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MEDIA UPDATE

Novartis builds on neuroscience pipeline and xRNA platform capabilities with acquisition of DTx Pharma

- *DTx Pharma is a preclinical stage biotechnology company that focuses on developing siRNA therapies for neuroscience indications, leveraging its proprietary fatty acid ligand-conjugated oligonucleotide (FALCON) platform*

- *Deal includes DTx-1252, a potential therapy for the neuromuscular disorder Charcot-Marie-Tooth disease type 1A (CMT1A), and two additional preclinical programs for other neuroscience indications*

- *Acquisition expands Novartis capabilities in RNA-based therapeutics, adding DTx's FALCON platform to the Novartis siRNA toolkit*

Basel, July 17, 2023 — Novartis announced today that it has acquired DTx Pharma, a San-Diego based, preclinical stage biotechnology company focused on leveraging its proprietary FALCON platform to develop siRNA therapies for neuroscience indications.

DTx's lead program, DTx-1252 targets the root cause of CMT1A—the overexpression of PMP22, a protein that causes the myelin sheath that supports and insulates nerves in the peripheral nervous system to function abnormally. DTx-1252, which was recently granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA),[1] decreases the expression of this protein in Schwann cells, the target cell type for the development, maintenance, and function of peripheral nerves. The FALCON platform facilitates this targeted approach by conjugating siRNAs to naturally occurring fatty acids to improve the biodistribution and cellular uptake to tissues and cell types of interest.[2]

"We look forward to continuing the development of DTx's therapeutic programs and bringing new hope to patients with neuromuscular and other neurological disorders for which there have historically been few treatment options," said Fiona Marshall, President of the Novartis Institutes for BioMedical Research (NIBR). "We are also excited to bring DTx's FALCON technology to Novartis and explore its potential to deliver drugs to extrahepatic tissues."

Charcot-Marie Tooth disease (CMT) is a group of inherited disorders affecting the nervous system. CMT1A is the most prevalent subtype of CMT, impacting up to approximately 150,000 patients in the U.S. and Europe.[3] A slow-progressing, degenerative disease of the peripheral nervous system, the disease causes progressive muscle wasting, neuropathic pain, and difficulty walking, and can have a significant impact on quality of life. DTx-1252 could potentially be a first-in-class treatment for the disease, as there are currently no approved treatments to address the underlying genetic cause of CMT1A.[4]

In addition to DTx-1252, the deal also brings two additional preclinical programs for other neuroscience indications to Novartis and expands the Novartis siRNA toolkit, building on its capabilities in xRNA, one of the Novartis technology platforms. The agreement underscores the Novartis commitment to Neuroscience, one of the company's five core therapeutic areas, and to pursuing innovative therapies for patients with neuromuscular disorders.

"The growing arsenal of therapeutic platforms available to us continues to enable the development of novel, high-value medicines—particularly in cases where the underlying biology of disease is well defined," said Robert Baloh, Global Head of Neuroscience for NIBR. "We look forward to continuing the development of DTx's potential first-in-class medicine for CMT1A and are hopeful we can bring a therapeutic option to patients living with this debilitating neuromuscular condition."

Under the terms of the agreement, Novartis will make an upfront payment of USD 500m and additional payments upon completion of pre-specified milestones.

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