



## News Release

---

### **Edgewise Therapeutics Announces FDA Authorization for Phase 2 Clinical Trial of EDG-5506 for the Treatment of Duchenne Muscular Dystrophy (DMD)**

- Initiation of LYNX Phase 2 clinical trial in individuals with DMD expected in Q4 2022 -

**Boulder, Colo., (September 7, 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 that the U.S. Food and Drug Administration (FDA) has authorized a clinical trial of EDG-5506 in children with DMD. The Company expects to begin dosing participants in the LYNX Phase 2 trial in the fourth quarter of 2022. EDG-5506 is an investigational orally administered small molecule myosin modulator designed to protect injury-susceptible fast skeletal muscle fibers in dystrophinopathies such as DMD and Becker muscular dystrophy (BMD).

“This is an important achievement for our team as we seek to expand our studies with EDG-5506 into individuals with DMD. Our team has worked with key opinion leaders and patient advocacy groups to thoughtfully design our LYNX Phase 2 clinical trial,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Edgewise. “The promising tolerability and changes in biomarkers of muscle damage that we have seen in studies with EDG-5506 in individuals with BMD supports our next step of expanding enrollment to individuals with DMD.”

The LYNX Phase 2 trial is a placebo-controlled trial to assess the effect of three doses of EDG-5506 over 12 weeks on safety, pharmacokinetics (PK) and biomarkers of muscle damage. Approximately 27 children with DMD aged 4 to 9 years on stable corticosteroids are expected to be enrolled at up to 12 sites across the United States. Participants will then continue in an open-label extension part of the trial for a total of 12 months to gain further insights into safety and functional measures. Importantly, this trial is designed to identify the doses of EDG-5506 that have the potential to reduce biomarkers of muscle damage and provide functional benefit to patients in a Phase 3 trial.

“As observed in many neuromuscular disorders, we need to have complementary approaches to address the underlying cause of the condition,” said Pat Furlong, Founding President and Chief Executive Officer, Parent Project Muscular Dystrophy. “By Edgewise expanding from Becker to Duchenne, this adds hope for those affected by Duchenne.”

#### **About Duchenne Muscular Dystrophy**

DMD is a severe, degenerative genetic disorder characterized by progressive impairment of muscle function. DMD affects an estimated one in every 3,500–5,000 male births, with an estimated 12,000–15,000 patients in the United States and approximately 25,000 patients in Europe. DMD, the most common type of muscular dystrophy, is caused by the absence of dystrophin, a protein that protects muscle from contraction-induced damage. Nearly all boys with DMD require the use of a wheelchair by the time they are young teens. Median life

expectancy for a patient with DMD is around 30 years old. There is no cure for DMD and currently limited options are available for treatment of DMD.

### **About EDG-5506**

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 designed to selectively limit the exaggerated muscle damage caused by the absence or loss of functional dystrophin. By impacting the progressive muscle damage that leads to functional impairment, 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 FDA 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 pharmacodynamics of EDG-5506 in adult healthy volunteers (Phase 1a) and in adults with BMD (Phase 1b) ([NCT04585464](#)). In ARCH, a follow-on open-label, single-center trial ([NCT05160415](#)) assessing long-term safety and PK, decreases in biomarkers of muscle damage have been demonstrated in individuals with BMD. CANYON, a Phase 2 trial ([NCT05291091](#)) is assessing safety, PK, biomarkers and functional measures in participants with BMD.

### **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](http://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 regarding the LYNX Phase 2 clinical trial, including the expected timing of initiation of such trial and the potential results from such trial; statements by Edgewise's Chief Medical Officer; 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 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 ongoing and future clinical trials; 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.

###

**CONTACT:**

**Investors & Media**

Michael Carruthers  
Chief Financial Officer  
[ir@edgewisetx.com](mailto:ir@edgewisetx.com)