CHET Outcomes
CENTER FOR HEALTH + TECHNOLOGY

UNIVERSITY of ROCHESTER

5 PIVOTAL STUDIES

Our disease-specific instruments were shown to be more responsive in detecting clinically-relevant changes compared to traditional functional measures

MDHI — Myotonic Dystrophy Type 1

Dyne therapeutics announces positive initial clinical data from ACHIEVE trial in DM1 patients and DELIVER trial in DMD patients demonstrating promise of the FORCE™ platform in developing therapeutics for rare muscle diseases

From their press release:

"Improvement in Myotonia (vHOT) as well as Fatigue (MDHI) Observed in Lowest Dose ACHIEVE Cohort at 6 Months"

Dyne™ THERAPEUTICS

HD-HI — Huntington's Disease

Neurocrine Biosciences Announces FDA Approval of INGREZZA® (valbenazine) Capsules for the Treatment of Chorea Associated With Huntington's Disease

The Huntington's Disease-Health Index (HD-HI): Measuring Changes in Disease Burden in Response to Valbenazine During the KINECT-HD Trial

NEUROCRINE® BIOSCIENCES
You Deserve Brave Science®

Safety and efficacy of valbenazine for the treatment of chorea associated with Huntington's disease (KINECT-HD): a phase 3, randomised, double-blind, placebo-controlled trial

Prof Erin Furr Stimming MD a, Daniel O Claassen MD b, Elise Kayson MS c, Jody Goldstein BS c, Raja Mehanna MD a, Hui Zhang PhD d, Grace S Liang MD d, Dietrich Haubenberger MD d

SMA-HI — Spinal Muscular Atrophy

Was used in a current clinical study looking at *Spinraza* efficacy in Adults with Spinal Muscular Atrophy

SPINRAZA® (nusinersen) injection 12 mg/5 mL

The SMA-HI showed a 5 point improvement in total disease burden over 14 months

Six month results of spinraza in adults with spinal muscular atrophy (SAS) study

Zaidman CM, Proud CM, Thonhoff JR, et al. Neurology. 2021;96(15):2446.

UNIVERSITY of WASHINGTON

CMT-HI — Charcot Marie Tooth

Applied Therapeutics Announces Positive Results from a 12-month Interim Analysis of Govorestat (AT-007) in the Ongoing INSPIRE Phase 3 Trial in Sorbitol Dehydrogenase (SORD) Deficiency

From their press release:

"Highly statistically significant effects on the CMT Health Index (CMT-HI) patient reported outcome measure (p=0.01), with benefit of govorestat on categories of lower limb function, mobility, fatigue, pain, sensory function, and upper limb function"

APPLIED THERAPEUTICS

FSHD-HI — Facioscapulohumeral Muscular Dystrophy

NIH STARFiSH: Study of Testosterone and rHGH in Facioscapulohumeral Muscular Dystrophy: Planning Infrastructure Execution and Readiness

STARFiSH PIER

- Primary outcome: safety and toreability
- Secondary outcomes: strength, function, muscle mass, disease burden (FSHD-HI)

Results from a 36-Week Open-Label Study of Recombinant Human Growth Hormone and Testosterone in Facioscapulohumeral Muscular Dystrophy (FSHD)

C. Heatwole, E. Leubbe, J. Hamel, P. Mongiovi, E. Ciafaloni, N. Dilek, W. Martens, D. Weber, R. Hani, J. Allen, C. Smith, S. Howell, S. Rosero, K. Eichinger, L. Baker, J. Dekdebrun, J. Hilbert, A. Varma, M. McDermott, C. Thornton, R. Moxley. American Academy of Neurology (AAN) Annual Meeting. Boston, MA. 4/22/23

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Advantages of disease-specific PROs

- Higher relevance and content validity for target population
- Lower burden to patients and caregivers
- Strong correlation to functional capabilities
- Ability to lower sample size requirements
- Ability to emphasize relevant symptomatic themes of a specific population while excluding non-relevant issues
- Increased sensitivity to detect small, but clinically relevant changes in disease-specific health
- Better suited to measure disease progression or therapeutic gain over time