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Muscular Dystrophy Association to host first Patient Focused Drug Development Meeting for Pompe Disease March 9, 2020 in Washington D.C.

Panelists will be attending live and via webcast, including live polling, to ensure a broad range of experiences and lifestyles are shared with FDA, Biopharmaceutical companies and key stakeholders.

NEW YORK, NY, Nov. 21, 2019 – The Muscular Dystrophy Association (MDA) announced today that the U.S. Food and Drug Administration (FDA) has approved its externally-led Patient Focused Drug Development (PFDD) meeting on Pompe disease on March 9, 2020 in Hyattsville, Maryland. MDA is partnering with the Acid Maltase Deficiency Association (AMDA) and United Pompe Foundation (UPF) to bring the Pompe community to Washington, D.C. in person and online. MDA encourages all members of the community to register for this meeting in person or online [here](#).

“We’re honored to bring this patient community together in collaboration with AMDA and UPF, and to have leaders from the FDA and physician community join us to discuss this serious neuromuscular disease, said Kristin Stephenson, MDA’s EVP, Chief Advocacy and Care Services Officer. “This meeting is part of MDA’s mission to raise the voice of the patient community in order to help accelerate the further development of treatments and cures for people living with over 43 neuromuscular diseases.”

The PFDD meeting is an opportunity for patients and families living with Pompe disease to share their perspective and inform the FDA and other key stakeholders about their experiences living with Pompe disease and how they view the potential benefits and risks of treatments for Pompe. This input will help inform FDA’s regulatory decision making and oversight during therapy development and in the regulatory review process.

At the meeting, patients living with Pompe disease and their family members and caregivers will have the opportunity to share their insights and experiences living with Pompe disease via moderated panel discussions and audience participation. The discussions will address symptoms and impact of Pompe disease on daily life, current experiences with treatment, and perspectives on and expectations for potential future treatments. Live polling will be also conducted to capture patient perspectives on benefits, risks, and preferences for treatment.

Following the meeting, MDA will publish a Voice of the Patient report that articulates the proceedings and main takeaways from the meeting for use by the patient, medical professional, biopharmaceutical, and regulatory stakeholder communities. This report will provide lasting lessons on life with Pompe disease and the community's viewpoints on potential therapeutic interventions.

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About the Muscular Dystrophy Association

MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS and related neuromuscular diseases. We do this through innovations in science and innovations in care. As the largest source of funding for neuromuscular disease research outside of the federal government, MDA has committed more than \$1 billion since our inception to accelerate the discovery of therapies and cures. Research we have supported is directly linked to life-changing therapies across multiple neuromuscular diseases. MDA's Neuromuscular ObserVational Research (MOVR) data hub gathers longitudinal clinical data for multiple neuromuscular diseases to improve health outcomes and accelerate therapy development. MDA supports the largest network of multidisciplinary clinics providing best in class care at more than 150 of the nation's top medical institutions, and our national resource center serves the community with one-on-one specialized support and we offer educational conferences, events, and materials for families and healthcare providers. Each year thousands of children and young adults learn vital life skills and gain independence at summer camp and through recreational programs, at no cost to families. For more information visit mda.org.

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