

RARE DISEASES: NEUROMUCULAR CONDITIONS

A COMPETITIVE EDGE FOR CLINICAL DEVELOPMENT



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

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

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

MAKING THE COMPLEX
SEAMLESS[™]



M E D **P A C E**