



# The Race to Treat and Cure ALS

The Laureate Society  
PROSPECTUS







Dear Friend,

On behalf of The ALS Association and all of the people we are privileged to serve, I invite you to play a leading role in the fight against Amyotrophic Lateral Sclerosis – Lou Gehrig’s Disease – at a crucially important time. In this Prospectus, you will learn more about ALS; about why the work of The ALS Association is so pivotal in the effort to treat, cure and end ALS; and why our newly established Laureate Society will have a transformative impact as we work to achieve our shared vision of A World Without ALS. I hope you will join us in this great effort.

Best wishes from all of us at The ALS Association.

Sincerely,

Jane Gilbert  
President & CEO  
The ALS Association

...every 90 minutes  
someone  
dies from  
ALS



**ALS**, Amyotrophic Lateral Sclerosis (ALS), Lou Gehrig's Disease, is a cruel and indiscriminate killer. It takes the lives of young and old, male and female, members of every race, ethnicity and faith. Loving parents. Beloved sons and daughters. Cherished spouses, siblings and friends. They are dedicated contributors to workplace and community—when a disease so devastating it felled even the baseball legend known as the “Iron Horse” begins to take hold, attacking and killing motor neurons in their bodies, denying them the ability to move and to speak, and eventually robbing them of the ability to breathe.

Some 30,000 people have ALS in the United States right now, 300,000 worldwide. ALS strikes military service veterans at a higher rate of incidence than it strikes in the general population. In this country 5,600 people are newly diagnosed with ALS each year and an equal number die. They fight, they endure, and they contribute to family, community, and society. They hope and they love, but in the end they die—much too soon—of ALS. Normal life expectancy from diagnosis is 2-5 years. Persons diagnosed with ALS have little time to live and no time to lose.

**They—and we—are racing to find therapy to treat ALS and a cure to end it.**

### Jenny has ALS.

She has a husband and three pre-teen children. She knows that ALS probably will take her life in just a few years, but she also knows that some people survive for 10, 12 years or longer. She has hope. She knows there is research—including clinical trials—that might prolong her life and increase its quality. She continues to be a loving and caring mother for her children, even as they care for her. She wants to see her children grow to adulthood, and she would give anything to someday see her grandchildren. For Jenny, finding treatment for ALS is a race to the finish line and her only relevant time frame is right now.

### Ron does not have ALS.

But his wife Jan died of ALS three years ago. Her ALS was familial (genetic) and struck in prior generations of her family. Ron is raising their two children and doing all he can to support research that will produce treatment and a cure for familial ALS. For Ron, racing to the finish line means finding treatment and a cure for ALS before one of his children or grandchildren is struck down.

**Today, there is research that might help prolong the lives of people affected with ALS, like Jenny.**

Your support will  
help save  
thousands  
of lives.



# The Laureate Society

Members of The Laureate Society provide transformational support for **research, compassionate care** and our **chapters**. They provide strategic support for **advocacy** and **educational programs**, and they provide high level counsel to The Association.

Members make gifts and pledges over a maximum 5-year period of \$1 million or more. Membership is open to individuals, families, foundations and corporations. Members are at the forefront of a successful effort to end ALS.

Funding of projects by Laureate Society Members is conducted within the framework of The ALS Association for selecting and funding research, compassionate care, advocacy and educational projects. Members work collaboratively with The ALS Association board and staff and its scientific advisory boards on funding opportunities. Funding of projects with Member contributions is done in a pooled resource, joint decision model. Members meet twice a year to review and recommend projects for funding and to provide counsel to The Association.

## Members of The Laureate Society will:

- Influence the formation of public policy and public funding for ALS.
- Participate in the development of strategic direction for The ALS Association.
- Promote the scientific work of leading genetic, cellular, clinical and other researchers and have personal connection with research industry leaders.
- Support the work of ALS Association chapters, centers and clinics throughout the United States.
- Receive top level recognition for their commitment to the great mission of treating, curing and ending ALS.

**Your support will help save thousands of lives.**

The ALS Association  
helps individuals with  
ALS and their families cope  
with the daily challenges  
of living with the disease.



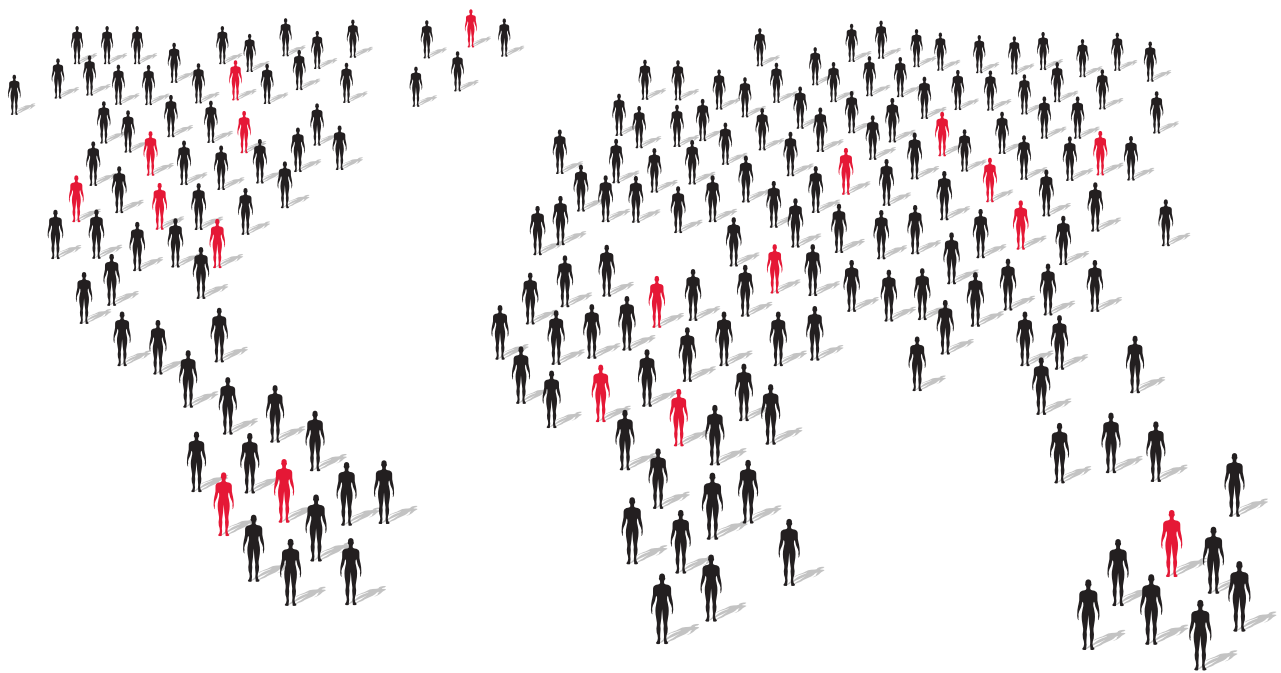
**Research.** The ALS Association is a global leader in the search for treatment and a cure for ALS and in accelerating progress toward effective therapy. The Association has built, supported and led a worldwide research effort devoted solely to ALS. When The Association was established in 1985 there was no hope for treatment of ALS and little understanding of disease mechanisms and progression. That has changed, owing in large part to the work of investigators funded and supported by The ALS Association and to its partnerships with pharmaceutical and biotech companies, academic institutions and other organizations. Since its founding, The ALS Association has initiated, funded or collaborated in many of the most significant developments in ALS research. Among these are:

- **Finding the ALS gene, SOD1**, that led to the groundbreaking discovery of the genetic mutations responsible for 20 percent of all familial ALS cases. (1993)
- **Development of a mouse model** that closely mimics ALS in humans and provides researchers vital information about the disease. (1994)
- **The discovery that a novel therapeutic** silences the genes that cause ALS symptoms to appear in ALS mice and may help to slow or possibly stop the progression of the disease. (2005)
- **Finding that human stem cells can be made into motor neurons**, the cells of the nervous system destroyed by ALS. (2008)
- **The discovery of ALS6**, another gene responsible for about 5% of familial ALS cases. This discovery is a culmination of the Gene Identification Project initiated by The ALS Association in 2002.
- **The commencement, in collaboration with Isis Pharmaceuticals, Inc., of a clinical trial to lower a gene that causes ALS**, a very promising treatment. (2010)

**Compassionate Care.** Our network of 42 chapters, 34 ALS Certified Centers<sup>SM</sup>, and our caregiving leadership team ensure that persons with ALS receive the best care and support.

- **Certified Centers** provide expert diagnosis and multidisciplinary clinical care shown to positively impact survival and quality of life.
- **Chapters** provide support to those living with ALS and their families; information and resource referral; support group facilitation; equipment and communication device loan programs.
- **Centers and Chapters** are critical in increasing awareness of ALS within the community and providing educational outreach to healthcare practitioners.

**We need to expand these services — to provide broader reach to more people with ALS and families.**



**Advocacy.** We are a powerful voice in advancing public policy initiatives and funding for ALS. Our efforts have empowered people with ALS, improved care, and expanded ALS research. Among our accomplishments:

- **Working with Congress** as it committed more than \$420 million in federal funding for ALS research.
- **Securing vital health and disability benefits for military veterans** with ALS, their families and survivors.
- **Eliminating the Medicare waiting period for people with ALS** (the only time Congress has amended this law).
- **Enacting the ALS Registry Act** to create the first nationwide ALS patient registry.

**Progress to be sure.** Today there are more researchers than ever before working on ALS. They are equipped with powerful tools and increased knowledge of the disease. Discoveries of genetic causes of ALS and advances toward treatment through stem cell therapy are taking place right now. We have new understanding of environmental factors contributing to ALS. The ALS Association itself has raised and directed more than \$55 million to support ALS research in the past decade.

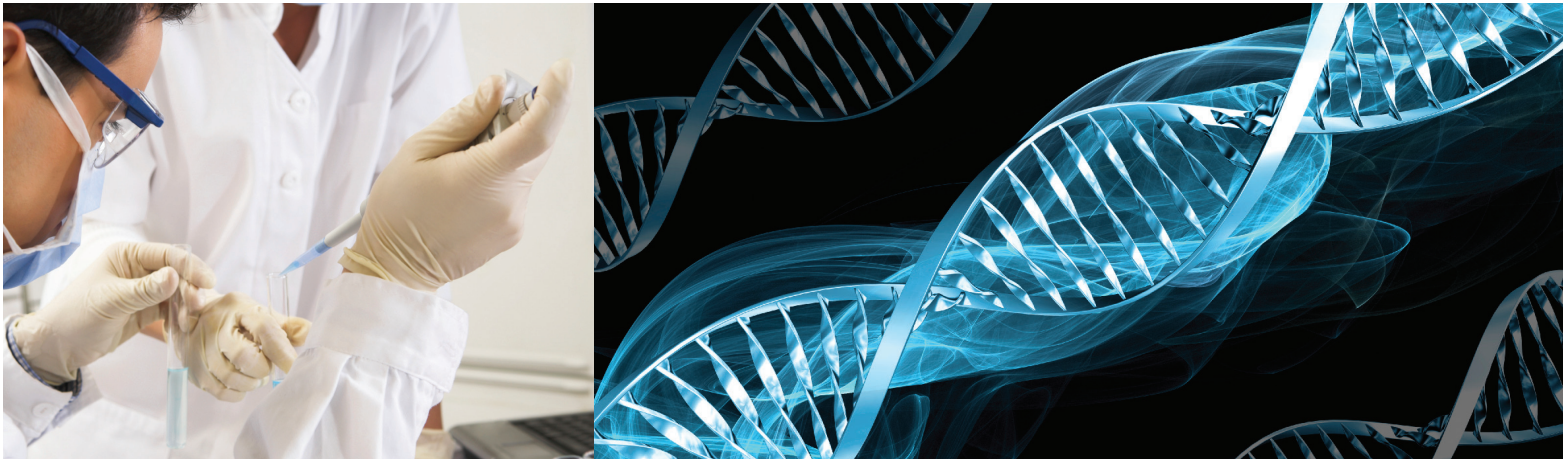
Meanwhile, expert and compassionate professionals in our chapters, clinics and certified centers are doing everything they can to make life better for those who have ALS. And our advocacy program continues to produce resources, visibility and support for the ALS community.

**But for the thousands of people with ALS today and those diagnosed in the months ahead, progress and results are not the same thing.** There still is no cure or effective treatment for ALS. Unless we can treat and prevent ALS soon, Jenny will die and Ron's children may receive the devastating diagnosis that they have ALS.

**We are in a race that we must win.**

Right now as many as  
**30,000**  
**Americans**  
are fighting for their  
lives against ALS.

TREAT ALS is designed  
to speed progress in  
the race to  
save lives.



**The ALS Association's TREAT ALS** (T<sup>r</sup>anslational **R**esearch **A**dvancing **T**herapies for ALS) program is designed to speed progress in the race to save lives. TREAT ALS accelerates the drug discovery process by forging relationships between academic research institutions and industry. Through TREAT ALS, contracts are provided to biotech and other industry partners to support the development of novel treatments and to establish preclinical data that will expedite clinical trials. TREAT ALS supports pilot clinical trials and The ALS Association has established a network to improve access to trials throughout the United States. Each component of TREAT ALS is intended to support great research and to produce results quickly.

TREAT ALS harnesses the tools of scientific discovery and development—laboratory research, model systems for ALS, the development of biomarkers to identify disease progression, and clinical trials to validate safety and efficacy—and uses them to drive a process that begins in early discovery and culminates in clinical management of ALS.

Researchers funded by The Association have been international leaders in the development of model systems for the disease and in finding biomarkers for ALS. The ALS Association has the relationships with academic institutions, biotech and pharmaceutical companies and government agencies to make it all work.

To win the fight against ALS, we need an integrated program that attracts generous investment; supports innovative science; expedites clinical and regulatory process toward therapy development and delivers safe and effective therapy to persons with ALS at the earliest possible moment.

**We need TREAT ALS, empowered by transformative funding from members of The Laureate Society.**



*Lucie Bruijn, Chief Scientist  
for The ALS Association.*

## **THE ALS ASSOCIATION SCIENTIFIC ADVISORY BOARD**

David Borchelt, Ph.D., *University of Florida*

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Disease Initiative*

Alfred Sandrock, M.D., Ph.D., *Biogen Idec, Inc.*



**The Association is funding projects in every phase of the development pipeline.** TREAT ALS has been instrumental in moving to clinical trial a very promising therapeutic research known as antisense and has funded the past work of the chief neurologist and the neurosurgeon leading a stem cell injection trial at Emory University in Georgia.

It is going to cost many millions of dollars to reach the finish line in the race to treat and cure ALS, and to continue to care for those in every state who are living with ALS. The average cost today of putting a new drug through regulatory requirements and clinical trials is more than \$1 billion. ALS is a complex disease and a combination therapy is likely to be required.

**Defeating ALS will be expensive and difficult. But it will be done.**

## TREAT ALS PIPELINE

Global Peer-Reviewed Biomedical Research

**TREAT ALS provides crucial tools necessary to defeat Lou Gehrig's Disease.**

- TREAT ALS speeds progress by:
  - Contracting with partners to develop treatments.
  - Establishing pre-clinical data.
  - Expediting clinical trials and delivery of effective therapy.
- Powered by our leadership and global network of top researchers.
- Inspired, funded, and accelerated by The Laureate Society.

LABORATORY

→ CLINIC





## Our Invitation

We seek the present means to treat ALS, to halt or reverse the destructive and ultimately fatal march of Lou Gehrig's Disease as it takes hold of the lives of the individuals diagnosed with ALS, and to provide the best possible service and care for people living with ALS. Support through The Laureate Society will enable us to have these resources at the earliest possible time. Advances in molecular biology, genetics and stem cell science have provided researchers with the most powerful tools they have ever had in the fight against ALS. Our chapters, centers and clinics provide compassionate and expert care for those living with ALS throughout the United States. But we need more financial resources to finish the race, and we must move faster to win it for Jenny, for Ron's children and grandchildren and for thousands of others.

We invite you to join The Laureate Society, a select and committed group of individuals who will succeed in developing effective ALS therapy through TREAT ALS and will lead the way to achieving our shared vision of A World Without ALS.

For more information or to make an appointment to discuss support  
for The ALS Association through The Laureate Society, contact:

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