PUBLIC STATEMENT REGARDING GALGT2 PROJECT FUNDING:

Six grassroots disease foundations founded by parents of boys diagnosed with Duchenne muscular dystrophy: Team Joseph (Michigan); Little Hercules Foundation (Ohio); Walking Strong (California); Charley's Fund (Massachusetts); JB's Keys (Massachusetts); and Hope for Gus (New Hampshire), are excited to announce their collaboration to provide a $284,620 production grant for a surrogate gene therapy delivering the *GALGT2* gene to potentially provide significant clinical benefit to boys affected by DMD. This study will be conducted at Nationwide Children's Hospital in Columbus, Ohio.

Kevin M. Flanigan, MD, is an attending neurologist at Nationwide Children's and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. Dr. Flanigan is a principal investigator in the Center for Gene Therapy in The Research Institute at Nationwide Children's and a leading expert in neuromuscular disorders. This grant supports the development of a surrogate gene therapy for DMD that uses a viral gene transfer vector to deliver the *GALGT2* gene, which encodes GalNAc transferase, an approach developed by Dr. Paul Martin of the Center for Gene Therapy, a leading expert in glycosylation. The protein GalNAc transferase is normally expressed only at the neuromuscular synapse and at the myotendinous junction where it glycosylates dystroglycan. In animal models, delivery of *GALGT2* results in a change to glycosylation across the entire muscle membrane, with upregulation of utrophin and other proteins that stabilize the muscle membrane, resulting in correction of muscle pathology and force deficits. This is a particularly promising approach because it is a potential therapy for any boy with DMD, regardless of mutation.

Our extensive, pre-clinical data supports our theory that GalGT2 has great potential to ameliorate the devastating effects of Duchenne," says Dr. Flanigan. "We are excited to move this therapy forward into a clinical setting for DMD patients and financial support from Duchenne organizations allow us to continue to work on developing treatments for DMD."

"We are thrilled to support Dr. Flanigan's efforts to move this promising therapy forward for our sons and all patients battling Duchenne," says Marissa Penrod, President and Founder of Team Joseph. Kelly Maynard, Founder and President of Little Hercules Foundation states, "collaboration is crucial to efficiently identifying and funding treatments for DMD where none currently exist."

About Duchenne muscular dystrophy: Duchenne is a progressive neuromuscular disorder that causes a loss of motor, pulmonary, and cardiac function, and premature death. It is the most common lethal pediatric genetic disorder, and it affects every 1 in 3,500 live

male births and some females. Duchenne is caused by the body's inability to create dystrophin, a large protein found in muscle cells. Duchenne has no FDA approved treatment or cure and is 100% fatal. Children with Duchenne are born normal and decline over time, usually losing their ability to walk around the age of 12 and succumbing to the disease in their early to mid-twenties.

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