ALS
THERAPY DEVELOPMENT
INSTITUTE

2019 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,

It's hard to believe that it has been twenty years since the ALS Therapy Development (ALS TDI) was first founded. It's truly incredible to see all that we've been able to accomplish, and how much we have grown, thanks to the support of our community. Today, we are the most comprehensive research lab in the world dedicated to ALS. And while I am extremely proud of what we have accomplished, we must continue to work relentlessly until there are treatments for every single person living with ALS.

In 2019, ALS TDI continued to advance its mission to find effective treatments for ALS on multiple fronts. We instituted a first-in-kind in-home blood collection program, allowing participants in our Precision Medicine Program to easily contribute blood samples to help our search for reliable biomarkers for ALS progression. We tested 22 new potential ALS treatments in animal models, bringing the total number of drugs tested in our lab to over 400, the largest amount tested by any ALS lab.

In addition, through our continued collaboration with Google, ALS TDI scientists helped demonstrate that it is possible to train speech recognition software to better understand and interpret voices that have been affected by bulbar ALS symptoms. Our collaboration with Google grew to include the development of voice recognition tools for dysarthric people with ALS through the initiation of Project Euphonia.

Further cementing ourselves as leaders in the ALS research-space, ALS TDI had our findings published in five peer-reviewed papers, making it our most prolific year of published research. Our papers detailed findings on the following: therapeutic antibodies targeting misfolded SOD1; protein aggregates in the spinal cord that may be protective against ALS disease progression; best practices of genotyping in the leading mouse model of ALS; development of speech recognition tools for dysarthric people with ALS; and cell-based assay systems designed to study and discover therapeutics targeting C9ORF72 dipeptide repeat mediated toxicity.

As always, none of this would have been possible without the support of the community. Thank you for continuing to fundraise and donate to support our ALS research. From participating in signature programs to creating your own successful fundraising events, the support of the community allowed us to move promising research projects forward in 2019.

As we look to 2020, we are confident that, with your continued support, we will get closer to finding effective treatments and cures.

With sincere thanks for your continued support,

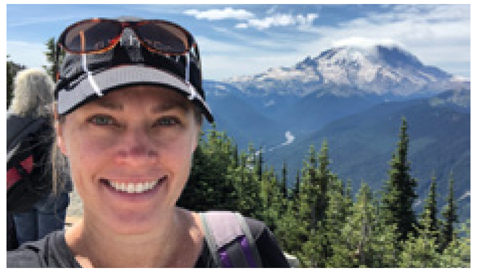
Steve Perrin, Ph.D.
President and Chief Executive Officer
ALS Therapy Development Institute

2019: THE YEAR IN MILESTONES

It is our mission to discover and develop effective treatments for ALS. As a nonprofit biotech lab, we rely on the support of our community to fund our ALS research. Each year our community hosts over 300 fundraisers around the world that raise millions of dollars to help end ALS.

The 9th annual White Coat Affair gala had its largest attendance yet. The event raised over $400,000 for ALS research at ALS TDI and included an impressive line-up of speakers. Jamie and Ben Heywood, the founders of ALS TDI, talked about the fearlessness of looking at a problem in a new way. Pat Quinn, person with ALS and co-founder of the ALS Ice Bucket Challenge, shared that legacy is about creation, not loss of life.

Ales for ALS™ has raised over $2,300,000 since 2013 for ALS research at ALS TDI. To celebrate their Ales for ALS™ brews and raise even more funds, breweries hosted summer Brewfest events. Faction Brewing hosted their 6th annual Ales for ALS™ Festival in Alameda, CA that included more than 40 local breweries. The inaugural Brewing for a Cure event in Cambridge, MA featured 25 local breweries and raised over $140,000.



Ragnar for Research fundraising events presented a new and unique way to fundraise for ALS research. ALS TDI partnered with Ragnar, the largest overnight relay series in the nation, to gather teams of runners in relay-style trail runs. Three events took place throughout 2019 in Washington, Texas, and Florida and raised $85,000 for ALS research.

The Leadership Summit was more powerful than ever, with guests like Google who showed a special preview of "The Age of A.I.," a new YouTube Originals documentary series that features ALS TDI! The full-day conference featured talks from experts like ALS TDI's CEO and CSO and Michael Brenner Ph.D., Research Scientist at Google. The event premiered a new social poster session with ALS TDI researchers.

Community events brought people together to end ALS. Created by two New England first responders with ALS, Arrest and Extinguish ALS held their first fundraising event in May and raised more than $65,000 for ALS research. Led by two brothers, David and Scott Lloyd, the Racing For ALS campaign hosted its first event at the Virginia International Raceway in June and raised more than $74,000 throughout 2019. In California, Rock, Roll, and Stroll raised more than $21,000 at their first fundraising 5K event in November.

RESEARCH ACCOMPLISHMENTS

With clinical, preclinical, and translational research we rapidly pursue for slowing, stopping, and ending ALS. In 2019, ALS TDI saw major expansion of all our research programs, making significant progress toward our mission to end ALS.

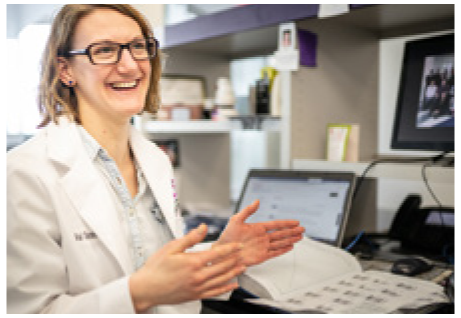
We learn from you. ALS TDI has implemented a first-in-kind “In-Home” blood sample collection program to better leverage our partnership with people with ALS. Through blood sample collection, we are able to identify biomarkers in the blood that could help inform strategies to combat ALS disease progression.

A biomarker is a kind of biological “fingerprint” – something about a living thing that can tell doctors or scientists something about that organism.

One of the most sought-after goals in ALS research is the discovery of a blood biomarker for the disease. Today, doctors and researchers must rely on subjective observations and questionnaires like the revised ALS Functional Rating Scale (ALSFRS-r) to track disease progression. Finding a blood biomarker – something in the blood that correlates to the presence and severity of ALS symptoms – could mean finally being able to put an objective measurement to these questions. It could also provide an important benchmark for drug development – possibly allowing researchers to see if the drugs they are testing in clinical trials are effective.

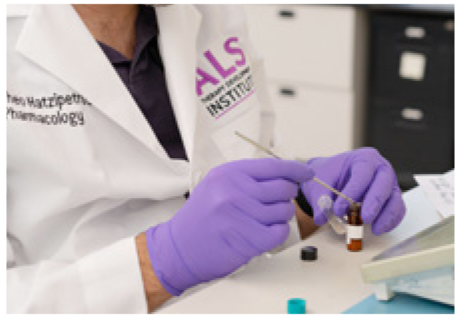
One of the best ways to find a biomarker for any progressive disease is to test blood samples from patients taken over time, or “longitudinally,” as their condition worsens.

To meet this challenge, we have established the first in-home blood collection program in ALS as part of our Precision Medicine Program (PMP). By partnering with Phlebotek, a mobile phlebotomy service, we are able to collect samples from people living with ALS who are participating in the PMP without asking them to leave their homes.



We are experts. We published papers that detailed findings regarding: therapeutic antibodies targeting SOD1, protein aggregation in the spinal cord, genotyping in the leading SOD1 mouse model, and development of speech recognition tools for dysarthric people with ALS.

We continue to grow. We acquired a compound library of 30,000 small molecules that can be screened rapidly in ALS-related cells. Screening a compound library of this size represents a sizable expansion of our scope. It has the potential to identify better quality leads, earlier on in the process; allowing us to pursue the most promising ideas, faster.



We are relentless. We continued to operate one of the largest preclinical pharmacology programs in all of neurodegeneration. In 2019, we rigorously tested 22 new potential ALS treatments, bringing the total number of drugs tested in our lab to over 370, more than any other ALS research lab in the world.

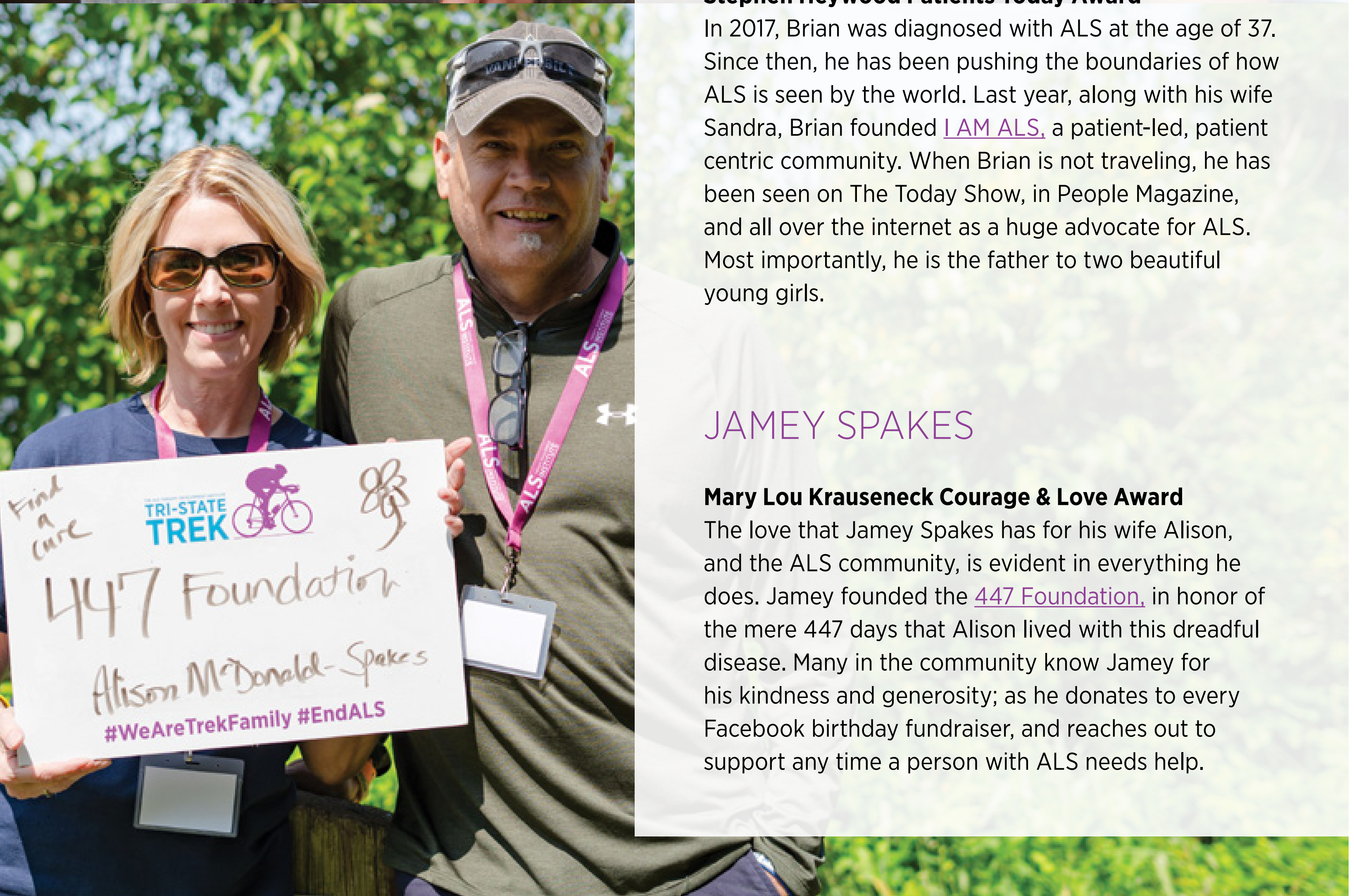
We are versatile. We studied an even more versatile and comprehensive selection of ALS models and established a new facility to house zebrafish-based models of ALS.

LEADERS IN THE ALS COMMUNITY

The ALS Therapy Development Institute (ALS TDI) Leadership Awards are presented each year to honor those who lead us in the battle against ALS. This year's recipients include Brian Wallach, Glynis Murray, Lisa Cross Bonahoom, Jamey Spakes, and Hugh Macdonnell. The recipients were chosen from nominations made by members of the ALS community.

BRIAN WALLACH

Stephen Heywood Patients Today Award
In 2017, Brian was diagnosed with ALS at the age of 37. Since then, he has been pushing the boundaries of how ALS is seen by the world. Last year, along with his wife Sandra, Brian founded I AM ALS, a patient-led, patient centric community. When Brian is not traveling, he has been seen on The Today Show, in People Magazine, and all over the internet as a huge advocate for ALS. Most importantly, he is the father to two beautiful young girls.



JAMEY SPAKES

Mary Lou Krauseneck Courage & Love Award
The love that Jamey Spakes has for his wife Alison, and the ALS community, is evident in everything he does. Jamey founded the 447 Foundation, in honor of the mere 447 days that Alison lived with this dreadful disease. Many in the community know Jamey for his kindness and generosity; as he donates to every Facebook birthday fundraiser, and reaches out to support any time a person with ALS needs help.

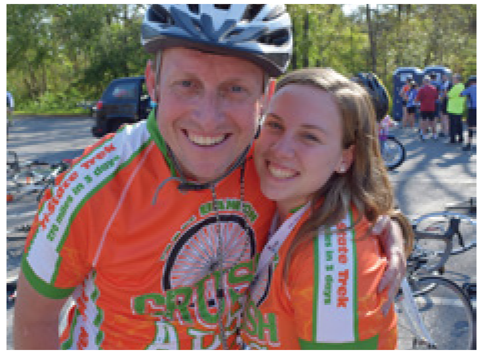
LISA CROSS BONAHOOM

Mary Lou Krauseneck Courage & Love Award
Lisa Cross Bonahoom was the first in her family to get ALS. Later, after her sister was diagnosed with ALS, they discovered they have a familial version of the disease. Lisa is actively working to help find treatments by volunteering her genes as well as her time at different fundraising events. Her courage and energy have led many friends to give time and money to the cause. He warmth and love inspires hope in others as they fight to end ALS.

GLYNIS MURRAY

Stephen Milne Adventurous Spirit Award
There are very few people who work as hard at awareness than Glynis Murray. Her husband, Vince, was diagnosed with ALS the day before their wedding and passed just two years later. Now, she flies around the country to support ALS fundraisers—dressing like a dinosaur, singing in the car, and even getting a pepper tattoo to support the #ALSPepperChallenge! She continues to work tirelessly to make sure that no one else loses the ones they love to ALS.

HUGH MACDONNELL



Fran Delaney Challenge & Respect Award
Hugh Macdonnell lost his mother in law, Betty Anne Chapman, to ALS in 2013. Not only does Hugh's entire family work to make sure that others don't suffer the loss theirs did, but he has marshalled scores of people in his community to join the fight. With Hugh's help and leadership, the Tri-State Trek's Team Crush ALS/Team Bannon has raised over $1M for ALS research at ALS TDI.

FINANCIAL REVIEW

For fiscal year ending December 31, 2019, ALS TDI was the grateful recipient of more than $17 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 call us at 617.441.7200.

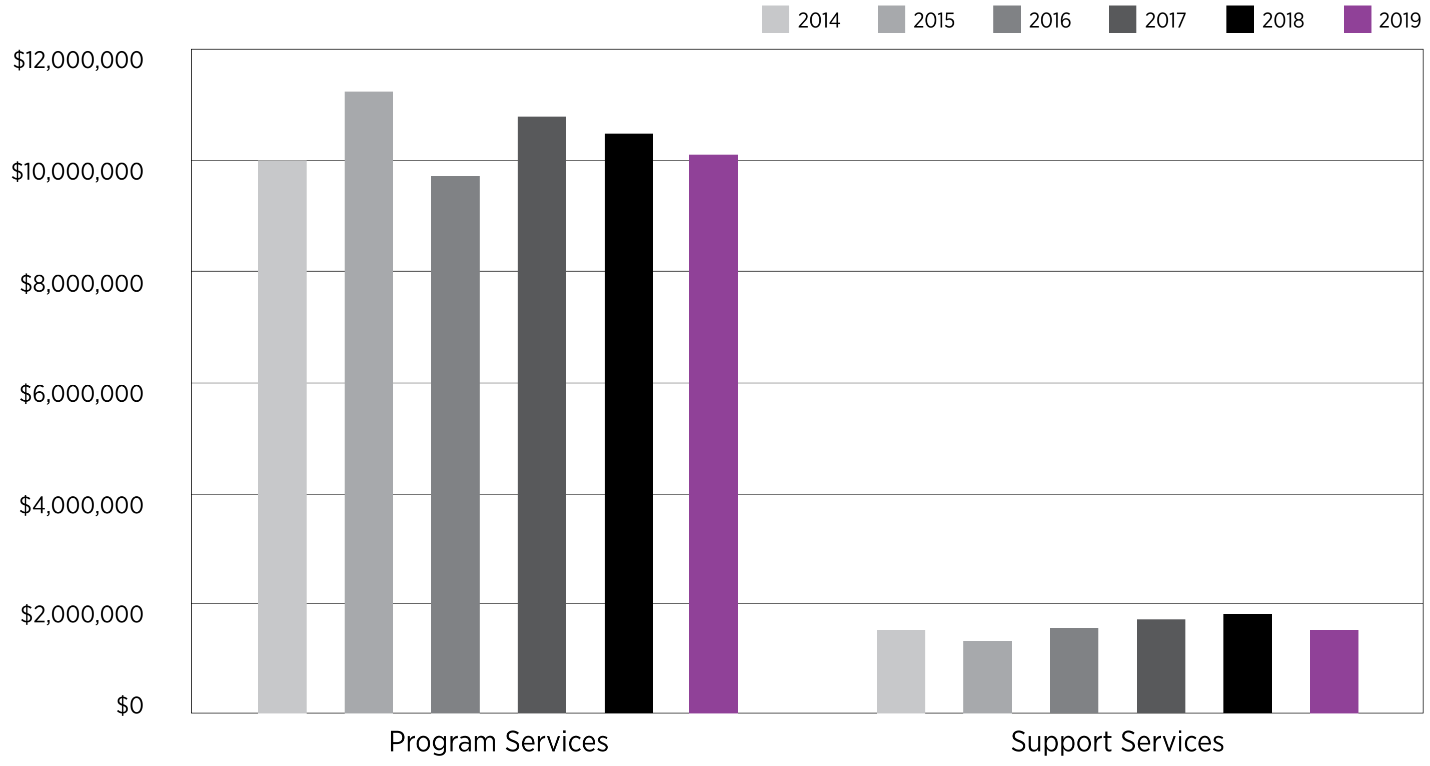
Assets	Current Assets		Other Assets	
	Cash And Cash Equivalents	$5,189,348	Pledges Receivable	$245,098
	Pledges & Accounts Receivable	$1,919,879	Property & Equipment, Net	$334,061
	Other Current Assets*	$724,042	Patents	$580,456
			Investment in Subsidiary	$7,263,532
			Other Long Term Assets	$1,213,131
	Total Assets			**$17,469,547**
Liabilities & Net Assets	Current Liabilities			$1,879,434
	Long Term Liabilities			$499,983
	Net Assets			$15,090,130
	Total Liabilities & Net Assets			**$17,469,547**
Operating Income	Grants			$883,883
	Special Events, Net			$10,422,221
	Contributions			$1,006,349
	Released From Restrictions			$250,000
	Fee For Service			$410,883
	Donated Goods & Services			$22,979
	Interest & Other			$335,650
	Total Public Support			**$13,331,965**
Operating Expenses	Program		Support	
	R&D		General Fund	$910,653
	Science	$7,130,128	Fundraising	$532,015
	Informatics	$590,174	**Total Support**	**$1,442,668**
	Patient Services	$80,147		
	Communications	$2,103,270		
	Business Development	$135,482		
	Total Program	**$10,039,201**		
	Total Expenses			**$11,481,869**

** This financial information is derived from audited financial statements, available upon request.*

2014-2019 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.

	2014	2015	2016	2017	2018	2019	Total Spent
Program Services	$9,977,506	$11,257,021	$9,705,301	$10,771,564	$10,587,060	$10,039,201	$62,337,653
Support Services	$1,524,655	$1,425,127	$1,569,649	$1,766,028	$1,815,788	$1,442,668	$9,543,915



***Ledford, H. "Keeping the Lights On." Nature, 20 November 2014*

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