TyPed - Natalizumab for the treatment of pediatric-onset multiple sclerosis in Portugal

First published: 29/03/2019

Last updated: 01/04/2024

Study Finalised

Administrative details

EU PAS number

EUPAS28904

Study ID

29436

DARWIN EU® study

No

Study countries

☐ Portugal

Study description

The primary objective of the study is to characterize the efficacy of natalizumab in patients diagnosed with POMS who have been treated for > 12 months with natalizumab in Portugal. The secondary objective of the study is to characterize POMS patients treated with at least one natalizumab infusion in Portugal, in terms of safety and tolerability of the drug.

Study status

Finalised

Research institutions and networks

Institutions

Biogen

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Multiple centres: 6 centres are involved in the study

Contact details

Study institution contact

Study Director Biogen ctrr@biogen.com

Study contact

ctrr@biogen.com

Primary lead investigator

Study Director Biogen

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 13/12/2017

Study start date
Actual: 23/04/2018

Date of final study report
Actual: 10/12/2018

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biogen

Study protocol

PRT_TYS_16_11106_TyPed_Protocol_v1_clean8965103509623323372_Redacted.pdf
(371.25 KB)

Regulatory

Was the study required by a regulatory body?

No

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

Non-EU RMP only

Other study registration identification numbers and links

PRT-TYS-16-11106

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

The primary objective of the study is to characterize the efficacy of natalizumab in patients diagnosed with POMS who have been treated for > 12 months with natalizumab in Portugal.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

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

NATALIZUMAB

Medical condition to be studied

Multiple sclerosis

Population studied

Short description of the study population

Patients diagnosed with Pediatric-onset multiple sclerosis (POMS) who have been treated for > 12 months with natalizumab in Portugal.

To be eligible to participate in this study, candidates must meet the following eligibility criteria:

1. Ability to understand the purpose and risks of the study and provide signed and dated informed consent and authorization to use protected health information (PHI) in accordance with national and local privacy regulations or, if at inclusion date patient is less than 18 years old, signed informed consent obtained from patient’s legal representative (parent or guardian).
2. Diagnosis of MS as defined by the International Pediatric Multiple Sclerosis Study Group and by the McDonald criteria 2010.
3. Age under 18 years old at the beginning of treatment with natalizumab.
4. At least 1 infusion of natalizumab in the context of POMS

Age groups

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Special population of interest

Other

Special population of interest, other

Multiple sclerosis patients

Estimated number of subjects

21

Study design details

Outcomes

•Annual relapse rate (ARR)•Proportion of subjects with EDSS progression•Proportion of subjects relapsed•Association of demographics and disease characteristics with ARR and time to relapse•Proportion of patients with no gadolinium-enhancing lesions•Proportion of patients with no new or enlarging T2 hyperintense lesions•Proportion of patients with No Evidence of Disease Activity, •Incidence adverse events(AE) and serious adverse events, discontinuation due to an AE •Discontinuation due to Anti-John Cunningham virus + serostatus/ progressive multifocal leukoencephalopathy risk concern•Discontinuation due to anti-natalizumab positive antibodies detected•Number of subjects hospitalized/with ER visits/requiring IV steroid treatment/who visit specialists due to MS relapse

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Data analysis plan

A descriptive analysis of all collected data and endpoints will be performed at the end of the study. All categorical data will be described by their absolute and relative frequencies. Continuous variables will be described by their minimum, maximum, median, mean and standard deviations. In the analysis of effects of baseline demographics and clinical characteristics on ARR at 12 months and on time to relapse will be performed with a 95% significance level. Because the sample size was chosen based solely on availability and was not to power for any endpoint, all analyses are exploratory in nature.

Documents

Study results

TyPed Study Report_v3_20190125__Redacted.pdf(838.93 KB)

Data management

Data sources

Data sources (types)

Electronic healthcare records (EHR)

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

Unknown