Rare Disease Research:
Providing Exceptional Solutions for Clinical Trials in Rare Diseases

Han C. Phan, MD
Director of Clinical Research
Eduardo Sanabria-Figueroa, PhD
Clinical Trials Liaison

Rare Disease Research, LLC
1891 Howell Mill Road NW, Ste B
Atlanta, GA 30318

Website: www.RareDiseaseResearch.com
Phone: 678 - 883 - 6897
Email: info@RareDiseaseResearch.com
Who are we?

- **Rare Disease Research** (RDR) specializes in clinical trials for patients with neurogenetic disorders
  - Duchenne Muscular Dystrophy, Spinal Muscular Atrophy, Limb-Girdle Muscular Dystrophy, Myasthenia Gravis, Niemann Pick Type C, Epilepsy, Cerebral Palsy, Metachromatic Leukodystrophy, and more

- **Our Mission**
  - We are an independent company that strives to provide access to investigational treatments for pediatric and adult patients with rare conditions that otherwise would not have the opportunity to participate in cutting-edge clinical research efforts

- **Our Vision**
  - Cultivating trustworthy relationships with our collaborators in order to facilitate access to investigational therapies for ALL our patients with Rare Diseases

- As an independent research center, we are able to work with any sponsor, indication, or study.
  - Even studies for a single patient!!!
Clinical Trials for DMD

- **EPIDYS**
  - Givinostat, two oral doses per day, ≥ 6 y/o, ambulant
- **Fibrogen**
  - Pamrevlumab (mAb), IV infusion every two weeks, ≥ 12 y/o, non-ambulant
- **MIS51ON**
  - Exondys 51 (Eteplirsen), weekly IV infusion, ages 7 to 13 y/o, ambulant
- **Investigator-Initiated Study**
  - Vyondys 53 (Golodirsen), IV infusion, ≥ 7 y/o, non-ambulant
- **Gene therapy study (Pfizer)**
  - AAV9/mini-dystrophin, single dose, ≥ 4 to < 8 y/o, ambulant
- **Future Gene Therapy studies, including CRISPR-based therapies**

We believe everyone deserves access to clinical trials!!!