

# MYOARETE

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| FOUNDED:         | December, 2020  |
| INDUSTRY:        | Pharma /Biotech |
| MARKET:          | Muscle Diseases |
| PRIOR INVESTORS: | Founder & UPENN |
| RAISING:         | \$5 Million USD |

**THE PROBLEM** Duchenne Muscular Dystrophy (DMD) is a devastating genetic disease in boys. Despite advances in medical management and therapeutic development, affected kids lose the ability to walk in their teens and die, typically of cardiac and respiratory failure before they turn 30. DMD occurs due to gene mutations resulting in the loss of the Dystrophin protein. A functionally near identical backup copy of the protein called Utrophin or Dystrophin-Related Protein exists, however, it is expressed at low levels that are insufficient to prevent disease formation. Recently approved DMD-specific treatments are promising, but since they are mutation-specific apply to only approx. 20% patients. Thus, there is a great unmet clinical need for therapies effective in all patients of DMD.

**THE MARKET** DMD affects 1 in 3500 boys and approximately 17,000 DMD patients live in the US. The market has shown strong growth since the FDA's approval of Eteplersen (Sarepta) as the first DMD-specific treatment in September 2016. This is exemplified by increases in the annual net sales at Sarepta from \$5.4M in 2016 to \$380.8M in 2019, based on drugs that are applicable to only ca. 20% patients. The global market size is estimated to be \$4.1 billion by 2023. There is currently no FDA approved DMD-specific treatment for the vast majority (80%) of DMD patients.

**OUR APPROACH** **MYOARETE** will develop and commercialize newly identified utrophin upregulation therapies that treat all DMD patients. Utrophin when upregulated, functionally substitutes for the missing dystrophin and has been validated in preclinical studies, but not as yet translated to patients. **MYOARETE** will leverage newly described platforms developed by the founder's laboratory at the University of Pennsylvania. that target post-transcriptional repression mechanisms to harness utrophin upregulation-based treatments. Utrophin upregulation treatments are predicted to improve function, slow disease progression and increase lifespan in all DMD patients, regardless of mutation status. UPENN has patents and patents filed on the intellectual property, which are being exclusively licensed to **MYOARETE**.

**COMPETITIVE ADVANTAGE** Unlike the current FDA-approved DMD therapy **MYOARETE'S** product is expected to benefit all DMD patients, regardless of mutation status. In addition, toxicity due to immune reactions or rejection is not expected with **MYOARETE'S** approach of increasing the expression of utrophin, a protein that is already present in muscle. Notably, no toxicities have been noted with utrophin upregulation in animal studies. The platform and products are protected by patents and PCT filings.

**FOUNDER** Prof. Tejvir S. Khurana is a Professor of Physiology at the Perelman School of Medicine & Pennsylvania Muscle Institute, University of Pennsylvania and is a leading authority in the field. He identified Dystrophin-Related protein or DRP (now renamed utrophin) as a graduate student at Harvard University. He has made ground-breaking discoveries in muscle biology including mechanisms of utrophin regulation and development of novel therapeutic strategies for muscle diseases based on grant support from numerous foundations including the National Institutes of Health (NIH) and the Muscular Dystrophy Association (MDA). His honors include membership of the American Society of Clinical Investigation (ASCI) and The Presidential Early Career Award for Scientists and Engineers (PECASE) award from the OSTP, The White House.

**FUNDING NEEDED** We are seeking \$5 million USD for conducting preclinical and IND-enabling studies.

**CONTACT** Founder: Tejvir S. Khurana, MD, PhD. tsk@penmedicine.upenn.edu  
Investor Relations: Neal Lemon, PhD, MBA nlemon@upenn.edu