



News Release

Edgewise-Funded Natural History Trial of Becker Muscular Dystrophy (BMD) Now Enrolling

- Trial to inform the disease progression of individuals with BMD -

Boulder, Colo., (April 14, 2022) – Edgewise Therapeutics, Inc., (NASDAQ: EWTX), a clinical-stage biopharmaceutical company focused on developing orally bioavailable, small molecule therapies for the treatment of rare muscle disorders, today announced the initiation of an Edgewise-funded observational trial in individuals with BMD. The trial is designed to understand the disease progression of individuals with BMD as assessed by functional measures and imaging endpoints. This global, multi-center trial is led by the GRASP (General Resolution and Assessments Solving Phenotypes) consortium and Virginia Commonwealth University (VCU), in collaboration with ImagingDMD University of Florida (UF).

The trial is expected to recruit approximately 150 individuals with BMD to collect information over a two-year period that may provide a more comprehensive understanding of the disease and help advance potential future therapies, like Edgewise’s lead clinical candidate, EDG-5506. This observational study ([NCT05257473](#)) will enroll individuals, ages 8 and older, at multiple locations across the United States and Europe. As a natural history study, it will not test any investigational drugs, but rather monitor participants over time to examine their disease course.

“The severity and progression of symptoms common in BMD, like progressive muscle weakness, vary from person to person and have posed a challenge in clinical trial design,” said Joanne Donovan, Ph.D., M.D., Chief Medical Officer, Edgewise Therapeutics. “With this study, we hope to evolve assessments of disease progression as we advance our lead clinical candidate, EDG-5506, into Phase 2 clinical trials for adolescents and adults with BMD.”

About Becker Muscular Dystrophy

BMD is a serious, progressively debilitating, and potentially fatal inherited X-linked neuromuscular disorder. BMD results from mutation of the dystrophin gene yielding unstable and/or dysfunctional dystrophin expression in muscles. Individuals with BMD, typically males, have ongoing muscle fiber (myofiber) degeneration that eventually leads to fibrosis, progressive loss of skeletal muscle function, and that can lead to severe disability and early death. BMD typically presents with juvenile onset of muscle wasting and progressive symmetrical, proximal muscle weakness, calf hypertrophy, activity-induced muscle cramping and elevated creatine kinase activity. While the course of BMD is variable, it is unidirectional in terms of the inevitable progressive limb weakness resulting in severe disability. BMD is also associated with early mortality from cardiac disease. The incidence of BMD is approximately 1 in every 18,450 live male births. It is estimated that there are between 4,000 to 5,000 individuals with BMD in the U.S., with similar numbers of individuals living with BMD in Europe. Despite

the seriousness of the disease, for many with BMD, the disease remains one of considerable unmet medical need as there are no approved therapies in the U.S.

About EDG-5506 for DMD and BMD

EDG-5506 is an orally administered small molecule designed to address muscle damage induced by mechanical stress in dystrophinopathies including DMD and BMD. EDG-5506 presents a novel mechanism of action to selectively limit the exaggerated muscle damage caused by the absence of functional dystrophin. EDG-5506 has the potential to benefit a broad range of patients suffering from debilitating rare neuromuscular disorders. It is anticipated to be used as a single agent therapy, but it may also provide a synergistic or additive effect in combination with available therapies and therapies currently in development. In August 2021, the U.S. Food and Drug Administration granted Fast Track designation to EDG-5506 for the treatment of individuals with BMD.

The Company has completed a Phase 1 clinical trial of EDG-5506 designed to evaluate safety, tolerability, PK and PD of EDG-5506 in adult healthy volunteers (Phase 1a) and in adults with BMD (Phase 1b). Go to clinicaltrials.gov to learn more about this clinical trial ([NCT04585464](https://clinicaltrials.gov/ct2/show/study/NCT04585464)). A follow-on open-label, single-center study is assessing the long-term safety and PK of EDG-5506 in adults with BMD ([NCT05160415](https://clinicaltrials.gov/ct2/show/study/NCT05160415)). We anticipate initiation of Phase 2 trials in individuals with BMD in the first half of 2022 and DMD in the second half of 2022.

About Edgewise Therapeutics

Edgewise Therapeutics is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of innovative treatments for severe, rare neuromuscular and cardiac disorders for which there is significant unmet medical need. Guided by its holistic drug discovery approach to targeting the muscle as an organ, Edgewise has combined its foundational expertise in muscle biology and small molecule engineering to build its proprietary, muscle-focused drug discovery platform. Edgewise's platform utilizes custom-built high throughput and translatable systems that measure integrated muscle function in whole organ extracts to identify small molecule precision medicines regulating key proteins in muscle tissue. To learn more, go to: www.edgewisetx.com or follow us on [LinkedIn](#), [Twitter](#) and [Facebook](#).

About VCU and VCU Health

Virginia Commonwealth University is a major, urban public research university with national and international rankings in sponsored research. Located in downtown Richmond, VCU enrolls nearly 30,000 students in 238 degree and certificate programs in the arts, sciences and humanities. Twenty-three of the programs are unique in Virginia, many of them crossing the disciplines of VCU's 11 schools and three colleges. The VCU Health brand represents the VCU health sciences academic programs, the VCU Massey Cancer Center and the VCU Health System, which comprises VCU Medical Center (the only academic medical center in the region), Community Memorial Hospital, Tappahannock Hospital, Children's Hospital of Richmond at VCU, and MCV Physicians. The clinical enterprise includes a collaboration with Sheltering Arms Institute for physical rehabilitation services. For more, please visit vcu.edu and vcuhealth.org.

About ImagingDMD UF

ImagingDMD UF was founded as a joint research effort to investigate the use of magnetic resonance imaging (MRI) as a non-invasive biomarker to monitor disease progression in a natural history study of Duchenne muscular dystrophy. Led by a diverse team of experts and based on 12 years of data collection, ImagingDMD UF has developed advanced, standardized MR imaging acquisition and analysis techniques to assess skeletal muscle pathology and overall muscle composition. Applying this valuable expertise, ImagingDMD UF works closely with research groups and pharmaceutical companies to facilitate national and international clinical trials for diverse muscular dystrophies. ImagingDMD UF is currently in the process of having MRI validated by the FDA as an accepted quantitative endpoint in clinical trials of neuromuscular diseases. With a worldwide

network of certified imaging sites with specific neuromuscular imaging expertise, and advanced infrastructure for data transfer, handling and processing, ImagingDMD UF has the ability to provide MRI services for large multi-center trials from study start through closeout in compliance with national and international regulatory agencies. This includes assisting with protocol development, determining study feasibility, providing comprehensive study management of all MR-related study activities including centralized data management and quality assurance, and to deliver precise, reliable MRI outcome measures.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as that term is defined in Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Statements in this press release that are not purely historical are forward-looking statements. Such forward-looking statements include, among other things, statements regarding the potential of, and expectations regarding, Edgewise's drug discovery platform, product candidates and programs, including EDG-5506; statements regarding enrollment of individuals in the BMD natural history trial; statements regarding Edgewise's expectations relating to the results from its preclinical studies, its clinical trials and the BMD natural history trial; statements about the expected timing of Edgewise's initiation of its preclinical studies and clinical trials including its Phase 2 clinical trials of EDG-5506 in BMD and DMD; and statements regarding Edgewise's pipeline of product candidates and programs. Words such as "believes," "anticipates," "plans," "expects," "intends," "will," "goal," "potential" and similar expressions are intended to identify forward-looking statements. The forward-looking statements contained herein are based upon Edgewise's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those projected in any forward-looking statements due to numerous risks and uncertainties, including but not limited to: risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics and operating as an early clinical stage company including the potential for Edgewise's product candidates to cause serious adverse events; Edgewise's ability to develop, initiate or complete preclinical studies and clinical trials for, obtain approvals for and commercialize any of its product candidates for muscular dystrophy patients or other patient populations; the timing, progress and results of preclinical studies and clinical trials for EDG-5506 ; Edgewise's ability to raise any additional funding it will need to continue to pursue its business and product development plans; negative impacts of the COVID-19 pandemic on Edgewise's operations, including preclinical and clinical trials; the timing, scope and likelihood of regulatory filings and approvals; the potential for any clinical trial results to differ from preclinical, interim, preliminary, topline or expected results; Edgewise's ability to develop a proprietary drug discovery platform to build a pipeline of product candidates; Edgewise's ability to enroll and maintain patients in its preclinical studies and its clinical trials; the enrollment of individuals in the BMD natural history trial; Edgewise's manufacturing, commercialization and marketing capabilities and strategy; the size of the market opportunity for Edgewise's product candidates; the loss of key scientific or management personnel; competition in the industry in which Edgewise operates; Edgewise's reliance on third parties; Edgewise's ability to obtain and maintain intellectual property protection for its product candidates; general economic and market conditions; and other risks. Information regarding the foregoing and additional risks may be found in the section entitled "Risk Factors" in documents that Edgewise files from time to time with the Securities and Exchange Commission (the "SEC"). These forward-looking statements are made as of the date of this press release, and Edgewise assumes no obligation to update the forward-looking statements, or to update the reasons why actual results could differ from those projected in the forward-looking statements, except as required by law.

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