

ALS

THE THERAPY DEVELOPMENT
INSTITUTE

2017
Annual
Report



Dedicated to finding a cure for ALS

Our Mission

The ALS Therapy Development Institute and our scientists actively discover and develop treatments for ALS. We are the world's first and largest nonprofit biotech focused 100 percent on ALS research. Led by people with ALS and drug development experts, we understand the urgent need to slow and stop this disease.

Letter from the CEO

Board of Directors

As of November 2018

Augie Nieto, *Chairman*
Steven Perrin, *PhD., Vice Chairman*
Stan Appel, *M.D.*
Kent Bransford, *M.D.*
Alexander Cappello
Eric Craven
Jennifer Gore Dwyer
Steve Fowler, *Board Member Emeritus*
James Allen Heywood
John Heywood, *Ph.D., Sc.D.*
Spiros Jamas, *Sc.D.*
Keith P. Melanson, *Governance & Nominating Committee Chair*

Leslie Michelson
Andrew Niblock
Lynne Nieto
Theodore Reich, *Treasurer and Audit Committee Chair*
Rob Rodin, *Executive Compensation Committee Chair*
Paul A. Sallaberry
Robert Sepucha
Michael M. Smith
Julie Swan

Staff

As of November 2018

Bashar Al-Nakhala	Anna Gill	Emer Martin	Carolyn Rodgers
Kaycee Asquith	Cynthia Gill	Carlos Maya	Ricardo Sanchez
Kelly Campbell	Robert Goldstein	Jennifer McErlain	Shannon Shryne
Isarelis Carrion	Alicia Favicchio Grossi	Maeve McNally	Gretchen Simoneaux
Tanille Carter	Carol Hamilton	Andrew Moreno	Erin Stevens
Angel Chanchea-Moser	Terri Handler	Roxanne Murden	Shawn Sullivan
Taylor Charbonneau	Theo Hatzipetros, <i>Ph.D.</i>	Melissa Nollstadt	Val Tassinari
Nicole Chen	Shumika Jean-Baptiste	Erika Norabuena	Ken Thompson
Kyle Denton, <i>Ph.D.</i>	Josh Kidd	Colette O'Neill	Fernando Vieira, <i>M.D.</i>
Emily Dhondt	Haley Knight	Chelsea Owens	Ben Wakefield
Ben Engle	Allison Kosciak	Steven Perrin, <i>Ph.D.</i>	Monica Wang
Caitlin Faulds	Megan Kosciak	Alan Premasiri	Yiding Yan
Matthew Ferola	Beth Levine	Aarthi Ravi	
Alan Gill, <i>Ph.D.</i>	Matvey Lukashov, <i>Ph.D.</i>	Clare Reich	



Dear Friend,

I have been honored to be the CEO and Chief Scientific Officer at the ALS Therapy Development Institute (ALS TDI) for 10 years. Due to the continued growth in support of the research efforts at ALS TDI, we continue to expand our scientific expertise and commitment to developing effective treatments for ALS.

2017 has been an amazing year where our scientific team replicated two published studies for potential treatments, both showing therapeutic benefit to slow down disease in ALS preclinical models: gene therapy decreasing mutant SOD1 protein and CuATSM that stabilizes the mutant SOD1 dimer into a non-toxic form. ALS TDI has now demonstrated therapeutic benefit with four potential ALS treatments, making ALS TDI the organization that has tested more potential treatments in models of ALS, and validated or discovered more potential therapeutics that show significant promise in models of ALS.

ALS TDI continues to grow the Translational Research Team to expand the scope of the first of its kind Precision Medicine Program (PMP). We continued to advance the second phase of the program in an effort to enroll up to 750 participants in the PMP. In order to leverage the vast data from the PMP, we entered strategic collaborations with Google and the Broad Institute to help identify new clinical endpoints and biomarkers. These collaborations are already leading to new algorithms to stratify ALS patients into subgroups. This will enhance the efficiency of clinical trial processes and move potential treatments to people living with ALS faster than ever before.

During this last year we continued to advance our lead candidate, AT-1501, closer to clinical trial. We completed the development of the drug product manufacturing process for AT-1501, packaging AT-1501 into vials for shipping to ALS clinical sites.

We have made significant progress in the last 10 years at ALS TDI. Nothing we do is possible without the leadership of people with ALS on our board, in our fundraising, and as participants in the clinical trials and research studies we operate. It is only when researchers partner with people living with ALS that we can expect the greatest advancements towards effective treatments for ALS.

With sincere thanks for your continued support.

Steve Perrin, *Ph.D.* | President and Chief Executive Officer
ALS Therapy Development Institute

It is our mission to discover and develop effective treatments for ALS. We can only do that when people join us in our efforts to raise awareness of ALS and funds for our critical research.



This year, the [Tri-State Trek](#) was more successful than ever! The 270-mile bike ride from Boston, MA to Greenwich, CT raised over \$800,000 for ALS research at ALS TDI and included over 450 riders, crew members and volunteers. For the first time in the ride's history, people with ALS were invited to join riders for the powerful last nine miles.



Andrew Niblock, an educator at the Greenwich Country Day School (GCDS) in Greenwich, Connecticut, was diagnosed with ALS in 2016. Andrew was named to the Board of Directors of ALS TDI in 2017, making him the third person with ALS to serve on the board at this time. Andrew also rode the last nine miles of the Tri-State Trek in 2017, and was cheered across the finish line by hundreds of kids from GCDS.



Tens of thousands of [Orangetheory Fitness](#) members made donations totaling more than \$2 million via Augie's Quest in support of research at ALS TDI.



ALS TDI co-hosted a week-long series of events around the [International Symposium on ALS/MND Research](#) in Boston in December. It was the largest meeting of ALS researchers, neurologists and people living with ALS in history, with more than 1,500 people in attendance from around the world.



The Haberstroh Family started the "[ALS Pepper Challenge](#)," a viral social media campaign has ended up raising significant ALS awareness and over \$600,000 for ALS research at ALS TDI! Thousands took the challenge, including celebrities Andy Cohen, Kelly Clarkson, Garth Brooks and Trisha Yearwood, Jake Tapper and Jimmy Kimmel, to name a few!

People with ALS are at the center of all we do at ALS TDI. They participate in our research, and selflessly give their time and funds, to raise awareness of the disease and funding for our critical work.

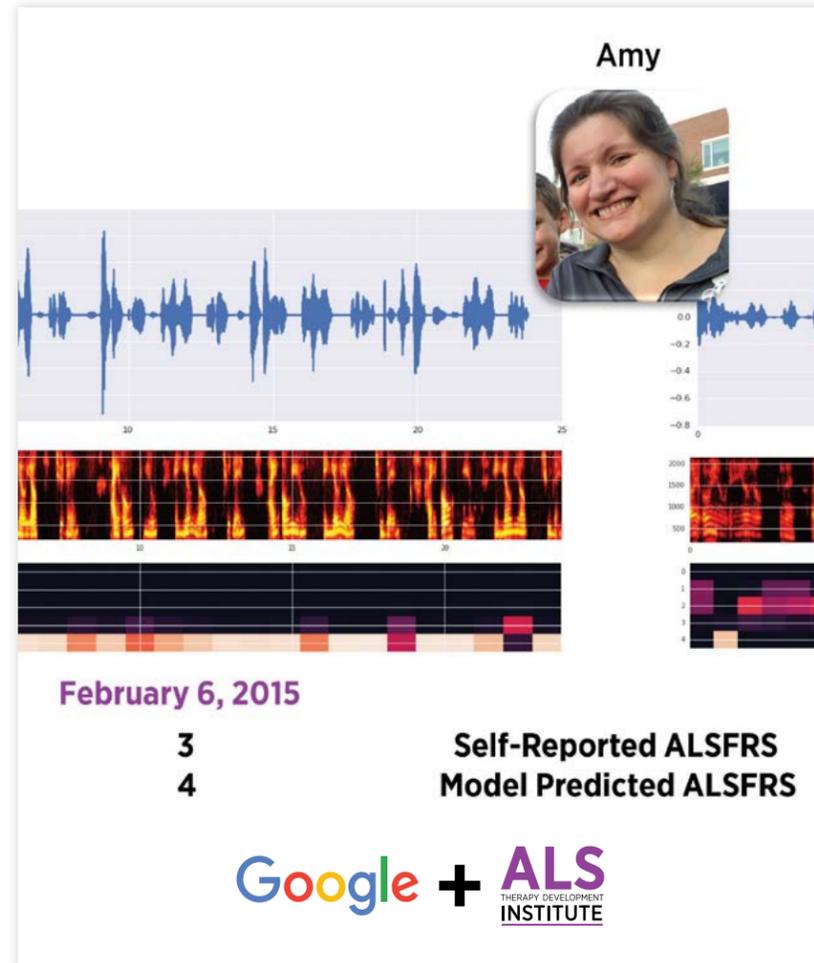
Google + ALS TDI + People with ALS = Faster Trials

When the ALS Therapy Development Institute (ALS TDI) launched its [Precision Medicine Program \(PMP\)](#) in the summer of 2014, we planned to enroll just 25 people living with ALS. In 2017, we enrolled 88 people in the program, bringing the total number of participants to 416 in over 20 countries—a significant achievement, made possible with the generous support of the ALS community.

This program has created a one-of-a-kind database integrating genetics, demographics, lifestyle, voice recordings, accelerometer, blood biomarker, and cell biology data.

In partnership with the research team at ALS TDI, in 2017 Google scientists began analyzing thousands of voice recordings of people with ALS who participate in the PMP. This work has led to the development of artificial intelligence based tools that allow for unbiased assessment of ALS bulbar symptoms.

As we continue our working partnership with PMP participants, Google scientists, and others, we will continue to utilize findings from analyses of genetics, blood based biomarkers, clinical disease progression metrics, and cell biology experiments to discover therapeutic targets and streamline clinical development processes for ALS.



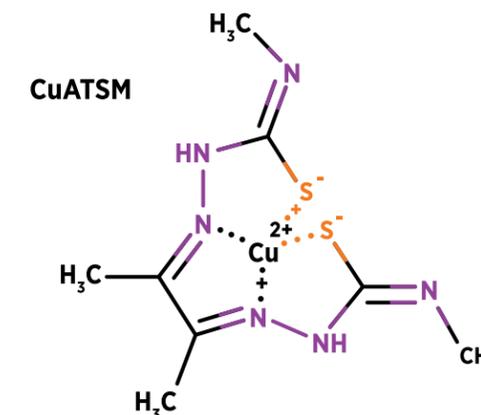
ALS TDI scientists validated the effectiveness of a gene therapy approach to treat SOD1 mediated ALS developed at the Institute of Myology in France. Based on this confirmatory study, Prize4Life awarded the French researchers a \$1 million “Treatment Prize.”



Advanced [AT-1501](#) toward clinical trial, including the creation of a Clinical Advisory Board at our majority-owned subsidiary, [Anelixis Therapeutics](#). We completed the development of the drug manufacturing process, putting a specific amount of AT-1501 into vials for shipping to ALS clinical sites. We also completed the safety testing of AT-1501 which is required for FDA filing and first in-human trial to start in 2018.



Through ALS TDI’s [Precision Medicine Program](#), scientists have made strides toward applying accelerometers for the remote tracking of ALS disease progression objectively and sensitively. This approach has potential to reduce clinical trial duration and reduce the number of participants necessary to reveal drug efficacy.



Scientists at ALS TDI published results showing that they **independently validated the efficacy of Copper ATSM (CuATSM)** at slowing ALS disease progression in the SOD1 mouse model. This is the third potential treatment tested at ALS TDI that has shown positive data in models of ALS.

“The chance to recognize leaders in the ALS community is a humbling one,” says Steve Perrin, Ph.D, CEO of ALS TDI. “This battle is a personal one, and we meet incredible people who are hit with this disease every day. The nominations we received this year were overwhelming and came from all over the country. The ALS community is full of people who

lead us, inspire us and remind us of all the reasons that we can never give up. [Rick, Collin, Emily, Michael, Osiel and Bella](#) are examples of leadership, and it is our privilege at ALS TDI to recognize them on behalf of the ALS community.”

Rick Marks



Fran Delaney Challenge & Respect Award

Rick Marks of Winchester, Massachusetts, was diagnosed with ALS in October 2016. He became an advocate for ALS research and was involved in multiple endeavors, including [hiking 600 miles of the Appalachian Trail](#) from Massachusetts to Maine in the summer of 2017. Rick’s hike raised significant awareness of ALS through media coverage by *People Magazine*, *Inside Edition* and television stations across the country. Proceeds from Rick’s hike went to ALS ONE, a partnership to advance treatments for ALS that includes the work done by ALS TDI.

Collin & Emily Hadley



Mary Lou Krauseneck Courage & Love Award

Collin Hadley was diagnosed with ALS in October 2014. He and his wife, Emily, got very involved in the ALS community; through an online blog [“Curveball Hadley,”](#) they documented their experiences participating in clinical trials, including a stem cell-based trial conducted by Brainstorm Cell Therapeutics, and the Precision Medicine Program at ALS TDI. Collin and Emily also held fundraisers in their local Fort Worth, Texas community, including a concert and barbecue event.

Michael “Boomer” Wallace



Stephen Milne Adventurous Spirit Award

Following his diagnosis with ALS in June 2016, Boomer became dedicated to the battle to end ALS. Through business connections, he began working in earnest to [create broad awareness of ALS](#). During the 2017 May ALS Awareness Month, his efforts led to major campaigns among top retailers including Ramy Brook, Lettuce Entertain You, Rue La La, Lilly Pulitzer and many others in support of ALS research.

Osiel & Bella Mendoza



Stephen Heywood Patients Today Award

Osiel was diagnosed with ALS in October 2016 at age 21. Less than a year later, he married Bella, his girlfriend since age 13. Osiel and Bella are committed to raising awareness of ALS. Together, they created the [“#YokeUp4ALS Challenge,”](#) which went viral with thousands of people accepting the Challenge and donating to ALS research. Osiel has become deeply involved in the work at ALS TDI: speaking to scientists about his battle, partnering with the development and marketing teams to share his story, and meeting with other newly diagnosed families. Osiel spoke at the 2017 White Coat Affair Gala in Boston.

For fiscal year ending December 31, 2017, ALS TDI was the grateful recipient of more than \$16 million in contributions and other income—the most raised in a single year for research at ALS TDI.

This financial information is derived from audited financial statements. Copies of

audited financial statements are available upon request. For a complete copy of our IRS Form 990 or Independent Auditors Report, please [visit our website at als.net](http://als.net) or call us at 617.441.7200.

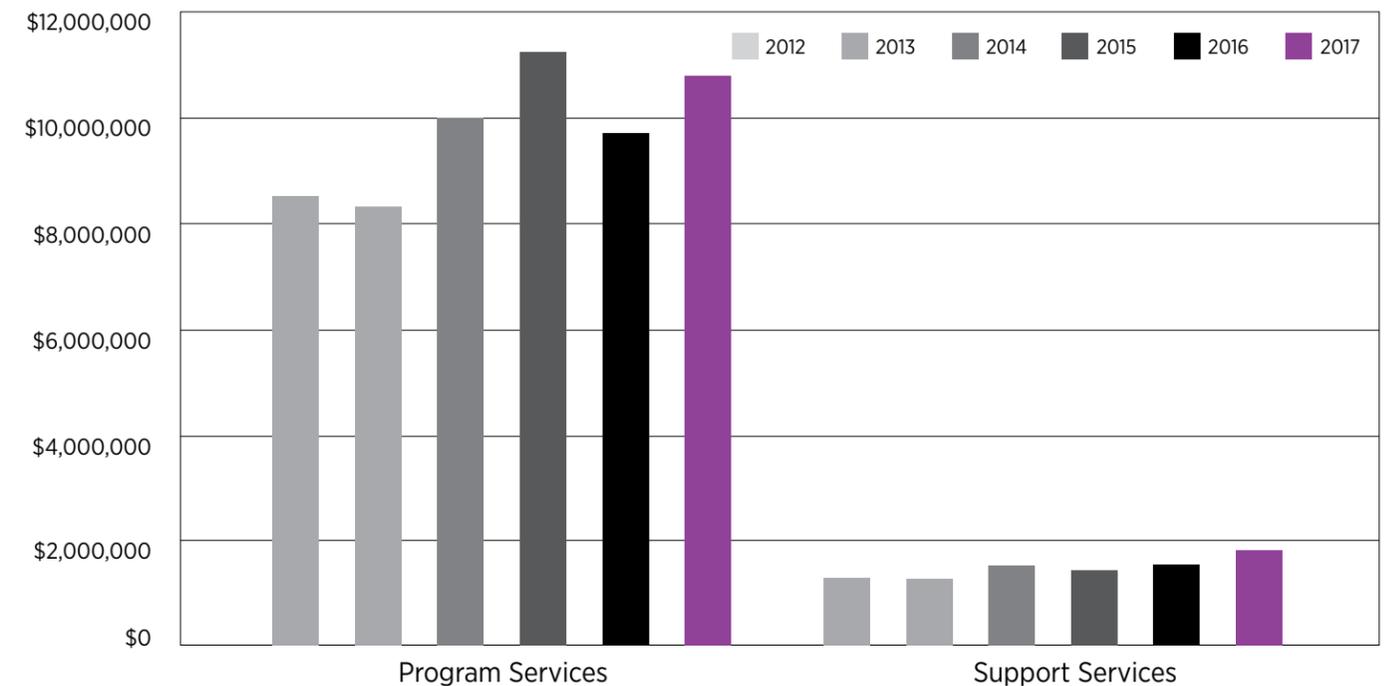
Financial Review



2012-2017: A Financial Comparison

Assets		Current Assets	Other Assets
	Cash And Cash Equivalents	\$4,187,524	Pledges Receivable \$720,971
	Pledges & Accounts Receivable	\$529,147	Property & Equipment, Net \$781,861
	Other Current Assets*	\$1,635,625	Patents \$333,320
			Investment in Subsidiary \$4,839,396
			Other Long Term Assets \$567,739
Net Assets			\$13,595,583
Liabilities & Net Assets			
	Current Liabilities		\$2,183,423
	Long Term Liabilities		\$701,913
	Net Assets		\$10,710,247
Total Liabilities & Net Assets			\$13,595,583
Operating Income			
	Grants		\$1,931,946
	Special Events, Net		\$10,932,408
	Contributions		\$1,457,011
	Released From Restrictions		\$25,000
	Fee For Service		\$1,374,412
	Donated Goods & Services		\$4,390
	Interest & Other		\$302,743
Net Public Support			\$16,027,910
Operating Expenses		Program	Support
	R&D		General \$731,493
	Science	\$7,959,013	Fundraising \$1,034,535
	Informatics	\$385,959	Total Support \$1,766,028
	Patient Services	\$157,331	
	Communications	\$2,036,328	
	Business Development	\$232,933	
Total Program		\$10,771,564	
Total Expenses			\$12,537,592

	2012	2013	2014	2015	2016	2017	Total Spent
Program Services	\$8,454,877	\$8,301,674	\$9,977,506	\$11,257,021	\$9,705,301	\$10,771,564	\$58,467,943
Support Services	\$1,276,210	\$1,262,610	\$1,524,655	\$1,425,127	\$1,569,649	\$1,766,028	\$8,824,279



Anelixis
THERAPEUTICS

In 2015, the ALS Therapy Development Institute established [Anelixis Therapeutics](http://AnelixisTherapeutics.com), as a majority-owned subsidiary to help it advance potential treatments for ALS. ALS TDI aims to partner with Anelixis in the future to attract new and different types of funding and revenue to advance potential treatments, such as AT-1501.



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