At Praxis Precision Medicines (Praxis), we are committed to exploring novel therapies to treat rare diseases, including SCN8A-DEE (Developmental and Epileptic Encephalopathy). We believe that partnership with SCN8A patients, families, and the advocacy community is essential to best understand what it is like to live with an SCN8A related disorder. Together, we can maximize our potential to help families impacted by SCN8A.

We come to the community to share some exciting news, offer our partnership, and issue an invitation.

Meeting Milestones Leads to Making Progress

Recently, Praxis received a Rare Pediatric Disease (RPD) designation from the FDA for its investigational medication, PRAX-562, which acts as a persistent sodium current blocker. We are encouraged by the FDA's decision because it recognizes SCN8A-DEE as a rare pediatric disease, and that the scientific data emerging to date show promise for the continued development of PRAX-562. This designation is significant because it means the FDA sees potential in our available preclinical findings to evaluate this therapy in pediatric patients under the age of 18.

We are actively working with the FDA to answer questions necessary for consideration of an Orphan Drug Designation (ODD). An ODD is a special status granted by the FDA to a drug or biological product that will treat a rare disease. A drug may qualify for an ODD if both the drug and the disease meets specific criteria under the Orphan Drug Act and FDA regulations. Such a designation is important because it provides certain incentives to support development of products for rare diseases. Once we have more information, we will share that with the community.

Each of these milestones leads us closer to finding therapies that may treat SCN8A. We are confident that securing these designations will help us improve the lives of children and families sooner and should support efficient development of potential therapies. But we can't do it without you.

Working Together to Make Things Better

At Praxis, we are passionate about science. But we are equally passionate about the people the science may help. Only by working together can we achieve our goal of finding new treatments for rare diseases, like SCN8A. You are the experts in what it is like to live with a rare disease. Your knowledge is critically important in the search to find targeted therapies. We are eager to work with you, learn from you, and share with you the latest scientific information Praxis generates.

Over these last few years, we have learned a lot from SCN8A advocacy leaders about what it is like to live with and care for someone with a mutation in the SCN8A gene. We have been fortunate to welcome children with SCN8A and their families to the Praxis office, to speak at

SCN8A gatherings and events, and to participate in ongoing discussions with advocacy leaders. And we recognize there is still so much to discover. We hope to continue conversations with advocates and families, find innovative ways of sharing insights and data, and further our understanding of this very complex condition and the impact it has on the lives of those affected.

Learning More from Each Other

In that spirit of learning, we would like to invite you to join us, along with SCN8A advocacy leaders, for a virtual community discussion that will focus on introducing the Praxis team to you, answering many of the questions we've received, and providing information on our development programs.

We'll be sharing the time and web link for the virtual community discussion with Praxis in the coming weeks via the Praxis LinkedIn and Twitter social media platforms, as well as through patient advocacy groups; we invite you to follow us to receive updates. In the meantime, we recognize that there have been a lot of questions on your minds already and we hope to share some insights about the drug development process. We are thrilled that there is a community for SCN8A patients and families, and we recognize that advocacy groups help connect you to each other and to critical support and information. To continue to learn from you and share information with the community, we invite you to reach out to us at patientadvocacy@praxismedicines.com with additional questions or thoughts.

Q1. Where are you in the development process for PRAX-562?

A1. PRAX-562 is in Phase I of clinical development, which means that human studies have started. These studies help us to understand the safety of the study medication in humans. Current studies have focused on adult safety and we plan to study the medication in children who have SCN8A mutations. If the results are favorable, we will work with the FDA to start clinical trials as quickly as possible in patients with SCN8A mutations.

Q2. How does PRAX-562 work?

A2: PRAX-562 is a selective small molecule and the first persistent sodium current blocker in development for the treatment of a wide range of rare central nervous system disorders, including SCN8A.

We know there are more questions on your minds, and we look forward to addressing them at the webinar.

In partnership,

The entire Praxis Precision Medicines Team