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

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Disclaimer
EDG-5506 is an investigational drug that is not approved in any territory. The authors are employees or consultants for Edgewise Therapeutics and may hold stock and/or stock options.

The Edgewise Approach: Protect susceptible muscle fibers

- Some muscle fibers are more susceptible to damage due to the lack of functional dystrophin.
- We've made an investigational therapy, EDG-5506, that is designed to protect these susceptible muscle fibers from damage, regardless of mutation.
- In diseased animal models, EDG-5506 protected susceptible muscle fibers and prevented long-term development of damage.

Injured Muscles Release Muscle Protein Biomarkers

- Multiple muscle proteins enter the bloodstream and can be measured, which we term biomarkers of muscle damage.

BMD and Duchenne Muscular Dystrophy (DMD) Represent a Continuum of the Same Disease; Edgewise’s Approach Aims to Treat Across the Disease Spectrum, Regardless of Dystrophin Mutation

Endpoints:
- CK Activity (U/L) Decreased after 6 Months of Treatment with EDG-5506
- TNNI2 Concentration (ng/ml) with dystrophin fibers support each other
- Dystrophin Floor (µM)
- BMD and DMD Represent a Continuum of the Same Disease, Edgewise’s Approach Aims to Treat Across the Disease Spectrum, Regardless of Dystrophin Mutation

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

Phase 2 Study in Adults and Adolescents with Becker Population:
- Age 12 to 50 years old, inclusive
- Confirmed mutation in dystrophin gene with characteristic Becker phenotype
- Ambulatory
- Not on corticosteroids

Design:
- 12-month placebo-controlled

Endpoints:
- Biomarker (CK) at 12 months
- Safety
- MRI fat fraction of upper leg
- Functional assessments to include NSAA, NSAD

EDG-5506: 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

At Edgewise, patients are at the core of everything we do.