EDG-5506 Targets Fast Skeletal Myosin to Protect Dystrophic Muscle and Reduce Muscle Damage Biomarkers in a Phase 1 Trial in Becker Muscular Dystrophy

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Participants in the BMD Phase 1b Had Significant Baseline Functional Impairment

EDG-5506 was Well-Tolerated in BMD Subjects

EDG-5506 Concentrates in Dystrophic Muscle Above Levels Predicted to Provide Meaningful Clinical Benefit

Key Biomarkers of Muscle Damage Significantly Decreased with EDG-5506

Robust, Significant and Time-Dependent Decreases in Elevated BMD Biomarkers

With SOMAscan CK and Fast Troponin Reduced to Levels Near Those Observed in Healthy Following Treatment with EDG-5506

Consistent and Progressive EDG-5506 Effect on Exercise Responsive Markers

EDG-5506: Well-Tolerated with Decreases in Biomarkers of Muscle Damage in BMD Subjects

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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.

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EDG-5506 was generally well tolerated with no serious adverse events observed. Most common AEs were somnolence and diarrhoea, which were generally mild and transient

Plasma CK showed good oral absorption with or without food, and an extended half-life, consistent with extensive distribution to muscle as was observed preclinically

EDG-5506 muscle concentrations well above anticipated efficacious levels

Participants were monitored as necessary for 9 days, with increased exposure up to 14 days after completion of dosing

SOMAcan shows enhanced protein profiling of elevated plasma markers in BMD

Baseline plasma samples (n=17) were compared to baseline samples taken from healthy volunteers (n=17)

Protein levels identified a fingerprint of elevated proteins in BMD

Most significant proteins are from muscular and metabolic pathways consistent with muscle injury