February 2022

SCN2A Community Questions

At Praxis, we're on a mission to develop novel therapies for SCN2A. We believe collaboration with the SCN2A community is critical in this work. We have compiled some questions we have received from you, along with our responses, to keep you informed on our journey.

Is this true? If so, is it in all er?

A1. The predominance of where SCN2A and SCN8A channels exist in neurons (brain cells) shifts throughout the first few years of life. They do not go away completely.

Q2. Will PRAX-222 eliminate the production of all SCN2A, or only SCN2A with the mutation?

A2. PRAX-222 is not allele-specific, meaning it may have some impact on the gene with an abnormality and the gene that does not have a variant. The amount to which this may occur is difficult to predict, and we are taking this into consideration as we design our initial studies for PRAX-222.

Q3. I know a girl whose coding of SCN2A and SCN1A in her DNA is missing. And she is really in poor clinical condition, seizures, and severe developmental delay. In that case, do you think PRAX would help?

A3. We would first recommend a more detailed discussion between the family and their doctor before entering a clinical study.

Q4. Based on the preclinical studies, what were the positive and negative results (if any) of these two therapies?

A4. We are very early in our research, so we are still investigating. Over the course of the studies, we will share what we learn.

Q5. Are these two potential therapies OK for both SCN2A Gain of Function and Loss of Function mutations? Are they OK for Epileptic Encephalopathies, then?

A5. Currently, PRAX-222 would only be studied in patients with a SCN2A gain of function mutation; we are working with The Florey Institute of Neuroscience and Mental Health to understand the potential impact of an ASO on SCN2A loss of function mutations. Additionally, we are working to better understand the best populations to target for PRAX-562.

Q6. What stage of clinical development is PRAX-562 in? Does this mean that it will soon be available as a therapy to treat this condition in the US?

A6. We are early in the research process and are still conducting early studies. We have recently received both Rare Pediatric Disease and Orphan Drug Designations from the FDA. This is exciting news for us at Praxis and we hope also for you. Receiving this designation means that the FDA's Office of Orphan Product Development recognizes that SCN2A is a rare pediatric disease, and that the scientific data emerging to date show promise for the continued advancement of PRAX-562. These early interactions with the FDA on nonclinical data are critical for understanding if our rationale for investigating a disease is sound.

Q7. In the event PRAX-562 and/or PRAX-222 are approved by the FDA, are you anticipating whether there will be parameters related to who will have access to them?

A7. We are still in the research phase of drug development, so we cannot speak to if or when PRAX-562 or PRAX-222 will be available. Drug development can sometimes take years, so we still have a lot of important work to do to understand if either potential therapy can help people with SCN2A gene mutations. We will continue to keep the community apprised on our progress in both programs.

Q8. My child has been enrolled in the Natural History Study of the disease for 18 months. Is there anything else we can do further to taking part to the said study?

A8. Praxis is grateful that you are participating in Dr Howell's Natural History Study. As a community, every piece of data we can collect on SCN2A gene mutations helps us better understand how complex it is. We will certainly let you know if Praxis is leading any additional research or opportunities to share data.

Q9. How will the patients be selected for the clinical trials of PRAX-562 and PRAX-222?

A9. Inclusion and exclusion criteria for the upcoming studies will be detailed in each study's clinical protocol. Once the protocol is finalized and approved, it will be made available via advocacy groups and clinicaltrials.gov.

Q10. Is there a possibility to transfer or share existing surveys that many of us parents have already filled out (e.g., Simons Searchlight, Natural History Study) to the Ciitizen platform?

A10. The Simons Searchlight and SCN2A Natural History Studies are conducted by independent investigators. At present, these different studies are not connected. Therefore, we would recommend anyone participating in Ciitizen to send their records directly to Ciitizen irrespective of participating in another study.

Q13. I'm having trouble sending EEGs for my child to Ciitizen, and I'm running into issues with the platform. Who should I reach out to?

A13. Both the US and International SCN2A cohorts have closed. If you were part of either group and experienced difficulties that have not been resolved, please contact scn2aciitizen@praxismedicines.com and share your experiences and concerns. They should be able to help ensure you get an answer from Ciitizen to troubleshoot.

Q14. If a family is receiving bills from the hospitals for Ciitizen requesting records, where should we direct them?

A14. That is a question best addressed by Ciitizen. Please reach out to Virginie at virginie@ciitizen.com to inquire.

Do you have a question?
We invite you to contact us at patientadvocacy@praxismedicines.com