Rare Disease Research:

Providing Exceptional Solutions for Clinical Trials in Rare Diseases

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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!!!