



# What is ALS?

## About ALS

[Amyotrophic lateral sclerosis](#) (ALS, Lou Gehrig's disease) is a progressive neurodegenerative disease that leads to paralysis, due to the death of motor neurons in the spinal cord and brain. There is no known cure for the disease. About 5,000 people in the US are diagnosed with ALS each year; the incidence is similar to multiple sclerosis. However, with no effective treatment, the average patient survives only two to five years following diagnosis. There are about 30,000 people in the US diagnosed with ALS today. The worldwide population of ALS patients is estimated at 450,000.

## About ALS TDI

The [ALS Therapy Development Institute](#) (ALS TDI) is the world's leader in research aimed at the discovery and development of effective treatments and a cure for ALS, commonly known as "Lou Gehrig's disease." Built by and for patients, the Cambridge, MA based research institute operates as a 501c3 nonprofit: combining the optimal entrepreneurial practices of a for profit biotechnology and pharmaceutical company with the passion and urgency of a nonprofit mission. For more information, please visit us online at [www.als.net](http://www.als.net).



## ALS Facts

- Every 90 minutes someone is diagnosed with ALS
- Currently, there are no effective treatments or a cure
- The average person survives only two to five years from diagnosis
- It can take a year or longer to diagnose
- 30,000 people are diagnosed with ALS in the United States
- 450,000 people are living with ALS worldwide
- The incidence of ALS is similar to that of multiple sclerosis
- About 10% of ALS cases are linked to mutations in nearly 20 genes; the rest are considered sporadic
- ALS shows no prejudice, affecting people of all ages, races and ethnicities

Phone: 617-441-7200

Email: [info@als.net](mailto:info@als.net)

Website: [www.als.net](http://www.als.net)

ALS Today Magazine: <http://blogs.als.net>

# ALS TDI Background

## Mission

To discover and develop effective treatments and cure for ALS.

## History

The history of the ALS Therapy Development Institute is the history of the people in the community we serve. Hundreds of families and thousands of supporters make our important work possible each and every day. In 1999, James Allen Heywood founded ALS TDI as an independent research center with a singular focus: develop effective therapeutics that slow and stop amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease). As a 501(c)(3) nonprofit organization, ALS TDI is the world's first nonprofit biotech company. Since its founding, ALS TDI has raised more than \$100 million to fund its research into effective treatments.

Today, the Institute maintains the most robust drug development pipeline of its kind in the world with dozens of potential treatments using a wide-range of therapeutic strategies. The ALS Therapy Development Institute represents a paradigm shift in the way that drug discovery and drug development is conducted for an orphan disease such as ALS.

## Research

Since 1999, ALS TDI has screened hundreds of potential compounds independently and in partnership with academic centers and pharmaceutical and biotechnology companies. The research institute is internationally recognized for its work to optimize preclinical models of neurodegeneration, such as the SOD1 mouse model, for clinical translation. With an annual budget of \$10 million, it annually screens 20-30 potential treatments at its core facility and has made more than \$5 million in venture philanthropy investments to date on outside projects with potential treatments for ALS. The Institute individually funded and is executing a Phase IIA clinical trial of TDI-132 (Novartis' Riluzole®) in ALS at four clinics in the United States. ALS TDI is funded by grants, bequests and more than 140 events annually, and supported by members of the ALS community worldwide.

## The ALS Therapy Development Institute is

- A collaboration of hope and science for ALS patients today
- Funded annually by 140+ events, grants and bequests
- Driven by a peer and industry-reviewed research plan
- Spends over 85% on mission, not on fundraising or administrative costs
- Employs 30 full-time drug development experts

## Leadership



### Steve Perrin, Ph.D., CEO and CSO

Dr. Perrin joined ALS TDI in 2006 as part of an unprecedented collaboration between the Muscular Dystrophy Association, Augie's Quest and ALS TDI. Through his leadership, Dr. Perrin has helped the Institute become the world leader in preclinical drug development for ALS. Prior to joining TDI, Dr. Perrin held positions at the Hoechst-Ariad Genomics Center, Aventis Pharmaceuticals, and more recently, as director of molecular profiling at Biogen Idec. He earned his Ph.D. at Boston University Medical Center studying the transcriptional regulation of genes controlling adipocyte and myocyte differentiation.



### Augie Nieto, Chairman

A pioneer in the fitness industry, Augie Nieto co-founded Lifecycle, Inc. in 1980, and in the course of 20 years grew the company, now called Life Fitness, Inc., to be the largest commercial manufacturer of fitness equipment in the world. In March 2005, Nieto's life took an unexpected turn; he was diagnosed with ALS. Since joining the board at ALS TDI, Nieto has raised over \$30 million for research. He is instrumental in the management of the Institute and acts as a spokesman for the company.