Medicinsk behandling til patienter med FSHD og Limb-girdle Muskel Dystrofi

Opdatering på igangværende medicinske projekter i Danmark

Møde i Dansk konsortium for neuromuskulære sygdomme

Mandag den 4. september 2023, Musholm, Korsør

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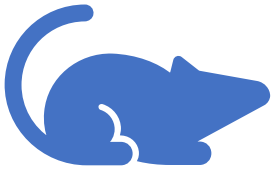
Facio-Scapulo-Humeral Muskeldystrofi (FSHD)

Losmapimod (Fulcrum Therapeutics)

Phase 3



RO7204239 (Hoffmann-La Roche)

Phase 2

Sponsor: Fulcrum Therapeutics
Navn: REACH
Protokol: 1821-FSH-301

Et fase 3, globalt, randomiseret, dobbeltblindet, placebokontrolleret, 48-ugers, parallelgruppeforsøg af effektiviteten og sikkerheden af losmapimod til behandling af patienter med facioscapulohumeralmuskeldystrofi (REACH)

Losmapimod selectively inhibits enzymes p38α/β mitogen-activated protein kinases (MAPKs), which are modulators of DUX4 expression and mediators of inflammation.

Effekt: Reduktion af DUX4 expression

losmapimod 15 milligrams (mg) or placebo orally twice daily (Tablet)

Sponsor: Hoffman La Roche
Navn: Manouvre
Protokol: BN43703

Et Multicenter, Randomiseret, Placebokontrolleret, Dobbelblindet Fase II-forsøg med henblik på at vurdere Farmakodynamikken, Sikkerheden, Tolerabiliteten, Farmakokinetiken og effekten af RO7204239 hos deltagere med Facioscapulohumeral Muskeldystrofi

RO7204239, a humanized monoclonal antibody that binds to human latent myostatin

Effekt i form af øget muskelvækst

Sub-kutan injection 1 x om måneden

Limb-Girdle type R9/2I Fukutin Related Protein

ATA001 (Atamyo Terapeutics)

- Phase 1/2

BBP-418 (ML Bio Solutions)

- Phase 3

EDG-5506 (Edgewise Therapeutics)

- Phase 2

Sponsor: Atamyo Therapeutics
Navn: ATA-001-FKRP
Protokol: ATA-001-FKRP

Et fase 1-2 multicenterforsøg (2 faser), der har til formål at evaluere sikkerheden og effekten af intravenøs GNT0006, en adeno-associeret viral vektor med FKRP-genet hos patienter med FKRP-relateret limb-girdle muskeldystrofi (LGMD R9, tidligere LGMD2I)

Genterapi med en AAV9 vector

Effekt – genoprette glycosylering af alfa-dystroglycan

Sponsor: ML Bio Solutions
Navn: Fortify
Protokol: MLB-01-005

A Phase 3 Randomized, Placebo-controlled, Double-blind Study to Evaluate the Efficacy and Safety of BBP-418 (ribitol) in Patients with Limb Girdle Muscular Dystrophy 2I (LGMD2I)

BBP-418 is designed to supply supraphysiological levels of an endogenous substrate upstream of the mutant FKRP enzyme to help drive residual activity of the enzyme to glycosylate αDG

(Afventer endelig protokol godkendelse i EU)

Pulver der indtages dagligt

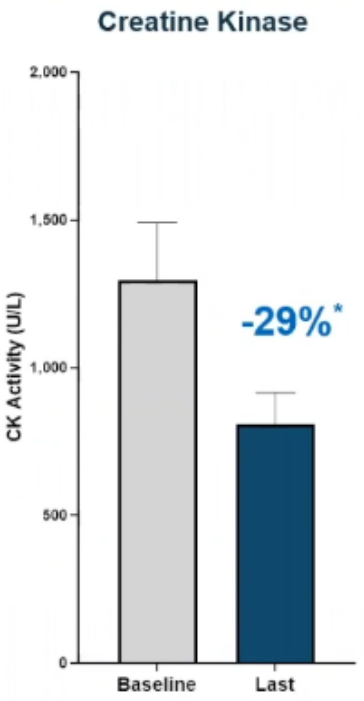
Sponsor: Edgewise Therapeutics
Navn: EDG-5506
Protokol: EDG-5506

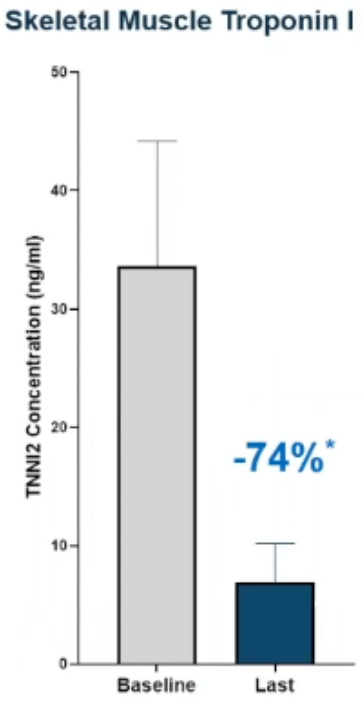
Både til Becker Muskel Dystrofi og LGMDR9 Fukutin Related Protein

EDG-5506, reduces stress by selectively targeting the muscle motor protein myosin that regulates contraction of fast skeletal muscle fibers.

The tissue specificity of EDG-5506 means that the function of other muscles such as slow skeletal, smooth and cardiac muscles are not affected. Allowing fast fibers to contract without injury should preserve muscle health in Duchenne and Becker and potentially enhance physical function.

4 months treatment of BMD





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Vigtige studier:

Natural history studier – GNT og MLB