Company Overview

Valerion Therapeutics, a biotech company based in Boston, Massachusetts, is focused on advancing innovative therapies for the treatment of rare neuromuscular diseases. Our experienced leadership team, including Deborah Ramsdell, Chief Executive Officer and Hal Landy, MD, Chief Medical Officer, have significant expertise in the development of rare disease therapies, including Pompe disease.

Compound/ Indication	Discovery	Preclinical	Phase 1	Phase 2	Phase 3
VAL-1221 Pompe Disease					
VAL-0417 Lafora Disease					
VAL-0620 Myotubular Myopathy					
Other Research Programs					

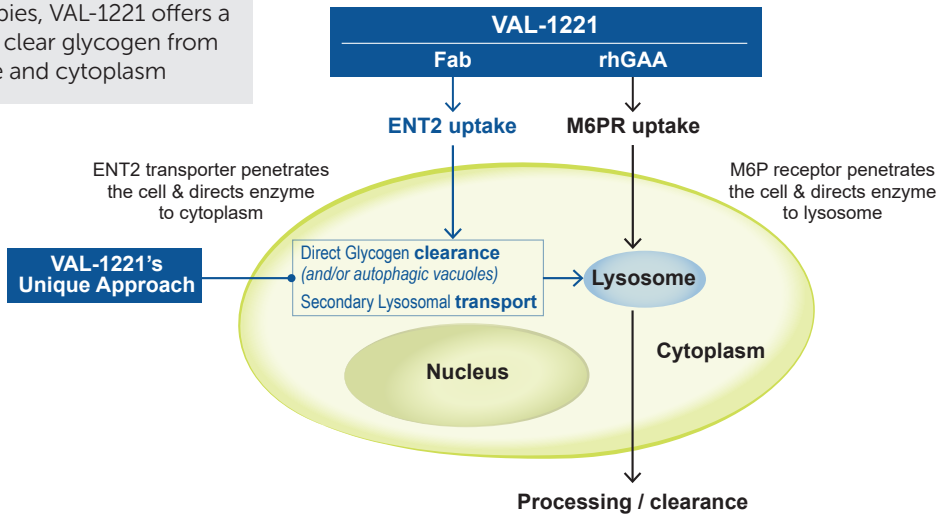
VAL-1221 for Pompe Disease

Valerion's lead program, VAL-1221, is in clinical development for Pompe Disease. VAL-1221 is uniquely designed to target delivery of therapeutic enzymes into skeletal and cardiac muscle, and unlike other therapies, VAL-1221's unique mechanism of action clears glycogen from both the lysosome and the cytoplasm.

- Pompe Disease, a rare multi-system genetic disorder with an approximate incidence of up to 1 in 9,000 births in the U.S., is characterized primarily by skeletal muscle weakness causing problems with ambulation and respiratory function. Patients with Pompe disease have a deficiency in a protein enzyme (acid alpha-glucosidase or GAA) which is responsible for the degradation of a complex sugar called glycogen. This enzyme deficiency leads to an accumulation of glycogen in skeletal, smooth and cardiac muscle tissue, causing damage to tissue structure and function.

Mechanism of Action

Unlike existing therapies, VAL-1221 offers a dual mechanism to clear glycogen from both lysosome and cytoplasm



Valerion employs its proprietary drug delivery platform to enable enhanced intracellular delivery of a range of active therapeutic agents directly into the targeted cell. This cell-penetrating mechanism provides a novel way to treat diseases with limited or no current therapeutic options.

VAL-1221 Pompe Disease: Ongoing Phase 1/2 Study

Study Design:

12 ambulatory and ventilator-free patients

- ≥18 yrs previously treated with Myozyme or Lumizyme for at least 6 months

Treatment:

- 3 dosing cohorts: VAL-1221 at 3, 10, 30 mg/kg via IV every 2 weeks
- Control: Myozyme/ Lumizyme at usual dose

Study duration:

3 months with extension of VAL-1221 treatment for up to 1 year

- Myozyme/ Lumizyme patients can roll-over to VAL-1221 after 3 months

Endpoints:

Primary:

- Safety, tolerability and immunogenicity

Other:

- Pharmacokinetics (PK) and pharmacodynamics (PD)
- Six minute walk test
- Pulmonary function testing (MIP, MEP, FVC)
- Quantitative & qualitative muscle testing
- Patient-reported outcomes/quality of life/disability

Initial Clinical Results & Next Steps

Initial findings from first dosing cohort of Phase 1/2 study*

- VAL-1221 has been well-tolerated to date
  - Safety profile similar to approved treatments
- No serious or unexpected adverse events or safety concerns observed
  - After 3 months treatment with VAL-1221 at 3mg/kg
- No discontinuations from the study
- All patients from cohort 1 enrolled in the open-label extension phase

Next Steps:

- Dose escalation in cohort 2 complete
- Cohort 3 enrollment scheduled to complete in March
- Topline results expected Q3 2018
- Planned registration study Q4 2018

*Presented at 2018 **WORLDS**ymposium February 2018