Follistatin Gene Therapy Increases Function in Inclusion Body Myositis Patients

CLEVELAND, OH and COLUMBUS, OH--(Marketwired - May 6, 2016) - A presentation at the American Society of Gene and Cell Therapy (ASGCT) annual meeting in Washington DC described promising safety and initial efficacy data in sporadic inclusion body myositis (sIBM) patients treated with follistatin gene therapy.

In total, nine sIBM patients received follistatin gene therapy treatments and have been followed up to four years. No drug related adverse events have been reported in the study, which was conducted at Nationwide Children's Hospital in Columbus, OH. Six of the treated sIBM patients received gene therapy treatments in both legs and were evaluated for six minute walk test (6MWT) performance at multiple intervals. All six patients have showed an improvement over baseline levels and, on average, improved six minute walk distance by 3.1 meters per month following treatment. The investigators also conducted a concurrent twenty patient sIBM natural history study; the untreated patients in that study showed a decline of 2.3 meters per month.

Jerry Mendell, M.D., Director of Nationwide Children's Hospital's Center for Gene Therapy and PI on the trial, presented the data. "From our observations, follistatin gene therapy is showing significant promise for patients with sIBM and other muscle-wasting disorders. We were gratified to discuss these encouraging findings with our colleagues at the ASGCT annual meeting, and we look forward to the day when this therapy becomes a standard treatment for patients affected by neuromuscular disease," Dr. Mendell noted.

The therapy, developed at Nationwide Children's Hospital by Dr. Mendell and Dr. Brian Kaspar, is based on adeno-associated virus delivery of follistatin 344 to increase muscle strength and prevent muscle wasting. It could have broad ranging applications in muscular dystrophies, cancer treatment induced muscle wasting and age related muscle wasting. In January 2015, the investigators published results of a six patient Becker muscular dystrophy study, establishing initial safety and efficacy in that indication. Both the sIBM and Becker studies were funded by a grant from Parent Project Muscular Dystrophy. A third clinical study of patients with Duchenne muscular dystrophy is ongoing.

The underlying intellectual property was exclusively licensed in 2012 from Nationwide Children's Hospital to Ohio-based Milo Biotechnology. Milo's CEO Al Hawkins stated, "The safety record of this drug, which spans 20 patients and more than four years of follow-up, is outstanding. This result further demonstrates that the follistatin gene therapy could have significant benefits for patients with a range of muscle wasting disorders."

About Nationwide Children's Hospital

Ranked 7th of only 10 children's hospitals on U.S. News & World Report's 2014-15 "America's Best Children's Hospitals Honor Roll" and among the Top 10 on Parents magazine's 2013 "Best Children's Hospitals" list, Nationwide Children's Hospital is one of the nation's largest not-for-profit freestanding pediatric healthcare networks providing care for infants, children and adolescents as well as adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research. More information is available at NationwideChildrens.org/Research.

About Milo Biotechnology

Milo Biotechnology is a clinical stage company developing therapies to strengthen muscle and improve the lives of patients with neuromuscular diseases. Its lead program, AAV1-FS344, leads to the local expression of follistatin, a potent myostatin inhibitor. Visit www.milobiotechnology.com for more information.
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