



European Commission Grants Orphan Medicinal Product Designation for CAT-1004, Catabasis Pharmaceuticals' Investigational Therapy for the Treatment of Duchenne Muscular Dystrophy

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 26, 2015-- [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced that the European Commission (EC) has granted orphan medicinal product 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 is a potential disease-modifying therapy being developed to reduce muscle inflammation and degeneration and promote muscle regeneration in patients affected by DMD regardless of the underlying dystrophin mutation. The U.S. Food and Drug Administration (FDA) has previously granted Fast Track, Orphan Drug, and Rare Pediatric Disease Designations for CAT-1004 for the treatment of DMD.

"Receiving orphan medicinal product designation in Europe supports our global regulatory strategy for the development of CAT-1004 and our goal of providing an important new treatment option to patients around the world who are affected by DMD," said Jill C. Milne, Ph.D., co-founder and chief executive officer of Catabasis. "We look forward to presenting our clinical development program for CAT-1004, including the ongoing Phase 1 / 2 MoveDMDSM trial, at the Action Duchenne International Conference in London on November 6."

"We are highly encouraged by the European Commission's leadership in their designation of CAT-1004. We feel this may facilitate the future development of the compound in Europe, and most importantly, may give the boys a different treatment approach for Duchenne. We shall look forward to working more closely with Catabasis as their future plans develop," commented Diana Ribeiro, Director of Research at Action Duchenne.

About European Orphan Medicinal Product Designation

In the European Union (EU), orphan medicinal product legislation provides incentives for drug development sponsors to develop products for diagnosing, preventing or treating life-threatening or very serious conditions that are rare and affect not more than five in 10,000 persons in the EU. The European Medicines Agency, through its Committee for Orphan Medicinal Products (COMP), is responsible for reviewing designation applications from persons or companies who intend to develop orphan medicines and making a recommendation to the European Commission. Sponsors may receive regulatory and financial incentives including, up to 10 years of market exclusivity if the product candidate is granted marketing authorization in the EU. Orphan designation also makes companies eligible for EMA assistance in optimizing the candidate's clinical development through participation in clinical trial design and preparation of the product marketing application, consideration for reduced regulatory fees, and a potential EU-funded research grant.

About Action Duchenne

Action Duchenne is the UK charity dedicated to finding a cure or viable treatments for Duchenne Muscular Dystrophy; whilst improving the lives of everyone affected by the condition. For more information, please visit www.actionduchenne.com.

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, fast track and rare pediatric disease 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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