RARE DISEASES: NEUROMUSCULAR CONDITIONS

GLOBAL, PHASE I-IV EXPERIENCE

- Charcot-Marie-Tooth Disease
- Duchenne Muscular Dystrophy
- Facioscapulohumeral Muscular Dystrophy
- Familial Amyloidosis
- Polyneuropathy

- Mucopolysaccharidosis
- Myasthenia Gravis
- Pompe Disease
- Spinal Muscular Atrophy



IMAGING CORE LAB

Capabilities Include Advanced Quantitative Magnetic Resonance Imaging Measures For:

- Muscle Fat Fraction (MRI-PDFF, MRS)
- Elastography
- Muscle cross-sectional area or volume
- Anatomic and functional brain mapping

DIFFERENTIATORS & BENEFITS

- In-house neurologists, child neurologists, regulatory experts, imaging experts, and clinical operations professionals that are skilled in neuromuscular disease studies
- Partnerships with endpoint training and oversight groups (including for physical function assessments) and in-house data management programming to improve trend oversight
- Specialized project teams that can leverage site relationships and utilize novel endpoints such as actigraphy, home pulmonary function testing, physical function testing, and imaging
- Experience in complex drug delivery, such as intrathecal/ intraparenchymal dose administration and CSF collection processes
- Extensive expertise in gene editing/gene transfer technology, and other advanced therapies
- Global central labs with safety and biomarker analysis to support neuromuscular disease studies

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MAKING THE COMPLEX SEAMLESS®



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