

COMPANY OVERVIEW

Aquilus Pharmaceuticals (Aquilus) is a biotech company focused on the treatment and management of amyotrophic lateral sclerosis (ALS), neuropathic pain, and other neurological disorders. The company has a portfolio of highly potent and proprietary matrix metalloproteinase (MMP) inhibitors. As a family, MMPs can cleave virtually any component of the extracellular matrix, thereby facilitating cell migration and affecting cellular signaling that regulates cell proliferation, differentiation, and cell death.

Website: Aquiluspharma.com

MMP's and ALS

A growing body of evidence has linked MMP-9 and MMP-2 with the severity and rate of progression of ALS. Currently, there are no FDA approved MMP inhibitors.

LEAD COMPOUND

AQU-118 is a dual MMP-9 and MMP-2 inhibitor for the treatment of ALS and neuropathic pain. Aquilus' primary goal is to pursue clinical development of AQU-118 as a first-in-class treatment for ALS.

- AQU-118 affects various key inflammatory pathways that reduce nerve injury and cell death (apoptosis)
- orally bioavailable; exhibited excellent safety in mice, rats, and dogs
- AQU-118, when given orally, has been found to significantly reduce nerve damage in several different rodent models of nerve injury & ALS.
- Oral dosing with AQU-118 in the overexpressed TDP-43 mouse model (TDP43A315T mutation) mitigated loss in weight and muscle grip strength and reduced involuntary muscle contractions (i.e. dystonia) as compared to control group.
- All Investigational New Drug (IND)-enabling studies have been completed
- IND submission for AQU-118 with the FDA by the 4th quarter of 2024
- Begin clinical testing in 2025

FUNDING OVERVIEW

Aquilus is pre-Series A company funded through over \$5M in grant awards from the ALS Association Lawrence and Isabel Barnett Drug Development Program, the National Institutes of Health (NIH), and the Department of Defense (DOD) ALS Research Program. Aquilus is looking for investors to fund the testing of AQU-118 in a Phase 1/2 clinical trial for the treatment of ALS.

INTELLECTUAL PROPERTY (IP)

Aquilus has obtained fully issued patents covering Composition of Matter for AQU-118 in the United States and internationally. Additionally, Aquilus has fully issued Method of Use IP for AQU-118 for treating neuropathic pain, addiction, tolerance, and withdrawal in the United States and internationally. Aquilus filed new Method of Use IP for AQU-118 for the treatment of ALS and other neurodegenerative disorders including Alzheimer's disease, Parkinson's disease, muscular dystrophy, Huntington's disease, and others.

RECENT MILESTONES AND ACHIEVEMENTS

Completed all preclinical studies & received Human Research Ethics Committee (HREC) approval to begin Phase 1 SAD/MAD trial in Australia in 2025

MANAGEMENT

[Irving Sucholeiki](#), Ph.D., Founder, President and CEO. As founder and President of a previous drug discovery company (Solid Phase Sciences Corporation), Dr. Sucholeiki had extensive experience in all aspect of running a research-based drug discovery company. Dr. Sucholeiki has over thirty issued or pending patent applications covering all aspects of drug discovery.

[Darrell J. Nix](#), Ph.D., VP of Research & Development. Dr. Nix has extensive experience in pre-clinical DMPK, toxicology, pharmacology, and clinical pharmacology through the submission of several IND/CTD filings with the US FDA, EMEA, and the Japanese Ministry of Health and Welfare.

[Roy Sucholeiki](#), M.D., VP of Clinical Development and Chief Medical Officer. Board certified, practicing neurologist and Former Director of the neurodiagnostic laboratory at Northwestern Medicine – Central DuPage Hospital. Dr. Roy Sucholeiki has over 25 years of clinical and academic research experience.

COLLABORATORS/ADVISORS

In the area of ALS, Aquilus currently has active and past research collaborations with

- Professor Robert Bowser, Chairman of the Department of Neurobiology at the Barrow Neurological Institute in Arizona
- Merit Cudkowicz, M.D., MSc., Professor, Harvard Medical School & Director, Sean M. Healey & AMG Center for ALS
- Sabrina Paganoni, M.D., Associate Professor, Harvard Medical School & Co Director, MGH Neurological Clinical Research Institute
- Professor Daniela Zarnescu, Professor of Molecular & Cellular Biology, University of Arizona
- Professor Rita Sattler, MSc., Ph.D., Associate Professor, Barrow Neurological Research Institute
- **Partnerships**
- ALS Association
- Department of Defense
- National Institutes of Health
- ALS Patient Support Network
- EverythingALS