Dystrogen Therapeutics *(Chicago, IL)*

Overview

- Clinical-stage **Chimeric Cell** company using its **novel and proprietary platform** to develop therapeutics to treat rare diseases
- Chimeric cell therapy represents a unique and **novel modality** with **broad applications** across multiple therapeutic categories which could **revolutionize the treatment paradigm** for broad spectrum of genetic disorders
  - **Engineered Cell Technology** – for Duchenne muscular dystrophy (DMD), a **rare, progressive disease without a cure** (caused by dystrophin gene mutations) - the therapy is created by "fusing" myoblasts of normal donor origin (allogenic) with myoblasts of the DMD-affected patient (autologous), to create a new generation of dystrophin expressing chimeric (DEC) cells which contain the phenotype of the normal donor while preserving the surface markers of the DMD recipient. DEC cells express significantly higher levels of CD56 and dystrophin when compared to DMD affected myoblasts, thus more closely resembling healthy myoblasts. Because they are chimeras, DEC cells display the recipients' immune phenotype, thus does not require immunosuppression to support engraftment
  - **Acts like a Trojan horse –** once injected**,** DEC cells are recognized by the recipient as "self" and evade the patient's immune system (are not rejected), and thus engraft, producing the relevant and missing protein from a full-length, healthy gene (delivered by the normal donor)
- **Lead program**, DT-DEC01, targeting **DMD**
  - Currently in **Phase 1 Pilot human study** following **robust safety and systemic (cardiac, pulmonary, skeletal muscle) efficacy maintained up to 180 days** in preclinical studies
  - First patient dosed with preliminary safety results expected in January 2022

Pipeline

Program	Indications	Phase
DT-DEC01	DMD	Phase 1
DT-200	Neuro-Degenerative	Discovery
DT-201	Sarcopenia	Discovery
DT-202	Graft-vs-Host Disease	Discovery

Phase 1 DMD Trial Overview

- DMD Affects 20,000 US males and 300,000 worldwide
- 10 patient, 3 arm dose escalation study based on patient weight
- Primary endpoints: safety
  - First 6 months active follow-up / 18-months passive follow-up
- Secondary endpoints: efficacy related, mean change of baseline of:
  - NorthStar Ambulatory Assessment (NSAA), range of motion (ROM), performance of upper limb (PUL), 6MWD at month 1, 3, 6,12,18 and 24
  - Muscle strength by myometry and 5-grade Lovett scale at month 1, 3, 6,12, 18 and 24. Cardiac muscle evaluation by ECHO
  - Laboratory parameters: hematological, biochemical (including: CK, CKMB), liver function panel, and other standard tests

Distinct Advantages over Current DMD Therapeutics

- **Universal therapy** for all DMD genetic mutations
- Produces the **dystrophin protein without genetic manipulation , unlike microdystrophin the viral vector modified** therapy approach targeting DMD
- **Does not modify genes;** no related toxicity or off-target mutations and no viral vector integration issues
- **Does not require pretreatment or conditioning**
- **Does not require immunosuppression to support engraftment**
- **Redosing possible without sensitization** and **no immunosuppression** required
- Can be used as a **monotherapy or in combination** with other therapeutics
- The Company has **patent protection through 2036**

Leadership Team

Maria Siemionow
Founder and CSO

- Performed the first US human face transplant at the Cleveland Clinic
- Professor of Orthopaedics at the University of Illinois at Chicago President of the American Society for Reconstructive Transplantation and numerous leadership roles at other transplantation and regenerative medicine organizations

Kris Siemionow
Co-Founder and CEO

- Chief of Spine Surgery and Associate Professor of Orthopaedics and Neurosurgery at the University of Illinois in Chicago
- Chief Medical Officer of Surgalign Holdings (NASDAQ)
- Co-founder Holosurgical Inc: acquired by Surgalign Holdings

Paul Lewicki
Co-Founder and Board Member

- Board Member of Surgalign Holdings
- Founder and CEO of StatSoft (acquired by Dell in 2014)
  - StatSoft had 30 overseas offices in all major markets and over 1 M B2B users across various industries

Source: Company filings and FactSet. Market data as of 10/05/2021.
(1) Also serves on the Board of Directors.