PRAXIS

September 2021

connecting for change

UPDATES FOR THE RARE EPILEPSY COMMUNITY

We're pleased to share this special Praxis Precision Medicines SCN2A and SCN8A community update. We invite you to learn more about what Praxis has been working on this Spring / Summer, and we thank you for your insights, interest, and participation.

NEWS TO KNOW

Our sincere appreciation goes out to all the SCN2A and SCN8A families who have participated in the Ciitizen program! Earlier this year, Praxis partnered with Ciitizen, a healthcare technology company that helps patients get full control of their medical records, to conduct digital observational studies about the signs and symptoms of SCN2A and SCN8A-related disorders. Ciitizen is currently collecting medical records and analyzing the data from electroencephalograms (EEGs), genetic tests, and doctor's notes. As we learn more, we plan to find ways to share what we've learned with advocates and families.

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PRAXIS RESEARCH UPDATES

PRAX-562: *Praxis' selective persistent sodium current blocker*

- Praxis plans to initiate a Phase 2 trial of PRAX-562 for treatment of developmental epileptic encephalopathies (DEEs), including SCN8A-DEE and SCN2A-DEE, in the first half of 2022. In Phase 2 trials, the study drug is administered to a group of patients with the disease or condition for which the drug is being developed.
- Praxis has completed the dosing and safety follow-up period for its single ascending dose (SAD) and multiple ascending dose (MAD) groups up to 150 mg and 120 mg, respectively in its Phase 1 healthy volunteer study. No dose-limiting toxicity has been observed, meaning that side effects were not serious enough to prevent an increase in dose or level of treatment. Safety, tolerability, pharmacokinetic (*i.e.*, how the drug moves through the body), and preliminary biomarker data will be reported by the end of 2021.

PRAX-222*: Praxis' antisense oligonucleotide (ASO) designed to reduce the expression of the SCN2A (Nav1.2) sodium channel*

- Praxis plans to complete the ongoing Investigational New Drug (IND) enabling toxicology study by the end of 2021 for PRAX-222. This is a preclinical animal study; the results of which Praxis intends to use to support its anticipated IND submission to the U.S. Food and Drug Administration (FDA). An IND is a request for authorization to begin clinical trials in humans.
- If the anticipated IND submission is cleared, Praxis intends to initiate a Phase 1/2 trial of PRAX-222 for the treatment of SCN2A-DEE in the first half of 2022. A Phase 1/2 trial is a study that tests the safety, side effects, dosage, tolerance, and efficacy endpoints of a new treatment in patients.

COMMUNITY CONNECTIONS

- ***Welcomed*** **at "A Conversation Between Praxis and SCN8A Community" – May 5.** The Cute Syndrome Foundation facilitated a great discussion that provided updates on the state of the PRAX-562 program, while also sharing more about Ciitizen from the perspective of Kelley Dalby, a Praxis employee and SCN2A parent, who enrolled in the Ciitizen program.

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- *Attended* **"2021 Rare Drug Development Symposium" – June 9–11.** In partnership with the Orphan Disease Center of the University of Pennsylvania, Global Genes presented on topics ranging from clinically meaningful measures to the importance of research participation in small populations. The report from the conference is available to the public here.

- *Attended* **"Living Rare, Living Stronger Patient & Family Forum" – June 26–27.** Hosted by NORD, this two-day event included sessions focused on addressing issues important to advocates, families, and patients. Sessions included information on finding and preparing for clinical trials, shared decision making with one's care team, aging with a rare condition, and finding and building community. See here for the event agenda.

- *Welcomed* **at "Virtual Community Discussion on Data" – July 13.** Wishes for Elliott and the Shay Emma Hammer Research Foundation invited Praxis to join a roundtable discussion on the importance of data in driving science forward. Praxis spoke about the recent Ciitizen launches in SCN2A and SCN8A and how observational studies, like what Ciitizen is leading, allow for greater access to data by researchers in the field. More insight into complex genetic epilepsies can help provide a long-term insight and awareness around the progression of these diseases.

- *Attended* **"NORD Patient Advocacy and Engagement Working Call" – July 18.** Praxis listened as the FDA spoke to what is happening within the agency to help advance clinical research in rare diseases.

- *Hosted* **"SCN2A Caregiver Advisory Council Kickoff Meeting" – September 8.** Praxis welcomed nine of the 11 global Advisory Council members for its inaugural meeting. The Praxis Rare Disease Program team is grateful to the Council for its thoughtful recommendations and engagement.

- *Attended* **"SCN8A & SCN2A European Conference & Family Gathering" – September 10-11.** Praxis joined advocacy leaders, families, researchers, and clinicians for important presentations on the latest developments in both SCN2A and SCN8A. Information was also shared by other genetic epilepsy leaders on topics related to devices and technology, health economics data, and the power of the patient and caregiver voice.

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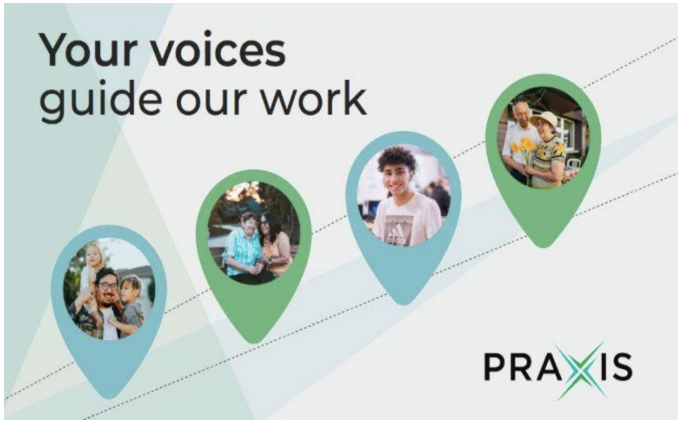
INSPIRED BY YOU

The Cute Syndrome Foundation released its formulation survey, which included incredible insights from SCN8A families around the most preferred ways to administer medications.



The insights provided help ensure Praxis fully understands how to best develop the study medications for upcoming clinical trials and what families need in terms of instructions and support. We are grateful for these insights, and **we sincerely thank the families who contributed to the survey** for sharing their thoughts!

COMING SOON

- **September 27–29** – Global Genes Rare Summit
- **October 16 –** SCN2A Families UK Conference

LET'S STAY IN TOUCH

Have questions or would like to connect? Please feel free to reach out to us at patientadvocacy@praxismedicines.com.

You can also connect with Praxis Medicines on Twitter and LinkedIn.