[352] MRNA THERAPEUTICS FOR THE TREATMENT OF RARE DISEASES

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ART BioScience develops mRNA-based drug for treating Duchene Muscular Dystrophy. By 2019 Duchene market is about to reach an estimated $1 billion—because of the novel mutation-specific drugs that are being developed. However, a large segment of DMD patients will be ineligible for these promising drugs, leaving vast opportunity for delivering new therapies.

The potential prospects are small-mid-big pharma companies that focus in orphan diseases and/or gene/mRNA therapy. The expected business model is either co-development deal, including upfront/milestones/royalties payments and/or commercialization deal that ART BioScience will provide product ready for sell and will be able to gain greater value. The decision for these and other options will be made in a later phase.

Synthetic mRNA, encoding human dystrophin is estimated to reach the length of about 11,000, (full-length) or 5,000-10,000 nucleotides (truncated). Delivered to the muscle cells, it will lead to production of functional protein, providing universal treatment, regardless of the patient's genetic background (mutation's nature and location), opening the whole new area of therapeutics.

ART BioScience designed and produced a number of truncated variants of DMD mRNA. Delivered into the muscle by local injection, the RNA leads to the production of dystrophin. This results in reduced muscular damage in model animals. We are working on developing more efficient and stable mRNAs, and improved local and systemic delivery formulations.

To date, the founders have privately invested in the company. Recently, our program has been accepted by the Israel Innovation Authority in the framework of the "Program for Emerging Companies". The company is looking to raise $7.0 M in 3 milestone-driven tranches to aggressively progress our lead candidate to be administered locally via intramuscular injection in Proof of Concept clinical trial.