Epirium Bio Announces Commencement of Phase 1 Clinical Trial of EPM-01 in Becker Muscular Dystrophy

-- Additionally, European Commission Grants Investigational Medicine EPM-01 Orphan Designation for the Potential Treatment of Becker Muscular Dystrophy --

San Diego, Calif. – August 12, 2020 – Epirium Bio Inc., a clinical-stage biopharmaceutical company pursuing novel and clinically significant therapeutic approaches for neuromuscular, neurodegenerative and mitochondrial disorders, today announced enrollment of the first participants in its Phase 1 clinical trial of EPM-01 in Becker muscular dystrophy. EPM-01 is a novel, oral, synthetic compound that has demonstrated compelling proof-of-concept in multiple preclinical and clinical studies of mitochondrial dysfunction. The Phase 1 study will be conducted by investigators at three leading academic centers in the United States, including University of California, Davis, and Washington University School of Medicine, St. Louis.

Additionally, the company announced that the European Commission (EC) granted orphan designation for EPM-01 as a potential treatment for Becker muscular dystrophy, a rare, genetic, neuromuscular disorder that affects approximately 10,000 people in the European Union (EU) and 8,000 people in the United States. EPM-01 was previously granted orphan drug designation by the U.S. Food and Drug Administration (FDA) for the treatment of Becker and Duchenne muscular dystrophy.

“The initiation of enrollment in our Phase 1 study and the EC designation of orphan drug status for EPM-01 in Becker muscular dystrophy are both important milestones for this development program, which is based on a differentiated therapeutic approach to this disease,” said Ransi Somaratne, M.D., FACC, chief medical officer of Epirium Bio. “Patients living with Becker muscular dystrophy are in need of dedicated therapies, as there are no approved treatments for this inherited disorder. We look forward to the results of our Phase 1 trial, which will help us select the optimal dose of EPM-01 for a pivotal clinical study in this patient population.”

“This advancement into Phase 1 enrollment for EPM-01 represents a major step forward in the development of a novel therapy for people living with Becker muscular dystrophy,” said Russ Cox, president and chief executive officer of Epirium Bio. “I expect this study will provide valuable insights to inform the next phases of clinical development and bring us closer to being able to treat patients with this degenerative and life-threatening disease.”

Phase 1 Clinical Trial Design

The Phase 1 open-label, dose-escalation study is evaluating the safety, preliminary clinical efficacy and potential biomarkers of three doses of EPM-01 in participants with Becker or Becker-like muscular dystrophy. The study is expected to enroll approximately 20 male participants, ages 16 to 59, who are ambulatory and have confirmed mutations of the dystrophin gene. After the six-month dose-escalation period, participants will continue treatment at the highest tolerated dose for an additional six months with a total one-year follow-up duration after dose initiation.

For more information, visit clinicaltrials.gov (NCT04386304).

About EU Orphan Designation
Orphan designation in the EU is granted by the EC based on a positive opinion issued by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products. To qualify, an investigational medicine must be intended to treat a seriously debilitating or life-threatening condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient non-clinical or clinical data to suggest the investigational medicine may produce clinically relevant outcomes. Orphan designation provides companies with benefits and incentives including clinical protocol assistance, differentiated evaluation procedures for Health Technology Assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees, and 10 years of market exclusivity.

About Becker Muscular Dystrophy
Becker muscular dystrophy is a rare, genetic, degenerative disease that causes progressive muscle weakening and loss, primarily affecting skeletal and cardiac muscles. The disease mainly affects boys and usually start to manifest between age 10 and 15. It is estimated to affect between one in 18,000 and one in 30,000 male births globally. Becker muscular dystrophy is caused by mutations in the gene responsible for the production of dystrophin, a protein that forms an important component of muscle fibers. As patients do not have enough working dystrophin, muscle fibers gradually break down, leading to muscle weakness. Becker muscular dystrophy causes long-term disability and is life-threatening because of its effects on the heart and the muscles used to breathe. No therapies are currently approved for Becker muscular dystrophy in the United States or the EU.

About EPM-01
EPM-01 is a synthetic compound that appears to affect multiple physiologic pathways including cellular energetics and muscle regeneration through key regulatory steps. Reduced levels of abnormally functioning dystrophin lead to a vicious cycle of harmful processes in the muscles of patients with Becker muscular dystrophy, including calcium dysregulation, mitochondrial dysfunction, inflammation and ultimately loss of functioning muscle tissue to fibrosis. Based on its mechanism of action, EPM-01 is expected to address multiple steps in this destructive cycle to break or slow its progression, potentially leading to clinical and functional benefit in patients with severe mitochondrial disorders.

About Epirium
Epirium is a clinical-stage biopharmaceutical company that uses unique insights related to the biology of mitochondrial function and tissue regeneration to pursue novel and clinically significant therapeutic approaches for neuromuscular, neurodegenerative and mitochondrial disorders. The Company has identified and established an IP-protected platform of small molecules that constitute a new class of therapeutics with the potential to improve mitochondrial structure and function, as well as stimulate mitochondrial biogenesis and tissue regeneration. Epirium has advanced its first clinical candidate (EPM-01) in Becker muscular dystrophy and plans to follow with drug development targeting other disorders associated with mitochondrial dysfunction and tissue degeneration. To learn more, please visit www.epirium.com.

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