



## **Q&A on Core Panel Developing SCN8A Treatment Guidelines**

**What are the key phases, time frames, roles, and approximate time commitments for members of the Core Panel?**

### ***Phase I: PROCESS AND INITIAL ANALYSIS PHASE***

November - Spring 2022 --> time commitment is approximately 2-4 hours/week

1. Establish process guidelines and policies for Core Panel and full project.
2. Define focus and organization of several small working groups of Core Panel members (composed of 2 clinicians and one family representative) who will perform a literature review on specific areas of focus to document established evidence.
3. Develop 1st draft questionnaire of Diagnosis and Treatment Guidelines for scrutiny by Core Panel for review and comments by larger group of reviewers.
4. Nominate, vet, and select expert physicians and family members to serve on the Review Panel.

### ***Phase II : REVIEW/RECONCILIATION/REFINEMENT PHASE***

Spring - Fall 2022 --> time commitment is approximately 10-12 hours total

1. Review data from each round of the Delphi along with respondent comments. Determine areas of consensus, and, where necessary, revise statements for the next Delphi round.
2. There will likely be 3 rounds of the Delphi – each round being shorter than the previous one.
3. Refine questions/statements of possible consensus based on feedback from the full review panel.

### ***Phase III FINALIZATION PHASE***

Fall 2022 - early Winter --> time commitment is approximately 15-20 hours total

1. Finalize Consensus Guidelines - release, publication, submission to professional association for possible adoption based on the Delphi. This should hopefully be an in-person meeting - perhaps at AES in Nashville,



TN in December 2022.

2. Each Core group member will be responsible for drafting a portion of the manuscript, providing critical feedback on the entire manuscript, and incorporating final changes to achieve full consensus.

### **What kind of support structure is in place to facilitate the work of Core Panel members?**

The International SCN8A Alliance has partnered with the COMBINEDBrain team who bring expertise in neurosciences and rare diseases to provide active and ongoing support to the Core Panel and facilitate each stage of the process. They will provide both administrative support for the detailed logistics in convening meetings, distributing drafts, as well as providing preliminary analysis of published literature, pertinent data from the 7 year longitudinal SCN8A Registry, and preliminary assessment of the quality of available evidence.

For each round, the 8AAlliance/CB team will provide data analysis, review each submission and determine consensus for review by the core group. The support team will manage the process, documentation, discussions, monitor and follow up on open issues and work with panel members to keep the overall project on track.

### **How clear are models of similar efforts - and do they help inform this process?**

The Modified Delphi process is well established as a scientific method to seek consensus. [Here is a link](#) to a publication describing the process and outcome for a Standard of Care for Dravet patients in 2016. Dr. Elaine Wirrell, who played a lead role in managing the Dravet process, as well as an ongoing update, will be serving as a process guide for this effort. As SCN8A is far more heterogeneous than Dravet, we anticipate more complexity in our process and more challenges achieving consensus around specific treatment guidelines for the whole population.

### **Will substitutes be allowed when a primary Core Panel Member may be unavailable?**



Yes. If you have a co-parent or partner who can attend the meetings and can fully catch you up on discussions and next steps, it will be acceptable to send that person as a substitute, when necessary.

**Will there be compensation for time by Core Panel members?**

We will follow the practice used in a similar effort to develop the first Standard of Care for Dravet.

Family representatives on the Core Panel who are active in all three phases of the Delphi process and participate as final authors of the consensus paper will be offered a \$750 Honoraria for their time and efforts.