# Retrospective non-interventional study of stiripentol use in Dravet patients in the USA (Retrospective US Study - STP228)

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# Administrative details

**Study description** 

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This is a retrospective, non-interventional, observational, multicentre chart review study to be conducted in patients who have a diagnosis of Dravet syndrome and received stiripentol for a minimum of 3 months. The goal of the study is to understand the management of Dravet patients who received stiripentol in the United States of America after the drug was made available. US sites where investigators are treating or have treated patients with Dravet syndrome will be selected, and investigators will be neurologists and epileptologists who have initiated stiripentol therapy in Dravet patients since 21-Aug-2018. This study will aim to collect data from 100 patients records from approximately 10 sites in the US.

#### **Study status**

Ongoing

Research institutions and networks

Institutions

Multiple centres: 10 centres involved in the study

### Contact details

Study institution contact

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Study contact

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**Primary lead investigator** 

#### Elaine WIRRELL

**Primary lead investigator** 

# Study timelines

#### Date when funding contract was signed

Actual: 08/03/2022

#### Study start date

Actual: 09/05/2022

#### Data analysis start date

Planned: 01/10/2023

#### **Date of final study report**

Planned: 01/01/2024

# Sources of funding

• Pharmaceutical company and other private sector

# More details on funding

**BIOCODEX GENTILLY** 

# Regulatory

Was the study required by a regulatory body?

#### Is the study required by a Risk Management Plan (RMP)?

Not applicable

# Methodological aspects

# Study type

# Study type list

#### Study type:

Non-interventional study

#### Scope of the study:

Drug utilisation

#### Main study objective:

The goal of the study is to understand the management of Dravet patients who received stiripentol in the United States after the drug was made available. The main study objectives are to: • Characterize the treatment patterns of stiripentol in the US following marketing authorization • Evaluate the efficacy of stiripentol treatment on seizure frequency • Estimate the impact of stiripentol

# Study drug and medical condition

#### Name of medicine

# **Study drug International non-proprietary name (INN) or common name**STIRIPENTOL

#### **Anatomical Therapeutic Chemical (ATC) code**

(N03AX17) stiripentol stiripentol

#### Medical condition to be studied

Severe myoclonic epilepsy of infancy

# Population studied

#### Age groups

Preterm newborn infants (0 - 27 days)

Term newborn infants (0 - 27 days)

Infants and toddlers (28 days - 23 months)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

#### **Estimated number of subjects**

100

# Study design details

#### **Outcomes**

Frequency decreased of bilateral convulsive, Frequency of status epilepticus

#### Data analysis plan

Statistical analyses will be descriptive

#### **Documents**

#### **Study publications**

De Liso P, Chemaly N, Laschet J, Barnerias C, Hully M, Leunen D, Desguerre I, C...

Thanh TN, Chiron C, Dellatolas G, Rey E, Pons G, Vincent J, Dulac O. Long-term

Chiron C. Stiripentol for the treatment of seizures associated with Dravet synd... Myers KA, Lightfoot P, Patil SG, Cross JH, Scheffer IE. Stiripentol efficacy an...

# Data management

# **ENCePP Seal**

The use of the ENCePP Seal has been discontinued since February 2025.

The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

#### Data sources

Data sources (types)

Other

Data sources (types), other

# Use of a Common Data Model (CDM)

#### **CDM** mapping

No

# Data quality specifications

#### **Check conformance**

Unknown

#### **Check completeness**

Unknown

#### **Check stability**

Unknown

#### **Check logical consistency**

Unknown

# Data characterisation

#### **Data characterisation conducted**

No