

STEADFAST BACKGROUND FOR THE FRIEDREICH'S ATAXIA COMMUNITY

The Friedrich's Ataxia Research Alliance (FARA) and Horizon Pharma plc would like to share additional background regarding the Phase 3 STEADFAST clinical trial, as well as information about our ongoing collaboration moving forward. We have received many questions among those who participated in the clinical trials, as well as others in the FA community. We want to ensure that you know we have heard your feedback, and that we are working together in the following ways.

- **Efforts are Underway to Analyze the STEADFAST Clinical Trial Data**

Given the patient reported experiences from people who participated in the study, we know that many people throughout the FA community have additional questions about STEADFAST. FARA, Horizon and David Lynch, M.D., Ph.D., professor of neurology at Children's Hospital of Philadelphia (CHOP) and principal investigator of the study, are working together to analyze all of the STEADFAST data. We are working diligently to better understand the results and continue collecting and analyzing data from the open label and long-term extension studies. FARA and Horizon want to make sure we provide transparent information in a timely fashion, and we will provide further updates as they become available.

- **The Study Has to Be Stopped Based on the Results**

Because both the primary and secondary endpoints were not shown to be statistically significant, the clinical trials have to be stopped, and this is a process that we are currently working through. People currently enrolled in one of the STEADFAST extension studies have the option, in consultation with their study physician, of continuing on the study drug until their last appointment is scheduled. As we work out the details of this transition we encourage all study participants to speak with their study physician regarding next steps with regard to their individual situation.

- **Horizon Remains Engaged with the FA Community**

Horizon is deeply appreciative for the collaboration among the FA community throughout the STEADFAST clinical trial, especially the people who enrolled in the study and maintained schedules and appointments, as well as their families. The company is particularly grateful for the opportunity to become part of the FA community over the last several years. Horizon will continue to support FA initiatives and this includes rideATAXIA and other education and awareness events during 2017. Employees of the company are profoundly moved by their interactions with people impacted by FA – this was shared in an article by Horizon CEO Tim Walbert, which you can read [here](#).

We would like to thank everyone who has contributed to pushing this research forward. We greatly appreciate your feedback and collaboration. We know that there are many questions and we are doing our best to answer them as quickly as possible. We will continue to provide timely, transparent communications as additional information becomes available.