Society for Investigative Dermatology (SID) 2021
Deadline: (Late Breaking Submission) March 31st, 2021

Title: Treating pemphigus vulgaris (PV) and foliaceus (PF) by inhibiting the neonatal Fc receptor: phase 2 multicentre open-label trial with efgartigimod

Authors: Matthias Goebeler[1], Zsuzsanna Bata-Csörgő[2], Clara De Simone[3], Biagio Didona[4], Eva Remenyik[5], Nataliya Reznichenko[6], Enno Schmidt[7], Johanna Stoevesandt[1], E. Sally Ward[8], Wim Parys[9], Hans de Haard[9], Patrick Dupuy[9], Peter Verheesen[9], Pascal Joly[10]

Affiliations: [1]Department of Dermatology, Venereology and Allergology, University Hospital Würzburg, Würzburg, Germany; [2]Department of Dermatology and Allergology, University of Szeged, Szeged, Hungary; [3]Catholic University Policlinic A. Gemelli, Rome, Italy; [4]Dermatopathic Institute of the Immaculate, Rome, Italy; [5]University of Debrecen, Debrecen, Hungary; [6]Zaporizhzhya State Medical University, Zaporizhzhya, Ukraine; [7]Department of Dermatology, University of Lübeck, Lübeck, Germany; [8]University of Southampton, Southampton, UK; [9]argenx, Ghent, Belgium; [10]Department of Dermatology, Rouen University Hospital, Rouen, France

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Efgartigimod, an engineered Fc fragment that inhibits the activity of the neonatal Fc receptor (FcRn), was evaluated in an open-label phase 2 adaptive trial (NCT03334058). Thirty-four mild to moderate PV or PF patients were enrolled to evaluate the safety, pharmacodynamics, pharmacokinetics, and efficacy of efgartigimod. In four sequential cohorts, efgartigimod was dosed at 10 or 25 mg/kg intravenously with various dosing frequencies, as monotherapy or add-on therapy to low-dose oral prednisone. Efgartigimod demonstrated a favorable safety and tolerability profile, consistent with previous studies of this FcRn inhibitor. We observed a strong association between serum IgG level reduction, autoantibody level reduction and improvement of pemphigus disease area index (PDAI) scores and clinical outcomes. 90% (28/31) of patients achieved disease control with a median time of 16 days. Fourteen of 22 (64%) patients on efgartigimod treatment with prednisone 0.1-0.5 mg/kg/d achieved complete remission (10 mg/kg: median 35 days, range 13-93; 25 mg/kg: 43 days, range 41-287). These results add to the interim analysis previously presented and support the further evaluation of efgartigimod as a therapy for pemphigus.