Valerion to Present Initial Clinical Data with VAL-1221 in Pompe Disease

- Poster Presentation at WORLDSymposium -

CONCORD, Mass. February 5, 2018 —Valerion Therapeutics, a clinical-stage biotechnology company that specializes in the development of therapies for orphan genetic diseases, today announced that a poster highlighting initial results from the first cohort of its ongoing Phase 1/2 clinical study with VAL-1221 in patients with late-onset Pompe disease will be presented at the 14th Annual WORLD*Symposium*™ 2018, being held February 5-9, 2018 in San Diego, CA.

"We are delighted to present these initial clinical findings from the first dosing cohort of our ongoing clinical study with VAL-1221 and look forward to providing further updates as the trial progresses into higher dosing cohorts," said Deborah Ramsdell, CEO of Valerion. "By targeting and clearing both lysosomal and extra-lysosomal glycogen in the cytoplasm, VAL-1221 has the potential to provide new therapeutic options to patients with late-onset Pompe disease."

Poster Presentation:

- **Treatment of Pompe Disease with VAL-1221** (Poster #323)
  Wednesday, February 7, 2018 4:30 – 6:30p.m. PT
  Valerion Therapeutics, Duke University Medical Center, University College London

About the Phase 1/2 Study
The Phase 1/2 trial is a randomized, international, parallel active control, single- and multiple-ascending dose escalation study designed to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and preliminary efficacy of VAL-1221 in up to 12 ambulatory and ventilator-free patients with late-onset Pompe disease previously treated with Myozyme or Lumizyme. Exploratory efficacy endpoints include the six-minute walk test, pulmonary function testing, quantitative and qualitative muscle testing and patient-reported outcomes. Following the three-month treatment period, responding patients will be eligible to roll-over to an open-label extension study. The trial will initially be conducted at Duke University Medical Center and The National Hospital for Neurology and Neurosurgery of London. Please visit clinicaltrials.gov for more information about the study.

About VAL-1221
VAL-1221, is an investigational fusion protein therapeutic that combines Valerion's antibody-mediated delivery technology with recombinant human acid alpha-glucosidase (rhGAA) to drive improved delivery of rhGAA to muscle. It is uniquely designed to target both lysosomal and extra-lysosomal glycogen in the cytoplasm. In cultured Pompe disease fibroblasts and in Pompe (GAA-deficient) mice, VAL-1221 was found to reduce lysosomal glycogen accumulation as effectively as current enzyme replacement therapies, and penetrated living cells independent of the mannose-6-phosphate receptor (M6PR), the mechanism of cell entry seen with current treatments that direct the ERT enzyme to the lysosome. VAL-1221 also enters cells through an equilibrative nucleoside

transporter, ENT-2, and was shown to clear cytoplasmic glycogen in a hypoxia model. VAL-1221 is currently in clinical development for the treatment of patients with late-onset Pompe disease.

About Pompe Disease

Pompe disease is a rare genetic disease with an approximate incidence of 1 in 9,000 births in the United States characterized primarily by skeletal muscle weakness causing problems with ambulation and respiratory function. The most severely affected infants usually present within the first 3 months after birth. They also have characteristic cardiac problems (dysfunction due to cardiac enlargement) in addition to generalized skeletal muscle weakness and a life expectancy of less than 2 years, if untreated (classic infantile Pompe disease). Less severe forms of Pompe disease with onset during childhood, adolescence, or adulthood, rarely manifest cardiac problems, but gradually lead to walking disability and reduced respiratory function. In essence, Pompe disease is a rare, multisystem disorder caused by pathogenic variations in the GAA gene containing the information for production and function of a protein enzyme called acid alpha-glucosidase (GAA). Because of the deficiency in this protein, a complex sugar named 'glycogen' cannot be degraded to a simple sugar like glucose. This causes the glycogen to accumulate in all kinds of tissues, but primarily in skeletal muscle, smooth muscle and cardiac muscle, where it causes damage to tissue structure and function.

About Valerion Therapeutics

Valerion Therapeutics, part of the Alopexx Enterprises portfolio of companies, specializes in the development of therapies for orphan genetic diseases through its proprietary antibody-mediated delivery platform, which enables enhanced intracellular delivery of a range of active therapeutic molecules by way of a transport mechanism present in muscles and neurons. Valerion's unique product candidates target disease tissues via a novel antibody (3E10) with cell-penetrating properties dependent on a tissue-localized membrane transporter (ENT2). Because the ENT2 transporter is naturally enriched in critical organs (ex. heart and skeletal muscle), Valerion constructs targeted fusion and chemical conjugation products (proteins, drugs and oligos); providing a novel way to treat a number of diseases with limited or no current therapeutic options. For more information about our platform and pipeline please visit www.valerion.com

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