Pathway Development Consortium Releases Draft White Paper to Facilitate AAV Gene Therapy Development

- Draft white paper published today by the Pathway Development Consortium provides a framework for applying the accelerated approval pathway to AAV gene therapy development -

- Pathway Development Consortium is building a collaboration of patients, industry, regulators, academia, payers and other stakeholders -

Washington, DC, November 2, 2021 – The Pathway Development Consortium (PDC), a new public-private collaboration founded by REGENXBIO Inc. (Nasdaq: RGNX) and Solid Biosciences Inc. (Nasdaq: SLDB), today announced the publication of a draft white paper, Draft Framework for AAV Gene Therapy Development, which proposes a framework that can be applied to AAV gene therapies to facilitate the use of the accelerated approval pathway at the US Food and Drug Administration (FDA). The white paper identifies different categories of AAV gene therapies that target the underlying monogenetic changes that cause disease and proposes generalized approaches that would clarify the evidence needed to support FDA approval. The PDC is actively seeking feedback from key stakeholders on this framework at info@pathwaydevelopmentconsortium.org.

The publication of the white paper coincides with the formal launch of the PDC, a multistakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.

“The Agency recognizes the importance of broad stakeholder engagement to facilitate and expedite the development of AAV gene therapies for rare diseases,” said Peter Marks, MD, PhD, Director of the FDA’s Center for Biologics Evaluation and Research (CBER). “FDA looks forward to hearing more about this consortium’s efforts to help bring promising gene therapies to patients in need.”

“The Pathway Development Consortium is embarking on critical work aiming to expeditiously advance AAV gene therapies for patients living with rare diseases,” said Rachel Sherman, MD, MPH, President at Rachel Sherman Partners and former Principal Deputy Commissioner at the FDA. “After a long history of building public-private collaborations throughout my career at FDA, I have seen firsthand the impact that can be made when all stakeholders come together to create groundbreaking solutions. This draft framework proposed by the PDC is an innovative approach to facilitate the development and regulatory approval of important AAV gene therapies.”

“The Pathway Development Consortium has the unique potential to bring together the diverse perspectives in the rare disease community with the interest of the patient at the forefront,” said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO and co-founder of the PDC, in partnership with Ilan Ganot, Co-Founder, President and Chief Executive Officer of Solid Biosciences. “We founded the PDC together because we share the vision that this collaboration can meaningfully guide how AAV-based gene therapy treatments can be more rapidly made available to patients. The ability to regularly dialogue with the FDA is critical and we are grateful for their support of this endeavor,” said Ganot.

The PDC’s activities kicked off with a roundtable discussion held in April 2021 to focus on Duchenne muscular dystrophy (Duchenne), a progressive muscle-wasting genetic disease that primarily affects boys. Children with Duchenne are typically diagnosed between the ages of 3 and 5 years old, lose the ability to walk by their early-teens and have a life expectancy of approximately 25 years. The roundtable focused on finding a path forward for meaningful clinical endpoints in clinical trials and brought together more than 120 attendees from the Duchenne patient community, industry, academia and the FDA. In September 2021, the PDC’s first white paper was published, identifying areas where attention is needed to facilitate development of AAV gene therapies for Duchenne.

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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