A Prospective, Observational, Post-Authorisation Efficacy Study to Assess Long-term Effectiveness of Risdiplam in Patients with Genetically Confirmed 5q SMA

First published: 29/06/2022

Last updated: 02/07/2024

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/48479

EU PAS number

EUPAS47916

Study ID

48479

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Germany
- ☐ Sweden
- ☐ Switzerland

Study description

This study is a multi-country, non-interventional, prospective, longitudinal cohort study that will use a hybrid method of 2 sources of data collection. The data will be extracted from existing Spinal Muscular Atrophy (SMA) patient registries as Secondary data use (SDU) and collected de novo from sites in countries not covered by the SDU under Primary data collection (PDC). Study countries will be primarily in Europe, although non-European countries (e.g. those in North America or Australasia) may be included to achieve the target sample size.

Study status

Ongoing

Research institutions and networks

Institutions

Evidera

☐ United Kingdom

First published: 20/11/2013

Last updated: 07/03/2024

Institution | Laboratory/Research/Testing facility | Non-Pharmaceutical company | ENCePP partner

TREAT-NMD Services Ltd

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Other

Translational Research in Europe for the Assessment and Treatment of Neuromuscular Disorders (TREAT-NMD) Europe, SMArtCARE Germany, Neuromuscular Diseases in SwedenSjukdomar i Sverige (NMiS) Sweden

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Nahila Justo

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 04/06/2021

Study start date
Planned: 30/06/2022

Actual: 27/06/2022

Date of interim report, if expected
Planned: 30/09/2025

Date of final study report
Planned: 30/12/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Hoffmann-La Roche

Study protocol

Prot BN43428 risdiplam v1, Published Output-1_Redacted.pdf(1.94 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Other study registration identification numbers and links

BN43428

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Main study objective:

The primary objectives are as follows: -To describe the real-world, long-term effectiveness of risdiplam on disease progression & to compare the impact of potential effect modifiers (symptomatic status, SMN2 copy number) on long-term effectiveness -To compare the real-world, long-term effectiveness

outcomes between a cohort of risdiplam-treated patients & cohort of DMT-naive patients

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

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

RISDIPLAM

Medical condition to be studied

Spinal muscular atrophy

Population studied

Age groups

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Hepatic impaired

Immunocompromised

Renal impaired

Estimated number of subjects

600

Study design details

Outcomes

- Time to all-cause mortality (survival) by the end of study participation - Prolonged/permanent ventilation-free survival - Developmental motor milestone achievement - Motor function assessed using Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), Hammersmith Functional Motor Scale Expanded (HFMSE), or Revised Upper Limb Module (RULM), - Time to onset of symptoms (within the pre-symptomatic group) - Time to need for nutritional support/tube feeding - Length of stay in hospitalisations and reasons for hospitalisations - Withdrawal of risdiplam treatment and reasons for withdrawal of treatment (risdiplam cohort only)

Data analysis plan

The raw data will be extracted from different data sources, followed by homogenization, pooling and then central analysis. All effectiveness outcomes will be summarized descriptively per cohort by an approximate 6-month timepoint. Continuous variables will be described with summary statistics such as n, mean, standard deviation, median, minimum, and maximum values. Also, treatment differences and 95% CIs will be presented. For each categorical variable, odds ratio, or relative risks, 95% CIs, frequency, and percentage will be reported. The time to event for each outcome will be reported in months,

summarised descriptively and analysed using Kaplan-Meier method and Cox proportional hazards regression model. Median survival time estimates and survival probabilities at each study period time point will be analysed. The other objective will be achieved by comparing the two cohorts and using statistical techniques like inverse probability weighting and multivariable regression analysis.

Data management

Data sources

Data source(s)

Translational Research in Europe - Assessment and Treatment of Neuromuscular Diseases

Longitudinal Data Collection from Patients with Spinal Muscular Atrophy (SMArtCARE)

Data source(s), other

Neuromuscular Diseases in Sweden – Neuromuskulära Sjukdomar i Sverige (NMiS) Sweden

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Data sources (types)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection

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