**AMP BGTC Announces FDA Clearance of Investigational New Drug Application for Gene Therapy Targeting Multiple Sulfatase Deficiency**

NORTH BETHESDA, MD — The Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC) announces that the U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for an investigational gene therapy designed to treat children with multiple sulfatase deficiency (MSD**),** an ultra-rare disease. MSD is a lysosomal storage disorder caused by a mutation in the SUMF1 gene. It has devasting, progressive symptoms that affect growth and development of children. MSD is a life-limiting condition with an average life expectancy of 13 years. Current treatment options focus on managing symptoms and improving the child's quality of life. The planned first-in-human study will evaluate the safety and tolerability of the investigational gene therapy while exploring its potential therapeutic effects.

The MSD IND application is sponsored by the National Center for Advancing Translational Sciences (NCATS), a part of the National Institutes of Health (NIH). NCATS is one of several NIH Institutes and Centers that participate in AMP BGTC, a public-private partnership between the NIH, the U.S. Food and Drug Administration, biopharmaceutical and life science companies, and nonprofit organizations. AMP BGTC is managed by the Foundation for the National Institutes of Health and has the overall goal of helping to speed the development and delivery of customized or “bespoke” gene therapies that could treat the millions of people affected by rare diseases.

Although the MSD program entered the BGTC after substantial preclinical development had been completed, the consortium enabled progress to the clinic through both manufacturing and clinical development activities. BGTC supported multiple critical manufacturing efforts, including the successful manufacturing of the clinical grade product that will be used in the trial.

The consortium also facilitated the collaborative development of the clinical protocol and IND application through input from BGTC manufacturing, regulatory and clinical subject matter experts, as well as a protocol review committee including patient advocates and parents of a child living with MSD. This coordination ensured the study design reflected both scientific priorities and the needs of the MSD community.

This achievement reflects years of scientific research and invaluable BGTC partnership of patients, families, clinicians, researchers, consortium partners and advocacy organizations dedicated to advancing new solutions for the rare disease community. The effort was driven by the foundational work of Steven Gray, PhD, and Rachel Bailey, PhD, of the University of Texas Southwestern Medical Center who developed the gene therapy; Cathleen Lutz, PhD, and Maximiliano Presa, PhD, of the Jackson Laboratories who conducted the pivotal preclinical testing of the gene therapy, the leadership of principal investigators Laura Adang, MD, PhD, and Rebecca Ahrens-Nicklas, MD, PhD of the Children’s Hospital of Philadelphia; and the unwavering support of patient advocate Sarah Cortell Vandersypen of United MSD Foundation, and the MSD community.

As the program advances, the BGTC remains committed to working alongside the broader scientific and patient communities to develop transformative therapies and improve outcomes for those affected by rare genetic diseases. To learn more about this milestone, the FNIH NCATS and CHOP have shared the following resources:

- BGTC webpage
- Family/Community Letter
- CHOP MSD Page: Multiple Sulfatase Deficiency (MSD) | Children's Hospital of Philadelphia
- Regulatory Playbook
- CHOP partnered educational videos
- United MSD Foundation Families Page