TAK-755-4007: A Post-Authorization Safety Study (PASS) to Further Evaluate Real-World Safety in Patients with Congenital Thrombotic Thrombocytopenic Purpura (cTTP) Treated with Adzynma

First published: 14/01/2026

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Study Ongoing

Administrative details

EU PAS number

EUPAS1000000870

Study ID

1000000870

DARWIN EU® study

No

Study countries

Austria

France

Germany
Italy
Poland
Spain
Switzerland
United Kingdom

Study description

Congenital thrombotic thrombocytopenic purpura (cTTP) is a rare blood disorder that some people are born with. It is caused by a problem in a gene passed down from parents to children, which affects the body’s ability to produce a enzyme called ADAMTS13.

This enzyme helps to cut down a larger form of protein called von Willebrand Factor (VWF). People with cTTP have low levels of ADAMTS13.

Without ADAMTS13, large forms of vWF build up and cause blood clots in small blood vessels. These clots can block blood flow to vital organs, causing serious health problems. Adzynma is a human ADAMTS13 protein made in the laboratory. It works the same way as natural ADAMTS13 does and may provide higher levels of ADAMTS13.

The main aim of this study is to learn more about the risk of children and adults with cTTP treated with Adzynma developing antibodies that prevent Adzynma from working properly (called neutralizing antibodies) within 6 months after the first treatment and to understand the risk of allergic reactions within 7 days of the first treatment with Adzynma. Other aims are to better understand how safe treatment with Adzynma is over a longer period of time (called long-term safety) in children and adults with cTTP and to gather information about pregnancies and babies of women who have received Adzynma while pregnant.

Only data already available in the medical records of the people who received

Adzynma for the treatment of cTTP in normal clinical practice will be reviewed and collected during this study.
Only data already available in the medical records of the participants who received Adzynma for the treatment of cTTP will be reviewed and collected during this study.

Study status

Ongoing

Contact details

Study institution contact

Study Contact Takeda TrialDisclosures@takeda.com

Study contact

TrialDisclosures@takeda.com

Primary lead investigator

Study Contact Takeda

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 13/09/2024

Actual: 13/09/2024

Study start date

Planned: 31/05/2026

Actual: 26/05/2026

Date of final study report
Planned: 01/12/2030

Sources of funding

More details on funding

Takeda

Study protocol

TAK-755-4007-clinical-study-protocol-redact.pdf (1.54 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

This non-interventional, retrospective, post-authorization safety, cohort study utilizes secondary data from medical records to evaluate the safety of Adzynma in real-world clinical practice.

Main study objective:

The main objective of this study is to learn more about the risk of children and adults with cTTP treated with Adzynma developing antibodies that prevent Adzynma from working properly (called neutralizing antibodies) within 6 months after the first treatment and to understand the risk of allergic reactions within 7 days of the first treatment with Adzynma.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

ADZYNMA

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

RADAMTS13

Anatomical Therapeutic Chemical (ATC) code

(B01AD13) apadamtase alfa and cinaxadamtase alfa

apadamtase alfa and cinaxadamtase alfa

Medical condition to be studied

Congenital thrombotic thrombocytopenic purpura

Population studied

Short description of the study population

Pediatric and adult participants who have received Adzynma for the treatment of cTTP, either prophylactically or as on-demand therapy for acute episodes- including those who became pregnant during Adzynma treatment.

Age groups

- **Paediatric Population (< 18 years)**
- **Adult and elderly population (≥18 years)**

Estimated number of subjects

50

Study design details

Outcomes

The primary outcomes will assess

1. Number of Participants With Hypersensitivity Reactions Following the Index Adzynma Infusions

2. Number of Participants With Neutralizing Antibodies to ADAMTS13 Following the Index Adzynma Infusions

The secondary outcomes will assess

3. Number of Participants With Long Term Safety Risk of Hypersensitivity Reactions Following the Index Adzynma infusion

4. Number of Participants With Risk of Hypersensitivity Reactions After Each Administration of Adzynma Following the Index Infusion

5. Number of Participants With Treatment-emergent Adverse Events (TEAEs)

6. Gestational age at the Time of Infant Birth

7. Number of Participants With Spontaneous Abortion

8. Number of Participants With Stillbirth

9. Number of Participants With Induced Abortion

10. Number of Participants With Live Birth

11. Number of Participants With any Pregnancy Related Complications

12. Number of Participants Categorized According to Gestational age of Greater Than or Equal to (>=) 37 Gestational Weeks and Less Than (<) 37 Gestational Weeks at Birth

13. Number of Infants Categorized According to Year of Birth

14. Number of Infants With Normal Birth Weight

15. Number of Infants With Small for Gestational Age (SGA)

16. Number of Infants With Congenital Anomaly Detected at the Time of Birth

17. Number of Participants by Breastfeeding Status While Receiving Adzynma

18. Number of Infant Categorized by Growth and Development Outcomes.

Data analysis plan

The statistical analysis of the data will be primarily descriptive. Categorical variables will be presented as frequencies and percentages. Continuous variables will be presented as mean with standard deviation (SD) or standard error (SE) and range for normally distributed variables; and as mean and SD, median, interquartile range (IQR), and range for non-normally distributed variables.

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)

Electronic healthcare records (EHR)

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

Yes

CDM Mappings

CDM name

CDISC SDTM

CDM website

https://www.cdisc.org/standards/foundational/sdtm

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Yes