

ALS

THERAPY DEVELOPMENT
INSTITUTE

2018 Annual Report



DEDICATED TO CURING ALS

OUR MISSION

The [ALS Therapy Development Institute \(ALS TDI\)](#) and its researchers discover and develop potential treatments for ALS. It is the world's first and largest nonprofit biotech focused 100 percent on ALS research. Led by drug development experts and people with ALS, ALS TDI understands the urgent need to slow and stop this disease.

LETTER FROM THE CEO



Dear Friend,

When the Heywood family first founded the ALS Therapy Development Institute (ALS TDI) in an effort to discover an effective treatment for their brother, Stephen, they imagined an organization that combines the heart of a nonprofit with the spirit, drive, and innovation of a biotech company. I am proud to say that, throughout 2018, our team and our community demonstrated that this vision still rings true today.

2018 was an exceptional year. After 10 years of development, AT-1501, a treatment discovered right here at our lab, began a Phase 1 clinical trial—an incredible milestone for ALS TDI as well as for our donors, our partners, and the ALS research community worldwide. While we are proud of our accomplishments, we know we can't slow down now.

To this end, in 2018 we continued to rigorously test more potential treatments for ALS than all other ALS research labs combined, bringing the total number of drugs tested at our lab to over 400. In addition, over 550 people with ALS are enrolled as research partners in our Precision Medicine Program (PMP), helping us move closer to our goals of discovering new treatments for ALS and making clinical trials faster and more efficient.

Despite all the progress made this year, we know that 2018 also brought incredible loss to families and people battling this disease. Loss of independence, loss

of abilities, and the devastating loss of loved ones. Not a day goes by at ALS TDI that we don't mourn someone who has passed, and talk to others who are currently fighting. These conversations motivate us to keep up the momentum of 2018 and do even more in 2019.

We know that ALS is extremely complex and treating it will not be a one-drug-fits-all solution. Our strategy seeks to find many effective treatments, as quickly as possible, until the needs of every person with ALS are met. As we move forward, we remain laser focused on our mission to discover and develop effective treatments for ALS—screening more potential treatments for ALS, enrolling more people in our PMP, and building upon strong collaborations with our research partners. We truly can't do any of this without your help.

With sincere thanks for your continued support,

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

Board of Directors <i>As of June 2019</i>	Augie Nieto, <i>Chairman</i>	Leslie Michelson
	Steven Perrin, Ph.D., <i>Vice Chairman</i>	Andrew Niblock
	Stanley Appel, M.D.	Lynne Nieto
	Kent Bransford, M.D.	Theodore Reich, <i>Treasurer and Audit Committee Chair</i>
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	James Allen Heywood	Julie Swan
	John Heywood, Ph.D., Sc.D.	
	Spiros Jamas, Sc.D.	
	Keith P. Melanson, <i>Governance & Nominating Committee Chair</i>	

Staff <i>As of June 2019</i>	Bashar Al-Nakhala	Beth Levine
	Kaycee Asquith	Matvey Lukashev, Ph.D.
	Gabrielle Bellitti, M.S.	Emer Martin
	Alessandro Calo	Carlos Maya
	Isarelis Carrion	Eliza McDonough
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	Nicole Chen	Maeve McNally
	Dileep Dadlani	Andy Moreno
	Kyle Denton, Ph.D.	Kaly Mueller
	Emily Dhondt	Roxanne Murden
	Ben Engle	Melissa Nollstadt
	Caitlin Faulds	Chelsea Owens
	Matthew Ferola	Steven Perrin, Ph.D.
	Alan Gill, Ph.D.	Alan Premasiri
	Anna Gill	Clare Reich
	Cynthia Gill	Carolyn Rodgers
	Alicia Favicchio Grossi	Ricardo Sanchez
	Carol Hamilton	Erin Stevens
	Terri Handler	Landan Stevens
	Theo Hatzipetros, Ph.D.	Shawn Sullivan
	Nicholas Hellmann	Val Tassinari
	Josh Kidd	Ken Thompson
	Haley Knight	Fernando Vieira, M.D.
	Allison Kosciak	Ben Wakefield
	Megan Kosciak	Monica Wang
	Meghan Lawlor	Yiding Yan
	David LeBlanc	



In 2018, ALS TDI launched its Marathon program, receiving one bib for the Boston Marathon and five bibs for the [TCS New York City Marathon](#), as a charity partner with the New York Road Runners. Sean Beaudry, Meg Gardiner, Hindy Ginsberg (pictured here), David McNeice, and Ellen and Doug Reich were selected and each ran and raised funds in honor of a loved one with ALS. Together they raised over \$120,000 to help #EndALS.

THE YEAR IN MILESTONES

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 bring awareness to ALS and the need to fund our critical research.



In March, Augie's Quest and Orangetheory Fitness joined forces for the [#IBurnForALS campaign](#). Members at Orangetheory Fitness studios ran, rowed, and lifted, earning “splat points”, which they then turned into donations totaling \$4,010,818 for ALS research. This is the second year that Orangetheory Fitness has led this campaign, bringing the grand total raised by its members to more than \$6 million.



The ALS Pepper Challenge, started by the Haberstroh family in honor of their mom, Patty, swept the globe in 2018 raising over \$600,000. Patty, who was diagnosed with ALS at the end of 2017, has been a social worker for over a decade, and is a mother of four and grandmother of six. She has dedicated her life to helping others and now is calling on us to help her #EndALS.



In June, we celebrated the 16th anniversary of the [Tri-State Trek](#). The 270-mile bike ride from Boston, MA to Greenwich, CT raised over \$700,000 for ALS research at ALS TDI and included over 450 riders, crew members, and volunteers. Four people with ALS, Andrea Peet, Lisa Chernob, Bobby Forster, and Andrew Niblock, joined the riders for the powerful final nine miles into Roger Sherman Baldwin Park.



The [Leadership Summit](#) made a return in November 2018. The full-day conference featured talks from experts including ALS TDI's CEO and CSO and a thought-provoking presentation from Michael Brenner Ph.D. (pictured left), a Research Scientist at Google, about the use of artificial intelligence for symptom assessment in ALS.



RESEARCH ACCOMPLISHMENTS

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.

We will not stop. To date, over 550 participants have enrolled in the ALS Therapy Development Institute's [Precision Medicine Program \(PMP\)](#)—the most comprehensive and longest running translational research study in ALS.

In recent years, we've learned a lot about ALS, but there is still so much that we don't know. We do know that ALS is a complex disease and will not be a one-treatment-fits-all solution. Only by listening to people with ALS, collecting unbiased data about their past and progression, can we better understand the processes that drive and influence ALS and screen and target treatments more precisely.

That's why our researchers partner with people with ALS around the world to share and gather data on medical histories, family histories, genetics, biomarkers, and patient cell biology to better understand the disease. In return for providing invaluable data, participants receive access to helpful tools to help them make better decisions on their treatment and care.

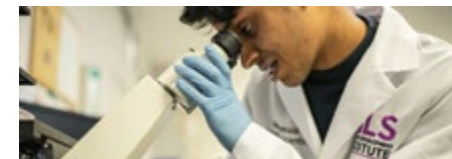
Our ultimate goals are to discover new treatments for ALS and make clinical trials faster and more efficient. We won't stop until the needs of every person with ALS are met completely.



We have one mission: [End ALS.](#) We continued to operate one of the largest preclinical pharmacology programs in all of neurodegeneration. We rigorously screened 20 potential treatments, bringing the total number of drugs tested at our lab to over 400—more than any other ALS research lab in the world.



We collaborate. [We partnered with Google](#) to leverage their expertise in artificial intelligence to analyze the “big data” collected from our PMP, making significant strides towards the development of unbiased, quantitative and sensitive measures of ALS disease progression.



We make history. We became the first nonprofit biotech in any disease to [discover and develop a potential treatment](#), AT-1501, and bring it from our own lab, through FDA review and into clinical trial.



We learn from you. With the help of uBiome, we initiated an ALS-specific research study in which we collect five different sample types from participants to assess the possible role of the microbiome in ALS disease progression.



CORY BURELL

Stephen Heywood Patients Today Award

Cory Burell was diagnosed with ALS in February 2017 when he was just 33 years old. As a husband, and father of two, Cory knew he was going to fight. Cory spent the next two years advocating for those with ALS through his posts on social media and [ALS forums](#), and by helping collate feedback for the FDA guidance document on clinical trial design in ALS. He was a partner in ALS TDI's research through its [Precision Medicine Program](#) as well as hosting fundraising events and campaigns, like a Skeeball Tournament. Cory passed away in March 2019.

LEADERS IN THE ALS COMMUNITY

"The battle against ALS takes a lot of leadership and support," says Steve Perrin, Ph.D., CEO of ALS TDI. "[This year's awardees](#) included two individuals, Chris and Jeff, from organizations that provide tremendous on the ground support to people with ALS and their families. It also included two incredible people with ALS, Cory and Patty, who have become very well-known in the ALS community. It was our privilege to recognize these individuals at this year's Leadership Summit in Boston."

PATTY HABERSTROH



Stephen Milne Adventurous Spirit Award

Patty Haberstroh lives in Westport, CT and was diagnosed with ALS in October 2017. She and her family started the "[ALS Pepper Challenge](#)," a viral social media campaign that raised awareness and over \$600,000 for ALS research at ALS TDI. People all over the world took the challenge, including celebrities like Andy Cohen, Kelly Clarkson, and Jimmy Kimmel, to name a few. Patty is a mother of four and grandmother of six.

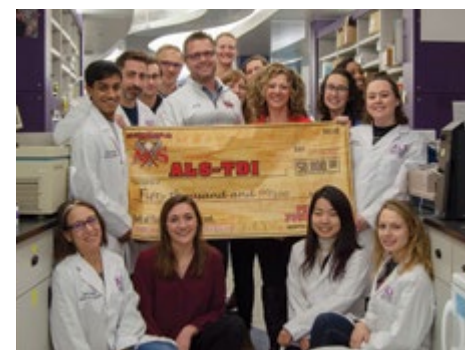
CHRIS CURTIN



Mary Lou Krauseneck Courage & Love Award

Larchmont, NY resident Chris Curtin began working with people with ALS in 2004 when he was a caregiver to a dear friend, Claire Collier, for five years. He was a founding board member of the [MAC Angels Foundation](#), and served as Board President until he became Services Director in 2014. Chris is a regular participant in ALS TDI events like the Tri-State Trek, riding 270 miles and raising funds for ALS research.

JEFF SWICK & PHAALS FOUNDATION



Fran Delaney Challenge & Respect Award

Jeff Swick of Fort Jennings, OH created the [Playing Hardball Against ALS \(PHAALS\) Foundation](#) in 2012 to help people with ALS and their families. The Foundation has raised over \$500,000 to provide support to over 100 ALS families. Jeff and PHAALS have committed to advancing ALS research at ALS TDI through their HOF Research Grant.

FINANCIAL REVIEW

For fiscal year ending December 31, 2018, ALS TDI was the grateful recipient of more than \$15.5 million in contributions and other income.

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 or call us at 617.441.7200.

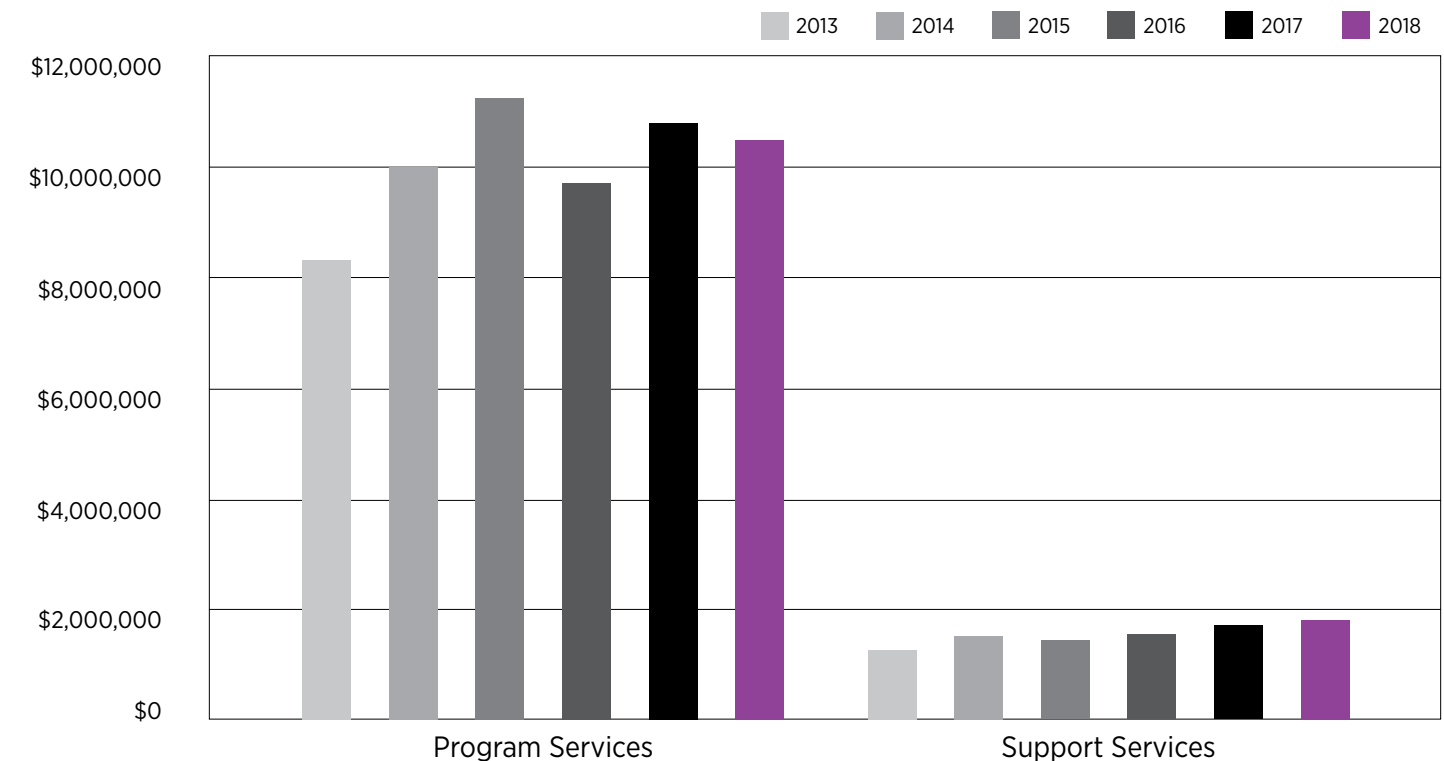
Assets		Current Assets	Other Assets
		Cash And Cash Equivalents \$4,317,383	Pledges Receivable \$485,390
		Pledges & Accounts Receivable \$706,799	Property & Equipment, Net \$569,386
		Other Current Assets* \$1,009,808	Patents \$431,891
			Investment in Subsidiary \$7,263,532
			Other Long Term Assets \$868,071
Total Assets		\$15,652,260	
Liabilities & Net Assets		Current Liabilities \$1,679,181	
		Long Term Liabilities \$553,795	
		Net Assets \$13,419,284	
Total Liabilities & Net Assets		\$15,652,260	
Operating Income		Grants \$521,544	
		Special Events, Net \$11,835,436	
		Contributions \$1,023,879	
		Released From Restrictions \$500,000	
		Fee For Service \$1,175,932	
		Donated Goods & Services \$73,289	
		Interest & Other \$481,805	
Total Public Support		\$15,611,885	
Operating Expenses		Program	Support
		R&D	General Fund \$731,267
		Science \$7,198,048	Fundraising \$1,084,521
		Informatics \$499,656	Total Support \$1,815,788
		Patient Services \$216,776	
		Communications \$2,365,946	
		Business Development \$306,634	
Total Program		\$10,587,060	
Total Expenses		\$12,402,848	

2013-2018 A FINANCIAL COMPARISON

As a nonprofit, we rely on donations to accelerate our research. 87 cents of every dollar goes directly to finding treatments and cures at ALS TDI, compared to the average 75 cents** at other research labs, and we have been [rated a four-star nonprofit by Charity Navigator](#) for five years in a row.



	2013	2014	2015	2016	2017	2018	Total Spent
Program Services	\$8,301,674	\$9,977,506	\$11,257,021	\$9,705,301	\$10,771,564	\$10,587,060	\$60,600,126
Support Services	\$1,262,610	\$1,524,655	\$1,425,127	\$1,569,649	\$1,766,028	\$1,815,788	\$9,363,857





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