

ALS Therapy Development Institute



300 Technology Square, Suite
400
Cambridge, MA 02139
(617) 441-7200
www.als.net



Mission

Founded in 1999 as the world's first nonprofit biotechnology company, ALS Therapy Development Institute (ALS TDI) seeks to develop effective therapeutics that slow and stop amyotrophic lateral sclerosis (ALS). It works to bridge the gap of ALS therapeutic development and facilitate the clinical development and commercialization of medical solutions for ALS patients.

ALS TDI was started in 1999 by James (Jamie) Heywood three months after his brother Stephen was diagnosed with ALS.

Strategy/Approach

ALS TDI combines the power of a 501(c)(3) nonprofit with the best practices of a for-profit biotech or pharmaceutical company. It aims to leverage more than a decade of treatment-focused ALS research and apply it on an industrial scale to get therapeutics to today's ALS patients as quickly, efficiently, and cost-effectively as possible. Along with the research done at its own facility, the institute partners with dozens of scientific collaborators around the globe. It also enters into strategic funding alliances with foundations and nonprofits from across the spectrum of neurodegeneration.

ALS TDI also offers patient resources, such as a clinical trial finder. While not endorsing any particular clinical trial, the institute provides up-to-date information about all known ALS trials taking place worldwide.

Research Portfolio

ALS TDI funds the research efforts of its own in-house lab. Its research strategy focuses on four separate areas:

- **Gene Therapy** – The current state of the art for gene therapy studies in ALS is hampered by the ability to effectively insert functional genes into cells in the central nervous system and maintain and maximize long-term function of the gene after injection. In order to reduce potential toxicity, it is often necessary to target the gene therapy construct to specific tissues or even specific cell types within a given tissue.
- **Research Biology** – Uses genomics and proteomics to profile ALS tissues. The identification of differences in DNA sequences, RNA quantities, and the quantities and shapes of proteins in ALS-related tissues when compared to healthy tissues leads to clearer understanding of the underlying biology of ALS and to the identification of promising ways to slow, stop, and reverse the disease.
- **Validation and Biomarkers** – Confirms the activity of promising therapeutics prior to initiation of longer and more costly in vivo (animal) studies. Helps to

identify earlier disease interventions and additional end points.

- **Testing Therapeutics** – Involves testing small molecules, gene therapies, biologics, and stem cell products by utilizing an animal model of disease.

The institute's **Laboratory Information Management System** is updated in real time. It provides information about ALS TDI's active research pipeline and lists potential therapeutics that are being pursued today.

Partnership Practices

ALS TDI works collaboratively with biotech, pharma, academia, and nonprofits to accomplish its mission.

- **Internal Project Partnerships** – Using proprietary genomic and proteomic data collected by ALS TDI and leveraged against the published literature, ALS TDI has identified several highly relevant ALS associated molecular targets. The current pipeline contains 30 programs targeting various aspects of neurodegenerative disease pathology. It is currently seeking partners interested in making strategic investments in projects in its pipeline or translating late-stage projects from ALS TDI toward the clinic. ALS TDI develops appropriate intellectual property protection on several molecules each year following the development of supporting data.
- **External Project Partnerships** – ALS TDI has developed a continuum of partnership models to support the advancement and translation of potentially life-changing therapeutics into the clinic and ultimately the marketplace. The institute works with pharmaceutical and biotechnology organizations, academic leaders, and start-ups. In 2011, ALS TDI had six contract research agreements with the largest global drug development companies and 12 collaborations with universities and other corporations.

ALS TDI partners with biotech and pharmaceutical companies. Partnerships are aimed at scaling up novel gene therapies, biologics, and small particle production. Examples include:

- **Applied Proteomics** became a strategic partner in ALS TDI's effort to characterize protein expression changes associated with disease onset and progression.
- **Asklepios Biopharma** and ALS TDI collaborated on the development of novel adeno-associated viral vectors to be used in drug-development research.
- **Aestus Therapeutics, Inc.** has a multi-year collaboration to test several potential small-molecule

compounds to slow or stop the progression of ALS. It works to identify therapeutics for screening against targets associated with ALS onset or progression. All screening will be conducted at ALS TDI.

Financials

ALS Therapy Development Institute is a 501(c)(3) tax-exempt nonprofit organization. The most recent financial information available is from 2012.¹ More than 86 percent of its budget is spent on research that advances its mission.

Year ending 12/31/12:

- Revenue: \$9,512,367
- Assets: \$7,256,370
- Gifts received: \$6,444,164
- Expenditures: \$9,731,087

Key Accomplishments

- World's first nonprofit biotech built by, and for, ALS patients.
- Largest dedicated ALS lab in the world.
- Raised private capital of about \$70 million to date.
- Current pipeline of 20 products.
- 500 events every year, all organized by supporters.
- About \$12 million spent on research each year.
- 500 drugs developed and tested.
- 32 active trials.
- Prize4Life selected ALS TDI model standards as the basis for a \$1 million biomarker and therapeutic prize.
- Full in vivo PK, efficacy, and PD capabilities using SOD1 mouse (ex: partnered with the Muscular Dystrophy Association, the Alzheimer's Drug Discovery Foundation, and the Association for Frontotemporal Degeneration to fund the characterization of a new model of neurodegeneration called the TDP-43 mouse in 2011-2012, another coming in 2013).
- In 2011, ALS TDI had six contract research agreements with the largest global drug development companies and 12 collaborations with universities and other corporations.

Leadership

The ALS Therapy Development Institute is governed by a **Board of Directors** and managed by a Massachusetts-based **Executive, Research, and Operations staff**.

- **President and CEO, Chief Scientific Officer, and Vice Chairman:** Steven Perrin, PhD, sperrin@als.net
- **Vice President of Research:** Al Gill, PhD, agill@als.net
- **Development Director, Lead Management:** Alicia Grossi, agrossi@als.net
- **Vice President of Communications & Public Relations:** Robert Goldstein, rgoldstein@als.net

¹ Information obtained from Foundation Center Directory, <http://fconline.foundationcenter.org>, July 2013.