The Work Continues...

Target ALS Annual Report 2020

Founder’s Letter

Dear friends and colleagues,

2020 was an extraordinary year. Faced with a global pandemic, individuals and organizations encountered remarkable challenges. I am deeply encouraged by the creativity and resilience shown by the Target ALS community, driven by a belief that the work continues — even, and perhaps especially — in such an unprecedented year.

We are excited to share this report featuring news about our progress and incredible partnerships, which help ensure we can advance our mission every day. Thank you for supporting us and our vision to realize a world in which no one dies from ALS.

Daniel L. Doctoroff
Founder

Team

Our expansive, multi-dimensional team represents the values, commitments, and vision of Target ALS. Together, we drive measurable scientific progress on behalf of ALS patients, caregivers, and their families.

Board Members

Daniel L. Doctoroff
Founder & Board Chair, Target ALS
Founder & CEO, Sidewalk Labs

John Dunlop
CSO, biotechnology company
(in stealth mode)

Zach Hall, Ph.D.
Chair, Target ALS Conflict of Interest Committee
Former Director, NIH NINDS

Richard Hargreaves, Ph.D.
Corporate Vice President, Bristol-Myers Squibb

Carole Ho, M.D.
Chief Medical Officer & Head of Development,
Denali Therapeutics

Story Landis, Ph.D.
Former Director, NIH NINDS
Board Liaison, Independent Review Committee

Mike Poole, M.D.
Senior Advisor & Head, COVID-19 Therapeutics Accelerator,
Bill and Melinda Gates Foundation

Stacie Weninger, Ph.D.
President, F-Prime Biomedical Research Initiative

Chief Advisors

Chris Henderson, Ph.D.
SVP, Head of Research, Biogen

Leonard Petrucelli, Ph.D.
Professor of Neuroscience, Mayo Clinic

Independent Review Committee

Robert Miller, Ph.D.
Chair, Target ALS IRC
Interim Vice Dean for Research & Academic Affairs, George Washington University

Michael Ahlijanian, Ph.D
Chief Scientific Officer, Pinteon Therapeutics

Manzoor Bhat, Ph.D., M.S.
Professor & Chair, Dept. of Cellular & Integrative Physiology, UT Health Science Center, San Antonio

Moses Chao, Ph.D.
Professor, Department of Neuroscience and Physiology, New York University

Biljana Djukic
Director, Head of Translational Sciences, Takeda

Lisa Ellerby
Professor, Buck Institute

Kenneth Fischbeck, M.D.
NIH Distinguished Investigator, NIH NINDS

Jonathan Glass, M.D.
Professor of Neurology & Pathology, Director of the Emory ALS Center, Emory University

Zaven Kaprielian, Ph.D.
Project Chief Scientific Officer, Dementia Discovery Fund U.S.

Jim Kupiec, M.D.
Chief Clinical Development Officer, Cassava Sciences

Lynne Maquat, Ph.D.
Director, Center for RNA Biology, University of Rochester Medical Center

Jere Meredith, Jr., Ph.D.
Senior Director Neuroscience, Arvinas

Robert Mays, Ph.D.
Vice President of Regenerative Medicine & Head of Neuroscience Programs, Athersys

Richard Olson, Ph.D.
Director, Small Molecule Drug Discovery Bristol Myers Squibb

Mahendra Rao, M.D., Ph.D.
Chief Executive Officer, panCELLa

Rajiv Ratan, M.D., Ph.D.
Executive Medical Director, Burke Neurological Institute, Cornell University

Ian Reynolds, Ph.D.
President, YaghPenn Consulting

Tracy Saxton
Managing Director, Dolby Family Ventures

Eric Schaeffer, Ph.D.
Chief Scientific Officer, Aprés Therapeutics

Holly Soares, Ph.D.
Head of Precision Medicine, VP, Pfizer

Ajay Verma, M.D., Ph.D.
Executive Vice President, Head of Research and Development, Yumanity Therapeutics

Frank Walsh, Ph.D.
Founder and Chief Executive Officer, Ossianix

Andrew Wood, Ph.D.
VP, Clinical Neuroimaging Research, CHDI

Staff

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

Kenneth Devaney
Chief Operations Officer

Steve Schonberg
Director of Communications

Burhan Siddiqui
Finance Associate

Team

By the Numbers

Driving Progress in Unprecedented Times

The COVID-19 outbreak threatened to disrupt and slow momentum of hard earned progress towards therapeutic and biomarker development. Understanding that the work must continue, Target ALS forged ahead with an ambitious scientific agenda: funding a cadre of new scientific collaborations and organizing the field's first comprehensive focus on discovery and development of reliable biomarkers. Once developed, these biomarkers will enable earlier diagnosis, as well as reliable tracking of the disease and effects of novel treatments.

Key Stats

Announced $20 million in new global research efforts

Funded 12 collaborative research consortia comprised of 20 research institutions & pharma/biotech companies and over 70 scientists around the world

Launched the world's most comprehensive ALS biomarker discovery and development effort – The Target ALS Diagnosis Initiative – with a $15 million initial commitment

60% of Target ALS-funded consortia have resulted in pharma/biotech industry-based programs and five drugs are currently in clinical trials

Bloomberg Philanthropies

Philanthropic Support Generates $37 Million, Fueling Progress Toward the First Effective Treatments and Biomarkers

In 2020, we were honored to receive a total of $37 million toward our mission, further accelerating progress toward effective treatments for ALS

The success of our fundraising efforts resulted from the broad, generous support of individual, philanthropic and corporate donors, including our founding donors Dan Doctoroff and Bloomberg Philanthropies as well as participants in our grassroots event with Spartan race -- “The Toughest Challenge for the Hardest Fight”. These contributions recognize our foundation’s successful efforts to revolutionize the battle against ALS. In just seven years, the Target ALS Innovation Ecosystem has funded and/or helped advance over 400 projects. 60% of Target ALS-funded consortia have resulted in pharma/biotech industry-based programs and five drugs are currently in clinical trials.

$25 million gift

Diagnosis Initiative

Launched an Unprecedented Research Initiative to Discover ALS Biomarkers

Target ALS launched an unprecedented effort to discover ALS biomarkers, which will help researchers diagnose the disease early, track its progression and provide reliable measures for effectiveness of new treatments. Reliable biomarkers have been critical for advancements in the fight against many diseases, but have yet to be discovered for ALS.

Named The Target ALS Diagnosis Initiative, the organization is investing an initial $15 million in collaborative grantmaking and the development of new, critically needed scientific resources. It was developed specifically to meet critical needs identified through feedback from more than 100 stakeholders across academia and the pharma/biotech industry.

The Target ALS Diagnosis Initiative will strategically focus on three key pillars:

- Competitive academic-pharma/biotech industry collaborative biomarker-focused projects
- New ALS biomarker-focused, pre-competitive collaborative projects
- An integrated Biofluid and Genomics core that will provide expedited access to human biosamples with no strings attached

Diagnosis Initiative

Initial Round of Funding for The Target ALS Diagnosis Initiative Announced

In November 2020, Target ALS announced $2.4 million funding over two years for four research consortia – bringing together a broad spectrum of expertise in neurodegenerative diseases – that will be supported through this new initiative.

AC Immune's high-sensitivity biofluid assays for detection of TDP-43 as an ALS biomarker
Ruth Luthi-Carter (AC Immune), Ghazaleh Sadri-Vakili, Clotilde Lagier-Tourenne, Steven Arnold and James Berry (Massachusetts General Hospital)

Identification of novel biomarkers for TDP-43-associated protein expression and signaling in longitudinal CSF samples
Yi Chen (Biogen), Jiou Wang and Phil Wong (Johns Hopkins University)

Neuron-derived exosomes as a biomarker platform for Amyotrophic lateral sclerosis
Erez Eitan (NeuroDex Inc.), Sabrina Paganoni (Massachusetts General Hospital), Jeff Rothstein (Johns Hopkins University)

Characterization of Neurofilament light chain isoforms and post-translational modifications in blood and CSF of ALS patients using mass spectrometry
Toby Ferguson (Biogen), Tim Miller and Cindy Ly (Washington University), Randall Bateman (Washington University)

Diagnosis Initiative

Target ALS also partnered with The ALS Association in 2020 to commit $1.4 million in pre-competitive funding for two projects targeting biomarker discovery.

Specifically, the projects are focused on the RNA-binding protein TDP-43. Presence of TDP-43 aggregates is a hallmark of the majority of ALS cases (~97%) and offers a potential to identify an impactful biomarker for the disease. These projects include the goal of a new assay to measure TDP-43 and to identify the forms/ fragments o present in CSF from ALS patients.

biomarker discovery

Target ALS/AFTD Collaboration

Target ALS and AFTD Advance a Groundbreaking Partnership, Galvanizing Scientists/Researchers From Two Related Diseases to Advance Work Toward Viable Treatments and Biomarkers

Groundbreaking Partnership

The Association for Frontotemporal Degeneration (AFTD) and Target ALS announced a groundbreaking partnership and call for proposals to identify treatments and biomarkers that would benefit both ALS and FTD. These two devastating, progressive diseases are now understood to overlap in genetic causes and biological mechanisms. By joining forces, Target ALS and AFTD are leveraging the combined expertise of scientists in two research fields, fueling collaboration in support of the most promising ideas.

Funding Awards

Funding Awards
Funding Awards

In May, Target ALS and AFTD announced $5 million in funding awards for work by six teams who represent scientists from academia and the pharma/biotech industry. The following six collaborative projects will work to discover and develop novel therapeutic strategies and/or biomarkers applicable to both ALS and FTD:

Mechanistic validation of HDAC6 inhibitors as a disease-modifying therapeutic for ALS and FTD
Janice Kranz (Eikonizo Therapeutics), Ludo Van Den Bosch (VIB-KU Leuven), Yongjie Zhang (Mayo Clinic), Koen Van Laere (UZ/KU Leuven)

Targeting G3BP1 and the stress granule response as a therapy for ALS & FTD
Dominique Cheneval (Novation Pharmaceuticals, Inc.), Christine Vande Velde and Alex Parker (Université de Montréal/CRCHUM)

Small Molecules Interacting with RNA (SMiRNA™) as a Therapeutic Strategy for C9ALS/FTD
Sai Velapaudi (Expansion Therapeutics), Matt Disney (Scripps Research Institute)

Small Molecule Screen to Identify Selective Inhibitors of Aberrant TDP-43 Biocondensates in a Disease-Relevant Model
Sophie Parmentier Batteur and Shahriar Niroomand (Merck & Co.), Jim Shorter (University of Pennsylvania), Chris Donnelley (University of Pittsburgh)

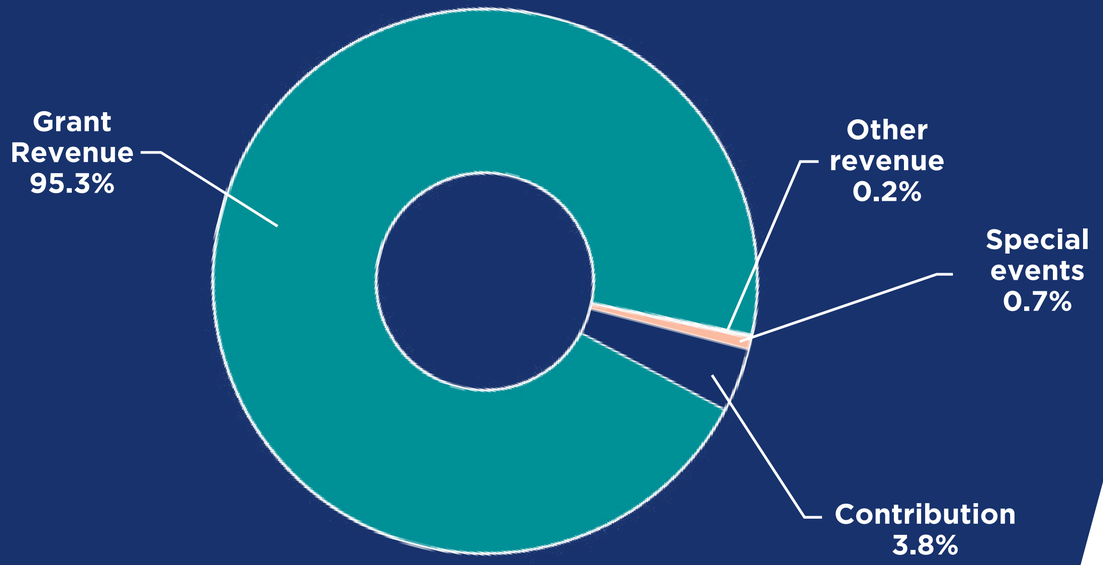
Antisense Oligonucleotides to Restore Expression of Full Length Stathmin 2 in Sporadic ALS
Daniel Elbaum (QurAlis), Kevin Eggan (Harvard University), Bob Brown and Jonathan Watts (University of Massachusetts Medical School)

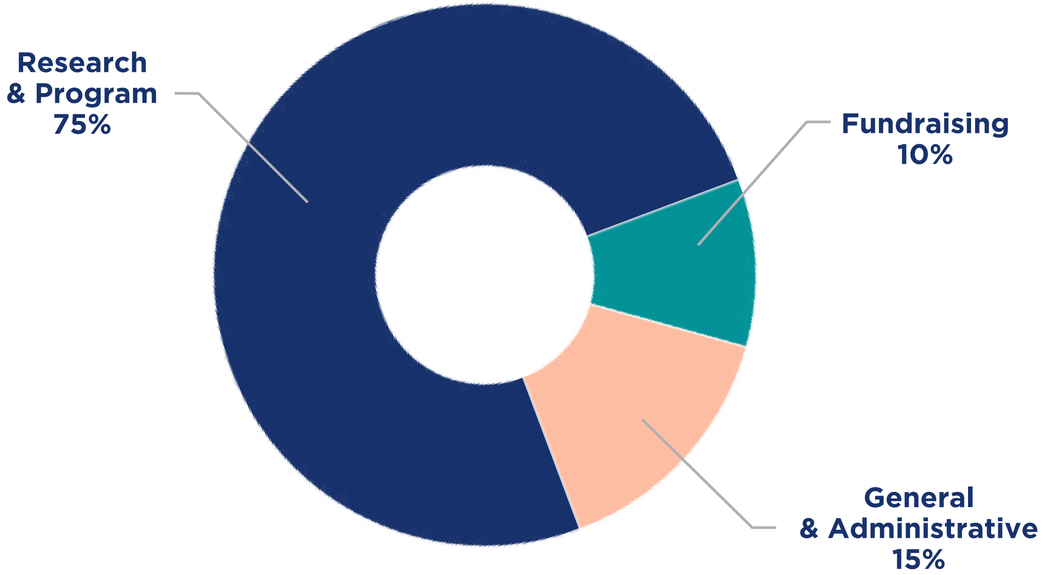
Poly(GR) and poly(GA) as Biomarkers and Therapeutic Targets in C9ORF72-ALS/FTD
Mark Kankel (Biogen), Fen-Biao Gao (University of Massachusetts Medical School), Sami Barmada (University of Michigan Medical School)

Target ALS/AFTD Collaboration

Financials

TALS





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