

Seeking Novel Solutions to Treat Rare Genetic Skeletal Muscle Diseases

Our client, a global leader in the creation and commercialization of pioneering therapies for rare genetic diseases, is looking for novel approaches to target the primary cause of rare genetic skeletal muscle diseases, to transform the way patients feel, function, and survive.

In particular, the team are focused on Muscular Dystrophy (Duchenne, Becker, Emery-Dreifus, Ullrich, Oculopharyngeal or Facioscapulohumeral) Myotonic Dystrophy Type 1 and 2, Pompe Disease, NemaLine Myopathy, LAMA2 / Congenital Muscular Dystrophy Type 1, X-linked Centronuclear Myopathy, Limb-Girdle Muscular Dystrophy, and RYR1-related myopathies.

The team is interested in a range of approaches including

