A multicenter, Phase 2a, open-label, non-randomized study evaluating the efficacy, safety, and tolerability of BIVV020 in adults with persistent/chronic immune thrombocytopenia (ITP)

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To evaluate the effect of BIVV020 on the durabilityof platelet response in participants withpersistent/chronic immune thrombocytopenia (ITP)Secondary• To assess the safety and tolerability of BIVV020• To assess the pharmacokinetics (PK) of BIVV020•…

Ethical review	Approved WMO
Status	Completed
Health condition type	Coagulopathies and bleeding diatheses (excl thrombocytopenic)
Study type	Interventional

Summary

ID

NL-OMON52011

Source
ToetsingOnline

Brief title
PDY16894

Condition

- Coagulopathies and bleeding diatheses (excl thrombocytopenic)
- Immune system disorders congenital
- Autoimmune disorders

Synonym
immune thrombocytopenia, ITP

Research involving

Human

Sponsors and support

Primary sponsor: Genzyme Europe BV
Source(s) of monetary or material Support: Genzyme Europe BV (Sanofi)

Intervention

Keyword: BIV0020, immune thrombocytopenia, open-label, phase 2a

Outcome measures

Primary outcome

Naïve participants: Proportion of participants with

a platelet count >=50 × 109/L at >=50% of scheduled

visits, or for participants with baseline platelet count

<15 × 109/L, a >=20 × 109/L increase in platelet

count from baseline at >=50% of scheduled visits,

without receiving rescue ITP therapy, as assessed

from Week 3 to Week 24.

• Participants who previously received sutimlimab:

Proportion of participants with maintenance of

platelet count >=30 × 109/L at >=50% of scheduled

visits, without receiving rescue ITP therapy, as

assessed from Week 3 to Week 24.

Secondary outcome

Standard clinical and laboratory parameters and

adverse events

• Plasma concentrations of BIVV020

• Response rate at Weeks 24 and 52, defined as

a platelet count >=50 × 109/L and a greater than

2 -fold increase from baseline, measured on

2 occasions at least 7 days apart, with the absence

of bleeding (bleeding is defined as bleeding with

a score >=2 on the WHO bleeding scale), and the

lack of combination ITP therapy during this period.

• Time from baseline to first platelet response,

defined as greater than or equal to each of the

following values: 50 × 109/L and 100 × 109/L

(confirmed by 2 measurements at least 7 days

apart)

• Proportion of participants who did not require

rescue therapy for an acute episode of

thrombocytopenia after Week 3

• Incidence and titer (if relevant) of anti-BIVV020

antibodies

Study description

Background summary

Individuals with ITP who are refractory to current therapies may respond to inhibition of the
proximal portion of the CP. This hypothesis is supported by data obtained using a first-generation
CP inhibitor, sutimlimab, which targets the active and inactive conformations of human serine

protease C1s. The current study will assess the efficacy, safety, and tolerability of a second-generation CP inhibitor, BIVV020. BIVV020 selectively targets the activated conformation C1s and has a prolonged half-life compared with sutimlimab, allowing for SC maintenance administration.

Study objective

To evaluate the effect of BIVV020 on the durability of platelet response in participants with persistent/chronic immune thrombocytopenia (ITP)

Secondary
• To assess the safety and tolerability of BIVV020
• To assess the pharmacokinetics (PK) of BIVV020
• To assess the response rate of treatment with BIVV020
• To assess the time to response
• To assess the effect of treatment with BIVV020 on the requirement for rescue ITP therapy
• To assess the immunogenicity of BIVV020

Study design

This is a Phase 2a open-label, non-randomized, international, multicenter study to evaluate the efficacy, safety, and tolerability of BIVV020 in adults with persistent/chronic primary ITP.
• The study will enroll approximately 12 participants: up to 6 participants who have previously received and responded to sutimlimab (BIVV009) in study TDR16218, as well as participants who have not previously received sutimlimab.

Intervention

An intravenous (IV) loading dose of BIVV020 at 50 mg/kg will be administered on Day 1, and will be followed by maintenance doses of 600 mg SC weekly starting on Day 8.

Study burden and risks

Risks and burdens related to blood collection, study procedures and possible

advers events.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Male and female participants >=18 years of age at the time of signing the informed consent
- Confirmed diagnosis of primary ITP; for participants who previously received sutimlimab in study TDR16218 (NCT03275454), a response to sutimlimab must have been obtained, as defined by platelet count >=30 × 10^9/L on 2 visits at least 7 days apart
- For participants who have not previously received sutimlimab: persistent/chronic ITP (ITP lasting for >= 6 months) and all the following conditions:

a) Platelet count <=30 × 10^9/L on 2 occasions at least 5 days apart during the Screening Period;
b) Lack of an adequate platelet count response (as defined by maintenance of sustained platelet count >=30 × 10^9/L in the absence of bleeding) to at least 2 ITP treatments, 1 of which was a thrombopoietin receptor agonist. Other ITP treatments include: IVIg, anti-D immunoglobulin, corticosteroids, splenectomy, rituximab, cyclophosphamide, azathioprine, danazol, cyclosporin A, mycophenolate mofetil, or fostamatinib;
c) If receiving weekly thrombopoietin receptor agonist dosing, the last dose must have been administered >=7 days before the first dose of BIVV020. If receiving daily thrombopoietin receptor agonist dosing, the last dose must have been administered >=24 hours before the first dose of BIVV020;
d) If applicable, concurrent administration of ITP medications (eg. corticosteroids, IVIg, azathioprine, danazol, cyclosporin A, mycophenolate mofetil, or thrombopoietin receptor agonists) is acceptable provided the patient has been on a stable dose for at least 1 month;
e) If previously dosed with rituximab, the last dose of rituximab must have been administered at least 12 weeks before the first dose of BIVV020
- Documented vaccinations against encapsulated bacterial pathogens (Neisseria meningitidis, including serogroup B where available, Haemophilus influenzae, and Streptococcus pneumoniae) within 5 years of enrollment
- Contraceptive use for women of childbearing potential and men who are sexually active with a female partner of childbearing potential

Exclusion criteria

Participants are excluded from the study if any of the following criteria apply:
- Clinically significant medical history or ongoing chronic illness that would jeopardize the safety of the participant or compromise the quality of the data derived from his/her participation in the study
- Clinical diagnosis of SLE
- Clinically relevant infection within the month prior to enrollment
- History of venous or arterial thrombosis within the year prior to enrollment
- Secondary ITP from any cause including lymphoma, chronic lymphocytic leukemia, and drug-induced thrombocytopenia
- Positive hepatitis B surface antigen (HBsAg) or active HCV infection
- HIV infection
- Pregnant or lactating women
- Hemoglobin level <10 g/dL

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	10-05-2021
Enrollment:	1
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	BIVV020
Generic name:	not applicable

Ethics review

Approved WMO

Date:	23-02-2021
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden) metc-ldd@lumc.nl

Approved WMO

Date:	12-03-2021
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden)

	metc-ldd@lumc.nl
Approved WMO Date:	06-06-2021
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO Date:	24-06-2021
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO Date:	13-10-2021
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO Date:	25-11-2021
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
	metc-ldd@lumc.nl
Approved WMO Date:	19-04-2022
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2020-004162-18-NL
CCMO	NL75989.058.21

Study results

Date completed: 03-01-2023

Results posted: 14-11-2023

Actual enrolment: 2

First publication
01-09-2023