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

First published: 04/10/2023

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Study Ongoing

Administrative details

EU PAS number

EUPAS107005

Study ID

107006

DARWIN EU® study

No

Study countries

☐ United States

Study description

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?

No

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

Medicinal product name

DIACOMIT

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

Patient charts records

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