

October 13, 2022

Dear Duchenne and Becker Community:

Today we issued a <u>press release</u> announcing positive 6-month interim results from the ongoing ARCH study, an open label study of EDG-5506 in adults with Becker muscular dystrophy. EDG-5506 is an investigational orally administered myosin modulator designed to protect injury-susceptible fast skeletal muscle fibers in dystrophinopathies such as Duchenne and Becker. The twelve adults with Becker enrolled in the ARCH study received an increasing dose level of oral EDG-5506.

After 6 months of EDG-5506 dosing, EDG-5506 continues to be well-tolerated in all participants with no discontinuations or dose reductions. Consistent with prior observations, treatment with EDG-5506 led to a significant decrease in key biomarkers of muscle damage, including creatine kinase (CK) and fast skeletal muscle troponin I. With 6 months of EDG-5506 dosing, North Star Ambulatory Assessment (NSAA) scores are trending in a positive direction, i.e., improving, and a similar trend was observed with the North Star Assessment for Limb Girdle Type Muscular Dystrophies (NSAD) scores, an expanded functional outcome scale developed for adult populations which includes higher difficulty tasks. These NSAA data diverge from trajectories observed in natural history studies. There was also a positive trend, i.e., towards improvement, in self-reported pain scores.

Edgewise's Chief Scientific Officer, Alan Russell, PhD, and Chief Medical Officer, Joanne Donovan, MD, PhD, will present these findings on October 14 at the 2022 Annual Congress of the World Muscle Society in Halifax, Nova Scotia. We are encouraged by these results and believe they provide further support that reducing contraction-induced damage in dystrophic muscle has the potential to preserve and improve muscle function while preventing disease progression in dystrophinopathies. These results also help guide our development plan in Duchenne and Becker.

We are participating in the following upcoming events through the end of 2022. Of note, we hope you can join us during one of the Community Webinars where our team will discuss and answer questions about our new Phase 2 clinical trials for Duchenne and Becker.

Upcoming Events

- PPMD Community Webinar, October 19, Virtual
- Cure Duchenne Community Webinar, November 2, Virtual
- Defeat Duchenne Canada Family Forum, November 4-6, Hybrid
- Action Duchenne International Conference, November 11-12, Manchester, UK
- Jett Foundation Community Webinar, November 17, Virtual
- TREAT-NMD, December 7-9, Vancouver

As always, thank you to all the individuals, and their families and caregivers, who participate in trials to further research. We remain focused on improving the lives of those with rare muscle diseases, such as Duchenne and Becker, and will continue to provide updates as more is learned.

Sincerely,

The Edgewise Team

