Valerion Therapeutics Announces Positive Results Of Non-Clinical Research Study at SSIEM Annual Symposium

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CONCORD, Mass., Sept. 3, 2015 /PRNewswire/ -- Valerion Therapeutics, LLC yesterday announced positive results from a non-clinical "proof of concept" pilot study for a novel humanized antibody and acid alpha glucosidase (GAA) fusion candidate for the treatment of Pompe disease.

The studies performed with Valerion's proprietary product candidate [VAL-1221 (3E10Fab-GAA)] are part of a broad research collaboration between Valerion Therapeutics and Duke University. Valerion Therapeutics, which is part of the Alopexx Enterprises portfolio, is a developer of bio-therapeutics for neuromuscular genetic disorders with limited or no current treatment options.

VAL-1221 demonstrated efficacy in both cultured Pompe patient fibroblasts and in Pompe (GAA-deficient) mice, according to results announced at the Society for the Study of Inborn Errors of Metabolism 2015 Annual Symposium in Lyon, France. The pilot results were presented by the principal investigator of the study, Baodong Sun, PhD, of Duke University.

Importantly, VAL-1221 was observed in the study to penetrate living cells partially-independent of M6PR and not co-localize with lysosomal marker LAMP2. The results suggest that VAL-1221 is capable of entering the cytoplasm and potentially functioning outside the lysosome.

"The pilot study results demonstrate that VAL-1221 represents a novel enzyme-replacement candidate," said Dustin Armstrong, PhD, Chief Scientific Officer of Valerion. "It is a paradigm shift as this fusion is specifically designed to target both the intended tissues and its subcellular compartments, imperative for the most effective treatment of Pompe. We are hopeful ongoing studies will continue to be positive and further highlight the efficacy of VAL-1221 and the widespread utility of the Valerion delivery-technology."

Glycogen, a complex sugar, is known to accumulate in both the cytoplasm and lysosome of adult-onset Pompe patients; however, the currently approved enzyme-replacement product is limited to the lysosome for therapeutic activity. Armstrong said VAL-1221 may uniquely target extra-lysosomal glycogen, which is known to affect adult Pompe muscle.

Pompe disease is a rare genetic disorder that primarily affects muscle. The buildup of glycogen in certain organs and tissues, especially in the muscles, impairs their ability to function normally. Approved therapies have been shown to be effective in reducing the clinical manifestations of the disease.

About Valerion Therapeutics

Valerion Therapeutics (www.valerion.com) is an emerging science-driven company focused on the development of bio-therapeutics for orphan neuromuscular genetic diseases. Valerion generates unique product candidates utilizing a proprietary antibody-

based delivery technology platform. Valerion is able to construct targeted products to treat a number of neuromuscular diseases with limited or no current therapeutic options. Valerion is a member of the Alopexx Enterprises portfolio of companies (www.alopexx.com).

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