



## **Catabasis Announces Positive Phase 1 Data for CAT-1004 Validating the SMART Linker Technology Platform**

*CAT-1004 Components Act Synergistically to Inhibit the NF- $\kappa$ B pathway*

CAMBRIDGE, Mass., February 11, 2013 – [Catabasis Pharmaceuticals, Inc.](#), today announced that a three-part series of Phase 1 studies showed that CAT-1004, a SMART Linker conjugate of docosahexaenoic acid (DHA) and salicylate created using the company's proprietary technology, was safe and well tolerated. Data also show that the conjugated compound was absorbed following oral administration and metabolized to its active components intracellularly, providing proof of concept for the SMART Linker technology platform.

Biomarkers at both the gene and protein level showed that treatment with CAT-1004 resulted in significant inhibition of the NF- $\kappa$ B pathway. A single dose of CAT-1004 produced a 70 percent reduction in nuclear p65, a marker of NF- $\kappa$ B activation. This effect was not observed with placebo or when DHA and salicylate were given as a simple combination, demonstrating that CAT-1004 produces a synergistic effect on NF- $\kappa$ B.

"In addition to the excellent safety and tolerability data for CAT-1004, the Phase 1 studies showed that our SMART Linker technology enables our conjugate to escape hydrolysis in the GI tract and plasma, and enter cells where hydrolysis releases the components to act synergistically by targeting multiple points along the NF- $\kappa$ B pathway," said Jill Milne, Ph.D., co-founder and chief executive officer at Catabasis. "Importantly, these data validate our approach to leveraging pathway pharmacology in an innovative way leading to the development of new treatments for inflammatory and metabolic diseases."

In a three-part series of Phase 1 studies, 105 subjects received CAT-1004 or placebo for up to 14 days. No serious adverse events or safety concerns were identified. These studies will be presented at a future scientific meeting. Phase 2 studies are being planned to evaluate the safety and efficacy of CAT-1004 in patients with inflammatory bowel disease. In addition, Phase 2 studies to investigate the potential of CAT-1004 as a replacement for corticosteroids in patients with Duchenne muscular dystrophy are being planned.

### **About CAT-1004**

CAT-1004 is a novel chemical entity that is a conjugate of the omega-3 fatty acid docosahexaenoic acid (DHA) and salicylate. Catabasis' lead program amplifies the beneficial effects of omega-3 fatty acids and salicylate to target inflammation, an underlying cause of many chronic diseases. A growing body of peer-reviewed research, along with clinical experience, has demonstrated important therapeutic benefits of DHA and eicosapentaenoic acid (EPA). Salicylates are anti-inflammatories with proven clinical efficacy and safety, and have been used safely for years to treat inflammatory bowel disease and other diseases of inflammation. Inhibition of NF- $\kappa$ B in muscle satellite cells has been hypothesized to have a beneficial effect in Duchenne muscular dystrophy. In preclinical studies, CAT-1004 has been shown to simultaneously inhibit pro-inflammatory pathways and activate endogenous anti-inflammatory pathways to resolve inflammation.

### **About Inflammatory Bowel Disease**

Inflammatory Bowel Disease is a chronic inflammatory disease affecting the colon and small intestine, resulting in symptoms such as diarrhea, severe abdominal pain, weight loss, and vomiting, with a significant reduction in quality of life and the risk of life-threatening complications. The most common forms of the disease, Ulcerative Colitis and Crohn's disease, affect more than two million people in the U.S., Europe and Japan. Salicylates are an important therapy in the treatment of IBD.

### **About Duchenne Muscular Dystrophy**

Duchenne muscular dystrophy is caused by a mutation in the dystrophin gene, resulting in progressive muscle weakness. NF-kB mediated inflammation is believed to play an important role in clinical manifestations of weakened skeletal muscles that eventually results in cardiac and pulmonary impairment. Corticosteroids are the current standard of care treatment for DMD. While this treatment delays disease progression by several years, their prolonged use is typically associated with significant side effects and the treatment does not alter the ultimate outcome of the disease.

### **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's drug development programs are rooted in the principles of pathway pharmacology, the treatment of diseases by simultaneously modulating more than one target in a disease pathway. Using its proprietary SMART Linker technology, the company conjugates two drugs that act on different components of a disease pathway to produce new chemical entities with significantly enhanced efficacy and an improved safety and tolerability profile. The company has assembled a team of passionate and experienced scientists who are committed to improving the lives of patients. The company was founded in 2008 and is headquartered in Cambridge, Mass.

Please visit [www.catabasis.com](http://www.catabasis.com) for more information.

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