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New Technologies Initiative: Gateway to Treating Genetic Diseases - Sarepta Therapeutics - Skipping Makes Sense - Restoring Muscle Function in Duchenne Muscular Dystrophy

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Abstracts

Approval of KYNAMRO (ISIS/Sanofi, HoFH), a first systemic Oligonucleotide Antisense (ASO) based therapy has raised expectations from the compounds in pipeline of other companies with similar technology platforms. Highly differentiated, 2nd Generation DNA/RNA-based drugs that work at the fundamental level of gene and protein expression have the potential to bring a paradigm change in treating diseases esp. rare genetic disorders like – Familial Hypercholesterolemia (FH), Duchenne Muscular Dystrophy (DMD), TTR (Transthyretin amyloidosis), SMN (spinal muscular atrophy) etc. Noteworthy, amongst them besides ISIS Pharma (ISIS) are Sarepta Therapeutics (SRPT), Prosensa (Pvt.), Regulus (RGLS), Alnylam (ALNY), Idera (IDRA), Santaris (Pvt.), Acuity pharma (merged with Froptix corp.), Arrowhead Research Corporation (ARWR), etc. SRPT's lead compound – Eteplirsen (formerly AVI-4658, PhII, based on proprietary exon-skipping technology, exon 51) targets specific mutations in DMD, repairs the mRNA and restores production of functional dystrophin protein = improved muscle functions. Duchenne Muscular Dystrophy (DMD) is a genetic (X-linked recessive) degenerative disease and Becker muscular dystrophy (BMD) is a milder form of DMD where the onset of the disease and disability is delayed.

In this report, we highlight the potential of the SRPT's Phosphorodiamidate Morpholino Oligomer (PMO) and Exon-Skipping technology platform. Key Points described in the report, are:

SRPT: Valuation and Key Milestones

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Eteplirsen – Description, Preclinical profile of Eteplirsen, and Clinical Data and Competitive Landscape in DMD

DMD – Disease and Market opportunity

IP Status

Contracts or Collaborations

Technology Platforms – PMO and Exon-skipping technology platform

Overview, evolution of Antisense technology platforms

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