therapies for Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1) by expanding its collaboration with CRISPR Therapeutics and acquiring Exonics Therapeutics.

Vertex and CRISPR Therapeutics (CRSP) have expanded their collaboration and entered into an exclusive licensing agreement to discover and develop gene editing therapies for the treatment of DMD and DM1.

Vertex and Exonics Therapeutics have entered into a definitive agreement under which Vertex will acquire privately held Exonics, a company focused on creating transformative gene editing therapies to repair mutations that cause DMD and other severe neuromuscular diseases.

“Through the expanded collaboration with CRISPR and the acquisition of Exonics, we are bringing together the intellectual property, technologies, and scientific expertise needed to establish a leading gene editing platform for DMD and DM1. These transactions are highly aligned with our strategy of investing in scientific innovation to create transformative medicines for people with serious diseases,” said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. “We are continuing to build a toolbox of small molecule and nucleic acid technologies and capabilities that will allow us to drive scientific innovation to produce transformative medicines for a broad portfolio of diseases.”

“This agreement with Vertex reflects the strong collaboration we have built together in other programs and underscores Vertex’s commitment to gene editing,” said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. “We continue to make significant advancements in enabling in vivo approaches for gene editing and are excited about the possibility of developing potentially curative therapies for DMD and DM1 together with Vertex.”

“DMD and DM1 are devastating muscle diseases with no curative therapies available,” said Eric Olson, Ph.D., Founder and Chief Science Advisor of Exonics, and Professor and Chair of the Department of Molecular Biology at UT Southwestern Medical Center. “Vertex has a proven track record of developing important therapies for serious diseases and we are excited to combine our efforts to potentially develop a safe and efficacious one-time treatment for severe neuromuscular diseases.”