Quarterly Community News • Winter 2023

Edgewise THERAPEUTICS

It's Time for a New Approach to Tackle Rare Muscle Disorders

Clinical Trials

GRAND CANYON ARCH

Edgewise Initiates GRAND CANYON, a Global Pivotal Study of EDG-5506 in Becker Muscular Dystrophy (Becker)

We recently announced the start of enrollment of GRAND CANYON, a global pivotal study of EDG-5506 in individuals with Becker. GRAND CANYON is a multicenter, randomized, double-blind, placebo-controlled study to evaluate the safety and efficacy of EDG-5506 in adults with Becker. EDG-5506 is orally administered and is designed to prevent contraction-induced muscle damage, which we hypothesize will prevent muscle damage or possibly improve muscle function and delay the progression of disease. This will be evaluated using the North Star Ambulatory Assessment in addition to other functional assessments. This trial is anticipated to recruit approximately 120 ambulatory adults with Becker between the ages of 18 and 50 years old, at up to 50 sites in 10 countries. The treatment period for participants will be 18 months.

We also presented 12-month results from the ARCH open-label study of EDG-5506 in adults with Becker. The ARCH study has been evaluating different doses of EDG-5506 administered daily in 12 adults with Becker. After 12 months there were significant decreases in biomarkers of muscle damage and a trend toward improvement in the North Star Ambulatory Assessment score, compared to a predicted decline from natural history studies. EDG-5506 was well-tolerated with no one reducing dose or discontinuing use due to adverse events.

To learn more about the GRAND CANYON study:

- Watch this **webinar**, co-hosted with Parent Project Muscular Dystrophy sharing details about the trial and answering questions from the community.
- Go to clinicaltrials.gov **(NCT05291091)** or the **GRAND CANYON website** or reach out to us at **studies@edgewisetx.com**.

Clinical Trials continued

FOX LYNX

Edgewise Expands their EDG-5506 Clinical Program in Duchenne Muscular Dystrophy (Duchenne)

On October 26, we announced FOX, a new Phase 2 placebo-controlled trial in children and adolescent boys with Duchenne who have been previously treated with gene therapy. This study will enroll approximately 24 participants between the ages of 6 and 14 in multiple states across the U.S. Participants will then continue in an open-label extension for a total of 12 months. The study will examine safety, changes in biomarkers of muscle damage, changes in functional measures, such as the North Star Ambulatory Assessment, and self-reported/caregiver-reported outcomes.

We also announced that we will continue enrollment and dose escalation in the Phase 2 placebo-controlled LYNX trial. One of the new cohorts in LYNX will study EDG-5506 in boys with Duchenne between the ages of 4 and 7 who are not currently treated with corticosteroids. The LYNX trial has been enrolling at 14 sites across the US, with the first three cohorts over-enrolling. LYNX is designed to determine a dose of EDG-5506 that will reduce biomarkers of muscle damage and could potentially provide functional benefits. We hope to report Phase 2 interim data in the first half of 2024 and identify the dose for a Phase 3 trial to start in the second half of 2024.

- Go to clinicaltrials.gov to learn more about LYNX **(NCT05540860)** and FOX **(NCT06100887)** or reach out to us at **studies@edgewisetx.com**

Edgewise in the Community

Becker Education and Engagement Day (BEED)

BEED was developed by Becker advocates, clinical sites, advocacy groups and researchers to unite the Becker community. On December 2, BEED brought over 150 individuals impacted by Becker together at four locations across the U.S.: Sacramento CA, Aurora CO, Pittsburgh PA and Gainesville FL. At the event, participants learned about topics relevant to the Becker community including the latest research updates and created personal connections with others impacted by Becker.

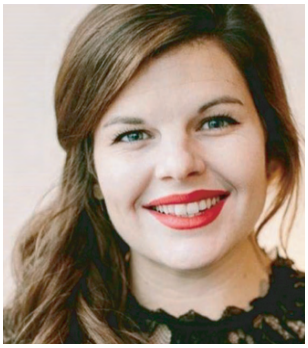
We are thankful to our partners for this inaugural event who helped make it a success: Children's Hospital of Colorado, Imaging NMD, Muscular Dystrophy Association, Parent Project Muscular Dystrophy, ReveraGen, UC Davis Health, University of Colorado Department of Neurology, University of Florida, University of Pittsburgh, and Virginia Commonwealth University.

Meet the Edgewise Patient Advocacy team

Abby Bronson

Vice President of Patient Advocacy

Abby joined Edgewise in 2020 to lead our patient advocacy outreach and build key relationships with the muscular dystrophy community. Her goal has always been to bring the patient voice into drug development, which we believe is integral to the process. Abby brings an extensive patient advocacy background, having most recently served in leadership roles at PPMD and NIH, and we are fortunate to have someone with her knowledge and passion for the patient community leading our team.



Katherine Krieger

Director, Patient Advocacy

Katherine joined Edgewise in 2022 to expand our reach in the muscular dystrophy community, leading efforts such as the Becker and Duchenne community councils. She comes with years of experience in the patient advocacy world in neuromuscular diseases, and most recently was at VOZ Advisors, where she helped rare disease biopharma clients integrate the patient voice throughout the development lifecycle. Previously, she was at the Muscular Dystrophy Association, serving in a variety of roles from Patient Access Liaison to Director of Care and Clinical Services.

Sarah Tencer

Associate Director, Patient Advocacy

Sarah joined Edgewise in October as the newest member of the Patient Advocacy team. She has her Masters in Social Work and has worked supporting biopharma companies in deepening relationships with patient advocacy groups, as well as developing family-facing programming at the Muscular Dystrophy Association, such as the national Summer Camp program and piloting recreation programs for the full family.