

FOR IMMEDIATE RELEASE



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Phrixus Pharmaceuticals announces Phase 2 clinical trial of Poloxamer 188 NF in non-ambulatory patients with Duchenne muscular dystrophy

- Phrixus to test Carmeseal-MD™ (P-188 NF) at Cincinnati Children's Hospital in a single-site, open-label trial against respiratory and cardiac end points -

ANN ARBOR, MI – X September 2017 – Phrixus Pharmaceuticals, Inc. (“Phrixus”), a company focused on therapies for Duchenne muscular dystrophy (DMD) and heart failure, today announced agreements with several DMD patient organizations and Cincinnati Children’s Hospital to conduct a first clinical trial of Carmeseal-MD™’s, active pharmaceutical ingredient Poloxamer 188 NF (P-188 NF), for the treatment of DMD in non-ambulatory patients. A number of endpoints will be evaluated including effects of P-188 NF on respiratory endpoints such as forced vital activity and on secondary endpoints related to cardiac and skeletal limb muscle performance.

“Older, non-ambulatory patients with DMD have few treatment options, especially with regard to heart failure and respiratory dysfunction, the two leading causes of death. Therefore, they are in urgent need of additional therapies that can slow or arrest disease progression,” said Dr. John L. Jefferies, Director, Advanced Heart Failure and Cardiomyopathy, Professor, Pediatric Cardiology and Adult Cardiovascular Diseases, The Heart Institute, Professor, Division of Human Genetics, Cincinnati Children’s Hospital Medical Center, who will be the Principal Investigator.

This single-center, open-label Phase 2 study will enroll 8 patients, non-ambulatory boys and young men with early heart failure and respiratory dysfunction on stable regimen of background therapies, including corticosteroids. Patients will be evaluated by cardiac MRI, pulmonary function testing and a number of upper body function tests, including performance of upper limb (PUL). Dosing will be for 52 weeks at 5 mg/Kg of P-188 NF, injected once-a-day subcutaneously, with drug product provided by the National Heart Lung and Blood Institute.

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Charley's Fund and Team Joseph led the funding of this trial, with significant support from Harrison's Fund, Hope for Gus, JB's Keys to DMD, Little Hercules, and Michael's

Cause. Phrixus has a history of support from the patient community, with previous funding provided by Coalition Duchenne, Hope for Gus, Michael's Cause, Ryan's Quest, and Save Our Sons. "We are excited to join forces with our colleagues in the nonprofit community to support the development of Carmeseal-MD as a treatment for Duchenne," said Dr. Benjamin Seckler, President of Charley's Fund. "This pilot study will generate initial data in the Duchenne population for a promising treatment that targets a major unmet need: Duchenne-related cardiac and pulmonary failure. This is an exciting step forward."

Said Cath Jayasuriya, Founder and Executive Director of Coalition Duchenne: "Coalition Duchenne is pleased to see momentum continue to build for research we first identified and sponsored in 2012 as part of our focus on cardiac and pulmonary issues. We're grateful for the support the community has provided for the initial stages of development that has led to full funding of this first clinical trial for this promising therapeutic."

Carmeseal-MD is available outside of the United States through Phrixus's Expanded Access Program and Ethicor Pharma Ltd., a distributor. For more information, please visit www.ethicorpharma.com or contact enquiries@ethicorpharma.com.

About Carmeseal-MD™

In animal models of DMD, Carmeseal-MD (Poloxamer 188 NF) has been shown to improve the efficiency of damaged hearts and the performance of damaged diaphragms with once-a-day subcutaneous administration at low doses. When infused into the bloodstream, it encounters and binds to microscopic tears in the muscle and prevents the pathological leakage of calcium into the cells, which keeps the muscle from performing as required. Carmeseal-MD is expected to have its effect in patients with DMD irrespective of the genetic defect that causes the disease.

About Duchenne muscular dystrophy (DMD)

DMD is the most devastating of the muscular dystrophies. It is a genetic disease that affects about 20,000 boys and young men in the United States and a comparable number in Europe. The hallmarks of DMD are skeletal muscle weakness followed by respiratory distress and heart failure. As a degenerative disease, it inevitably leads to premature death, mostly through heart failure and respiratory failure.

About Phrixus Pharmaceuticals, Inc.

Phrixus Pharmaceuticals, Inc. is developing Carmeseal as Carmeseal-MD™ (P-188 NF for subcutaneous injection) for DMD and as Carmeseal-HF™ (P-188 NF for intravenous administration) for acute decompensated heart failure. Phrixus has assembled the leading global patent portfolio for the use of poloxamers in DMD, heart failure and respiratory dysfunction. For more information: Thomas A. Collet, thomas.collet@phrixuspharmaceuticals.com or www.phrixuspharmaceuticals.com.

Forward-Looking Statement Disclaimer

This announcement may contain, in addition to historical information, certain forward-looking statements that involve risks and uncertainties. Such statements reflect management's current views and are based on certain assumptions. Actual results could differ materially from those currently anticipated. The company is developing several products for potential future marketing. There can be no assurance that such development efforts will succeed, that such products will receive required regulatory clearance or that, even if such regulatory clearance were received, such products would ultimately achieve commercial success.

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