

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.

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
- completed all Investigational New Drug (IND)-enabling studies and is on-track to submit IND application with the FDA by end of 2023
- begin clinical testing by third quarter of 2024

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 has completed all of the IND-enabling studies around AQU-118 and plans to submit its IND application with the FDA by end of 2023. 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. Recently, 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

- ☑ Biomarker study in serum of ALS patients confirming the relevance of MMP-9 to ALS
- ☑ Formulation of AQU-118 into capsules; completed long term stability testing
- ☑ Pre-IND meeting with FDA officials
- ☑ GLP dog cardiovascular, rat IRWIN, and rat respiratory studies
- ☑ GLP rat 28-day & 70-day toxicity studies
- ☑ GLP 70-day dog toxicity and rat micronucleus studies

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 Director of the neurodiagnostic laboratory at Central DuPage Hospital's Neurosciences Institute in Winfield, IL. Dr. Roy Sucholeiki has experience as principle investigator on various clinical trials such as a Phase 4 study of oxcarbazepine (Trileptal®) for Novartis, and a multi-center Phase III trial of Keppra XR® sponsored by UCB Pharma. He has served as a consultant regarding anticonvulsant drugs for major pharmaceutical companies.

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
- Professor Sabrina Paganoni, M.D., Associate Professor, Harvard Medical School & Co-Director, MGH Neurological Clinical Research Institute
- Professor Rita Sattler, Associate Professor, Barrow Neurological Institute
- Professor Daniela Zarnescu, Professor of Molecular & Cellular Biology, University of Arizona
- National Institutes of Health (NIH)

In the area of nerve injury and neuropathic pain, Aquilus has had collaborations with

- Professor Michael Henry, Professor of Endodontics at the University of Texas Health Sciences Center at San Antonio
- Professor Xue-Jun Song, Professor of Basic Science Research at the Parker Research Institute
- Dr. Marcie Fowler, Principal Investigator at the United States Army Institute of Surgical Research at Fort Sam Houston