Valerion Presents Preclinical Proof-of-Concept Data for VAL-0417 for the Treatment of Lafora Disease

- High Unmet Need for this Rare and Fatal Form of Epilepsy -

- Planning Underway for Initiation of Clinical Studies in Lafora Patients -

CONCORD, Mass., Sept. 6, 2017 /PRNewswire/ -- Valerion Therapeutics, a clinical-stage biotechnology company that specializes in the development of therapies for orphan genetic diseases, today announced preclinical proof-of-concept results providing early validation of VAL-0417 as a potential treatment for Lafora disease, a rare and fatal genetic form of epilepsy, characterized by an accumulation of aberrant glycogen-containing deposits known as Lafora bodies. VAL-0417 is a novel fusion protein that combines Valerion's delivery antibody (3E10) linked to amylase, to uniquely target Lafora bodies present in all tissues of Lafora disease patients. Data from this study were presented at the 2017 Lafora Disease Workshop, a satellite symposium of the 32nd International Epilepsy Congress being held September 2-6, 2017 in Barcelona, Spain.

"Current treatments for Lafora disease are only palliative with patients typically succumbing to their disease within ten years of symptom onset," said Deborah Ramsdell, CEO of Valerion. "Our goal with VAL-0417 is to rescue neurons from degeneration thereby minimizing progression of this devastating and underserved disorder. We look forward to completing the preclinical and IND-enabling studies necessary to initiate clinical studies in Lafora patients."

In a presentation titled "*Lafora Body Degradation by a Therapeutic Enzyme for the Treatment of Lafora Disease,"* Valerion researchers and collaborators from the University of Kentucky demonstrated *in vitro* and *in vivo*, the ability to deliver VAL-0417 to the skeletal muscle and brain with retention of up to 24 hours following intracerebroventricular **(**ICV) injection in Lafora mouse models.

"These early but encouraging data demonstrate preclinical proof-of-concept for VAL-0417 and further validate the Valerion platform," said Tracy McKnight, Ph.D., Director of Translational Research at Valerion. "We look forward to continued evaluation of VAL-0417 as a potential therapeutic approach to Lafora by enzymatically degrading neuronal glycogen to clear Lafora bodies in the brain."

About Lafora Disease

Lafora Disease (LD) is a rare, progressive, autosomal recessive neurodegenerative disorder characterized by intractable seizures, difficulty walking, muscle spasms, neurological deterioration, rapid cognitive decline, dementia, and death typically within 10 years of onset. It is caused by loss-of-function mutations in either the laforin gene (EPM2A) or malin gene (EPM2B) and is associated with gradual accumulation of Lafora bodies, aggregates of poorly branched, hyperphosphorylated, insoluble glycogen also known as polyglusan. LD usually begins in late childhood or adolescence.

About VAL-0417
VAL-0417 is a novel fusion protein that combines Valerion's delivery antibody (3E10) linked to amylase to uniquely target neuronal glycogen through enhanced delivery to affected tissues. In preclinical proof-of-concept studies, Valerion and collaborators at the University of Kentucky demonstrated *in vitro* and *in vivo*, the ability to deliver VAL-0417 to the skeletal muscle and brain with retention of up to 24 hours following intracerebroventricular **(**ICV) injection in Lafora mouse models.

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. skeletal muscle and CNS), Valerion constructs targeted fusion 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

Contacts:
Deborah Ramsdell, Valerion Therapeutics
deb.ramsdell@valerion.com
781-775-7688

or

Gina Nugent, Nugent Communications
(Investors and Media)
gina@nugentcommunications.com
617-460-3579

This information is being sent to you by:
Valerion Therapeutics
100 Main Street
CONCORD, MA 01742
www.valerion.com