Phase 1 Exploratory Efficacy of the Novel Enzyme Replacement Therapy NeoGAA in Treatment-Naïve and Alglucosidase Alfa-Treated Late-Onset Pompe Disease Patients

Loren Pena1, Richard Barohn2, Barry Byrne3, Claude Desnuelle4, Oezlem Goker-Alpan5, Shafeeq Ladha6, Pascal Laforet7, Eugen Mengel8, Alan Pestronk9, Jean-Yves Pouget10, Benedikt Schoser11, Volker Straub12, Jaya Trivedi13, Philip Van Damme14, John Vissing15, Peter Young16, Beth L. Thurborg17, Kenny Culm-Merdiek18, Ged Short19, and Ans van der Ploeg20 for the NEO1 Investigator Group

Duke University, Durham, NC, USA; University of Kansas Medical Center, Kansas City, KS, USA; University of Florida, Jacksonville, FL, USA; University Hospital of Nice, Nice, France; O & D Alphan LLC, Fairlawn, UK, USA; St. Joseph’s Hospital & Medical Center, Phoenix, AZ, USA; Paris Est Neuromuscular Center, Bicetre Hospital, France; Universitätsklinikum Hamburg-Eppendorf, Hamburg, Germany; Universitätsklinikum Mainz, Mainz, Germany; Universitaetsklinikum Ulm, Ulm, Germany; Medical College of Wisconsin, Milwaukee, WI, USA; CHU Timone APHM, Marseille, France; Friedrich-Baur Institut München, Munich, Germany; John Walton Muscular Dystrophy Research Centre, Newcastle upon Tyne, UK; University of Texas Southwestern Medical Center, Dallas, TX, USA; KU Leuven (University of Leuven) – Department of Neurosciences, VIB – Vesalius Research Center, and University Hospitals Leuven – Department of Neurology, Leuven, Belgium; Rigshospitalet, Copenhagen, Denmark; Universitätsklinikum Düsseldorf, Düsseldorf, Germany; Sanofi Genzyme, Cambridge, MA, USA; Enzurum MC, The Netherlands

*At the time of the study

The authors exerted sole scientific control, were responsible by Jane M. Gilbert, BSc, CMPP, of Envision Scientific Solutions, contracted by Sanofi Genzyme to provide publication support services. The authors had full access to all of the data in the study and take responsibility for the integrity of the data and the accuracy of the data analysis.

CONCLUSIONS
- neoGAA was well-tolerated in both groups (see WORLDSymposium™ Abstract 122, Poster 318)
- Pulmonary function (FVC, MEP, and MIP) generally improved or remained stable in both groups at Week 25 relative to baseline
- 6-Minute Walk Test distances were generally stable or increased with neoGAA without relationship to patient group or dose level
- Minimal changes across groups and all dose levels
- Cognitive fatigue, general fatigue, and sleep/rest fatigue mean scores were unchanged relative to baseline, in both groups at all dose levels
- Small reductions in glycogen levels seen in 4/9 patients in the Naïve Group and in 5/9 patients in the Switch Group with no relationship to neoGAA dose
- Muscle biopsy glycogen levels
- Quadriceps muscle biopsy: glycogen levels were low (~6%) in most patients in both groups at baseline, and remained mostly unchanged
- There was minimal change across all dose levels

METHODS
- Study Sites and Patients
  - Study sites: 17 centers (USA [7], France [3], Germany [3], Belgium [1], Denmark [1], The Netherlands [1], UK [1])
  - 24 patients enrolled; 21 completed (Figure 3, Table 1)

RESULTS
- Study design
  - 12 patients randomized to NEO1 Switch Group
  - 12 patients randomized to NEO1 Naïve Group

OBJECTIVES
- To evaluate the safety, tolerability, and exploratory efficacy of neoGAA, and to characterize its pharmacodynamic and pharmacokinetic profiles following repeat dose administrations in adult patients with late-onset Pompe disease
- Exploratory efficacy results are presented here
- Safety profile and pharmacokinetics are presented in WORLDSymposium™ 2016 Abstract 122, Poster 318

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METHODS
- Study Design and Patients
  - Multicenter, multinational, open-label, ascending dose study (NCT01898364)
  - 2 patient groups:
    - Naïve Group: patients naïve to alglucosidase alfa therapy
    - Switch Group: patients previously treated with alglucosidase alfa for ≥9 months

RESULTS
- Treatments:
  - Doses: 5, 10, and 20 mg/kg IV neoGAA every other week
  - 13 infusions over a 24-week treatment period (Figure 2)

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