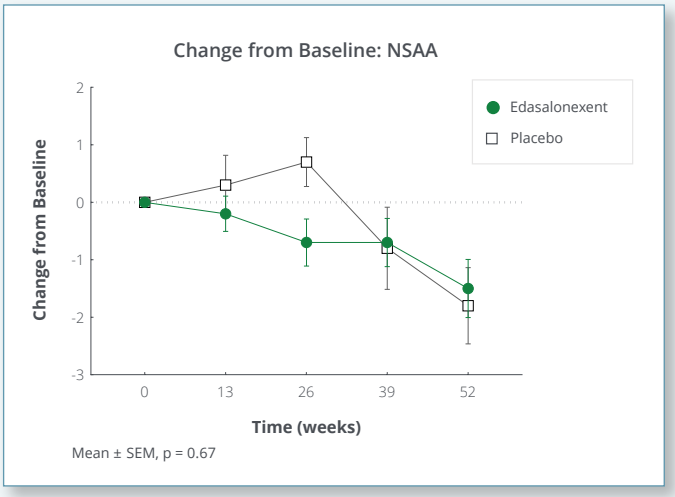
catabasis CONNECTION

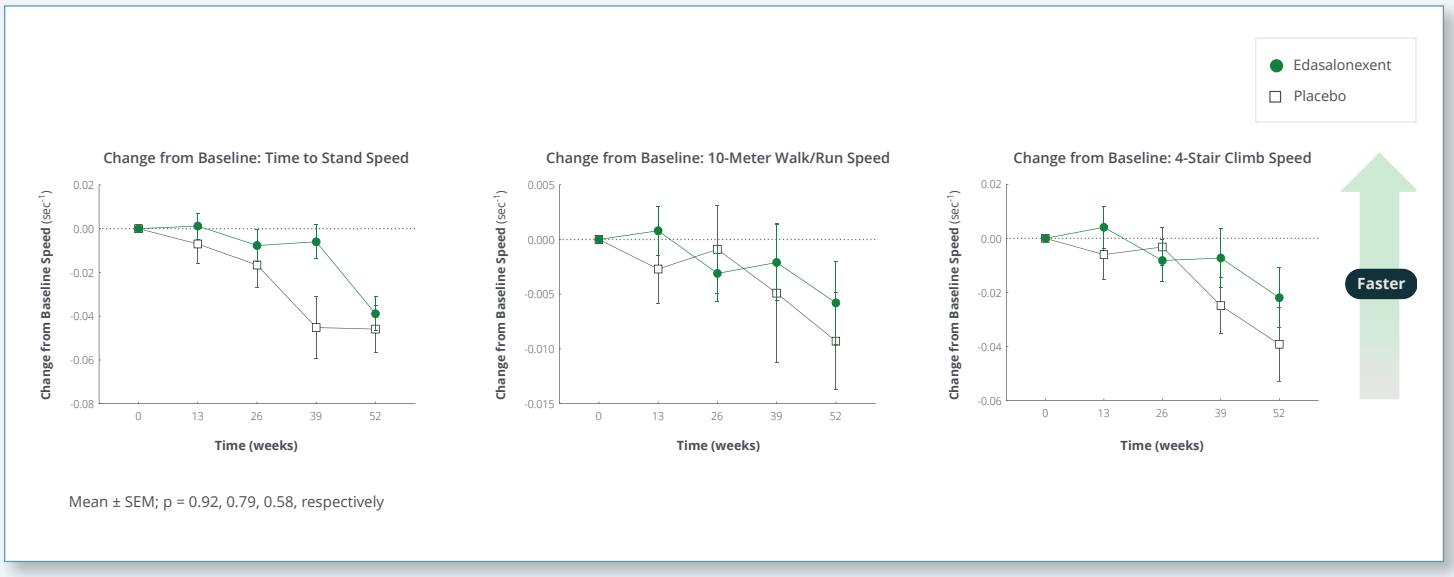
October 2020

POLARIS DMD

To the Duchenne Community,

We are very sorry to share today that the Phase 3 PolarisDMD trial of edasalonexent in Duchenne muscular dystrophy did not meet its primary endpoint, which was to measure the change in the North Star Ambulatory Assessment (NSAA) over one year of edasalonexent treatment compared to placebo. The secondary endpoints were the timed function tests (time to stand, 10-meter walk/run, and 4-stair climb) and also did not show statistically significant improvements. Muscle enzymes and heart rate did not show significant differences between edasalonexent and placebo.





Edasalonexent was well-tolerated, which is consistent with the safety profile we have seen to date. The majority of adverse events were mild in nature, and the most common treatment-related adverse events were diarrhea, vomiting, abdominal pain and rash. There were no treatment-related serious adverse events and no dose reductions. There were no adverse trends in blood tests and growth was maintained.

We would like to sincerely thank everyone who participated in and enabled the edasalonexent program. We are greatly appreciative of all the boys that participated in the trial and their families and caregivers. Thank you to everyone who helped make PolarisDMD possible: boys and their families, site personnel, investigators, trial sites and advocacy groups. We are extremely grateful for your support and understand the sacrifices made when participating in a clinical trial and even more so during the COVID-19 pandemic.

We are profoundly saddened and disappointed to share the difficult decision that development of edasalonexent, including the ongoing GalaxyDMD open-label extension trial, will be stopped. In addition to the muscle function and safety information, we will be analyzing the bone and cardiac data, and we are committed to sharing the data from the PolarisDMD trial with the Duchenne community to contribute to natural history data. We plan to submit the data from the Phase 3 PolarisDMD trial for presentation at upcoming scientific conferences as well as to publish these results in an effort to advance Duchenne research for the entire community.

We wish that we could be in touch with each trial participant individually, however due to confidentiality we are unable to reach out directly. We will be in close communication with the investigators and study site personnel. **Families involved in the PolarisDMD and GalaxyDMD trials who have questions are encouraged to contact their study site directly.**

All in the Duchenne community are welcome to join our webinar with PPMD on Tuesday, October 27 at 1:00pm ET where we will share more details and you can ask questions about the trial and the results.

Thank you,

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The information provided here is for those affected by Duchenne muscular dystrophy and their families and caregivers. Edasalonexent is an investigational drug that is not approved in any territory.

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