EDG-5506: A Novel Approach to Protect Muscle in Duchenne and Becker Muscular Dystrophy

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What does dystrophin do in muscle?

Dystrophin Protects Muscles from Stress During Contraction Rather than Powering Contraction

The primary role of dystrophin is to protect muscles from damage and not to directly influence muscle strength. When dystrophin is lacking, the mechanical stress that occurs during muscle contraction damages the muscle, leading to muscle weakness, fibrosis, and ultimately, loss of function.

EDG-5506 Protects Dystrophic Mouse Muscle

Dystrophin is severely reduced in mouse muscles treated with EDG-5506, indicating it protects muscles from stress during contraction.

Injured Muscles Release Muscle Protein Biomarkers

EDG-5506 Phase 1 Trial in Unaffected Adults and Those with Becker Muscular Dystrophy

What do we know about side effects?

- When dosed for two weeks, EDG-5506 was well-tolerated

How is EDG-5506 given?

- EDG-5506 is well absorbed and can be taken once a day with or without food

Does it get to the muscle?

- EDG-5506 is highly concentrated in muscle compared to the bloodstream, which tells us that it is getting to where it needs to be to protect the muscle

EDG-5506 is Being Developed for Becker and Duchenne Muscular Dystrophy

- Taken orally, intended to preserve and improve function in Becker and Duchenne patients with any mutation
- Goal to prevent damage to muscle by protecting the most susceptible fast muscle fibers
- Potential to be used alone or in combination with other therapeutic approaches for dystrophinopathies
- Designed to stop the damage where it begins

EDG-5506 Clinical Trials Ongoing and in Planning

- A 12-month open-label study is ongoing, including adults with Becker who were in the Phase 1 Trial (NCT01964118)
- A natural history study of those with Becker has begun (ages 8 to 65) (NCT02574743)
- A study in ambulatory adolescent and adult males with Becker is planned to start in the first half of 2022 (NCT05021015)
- An initial study in ambulatory boys with Duchenne is planned to start in the second half of 2022

Phase 2 Study in Adults and Adolescents with Becker

- Anticipated to be starting soon
- Population:
  - Age 12 to 65 years old, inclusive
  - Confirmed mutation in dystrophin gene with characteristic Becker phenotype
- Ambulatory:
  - On stable dose of corticosteroids
- Design: 6-month placebo-controlled
- Endpoints:
  - Safety
  - Biomarker (CK) at 12 months
  - MRI 1st fraction of upper leg
- Functional assessments to include NSAA, NSAD

Initial Phase 2 Study in Boys with Duchenne

- Planned to start in second half of 2022
- Population:
  - Age 4 to 9 years old, inclusive
  - Confirmed mutation in dystrophin gene with characteristic phenotype
  - Ambulatory:
    - On stable dose of corticosteroids
  - Design: 3-month placebo-controlled, followed by 9-month open-label
- Endpoints:
  - Safety, pharmacokinetics and biomarkers at 3 months
  - Functional assessments collected for longer term information

Disclaimer

EDG-5506 is an investigational drug that is not approved in any territory.

The authors’ employees, consultants for Edgewise Therapeutics and may hold stock and/or stock options.

Thank you to the patients and their families!