An Open-label Extension Study of ARGX-113-2009 to Evaluate the Long term Safety, Tolerability, and Efficacy of Efgartigimod PH20 SC in Adult Participants With Bullous Pemphigoid

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This study has been transitioned to CTIS with ID 2024-515832-59-00 check the CTIS register for the current data. To assess the long-term safety and tolerability of treatment with efgartigimod PH20 SC in participants with BP

Ethical review	Approved WMO
Status	Completed
Health condition type	Autoimmune disorders
Study type	Interventional

Summary

ID

NL-OMON56108

Source
ToetsingOnline

Brief title
BALLAD+

Condition

- Autoimmune disorders
- Skin and subcutaneous tissue disorders NEC

Synonym
Bullous Pemphigoid

Research involving
Human

Sponsors and support

Primary sponsor: argenx BV
Source(s) of monetary or material Support: industry

Intervention

Keyword: Bullous Pemphigoid, efgartigimod, extension, skin disease

Outcome measures

Primary outcome

• Incidence and severity of treatment-emergent adverse events (TEAEs), serious AEs (SAEs), and AEs of special interest (AESIs)

• Rate of treatment discontinuation because of safety concerns

Secondary outcome

To assess the long-term efficacy and durability of response with efgartigimod PH20 SC treatment in participants with BP :

• Proportions of participants who:

* Achieve complete remission (CR) and have been off oral corticosteroids (OCS) for >=8 weeks

* Achieve CR or partial remission (PR) and have been off OCS therapy for >=8 weeks

* Achieve CR and have been on minimal OCS therapy for >=8 weeks. (Minimal OCS therapy is defined as <=0.1 mg/kg a day of prednisone [or an equivalent dose of another OCS])

* Are in CR and have been off both OCS and efgartigimod PH20 SC for >=8 weeks

* Are in CR or PR and have been off both OCS and efgartigimod PH20 SC for >=8

weeks

• Duration of sustained remission

• Proportion of participants who relapse

• Time to relapse

• Incidence and severity of relapse

• Bullous Pemphigoid Disease Area Index (BPDAI) activity scores, Investigator Global Assessment of Bullous Pemphigoid (IGA-BP) scores, and itch numerical rating scale (NRS) over time

• Rate of treatment discontinuation due to efficacy concerns

Study description

Background summary

BP is a subepidermal AIBD that predominantly affects older adults. It is a chronic disease that significantly affects morbidity and QoL; additionally, the disease can worsen spontaneously, even when the patient is treated with the current standard of care. The pathogenesis of BP is driven by IgG and IgE autoantibodies against the hemidesmosomal proteins BP180 and BP230, acting as key antigens for pathogenic autoantibodies.

The current standard of care for BP is treatment with TCS or OCS, either of which can be combined with conventional immunosuppressant therapy. Unfortunately, corticosteroid therapy in patients with BP typically causes comorbidities, which can be severe and even life-threatening, especially in older adults.

In summary, there is currently an unmet medical need for new BP treatments that provide rapid CDA and remission, minimize (or even prevent) relapse, and reduce the burdens placed on patients caused by cumulative corticosteroid exposure. This OLE study aims to evaluate the safety, tolerability, long-term efficacy, immunogenicity, PRO measures, and PD of efgartigimod PH20 SC in adult participants with BP, who completed the antecedent study ARGX 113 2009.

Study objective

This study has been transitioned to CTIS with ID 2024-515832-59-00 check the CTIS register for the current data.

To assess the long-term safety and tolerability of treatment with efgartigimod PH20 SC in participants with BP

Study design

This is a phase 3, prospective, global, multicenter, OLE study to investigate the long-term safety, tolerability, efficacy, quality of life (QoL), pharmacodynamics (PD), and immunogenicity of efgartigimod PH20 SC in adult participants with BP who have completed ARGX-113-2009. All participants who complete the end-of-treatment period (EoTP) visit at week 36 in ARGX 113 2009 will be invited to enroll. The study will be conducted in the same sites as ARGX 113 2009.
In ARGX-113-2009, participants received efgartigimod PH20 SC or placebo with concurrent OCS, or rescue therapy (defined as concurrent BP therapy in this protocol). Depending on their clinical status at the time of rollover, participants may be treated with efgartigimod PH20 SC and participants who relapse during the study may also be treated with efgartigimod PH20 SC.

Intervention

At rollover, participants will be treated with efgartigimod PH20 SC according to their clinical status at the EoTP visit of ARGX-113-2009. A summary of the treatment interventions is provided in Table 5 in the protocol on page 38 -41

Study burden and risks

There is currently an unmet medical need for new BP treatments that provide rapid CDA and remission, minimize (or even prevent) relapse, and reduce the burdens placed on patients caused by cumulative corticosteroid exposure. This OLE study aims to evaluate the safety, tolerability, long-term efficacy, immunogenicity, PRO measures, and PD of efgartigimod PH20 SC in adult participants with BP, who completed the antecedent study ARGX 113 2009.

The favorable balance between the risks and anticipated efficacy/benefits supports the administration of efgartigimod PH20 SC to participants with BP in ARGX-113-2010.
More detailed information about the known and expected benefits and risks of efgartigimod*both as an IV formulation and as efgartigimod PH20 SC and reasonably expected AEs can be found in the current IB.

See also section 2.3 in the protocol for detailed information about the Benefit

and Risk Assessment.

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

1. Has completed the week 36 visit of ARGX-113-2009
2. Is capable of providing signed informed consent and complying with protocol requirements
3. Agrees to use contraceptive measures consistent with local regulations and the following:
a. Male participants: An acceptable method of contraception is a condom. All nonsterilized male participants must use this method from signing of the ICF until the date of the last dose of IMP.
b. Women of childbearing potential must have a negative urine pregnancy test at

baseline before receiving IMP. Women must use one of the contraception methods described in the protocol from signing the ICF until the last dose of IMP

Exclusion criteria

1. Clinically significant disease, recent major surgery (within 3 months of baseline), or intention to have surgery during the study; or any other medical condition that, in the investigator*s opinion would confound the results of the study or put the participant at undue risk
2. Known hypersensitivity to IMP or 1 of its excipients
3. Permanently discontinued IMP in ARGX-113-2009 due to an AE considered related to IMP and for whom the benefit/risk balance is not considered positive

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	03-08-2023
Enrollment:	2
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Efgartigimod
Generic name:	Efgartigimod

Ethics review

Approved WMO Date:	23-01-2023
Application type:	First submission
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	25-04-2023
Application type:	First submission
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	30-08-2023
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	13-09-2023
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	18-03-2024
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	04-04-2024
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	03-06-2024
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)
Approved WMO Date:	11-07-2024
Application type:	Amendment
Review commission:	METC Brabant (Tilburg)

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
EU-CTR	CTIS2024-515832-59-00
EudraCT	EUCTR2021-003063-10-NL
CCMO	NL83164.028.23