



PR NewsWire  
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### **Valerion Therapeutics Demonstrates a New Mechanism for Treating Pompe Disease**

Concord, MA-- March 3, 2017-- Valerion Therapeutics announced today that it has developed a fusion protein, VAL-1221, which combines its proprietary antibody delivery technology with recombinant human acid alpha-glucosidase (rhGAA) to improve the delivery of rhGAA into affected tissues of patients with Pompe disease (Glycogen Storage Disease, Type II; GSDII). Pompe disease is caused by a deficiency of the lysosomal enzyme, GAA, that leads to accumulation of glycogen in multiple tissues, with cardiac and skeletal muscles being the most severely affected. Glycogen, a complex sugar, is known to accumulate in both the lysosomes and cytoplasm of late-onset Pompe disease patients. However, the currently approved enzyme-replacement therapy is limited to the lysosome for therapeutic activity.

In a study recently published by Sun et al, Duke University, Division of Medical Genetics (*J Mol Med*, 2 Feb, 2017), Valerion's proprietary antibody-mediated enzyme replacement therapy [VAL-1221 (humanized 3E10Fab-GAA)] demonstrated efficacy in both cultured Pompe patient fibroblasts and in Pompe (GAA-deficient) mice. Importantly, not only did VAL-1221 reduce lysosomal glycogen accumulation as effectively as rhGAA (current enzyme replacement therapy or ERT) but it was also demonstrated to penetrate living cells independent of the mannose-6-phosphate receptor (M6PR), the mechanism of cell entry associated with current ERT which directs enzyme to the lysosome. These results suggest that VAL-1221 has potential benefit over current ERT by clearing both lysosomal and cytoplasmic glycogen.

Valerion is initiating a clinical trial in both the US (Duke University Medical Center) and the UK (The National Hospital for Neurology and Neurosurgery, London) next month to evaluate this novel therapy in patients with late-onset Pompe disease.

“We believe our findings are a game-changer in the treatment of Pompe disease,” said Deborah Ramsdell, Valerion's Chief Executive Officer. “We are excited about the potential to help patients who are looking for alternatives to the current approved therapy.”

This randomized, parallel active control, single and repeat dose, dose-escalation study will evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and preliminary efficacy of VAL-1221 in ambulatory and ventilator-free patients with late-onset Pompe Disease. Top line results are expected later this year.



“The approach is different from other ERT approaches as this has the ability to act on glycogen in the cytoplasm. This remains a challenge in the field of Pompe disease,” said Dr. Priya Kishnani, Principal Investigator at Duke University Medical Center. “Glycogen that is leached out (either due to shearing effect or rupture of lysosomes) into cytoplasm needs to be cleared. The collaboration with Valerion is an important one as it allows us to look at whether VAL-1221 has this additional benefit.”

### **About Valerion Therapeutics**

Valerion Therapeutics ([www.valerion.com](http://www.valerion.com)) is a biotechnology company focused on developing therapies for orphan genetic diseases utilizing a proprietary antibody-mediated delivery platform to target specific tissues.

This platform is capable of enhanced intracellular delivery of a variety of active therapeutic molecules via a well-known and broadly studied transport mechanism that is present in muscles and neurons. Pipeline candidates include therapies aimed at addressing orphan genetic disorders with limited or no current therapies. Valerion Therapeutics is part of the Alopexx Enterprises portfolio of companies ([www.alopexx.com](http://www.alopexx.com)).

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