

Email Subject Line: The EMBOLD Study: Now offering a fully in-home participation option

Subheading: New Anti-Seizure Options For Children With DEEs Are Long Overdue, consider if the EMBOLD study could be right for your child.

Dear {Caregiver}

We're excited to share with the DEE community that the EMBOLD study (PRAX-562-221) for children with early-onset SCN2A-DEE and SCN8A-DEE is now enrolling and offering families flexible participation options. The EMBOLD study is now offering a fully in-home participation option in which all study visits can be done from your home. We know traveling can be difficult for this patient population, so we are bringing the trial to you.

In the EMBOLD study, you have the option to participate fully remote from your home, in clinic at a study site, or a combination of both in-clinic and at-home visits. The choice is yours to make.

About the EMBOLD Study

The main purpose of this study is to learn about the effects of the study drug, PRAX-562, by assessing the safety and impact on numbers of seizures.

Part A of the study will last up to 26 weeks and consist of 11 study visits. Your family will have the option to complete these study visits completely remote from your home, in clinic at a study site or a combination of both in-clinic and at-home visits.

If PRAX-562 shows benefit in Part A, all participants will have the option to enroll into an open-label extension for up to an additional 48 weeks. In the open-label extension, all participants will continue to receive PRAX-562 and you can continue to participate in a flexible manner, in clinic and/or at home. PRAX-562 will be available after the open-label extension through an expanded access program for participants who showed clear benefit beyond previous therapeutic treatments.

What is PRAX-562 and how is it potentially different from currently available therapies?

PRAX-562 is an investigational medicine that has been designed to more precisely regulate the flow of sodium in and out of the cells in the brain. Currently available sodium channel blockers work to block the flow of sodium across all sodium channel types. PRAX-562 has been designed to maximize its effects against overactive sodium channels that are believed to cause seizure activity while minimizing the blocking of the normal activity needed for healthy brain functioning. We believe this precision approach may offer greater seizure control with reduced side effects.

Preclinical studies of PRAX-562 showed that it counteracted key causes of seizures in DEEs and provided greater seizure control in DEE animal models, up to complete elimination of seizures.

Further studies in healthy humans showed that PRAX-562 was generally well tolerated. These studies also showed that the precision approach could minimize titration needed to get to an effective dose. PRAX-562 is administered as a liquid formulation to be taken once daily, which may make it easier for

children with DEEs to take consistently, thus, potentially leading to more consistent therapeutic levels in the body over time. PRAX-562 is both keto friendly and able to be administered through a G-tube.

Why should my child participate?

- Flexible study design allows study participation to be completed fully remote from your home, in clinic at a study site, or a combination of both in-clinic and at-home visits
- If your family chooses to attend in-clinic study visits, all expenses for travel, lodging, meals and any other costs associated with study participation will be paid for by the sponsor
- All participants will receive PRAX-562 during the study
- Option to participate in an open-label extension after the double-blind portion is complete
 - In the open-label extension, all participants will have the opportunity to receive PRAX-562 for an additional 48 weeks after completion of Part A. You can also continue to participate in a flexible manner
- PRAX-562 will be available after the open-label extension through an expanded access program for participants who showed clear benefit beyond previous therapeutic treatments.

Could your child take part?

This study is enrolling about 20 participants (about 10 early-onset SCN2A and 10 early-onset SCN8A). Your child may be able to take part if they:

- Are 2 through 18 years old
- Have received a diagnosis of:
 - SCN2A gene mutation with onset of seizures in the first three months of life
 - OR**
 - SCN8A gene mutation with seizures
- Have at least eight motor seizures (seizures that involve movement) in the four weeks prior to screening
- Additional criteria to be assessed at screening

How do I find out if my child can participate??

Visit the EMBOLD study website <http://www.emboldstudy.com> and schedule a call with the Praxis Nurse Patient Navigator.

The Nurse Navigator will confirm your child's eligibility and connect you with an EMBOLD study site.

It is important to note that **PRAX-562 is an investigational drug**, which means that it has not been approved by any regulatory or health authorities, including the U.S. Food and Drug Administration (FDA). Participation in clinical research studies is completely voluntary and you may end participation for your child at any time.

