Acceleron Pharma’s ACE-031 Increases Lean Body Mass
in Phase 1 Single Dose Clinical Trial

CAMBRIDGE, Mass. – September 10, 2009 - Acceleron Pharma, Inc., a biopharmaceutical company developing novel therapeutics that modulate the growth of cells and tissues including red blood cells, bone, and muscle, today announced preliminary results from the ACE-031 Phase 1 single dose clinical trial demonstrating that ACE-031 increased lean body mass and muscle volume. The results from this randomized, placebo-controlled study were presented at the 14th International Congress of the World Muscle Society in Geneva, Switzerland.

“We are thrilled that this ACE-031 Phase 1 clinical trial generated a substantial amount of encouraging safety data as well as clear signs of biological activity on muscle, bone and fat all following just a single dose of ACE-031,” said Matthew Sherman, M.D., Chief Medical Officer at Acceleron. “ACE-031 produced rapid, sustained and dose-dependent increases in lean body mass and muscle volume, increased biomarkers of bone formation and positively altered biomarkers of fat mass. Based on these results, we’ve initiated a Phase 1 multiple dose study and look forward to further development of ACE-031 for the treatment of patients suffering from neuromuscular diseases.”

Summary Results

- ACE-031 was well tolerated at all dose levels and demonstrated a linear pharmacokinetic profile with an average half-life ranging from 10-15 days
- Single doses of ACE-031 at 1 mg/kg and 3 mg/kg produced dose-dependent increases in lean body mass measured by dual energy X-ray absorptiometry (DXA) as early as day 15 that were sustained through day 57
- Subjects given single doses of placebo had a 0.2% decrease in lean body mass at day 57 compared to a 2.4% increase in subjects receiving 1 mg/kg ACE-031 and a 2.6% increase in subjects receiving 3 mg/kg ACE-031
- Subjects given single doses of placebo had a 0.2% decrease in muscle volume assessed by MRI at day 29 compared to a 3.5% increase in subjects receiving 1 mg/kg ACE-031 and 5% increase in subjects receiving 3 mg/kg ACE-031
- ACE-031 favorably affected biomarkers of fat mass (increased adiponectin and decreased leptin) and bone formation and resorption (increased bone-specific alkaline phosphatase (BSAP) and decreased C-terminal type 1 collagen telopeptide (CTX)) at doses of 1 and 3 mg/kg

“ACE-031 holds great potential as a treatment for patients suffering from neuromuscular diseases such as muscular dystrophy and amyotrophic lateral sclerosis (ALS),” said John Knopf, Ph.D., Chief Executive Officer of Acceleron. “We are moving forward with great confidence and excitement with the ACE-031 program as well as our overall muscle franchise.”

Muscle is increasingly recognized as central to many biological processes and plays a major role in human health. The loss of muscle mass and strength is ultimately directly related to the cause of death in neuromuscular diseases such as muscular dystrophy and amyotrophic lateral sclerosis. Severe muscle loss in cancer leads to serious complications and a poor prognosis. Muscle loss is a natural consequence of aging, similar to bone loss, resulting in decreased muscle strength (frailty), reduced mobility and an increased risk of a fall and broken bones. In metabolic diseases, an imbalance of diet, energy utilization and skeletal muscle leads to poor metabolic function. By increasing muscle mass there is a corresponding decrease in fat mass and improvements in metabolic function.
About ACE-031

ACE-031, a soluble fusion protein based on the activin receptor type IIB (ActRIIB), is a biologic therapeutic that inhibits signaling through the ActRIIB receptor. By preventing signaling through ActRIIB, ACE-031 increases muscle mass and strength. In numerous and varied animal models of disease, ACE-031 significantly increased muscle mass and muscle strength. ACE-031, and a related molecule ACE-435, have shown encouraging preclinical results in animal models of age-related muscle loss, neuromuscular disease, cancer treatment-related muscle loss and metabolic diseases. ACE-031 is currently being studied in a Phase 1 multiple dose clinical trial in healthy volunteers. ACE-435 is expected to enter clinical trials in 2010.

About Acceleron

Acceleron is a privately held biopharmaceutical company committed to discover, develop, manufacture and commercialize novel biotherapeutics that modulate the growth of red blood cells, bone, muscle, fat and the vasculature to treat musculoskeletal, metabolic and cancer-related diseases. Acceleron’s scientific approach takes advantage of its unique insight into the regenerative powers of the TGF-beta superfamily of proteins. ACE-011 is currently being studied in two Phase 2 clinical trials in cancer patients. ACE-031 is currently being studied in a Phase 1 clinical trial in healthy volunteers. In addition, the company is advancing new product candidates that increase muscle mass, control angiogenesis, inhibit fat accumulation and increase hemoglobin. 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 include Advanced Technology Ventures, Bessemer Ventures, Celgene, Flagship Ventures, MPM BioEquities, OrbiMed Advisors, Polaris Ventures, QVT Financial, Sutter Hill Ventures and Venrock. For more information, visit www.acceleronpharma.com.

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