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## Carmeseal-MD™ protects skeletal limb muscle, the third major target muscle of therapeutic relevance in Duchenne muscular dystrophy (DMD)

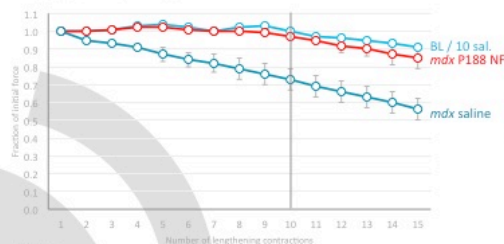
- Carmeseal-MD™, the first disease-modifying agent with the potential to protect all three dystrophic target muscles: skeletal limb muscle as well as diaphragm and heart -

ANN ARBOR, MI – 12 November 2015 – Phrixus Pharmaceuticals welcomes the results from a preclinical study that examines the effects of Carmeseal-MD (P-188 NF) in the *mdx* mouse, a leading preclinical model of DMD. This study, "[Membrane-stabilizing copolymers confer marked protection to dystrophic skeletal muscle in vivo](#)," demonstrates for the first time a strong protective effect of Carmeseal-MD on dystrophic skeletal limb muscle. Beneficial effects are shown to be critically dependent on the route of delivery and are seen only after subcutaneous, but not intravenous or intraperitoneal administration. Previously, [Carmeseal-MD had been shown to improve the performance of dystrophic heart and diaphragm](#) damaged by the lack of dystrophin, a key structural membrane protein. Carmeseal-MD, a membrane stabilizer, is now the first disease modifying agent that has the potential to treat the three major aspects of DMD in all patients regardless of mutation: loss of ambulation and limb muscle strength as well as respiratory dysfunction and heart disease, the last two being the leading causes of death in these patients.

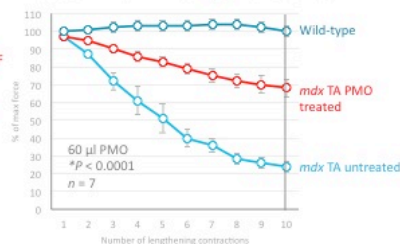
### P188 NF – impressive protection of skeletal muscle function in *mdx* mice even when compared to current exon-skipping therapy



Protection with P-188 NF



Protection with PMO skipper for exon 23



Notes

- Both studies done with *mdx* mouse hind limb
- P-188 NF data from Houang et al. Molecular Therapy — Methods & Clinical Development 2, Article number: 15042 (2015) doi: 10.1038/mtm.2015.42 (P-188 NF administered subq, not dose optimized, *in vivo*)
- PMO data from Sharp PS, Hema BJ and Wells DJ, 2011. Molecular Therapy vol. 19 no. 1, 165–171 (showing highest dose for exon skipping PMO, administered IM)

- more -

“The degree of protection enabled *mdx* skeletal muscle to function similarly to control mice, with the protection achieved comparable to transgenic animal studies using highly functional dystrophin molecules,” according to the [senior author, Dr. Joseph Metzger](#). This study brings the [number of peer-reviewed publications in support of use of P-188 NF in DMD to 17](#).

Phrixus is currently preparing for a Phase 2 trial of Carmeseal-MD in non-ambulatory patients with DMD with support from the NIH National Heart, Lung and Blood Institute.

Carmeseal-MD is already available in Europe as part of Phrixus’s European Access Program. Please contact Ethicor Pharma Ltd. at [enquiries@ethicorpharma.com](mailto:enquiries@ethicorpharma.com) or visit [www.ethicorpharma.com](http://www.ethicorpharma.com).

### ***About Carmeseal-MD™***

Carmeseal-MD (Poloxamer 188 NF or P-188 NF) is the first disease modifying agent in diseases characterized by membrane instability such as DMD, BMD, limb girdle muscular dystrophy and heart failure in the general population. In animal models of DMD and heart failure, it has been shown to improve the efficiency of damaged hearts. In animal models of DMD, it has been shown to improve the performance of damaged diaphragms and to protect skeletal limb muscle. When infused into the bloodstream, it encounters and binds to microscopic tears in the muscle membranes. This prevents the pathological leakage of calcium into the cells, which causes calcium overload and keeps the muscle from performing as required. Carmeseal-MD is the first agent that has the potential to treat the three major aspects of DMD: loss of ambulation and limb muscle strength as well as respiratory dysfunction and heart disease, the last two being the leading causes of death. It is expected to have its effect in all patients with DMD regardless of the genetic defect.

### ***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, most commonly through respiratory failure but now increasingly through heart 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](mailto:thomas.collet@phrixuspharmaceuticals.com) or [www.phrixuspharmaceuticals.com](http://www.phrixuspharmaceuticals.com) or [Phrixus on Facebook](#).

**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 as a result of a number of factors. 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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