



News Release

Edgewise Therapeutics to Report Phase 1b Topline Results of EDG-5506 in Individuals with Becker Muscular Dystrophy (BMD) on January 5, 2022, and Initiates ARCH Follow-On Open Label BMD Study

- *Webcast event to feature EDG-5506 topline results presentation and commentary by a leading neuromuscular disease expert –*
- *Management to provide 2022 Company outlook at the 40th Annual J.P. Morgan Healthcare Conference on January 10, 2022 –*

Boulder, Colo., (December 15, 2021) – 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 plans to report topline results from the Phase 1b clinical trial of EDG-5506 in individuals with BMD on January 5, 2022, at 9:00 am ET. The webcast event will feature a presentation of the results which support advancement of the program, and commentary by a leading neuromuscular disease expert. Additionally, the Company will provide details on the recently initiated follow-on open label study, EDG-5506-002 (ARCH).

“We look forward to sharing the Phase 1b BMD topline results of EDG-5506 during our webcast,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Edgewise. “We are also excited to initiate the ARCH study, which will enroll participants from our Phase 1b clinical trial as well as new participants with BMD.”

Further, Edgewise plans to share these findings and the Company’s 2022 outlook at the 40th Annual J.P. Morgan Healthcare conference on January 10, 2022.

Phase 1b BMD Topline Results Conference Call

Edgewise’s management will hold a conference call and webcast on **January 5, 2022, at 9:00 am ET** to discuss topline results from the Phase 1b clinical trial in individuals with BMD. To participate, please dial **844-200-6205** (domestic) or **929-526-1599** (international) and refer to access code **078871**. Visit the Edgewise [events page](#) to access the webcast, including replay and conference call slides.

The Phase 1b clinical trial enrolled 7 participants and assessed a 20 mg dose of EDG-5506 (n=5) or placebo (n=2) for 14 days. The clinical trial was designed to evaluate safety, pharmacokinetics (PK) and changes in biomarkers of muscle damage, such as creatine kinase and fast troponin I as well as biomarkers evaluated using SOMAscan, in adult males with BMD. Based on the clinical results to date, the Company expects to initiate Phase 2 clinical trials in individuals with BMD in the first half of 2022 and Duchenne muscular dystrophy (DMD) in the second half of 2022.

About the ARCH Open Label Study

ARCH will evaluate EDG-5506 in adult males with BMD. Participants will include those who completed the Phase 1b first-in-human study of EDG-5506, as well as new participants. The study will assess a

10 mg dose of EDG-5506 administered daily over 3 months. Safety, PK and changes in biomarkers of muscle damage such as creatine kinase and fast troponin I will be evaluated.

Edgewise Therapeutics Presentation at J.P. Morgan Annual Healthcare Conference

Edgewise Therapeutics' President and Chief Executive Officer, Kevin Koch, Ph.D., will present at the 40th Annual J.P. Morgan Healthcare Conference on January 10, 2022, at 5:15 pm ET / 2:15 pm PT. The presentation will be webcast live; a link for the webcast can be found on the Edgewise [events page](#) and will be accessible for replay following the presentation. It is recommended that users connect to the webcast several minutes prior to the start to ensure a timely connection.

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 4,000–5,000 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 the root cause of 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.

EDG-5506 has been studied in a Phase 1 clinical trial designed to evaluate safety, tolerability, PK and pharmacodynamics (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)).

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](#).

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 about the expected timing of Edgewise's initiation of Phase 2 clinical trials in individuals with BMD and DMD; timing for reporting topline results from the Phase 1b clinical trial of EDG-5506 in individuals with BMD, timing for providing information on the follow-on open label study of EDG-5506 (ARCH); statements regarding Edgewise's pipeline of product candidates and programs; and statements by Edgewise's Chief Medical Officer. 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 and Edgewise's other product candidates in its EDG-6289, EDG-002 and EDG-003 programs; 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 enroll patients in its ongoing and future clinical trials; Edgewise's ability to develop a proprietary drug discovery platform to build a pipeline of product candidates; 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. 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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