



ANNUAL REPORT 2023

LETTER FROM OUR CEO



Dear Friends,

Over the past decade, Target ALS has ushered in a transformative era in ALS research, championing collaboration and driving innovation to make significant strides toward effective treatments for those living with ALS. While we take pride in the progress we've made, we are more determined than ever to elevate our efforts as we step into the next phase of our journey.

In 2023, we continued investing in our Seven-Pillar Strategy, increasing our budget to nearly \$13 million. This allowed us to engage new talent and cutting-edge technologies, expand our Innovation Ecosystem globally, and enhance our capacity to meet the growing demands of our mission. We funded 85 grants to 69 scientists across 10 countries, fostering the collaborative consortia that accelerate research. We also provided vital support for emerging talent, empowering them to bring bold ideas that will drive tomorrow's breakthroughs.

Beyond grant funding, we were committed to democratizing research by lowering barriers and ensuring scientists have access to the resources they need for groundbreaking work. A key milestone in 2023 was the successful recruitment of the first 100 participants in our Natural History Study. This study will provide researchers with the most comprehensive collection of biosamples and datasets, which are crucial for understanding the biology of ALS and identifying new therapeutic targets and biomarkers.

Additionally, we launched a diversity initiative, welcoming our first international site for the study in Latin America and creating programs to engage underrepresented communities. These efforts ensured that our work benefits all people living with ALS.

While we've built tremendous momentum in 2023, we are only just beginning. In 2024, we plan to double our budget again to \$26 million, enabling us to continue executing our ambitious agenda. We will further expand our Natural History Study and scientific network internationally, exploring uncharted areas of research that may lead to groundbreaking discoveries, particularly in Sporadic ALS, which accounts for about 90% of cases and remains largely unexplored.

Your generous support is not only fueling these exciting collaborations and innovations but also helping us chart the scientific roadmap toward effective treatments for all forms of ALS. We stand on the brink of a transformational moment in the history of ALS. Now is the time to drive forward with renewed determination, transforming the hope we've inspired in the ALS community into reality.

Thank you for being a vital part of this journey.

A handwritten signature in blue ink that reads "Manish Raisinghani".



Manish Raisinghani
CEO, Target ALS

GRANT PROGRAMS



New Talent, New Ideas

In 2023, we focused on recruiting new talent to our Innovation Ecosystem. By encouraging ideas on ALS research from the next generation of scientists, we're paving the path for future breakthroughs. We provide support to these researchers at different stages of their careers to encourage their long-term interest in ALS. These scientists range from post-doctoral fellows to early-stage clinicians, all of whom play a distinct and important role of bringing new ideas for ALS therapeutics to fruition.

Collaboration Drives Progress

In February 2023, we funded six Industry-Led Consortia, groups including academic investigators with deep knowledge of disease biology and pharmaceutical or biotech companies with expertise in drug discovery. By requiring collaboration between groups with distinct skillsets, we accelerate potential treatments from the laboratory to the clinic. In just one year, three of these groups have achieved significant milestones by advancing molecules into in vivo testing, which evaluates pharmacological activity in mice. This marks an important step before potential drugs can be studied in human trials.



As an early career researcher, I aspire to lead an independent laboratory and mentor the next generation of passionate researchers, including clinician scientists, who are devoted to discovering the molecular underpinnings of ALS. **As a Target ALS Springboard Fellow, I am one step closer to achieving these goals."**

—Dr. Jose Norberto Vargas, Ph.D.
University College London



\$7.4M

Grant Spend

Funding Calls

- Springboard Fellowships
- Early Stage ALS Clinicians
- Industry-Led Consortia
- New Academic Investigators
- In Vivo Target Validation

85 Grants

69 Scientists

54 Institutions

10 Countries

RESEARCH CORES



Democratizing ALS Research

Our eight Scientific Core Facilities provide expedited no-strings-attached access to biosamples, datasets, and reagents for scientists worldwide to carry out their research. These critical resources are often not accessible, especially for ALS research, limiting scientist's ability to perform research and interest in the field. Our Research Cores break down that barrier and have supported over 1,100 ALS projects to date.

8

Research
Cores

\$3.4M

Research
Core Spend

299

Requests
Granted

Data Drives Discovery

In 2023, we continued to maintain and expand our Research Cores, specifically focusing on data. We made several new datasets available, including digitized images brain and spinal cord tissue from ALS cases. We also funded the generation of ST data, a complementary method of analysis that allows scientists to look at spatial changes in genes throughout the brain and spinal cord and how they relate to the motor neurons affected in ALS. To continue facilitating this work, we began creation of data portal and analysis platform in July 2023 to be launched next year. This platform will be the go-to source for data mining and analysis in ALS, driving discovery of new drug targets and biomarkers.

RESEARCH CORES

Genomic Datasets

Human Postmortem Tissue

Longitudinal Biofluids

Antibody

In Vivo Target Validation

Stem Cell

Viral Vector

Animal Models

FINANCIAL SUMMARY



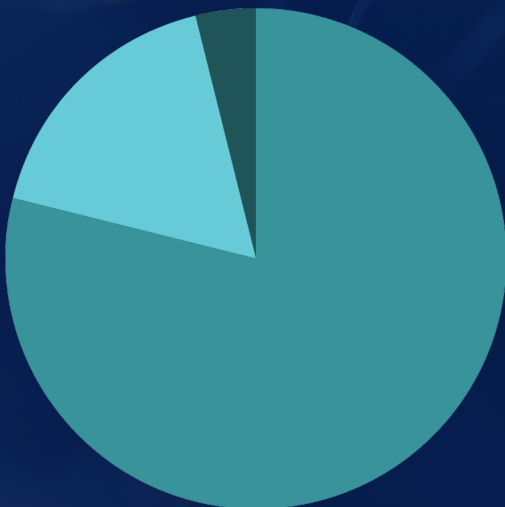
At Target ALS, we are committed to the highest level of financial responsibility and transparency. Your donations propel our mission to break down barriers to ALS research and find effective treatments. Together, we will realize a world where **Everyone Lives**.

Revenue and Support \$79.6M



- **Contributions and Grants**
\$79,148,833
- **Investments**
\$403,757
- **Other**
\$4,197

Expenses \$15.8M



- **Research**
\$12,536,830
- **Administrative**
\$2,714,125
- **Fundraising**
\$594,728

LETTER FROM OUR FOUNDER



Dear Friends,

Since our founding 10 years ago, our success has increasingly relied on collaborations and partnerships between academic researchers, industry leaders, other ALS nonprofits, and organizations focused on neurodegenerative diseases. However, one key constituent that we haven't worked closely with is the federal government. That changed significantly in 2023.

In Fiscal Year 2024, Congress allocated \$75 million under the ACT for ALS, empowering the National Institute of Neurological Disorders and Stroke (NINDS) and the Foundation for the National Institutes of Health (FNIH) to create the Accelerating Medicines Partnership for ALS (AMP ALS), which brings together resources from the private and public sectors to drive critical progress in ALS research.

This year, the NIH will allocate \$43.2 million of the \$75 million provided by Congress to AMP ALS. Along with Stephanie Fradette of Biogen and Amelie Gu-bitz of NINDS, I have the honor of co-chairing AMP ALS. The program will serve as a comprehensive knowledge portal, integrating existing datasets and establishing a database that includes clinical, genomic, and other -omics data, as well as biofluid and postmortem tissue samples. Researchers can mine this collection to develop ALS biomarkers and improve clinical measures for better trial design.

Target ALS has emerged as a key driver of this initiative, guided by our Senior Director of Scientific Programs, Dr. Amy Easton. As part of AMP ALS, NINDS recently launched the Access for All in ALS (ALL ALS) Consortium. Thirty-four clinical sites across the U.S. will participate in a comprehensive Natural History Study of ALS, modeled after our ongoing longitudinal biofluids study. The Barrow Neurological Institute, led by Dr. Robert Bowser, will coordinate the NIH study in the West, backed by a

\$16.7 million grant. All 10 U.S.-based Target ALS sites are participating in this consortium, significantly amplifying our efforts and vastly increasing biofluid and data collection.

Over the past decade, our collaborative Innovation Ecosystem has profoundly transformed the ALS research landscape. Sixty percent of Target ALS-funded consortia have initiated drug discovery programs led by industry partners. Seven clinical trials have emerged from our diverse portfolio, which includes collaborative consortia, individual grants for next-generation scientists, and core facilities that continue to accelerate research globally with urgency and purpose. Our work has proven that progress accelerates when we collaborate, furthering hope that we will achieve a world where everyone with ALS lives.



A handwritten signature in blue ink that reads "Dan Doctoroff".

Dan Doctoroff
Founder, Target ALS



www.targetALS.org