ballad

Bullous Pemphigoid Study

A Phase 2/3, Global, Multi-Center, Randomized, Double-Blinded, Placebo-Controlled, Parallel-Group Study Investigating the Efficacy and Safety of Efgartigimod PH20 SC in Adult Participants with **Bullous Pemphigoid (BP)**

About The Ballad Study

PART A	PART B
Phase 2 Proof-of-Concept 40 Participants	Phase 3 Confirmatory 120 Participants

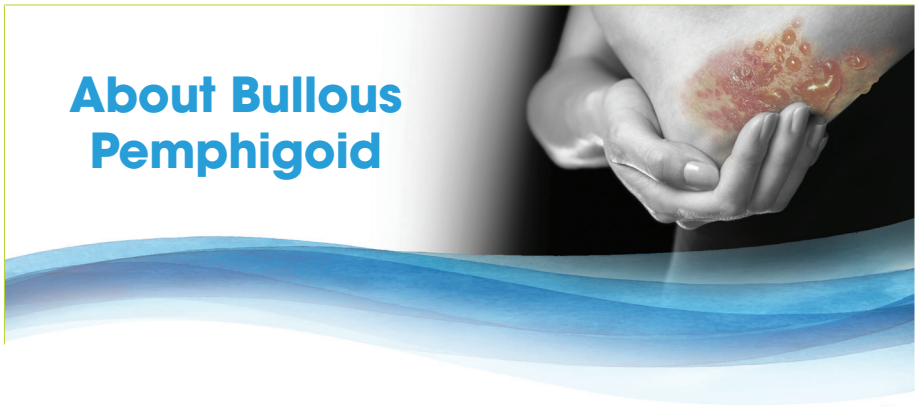
Parts A + B

- 1:1 randomization to efgartigimod or placebo
- Weekly subcutaneous injection, after receiving two injections on days 1 and 8
- Concurrent treatment with daily oral prednisone (or equivalent oral corticosteroid [OCS])
- At week 36, option to roll over to open-label extension (OLE) study or complete 7-week follow-up period

About Efgartigimod

Efgartigimod is an investigational drug currently being studied in a variety of disease states. Efgartigimod is currently not approved for use in BP by any regulatory agency, as efficacy and safety have not been established.

About Bullous Pemphigoid



BP is caused by the disruption of the dermoepidermal junction by pathogenic autoantibodies resulting in large, tense bullae on the skin, progressing to epidermal erosion, crusting and urticarial plaque formation.

Classification of moderate to severe BP - presence of bullae with or without the presence of urticarial (hives), eczematous, or erythematous plaques, and with or without pruritis.

- **Moderate:** Multiple (10 to 29) blisters or erosions and/or multiple erythematous, eczematous or urticarial lesions (involving 10-30% of the body surface area) and/or at the most, 2 large erosive areas (>5 cm) or large blisters (>5 cm).
- **Severe:** Extensive (≥30) blisters or erosions and/or extensive erythematous, eczematous or urticarial lesions (involving >30% of the body surface area) and/or 3 or more large erosive areas (>5 cm) or large blisters (>5 cm).

Please see clinicaltrials.gov NCT05267600 for more information about the study, including the full inclusion and exclusion criteria.

Primary Endpoint

- Complete remission while receiving efgartigimod PH20 SC or placebo and have been off OCS therapy for ≥8 weeks at week 36.

SCAN ME

Prohibited Treatments During the Study

- Topical corticosteroids, conventional immunosuppressants (e.g. azathioprine, cyclophosphamide, methotrexate, mycophenolate mofetil), or dapsone must be discontinued at the baseline visit; **no washout required.**
- Tetracyclines with or without nicotinamide at doses higher than the recommended daily allowance/dietary reference intake must be discontinued **within 2 weeks** of the baseline visit.
- Complementary therapies, such as traditional Chinese medicines, herbs, or procedures (e.g. acupuncture), must be discontinued within **4 weeks (or 5 half-lives)** of the baseline visit, if the investigator determines that such therapies may interfere with the study's efficacy assessments and/or potentially risk the safety of the participant.
- Sulfasalazine, IVIg, subcutaneous administration of immunoglobulin, immunoadsorption or plasma exchange must be discontinued **within 8 weeks** of the baseline visit.
- Other investigational product must be discontinued within **3 months (or 5 half-lives)** before the first dose of investigational medicinal product (IMP).
- Any monoclonal antibody (including rituximab or another anti-CD20 biologic) must be discontinued **within 6 months** of the baseline visit.

Sources: 1. Data on file argenx 2022. 2. https://clinicaltrials.gov/ct2/show/NCT05267600

Key Eligibility Criteria

INCLUSION

- ✔ Adults (≥18 years old) with a diagnosis of BP confirmed by positive histopathology and DIF before randomization to treatment assignment, and by positive serology (by IIF, CLEIA, or ELISA, according to local practice) at screening.
- ✔ Clinical signs of BP (ie, presence of bullae), with or without the presence of urticarial/eczematous/erythematous plaques or pruritus at the screening and baseline visits.
- ✔ Investigator Global Assessment of Bullous Pemphigoid (IGA-BP) score of 3 or 4 at screening and baseline, indicating moderate to severe BP.
- ✔ Karnofsky performance status of at least 60% at screening.
- ✔ Males: must agree to use an acceptable method of contraception from the time that the ICF is signed until the date of their last dose of IMP.
- ✔ Females: must agree to use a highly effective or acceptable contraception method from the time that the ICF is signed until the date of their last dose of IMP.

EXCLUSION

- ✖ Drug-induced BP or other forms of pemphigoid/autoimmune bullous diseases.
- ✖ Received unstable dose of treatments known to cause or exacerbate BP for at least 4 weeks prior to the baseline visit.
- ✖ Use of BP treatments other than OCS, topical corticosteroids, conventional immunosuppressants, or dapsone.
- ✖ History of malignancy unless deemed cured for ≥3 years except basal/squamous cell carcinoma, carcinoma in situ of cervix/breast, or prostate cancer (T1a or b) may be included at any time.
- ✖ Other serious diseases or recent surgery.
- ✖ Previously participated in a clinical study with efgartigimod.
- ✖ Active or chronic infection at screening.
- ✖ Primary or secondary hypogammaglobulinemia with total IgG serum levels <4 g/L at screening.
- ✖ Pregnant or lactating females.

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