ACCELERATING MEDICINES PARTNERSHIP®
BESPOKE GENE THERAPY CONSORTIUM

To: Members of the Multiple Sulfatase Deficiency (MSD) Patient Community

From: The AMP® Bespoke Gene Therapy Consortium (BGTC)

BGTC is committed to sharing accurate and timely information with the MSD patient community as we continue to work towards initiation of a first in human clinical trial. We know that many of you have been following progress of the BGTC Clinical Trial Portfolio since the announcement in May 2023 of funding for development of a gene therapy for MSD being awarded to a research team from the Children's Hospital of Philadelphia. Today, we announced that FDA has cleared the Investigational New Drug (IND) Application for AAV9/SUMF1, a gene therapy for MSD, that was submitted by the National Center for Advancing Translational Sciences (NCATS).

As the IND sponsor, NCATS is now leading efforts to prepare for dosing of participants in the first in human clinical trial for the AAV9/SUMF1 gene therapy. NCATS does not yet have all of the final details on key trial information such as: When will the clinical trial start? and How do I express interest in enrolling my child in the clinical trial? Over the next months, NCATS will be working closely with the principal investigators (Dr. Laura Adang and Dr. Rebecca Ahrens-Nicklas of the Children's Hospital of Philadelphia) to finalize the clinical trial protocol that was submitted to FDA for review and to develop the materials and processes that will be needed to inform the MSD community about the AAV9/SUMF1 first in human clinical trial.

NCATS has engaged the Duke Clinical Research Institute (DCRI) to serve as a Clinical Data Coordinating Center for the AAV9/SUMF1 first in human clinical trial and is working closely with DCRI to complete all of the steps that will be required to safely dose participants and collect clinical trial data in a way that will be acceptable to the FDA.

As we continue on our journey to prepare for the start of the AAV9/SUMF1 clinical trial, NCATS (the IND sponsor) and the principal investigators (Dr. Adang and Dr. Ahrens-Nicklas) will be taking the lead on communicating with the MSD patient community about the AAV9/SUMF1 first in human clinical trial. BGTC will continue to provide a Patient Advocacy Newsletter and will also be developing educational videos describing the additional work that must be done to safely and compliantly start a clinical trial after an IND clears FDA review.

For now, you can obtain more information about the AAV9/SUMF1 first in human clinical trial by sending your questions to: CIGT@chop.edu. You can follow BGTC activities here and subscribe to the BGTC Patient Advocacy Newsletter here.

The first in human clinical trial protocol for AAV9/SUMF1 has been developed with significant input from MSD parents and patient advocates. We thank them for their expertise and honest feedback. We cannot complete our journey without the continued support of the MSD Patient Community. We look forward to providing you with updates and more information in the near future.