Epirium Bio Announces Positive Phase 1 Clinical Trial Results Evaluating MF-300 in Healthy Volunteers,
A First-In-Class, Oral 15-PGDH Enzyme Inhibitor, For the Treatment of Sarcopenia

No safety concerns or dose limiting toxicities observed, pharmacodynamic analysis demonstrated evidence of dose dependent target engagement together with a biologic effect

San Diego, September 24, 2025. Epirium Bio Inc. (Epirium), a clinical-stage biopharmaceutical company advancing medicines for neuromuscular and fibrotic diseases, today announced positive results from its Phase 1 trial evaluating MF-300, a novel therapy in development for sarcopenia. The primary endpoint of safety was achieved, and all doses of MF-300 studied were generally well tolerated with no subject discontinuations. MF-300 produced dose-related pharmacodynamic (PD) responses which were observed early and sustained over time, whereas placebo showed no meaningful changes, supporting target engagement and biologic activity. Pharmacokinetic (PK) analyses demonstrated dose-related increases in exposure, and the observed half-life supports convenient once-daily oral dosing.

“We are encouraged by the success of the Phase 1 study. MF-300 demonstrated target engagement, reducing urinary metabolites by levels consistent with maximal gains in muscle force in MF-300 treated aged mice. Our study also confirmed mechanism, as MF-300 produced substantial mean increases in urinary PGE2 - comparable to levels observed in human muscle tissue after exercise - versus a mean decrease from baseline with placebo,” said Alex Casdin, Chief Executive Officer of Epirium.

Mr. Casdin added, “Our positive Phase 1 results support advancing MF-300 into a Phase 2 safety and efficacy study in patients with sarcopenia, a disease affecting over 20 million seniors, for which there are no FDA-approved therapies.”

Phase 1 Study Details

The randomized, double-blind, placebo-controlled single and multiple-ascending dose (SAD and MAD) trial was designed to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of MF-300 in healthy adults. MF-300 is an investigational, first-in-class, orally administered, 15-hydroxyprostaglandin dehydrogenase (15-PGDH) enzyme inhibitor currently in development for the treatment of sarcopenia, or age-related muscle weakness.

A total of 70 healthy volunteers were randomized to receive oral administration of MF-300 or placebo as part of five single-ascending dose cohorts or three multiple-ascending dose cohorts with once daily dosing for 5 days. MF-300 was well tolerated across all cohorts, with no serious adverse events and all participants completed the study. Most adverse events were assessed as mild. Pharmacokinetic analyses demonstrated a half-life supporting a convenient once-daily oral administration. Evidence of on-target biological activity was observed, in a dose-dependent manner, as measured by changes in PGE2 and its metabolites, guiding dose selection for Phase 2.

Dedicated SAD and MAD cohorts of older adults are currently ongoing. This part of the study will provide additional safety, PK, and PD data in a population relevant to sarcopenia, with results expected by the end of the year.

The company plans to initiate a randomized, placebo-controlled, Phase 2 clinical trial in patients with sarcopenia in mid-2026.

“Sarcopenia affects millions worldwide, leading to progressive loss of muscle strength, declining mobility, and risk of dependency — yet there are still no approved therapies. Positive early clinical studies, such as this one, represent an important step toward addressing this critical need,” said Dr. David Cella, a leading authority on patient reported outcomes in sarcopenia, and Director of the Institute for Public Health and Medicine - Center for Patient-Centered Outcomes, Northwestern University’s Feinberg School of Medicine.

About MF-300

MF-300 is an investigational, orally bioavailable small molecule that reversibly occupies the prostaglandin E2 (PGE2) binding site of 15-hydroxyprostaglandin dehydrogenase (15-PGDH). 15-PGDH metabolically degrades PGE2, generating non-functional PGE2 metabolites, and is transcriptionally upregulated in aged muscle. Preclinical data show that PGE2 plays a crucial role in promoting aged muscle strength by improving muscle quality (i.e., muscle strength independent of muscle mass) as well as function of the neuromuscular junction. In preclinical studies, oral administration of MF-300 increases physiologic levels of PGE2 in skeletal muscle in rats and it increases muscle force and improves muscle quality in aged mice. Inhibiting 15-PGDH in aged muscle may be a strategy to increase physiologic levels of PGE2 to improve muscle quality and function in sarcopenia.

About Sarcopenia

The U.S. Food and Drug Administration (FDA) estimates that up to a third of Americans over the age of 60 are affected by sarcopenia, a disease that increases the risk of falls, fractures, disability and all-cause mortality. Despite sarcopenia’s widespread prevalence and serious health implications, there are currently no FDA-approved therapies available to treat sarcopenia, highlighting the significant unmet medical need for this disease.

About Epirium Bio

Epirium, a biopharmaceutical company based in San Diego, California, has identified and established an IP-protected platform of orally bioavailable small molecules that constitute a new class of therapeutics with the potential to improve function in neuromuscular diseases, including sarcopenia and spinal muscular atrophy. Epirium has generated preclinical data in a broader scope of indications with significant unmet medical need, including fibrosis, which Epirium's development pipeline has the potential to address.

To learn more about Epirium, please visit www.epirium.com and follow us on LinkedIn.

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