22 March 2018
EMA/125720/2018
Committee for Medicinal Products for Human Use (CHMP)

# Assessment report for paediatric studies submitted according to Article 46 of the Regulation (EC) No 1901/2006

## Revolade

eltrombopag / eltrombopag olamine

Procedure no: EMEA/H/C/001110/P46/028

## Note

Assessment report as adopted by the CHMP with all information of a commercially confidential nature deleted.

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## Table of contents

# Introduction

On 21 December 2017, the MAH submitted a completed paediatric study for Revolade (eltrombopag), in accordance with Article 46 of Regulation (EC) No1901/2006, as amended.

A short critical expert overview has also been provided.

The MAH stated that the submitted paediatric studies do not influence the benefit risk for Revolade and that no consequential regulatory action is required. No changes to the paediatric information of the current EU Summary of Product Characteristics (SmPC) are proposed because of this study.

# 1. Scientific discussion

## *1.1. Information on the development program*

Eltrombopag is an orally bioavailable, small molecule thrombopoietin receptor agonist, approved for marketing in the EU through the centralised procedure on 15th March 2010.

Eltrombopag is indicated in the paediatric population for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients aged 1 year and above who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Two studies, the Phase II PETIT study (TRA108062) and Phase III PETIT2 study (TRA115450), formed the basis of the approval of eltrombopag in paediatric patients with chronic ITP in the EU. Study PETIT2 was considered the pivotal study while PETIT was presented as dose-finding supportive study. It is noted that the paediatric investigation plan (PIP) for the condition ITP (EMEA-000170-PIP01-07-M04) only discussed the PETIT study, which was conducted in compliance with an agreed paediatric investigation plan (EMEA-C-000170-PIP01-07-M03).

The hereby submitted study (CETB115BRU01) is an extension study conducted in Russia for pediatric chronic ITP patients who have previously been enrolled in the multicenter, multinational study PETIT2.

The MAH stated that study CETB115BRU01 "An extension study of Eltrombopag in pediatric patients with chronic immune (idiopathic) thrombocytopenia purpura (ITP)" is a stand alone study.

## *1.2. Information on the pharmaceutical formulation used in the study*

Two pharmaceutical formulations of eltrombopag have been approved in the European Union:

- 12.5 mg, 25 mg, 50 mg or 75 mg film-coated tablets for oral administration;
- 25 mg powder for oral suspension (PfOS) for oral administration.

No new data were submitted for the pharmaceutical formulation used in the study CETB115BRU01.

Study CETB115BRU01 was a single arm study where all enrolled patients received eltrombopag (tablets and/or PfOS).

## *1.3. Clinical aspects*

### 1.3.1. Introduction

The MAH submitted a final report for study CETB115BRU01: "An extension study of Eltrombopag in pediatric patients with chronic immune (idiopathic) thrombocytopenia purpura (ITP)".

### 1.3.2. Clinical study

### Clinical study CETB115BRU01:

"An extension study of Eltrombopag in pediatric patients with chronic immune (idiopathic) thrombocytopenia purpura (ITP)"

#### ➢ Description

Study CETB115BRU01 was a multicenter open-label Phase III extension study conducted in Russia to evaluate the long-term safety of eltrombopag in pediatric patients with chronic ITP who previously participated in the multinational study TRA115450 (PETIT2 study) and who had obtained clinical benefit from treatment with eltrombopag.

#### ➢ Methods

- ***Objective***

The primary objective of Study CETB115BRU01 was to provide continued treatment to pediatric subjects who completed the PETIT2 study and to describe the safety and tolerability of eltrombopag when administered to pediatric patients with previously treated chronic ITP.

No secondary or exploratory objectives were planned.

- ***Study design***

Study CETB115BRU01 was a multicenter open-label Phase III extension study conducted in Russia to evaluate the long-term safety of eltrombopag in pediatric patients with chronic ITP who previously participated in the multinational study TRA115450 (PETIT2 study) and who had obtained clinical benefit from the treatment. This study allowed dosing of eltrombopag at an individualized dose for each patient based upon platelet counts, being 50 Gi/L - 200 Gi/L the target platelet count range. The starting dose was based on the patient's dose at the end of the PETIT2 study, unless a dose adjustment was warranted due to platelet count. The maximum dose was 75 mg daily.

A screening period was followed by a single arm treatment period and a follow-up period in this study:

- A screening period of up to 28 days prior to Day 1 of treatment in this study.

- Open-label, single arm, dose-adjustment, long-term treatment period, in which patients continued treatment until at least one of the following criteria were met:
    - Patient reached 18 years of age: Patient was considered to have completed the study, and was discontinued from eltrombopag within 3 months of his/her 18th birthday.
    - Eltrombopag received local regulatory approval for pediatric chronic ITP.
- Follow-up period. All patients had to complete the 4-week follow-up period, as described below, as part of their participation in this study:
    - Patients who were elected not to continue treatment with eltrombopag (i.e., nonstudy treatment) after completion of the study treatment period, had weekly follow-up visits for 4 weeks after the last dose of eltrombopag.
    - Patients who continued treatment with eltrombopag (i.e., non-study treatment) after completion of the study treatment periods had a follow-up visit 4 weeks after the last dose of eltrombopag.

- ***Study population /Sample size***

*Study population*

Patients were eligible if they were pediatric patients with chronic ITP who previously participated in thePETIT2 Study and fully completed that study. No sample size re-estimation was planned for this study. The main exclusion criteria were any serious and/or unstable pre-existing medical, psychiatric disorder, or other conditions that could interfere with the patient's safety or compliance to the study procedures.

*Sample size*

Maximum twelve patients were planned to be enrolled in this study conducted in Russia across 4 centers. The sample size (n = 12) assumed by the MAH was based on the number of patients previously enrolled in the PETIT2 Study in that country.

- ***Treatments***

Study CETB115BRU01 was a single-arm study where all enrolled patients received eltrombopag (tablets or PfOS).

For patients between the ages of 6 to 17 years old, eltrombopag tablets was administered, however, patients between the ages of 6 to 11 years old may use PfOS if they have difficulty swallowing tablets and are receiving a dose of eltrombopag of < 40 mg. For patients between the ages of 1 to 5 years old, either eltrombopag tablets or PfOS was administered. Any change between PfOS and tablet formulations should take into account the differences in bioavailability between formulations. When switching from PfOS to tablets, the nearest tablet strength rounding up should be used.

This study allowed dosing of eltrombopag at an individualized dose for each patient based upon platelet counts, being 50 Gi/L - 200 Gi/L the target platelet count range. The starting dose was based

on the patient's dose at the end of the PETIT2 study, unless a dose adjustment was warranted due to platelet count. The maximum dose was 75 mg daily.

- ***Outcomes/endpoints***

The primary endpoint was the assessment of long-term safety. The frequency of all AEs was the primary variable (endpoint).

Safety assessments included clinical laboratory evaluation (hematology, clinical chemistry, urinalysis), vital signs, pregnancy, physical findings and other observations related to safety such as liver exams and ocular exams (cataracts) and frequency of all adverse events, categorized using Common Terminology Criteria for Adverse Events (CTCAE) toxicity grades. Any abnormal findings were recorded in the appropriate CRF form, e.g., Medical History or AE/SAE form.

The endpoints were summarized utilizing the All Treated Subjects (ATS) analysis set.

The efficacy was not formally evaluated during this study. Patients could continue treatment as per protocol defined criteria as long as clinical benefit was evident.

- ***Statistical Methods***

*Planned analysis*:

No formal statistical hypothesis tests were planned.

All patients who receive at least one dose of study medication were included in the All Treated Patients (ATS) analysis set population.

No data sets were planned for this study.

As it was anticipated that accrual would be spread thinly across centers and summaries of data by center would be unlikely to be informative, data from all participating centers were pooled prior to analysis.

As this is a single arm study, there are no treatment comparisons.

Categorical data were presented as frequencies and percentages. For continuous data, n, mean, standard deviation, median, minimum, and maximum were presented. Demographic and baseline characteristics were summarized.

As the duration of treatment for a given patient would depend on efficacy and tolerability, the duration of follow-up would vary between patients. Consequently there was no imputation for missing data.

No interim statistical analyses were planned for this study.

Results

- ***Recruitment/ Number analysed***

A total of 9 out of 12 patients planned to be included into the extension study CETB115BRU01 received eltrombapag treatment. Four patients (44.4%) completed the study while 5 patients (55.6%) discontinued from treatment and withdrew from the study.

The main reasons for discontinuation/withdrawal from treatment were decision by patient or proxy (in 2 patients), lack of efficacy (in 2 patients) and adverse event (1 patient).



**Table 10-1 Subject disposition (All treated subjects)**

| Disposition variable | All subjects N=9 n (%) |
|---|---|
| Subjects treated | |
| Treated | 9 (100) |
| Discontinued from treatment | 5 (55.6) |
| Reason for discontinuation | |
| Decision by subject or proxy | 2 (22.2) |
| Lack of efficacy | 2 (22.2) |
| Adverse event | 1 (11.1) |
| Subject study status | |
| Subject completed the study | 4 (44.4) |
| Subject withdrew from the study | 5 (55.6) |
| Reason for withdrawal | |
| Lack of efficacy | 2 (22.2) |
| Withdrew consent | 2 (22.2) |
| Adverse event | 1 (11.1) |

- ***Baseline data***

The minimum age of the patients in the study was 4 years and the maximum age was 15 years. The median age was 9.0 years.

Approximately half of the patients were female (4 patients) and half were male (5 patients).

All of the patients in this study were white.

**Table 11-1 Demographics and baseline characteristics (All treated subjects)**

| Demographic Variable | All subjects N=9 |
|---|---|
| Age (years) | |
| n | 9 |
| Mean (SD) | 9.7 (3.81) |
| Median | 9.0 |
| Min-Max | 4-15 |
| Sex-n (%) | |
| Female | 4 (44.4) |
| Male | 5 (55.6) |
| Race-n (%) | |
| White - White/Caucasian/European Heritage | 9 (100) |
| Weight (kg) | |
| n | 9 |
| Mean (SD) | 45.2 (21.81) |
| Median | 47.0 |
| Min-Max | 15-73 |

Relevant medical history and current medical conditions are listed below:

Relevant medical history and current medical conditions
(All treated subjects)

| Country/ Subject identifier | Age/ Sex/ Race | REPORTED | Medical history or condition status code* | Medical condition classification code** |
|---|---|---|---|---|
| [redacted] | 12/M/WH | ADIPOSITY | 1 | |
| | | ATOPIC DERMATITIS | 1 | |
| | | DYSKINESIA OF THE BILE DUETS | 1 | |
| | | DYSLIPIDEMIA | 2 | |
| | | EROSIVE ESOPHAGITIS | 2 | |
| | | EROSIVE GASTRODUODERITIS | 2 | |
| | | REACTIVE PANCREATITIS | 1 | |
| | | RIGHT KNEE INJURY | 2 | |
| | | VEGETORASCULAR OF THE NASAL MUCOSA | 1 | |
| [redacted] | 15/M/WH | ATOPIC DERMATITIS | 1 | |
| [redacted] | 6/M/WH | ANEMIA GRADE 2 | 1 | |
| [redacted] | 6/F/WH | ACUTE VIRAL HEPATITIS A | 1 | 41 |
| | | ALCOHOLIC LIVER DISEASE | 5 | 41 |
| | | AUTOIMMUNE HEPATITIS | 5 | 41 |
| | | CHRONIC HEPATITIS B | 5 | 41 |
| | | CHRONIC HEPATITIS C | 5 | 41 |
| | | CYTOMEGALOVIRUS HEPATITIS | 5 | 41 |
| | | DRUG ALLERGIES | 5 | OT |

*1=Current; 2=Past; 5=No medical condition
**41=Liver disease medical conditions; 42=Drug related liver disease medical conditions; 43=Other liver disease medical conditions; OT=Other (general)

Relevant medical history and current medical conditions
(All treated subjects)

| Country/ Subject identifier | Age/ Sex/ Race | REPORTED | Medical history or condition status code* | Medical condition classification code** |
|---|---|---|---|---|
| [redacted] | 6/F/WH | DRUG RELATED LIVER DISEASE | 5 | 42 |
| | | EPSTEIN BARR VIRUS INFECTIOUS MONONUCLEOSIS | 5 | 41 |
| | | FATTY LIVER | 5 | 41 |
| | | GALLBLADDER DISEASE | 5 | 41 |
| | | HEMOCHROMATOSIS | 5 | 41 |
| | | HEPATIC CIRRHOSIS | 5 | 41 |
| | | HEPATITIS E IGM ANTIBODY | 5 | 41 |
| | | HERPES SIMPLEX HEPATITIS | 5 | 41 |
| | | INFLAMMATORY BOWEL DISEASE | 5 | OT |
| | | LIVER METASTASES | 5 | 41 |
| | | LUPUS | 5 | OT |
| | | NON-ALCOHOLIC STEATOHEPATITIS | 5 | 41 |
| | | PSORIASIS | 5 | OT |
| | | RHEUMATOID ARTHRITIS | 5 | OT |
| | | SJOGRENS SYNDROME | 5 | OT |
| | | THYROID DISEASE | 5 | OT |
| | | VITILIGO | 5 | OT |

*1=Current; 2=Past; 5=No medical condition
**41=Liver disease medical conditions; 42=Drug related liver disease medical conditions; 43=Other liver disease medical conditions; OT=Other (general)

- ***Efficacy results***

The efficacy was not formally evaluated during this study.

- ***Safety results***

*Patient exposure*

The median (Q1,Q3) duration of exposure to Eltrombopag was 24.6 (20.3, 46.2) months. All patients received eltrombopag for at least 3 months. More than half of patients (6/9 patients, 66.7%) received Eltrombopag for at least 24 months. Only 1 patient received eltrombopag for up to 47.5 months.

**Table 12-1 Duration of exposure to Eltrombopag (All treated subjects)**

| | All subjects N=9 |
|---|---|
| Total number of subjects receiving eltrombopag-n (%) | 9 ( 100) |
| Duration of exposure (months) | |
| Mean (SD) | 28.5 (16.80) |
| Median | 24.6 |
| Q1-Q3 | 20.3-46.2 |
| Min-Max | 3-48 |
| Duration of exposure (months) categories-n (%) | |
| Less than 1 | 0 |
| At least 1 | 9 ( 100) |
| At least 3 | 9 ( 100) |
| At least 6 | 8 ( 88.9) |
| At least 12 | 8 ( 88.9) |
| At least 24 | 6 ( 66.7) |
| At least 36 | 4 ( 44.4) |
| At least 48 | 1 ( 11.1) |
| At least 60 | 0 |

*Adverse events*

Eight out of 9 (88.9%) patients reported at least one adverse event.

The most commonly affected SOCs were infections and infestations (in 4 patients), nervous system disorders (in 2 patients), and respiratory, thoracic and mediastinal disorders (in 2 patients).

The most common AEs by preferred term were nasopharyngitis (in 3 patients), epistaxis (in 2 patients), and headache (in 2 patients).

Only 1 patient experienced an AE that was assessed as Grade 3 in severity. This was an autoimmune hepatitis (SOC “Hepatobiliary disorders”).

None of the reported AEs were considered to be related to eltrombopag.

**Table 12-2 Adverse events by system organ class and maximum severity (All treated subjects)**

| | All subjects (N=9) | |
|---|---|---|
| System organ class | All grades n (%) | Grade ≥3 n (%) |
| Number of subjects with at least one event | 8 ( 88.9) | 1 ( 11.1) |
| Infections and infestations | 4 ( 44.4) | 0 |
| Nervous system disorders | 2 ( 22.2) | 0 |
| Respiratory, thoracic and mediastinal disorders | 2 ( 22.2) | 0 |
| Eye disorders | 1 ( 11.1) | 0 |
| Hepatobiliary disorders | 1 ( 11.1) | 1 ( 11.1) |
| Investigations | 1 ( 11.1) | 0 |
| Metabolism and nutrition disorders | 1 ( 11.1) | 0 |

Numbers (n) represent counts of subjects.
A subject with multiple severity grades for an AE is only counted under the maximum grade.
Only AEs occurring during treatment or within 30 days of the last study treatment are reported.
MedDRA version 19.0, CTCAE version 4.03.

**Table 12-3 Adverse events by preferred term and maximum severity (All treated subjects)**

| | All subjects (N=9) | |
|---|---|---|
| Preferred term | All grades n (%) | Grade ≥3 n (%) |
| Number of subjects with at least one event | 8 ( 88.9) | 1 ( 11.1) |
| Nasopharyngitis | 3 ( 33.3) | 0 |
| Epistaxis | 2 ( 22.2) | 0 |
| Headache | 2 ( 22.2) | 0 |
| Autoimmune hepatitis | 1 ( 11.1) | 1 ( 11.1) |
| Blood bilirubin increased | 1 ( 11.1) | 0 |
| Iron deficiency | 1 ( 11.1) | 0 |
| Pharyngitis | 1 ( 11.1) | 0 |
| Scleral haemorrhage | 1 ( 11.1) | 0 |
| Tonsillitis | 1 ( 11.1) | 0 |

Numbers (n) represent counts of subjects.
A subject with multiple severity grades for an AE is only counted under the maximum grade.
Only AEs occurring during treatment or within 30 days of the last study treatment are reported.
MedDRA version 19.0, CTCAE version 4.03.

There were no deaths during the study.

A total of 3 patients (33.3%) experienced serious adverse events (SAEs). In 2 patients SAEs were associated with haemorrhagic symptoms (one case of epistaxis and one case of scleral haemorrhage). Both SAEs were resolved and the dose of the drug was not changed.

One patient experienced SAE of autoimmune hepatitis that led to treatment discontinuation. The patient narrative for this event is shown below:

**Patient** [redacted] ***(Required hospitalization or prolongation of existing hospitalization)***

**Drug and dose**: Eltrombopag, 75 mg per day

**Patient details**: 6 years, female

**Event:** The subject received oral Eltrombopag from 29 July 2013 to 26 October 2013. The subject received no concomitant medication. On 26 October 2013, the subject developed grade 3 or severe acute hepatitis A. The subject was hospitalized. The subject also experienced vomiting, sickness and icterus of "biolousness of sclears". Clinical blood tests and biochemical tests showed platelet count 284, white blood cells 9.5, neutrophil 72, ALT 344 u/L and AST 307 u/L. Biopsy was not done. Treatment with Eltrombopag was discontinued on 26 October 2013 and the subject was withdrawn from the study. The event was unresolved at time of reporting. The investigator considered that there was no reasonable possibility that the acute hepatitis a may have been caused by Eltrombopag and that the event was possibly due to acute viral hepatitis A (preliminary diagnosis).

Follow up information received on 30 October 2013: The event term was changed from acute hepatitis A to autoimmune hepatitis, as hepatitis A, B and C antibodies were not identified. Follow up information received on 05 November 2013: No relevant medical conditions were reported for the subject. The investigator confirmed autoimmune hepatitis as the final diagnosis. The subject was discharged from hospital on 01 November 2013, and at this point the autoimmune hepatitis was not resolved. Follow up information received on 14 November 2013: The event resolved on 13 November 2013.

Follow up information received on 07 November 2013 and 15 November 2013: The subject was enrolled in study TRA115450 from 22 May 2012. During this study the subject felt better and had hemorrhagic syndrome which stopped with the study. In July 2013 the subject was enrolled in the TRA117366 study. After 2 weeks of treatment the dose was increased by 25 mg until the subject received 75 mg per day. On the 10 October 2013 platelets were 110 GI/L.

On the 26 October 2013 subject was hospitalized at children infectious clinic with vomiting, sickness and icterus of biliousness of scleras. Investigational product was stopped the same day. Clinical tests and biochemical tests showed platelets 284 GI/L, white blood cells 9.5, neutrophils 7.2, ALT 344 u/l and AST 307 u/l. Biopsy was not performed. Preliminary diagnosis was Hepatitis A. On 27 October 2013 platelets were 338 GI/L and 28 October 2013 platelets 522 GI/L. Other tests performed on 28 October 2013 for HBV, HAV, HCV, VEB, CMV, HBcorAg and HBsAg were negative. Retest on 29 October 2013 included ALT 320 u/l, ACT 200 u/l, bilirubin 12.3 mmol/l and platelets 586 GI/L. Subject discharged on 01 November 2013. Autoimmune Hepatitis not recovered as on 31 October 2013 the ALT 265 u/l, AST 177 u/l, bilirubin 6.4 mmol/l (ULN 21mmol/l) and platelets 521 GI/L.

Autoimmune Hepatitis resolved 13 NOV 2013. Retest on 13 November 2013 showed AST 21 u/l, ALT 19 u/l and platelets 245 GI/L. Follow up reconciliation report received on 29 Mar 2017: The subject started receiving oral Eltrombopag from 29 Jul 2013 to 15 Aug 2013 at a dose of 50 mg QD. On 16 Aug 2013, the dose of the study medication was adjusted to 62.5 mg QD due to low effect of IP. On 30 Aug 2013, the dose of the study medication was adjusted to 75 mg QD due to low effect of IP. The subject received oral Eltrombopag from 30 Aug 2013 to 26 Oct 2013 at a dose of 75 mg QD

None of the SAEs were considered to be related to eltrombopag by the investigator.

There were no unexpected safety findings in the pediatric patients with chronic ITP who had previously participated in the PETIT2 Study).

### 1.3.3. Discussion on clinical aspects

Study CETB115BRU01 was a multicenter, single-arm, open-label Phase III extension study conducted in Russia to evaluate the long-term safety of eltrombopag in paediatric patients with chronic ITP who previously participated in the multinational study TRA115450 (PETIT2 study) and who had obtained clinical benefit from treatment with eltrombopag. The primary objective of study CETB115BRU01 was to provide continued treatment to paediatric patients who completed the PETIT2 study and to describe the safety and tolerability of eltrombopag when administered to paediatric patients with previously treated chronic ITP. The study design appears adequate to describe the long-term safety of eltrombopag in this setting.

PETIT2 study was a multicenter, multinational (including 4 centers in Russia) Phase III pivotal study to support the approval of eltrombopag in paediatric patients with chronic ITP in the EU. It is noted that this pivotal study was not discussed in the agreed paediatric investigation plan (PIP) for the condition ITP (EMEA-000170-PIP01-07-M04).

The maximum sample size (n=12) of the hereby submitted study (CETB115BRU01) was based only on the number of patients previously enrolled in the study PETIT2 in Russia across 4 centers.

A total of 9 out of 12 patients planned to be included into the extension study CETB115BRU01 received eltrombapag treatment. Four patients (44.4%) completed the study while 5 patients (55.6%) discontinued from treatment and withdrew from the study. The main reasons for discontinuation/withdrawal from treatment were decision by patient or proxy (in 2 patients), lack of efficacy (in 2 patients) and adverse event (1 patient).

Data from all participating centers were pooled prior to analysis.

A total of 8 patients out of 9 (88.9%) reported at least one adverse event. According to the MAH, the incidence of AEs in the hereby submitted study corresponds with the results from patients who received eltrombopag in PETIT2 study (81.0%).

There were no deaths during the study.

A total of 3 patients (33.3%) experienced serious adverse events (SAEs). In 2 patients SAEs were associated with haemorrhagic symptoms (one case of epistaxis and one case of scleral haemorrhage). Both SAES were resolved without change in the dose of eltrombopag.

One patient experienced SAE of autoimmune hepatitis that led to treatment discontinuation.

None of the reported AEs or SAEs were considered to be related to eltrombopag by the Investigator. This consideration seems to be acceptable.

Additionally, there were no unexpected safety findings in the paediatric patients with chronic ITP who had previously participated in the PETIT2 study.

Overall, the safety profile appears consistent to that observed in the PETIT2 study, and continues to be consistent to the EU SmPC of eltrombopag.

## 2. Rapporteur's overall conclusion and recommendation

Eltrombopag (tablets or PfOS) has been authorised for oral use in the paediatric population for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patient aged 1 year and above who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

In accordance with Article 46 of Regulation (EC) No1901/2006, the MAH submitted a final report for study CETB115BRU01 and stated that this study is a standalone study.

Results obtained in this study were consistent to the EU SmPC. The scarcity of data from the few patients included in the follow-up study CETB115BRU01 do not provide relevant additional data that could change the view about the long-term safety profile of eltrombopag. Thus, on the basis of study results, it is considered that no further regulatory action is required.

☒ **Fulfilled:**

No regulatory action required.

## 3. Additional clarification requested

# Annex. Line listing of all the studies included in the development program

The studies should be listed by chronological date of completion:

## Non clinical studies

Not applicable

## Clinical studies

Product Name: Revolade Active substance: eltrombopag

| Study title | Study number | Date of completion | Date of submission of final study report |
|---|---|---|---|
| A three part, staggered cohort, open-label and double blind, randomized, placebo controlled study to investigate the efficacy, safety, tolerability and pharmacokinetics of eltrombopag, a thrombopoietin receptor agonist, in previously treated pediatric patients with chronic idiopathic thrombocytopenic purpura (ITP). | PETIT (TRA108 062) | February 2014 | 06 February 2015 |
| A two part, double-blind, randomized, placebo-controlled and openlabel study to investigate the efficacy, safety and tolerability of eltrombopag, a thrombopoietin receptor agonist, in previously treated pediatric patients with chronic immune (idiopathic) thrombocytopenic purpura (ITP). PETIT2: Eltrombopag in PEdiatric patients with Thrombocytopenia from ITP | PETIT (TRA115 450) | February 2014 | 06 February 2015 |
| An extension study of Eltrombopag in pediatric patients with chronic immune (idiopathic) thrombocytopenia purpura (ITP)" is a stand alone study | CETB115 BRU01 | 04 July 2017 | 21 December 2017 |