EVERYONE LIVES

TARGET ALS NEWSLETTER | VOL. 3 | WINTER 2023 / 2024

CELEBRATING 10 YEARS OF TARGET ALS

DRIVING PROGRESS: **HIGHLIGHTS FROM OUR SEVEN-PILLAR STRATEGY**

CHAMPIONING COLLABORATION ACROSS CONSTITUENCIES

TARGET ALS

LETTER FROM OUR CEO

Dear Friends,

Over the last ten years, Target ALS has sparked a new era in ALS research, championing collaboration and fueling innovation to advance breakthroughs that will lead to effective treatments for people living with ALS. While we are immensely proud of the impact we've already made, more importantly, we are galvanized to radically scale up our efforts as we embark on the next decade of our journey.

Guided by the trailblazing leadership of our Founder, Dan Doctoroff, and with your generous support, we are extremely close to achieving Dan's $250 million fundraising goal to realize our shared vision: a world where Everyone Lives with ALS.

In 2023, we intensified work across all aspects of our Seven-Pillar Strategy, doubling our budget to nearly $13 million to engage new talents and technologies, expand our Innovation Ecosystem globally, and build out the capabilities necessary to undertake the growing breadth and depth of our work. We funded 109 grants to 75 scientists in 11 countries for the collaborative consortia that we have proven accelerate research as well as support for new talent to enter the ALS space with bold ideas to drive tomorrow's successes. Beyond grant support, we democratize research by lowering barriers and providing access to the resources that scientists need to carry out their groundbreaking work. A major milestone, we successfully recruited the first 100 participants in our Natural History Study, which will provide researchers with the most comprehensive collection of biosamples and datasets needed to understand the biology of ALS and identify novel therapeutic targets and biomarkers. We also launched a diversity initiative, onboarding our first international site for the study in Latin America and offering programs to engage underrepresented communities, ensuring our work benefits all people living with ALS. While we have built incredible momentum this year, we are just getting started.

In 2024, we will again double our budget to $26 million to continue to execute our ambitious agenda. We aim to push both

> *As we celebrate our tenth anniversary, I am humbled by your encouraging support and proud of all that we have accomplished together.*

the physical boundaries of our ecosystem, further expanding our Natural History Study and scientific network internationally, as well as the biological frontiers of our understanding of ALS, exploring uncharted areas of study that may illuminate game-changing discoveries – specifically for Sporadic ALS, which accounts for about 90% of cases and is not yet well understood. Thanks to your generous support, we are not only providing funds for the exciting collaborations and innovative researchers featured in these pages, but we are drawing the scientific roadmap to discover effective treatments for all forms of ALS.

As we celebrate our tenth anniversary, I am humbled by your encouraging support and proud of all that we have accomplished together. We have arrived on the precipice of a transformational moment in the history of ALS. We have proven our model and laid the groundwork. We are decoding how and why this disease manifests and what we need to do to beat it. Now is the time to emphatically drive forward, turning the hope for effective treatments that our work has inspired in the ALS community into reality.

Thank you for joining us on this journey. From myself and the entire Target ALS team, we wish you a Happy New Year.

Sincerely,

Manish Raisinghani
CEO, Target ALS

Target ALS Seven-Pillar Strategy

PILLAR
01

Diversification and Expansion of Talent

To pave the path for future breakthroughs, we must encourage ideas on ALS research from the next generation of scientists. **We provide support to these researchers at different stages of their careers to encourage their long-term interest in ALS** through two of our established grant programs: New Academic Investigators and Springboard Fellowships. This year, we awarded grants to six New Academic Investigators and seven Springboard Fellows, advancing our understanding of disease biology in genetic and sporadic forms of ALS and developing novel therapeutics. In addition to funding, we cover all costs associated with utilizing Target ALS core facilities and offer grantees the opportunity to present and network at Target ALS Annual Meetings, eradicating barriers that could hinder emerging scientists from advancing their work.

New Academic Investigator Grants

New Academic Investigator Grants support scientists who have established their own lab in the last five years through a three-year award. One of this year's awardees, Javier Oroz, PhD, aims to improve the efficiency of ALS diagnosis, which remains notoriously difficult. Dr. Oroz's lab is studying the biological structure of TDP-43, a protein that is dysfunctional and aggregated in the brain of ~97% of ALS cases. By using cutting-edge structural biology techniques (scientific methods that give researchers a deep understanding of the 3D shape of proteins) and nanobodies (tiny molecules that can pinpoint parts of misfolded TDP-43 protein), Dr. Oroz aims to both identify TDP-43 in patient samples to reliably diagnose ALS and stratify patients based on disease progression to run more effective clinical trials.

New Academic Investigator

Javier Oroz, PhD	Principal Investigator Consejo Superior de Investigaciones Científicas, Madrid, Spain
	"Our work is based on understanding the structural basis of TDP-43 pathophysiology by advanced structural biology techniques. Our detailed mechanistic understanding of TDP-43's gain of toxic function is a superb opportunity to establish the protein as invaluable substrate for disease diagnosis. **Target ALS's support represents a unique opportunity to obtain access to valuable patient samples from different biobanks by networking us with clinicians worldwide."**

PILLAR
01

Diversification and Expansion of Talent

Springboard Fellowships

Springboard Fellowships facilitate the transition of postdoctoral fellows to establish their own lab through a portable 3-year grant. Dr. Aude Chiot, PhD, a Springboard Fellow, studies selective resilience of motor neurons in ALS. While motor neuron degeneration is the hallmark of the disease, some motor neurons, like those in the oculomotor cortex (part of the brain that controls eye movement), are spared. Dr. Chiot's work compares populations of microglial cells — cells that are responsible for inflammation and immune response in the nervous system — around neurons that degenerate in ALS versus those that are spared. Through this comparison, she aims to decipher how the spared neurons are protected by microglia, identifying innovative drug targets to treat ALS.

Springboard Fellow

Aude Chiot, PhD	Postdoctoral Fellow Oregon Health and Science University
	The generous support of the Springboard Fellowship from Target ALS is absolutely pivotal to the success of my research work. Not only does this funding provide the resources necessary for conducting in-depth investigations into the mechanisms underlying motor neuron resilience in ALS, but it also serves as a significant recognition of the importance and potential impact of our work within the scientific community. Moreover, the grant facilitates the expansion of my professional network, fostering collaborations with peers and experts across diverse fields, which undoubtedly enhances the breadth and depth of our research. **This support not only advances my current research goals but also lays a solid foundation for my future endeavors as a Principal Investigator, ensuring the continuity of vital research in the fight against ALS."**

PILLAR
02

Maintenance and Expansion of Scientific Core Facilities

Our eight Scientific Core Facilities democratize ALS research by providing expedited no-strings-attached access to biosamples, datasets, and reagents for scientists worldwide to carry out their research. **Over 1,100 ALS projects have already benefited from use of these resources worldwide**.

An enduring barrier to investigating the evolution of ALS over time in patients, known as a Natural History Study, is the lack of ethnic, genetic, and geographic diversity among study participants. A diverse participant pool allows for identification of novel genetic risk factors, deeper understanding of environmental contributors to disease progression and development, and a more complete grasp of disease epidemiology. Together, these learnings can lead to the discovery of biomarkers not realized in studies of more uniform populations and new drug targets to support treatment development across the spectrum of patients.

Breaking down this barrier, we endeavored to create the most diverse and comprehensive Natural History Study for ALS through our Longitudinal Biofluids Core and make all data and samples available to researchers worldwide to accelerate biomarker discovery. The study aims to enroll at least 800 ALS patients and 200 healthy controls, **ensuring participation from a diverse population through a three-pronged strategy**:

- Establish strategic partnerships with international ALS clinics
- Provide reimbursement for travel, meals, and lodging for all study participants
- Initiate outreach to underrepresented communities and offer a low–burden method to participate in research via at-home blood collection kits

As part of our initiative to engage international sites, we are proud to work with Dr. Martha Peña from Instituto Roosevelt in Bogotá, Colombia. Dr. Peña has been working with the ALS community in Colombia since 2005. The diverse ethnic backgrounds represented in Latin America have led to complex genotypes among people with ALS. By joining the Target ALS Natural History Study, Dr. Peña will expand our investigation of the relationship between the genetic makeup of people with ALS and their clinical presentations.

ALS biofluid onboarded and expected collection sites

U.S. SITES

1. Barrow Neurological Institute, AZ
2. Columbia University, NY
3. Georgetown University, Washington DC
4. Mayo Clinic, FL
5. Massachusetts General Hospital, MA
6. Northwestern University, IL
7. University of California San Diego, CA
8. University of Washington, WA
9. Washington University, MO
10. University of Puerto Rico, PR

OUTSIDE U.S. SITES

11. Neurology Research Corporation of Santiago, Chile
12. Instituto Roosevelt, Colombia
13. Hebrew University of Jerusalem, Israel

PILLAR
03

Applying the Target ALS Model to Related Diseases

Neurodegenerative diseases primarily involve the degeneration, or breakdown, of neurons. ALS is a disorder of motor neurons. These cells are found throughout the body and control muscle movement through signals from the brain; their degeneration leads to progressive muscle weakness. Other neurodegenerative diseases, like Frontotemporal Dementia (FTD), involve degeneration of neurons within the cortex of the brain, leading to cognitive and behavioral changes. Despite these differences, **ALS and FTD have been found to share common genetic causes and underlying disease biology.** Further, 50% of ALS patients have symptoms associated with FTD, with 15% receiving an FTD diagnosis. Better understanding of the ALS-FTD spectrum — how and why these diseases manifest, both separately and together — can pinpoint what factors contribute to disease development. These factors, or biomarkers, can help doctors better diagnose and track disease as well as help researchers identify new drug targets.

Dr. Hemali Phatnani, PhD, Assistant Professor of Neurological Sciences at Columbia University and Core Faculty Member and Director of the Center for Genomics of Neurodegenerative Disease (CGND) at the New York Genome Center, is working to answer elusive questions that can transform our understanding of ALS-FTD.

Dr. Phatnani's work involves applying spatially resolved multi-omic analysis (mapping what genes and proteins are active in which types of cells in the brain and spinal cord) to ALS and FTD cases. This analysis offers a hyperlocal understanding of cellular relationships in disease-impacted brain and spinal cord regions. Dr. Phatnani's efforts will decode which specific genes are changing in healthy or sick neurons and neighboring cells. Additionally, since the tissues come from well-characterized cases of ALS and FTD through the Target ALS Postmortem Tissue Core, we will learn how these cellular processes relate to the clinical presentation of disease.

Not only will this research validate spatial distribution of both genes of interest and cellular pathways expressed in ALS-FTD, it will develop novel methods for analysis of this type of data and provide a strategy to improve our understanding of other neurodegenerative diseases. Aligned with Target ALS's commitment to collaboration, this dataset will be shared with the larger scientific community to fuel innovation in discovery of new drug targets.

Hemali Phatnani, PhD	Assistant Professor, Columbia University Director, New York Genome Center
	"Target ALS funded my first research project, where we aimed to bring new technologies in spatial genomics to answer questions about disease progression and how gene expression changes happen in space and time over the course of disease. This project is a maturation of that work, which we are applying to samples from the Target ALS Postmortem Core and merging with studies in cell-based systems to really understand the role of ALS-associated mutations and how they affect interactions within cellular neighborhoods. The culture that Target ALS builds and reinforces has had a positive impact on the community. It's been wonderful to be a part of that and to watch how it has affected a generation of trainees. **The emphasis that they put on collaborative research, never losing sight of the translational importance of the work, bringing together academia and industry — all of these things are what sets Target ALS apart from others in the space.**"

PILLAR
04

Discovery and Development of New, Effective Treatments

Leveraging the unique strengths and expertise of different constituencies accelerates research and drives innovation. Our Innovation Ecosystem unites academia's deep understanding of disease biology with industry's expertise in drug development. This model has led to the initiation of seven clinical trials for ALS with unprecedented speed over the last decade, propelling research from the bench to the bedside.

One of these trials, **Denali Therapeutics' Phase 2/3 clinical trial of DNL343, launched from research funded through a Target ALS industry-led consortium** including Dr. Joseph Lewcock from Denali, Dr. Gene Yeo of UCSD, and Dr. Steve Finkbeiner of UCSF. The team studied the formation of stress granules (bundles of dysfunctional RNA and protein in a cell) that occurs when a cell is under stress, like neurons are in ALS. The researchers hypothesized that targeting factors that contribute to stress granule formation could improve neuron health. Through this research, E1F2B (a protein critical to cellular health) was identified as a potential drug target. In ALS, E1F2B activity is suppressed, leading to the formation of stress granules, which may be precursors to TDP-43 aggregation, a hallmark pathology in ALS. Denali's investigational drug, DNL343, aims to activate E1F2B, reducing the formation of stress granules to avoid neuron degeneration.

This year, we funded a related industry-led collaboration with Dr. Lewcock from Denali and Dr. Gene Yeo, Dr. Eric Bennett, and Dr. John Ravits from UCSD. The team has decoded the composition of stress granules and identified specific RNA-binding proteins (proteins critical for maintaining normal gene expression and proper function of a cell) as potential drug targets. Using Denali's proprietary delivery technology, the researchers aim to deliver molecules to stop the formation of the targeted proteins, thus reducing formation of stress granules and neuron degeneration.

This project is just one example of the many powerful new therapeutics that could emerge from our funded portfolio. **From our industry-led consortia alone, six treatments are under investigation — two of which hold promise for C9orf72 ALS (the most common genetic cause of ALS) while the other four have the potential to treat familial and sporadic ALS patients.** In addition, our most recent funding call served to bring five early stage ALS clinicians from around the world into our Innovation Ecosystem. These talented physician scientists will research novel treatments while simultaneously caring for ALS patients in the clinic, an invaluable perspective in the quest to treat this disease.

Eric Bennett, PhD

Professor, Cell and Developmental Biology

University of California, San Diego

"I am thrilled to be part of this Target ALS-sponsored academic/industry partnership with Gene Yeo's lab and the excellent team at Denali Therapeutics which is propelling our foundational research findings into a potential therapeutic targeting a fundamental cellular property of ALS/FTD. **Interactions with the larger Target ALS research community have accelerated our understanding of mechanisms governing ALS/FTD pathology and our collaborative research team is happy to contribute our deep expertise to this endeavor."**

PILLAR
05

Discovery and Development of Toolkit of Biomarkers

Early diagnosis, disease monitoring, and treatment evaluation all depend on one critical element: biomarkers. Finding reliable and accessible biomarkers (characteristics of disease that can be accurately measured and reproduced) has been an enduring challenge for ALS researchers. Because ALS is a disease of the nervous system, the few currently available biomarkers are measured in cerebrospinal fluid (CSF), the fluid which surrounds the brain and spinal cord. CSF draws can be painful and require a laboratory, hospital, or doctor's office visit, proving burdensome to patients. Identifying biomarkers in "peripheral" biofluids that are easier to collect, like blood, or establishing **digital biomarkers that monitor speech, gait, or other characteristics that can be measured non-invasively would significantly improve quality of life** by reducing delays in diagnosis and allowing for more frequent and improved measurements of disease progression that could support better care plans. Further, biomarkers allow scientists to study the effects of potential treatments, enhancing clinical trial design and development of therapies with true impact.

To explore digital biomarkers for ALS, we are funding a collaboration between Dr. Johannes Tröger of ki:elements, Dr. Jessica Robin of Winterlight Labs, and Drs. Anja Schneider and Andreas Hermann of the German Center for Neurodegenerative Diseases (DZNE). **The consortium is developing and validating a speech biomarker for ALS and FTD.** Speech changes are well documented in these patient groups and can indicate alterations in motor, cognitive, and respiratory functions. In the study, patients participate in short 10-minute speech assessments, collected remotely by telephone – a low-burden process that allows for increased participation from patients unable to travel to clinics or those who cannot manage more invasive monitoring techniques like CSF draws. Advances in Artificial Intelligence (AI) technologies including machine learning and natural language processing enable the researchers to identify indicators of disease and its progression. Establishing speech biomarkers for ALS will both improve our ability to track the disease and the overall patient experience.

Johannes Tröger, D.Sc.

Chief Scientific Officer

ki:elements

"The goal of our Target ALS funded project is to follow core cardinal ALS symptoms and their progression through speech. We will measure motor problems, cognitive problems, and respiratory problems all while patients don't have to travel long distances to clinical sites. **By simply picking up a telephone, patients allow us to collect symptom information and follow their clinical trajectory."**

PILLAR
06

Treatment of Ultra-Rare Forms of ALS

ALS exists in numerous forms, so a single approach is unlikely to provide effective solutions for every person living with the disease. Approximately 10% of ALS cases are familial, which means there is a family history of disease and suggests inheritance of a harmful gene mutation (a change in the DNA code that causes disease). Mutations in more than 30 ALS related genes have been identified. **While some mutations are more commonly associated, others are considered ultra-rare and are only found in a handful of patients.** Because of their rarity, these mutations aren't usually priorities in traditional drug development programs. However, they offer specific disease-causing targets primed to test technologies that could have broader applications in ALS and beyond.

We funded Silence ALS, a collaboration between n-Lorem Foundation and Columbia University to test antisense oligonucleotide (ASO) technology in ultra-rare forms of ALS. ASOs are small pieces of RNA or DNA that can bind to the messenger RNA created from a mutated gene, blocking its ability to make dysfunctional protein. n-Lorem is using ASO technology developed by Ionis, the leader in gene therapy for neurodegenerative disease. By focusing on ultra-rare forms, Silence ALS is able to perform N-of-1 studies, where a single patient is treated with an ASO tailored specifically for their mutation, fast-tracking the treatment to in-person testing. In 2022, a first patient was treated with an ASO from this collaboration, and we have committed to funding production of a second ASO for a second patient with an ultra-rare case of ALS. While these N-of-1 trials treat only one patient at a time, they act as proof-of-concept case studies that can validate the approach as a whole, leading to expansion of ASO technology across other forms of ALS.

Looking further, Target ALS anticipates the emergence of additional technology platforms which could have applications in treatment for ultra-rare and familial forms of ALS. Adeno-Associated Virus (AAV) and CRISPR technologies, for example, may have potential advantages over ASOs, including higher efficacy, improved safety, or longer-lasting benefit after a single, one-time injection. Our focus on ultra-rare forms of ALS both supports development of cutting-edge technologies and demonstrates our commitment to our vision of a world where Everyone Lives.

Sarah Glass, PhD

Chief Operating Officer

n-Lorem Foundation

"We focus on leveraging our knowledge of ASOs to discover, develop, and provide individualized treatments for patients with nano-rare mutations. **Through our partnership with Target ALS, we've had the opportunity to advance one of our drug discovery and development programs toward the clinic for a single nano-rare ALS patient.** For n-Lorem Foundation, our focus is to help the patients that we can help today and learn from that individual patient to bolster our knowledge of the disease with the goal to apply this knowledge and experience to help others. Our core values are in alignment with Target ALS and we're excited to continue the partnership."

PILLAR
07

Prudent Growth of Target ALS Capacity

Target ALS is not simply supporting ALS research; we are driving it. Such a bold venture requires a relentless, committed team with a sense of urgency on behalf of all people living with ALS and their families. To scale up our efforts and successfully implement our Seven-Pillar Strategy, we have welcomed new members to our staff and leadership that share our ideals and passion for our mission. **Our team has expanded its breadth of expertise across basic sciences, drug discovery, clinical trials, communications, grants operations and development. We are building the team that will defeat ALS.**

To guide our Executive Leadership as we expand the breadth and depth of our Innovation Ecosystem, we have recruited a distinguished group of pioneering academic leaders and pharmaceutical and biotech executives to serve as our Board of Directors.

Renowned physician-scientist, drug developer, and Head of Global Development and Neuroscience at Takeda, **Sarah Isabel Sheikh, BM BCh, MSc, MRCP, brings instrumental expertise in collaborative models to advance research and vast knowledge of clinical neuroscience, drug discovery, and clinical trials** as a member of the Target ALS Board of Directors. She has previously held positions at Biogen and Celgene in roles spanning neuroscience clinical development, strategy, and program leadership where she made important contributions to the development and approval of several medicines now used by patients across the world. A recent accomplishment, Sarah spearheaded the partnership between Takeda and AcuraStem, a biotechnology company focused on neurodegenerative diseases, to develop and commercialize AcuraStem's therapeutics targeting PIKFYVE, a novel target for ALS and related diseases.

Recognizing both the importance of drug development in areas of great unmet need, like devastating neurological diseases, and the importance of collaboration between academia and industry to tackle these challenges, Sarah founded and directed industry-academic fellowship programs that provided fellows with 'hands-on' drug development experience and mentorship from senior leaders. Sarah's commitment and experience driving collaboration seamlessly aligns with our Target ALS mission foundations. As a member of the Board, she ensures collaboration remains paramount as we continue to build momentum.

Sarah received her MSc in Cell Physiology and medical degree (BM BCh) from the University of Oxford (Corpus Christi College). She completed her clinical training in internal medicine at Oxford, where she was also a clinical tutor, and completed a neurology residency and fellowship in neuromuscular neurology at the Massachusetts General Hospital and Brigham and Women's Hospital, Harvard Medical School, Boston. She is a member of the Royal College of Physicians, London.

Sarah Sheikh, BM BCh, MSc, MRCP	Head, Neuroscience Therapeutic Area and Head, Global Development Takeda
	"Target ALS is the preeminent organization in ALS research that successfully connects academia, industry, and government. **The scientific depth, energy, ability to bring people together around a common cause and business acumen, is unparalleled.** I am honored to be able to contribute to Target ALS and the effort to bring transformative ALS medicines to patients faster."

CHAMPIONING COLLABORATION ACROSS CONSTITUENCIES

TARGET ALS

BY
Dan Doctoroff

CHAMPIONING COLLABORATION

ACROSS CONSTITUENCIES

Collaboration has always been the cornerstone of Target ALS. For the past ten years, our successful model has increasingly been built on academics working together with industry players, other ALS nonprofits, and other neurodegenerative disease organizations to rapidly advance breakthroughs so that eventually Everyone Lives with ALS.

The one key constituent that we haven't worked closely with is the federal government. That has changed radically over the last two years. Target ALS lobbied Congress and has strategically partnered with government agencies to both access significant funding sources for ALS research and engineer the vision on how these funds are used to accelerate progress in ALS research.

In December 2021, the Accelerating Access to Critical Therapies for ALS Act (ACT for ALS) was passed, allocating up to $100 million for ALS research. While Target ALS was not involved in the passage, we jumped in to establish a proper balance between funding for expanded access to

Dan Doctoroff
Founder, Target ALS

Our work has proven that progress can be expedited when we work together, inspiring hope that we will realize a world where Everyone Lives with ALS.

clinical trials (which was heavily supported by patient advocacy groups) and funding for the formation of public-private partnerships (PPPs) to improve our understanding of ALS and develop potential treatments. Ultimately, we reached a compromise, with the help of lobbying firms BGR and Cornerstone Group, to ensure that the National Institute of Neurological Disorders and Stroke (NINDS), the neurological division of the National Institutes of Health, would have greater discretion in funding decisions.

With our help, in Fiscal Year 2024, Congress appropriated $75 million under the ACT for ALS. That gave NINDS and the Foundation for the National Institutes of Health (FNIH) the confidence to develop a new program called Accelerating Medicines Partnership for ALS (AMP ALS). Modeled on similar programs for other diseases, AMP ALS unites resources from NINDS and private and nonprofit partners to drive progress in ALS research. This year, the NIH will allocate $43.2 million out of the $75 million that Congress appropriated for AMP ALS. Along with Stephanie Fradette of Biogen and Amelie Gubitz of NINDS, I am the co-chair of AMP ALS.

AMP ALS will be a knowledge portal, integrating existing datasets and establishing a first-of-its-kind comprehensive database of clinical, genomic, and other -omics data, as well as biofluid and post-mortem tissue samples. Scientists can mine and analyze this robust data and sample collection to identify and develop biomarkers for ALS diagnosis and progression and enhance clinical measures to improve trial design. Such a comprehensive database is also critical to leverage artificial intelligence and machine learning to discover drug targets and decode the causes of sporadic ALS, which accounts for the 90% of ALS cases where family history is not a factor. Target ALS has emerged as a major driver of the program with guidance from Amy Easton, PhD, our Senior Director of Scientific Programs.

As part of AMP ALS, NINDS recently announced the Access for All in ALS (ALL ALS) Consortium; Thirty-four clinical sites across the USA will participate in a comprehensive Natural History Study of ALS, modeled after our ongoing longitudinal biofluids study. The clinical center coordinating the international Target ALS Natural History Study, Barrow Neurological Institute led by Dr. Robert Bowser, PhD, was granted $16.7 million in support to also act as the coordinating site for the NIH study in the West. All 10 US-based Target ALS sites are involved in the consortium. This initiative will amplify our efforts, vastly increasing biofluid and data collection that will lead to groundbreaking discoveries in our search for effective ALS treatments.

Over the last decade, our collaborative Innovation Ecosystem has dramatically transformed the ALS research landscape. Sixty percent of Target ALS-funded consortia have generated drug discovery programs led by industry partners. Seven clinical trials have been launched from our portfolio of projects, which range from collaborative consortia to individual grants for next generation scientists as well as core facilities that continue to accelerate research worldwide with a sense of urgency and purpose. We continue to work across constituencies, seeking out strategic partnerships like AMP ALS that reinforce and advance our efforts. Our work has proven that progress can be expedited when we work together, inspiring hope that we will realize a world where Everyone Lives with ALS.

Wishing you a Happy New Year,

Dan Doctoroff
Founder, Target ALS


TARGET
ALS