



## **Acceleron Pharma's ACE-031 Increases Muscle Mass and Strength in Preclinical Studies**

*-- Novel Compound Shown to be Promising Potential Therapy for Multiple Diseases with Unmet Medical Need --*

LUCCA, ITALY – May 14, 2007 – Acceleron Pharma, Inc., a biopharmaceutical company developing therapeutics for the treatment of musculoskeletal, metabolic and cancer-related diseases, today announced results of a preclinical study with ACE-031 that demonstrated the ability of the compound to increase muscle mass and strength in normal mice and in murine models of muscular dystrophy. ACE-031 inhibits negative regulators of muscle to increase muscle mass and strength as a potential treatment for neuromuscular diseases and cancer-related muscle loss. The data was presented on Sunday, May 13 at the Gordon Research Conference on Myogenesis in Lucca, Italy.

“Acceleron is encouraged by the significant outcomes and positive data generated by ACE-031 spanning numerous preclinical models of muscle loss”, said Jas Seehra, Ph.D., Chief Scientific Officer of Acceleron. “ACE-031 and related Acceleron compounds consistently demonstrate substantial increases in skeletal muscle mass and strength.”

ACE-031, a protein therapeutic based on the activin receptor type IIB (ActRIIB), is the first of several compounds Acceleron is developing for the treatment of diseases involving the loss of muscle mass, strength and function. ActRIIB binds to myostatin (GDF-8) and other negative regulators of muscle mass and strength. Over-expression of myostatin has been shown to cause a loss in muscle mass and strength, and inhibition of myostatin results in the selective increase in skeletal muscle mass and strength. In preclinical models, ACE-031 increased skeletal muscle mass and strength in disease models of amyotrophic lateral sclerosis (ALS), muscular dystrophy, glucocorticoid-induced muscle loss and age-related muscle loss (sarcopenia). ACE-031 offers the potential for an entirely new type of therapy for the treatment of various muscle wasting conditions.

“The clinical potential of ACE-031 to selectively increase skeletal muscle will offer a growing number of patients an excellent opportunity to benefit from a promising therapy for the otherwise untreatable muscle loss associated with their disease,” said Matthew L. Sherman, M.D., Chief Medical Officer of Acceleron. “Based on the comprehensive preclinical program currently underway, Acceleron is working toward advancing ACE-031 into Phase 1 clinical trials later this year.”

### **Study Findings**

The presentation, entitled “Treatment with a Soluble Activin Receptor IIB Causes Increased Muscle Mass and Strength in mdx Mice” discussed data from preclinical studies in normal mice and in a mouse model of muscular dystrophy. These studies show a substantial increase in muscle mass and a dose-dependent increase in lean body mass in treated animals. These effects are significant in just two weeks following start of therapy and increase further as treatment continues. In the *mdx* murine model of muscular dystrophy, treated animals showed increases in muscle mass which translated into significantly increased muscle strength.

### **About Muscle Loss**

Loss of muscle mass and strength is a serious clinical consequence of several types of diseases and conditions: (1) neuromuscular diseases, such as ALS and muscular dystrophy, in which patients suffer from the loss of muscle strength and function which ultimately leads to respiratory failure and death (2)

cancer-cachexia, in which patients can lose muscle mass and body weight as a result of both their cancer treatments (hormone deprivation therapy causes loss of muscle) and the cancer itself (3) other cachexias, in which the muscle loss is associated with chronic diseases such as congestive heart failure, chronic obstructive pulmonary disease, and chronic kidney disease and, (4) age-related muscle loss, known as sarcopenia, in which generalized muscle loss and frailty leads to frequent falls and morbidity and mortality from broken bones. A drug candidate that would increase muscle mass and strength is expected to produce a major clinical benefit across this broad range of serious medical conditions in which there are currently no safe and effective therapies that selectively increase skeletal muscle mass and strength.

#### About Acceleron Pharma

Acceleron is a privately held biopharmaceutical company committed to discover, develop, manufacture and commercialize novel biotherapeutics that modulate the growth of bone, muscle, fat and the vasculature to treat musculoskeletal, metabolic and cancer-related diseases. Acceleron's scientific approach takes advantage of its unique insight on the regenerative powers of two protein families: the Growth and Differentiation Factor (GDF) and Bone Morphogenetic Protein (BMP) families. The company's lead program is an anabolic bone agent being developed to reverse bone loss in diseases such as osteoporosis and cancer. Additionally, the company is advancing several preclinical programs that increase muscle mass, control angiogenesis and inhibit fat accumulation. Acceleron utilizes proven biotherapeutic technologies and capitalizes on the company's internal GMP manufacturing capability to rapidly and efficiently advance its therapeutic programs. The investors in Acceleron are Advanced Technology Ventures, Flagship Ventures, OrbiMed Advisors, Polaris Ventures, Sutter Hill Ventures and Venrock.

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