



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

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Forward-Looking Statements

This presentation contains forward-looking statements that involve substantial risks and uncertainties of Edgewise Therapeutics, Inc. (“Edgewise” or the “Company”). All statements other than statements of historical facts contained in this presentation, including statements regarding our future financial condition, results of operations, business strategy and plans, and objectives of management for future operations, as well as statements regarding industry trends, 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; Edgewise’s product candidates and programs, including EDG-5506; the expected milestones and timing of such milestones for EDG-5506 including the expected timing of reporting of data for EDG-5506 and clinical trials; statements regarding the market opportunity for Edgewise’s product candidates; statements regarding Edgewise’s pipeline of product candidates and programs; and statements regarding Edgewise’s financial position including its liquidity. In some cases, you can identify forward-looking statements by terminology such as “estimate,” “intend,” “may,” “plan,” “potentially” “will” or the negative of these terms or other similar expressions.

We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including, among other things: negative impacts of the COVID-19 pandemic on Edgewise’s operations, including clinical trials; 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; Edgewise’s ability to develop, initiate or complete preclinical studies and clinical trials for, obtain approvals for and commercialize any of its product candidates; changes in Edgewise’s plans to develop and commercialize EDG-5506 or any other product candidates; the potential for clinical trials of EDG-5506 or any other product candidates to differ from preclinical,

interim, preliminary, topline or expected results; Edgewise’s ability to enroll patients in its ongoing and future clinical trials; operating results and business generally; Edgewise’s ability to raise funding it will need to continue to pursue its business and product development plans; regulatory developments in the United States and foreign countries; Edgewise’s reliance on third parties, contract manufacturers and contract research organizations; Edgewise’s ability to obtain and maintain intellectual property protection for its product candidates; risks associated with access to capital and credit markets; the loss of key scientific or management personnel; competition in the industry in which Edgewise operates; Edgewise’s ability to develop a proprietary drug discovery platform to build a pipeline of 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 risks are not exhaustive. New risk factors emerge from time to time, and it is not possible for our management to predict all risk factors, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in, or implied by, any forward-looking statements. You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we undertake no obligation to update publicly any forward-looking statements for any reason after the date of this presentation.

This presentation concerns product candidates that are under clinical investigation, and which have not yet been approved for marketing by the U.S. Food and Drug Administration (FDA). It is currently limited by federal law to investigational use, and no representation is made as to its safety or effectiveness for the purposes for which it is being investigated.

EDG-5506 is an investigational agent and is not approved in any territory

The Edgewise Approach: *Protect susceptible muscle fibers*



Some muscle fibers are more susceptible to damage due to the lack of functional dystrophin

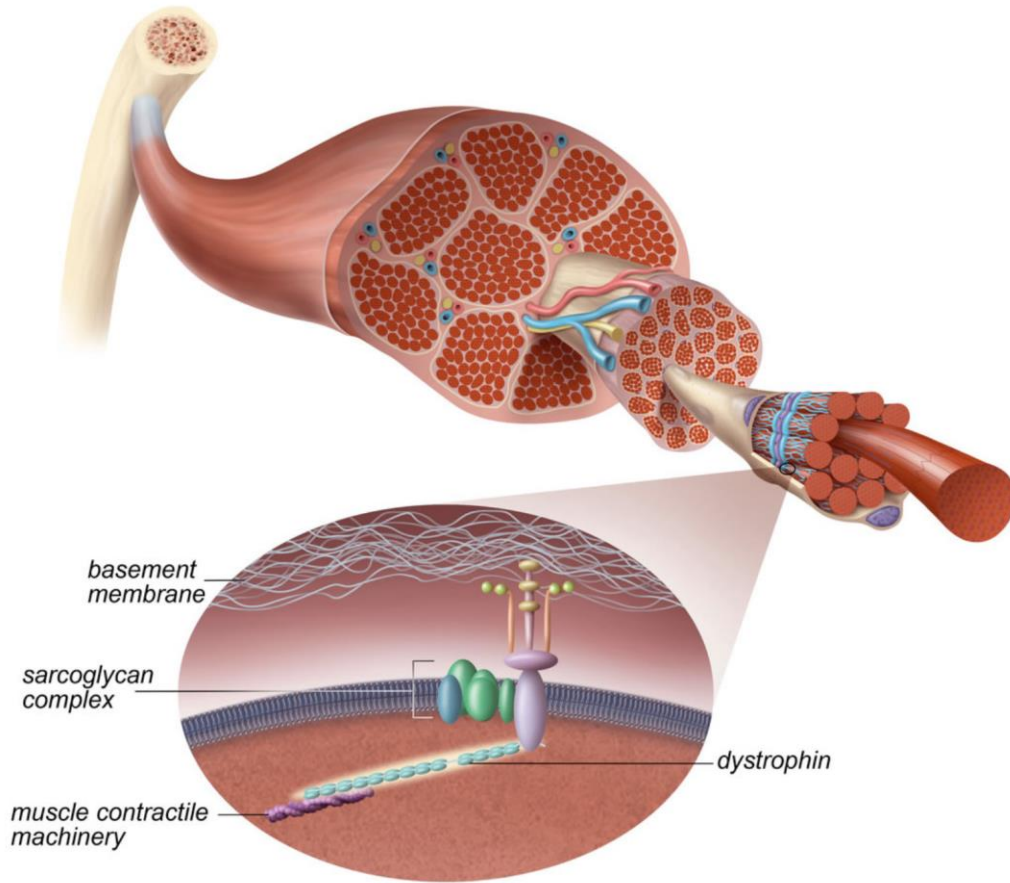


We've made an investigational therapy, **EDG-5506** that is designed to protect these susceptible muscle fibers from damage, regardless of mutation



In diseased animal models, **EDG-5506** protected susceptible muscle fibers and prevented long-term development of damage

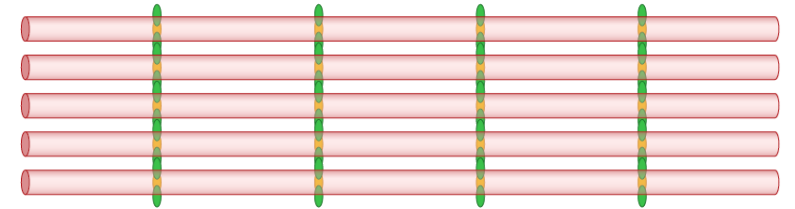
How Does Dystrophic Muscle Break Down?



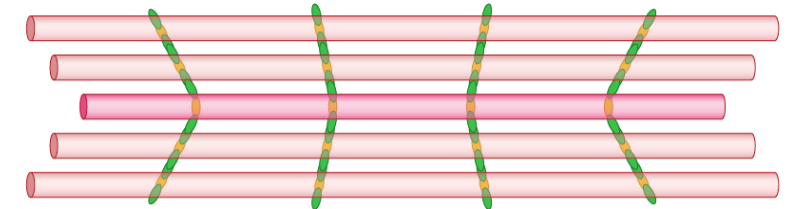
Dystrophin connects contractile proteins to the membrane and surrounding matrix of fibers

With dystrophin – fibers support each other

Rest



Contraction



Without dystrophin – contraction causes injury



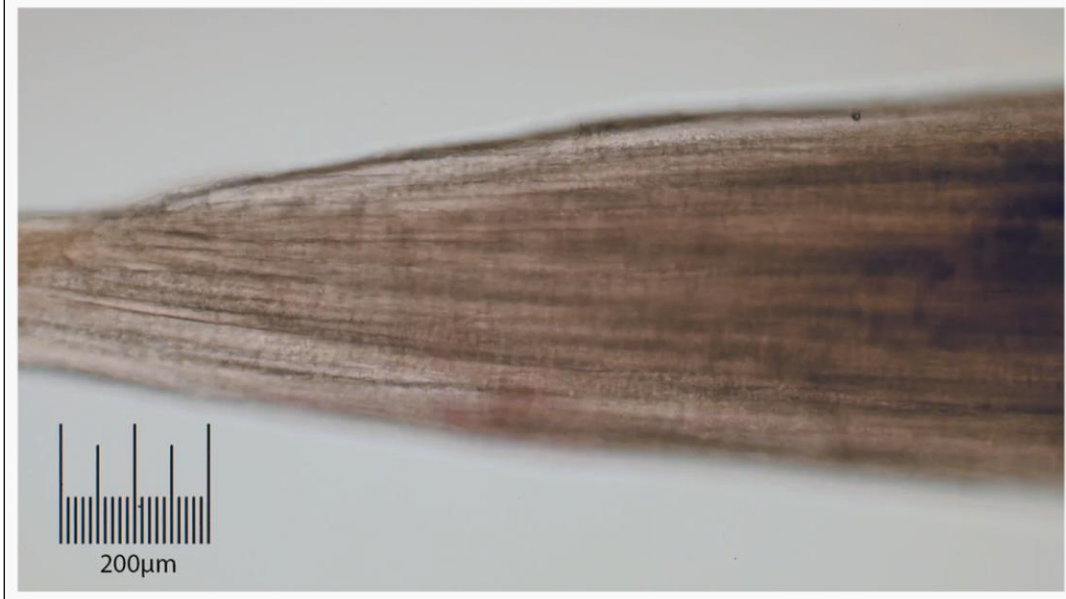
EDG-5506 Protects Dystrophic Mouse Muscle

Dystrophic mouse muscle is damaged during contraction



Contraction leads to visible changes...

EDG-5506 protects dystrophic mouse muscle during contraction



With EDG-5506, contractions don't cause these changes

Initial Phase 2* Trial in Boys with Duchenne - Design



Population

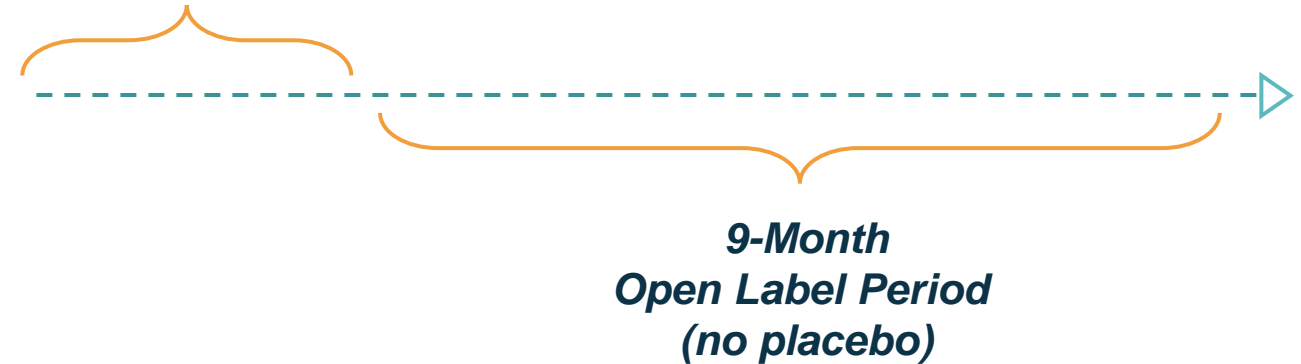


Approximately 27 individuals with a genetic diagnosis of DMD will be enrolled

- Boys age to 4-9 (up to their 10th birthday) years old
- Ambulatory and meets certain functional criteria
- On stable dose of corticosteroids
- Individuals may be on a stable dose of exon-skipping therapy

Upon screening and enrollment in trial, participants assigned to dose 1, 2, or 3; randomized to treatment or placebo

**3-Month
Treatment Period
(placebo-
controlled)**



**9-Month
Open Label Period
(no placebo)**

Initial Phase 2* Study in Boys with Duchenne



A year long, two-part trial to study the safety of EDG-5506 and to identify EDG-5506 doses for further study that have the potential to best reduce muscle damage.



Assessments and Visits

- Approximately 10 site visits required throughout 12-month trial, including screening
- At-home elements incorporated for routine monitoring
- Select functional assessments and blood tests, *no muscle biopsy*



Endpoints

- *Safety monitoring*
 - Heart monitoring
 - DEXA scan
 - Lung Function tests
- *Functional outcomes*
 - Muscle and hand grip strength testing
 - North-Star Ambulatory Assessment
 - 4-Stair climb
 - 100-meter timed walk test

Sites across the US will enroll for the LYNX trial

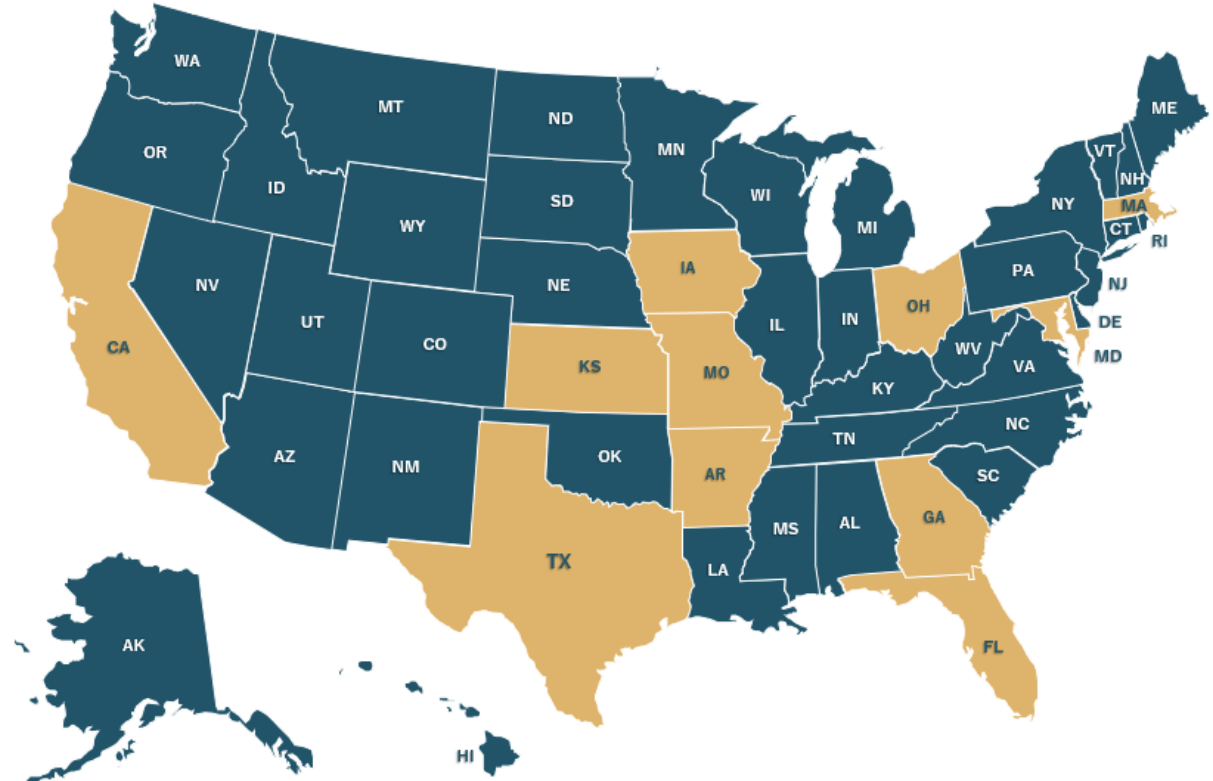


Sites actively recruiting

- Little Rock, Arkansas
- Los Angeles, California
- Atlanta, Georgia
- Iowa City, Iowa
- Kansas City, Kansas
- Gainesville, Florida
- Baltimore, Maryland
- Worcester, Massachusetts
- St. Louis, Missouri
- Cincinnati, Ohio
- Columbus, Ohio
- Fort Worth, Texas

Sites not yet recruiting

- Sacramento, California
- Aurora, Colorado



EDG-5506 is 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 muscle fibers
- Potential to be used alone or in combination with other therapeutic approaches for dystrophinopathies
- Designed to stop the damage where it begins



THANK YOU!

For questions or comments
please email us!

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