

# Kayak Study for SCN8A-DEE Fact Sheet

## What Is the Purpose of the Kayak Study?

The purpose of the Kayak Study is to evaluate the efficacy, safety, and tolerability of an investigational study drug (NBI-921352) compared to a placebo (inactive drug) as add-on therapy (i.e., in addition to existing treatments) in young people 2 to 21 years of age with SCN8A developmental and epileptic encephalopathy syndrome (SCN8A-DEE). This study, also known as a clinical trial, is looking at how the study drug affects participants' symptoms, quality of life, severity of seizures, and frequency of seizures.

## What Drug Is Being Studied and How Is It Taken?

The study drug, NBI-921352, is a selective sodium channel inhibitor, which means it acts on a particular channel in the brain that is thought to contribute to seizures in SCN8A-DEE. Both the study drug and the placebo are taken orally or via a gastrostomy tube (G-tube).

## What Should Participants Expect?

The Kayak Study consists of a screening period (during which eligibility will be confirmed) and a study treatment period of up to 18 weeks. After meeting eligibility criteria, participants will have 9 visits and 4 phone calls throughout the study. Most visits will last approximately 3 hours, although 3 of the visits will last approximately 8 hours. There are 2 treatment groups in this clinical trial - 1 group that will receive NBI-921352 (the investigational study drug) and 1 group that will receive placebo. Each participant will be randomly assigned to 1 of the 2 groups in equal numbers, and the assignment will not be revealed to the participant or the study team (unless medically necessary). Participants assigned to the placebo group will receive matching placebo (which looks just like NBI-921352 but does not contain active ingredients) for the trial duration. Following completion of the Kayak Study, eligible participants, including those who received the placebo during the Kayak Study, have the option to enroll into an extension study to receive NBI-921352 for about 2 years. The study doctor will be involved in assessing eligibility for the extension study.

Both the study drug and placebo consist of oral granules in a capsule. The entire contents of the capsule will be added to water or mixed with a semi-solid liquid such as applesauce, yogurt, or pudding. For participants with a G-tube, the contents of the capsules will be taken with juice. Participants should take the assigned study treatment 3 times a day approximately 8 hours apart.

If an individual qualifies to participate in this clinical trial, study-related medical exams and study-related laboratory tests will be performed at no cost to them. Compensation for travel or lodging support may also be available. For sites that have agreed to use it, a travel service is being provided to assist with booking flights, rail, car rentals, taxis, wheelchair vans, lodging, etc.

## Who Qualifies for the Kayak Study?

Eligible participants must:

- ◆ Be male or female 2 to 21 years of age, inclusive.
- ◆ Have a diagnosis of SCN8A-DEE, supported by both clinical and genetic findings.
- ◆ Have on average at least 1 countable motor seizure (defined as a generalized tonic-clonic seizure [aka grand mal seizure], tonic, atonic or focal [aka partial] onset seizures with noticeable motor component [e.g., involving muscle contractions, a lack of movement, etc.]) per week and not be seizure-free for more than 20 consecutive days.
- ◆ Be receiving treatment with at least 1 other anti-seizure medication (ASM), but no more than 4 ASMs.
- ◆ Have failed to achieve seizure freedom with at least 2 previously tried ASMs.
- ◆ Not have symptoms that would be more consistent with another epilepsy disorder such as Dravet syndrome (e.g., fever-induced episodes of status epilepticus, frequent myoclonic seizures, worsening on sodium channel blockers, absence seizures with generalized spike-and-wave electroencephalogram [EEG] as the sole seizure type).
- ◆ Not be currently taking cannabinoids (other than Epidiolex®/Epidyolex®) or systemic steroids.

There are additional eligibility requirements, which the study doctor can explain to interested individuals.

## Where Can I Learn More about the Kayak Study?

To learn more, visit [KayakStudy.com](http://KayakStudy.com).

