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## MDA AWARDS \$120,000 TO CATABASIS PHARMACEUTICALS

### Funds Will Support Testing of Anti-Inflammatory Compounds in Mice

TUCSON, Ariz., and CAMBRIDGE, Mass., April 4, 2012 — The Muscular Dystrophy Association announced today that it has awarded \$120,000 to Cambridge, Mass.-based Catabasis Pharmaceuticals as part of a strategic partnership under which the biopharmaceutical company will test two compounds called CAT-1004 and CAT-1040 in the mdx research mouse model of [Duchenne muscular dystrophy \(DMD\)](#).

The award was made through [MDA Venture Philanthropy \(MVP\)](#), the drug development arm of MDA's translational research program.

"MDA is pleased to support further testing of CAT-1004 to assess the compound's ability to counter muscle-damaging inflammation and support muscle regeneration," said Jane Larkindale, MDA director of translational research. "We're excited to help determine if this compound, or if the related compound CAT-1040, will be helpful in Duchenne muscular dystrophy — and, perhaps, for other diseases in MDA's program."

Catabasis plans to determine in the new four-month study whether either or both CAT-1004 and CAT-1040 reduce inflammation in muscle tissue and improve muscle function. If favorable results are obtained, the company plans to begin clinical trials in people with DMD.

"We want to thank MDA for this important funding, which validates the preclinical work we have done with CAT-1004 in DMD mouse models," said Michael Jirousek, co-founder and

chief scientific officer of Catabasis. "The short-term preclinical findings in DMD models are very encouraging, and we look forward to conducting this longer-term study with both CAT-1004 and CAT-1040. There is a significant need for new treatment options with improved safety and efficacy for people with DMD. If the preclinical findings prove positive, we will initiate a clinical trial in patients with the disease, which could potentially lead to a new treatment and approach to slow the rate of muscle degeneration in young boys living with DMD."

CAT-1004 is an orally available, anti-inflammatory agent. In a previous MDA-supported mouse study, the compound was compared with the corticosteroid prednisolone (similar to prednisone). In the study, CAT-1004 worked as well as prednisolone at reducing muscle inflammation without the prednisolone side effect of reduced muscle weight. Mice treated with the compound had the same or fewer degenerating muscle fibers than those treated with prednisolone and also had increased numbers of regenerating muscle fibers. Those treated with prednisolone had decreased numbers of regenerating fibers.

Catabasis recently completed a phase 1 safety, tolerability and pharmacokinetics (what the body does to a drug) study of CAT-1004 in healthy human volunteers, in which the drug was found to be safe and well-tolerated. The company says that the pharmacokinetics results from the study offer chemical proof of concept that the approach works in humans and that CAT-1004 offers broad utility in treating diseases of inflammation.

Catabasis has an open Investigational New Drug application for the compound with the U.S. Food and Drug Administration, which allows it to conduct clinical trials of the investigational drug in the United States.

### **About DMD**

DMD is a degenerative muscle disease, affecting boys almost exclusively, that involves progressive degeneration of voluntary and cardiac muscles, with resulting weakness and heart abnormalities. The disease manifests in early childhood, causing delayed motor milestones and, in some cases, cognitive, behavioral or language abnormalities. Loss of the ability to walk occurs in most children with DMD between the ages of 10 and 12 years; weakened cardiac and respiratory muscles severely limit life span.

The disease is caused by any number of mutations in the X-chromosome gene that carries instructions for the muscle protein dystrophin. Without dystrophin, muscle fibers are abnormally fragile and break down under the stress of contractions.

Corticosteroid medications, such as prednisone and its chemical cousin prednisolone, slow disease progression.

### **About MDA Venture Philanthropy (MVP)**

MVP is the Muscular Dystrophy Association's drug development program, which operates within MDA's translational research program. MVP is exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular diseases. For more information, visit [mda.org](http://mda.org) and follow MDA on Facebook ([facebook.com/MDANational](https://facebook.com/MDANational)) and Twitter (@MDAnews).

### **About Catabasis**

Catabasis is a clinical-stage company dedicated to the discovery and development of innovative, effective and safe medicines to treat inflammatory and metabolic diseases. The company has assembled a team of passionate and experienced scientists who are committed to improving the lives of patients. Catabasis has developed a pipeline of molecules that produces mechanistic synergy on a targeted pathway. The company's technology platform utilizes pathway pharmacology and has produced a robust pipeline of new chemical entities (NCEs). Catabasis' approach dramatically enhances the therapeutic potential of docosahexaenoic acid (DHA) and eicosapentaenoic acid (EPA) by improving their delivery, potency and efficacy through SMART linker technology. Founded in 2008, Catabasis is headquartered in Cambridge, Mass. Please visit [catabasispharma.com](http://catabasispharma.com) for more information.

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