

# Epilepsy DISEASE-STATE FACT SHEET

## Epilepsy Overview

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| Epilepsy is the fourth most common neurological condition | |
| Affects more than  65 million people worldwide |  |
| At least  3.4 million people  in the U.S. live  with seizures | including  470,000  children |
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Epilepsy—one of the most common neurological disorders—is characterized by abnormal electrical activity in the brain that leads to seizures.

Seizures can be:

* Brief lapses of attention
* Brief episodes of involuntary movement in part of   
  the body (partial)
* Prolonged convulsions of the entire body (generalized).

Seizures are unpredictable, and can vary in frequency, from less than one per year to several per day.

Epilepsy, and particularly childhood epilepsy, not only impacts individuals, but may also impact their families and communities who must adapt and come together to help patients cope with and manage their disease.

### Who is at Risk for Developing Epilepsy?

Anyone of any age can develop epilepsy, but it is more common in young children or older adults. The cause of the disease is unknown in about 50% of cases globally.



NOT ACTUAL PATIENT

\**Active Epilepsy is defined as diagnosed epilepsy, with patient on medication and experiencing one or more seizures per year.*

### Rare Pediatric Epilepsy

Epilepsy affects children at different ages and to different degrees, from mild to severe. According to the latest estimates, about 0.6% of all children aged 0–17 years have \*active epilepsy.

A rare epilepsy is one that:

* Affects fewer than 200,000 people in the   
  United States.
* Might be part of a disorder or syndrome that is defined by a particular cause, certain type of seizure(s) and/or refractory seizures, and a specific constellation of symptoms affecting other systems in the body.
* In addition to seizures, symptoms of these rare epilepsies may include low muscle tone, ataxia (lack of muscle coordination), epileptic encephalopathy, and intellectual and developmental delays.

Add “Not actual patient” to the photo

Rare pediatric epilepsy has few or limited therapeutic options available, which makes this an area of significant unmet medical need.

## Neurocrine Biosciences Epilepsy Portfolio

The Neurocrine Biosciences epilepsy portfolio consists of compounds in development to target rare epilepsies and epilepsies with limited existing treatment options.

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| NBI-921352  (XEN901) | NBI-921352 is a selective Nav1.6 sodium channel inhibitor in clinical development with potential in SCN8A developmental and epileptic encephalopathy (SCN8A-DEE) and other forms of epilepsy. Neurocrine Biosciences acquired exclusive rights to NBI-921352 from Xenon Pharmaceuticals, Inc.  NBI-921352 received a Rare Pediatric Disease Designation from the FDA. Neurocrine Biosciences intends to develop NBI-921352 for the treatment of pediatric SCN8A-DEE, as well as Focal-Onset Seizures, initially in an adult population. |
|  | *SCN8A-DEE is a rare, severe syndrome linked to gain-of-function mutations in the SCN8A gene that codes for the Nav1.6 sodium channel and affects how brain cells conduct electrical impulses in the brain. It is characterized by severe epilepsy, early onset developmental delay, cognitive impairment, and other medical challenges. Seizures associated with this syndrome are highly refractory to currently available antiseizure medication. The seizures, which begin occurring at a median age of four months, can vary in frequency, with some patients experiencing up to several per day. Over 90% of children with SCN8A-DEE are non-verbal, and half are not ambulatory. About 10% of people with SCN8A encephalopathy die from sudden unexpected death in epilepsy*. *There are currently no approved therapies for this form of pediatric epilepsy.*  *Focal-Onset Seizures*  *Focal or partial seizures begin in one area of the brain and are the most common type of seizures in adults. They can involve involuntary movements with alteration or loss of awareness and can last up to several minutes. These seizures can impact the ability of patients to carry out activities of daily living (ADLs) and can negatively impact quality of life.* |
| NBI-827104  (ACT-709478) | NBI-827104 is a selective, orally active and brain penetrating T-type calcium channel blocker that is in clinical development for the treatment of a form of rare pediatric epilepsy, known as epileptic encephalopathy with continuous spikes and waves during sleep (EE-CSWS). Neurocrine Biosciences acquired the exclusive rights to NBI-827104 from Idorsia Ltd.  NBI-827104 received an FDA IND acceptance for a Phase II study in CSWS, which is planned for 2021. |
|  | *EE-CSWS is a rare pediatric epilepsy that impacts approximately 7,000 children in the United States. Typical onset occurs between two and four years old with most seizures occurring during sleep and infrequently in the daytime. Diagnosis of CSWS is based on a unique electroencephalographic (EEG) pattern for electrical status epilepticus in sleep (ESES), together with cognitive stagnation and regression. There is currently no approved treatment for the disorder.* |

## Behind the Seizure®

Many rare pediatric epilepsy syndromes associated with global developmental delay and/or cognitive impairment in infancy or early childhood may be caused by single-gene mutations, such as SCN8A-DEE. Identifying the potential genetic underpinnings of different forms of epilepsy could support the development of more precision treatments.

To support research into the genetics of epilepsy, Neurocrine Biosciences is a sponsor of Invitae’s Behind the Seizure® program in the U.S. and Canada. This program provides free access to comprehensive testing for epilepsy-related genetic variations to any child under eight years old who has had an unprovoked seizure.

NOT ACTUAL PATIENT