Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

NEWS RELEASE

FOR IMMEDIATE RELEASE

CONTACT: Will Nolan: 201.250.8440

Parent Project Muscular Dystrophy To Collaborate With Catabasis Pharmaceuticals, Inc. On CAT-1004 Development

Hackensack, NJ – December 11, 2014 – Parent Project Muscular Dystrophy (PPMD), a nonprofit organization dedicated to the fight to end Duchenne muscular dystrophy (Duchenne), announced today a collaboration with Catabasis Pharmaceuticals, Inc. (Catabasis) to assist in a Phase 2 clinical trial of its CAT-1004 product candidate for the treatment of patients with Duchenne. As part of this collaboration, PPMD will provide funding to support participant travel. The trial is expected to begin in the first half of 2015.

Duchenne muscular dystrophy is the most common fatal genetic disorder diagnosed in childhood, affecting approximately one in every 3,500-5,000 live male births. The loss of a key muscle protein called "dystrophin" causes muscle wasting and weakness, eventually leading to the loss of ambulation, difficulty breathing, and heart failure. Death typically occurs in the mid- to late 20s.

CAT-1004 is designed to block a key inflammatory pathway, mediated by a molecule called "NF-κB," that is thought to contribute to the muscle wasting and loss that occurs in Duchenne. In mice that lack dystrophin, CAT-1004 has been shown to reduce inflammation and increase muscle regeneration, and the drug was well-tolerated and demonstrated a significant reduction of activated NF-κB in a study of healthy volunteers. Late last month, the U.S. Food and Drug Administration (FDA) granted CAT-1004 Orphan Drug Designation for the treatment of Duchenne.

PPMD's Founding President and CEO, Pat Furlong, remarked, "As more and more potential therapies make it to clinical trial, it is critical that we ease the burden of patients willing to participate in these important next steps – especially in a rare disease like Duchenne, where the patient population is limited. Subsidizing travel expenses is one way PPMD can at least help ease the financial burden, especially in a therapy we believe in like CAT-1004. The team at Catabasis is committed to helping the Duchenne community so it is our obligation to our children and their future to help Catabasis help us."

"We are very pleased to be collaborating with PPMD for the execution of our Phase 2 trial of CAT-1004 in DMD", said Jill

Milne, PhD, Catabasis co-founder and CEO. “PPMD’s willingness to help with patient participation in our innovative trial is a great example of PPMD’s commitment to finding a cure for this devastating disease.”

To learn more about other projects PPMD is currently funding, visit our website.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is the most common fatal genetic disorder diagnosed in childhood, affecting approximately one in every 3,500-5,000 live male births (about 20,000 new cases worldwide each year). Because the Duchenne gene is found on the X-chromosome, it primarily affects boys; however, it occurs across all races and cultures.

Duchenne results in progressive loss of strength and is caused by a mutation in the gene that encodes for dystrophin. Because dystrophin is absent, the muscle cells are easily damaged. The progressive muscle weakness leads to serious medical problems, particularly issues relating to the heart and lungs. Young men with Duchenne typically live into their late twenties.

Duchenne can be passed from parent to child, but approximately 35 percent of cases occur because of a random spontaneous mutation. In other words, it can affect anyone. Although there are medical treatments that may help slow its progression, there is currently no cure for Duchenne.

About Parent Project Muscular Dystrophy

Duchenne is a fatal genetic disorder that slowly robs young men of their muscle strength. Parent Project Muscular Dystrophy (PPMD) is the largest, most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne muscular dystrophy—our mission is to end Duchenne.

We invest deeply in treatments for this generation of young men affected by Duchenne and in research that will benefit future generations. We advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. We demand optimal care, and we strengthen, unite and educate the global Duchenne community.

Everything we do—and everything we have done since our founding in 1994—helps boys with Duchenne live longer, stronger lives. We will not rest until every young man has a treatment to end Duchenne. Go to www.ParentProjectMD.org for more information or to learn how you can support our efforts and help families affected by Duchenne.

About Catabasis Pharmaceuticals, Inc.

Catabasis Pharmaceuticals is leveraging its pathway pharmacology drug development platform to bring important medicines to patients with severe lipid disorders and rare diseases. The Company's mission is to address difficult-to-treat diseases through the simultaneous modulation of multiple targets in a disease pathway. For more information on our technology and pipeline of drug candidates, please visit www.catabasis.com.

###