TARGET ALS

ANNUAL REPORT 2024

EVERYONE LIVES

Dear Friends,

As we reflect on 2024, we are proud of the milestones that brought us closer to a world where Everyone with ALS Lives. From launching our groundbreaking Data Engine to expanding our Global Natural History Study into Latin America—and completing our $250 million capital campaign—the year was a powerful testament to our core values: radical collaboration, deliberate disruption, and impatient optimism.

Our scientific strategy continues to focus on three critical areas: understanding ALS biology, advancing drug discovery, and developing biomarkers to support diagnosis, monitoring, and treatment evaluation. In 2024, we balanced our funding across these priorities—38% for biology, 38% for drug discovery, and 24% for biomarker development—while staying agile to fund emerging breakthroughs, like cryptic exons and their role in ALS.

At the same time, we expanded our Research Cores—a unique suite of tools and resources that remove barriers to scientific progress. With the launch of the Data Engine, researchers now have access to multi-omic datasets paired with biofluid and tissue samples, helping translate ideas into potential treatments faster than ever.

Through our ALS Global Research Initiative (AGRI), we're also conducting our own studies to address the lack of diversity in ALS research. In 2024, we expanded internationally, enrolling participants from Colombia, Puerto Rico, South Korea, and Israel, and launched our first Community Pop-Up Clinic in Los Angeles to engage underrepresented groups. These efforts are fueling new insights into genetic, environmental, and biomarker-related contributors to ALS.

We are deeply grateful to the many people who drive our mission forward:

- **People living with ALS and their families**, whose strength inspires everything we do
- **Researchers**, who are pushing the boundaries of what's possible
- **Supporters**, whose belief in our work makes progress possible
- **Our dedicated team**, who ensure every dollar is used strategically to accelerate impact

Together, we're not just funding research—we're shaping the future of ALS science.

Thank you for being a vital part of this journey.

Daniel L Doctoroff

Dan Doctoroff
Founder and Chairman
Target ALS

Manish Raisinghani, M.B.B.S., Ph.D.
Chief Executive Officer
Target ALS

Research we FUND

Target ALS has become the largest private funder of ALS research across the globe. With gratitude to the unrestricted nature of our donor funds, we have the unique ability to fund the best talent and most promising ideas anywhere in the world. In 2024, we launched several funding opportunities to fuel innovative science in our three main focus areas:

1. **Understanding the basic biology and underlying causes of ALS**, providing scientists with new ideas for transformational therapies.
2. **Accelerating drug discovery** by supporting proof-of-concept studies for new therapeutics required for advancement into clinical trials.
3. **Developing biomarkers**, critical for new diagnostics and early detection, identifying and stratifying subtypes of ALS, and monitoring disease progression.

Each grant program was strategically designed to leverage emerging biology and technology and to address areas of high unmet need. In addition, all Target ALS funding opportunities aim to encourage multi-disciplinary collaborations and attract new scientists and ideas into the field. This year, we were able to **launch six competitive funding opportunities and fund 67 projects, investing $15.5 million in grants.**

Basic Biology Consortia

Understanding ALS Biology

Six collaborative teams have been awarded grants through our 2024 Basic Biology Consortia Program to study the biology of sporadic ALS, which accounts for 90% of cases. By encouraging cross-disciplinary collaboration and the use of new technologies, the program aims to uncover new drug targets and biomarkers—advancing treatment options for all people living with ALS.

New Therapeutic Modalities

Drug Discovery

Recent advances in technology have enabled new therapeutic approaches for Central Nervous System (CNS) disorders. In 2023, the FDA approved Tofersen—the first effective treatment for SOD1 ALS, a genetic form of the disease affecting ~2% of patients. Delivered via monthly spinal injection, Tofersen reduces mutant SOD1 levels and has shown significant clinical benefit. Its success demonstrates the potential of targeting genetic mutations, while also underscoring the need for therapies that can cross the blood-brain barrier and be delivered less invasively. To advance this next generation of DNA- and RNA-targeting treatments, Target ALS launched a new collaborative funding opportunity in late 2024, with selected consortia to be announced in Spring 2025.

In Vivo Target Validation

Drug Discovery

In 2024, Target ALS launched two In Vivo Target Validation grant programs to support preclinical testing of promising ALS therapeutics in animal models—a critical step in advancing treatments from lab to clinic.

TDP-43 Program | Biospective

Five biotech companies received in-kind support to test therapies in a mouse model expressing mutant TDP-43, a protein implicated in 97% of ALS cases. These studies aim to generate key data for Investigational New Drug (IND) applications to the FDA.

C9orf72 Program | The Jackson Laboratory, in collaboration with the ALS Association

Two projects were selected to evaluate therapies targeting the most common genetic cause of ALS, with studies launched in early 2025.

Together, these programs expand "shots on goal" in the ALS drug pipeline by overcoming cost and access barriers to in vivo testing.

Neurology Resident Grants

Drug Discovery

Biomarker Development

Clinicians and clinician-scientists play a vital role in ALS research, bridging patient care with scientific discovery. To support the next generation of leaders in this space, Target ALS launched the Neurology Resident Grant program in March 2024. Three early-career clinician-scientists have been awarded funding to pursue innovative projects that aim to translate research into real-world impact—advancing drug and biomarker development while improving care for people living with ALS.

Biomarker Consortia Grants

Biomarker Development

Biomarkers are essential tools for diagnosing disease, tracking progression, and improving clinical trial design. In ALS, identifying reliable biomarkers has been especially challenging due to the complexity of the brain and spinal cord. To address this, Target ALS launched the Biomarker Consortia Grant Program, drawing 139 scientists across 34 collaborative proposals from 17 countries. Five teams were selected to advance biomarker discovery—paving the way for more precise diagnostics, better trial design, and personalized treatment strategies for people living with ALS.

About our review process

At Target ALS, fairness and transparency are core to how we fund research. All funding decisions are made solely by the Independent Review Committee (IRC)—with no involvement from Target ALS staff or leadership—to ensure every application is evaluated on merit. What sets our process apart?

- **Expertise across sectors**: The IRC is composed of leading scientists from both academia and the biotech/pharma industry, with balanced 50/50 representation to reflect the evolving nature of ALS research.
- **No conflicts of interest**: IRC members cannot apply for or receive Target ALS funding for their own work.
- **Strong governance**: All members follow a strict conflict of interest policy and confidentiality agreement.
- **Merit-based selection**: There is no set number of grants per cycle—the IRC selects only the strongest proposals for funding.

Research we ENABLE

Target ALS Research Cores: Empowering Global ALS Discovery

Target ALS is accelerating ALS research by offering no-strings-attached access to critical tools and resources through our Research Cores—eliminating barriers like limited access, high costs, and lack of standardization to advance drug and biomarker discovery.

Target ALS Data Engine

Launched in March 2024, the Target ALS Data Engine accelerates biomarker and drug discovery by providing no-strings-attached access to comprehensive, multi-omic data from our Research Cores. Developed with DNAstack and Verily, the platform enables researchers to analyze DNA, RNA, and protein data to uncover new ALS targets. Updated regularly and freely accessible, the Data Engine has already been used by over 250 scientists worldwide—driving faster, more collaborative research.

Postmortem Tissue Core

The Target ALS Postmortem Tissue Core is the world's largest ALS-focused biobank, providing high-quality brain and spinal cord tissue—and associated clinical data—to researchers worldwide. With over 50,000 samples from 532 donors and six geographically distributed sites, including a new international site at the University of Edinburgh, the Core has supported more than 300 labs and hundreds of studies over the past decade to drive discovery in ALS and related diseases like FTD.

Longitudinal Biofluids Core

The Target ALS Longitudinal Biofluids Core offers the most comprehensive collection of ALS biofluids—CSF, blood, and urine—paired with rich clinical and demographic data to support biomarker and treatment development. Samples are collected through our Global Natural History Study and distributed globally, fulfilling 12 approved requests in 2024—some within just 12 days. The Core is powered by a growing international consortium of 14 ALS clinics, including a new site in Bogotá, Colombia. Researchers also gain access to integrated datasets via our Data Engine, enabling advanced, multi-layered analysis of disease progression across diverse populations.

Stem Cell Core

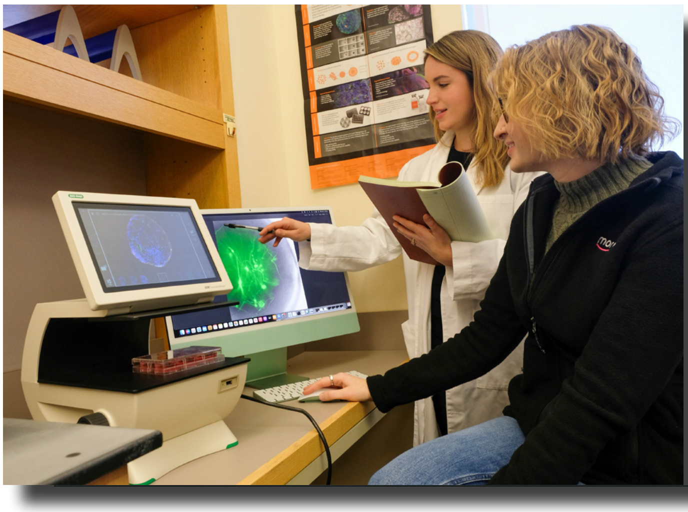
The Target ALS Stem Cell Core provides researchers with high-quality induced pluripotent stem cell (iPSC) lines derived from people with ALS and healthy controls—critical tools for studying disease mechanisms and testing new therapies. Unlike traditional animal models, iPSCs can be turned into human motor neurons and glial cells, offering more accurate insights into ALS biology. To address challenges with reproducibility and genetic variability, Target ALS launched an international consortium in 2024 to develop standardized, genetically defined iPSC lines—including isogenic controls for common ALS mutations. These rare, technically complex resources, along with detailed protocols and data, will be shared globally to accelerate ALS research and therapeutic development.

Animal Models Core

The Target ALS Animal Models Core accelerates preclinical research by removing common barriers to accessing and using ALS animal models. It supports three key areas: funding therapeutic testing at contract research organizations (CROs), developing new mouse models, and generating multi-omic data for integration into the Data Engine. In 2024, we launched a partnership with Biospective to support studies using a well-characterized TDP-43 mouse model—representative of pathology found in 97% of ALS cases. This effort helps researchers without in-house animal facilities advance promising drug candidates toward clinical trials.

Reagents Core

The Target ALS Reagents Core provides high-quality antibodies to support ALS research, including a new, validated antibody for phosphorylated TDP-43 (pTDP-43)—a hallmark of ALS pathology present in 97% of cases. Developed in collaboration with Dr. Len Petrucelli and the Developmental Studies Hybridoma Bank, this antibody addresses a long-standing barrier by offering reliable, affordable access for researchers worldwide. Demand was immediate and high, with the first batch fully distributed within a month. To meet ongoing needs, production will triple in early 2025, accelerating efforts to understand and treat ALS.



Dr. Kathryn Morelli, PhD, and Abby Kirch, PhD student, working in the Morelli Lab at University of Vermont. Dr. Morelli is the recipient of a Target ALS Springboard Fellowship and has utilized several Target ALS Research Cores, including the Stem Cell Core and Reagents Core to further her work developing RNA-targeted therapies for ALS.

Research we CONDUCT

ALS Global Research Initiative (AGRI)

Target ALS launched AGRI to address critical gaps in ALS research by conducting two large-scale clinical studies aimed at identifying causes of ALS and discovering novel biomarkers to diagnose, predict, and monitor disease progression—particularly across diverse populations.

Global Natural History Study (GNHS)

A longitudinal study collecting biofluids (CSF, blood, urine) and clinical data from people with ALS and healthy controls. With enrollment more than doubling in 2024 (now 182 participants), the study spans 14 global sites and has banked over 17,000 samples. Data is accessible via the Target ALS Data Engine to accelerate biomarker discovery and therapeutic development.

Community-Based Pop-Up Clinics

One-time blood and data collection events focused on underrepresented racial and ethnic groups to study genetic and environmental risk factors. The goal is to enroll 5,000 ALS participants and 1,000 controls. The first event took place 2024 at Kaiser Permanente in Los Angeles, with strong participation and diverse representation. A second event is scheduled for February 2025 in Phoenix, AZ.

AGRI is advancing inclusive ALS research by breaking down barriers to participation and ensuring that future treatments are safe and effective for everyone living with ALS—regardless of race, ethnicity, or geography.

The Target ALS Annual Meeting:

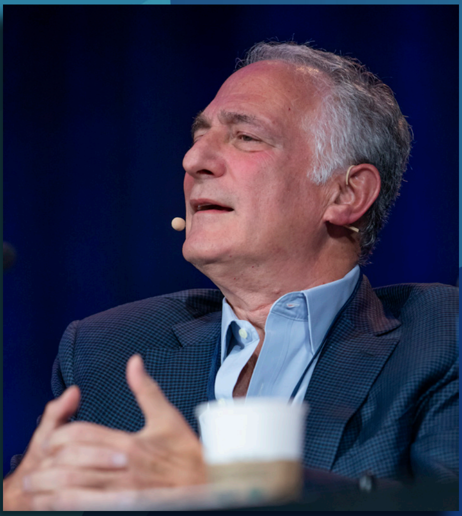
Driving Collaboration and Breakthroughs

Held each spring, the Target ALS Annual Meeting is the premier global conference for ALS research, uniting our Innovation Ecosystem across academia, biotech, pharma, venture capital, and nonprofit sectors. The 2024 meeting marked our largest yet, with over 850 attendees joining in person and virtually.

What sets this meeting apart is its focus on radical collaboration—featuring the sharing of confidential, cutting-edge research and real-time expert feedback. By bringing every key stakeholder into one room, the meeting sparks partnerships that accelerate the path from discovery to treatment.

As our ecosystem grows, so does the momentum toward a future where Everyone Lives. We look forward to continuing this progress at the 2025 Annual Meeting.

Dan Doctoroff addressing the room during his opening remarks.



Dr. Fanny Elahi, Mount Sinai, member of the Target ALS IRC, moderating a Q&A session.

Scientists listening to presentations at the 2024 Target ALS Annual Meeting.

Drs. Caroline Ingre, Karolinska Institutet, (center) and Mai Yamakawa, UCLA, (right), receipients of Target ALS Early Stage ALS Clinician grants, taking questions after their presentation, moderated by Dr. Michela Deleidi, DZNE, (left).

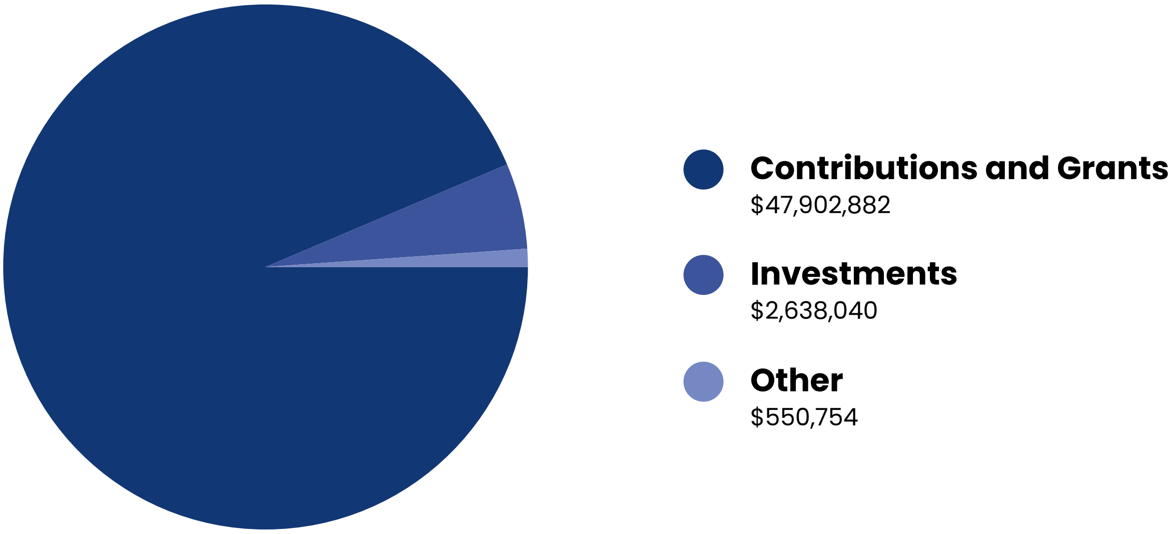
Financial Summary

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. Our audited financial reports and 990s are available on our website at www.targetals.org/financial-reports.

Together, we will realize a world where **Everyone Lives**.

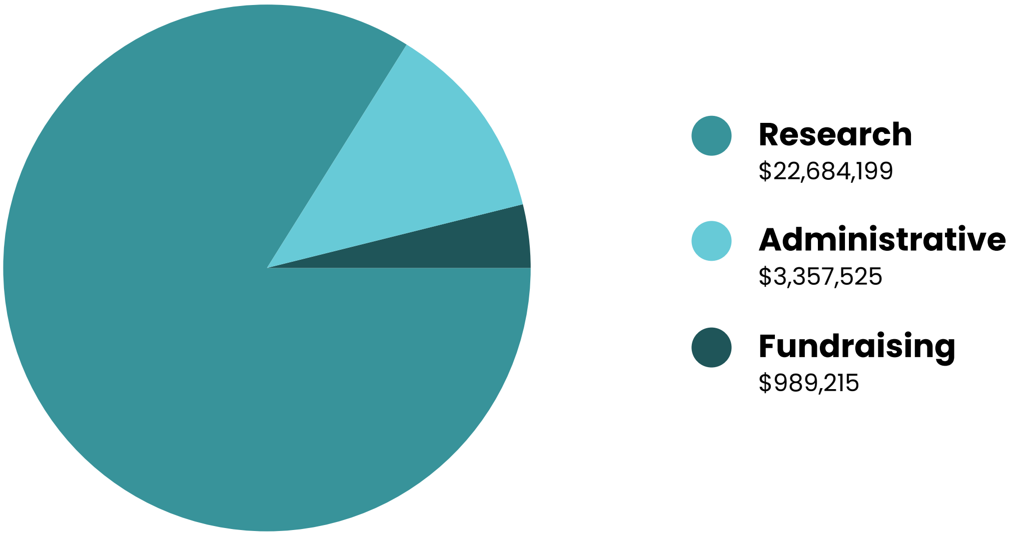
Revenue and Support

$51.1M



Expenses

$27M



Our Team

Board of Directors

- **Daniel L. Doctoroff**, Founder & Board Chair, Target ALS, Founder & Former CEO, Sidewalk Labs Former CEO, Bloomberg LP Former Deputy Mayor of Economic Development and Rebuilding for the City of New York
- **Andy Berndt**, Vice President and Co-Founder, Google Creative Lab
- **Alisa Doctoroff**, Philanthropist
- **John Dunlop, Ph.D.**, Former Chief Scientific Officer, Aliada Therapeutics
- **Steven Gruber**, Investor
- **Zach Hall, Ph.D.**, Former Director, NIH NINDS
- **Richard Hargreaves, Ph.D.**, Corporate Vice President, Bristol-Myers Squibb
- **Chris Henderson, Ph.D.**, Chair, Scientific Advisory Board, Alltrna
- **Carole Ho, M.D.**, Chief Medical Officer & Head of Development, Denali Therapeutics
- **Allison Jaffin**, Chief Operating Officer, Bloomberg Philanthropies
- **Brad S. Karp**, Chairman, Paul, Weiss, Rifkind, Wharton & Garrison LLP
- **Story Landis, Ph.D**., Former Director, NIH NINDS, Board Liaison, Independent Review Committee
- **Sarah I. Sheikh, BM BCh, MSc, MRCP**, Senior Vice President, Neuroscience, Takeda
- **Stacie Weninger, Ph.D.**, President and Venture Partner, F-Prime Biomedical Research Initiative

Independent Review Committee

- **Robert Miller, Ph.D.**, Chair, Target ALS IRC, Vice Dean for Research & Academic Affairs, George Washington University
- **Michael Ahlijanian, Ph.D.**, Chief Scientific Officer, Pinteon Therapeutics
- **Eva-Maria Albers, Ph.D.**, Professor and Group Leader, IDN, Johannes Gutenberg Universität Mainz
- **Sophia Bardehle, Ph.D.**, Sr Principal Scientist, In Vivo Pharmacology, Abbvie
- **Manzoor Bhat, Ph.D., M.S.**, Vice Dean for Research (Interim), Professor & Chair, UT Health Science Center, San Antonio
- **Martha R.C. Bhattacharya, Ph.D.**, Associate Professor of Neuroscience and BIO5 Institute, University of Arizona
- **Dean Brown, Ph.D.**, SVP and Head of Discovery Sciences, Jnana Therapeutics
- **Elizabeth D. Buttermore, Ph.D.**, Director of Translational In Vitro Models, Rosamund Stone Zander Translational Neuroscience Center, Boston Children's Hospital
- **Moses Chao, Ph.D.**, Professor, Department of Neuroscience & Physiology, New York University
- **Merit Cudkowicz, M.D.**, Chief, Neurology Department; Director, Sean M. Healey & AMG Center for ALS; Julieanne Dorn Professor of Neurology, Harvard Medical School
- **Vardhan Dani, Ph.D.**
- **Michela Deleidi, M.D., Ph.D.**, Helmholtz Group Leader, DZNE German Center for Neurodegenerative Diseases, University of Tübingen
- **Nino Devidze, Ph.D.**, VP, Medical, SSI Strategy
- **Biljana Djukic, Ph.D.**, Translational Medicine and Early Development Lead, Neurology and Rare Diseases, Sanofi

Independent Review Committee (continued)

- **Fanny Elahi, M.D., Ph.D.**, Asst. Professor of Neurology & Neuroscience; Asst. Professor of Pathology, Molecular and Cell-Based Medicine, Icahn School of Medicine at Mount Sinai
- **Lisa Ellerby, Ph.D.**, Professor, Buck Institute
- **Toby Ferguson, M.D., Ph.D.**, Chief Medical Officer, Voyager Therapeutics
- **Ana Fiszbein, Ph.D.**, Assistant Professor, Boston University
- **Brad Friedman, Ph.D.**, Senior Principal Scientist, Department of OMNI Bioinformatics, Genentech, Inc.
- **Judith Frydman, Ph.D.**, Donald Kennedy Chair, School of Humanities and Sciences and Professor of Genetics, Frydman Lab, Stanford University
- **Sonia Gandhi, M.D., Ph.D.**, Professor of Neurology, University College London,, The Francis Crick Institute
- **Volkan Granit, M.D., MSc**, Medical Director, Clinical Development, Biohaven Pharmaceuticals
- **Orla Hardiman, M.D., FRCPI**, Professor of Neurology/Head of Academic, Clinical Medicine, Trinity College of Dublin
- **Erika Holzbaur, Ph.D.**, Professor of Physiology, University of Pennsylvania, Perelman School of Medicine
- **Eric Huang, M.D., Ph.D.**, Professor of Pathology, University of California, San Francisco
- **Aimee Kao, M.D., Ph.D.**, John Douglas French Foundation Endowed Professor; Director, Medical Scientist Training Program, Memory and Aging Center, University of California, San Francisco
- **Zaven Kaprielian, Ph.D.**, Chief Scientific Officer, Remix Therapeutics
- **Holly Kordasiewicz, Ph.D.**, Vice President, Neurology Research, Ionis Pharmaceuticals
- **Sarah Kucenas, Ph.D.**, Professor of Biology, Cell Biology, and Neuroscience, University of Virginia
- **Jim Kupiec, M.D.**, Chief Clinical Development Officer, Cassava Sciences
- **Claire Le Pichon, Ph.D.**, Principal Investigator, NIH Unit on the Development of Neurodegeneration
- **Pamela Maher, Ph.D.**, Research Professor, Cellular Neurobiology Laboratory, Salk Institute
- **Robert Mays, Ph.D.**, Executive Vice President of Regenerative Medicine & Head of Neuroscience Programs, Athersys
- **Alexander McCampbell, Ph.D.**, Global Head of Neuroscience & Rare Diseases Research, Roche
- **Jere Meredith, Jr., Ph.D.**, Senior Director, Head of Neurology Discovery, Eisai Co, Ltd
- **Axel Methner, M.D.**, Professor of Neurology, University Medical Center Mainz
- **Kelly R. Monk, Ph.D.**, Professor and Co-Director, Vollum Institute, Oregon Health & Science University
- **Ralda Nehme, Ph.D.**, Principal Investigator and Director of Stem Cell Program, Stanley Center for Psychiatric Research, Broad Institute of MIT and Harvard
- **Richard Olson, Ph.D.**, Former Director, Small Molecule Drug Discovery, Bristol Myers Squibb
- **Manuela Polydoro, Ph.D.**, VP, Head of Translational Medicine, Muna Therapeutics
- **Mahendra Rao, M.D., Ph.D.**, Chief Executive Officer, Implant Therapeutics
- **Ian Reynolds, Ph.D.**, President, YaghPenn Consulting
- **Tracy Saxton, Ph.D.**, Managing Director, Saxton Biopharma Consulting, LLC
- **Rob Scannevin, Ph.D.**, Chief Scientific Officer, Verge Genomics
- **Kimberly Scearce-Levie, Ph.D.**, Vice President, Translational Sciences, Denali Therapeutics
- **Eric Schaeffer, Ph.D.**, Chief Scientific Officer, Paros Bio
- **Abdoulaye Sene, Ph.D.**, VP, Translational Science, Ophthalmology, Kartos Therapeutics
- **Holly Soares, Ph.D.**, Head of Precision Medicine, VP, Pfizer
- **Ajay Verma, M.D.**, Ph.D.
- **Ottavio Vitolo, M.D.**, Neuropsychiatrist, Massachusetts General Hospital
- **Frank Walsh, Ph.D.**, Founder and Chief Executive Officer, Ossianix
- **Andrew Wood, Ph.D.**, VP, Clinical Neuroimaging Research, CHDI
- **Nicholas Wood, Ph.D.**, UCL Professor of Genetics and Neurology, University College London, Institute of Neurology
- **Choya Yoon, Ph.D.**, Associate Principal Scientist, Merck
- **Stephen Zicha, Ph.D.**, Senior Director, Eli Lilly and Company

Staff

- **Manish Raisinghani M.B.B.S., Ph.D.**, Chief Executive Officer
- **Jessica Chapman, MBA, CAP**, Chief Development Officer
- **Kenneth Devaney**, Chief Operations Officer
- **Amy Easton, Ph.D.**, Senior Director of Scientific Programs
- **Yuliya Rzad**, Chief Grants Officer
- **Courtney Christa**, Accounts Payable Specialist
- **Laura Dugom**, Clinical Research Scientist
- **Ellen Guss, Ph.D.**, Scientific Programs Coordinator
- **Aiden Hipwell, MBA**, Marketing Coordinator
- **Ruby Hoglund**, Administrative Assistant
- **Stephanie Ishoo**, Manager, Research Communciations and Marketing
- **Ariella Leicht,** Development Operations Manager
- **Christine Aranyos Prouty**, Director of Philanthropy
- **Clint Ramnarine**, Grants and Database Manager
- **Burhan Siddiqui**, Accounting and Financial Systems Manger
- **Tammy Sutherns**, Manager of Mission Communications

Upper Left: Target ALS Board of Directors 2024
Upper Right: Target ALS Independent Review Committee 2024
Left: Target ALS Staff 2024

All photos taken at the 2024 Target ALS Annual Meeting

Thank you for being an essential part of this mission. Your trust, generosity, and belief in our vision empower us to make an impact every day. We look forward to building on this momentum in the year ahead and making 2025 a year of unprecedented progress — together.

TARGET
ALS

www.targetALS.org