News From Edgewise January 5, 2022

Recent News Highlights

Positive Results Reported from the EDG-5506 Phase 1 Study in Becker Muscular Dystrophy (BMD)

Today we announced positive results from the Phase 1b portion of the first-in-human trial which enrolled individuals with Becker muscular dystrophy. The seven adults with BMD enrolled in the Phase 1b clinical trial were administered 20 mg oral doses of EDG-5506 (n=5) or placebo (n=2) for two weeks to assess safety and pharmacokinetics, or the levels of the drug in the body.

Data Highlights:

- EDG-5506 was well tolerated in individuals with BMD. There were no serious adverse events and no discontinuations
- EDG-5506 concentrated in muscle beyond the levels where we saw positive effects in multiple animal models of muscular dystrophy.
- Biomarkers of muscle damage, specifically creatine kinase (CK), fast troponin and myoglobin, significantly decreased and approached the normal range.

In other words, in the individuals with Becker muscular dystrophy who participated in the Phase 1b, we found enough EDG-5506 in the muscle at levels we anticipate could provide meaningful clinical benefit. EDG-5506 was well-tolerated and muscle strength was preserved. Additionally, all indicators of muscle damage that we measured moved in the right direction towards levels in unaffected individuals. Taken together, this evidence allows us to progress to the next clinical trial phase where we will look at the effects of

longer term dosing on safety and biomarkers. For more information, see our press release.

This Phase 1b portion of the trial followed the Phase 1a portion, or the single and multiple ascending dose portion of the Phase 1 clinical trial assessing the safety and tolerability of escalating doses of EDG-5506 in healthy volunteers. Go to clinicaltrials.gov to learn more about this study (NCT04585464), or for more information, see our press release.

On behalf of all of us at Edgewise Therapeutics, we wanted to say a huge thank you to all those who participated in our Phase 1 trial. We do not take it for granted that personal sacrifices have to be made to participate in a research trial. Research is complex and takes a long time, but every individual that enrolls contributes to a greater understanding of muscular dystrophy and helps speed the development of new therapies. A positive step forward for all!

THANK YOU!

New Edgewise Clinical Trial

EDG-5506-002 (ARCH) A Study of EDG-5506 in Adult Males with Becker Muscular Dystrophy

The EDG-5506-002 study is an important step in our efforts to develop a novel therapeutic approach for rare muscle disorders. For more information see clinical trials.gov (NCT05160415)

Edgewise also intends to open a Phase 2 trial in adults and adolescents with Becker muscular dystrophy in the first half of 2022 and a Phase 2 trial in boys with Duchenne muscular dystrophy in the second half of 2022. Keep an eye out for updates.

EDG-5506 Receives Fast Track

Designation in BMD

The U.S. Food and Drug Administration (FDA) granted Fast Track designation for EDG-5506 for the treatment of individuals with Becker muscular dystrophy. Fast Track designation is a regulatory process that facilitates and expedites the review of new drugs that are intended for the treatment of a serious or life-threatening disease or condition. Investigational therapeutics that receive this designation receive benefits such as more frequent meetings with the FDA to discuss development of the drug candidate and, if relevant criteria are met, eligibility for accelerated approval and priority review. We are very pleased with this designation as it confirms the seriousness of Becker muscular dystrophy as a disease as well as confers the benefits that Fast Track designation provides which will help to accelerate the development of EDG-5506.

Did You Know?

Our Science: Edgewise's approach to protecting muscle

We have designed molecules that selectively reduce muscle stress in one type of skeletal muscle fiber that is more prone to injury in both DMD and BMD. By

reducing muscle stress that occurs with everyday use, we hope to prevent the skeletal muscle breakdown, inflammation and functional decline that accompany disease progression.

Our lead molecule, EDG-5506, reduces stress by selectively targeting the muscle motor protein myosin that controls contraction of fast skeletal muscle fibers. The selectivity of EDG-5506 means that the function of other muscles such as slow skeletal and cardiac muscle is not altered. Allowing fast fibers to contract without injury should preserve muscle health in Duchenne muscular dystrophy and Becker muscular dystrophy, and potentially enhance physical function.

Where We've Been Recently

- PPMD End Duchenne Tour Washington DC, December 4
- Duchenne Patient Academy, Duchenne Data Foundation, December 4
- Action Duchenne, November 13-14
- Jesse's Journey, November 6, Edgewise Presentation Replay
- Jett Foundation Community Webinar, October 10, Edgewise Presentation Replay
- Cure Duchenne FUTURES National Conference, October 8, Edgewise Presentation Replay

Mark Your Calendars for Important Events in Early 2022!

- Parent Project Italy Annual Conference, February 17- 20
- EveryLife Rare Disease Week on Capitol Hill, February 22- March 2
- Parent Project Italy Annual Conference February 17- 20
- PPMD Advocacy Conference, March 6-8
- MDA Clinical and Scientific Conference, March 13-16

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For more information about Edgewise, go to www.edgewisetx.com.

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