

# 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

**Last updated:** 16/03/2026

Study

Planned

## Administrative details

### EU PAS number

EUPAS1000000870

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### Study ID

1000000870


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### DARWIN EU® study

No

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### Study countries

 Austria

 France


 Germany

 Italy

 Poland

 Spain

 Switzerland

 United Kingdom

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## **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 an 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.

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### **Study status**

Planned

## Contact details

### **Study institution contact**

Study Contact Takeda [TrialDisclosures@takeda.com](mailto:TrialDisclosures@takeda.com)

**Study contact**

[TrialDisclosures@takeda.com](mailto: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

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### **Study start date**

Planned: 30/04/2026

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### **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

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### **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

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#### **Study type:**

## Non-interventional study

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### **Scope of the study:**

Safety study (incl. comparative)

### **Data collection methods:**

Secondary use of data

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### **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

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

RADAMTS13

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**Anatomical Therapeutic Chemical (ATC) code**

(B01AD13) apadamtase alfa and cinaxadamtase alfa  
apadamtase alfa and cinaxadamtase alfa

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**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.

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**Age groups**

- **Paediatric Population (< 18 years)**
  - **Adult and elderly population (≥18 years)**
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**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 ( $\geq$ ) 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.

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## **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

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**CDM website**

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

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## Data quality specifications

**Check conformance**

Unknown

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

Yes