



Catabasis Pharmaceuticals Receives Rare Pediatric Disease Designation from FDA for CAT-1004 for the Potential Treatment of Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 14, 2015-- [Catabasis Pharmaceuticals, Inc.](http://www.catabasis.com) (NASDAQ:CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to CAT-1004 for the treatment of Duchenne muscular dystrophy (DMD). CAT-1004 is designed to inhibit activated NF-kB, a protein that plays an important role in muscle health. CAT-1004 has the potential to reduce muscle inflammation and degeneration and promote muscle regeneration in patients with DMD regardless of the underlying dystrophin mutation. The FDA has previously granted Fast Track and Orphan Drug Designations for CAT-1004 for the treatment of DMD.

"We are pleased that the FDA's Office of Orphan Products Development has granted CAT-1004 a Rare Pediatric Disease Designation. CAT-1004 has the potential to make a meaningful impact for the patients and families affected by DMD through a disease-modifying mechanism that could be applicable to all patients with this disorder," said Jill C. Milne, Ph.D., co-founder and chief executive officer of Catabasis. "We look forward to continuing to advance this product candidate in our Phase 1 / 2 MoveDMD clinical trial, which is currently enrolling."

About Rare Pediatric Disease Designation

The FDA defines a "rare pediatric disease" as a disease that primarily affects individuals aged from birth to 18 years and affects fewer than 200,000 individuals in the U.S. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, upon the approval of a qualifying new drug application (NDA) or biologics license application (BLA) for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a Rare Pediatric Disease Priority Review Voucher that can be used to obtain priority review for a subsequent NDA or BLA. The Priority Review Voucher may be sold or transferred an unlimited number of times.

About CAT-1004

CAT-1004 is an oral small molecule that inhibits activated NF-kB, a protein that coordinates cellular response to muscular damage, stress and inflammation and plays an important role in muscle health. In skeletal muscle, activated NF-kB drives muscle degeneration and suppresses muscle regeneration. In animal models of DMD, CAT-1004 inhibited activated NF-kB, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials, CAT-1004 inhibited activated NF-kB and was well-tolerated with no observed safety concerns. The FDA has granted CAT-1004 orphan drug and fast track designations for the treatment of DMD. Catabasis is currently conducting the MoveDMD Phase 1 / 2 trial of CAT-1004 in 4-7 year-old boys with DMD.

About Catabasis

Catabasis Pharmaceuticals is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics using its proprietary Safely Metabolized And Rationally Targeted, or SMART, linker technology platform. The Company's SMART linker technology platform is based on the concept of treating diseases by simultaneously modulating multiple targets in one or more related disease pathways. The Company engineers bi-functional product candidates that are conjugates of two molecules, or bioactives, each with known pharmacological activity, joined by one of its proprietary SMART linkers. The SMART linker conjugates are designed for enhanced efficacy and improved safety and tolerability. The Company's focus is on treatments for rare diseases. The Company is also developing other product candidates for the treatment of serious lipid disorders. For more information on the Company's technology and pipeline of drug candidates, please visit www.catabasis.com.

Forward Looking Statements

Any statements in this press release about future expectations, plans and prospects for the Company, including statements about future clinical trial plans and other statements containing the words "believes," "anticipates," "plans," "expects," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products; availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of the Company's product candidates; and general economic and market conditions and other factors discussed in the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the three months ended June 30, 2015, which is on file with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this

press release represent the Company's views as of the date of this press release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.

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Source: Catabasis Pharmaceuticals, Inc.

Corporate and Media Contact
Catabasis Pharmaceuticals, Inc.
Andrea Matthews, 617-349-1971
amatthews@catabasis.com