Pathway Development Consortium Announces Publication in Human Gene Therapy on the Application of FDA's Accelerated Approval Pathway for AAV Gene Therapies for Patients with Duchenne Muscular Dystrophy

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- Publication in Human Gene Therapy provides rationale for the use of microdystrophin expression levels as a surrogate endpoint reasonably likely to predict clinical benefit for AAV gene therapy intended for treatment of patients with Duchenne muscular dystrophy -

- White paper submitted to the U.S. Food and Drug Administration by the Pathway Development Consortium supports the use of the accelerated approval pathway for AAV gene therapies intended to treat patients with Duchenne muscular dystrophy -

- Expands on Pathway Development Consortium's previously released white paper that provided a framework for applying the accelerated approval pathway to AAV gene therapy development -

- The work of the Pathway Development Consortium is a result of collaboration across a broad set of stakeholders, including patients, industry, academia, and clinicians -

WASHINGTON, Jan. 26, 2023 (GLOBE NEWSWIRE) -- The Pathway Development Consortium (PDC), a public-private collaboration founded by REGENXBIO Inc. (Nasdaq: RGNX) and Solid Biosciences Inc. (Nasdaq: SLDB), today announced the publication of a peer-reviewed manuscript, Micro-dystrophin expression as a surrogate endpoint for Duchenne muscular dystrophy clinical trials, in *Human Gene Therapy.*

This publication proposes microdystrophin expression levels as a surrogate endpoint reasonably likely to predict clinical benefit. The use of surrogate endpoints reasonably likely to predict clinical benefit could expedite access to therapies for serious diseases that have demonstrated a meaningful advantage over available therapy. Improvements in endpoints that are reasonably likely to provide patients clinical benefit allows patients access while studies are ongoing to verify and describe the predicted clinical benefit to patients under the U.S. Food and Drug Administration (FDA) accelerated approval pathway.

An extended version of the manuscript is available as a white paper on the PDC website and has been submitted to the FDA. This white paper clarifies the rationale for use of the accelerated approval pathway to advance AAV gene therapy development for patients with Duchenne muscular dystrophy and provides support for two surrogate endpoints reasonably likely to predict clinical benefit—muscle fat fraction (FF) obtained by magnetic resonance (MR) methods and microdystrophin expression levels.

"Multistakeholder collaborative efforts that bring together expertise from all backgrounds are critical to bringing new therapeutic options to people with Duchenne," said Pat Furlong, Founding President and CEO of Parent Project Muscular Dystrophy (PPMD). "The PDC's white paper on the use of the accelerated approval pathway for AAV gene therapies complements our recent work to update the Community-led Guidance for Dystrophinopathies to advance the development of potential therapies."

This white paper expands on the PDC's draft framework that outlined an approach for the use of FDA's accelerated approval pathway for different categories of AAV gene therapies that target the underlying monogenic changes that cause disease.

"The manuscript and white paper are important steps in providing the scientific rationale that enables use of the accelerated approval pathway to get new treatment options to patients with unmet medical needs," said Jeff Chamberlain, Ph.D., Professor in the Departments of Neurology, Medicine, and Biochemistry, the McCaw Endowed Chair in Muscular Dystrophy at the University of Washington School of Medicine, and Director of the Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center of Seattle. "The rare disease community needs to work collaboratively to fill treatment gaps and starting with the science is key to development of novel therapies," said Dongsheng Duan, Ph.D., Curators' Professor and Margaret Proctor Mulligan Professor in Medical Research at the University of Missouri's School of Medicine with a joint appointment in biomedical sciences at the College of Veterinary Medicine.

About Pathway Development Consortium
The Pathway Development Consortium (PDC) aims to guide the recent decades of AAV gene therapy research into a future of innovative, potentially life-saving therapies. The PDC's goal is to foster collaboration and partnership among patients, industry, regulators, academia, payers and other stakeholders. For this reason, REGENXBIO and Solid Biosciences joined together to launch the PDC with the vision to construct an ideal pathway to ensure that all born with serious genetic conditions can find their way to effective AAV gene therapies. To learn more, visit https://www.pathwaydevelopmentconsortium.org/.

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