

ALS

THE THERAPY DEVELOPMENT
INSTITUTE

2016
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

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Alan Gill, Ph.D.	Beth Levine	Alan Premasiri	Yiding Yan
Cynthia Gill	Matvey Lukashev, Ph.D.	Aarthi Ravi	



Dear Friend,

It is an overwhelming feeling knowing how many people with ALS and their families are part of the ALS Therapy Development Institute (ALS TDI) today. Thousands of families fuel our work at ALS TDI and it is with them in mind that I share our annual report with you.

A prominent set of accomplishments this year focused on the expansion of our partnerships with the ALS community: people living with ALS, the ALS researcher and clinical community, and pharmaceutical and biotechnology companies working on treatments and cures for ALS.

The mission of the ALS Therapy Development Institute has been, and will always be, laser focused: to discover and develop effective, targeted treatments for ALS. With our multifaceted approach, we are closer to accomplishing this goal.

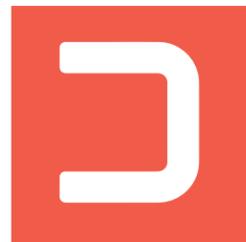
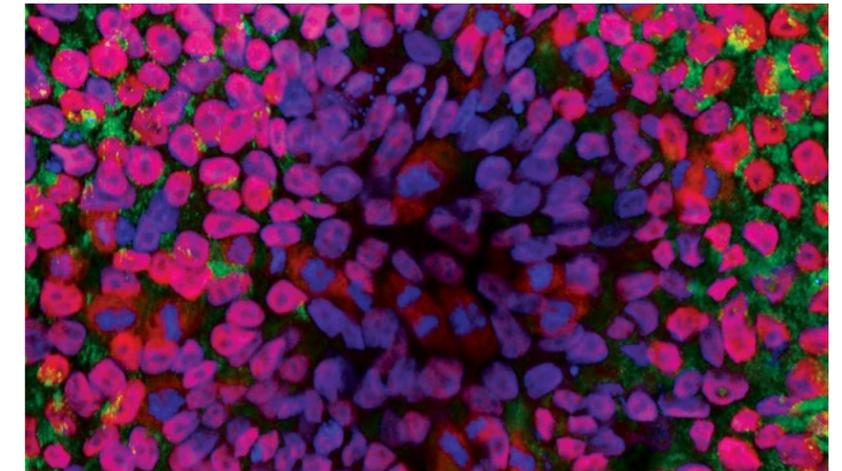
The validation of Copper ATSM as a potential treatment for ALS now inaugurates the ALS Therapy Development Institute as the only ALS research organization that has discovered or validated four potential treatments for ALS, using rigorous and established best practices. The other three potential treatments—an antibody targeting mis-folded SOD1 (NI-206), a gene therapy targeting the SOD1 gene, and AT-1501, an immune modulatory treatment—are all moving into clinical development for people living with ALS. It is crucial that we find the resources to advance these exciting potential treatments through clinical trials.

One of the things I am most proud of is our Precision Medicine Program (PMP), the world's first of its kind in ALS. More than 450 people living with ALS have enrolled in the Precision Medicine Program since the fall of 2014. Since its inception, the PMP initiative was designed to optimize tools in telehealth to develop new quantitative outcomes for people living with ALS; identify genetic markers in the genomes of people living with ALS to stratify ALS; improve the clinical trial process and dramatically increase the likelihood of developing effective targeted treatments for ALS; develop cell-based models of ALS to discover new mechanisms leading to the onset and progression of disease; and aggregate this complex information into new data models and share with the participants in real time. This program has changed how we work at ALS TDI and I believe its outcomes will significantly change how the rest of the field works with people with ALS in clinical trials in the future.

With sincere thanks for your continued support.

Steve Perrin, Ph.D. | CEO and CSO
 ALS Therapy Development Institute

2016 was a year in which progress was made both inside and outside the lab. New partnerships were formed with highly respected organizations, signature and community events helped boost awareness and raise funds for research, and the ALS community supported every effort to help us find effective treatments for ALS.



Created partnerships with UMass Medical and MGH called [ALS ONE](#) to accelerate potential treatments for ALS through preclinical and clinical research.

Entered into [a research collaboration with Denali Therapeutics](#) to investigate potential new endpoints for use in ALS clinical trials.

[The ALS Therapy Development Institute Tri-State Trek](#) broke records, raising \$802,000 for ALS research.

Demonstrated the stability and scalability of [AT-1501](#), the most promising treatment ever tested at ALS TDI.

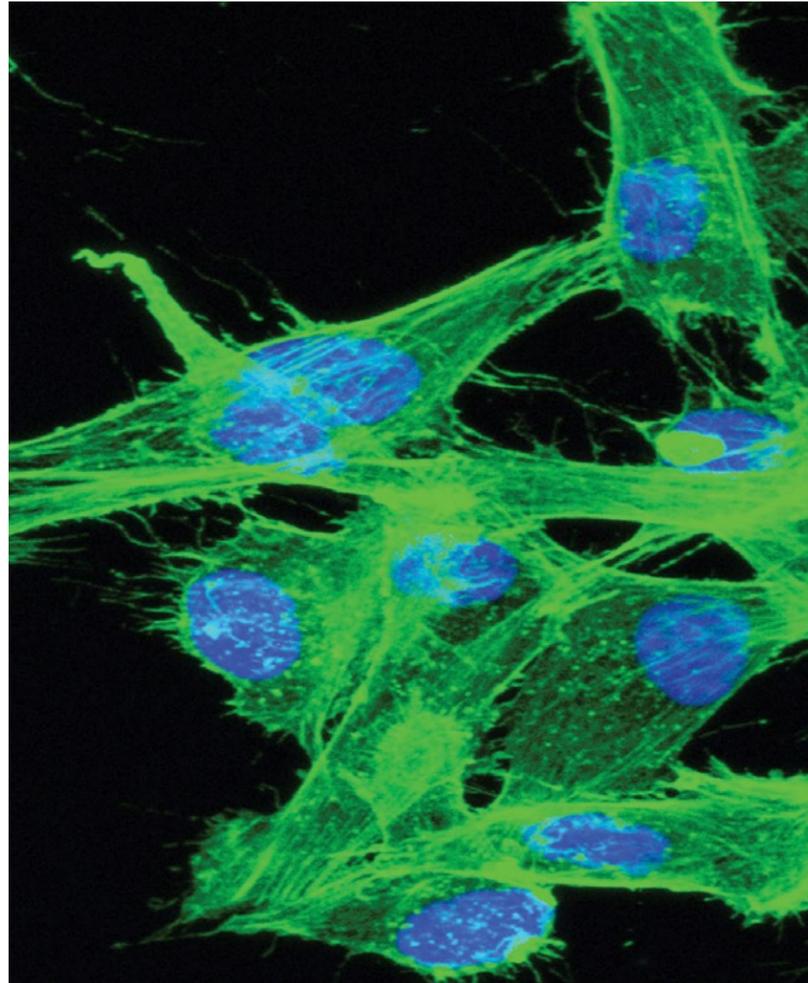
ALS TDI was featured as a leader in its field in a [VICE on HBO® documentary](#) on ALS.

Enrolled 300th participant in the ground-breaking [Precision Medicine Program](#), making it the fastest enrolled observational study in the history of ALS research. Expanded enrollment to include another 450 people with ALS.

[Ales for ALS™](#) hit fundraising milestone of \$1 million. More brewers than ever participated in the 2016 program.

Validated the efficacy of [Copper ATSM](#) in the SOD1 mouse model, making it the first proposed treatment ever to be validated preclinically in three independent research centers.

We are proud of our accomplishments in 2016, but we understand that we still have some way to go to ensure that an effective treatment reaches those diagnosed with ALS as soon as possible. We will not stop until we achieve our mission of seeing effective treatments in the hands of people with ALS.



Precision Medicine Program (PMP)

When the ALS Therapy Development Institute (ALS TDI) launched its Precision Medicine Program (PMP) in the summer of 2014, we had a plan to enroll just 25 people living with ALS. In July of 2016 we enrolled the 300th person in the program – a huge achievement, made possible with the generous support of our community.

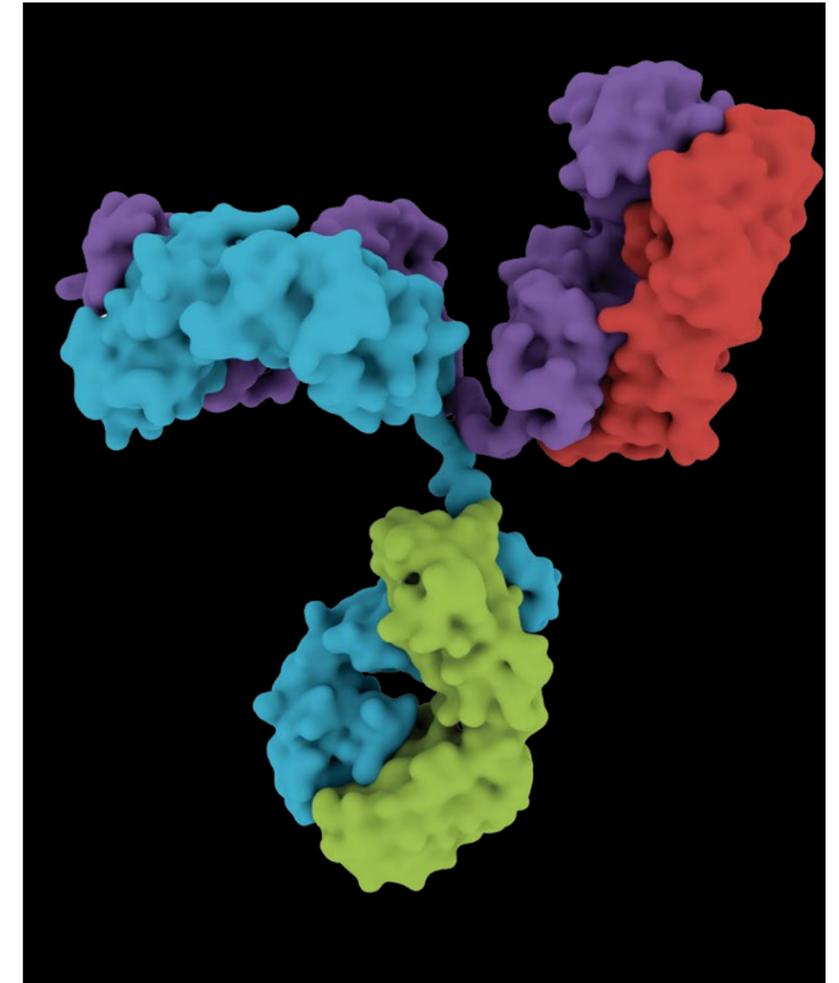
So far, this program has created the largest patient-linked database integrating genetics, voice recordings, lifestyle, demographics and accelerometer data. Using these data, we can create patient cell lines modeling human disease, helping us focus our research on specific disease-relevant biologies among other things. As a result of the success of the Precision Medicine Program so far, we broadened the plan to enroll an additional 450 people with ALS as partners in research, to help us discover effective treatments and a cure for ALS.

Promising Treatment

In 2016 we worked with Lonza to test the stability and scalability of manufacturing AT-1501. AT-1501 is an antibody therapeutic that acts in a highly targeted way to tamp down the immune system. It has produced the most exciting and comprehensive data we've seen in over 300 drugs tested at ALS TDI. In the SOD1 mouse model, AT-1501:

- extended life span significantly, beyond any other drug that has been advanced into human ALS clinical trial;
- delayed disease onset;
- improved body weight, signaling that muscle is healthier;
- improved the percentage of neuromuscular junctions that remain intact, allowing muscle to remain functional;
- decreased indications of inflammation in nerves and spinal cord.

AT-1501 is now ready for clinical translation. However, it will not make it to trial without immediate philanthropic support. To move this compound through a Phase 2 clinical trial, we will need to raise \$24 million. Your generous donations directly impact how quickly we can do this. If you would like more information on AT-1501, please visit als.net/at-1501/.



Each year since 2005, the ALS Therapy Development Institute has presented Leadership Awards to individuals nominated by the ALS community. In 2016, we honored the following people for their stories of inspiration, and unwavering dedication to the mission of our organization.

The Gosnell Family



Fran Delaney Challenge & Respect Award

Shortly after Kevin Gosnell was diagnosed with ALS in 2015, he launched an unprecedented effort called ALS ONE, bringing together researchers from UMass Medical, Harvard, MGH and the ALS Therapy Development Institute. Kevin challenged ALS researchers to think differently and to work together differently. Always with respect for the challenges faced by researchers and ALS families alike, Kevin and his family became highly effective speakers and leaders within the ALS community, raising nearly \$6 million in funding commitments in a short amount of time. Kevin lost his battle with ALS in August 2016.

Ernest (Rusty) Perry



Mary Lou Krauseneck Courage & Love Award

Rusty was diagnosed with ALS in 2012, and searched the world for the most impressive ALS research and potential treatments. He and his community created Hunting & Fishing for a Cure, and raised several million dollars for research efforts at Houston Methodist and the ALS Therapy Development Institute. Rusty and his family, and their organizing committee built their success together with a deep sense of love and courage, empowering their community to become involved and make a difference in the battle to end ALS. Rusty lost his battle to ALS in December 2016.

Smith & Hanses Family



Stephen Milne Adventurous Spirit Award

Cheryl Smith has seen ALS in her family for generations, including today, with several family members battling the disease, including her brother Scott “Bubba” Hanses. In 2013, together with her husband, Mike Smith, their children and brewer friends, the Smith and Hanses Family created Ales for ALS™. Now in its fourth year, Ales for ALS™ is a successful national program which raises funds for ALS research as well as ALS awareness inside more than 100 tap rooms across the United States.

Andrea Lytle Peet



Stephen Heywood Patients Today Award

Andrea Lytle Peet was diagnosed with ALS at 33. Through her blog and fundraising efforts, Andrea, and her husband David are passionate and effective ALS educators and have set up a non-profit, Team Drea, to boost awareness and research funding. An athlete her entire life, she completed her tenth triathlon AFTER her diagnosis and today, continues to ride her recumbent trike in races around the country to raise much needed funding and awareness for ALS. Andrea also serves as a National ambassador for ALS TDI.

For fiscal year ending December 31, 2016, ALS TDI received more than \$12.3 million in contributions and other income. This represents a funding increase of almost \$2 million from the previous year, enabling ALS TDI to invest further in science programs, such as AT-1501 and the Precision Medicine Program.

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.

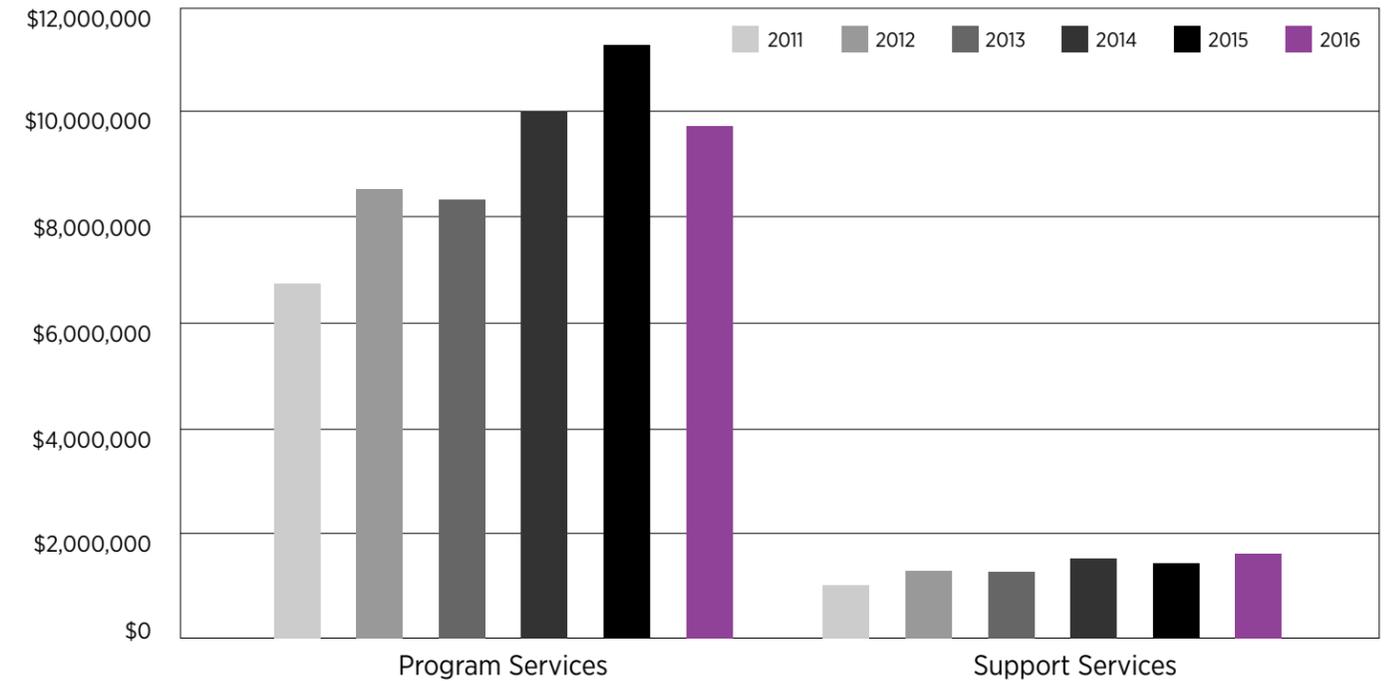
Financial Review



2011-2016: A Financial Comparison

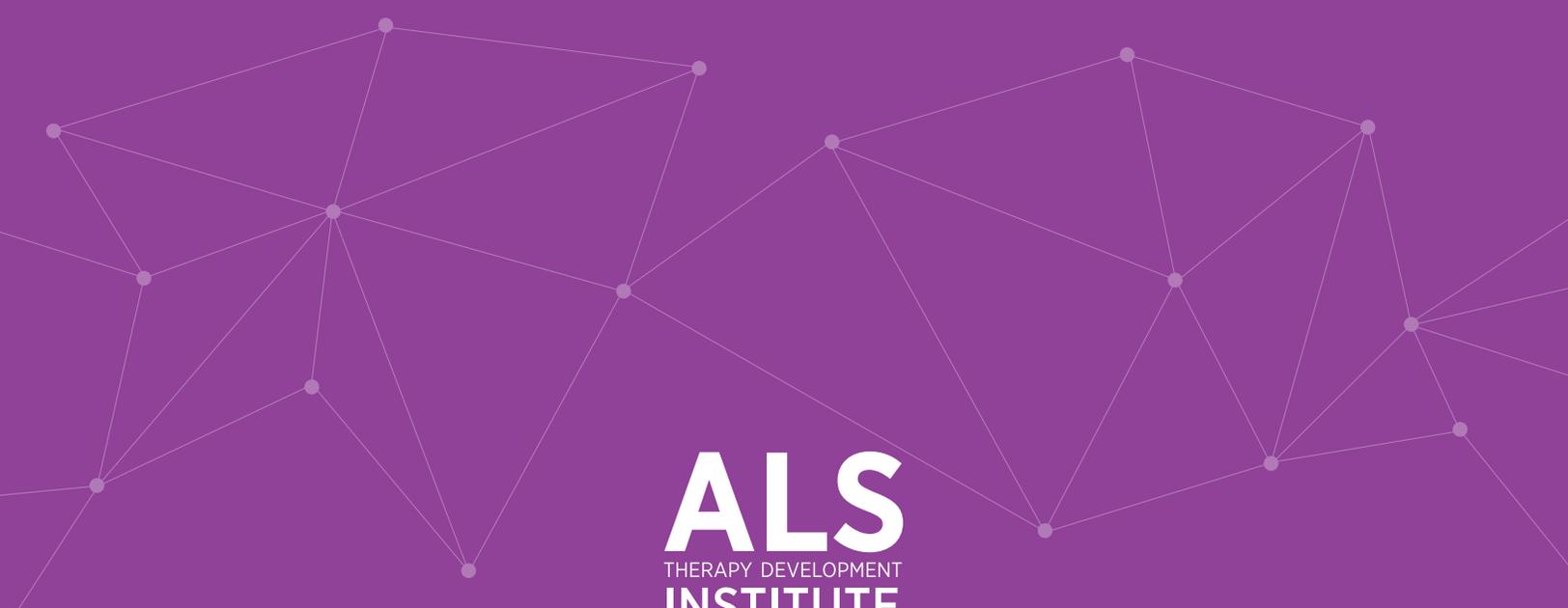
Assets				
	Current Assets		Other Assets	
	Cash And Cash Equivalents	\$4,562,414	Pledges Receivable	\$225,000
	Pledges & Accounts Receivable	\$154,172	Property & Equipment, Net	\$841,903
	Other*	\$3,059,669	Patents	\$241,060
	Net Assets		\$9,084,218	
Liabilities & Net Assets				
	Current Liabilities		\$2,016,266	
	Long Term Liabilities		\$1,071,984	
	Net Assets		\$5,995,968	
	Total Liabilities & Net Assets		\$9,084,218	
Operating Income				
	Grants		\$1,358,875	
	Special Events, Net		\$8,441,190	
	Contributions		\$669,386	
	Released From Restrictions		\$530,000	
	Fee For Service		\$978,799	
	Donated Goods & Services		\$6,613	
	Interest & Other		\$385,101	
	Net Public Support		\$12,369,964	
Operating Expenses				
	Program		Support	
	R&D		General	\$659,258
	Science	\$6,833,675	Fundraising	\$910,391
	Informatics	\$398,601	Total Support	\$1,569,649
	Patient Services	\$160,585		
	Communications	\$2,056,930		
	Business Development	\$255,510		
	Total Program	\$9,705,301		
	Total Expenses		\$11,274,950	

	2011	2012	2013	2014	2015	2016	Total Spent
Program Services	\$6,707,315	\$8,454,877	\$8,301,674	\$9,977,506	\$11,257,021	\$9,705,301	\$54,403,694
Support Services	\$1,035,726	\$1,276,210	\$1,262,610	\$1,524,655	\$1,425,127	\$1,569,649	\$8,093,977



Anelixis
THERAPEUTICS

In 2015, the ALS Therapy Development Institute established Anelixis, Inc., as a wholly 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 to advance potential treatments, such as AT-1501.



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