

An international disease registry collecting data on manifestations, interventions and outcomes in patients with tuberous sclerosis complex - TOSCA

First published: 18/12/2012

Last updated: 02/07/2024

Study

Finalised

Administrative details

PURI

<https://redirect.ema.europa.eu/resource/36194>

EU PAS number

EUPAS3247

Study ID

36194

DARWIN EU® study

No

Study countries

Australia
Austria
Belgium
China
Denmark
Estonia
France
Germany
Greece
Israel
Italy
Japan

Korea, Republic of
Latvia
Lithuania
Netherlands
Norway
Poland
Portugal
Romania
Russian Federation
Slovakia
Slovenia
South Africa
Spain
Sweden
Taiwan
Thailand
Türkiye
United Kingdom

Study description

Tuberous Sclerosis Complex (TSC) is an autosomal dominant genetic disorder caused by inactivating mutations in the TSC1 or TSC2 genes. Lesions in TSC patients can occur in the brain, kidneys, heart, liver, lungs and skin, and can manifest with renal and or pulmonary complications, autism, mental retardation and epilepsy. There is common agreement that there are still gaps in understanding the course of TSC manifestations and their prognostic role, rare symptoms and co-morbidities, interventions, treatments and their outcomes, and quality of life. The registry will address many of these gaps by collecting data from patients across many countries worldwide. The data collected might influence and improve patient's treatment standards and flows. An additional purpose of this registry is to inform the research in TSC. Data on the long-term safety of the prescribed treatment with Votubia® (everolimus) in the licensed indications will be collected in a TOSCA safety sub-study.

Study status

Finalised

Research institution and networks

Institutions

Novartis Pharmaceuticals

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01/02/2024

Institution

Multiple centres: 250 centres are involved in the study

Contact details

Study institution contact

Novartis Clinical Disclosure Officer

Study contact

trialandresults.registries@novartis.com

Primary lead investigator

Novartis Clinical Disclosure Officer

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned:

04/01/2012

Actual:

04/01/2012

Study start date

Planned:

10/08/2012

Actual:

10/08/2012

Date of final study report

Planned:

10/08/2027

Actual:

12/06/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharmaceuticals

Study protocol

[CRAD001MIC03_protocol_v03_30Jul2014_Redacted.pdf](#)(4.93 MB)

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

CRAD001MIC03

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition
Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Other

Safety study (incl. comparative)

If ‘other’, further details on the scope of the study

To map TSC manifestations, identify rare symptoms/co-morbidities, record interventions and outcomes, define an evidence base for disease assessment/therapy, evaluate long-term safety of Votubia®

Data collection methods:

Combined primary and secondary data collection

Main study objective:

Registry: Map the course of TSC manifestations, identify rare symptoms and comorbidities, contribute to create an evidence base for disease assessment and therapy, inform research in TSC, measure quality of life, collect information on sexual maturation/endocrine assessments. PASS: Document long term safety of Votubia® in TSC patients treated in the licensed indications.

Study Design

Non-interventional study design

Cohort
Other

Non-interventional study design, other

Prescription event monitoring, Disease Registry, Post-Authorization Safety Study

Study drug and medical condition

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

EVEROLIMUS

Medical condition to be studied

Tuberous sclerosis complex

Population studied

Short description of the study population

Patients were eligible for inclusion in the TOSCA PASS if they met all of the following criteria:

1. Patients participating in the TOSCA disease registry.
2. Patients on treatment with everolimus prescribed for the licensed indications in the

European Union (EU).

3. Patients had to sign the TOSCA PASS informed consent form (ICF) (parental/guardian consent, if applicable) before any data or information was provided into the safety sub-study.

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)

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

Other

Special population of interest, other

Tuberous sclerosis patients

Estimated number of subjects

2000

Study design details

Outcomes

REGISTRY: Patients with each TSC manifestation, overall survival, incidence and prevalence of rare symptoms/ co-morbidities, frequency of interventions, outcome of manifestations, identification of scientific hypotheses. PASS: Incidence of adverse events (including important potential and identified risks), serious adverse events, Votubia®-related adverse events.

Data analysis plan

All patients enrolled in the registry will be considered in the analysis. Demographic and clinical parameters will be tabulated for the descriptive statistical analyses of relevant variables. Given the descriptive nature of this registry and the lack of a specific hypothesis to be tested, there is no formal sample size calculation. TOSCA PASS: The primary variable of the TOSCA PASS is the incidence of adverse events, serious adverse events and Votubia®-related adverse events in the observation period. The incidence of AEs and SAEs will be summarized by system organ class and preferred term using the MedDRA dictionary. Similar summaries will also be produced for treatment-related AEs and SAEs.

These listings will cover both events that occur during the on-treatment and post-treatment period. Other variables are the following: • Incidence of events of special interest, • Everolimus blood concentration if available.

Documents

Study results

[RAD001MIC03 final PASS CSR_Redacted.pdf\(439.79 KB\)](#)

Data management

Data sources

Data sources (types)

[Electronic healthcare records \(EHR\)](#)

[Other](#)

Data sources (types), other

The data for this study will be retrieved from hospital discharge files, clinical records, electronic medical records, patients' questionnaires, and ad hoc clinical databases.

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