OUR MISSION

ALS ASSOCIATION
The ALS Association is the only national nonprofit organization fighting the disease on every front – supporting cutting-edge research, enhancing access to clinical care, and promoting better public policies. Our nationwide network of chapters comprise one team with a single mission: to discover treatments and a cure for ALS, and to serve, advocate for, and empower people with ALS.

Everything we do at the Association is centered on people with ALS – the care we support, the policies we advocate for, and the research we advance. The only way we will realize our vision of a world without ALS is to make sure that all of these parts are fully integrated and working together.

- The public policies we advocate for increase funding for ALS research and improve access to care and assistive technology for people with ALS.
- The care services programs we support improve the quality of life for people living with ALS, and enables them to participate in clinical trials that advance research toward treatments and a cure.
- The research we fund informs the development of treatments and technologies for people with ALS, and furthers support for more investment in ALS research.

In the past year, the Association has made remarkable strides. Our global research program has supported projects and collaborations that led to breakthroughs in new therapies, diagnostic tools, and technologies to assist people living with ALS. Our nationwide network of more than 16,000 advocates helped protect and secure millions of dollars in funding for ALS research and enhanced access to treatments, including $10 million in the Department of Defense ALS Research Program and $10 million for the National ALS Registry. Our care services team served more than 19,000 people living with ALS, including 8,975 people served through our Certified Treatment Centers of Excellence and Recognized Treatment Centers.

Keep reading to learn more about the tremendous work being done throughout the Association. We will continue our work to help people with ALS live their lives to the fullest while we search for new treatments and a cure.
The ALS Association fights every day for people living with ALS, leading cutting-edge research to discover treatments and a cure for ALS and serving, advocating for, and empowering people living with ALS to live their lives to the fullest.

**RESEARCH**

**KIF5A ALS Gene Discovered**
A large collaborative effort involving over 250 researchers, led by Dr. John Landers (UMass Medical School) and Dr. Bryan Traynor (NIH), came together to achieve this exciting gene discovery, including organizations supported by the Association - Project MinE, Genomic Translation for ALS Care, Answer ALS, the Target ALS Postmortem Tissue Core, the New York Genome Center (NYGC) ALS Consortium, the CReATe Consortium, the NIH, and others.

**Antisense Drugs Achieved Significant Progress**
- Biogen announced encouraging results of SOD1 (BIIB067) phase I trial and is moving to the next clinical phase with urgency.
- Biogen also initiated the C9orf72 (BIIB078) phase I trial this fall and the first patients were dosed.

**Assistive Technology Advanced at Rapid Pace**
- Pison Technology, winner of our ALS Assistive Technology Challenge, is growing. Our $100,000 investment leveraged over $1.7 million in outside funding and their wearable muscle sensor technology is moving fast toward the market.
- Project Revoice was launched to help encourage people with ALS to voice bank and make it easier to use their synthesized voice.
- Telemedicine is on the rise to help people with ALS connect with their care teams, especially in rural areas, through the hard work of our chapter network and funded researchers.

**Significant Progress for ALS Clinical Trials**
- Amylyx Pharmaceuticals is close to finishing enrollment in its phase II AMX0035 trial and announced an open-label extension study.
- Origent Data Sciences made headway in clinical trial patient stratification using its unique computer algorithms.
- The mexiletine phase II trial showed positive results in reducing muscle cramping.
- The phase II ezogabine trial completed, meeting its primary goal to measure a reduction in motor neuron excitability in people with ALS following treatment.

**ADVOCACY**

Supported nearly 500 meetings with Members of Congress at the Advocacy Fly-In and National ALS Advocacy Conference. Empowered more than 16,000 ALS Advocates with six action alerts – resulting in nearly 4,700 advocacy messages to Congress.

Successfully protected the ALS Research Program at the Department of Defense (DOD), resulting in $10 million in appropriations and ensuring its place at the DOD.

Continued leadership on ensuring patient voice in the FDA’s Draft Guidance on ALS Drug Development by hosting a public forum that provided the FDA with targeted feedback and information from people with ALS, caregivers, and stakeholders.

Continue to play a key role in ensuring people with ALS have access to wheelchairs, speech-generating devices, and other complex technology through legislative and regulatory pressure.

**CARE SERVICES**

Nearly 20,000 people viewed, downloaded, or ordered our educational materials including the Living with ALS and Families and ALS resource guides and medical information packets.

8,975 people served through our Certified Treatment Centers of Excellence and Recognized Treatment Centers.

ALSA.org
TABLE OF CONTENTS

PART 1 - RESEARCH

SECTION 1: RESEARCH TALKING POINTS, NARRATIVE & STRATEGIC INITIATIVES

1. Research Narrative: TREAT ALSTM Global Research Program ........................................ 1.1.1
2. High-Level Talking Points .................................................................................................. 1.1.2
3. Impact on Research: The ALS Ice Bucket Challenge ..................................................... 1.1.3
4. Strategic Initiatives ........................................................................................................... 1.1.4

SECTION 2: INFOGRAPHICS

1. Current Projects .................................................................................................................. 1.2.1
2. Research Pipeline .............................................................................................................. 1.2.2
3. 2018 Priorities and Results to Date .................................................................................. 1.2.3
4. Research Strategy .............................................................................................................. 1.2.4
5. Grant Portfolio .................................................................................................................. 1.2.5

SECTION 3: DEVELOPMENT

1. Biomarker Funding Opportunities* ................................................................................... 1.3.1
2. Biomarkers* ...................................................................................................................... 1.3.2
3. Stem Cell Funding Opportunities* ................................................................................... 1.3.3
4. Genetics Funding Opportunities* ..................................................................................... 1.3.4
5. 2018 Advances in ALS Research Infographic ................................................................. 1.3.5
6. Scientific Focus Areas ....................................................................................................... 1.3.6
7. Abbreviated Glossary of Scientific Terms ....................................................................... 1.3.7
8. Biographies ....................................................................................................................... 1.3.8

Acknowledgements

Thank you to our Chapters – especially North Carolina, Golden West, St. Louis Regional, and Rocky Mountain – for all their valuable input and revisions. This was a large team effort involving the Chapters, along with National Development, Communications, Chapter Relations, Advocacy, Executive, Research, and Care Services teams.

*Customizable by chapter. All content is copyrighted by the ALS Association.
©January 2019

Spring 2019
PART 2 - CARE SERVICES

SECTION 1: HOW WE WORK
1. How We Work/Chapter Portfolio ................................................................. 2.1.1

SECTION 2: HOW WE SUPPORT CLINICAL CARE
1. How We Support Clinical Care/Map/Table .................................................. 2.2.1

SECTION 3: RESOURCE SHEETS
1. Care Services Impact ...................................................................................... 2.3.1
2. Education Resources ...................................................................................... 2.3.2
3. Assistive Technology Resources ................................................................. 2.3.3
4. Children’s Programs and Resources ............................................................. 2.3.4

PART 3 - ADVOCACY

SECTION 1: INTRODUCTION TO ADVOCACY
1. Summary ......................................................................................................... 3.1.1
2. History of ALS Advocacy ............................................................................. 3.1.2
3. Advocacy Timeline ....................................................................................... 3.1.3

SECTION 2: ADVOCACY GUIDANCE
1. FDA Guidance ............................................................................................... 3.2.1
2. How We Advocate ......................................................................................... 3.2.2

SECTION 3: PRIORITIES, IMPACT & COALITIONS
1. Public Policy Priorities .................................................................................. 3.3.1
2. Impact on Advocacy ....................................................................................... 3.3.2
3. Biographies .................................................................................................... 3.3.3
4. Index - Value of Coalition Memo ................................................................. 3.3.4
RESEARCH TALKING POINTS, NARRATIVE & STRATEGIC INITIATIVES
SUMMARY

The ALS Association’s global research program has remained at the forefront of ALS research since its inception in 1985. We are the largest private funder of ALS research worldwide, and our efforts have led to some of the most promising and significant advances in ALS research. Our approach is global – the world is our lab – enabling us to fund the top ALS researchers worldwide and ensure that the most promising research continues to be supported. We fund projects across the research pipeline, from basic research through clinical trials, and our support has led to several potential treatments currently in clinical trials. Since the ALS Ice Bucket Challenge in 2014, we have tripled the amount we spend in research every year – from $6 million to $16 million in the current year – and we are committed to maintaining – and even increasing – this level.

OUR HISTORY

Since its inception, The ALS Association’s global research program has led the way on ALS research. The program was established by Robert Abendroth, one of the founders of The ALS Association and long-standing member of the Board of Trustees. The program was originally modeled after a National Institutes of Health (NIH)-style program that funded basic research grants. When The ALS Association decided to expand our research funding, Mr. Abendroth invited Dr. Tom Maniatis, a world-renowned geneticist and molecular biologist, to lead and assemble an advisory board of experts to help identify critical gaps in ALS research. In 2001, we expanded our grant portfolio to include translational research studies, our postdoctoral fellowship, and clinical trial programs. As the program grew, it was renamed Translational Research Advancing Therapies for ALS, or TREAT ALS™. Today, Dr. Neil Thakur, Executive Vice President of Mission Strategy, directs the TREAT ALS™ global research program with guidance from The ALS Association’s Research Committee.

OUR APPROACH TO RESEARCH

Through TREAT ALS™, we are dedicated to a collaborative and global approach. Since our inception, we continue to accomplish significant advances in ALS research. Everything we do works toward discovering ALS treatments and a cure. We do not fund one laboratory, but instead take a global approach in funding the most promising...
researchers worldwide, whose projects span the entire research pipeline. This covers a wide breadth of scientific focus areas – each is critical to advancing research – while spurring innovation along the way.

Collaboration is the cornerstone of our research program. Rather than conducting research in our own proprietary laboratory, our unique approach to advancing ALS research involves forging partnerships among academic institutions, industry (pharmaceuticals and biotech firms), government, and other nonprofit organizations. We host yearly scientific workshops and symposia to bring the foremost ALS experts together to discuss and explore various ALS research topics, leading to the generation of novel research ideas.

We also focus on the future of ALS research by supporting and attracting bright young scientists to the ALS field through the Milton Safenowitz Postdoctoral Fellowship Program. We foster their creative ideas and hard work to stimulate advances and propel them to the next level to start their own ALS research laboratories. Since 2005, we have awarded 64 Milton Safenowitz Postdoctoral Fellowship awards. Currently, 22 are finishing their fellowships. Of the 42 awardees who have moved on from their postdocs, 76 percent remain in academia continuing to research ALS, 19 percent moved into industry jobs, and 5 percent hold non-research based roles.

Everything we do works toward discovering ALS treatments and a cure.

HOW WE WORK
Through TREAT ALS™, we maintain a large grant portfolio. All studies funded through the organization undergo competitive review. Every year, we receive hundreds of grant applications, and the number of applications received has doubled since the ALS Ice Bucket Challenge. Leading experts in ALS and related fields from around the world review our grant program to select the most promising ALS projects. Our grant review process is rigorous, following policies and procedures that are in place to maintain the utmost integrity of the research program. Members of our Research Committee provide oversight for final approval of the grants, and the Board of Trustees gives the final approval.

OUR ACHIEVEMENTS
To date, The ALS Association has funded more than $147 million in ALS research. Currently, we are funding over 108 projects in 13 countries, all selected through our competitive peer review process, involving top ALS scientists. We are the largest private funder of ALS research worldwide. Our unrelenting focus has resulted in some of the greatest ALS research discoveries in history. From the earliest stages of our program, The ALS Association has recognized novel approaches that have led to significant ALS research breakthroughs.
EXAMPLE OF OUR TRACK RECORD OF SUCCESS

The ALS Association has a strong track record of success when it comes to advancing research. The ALS Association was the first to invest in antisense technology targeting the second most common genetic cause of ALS: SOD1. We supported antisense research despite the high risk of the technology not coming to fruition. Our initial investment of $1.5 million to ALS researchers propelled the concept all the way from an academic laboratory in partnership with industry to testing the approach in the clinic. Recently, a phase I clinical trial for people carrying the SOD1 mutation has ended with promising results with plans to begin the next phase in the near future. Also, a clinical trial targeting C9orf72, the most common genetic cause of ALS, started and began enrolling in fall 2018.

Our translational approach facilitates the development of potential antisense treatments not only for ALS, but also for other neurodegenerative diseases, including Huntington’s disease, Alzheimer’s disease, and Frontotemporal dementia. In this way, the value of our initial investment has ballooned from $1.5 million to more than $100 million. In December 2016, the FDA approved antisense technology targeting spinal muscular atrophy, a common neuromuscular disease, which is the leading genetic cause of death in infants and toddlers. This is the first approved treatment for this fatal disorder. This success gives us much hope for the future of antisense therapies targeting ALS.

THE FUTURE

Building on success, our research program continues to evolve with an increased focus on people living with the disease. Driven by a sense of urgency, we are driving drug development while leveraging innovative partnerships with industry, the investment community, and federal agencies. In the years ahead, we will see an increase in the number of clinical trials, as many of the newer approaches in gene therapy continue to expand. Through engagement with the Federal Drug Administration (FDA) and the voice of people living with the ALS, clinical trial design and biomarker programs will be enhanced.

We are in an era of precision medicine that targets an individual patient’s disease process and takes into account individual variability in genes, environment, and lifestyle. As a result, many more large collaborative partnerships will emerge focusing on collecting clinical data closely linked to gene sequencing aimed at improving clinical trial design. Through our consortium initiatives, new therapeutic targets will be identified leading to new treatment approaches.

Treatments that significantly change the course of ALS and ultimately halt the disease continue to be a high priority in our programs. Improving the lives of people living with ALS is also a primary focus. In recognition of the interdependence of care and research, we will continue to leverage our clinical network to conduct a variety of qualitative and quantitative research with our clinics to help to improve patient care, communications, and mobility for those living with ALS.
GENERAL PROGRESS POINTS

• In August 2014, millions of people participated in the ALS Ice Bucket Challenge — raising awareness and donating more than $115 million to The ALS Association.

• The ALS Association continues to be a global leader in advancing ALS research. With the generous support of our donors, we are currently funding more than 108 active ALS research projects in 13 countries (as of Feb. 2019).

• In The ALS Association’s history, we have committed over $147 million to research.

• Due to the huge funding boost from generous donors during the ALS Ice Bucket Challenge, so far we have committed more than $89 million to research alone, not including investments in the other core pillars of The ALS Association. This commitment is focused on specific projects and large global initiatives to help drive progress toward finding treatments and a cure for ALS. For more information visit: www.alsa.org/ibcspending/

• We feel privileged with this great responsibility to spend these dollars wisely, transparently, and in ways that make the most impact on the fight against this disease.

• We are committed to maximizing all donations from the ALS IBC and beyond by partnering with other organizations to fund research, along with tremendous efforts by our ALS advocates and volunteers across the country. It is important to keep up the momentum to contribute to funding the most promising ALS research all over the world!

The ALS Ice Bucket Challenge of August 2014 raised more than $115 million for The ALS Association.

IN 2018, THE ALS ASSOCIATION:

• Committed an $18 million research budget to support promising projects all over the globe.

• Funded over 61 projects in 9 countries in new awards.

• Advanced ALS research to better understand the disease and contributed to the knowledge base of ALS research in the scientific community, where important results and comprehensive ALS reviews were published in top scientific journals by researchers and clinicians we funded.

• Invested in numerous ALS clinical trials through our Clinical Trial Pilot Program, facilitated partnerships between academia and industry to propel ideas from the lab into clinical trials, and invested in clinical trial infrastructure to ensure that trials are carried out at the highest level.
Many new clinical trials we are funding began enrollment.

Funded six new bright, young scientists through our Milton Safenowitz Postdoctoral Fellowship Program and two new clinician scientists through fellowship awards in partnership with the American Academy of Neurology (AAN).

Key advancements were made in wearable sensor technology by our ALS Assistive Technology Challenge winner – Pison Technology. The Association’s $100,000 initial investment into Pison has resulted in over $1.7 million in additional funding from other resources (local, federal, and angel investor grants) and has allowed their team to grow exponentially. The goal is to bring their assistive technology products to market as quickly as possible and are beginning to test their prototypes on people with ALS.

ELEVEN NEW STRATEGIC INITIATIVES SUPPORTED

Strategic initiatives are large global collaborations focused on the understanding of the disease, targeting new therapies, expediting clinical trials, and making RNA and DNA sequencing data available to the entire ALS research community. These initiatives will generate data and resources available for researchers globally. Central to all the major new collaborations are the people living with ALS.

In October 2014, The ALS Association announced initial ALS Ice Bucket Challenge spending decisions designed to ignite four new strategic initiatives – Project MinE, New York Genome Center for Genomics of Neurodegenerative Diseases (NYGC CGND), the Neuro Collaborative, and ALS Accelerated Therapeutics (ALS ACT) — to advance progress towards finding treatments for ALS.

Since then, The ALS Association has announced its support for seven new collaborative strategic teams – CREATE Consortium, Genomic Translation for ALS Clinical Care (GTAC), Answer ALS, NeuroLINCS, and ALS ONE, and in 2018 announced partnerships with GNS Healthcare and ALS Reproducible Antibody Platform (ALS RAP). Refer to “Our Strategic Initiatives” page for more information..

TWO NEW ALS ANTISENSE DRUGS

Antisense therapy, which The ALS Association has supported since 2004, prevents the production of proteins involved in disease, with the aim to slow or stop disease progression in people living with ALS.

The Neuro Collaborative is one of the major research strategic initiatives made possible by the ALS Ice Bucket Challenge. Its scientists have developed antisense drugs targeting two of the most common ALS genes, SOD1 and C9orf72.

This progress can be directly attributed to The ALS Association’s early investment of SOD1 antisense technology. The SOD1 antisense clinical trial started in early 2016, led by Biogen and Ionis Pharmaceuticals and recently reported promising results and is moving onto the next clinical trial phase in the near future. A C9orf72 antisense trial initiated and began enrolling in the fall 2018.
• Our investment in this innovative technology also translates to other neurodegenerative diseases, making our impact even greater. In December 2016, the FDA approved the antisense drug SPINRAZA™ to treat a broad range of people with spinal muscular atrophy (SMA), a common, fatal genetic disease in children.

**CLINICAL TRIALS**

• Currently, over 62 ALS clinical trials are enrolling, including 29 observational trials and 33 interventional trials. [Information obtained from NEALS]

• In mid-August, Orphazyme A/S announced that a person with ALS has received the first dose of arimoclomol in its phase III clinical trial.
  - The Association funded the successful phase II trial that led up to this point.
  - The phase III trial is enrolling people with both familial (inherited) and sporadic (non-inherited) ALS.
  - The study goal is to determine both safety and efficacy of the oral drug.
  - An open-label extension study will be offered after trial completion.

• A phase III clinical trial testing the safety and efficacy of levosimendan in preserving respiratory function in patients with ALS was initiated in summer 2018. The sponsoring company is Orion Pharma.

**DRUG DEVELOPMENT**

• *Anelixis Therapeutics, a subsidiary of ALS TDI, moved its compound AT-1501 into clinical trials and dosed its first patient.

• *A viral gene therapy targeting the ALS SOD1 gene was safe and effective in nonhuman primates, which paved the way for a human clinical trial.

• *Karyopharm Therapeutics announced that Biogen acquired compound KPT-350 with plans to advance it into clinical trials in the near future.

• Biohaven Pharmaceuticals began a phase I open-label study of BHV-0223, a sublingual form of riluzole. This formulation melts quickly under the tongue.

• *The mexiletine phase II trial showed positive results in reducing muscle cramping.

• *The phase II ezogabine trial completed meeting its primary goal to measure a reduction in motor neuron excitability in people with ALS following treatment.

*Notates research funded by The ALS Association
FIVE GENES IDENTIFIED SINCE THE ALS ICE BUCKET CHALLENGE

• Identifying ALS genes that contribute to ALS disease allows scientists to target them for therapy, essentially increasingly the likelihood that a TREATMENT will be found.

• Five new ALS genes that contribute to the development of the disease have been identified in the last two years, two of which, NEK1 and C21orf2, were announced in July 2016 and KIF5A was announced in March 2018. (Note: the five genes identified since the ALS IBC are KIF5A, NEK1, C21orf2, TUBA4A, and TBK1.)

• Researchers who are part of Project MinE’s global gene sequencing effort identified NEK1, which now ranks among the most common genes that contribute to the disease. The study involved contributions from more than 80 researchers in 11 countries.

• In March 2018, the KIF5A gene was discovered with money raised from the ALS Ice Bucket Challenge. Researchers from organizations Project MinE, Genomic Translation for ALS Care, Answer ALS, the Target ALS Postmortem Tissue Core, the New York Genome Center (NYGC) ALS Consortium, the CReATE Consortium, the National Institutes of Health (NIH), and others came together to collaborate and share detailed genetic and clinical information needed to make this important discovery. The Ice Bucket Challenge enabled The ALS Association to support the above unique collaborative initiatives in precision medicine to accelerate the discovery of new treatments for ALS.

• Global collaboration among scientists, which was made possible by ALS Ice Bucket Challenge donations, led to these important gene discoveries.

NEW FORMULATION OF RILUZOLE IS FDA APPROVED

ITF Pharma, Inc. announced that Tiglutik, a thickened liquid form of riluzole, was approved by the FDA to treat ALS in early September. This liquid formulation, in contrast to the pill form, should help individuals with swallowing difficulties. Approximately 80 percent of people with ALS develop difficulty swallowing because of gradual weakness and paralysis in the muscles of the face and throat, called bulbar muscles. As a result, some people with ALS end up crushing their pills to take their medications.

• It the first and only easy-to-swallow thickened liquid form of riluzole.

• Its most common side effects are in line with established side effects observed in oral riluzole.

• It can be administered orally twice-daily via a syringe.

• Tiglutik became commercially available in the U.S. in October 2018.
WHY SUPPORT FOR RESEARCH IS CRITICAL

- There is renewed hope in ALS science circles these days. The excitement is fueled in part by important new discoveries. In recent years, researchers have pinpointed a key genetic cause of the disease and begun sorting through, to an unprecedented degree, the way that a gene mutation plays out inside the brain’s motor neurons.

- We have tripled the amount of money allocated to research in the years since the Ice Bucket Challenge, leading to greater scientific output and a greater understanding of the disease pathways and potential strategies for new therapies.

- To get a drug from preclinical trials to market costs between $50M and $100M on average, according to the U.S. Department of Health and Human Services (HHS).

- It can take between $2 billion and $3 billion to develop one new pharmaceutical treatment from an idea to an approved drug.

- A clinical trial is the best way for researchers to find effective treatments, and, equally important, to weed out useless or harmful ones. Clinical trials are costly and may last months. When the treatment being tested proves to not be effective, it can be sorely disappointing. But clinical trials have proven to be the most reliable, and ultimately the fastest, way to discover treatments that really work.

- Unfortunately, people with ALS will pass away while waiting for experimental treatments to slowly crawl through the approval process, and some promising drugs are simply shelved because companies run out of money trying to finance multiple long and expensive clinical trials.

- The ALS Association funds top ALS researchers all over the globe, forming lasting global collaborations, and encouraging data sharing to avoid duplication. We invest in innovation, infrastructure, technologies, and partnerships, exploring all aspects of the disease across the research pipeline, from idea through phase II clinical trials.

- Funding from The ALS Association over the past 30 years has contributed to a better understanding of ALS, funded all the major ALS gene discoveries, improved care for people living with the disease, and explored new treatment approaches in clinical trials.

- Our work is not done. Researchers are close to major ALS scientific breakthroughs and therapies. They are motivated to finish the work they started! Together, we can help them get there.

For questions or more information, please contact Jill Yersak, Ph.D., Director, Mission Strategy, at jyersak@alsa-national.org.
THE ALS ICE BUCKET CHALLENGE

In August 2014, millions of people around the world dumped buckets of ice water on their heads to raise awareness and funds in support of the ALS community. The result was staggering – The ALS Association welcomed 2.5 million new donors, the majority of them millennials, and received $115 million in just six weeks; at least $100 million more was donated to other ALS organizations around the world. It was the most important moment in the history of ALS since Lou Gehrig’s farewell speech more than 75 years ago. Ultimately, the ALS Ice Bucket Challenge became the single biggest act of collaborative grassroots fundraising in history. This all stemmed from the efforts of three young men living with ALS, who inspired their communities, celebrities, and the world to join the fight against, and bring awareness to this devastating disease.

The ALS Ice Bucket Challenge became the single biggest act of collaborative grassroots fundraising in history.

ALS ICE BUCKET CHALLENGE AND RESEARCH

The ALS Ice Bucket Challenge and the infusion of funding it generated has had a significant impact on advancing ALS research globally. Since 2014, The ALS Association has invested more than $89 million in the most promising research projects. The ALS Association research budget more than tripled to an all-time high of $19 million, which propelled the organization to become the largest ALS research funder outside the U.S. federal government in any single year in the world. We proudly fund diverse, cutting-edge research through our competitive TREAT ALS™ global research program in laboratories around the world, rather than just one laboratory. With your help, we funded more than 176 active projects, including 61 new grant awards in 9 countries in the last year alone, in addition to a total of nine global collaborative initiatives that would not have been possible without the ALS Ice Bucket Challenge. Through collaborations with government, industry, academia, and other nonprofit organizations, The ALS Association aims to accelerate drug development so that people living with ALS receive treatments faster.
The ALS Association has announced nine important research strategic initiatives since the ALS Ice Bucket Challenge to advance the search for treatments and a cure for the disease. In this document, we have included details on the goals of each initiative, along with information on The ALS Association’s role and funding commitments.

Strategic initiatives are large global collaborations focused on the understanding of the disease, targeting new therapies, expediting clinical trials, and making RNA and DNA sequencing data available to the entire ALS research community. These initiatives will generate data and resources available for researchers globally. Central to all the major new collaborations are the people living with ALS.

Learn more about each strategic initiative here:

PROJECT MINE

$1.4 million original commitment partnered with Greater New York and Georgia Chapters. Total commitment since 2014 is over $2.1 million.

An international, large-scale research initiative devoted to discovering genetic causes of ALS and to ultimately finding a cure. The goal is to identify genes associated with ALS by performing whole genome sequencing on at least 15,000 ALS patients plus 7,500 healthy controls worldwide, resulting in an open-source ALS genome database, in conjunction with the collection of skin samples to make ALS patient-induced pluripotent stem cell (iPSC) lines. Our funding supports the U.S. arm of this initiative, led by Jonathan Glass, M.D. (Emory University) and John Landers, Ph.D. (University of Massachusetts Medical School). Original funding announced in October 2014.

NEW YORK GENOME CENTER – CENTER FOR GENOMICS OF NEURODEGENERATIVE DISEASES (NYGC CGND)

In 2015, The ALS Association funded $2.5 million in our original commitment and partnered with Greater New York Chapter. That commitment was matched with an additional $2.5 million contributed by the Tow Foundation. In 2019, the Association committed an additional $3.5 million, including a $1 million commitment from the Association’s Greater New York Chapter. That brings our total contribution to $6 million.

A consortium that is a collaboration between numerous global laboratories capable of generating and analyzing thousands of DNA sequences from people with ALS. The goal is to discover new genetic contributors of ALS to then translate into clinical solutions for ALS. It houses all data in a central repository that is freely available to the research community worldwide. Original funding announced in October 2014. Additional funding announced in February 2019.
GENOMIC TRANSLATION FOR ALS CARE (GTAC)

$3.5 million commitment partnered with Greater New York Chapter

A collaboration between Biogen and Columbia University Medical Center (CUMC) to better identify new targets for treatment development, in order to understand how different genes contribute to various clinical forms of ALS. This will translate into clinical trials that are more focused. This project will follow 1,500 people with ALS and collect detailed clinical data, sequence their DNA and store blood cell samples to generate iPSCs. This study will allow correlation of ALS clinical symptoms to genetic causes and help stratify patients for future clinical trials. Funding announced in August 2015.

CReATe

Clinical research in ALS and Related Disorders for Therapeutic Development (CReATe) Consortium: $450K commitment for biomarker study and biorepository and an $835,937 commitment to Drs. Paul Taylor, Jinghui Zhang, and Michael Benatar for DNA sequencing

A Rare Diseases Clinical Research Consortium (RDCRC) that forms part of the National Institutes of Health (NIH) Rare Diseases Clinical Research Network. The goal of CReATe is to identify new genes and novel disease pathways linked to ALS and related disorders. In addition to sequencing samples collected from study participants, CReATe is building a resource of biosamples that have attached detailed clinical information, providing a unique and critical resource for biomarker development. The biorepository will enable the discovery and validation of biomarkers relevant to therapy development for patients with ALS and related disorders. In partnership with The ALS Association, CReATe is funding pilot biomarker projects using this resource, as well as other biorepositories, including the Northeast ALS Consortium (NEALS) biorepository supported by The ALS Association. Funding announced September 2015.

• CReATe Connect: All ALS organizations associated with CReATe are a part of Connect

A part of the Rare Diseases Clinical Research Network (RDCRN) Contact Registry, CReATe Connect is an international online system to help facilitate communication between doctors/scientists and patients and their families. CReATe Connect provides a means for patients with these rare diseases (and their family members) to indicate their willingness to be contacted in the future about clinical research opportunities and to receive updates on the progress of research and new educational opportunities sponsored by CReATe.

NEUROLINCS

$2.5 million commitment partnered with the Greater Philadelphia Chapter

A partnership with NIH’s National Institutes of Neurological Disorders and Stroke (NINDS). This National Institutes...
of Health (NIH)-funded collaborative effort is between various research groups with expertise in iPSC technology, disease modeling, OMICS methods, and computational biology. The goal is to use iPSC lines from ALS patients and healthy controls and OMICS methods to identify unique cell signatures that are specific to various subtypes of motor neuron diseases, in order to better develop therapies and design clinical trials. Funding announced July 2016.

**ALS ACCELERATED THERAPEUTICS (ALS ACT)**

Original $10 million commitment matched with an additional $10 million contributed by ALS Finding a Cure®

A novel academic-foundation-industry partnership with ALS Finding a Cure, initiated with researchers from General Electric (GE) Healthcare and four academic NEALS sites to accelerate treatments for people living with ALS. It is using the following strategies to develop new therapeutic approaches for ALS: supporting development of neuroimaging tools as potential ALS biomarkers; supporting projects focused on decreasing the production of misfolded proteins, and reversing neuroinflammation, two major contributors to the disease process; supports NeuroBANK™ (see below); and supporting Phase IIA pilot clinical trials with relevant biomarkers aimed at developing novel high-potential ALS treatments. A TDP 43 PET Tracer Grand Challenge was launched as part of ALS ACT and was awarded to Dr. Timothy Miller in 2017 to develop a novel TDP-43 biomarker to track disease progression over time, improve diagnosis time, and understand whether a drug is hitting its target. Original funding announced October 2014.

- NeuroBANK™: original funding of $200,000 under ALS ACT – further expanded in August 2016 for an additional $1,679,091, bringing our total commitment to $1,879,091.

A patient-centric clinical research platform and central repository that sets the framework to allow for standardization of ALS patient information (including proteomic, genomic, and clinical data) that is linked across simultaneously-running research studies, locations, and modalities. It is designed to host, curate, and disseminate this information. Global Unique Identifier (GUID) technology generates a patient-specific character string that securely identifies a patient without revealing their true identity. NeuroBANK™ is part of NYGC projects, GTAC, Answer ALS, and other ALS research projects.

**NEURO COLLABORATIVE**

A $4.8 million commitment in October 2014 - funding through The ALS Association with contributions from the Orange County and Wisconsin Chapters. To date, we have committed a total of $7.8 million.

An initiative founded as a collaboration between three leading California laboratories aimed at discovering and developing new potential ALS therapies that can be delivered to pharmaceutical companies for further development. The three laboratories are the Svendsen Laboratory at Cedars-Sinai in Los Angeles, which will develop and maintain a Motor Neuron Core Facility to create iPSC lines from people with ALS that will be openly shared; the Cleveland Laboratory at the University of California San Diego, which will spearhead the development of antisense therapy against the C9orf72 gene, the most common genetic cause of ALS; and the Finkbeiner laboratory at the Gladstone Institutes, which is affiliated with University of California San Francisco, which will...
further develop robotic technology for screening drugs in motor neuron cell culture. The Cleveland laboratory is collaborating with Martin Marsala, M.D., at the University of California San Diego and Brian Kaspar, Ph.D., at the Research Institute at Nationwide Children’s Hospital in Ohio. In 2014, The ALS Association Golden West Chapter, along with Advisory Trustees Jim Barber and Linda Della, partnered with the National ALS Association to build the Neuro Collaborative concept. For more information, click here. Original funding announced October 2014.

ANSWER ALS

The ALS Association contributed to its development/business plan and is a partner with Team Gleason and others to advance this initiative. We plan to contribute funds as the program evolves.

An initiative spearheaded by Steve Gleason to challenge ALS researchers to come up with a solid plan to find a cure for ALS. Its strategy includes two impact goal arms. One is designed for immediate impact to help ALS patients live more productive lives by supporting affordable assistive technologies and services. The other arm is designed to contribute to the ultimate impact to fund a collaborative effort to bring together the world’s best ALS researchers to find a treatment or a cure in the next five-10 years. As part of this initiative, all DNA samples from participants will be sequenced by the New York Genome Center (NYGC), which will be funded through ALS Association research programs. In addition, NeuroBANK™ will be an integral part of the program. Projects funded as part of ALS ACT, the Neuro Collaborative, and NeuroLINCS form an important foundation for Answer ALS. Partnership announced in September 2015.

ALS ONE – MASSACHUSETTS ALS PARTNERSHIP

The ALS Association partnered with ALS ONE and ALS Finding a Cure to fund $2 million each for specific clinical and research initiatives to maximize collaborations to find treatments and a cure for ALS.

An initiative founded by Kevin Gosnell, a person who lost his battle to ALS, to bring together leading neurology experts and care specialists in Massachusetts to leverage their institutions’ strengths to expedite progress toward finding a treatment for ALS by 2020 while improving care now. Institutional partnerships include Massachusetts General Hospital, the ALS Therapy Development Institute (ALS TDI), the University of Massachusetts Medical School, and Compassionate Care ALS. Under the ALS ONE umbrella, we funded research projects of Dr. Steven Perrin from ALS TDI, Dr. Nazem Atassi from Mass General, and Dr. Robert Brown from U. Mass Medical School. The Association’s funding to ALS TDI helped move its lead compound AT-1501 from preclinical studies into a phase I clinical trial through TDI’s affiliate Anelexis Pharmaceuticals. Partnership announced January 2016. Funding announced in November 2016.
**GNS HEALTHCARE**

The ALS Association contributed $500,000 to GNS Healthcare to use artificial intelligence (AI) to create a comprehensive disease model to advance research into ALS.

GNS Healthcare will use its powerful machine learning platform, REFS, in conjunction with the rich Answer ALS patient datasets, which are accessible to clinicians and scientists throughout the ALS research community. The project will be led by Dr. Iya Khalil, chief commercial officer and co-founder of GNS Healthcare. The ALS Association’s partnership with GNS Healthcare will transform Answer ALS’s petabytes of patient data into mechanistic models, connecting genetic, molecular, and biochemical variables to clinical outcomes that will allow in silico experiments to be performed at a rapid rate on the computer. These rapid, high-throughput computational experiments will explore the numerous factors in the REFS Answer ALS data models that drive disease progression and drug response. Discoveries will then be evaluated and validated with wet lab experiments and, eventually, clinical studies. [Funding announced July 2018.](#)

**ALS REPRODUCIBLE ANTIBODY PLATFORM (ALS RAP)**

The ALS Association contributed $200,000 in partnership with the Motor Neurone Disease Association and the ALS Society of Canada for a total contribution of $600,000 to support ALS-RAP.

ALS-RAP was created as a public-private partnership comprised of world experts in antibody generation and validation, including Structural Genomics Consortium (SGC) and its associated labs at the Montreal Neurological Institute (MNI) McGill University in Montreal (Canada), the University of Oxford (UK), and the Karolinska Institute (Sweden). The funding will support the creation of an open-access pipeline to validate antibody research and provide the ALS research community with the highest quality reliable, renewable antibodies for ALS genes. Standard operating procedures will be established to characterize ALS antibodies – both commercially available and newly created – to ensure these exceed SGC and the Neuro’s stringent quality criteria to establish a public list of “gold-standard” antibodies that will ultimately lay a solid foundation for successful ALS therapies. [Funding announced in March 2018.](#)
The ALS Association’s TREAT ALS™ Global Research Program

108 Active Research Projects

In an effort to accelerate progress toward finding treatments and a cure for ALS, The ALS Association is currently funding 108 active research projects all over the globe.

Active Projects by Location

- Arizona 1
- California 6
- Connecticut 1
- D.C. 1
- Florida 11
- Georgia 1
- Illinois 2
- Massachusetts 24
- Maryland 8
- Michigan 2
- Minnesota 1
- Missouri 2
- North Carolina 2
- New Hampshire 1
- New York 10
- Ohio 1
- Oregon 1
- Pennsylvania 3
- Rhode Island 1
- Tennessee 3
- Texas 3
- Virginia 1
- Wisconsin 2
- Belgium 1
- Canada 3
- Chile 2
- China 1
- Finland 1
- Germany 1
- Ireland 1
- Italy 1
- Malaysia 1
- South Africa 1
- Switzerland 1
- United Kingdom 5
We support a wide breadth of scientific focus areas – each is critical to advancing ALS research.

**Harnessing Innovative Ideas:**
Basic research at the lab bench to find therapeutic targets

**Translating Concepts to Therapies:**
Drug development and biomarker discovery

**Advancing Treatments to Patients:**
Clinical trials

**Championing Patient Care Through Research:**
Research focused on improving lives more immediately, such as assistive technology, and improving patient and caregiver care

**Thirteen Scientific Focus Areas**
- Disease Mechanisms
- Environmental Factors/Epidemiology
- Disease Models
- Genetics
- Cognitive Studies
- Natural History Studies
- Assistive Technology
- Clinical Studies
- Stem Cells
- Precision Medicine
- Biomarkers
- Drug Development
- Nanotechnology

For more information, visit:
www.alsa.org/research/our-approach
www.alsa.org/research/focus-areas
The ALS Association’s collaborative and global approach to funding research continues to lead to significant advances by top ALS researchers all over the world.

**THE WORLD IS OUR LAB**

We fund novel, promising research around the globe covering all scientific focus areas, spurring innovation along the way.

- **Harnessing Innovative Ideas**
- **Translating Concepts to Therapies**
- **Advancing Treatments to Patients**

**TREATMENTS & CURES**

- **AWARDED NEW GLOBAL RESEARCH PROJECTS IN 13 COUNTRIES**: 61+
- **2018 research budget**: $18 MILLION
  - The ALS Association is the highest nonprofit research funder in the U.S.
- **11 global strategic initiatives**
- **1 Clinician Scientist Funded in 2018**
- **5 new Postdoctoral Fellows Funded in 2018**
- **5 NEW GENES**
  - identified since the ALS Ice Bucket Challenge to develop new therapies
- **62+ actively recruiting ALS clinical trials**
- **$89+ MILLION**
  - dedicated to research since the ALS IBC to advance treatments and a cure
- **two potential new antisense drugs and numerous other drugs on the horizon aimed to slow or stop the progression of ALS**

Spring 2019
Our program is far-reaching, innovative, collaborative, and powerful.

**INSPIRING PARTNERSHIPS**

Collaboration is the cornerstone of our research program. We partner with academia, industry, government, and other nonprofit organizations.

**Impact:** We lead by spurring long-lasting collaborations among researchers across all sectors, leading to globally-shared data, protocols, and research samples to accelerate research progress.

**ATTRACTING YOUNG, BRIGHT SCIENTISTS**

We encourage young scientists to enter and remain in ALS research and are dedicated to their continued success.

**Impact:** Over 76 percent of our postdocs remain in ALS research to start their own labs and mentor more young researchers. The other 14 percent hold jobs in industry, non-profit, and medical writing.

**INVESTING IN CLINICAL TRIALS**

We sponsor ALS clinical trials to accelerate drugs through the drug pipeline as quickly as possible.

**Impact:** We have funded numerous clinical trials over the years and have helped countless drugs move from ideas into trials. Cedars-Sinai’s combined stem cell-gene therapy trial is just one example.

**MAXIMIZING INVESTMENTS**

We secure matching gifts to significantly increase donor investment from the ALS IBC and beyond.

**Impact:** Our original $1.5 million investment in antisense technology infused over $100 million and one FDA-approved drug for spinal muscular atrophy, SPINRAZA™, and two potential new ALS drugs targeting SOD1 and C9orf72.

**WORKING WITH TOP ALS EXPERTS**

We collaborate with top ALS scientists, clinicians, consultants, entrepreneurs, and executives to create and lead an exceptional research program.

**Impact:** Our highly competitive research program funds the most ALS research dollars of any ALS nonprofit, with an $18 million research budget in 2018, and is held to rigorous standards to drive innovation.

**CHAMPIONING PEOPLE LIVING WITH ALS**

People living with ALS are at the center of everything we do and must receive the best care and support possible.

**Impact:** In 2018, we funded three clinical management projects focused on improving care for people living with ALS and their families.
Thanks to our generous donors, The ALS Association awards various research projects throughout the year as part of its competitive Translational Research for ALS (TREAT ALS™) Portfolio, which include the following:

- **Multiyear Investigator-Initiated Grants** to established investigators.

- **One-year Starter Grants** to investigators new to the ALS field or senior postdoctoral fellows establishing their own independent position.

- **Milton Safenowitz Postdoctoral Fellowships** to encourage and facilitate promising young scientists to enter the ALS field. Fellows work with a senior mentor and receive extensive exposure to the ALS research community through meetings and presentations.

- **Strategic Challenges** are crowdsourcing initiatives such as the ALS Assistive Technology Challenge to help people living with ALS communicate with ease (partnered with Prize4Life) and the TDP43 PET Tracer Grand Challenge to discover a biomarker to track TDP43 in the body (partnered with ALS Finding a Cure®).

- **Strategic Initiatives** that invite researchers to submit collaborative projects that address research gaps, areas of high risk-high reward, and/or areas that provide novel opportunities. This grant program encompasses large, collaborative research programs. For more information, visit the strategic initiative page and refer to the strategic initiative talking points.

- **Clinical Development Fellowships**, in partnership with the American Academy of Neurology (AAN), to support ALS clinician-researchers focused on projects involving people living with ALS.

- **Lawrence and Isabel Barnett Drug Development Program** fosters collaborations with companies/academia to fund milestone-driven research focused on preclinical studies to move treatment approaches closer to the clinic. Funding specifications and project criteria vary for each specific request for proposals.

- **Pilot Clinical Trials** to support up to and including phase II clinical trials that are associated with a comprehensive biomarker program to test novel, high-potential treatment approaches in people with ALS.

- **Clinical Management Awards** to fund research for improving clinical, psychological, and social management of ALS, focusing on both people living with ALS and their caregivers.

For questions, please contact Dr. Neil Thakur, EVP Mission Strategy, at nthakur@alsa-national.org.
The ALS Association Provides Funding at Every Stage of the Research Pipeline

DEVELOPMENT
Biomarkers are any measurable substance that changes in quantity, either appearing or disappearing over time, with a change in the body’s state. Examples are a chemical change in your blood, urine or cerebral spinal fluid, and structural change or chemical change in your brain. They are used to diagnose diseases and track effectiveness of potential treatments. Currently, there are no approved biomarkers for ALS.

**IMPACT ON ALS**

Today, researchers rely on clinical trial outcome measures such as the ALS Functional Rating Scale – Revised (ALSFRS-R), forced vital capacity (FVC), and others. Once developed, their potential is immense. Right now, the average time to ALS diagnosis is one year. We need to do better. Biomarkers will make ALS diagnosis faster and more accurate. They will also allow physicians the ability to track the disease in real time as ALS progresses in a patient, allowing for more proactive and targeted care. Clinical trials will be more easily stratified, allowing clinicians to test specific populations of people that have a high potential for the therapy to be effective. Biomarkers will also be used to track a person’s response to therapy. It will show whether a drug is hitting its target in the central nervous system and is working properly. Biomarkers will accelerate drug development of new treatment for ALS by making the clinical trials more efficient. This, in turn, increases a potential therapy’s value to pharmaceutical companies, as it will be readily apparent if the therapy is working as designed.

"The ALS Association is committed to supporting biomarker discovery. Identifying biomarkers is an extremely important step in the drug development pipeline to accelerate the discovery of novel treatments and a cure for ALS. We support a number of exciting biomarker research studies in addition to the TDP-43 Biomarker Grand Challenge, all aimed at pioneering and moving the ALS biomarker field rapidly forward."

– Dr. Neil Thakur, EVP Mission Strategy at The ALS Association

Biomarker Funding Opportunities

Developing a Faster, More Accurate Diagnosis

Customizable by Chapter
TDP-43 protein is present in aggregates (large clumps of protein) that are found in the brain and spinal cord of people with ALS, and other neurodegenerative diseases, such as Alzheimer’s disease and Frontotemporal dementia. Dr. Miller’s team is developing a positron emission tomography (PET) biomarker that is a fluorescent tracer that attaches to TDP-43 protein aggregates and can be observed in real time by PET imaging. The goal is for the tracer to not only contribute to the basic scientific understanding of TDP-43 disease processes, but also to ALS drug development targeting TDP-43 aggregates. Outcomes for drug development will not only impact ALS, but also could apply to other neurodegenerative diseases where TDP-43 aggregates are also observed.
Stem cells have the ability to divide for indefinite periods in a dish, providing an unlimited supply of cells to study. They can give rise to any specialized cell type in the body, including motor neurons and support neurons called glia, which are both lost in ALS. There are different types of stem cells, such as induced pluripotent stem cells (iPSCs), which are typically created from adult skin cells or blood. When derived from a person living with ALS, iPSCs are transformed into motor neurons, exactly reflecting the person’s genetic makeup – like an avatar in a dish.

**IMPACT ON ALS**

iPSCs have emerged as the most significant source of stem cells for ALS research and are important sources of neurons to model the disease in a dish. They have the potential to identify new disease pathways and individual susceptibilities to disease that cannot be revealed with other models. They serve as exceptionally valuable tools to find new treatments based on a person’s unique genetic makeup. Neurons derived from iPSCs can be tagged with fluorescent markers to allow tracking of individual neurons over time. This allows researchers to conduct drug screens to find compounds that improve the health of neurons, identifying a potential therapy. Motor neurons derived from iPSCs are even being used in parallel to people living with ALS (from which the cells are derived) during clinical trials to help predict whether a trial drug will positively impact the health of motor neurons.

“iPSCs have emerged as exceptionally valuable tools for modeling disease, screening for new therapies, and finding new treatments based on a person’s unique genetic makeup.”

– Dr. Neil Thakur, EVP Mission Strategy at The ALS Association
**Principal Investigator:** Dr. Clive Svendsen, Cedars-Sinai, Los Angeles, Calif.

**Funding Award:** FILL IN

“Funding from The ALS Association has allowed us to generate a set of valuable ALS iPSC lines and develop robust protocols for making motor neurons from them. As they are the cells that die in ALS, they should help us understand the underlying mechanisms of disease.” – Dr. Clive Svendsen

The Neuro Collaborative is a synergistic research model based in three laboratories in California that are working in collaboration to bring together complementary expertise to advance the understanding of ALS and to develop and expedite ALS therapeutic approaches. One arm of the Collaborative is Dr. Svendsen’s laboratory, which focuses on the establishment of a stem cell and motor neuron core facility to create and store clinical-grade iPSCs, which will be shared openly with the ALS research community, including with large collaborative initiatives such as Answer ALS, the Northeast ALS Consortium (NEALS), the National Institutes of Health (NIH), and the California Institute for Regenerative Medicine (CIRM), among others. The team is developing enhanced, standardized techniques that turn iPSCs into a range of motor neurons and genetically modify them to light up fluorescently to allow researchers to view and track individual motor neuron health over time. The iPSCs will be used for discovering the causes of ALS, developing new drugs through drug screening using a patient’s own motor neurons, and creating clinical-grade lines of iPSCs to be used in cutting-edge stem cell therapy trials that are underway at Cedars-Sinai and other institutions.
Researchers have demonstrated that 10 percent of ALS cases are familial, meaning the disease gene is inherited. The other 90 percent of ALS cases are sporadic, meaning they do not know the underlying cause. It is likely that a percentage of the sporadic cases are familial, but those genes are yet to be uncovered. In recent years, there has been a large boom in genome sequencing (where all of a person’s DNA is sequenced) due to decreased cost (approximately $2,000 per genome) and advances in sequencing technology. Currently, more than 30 ALS genes have been identified, and counting. The ALS Association supports big initiatives all over the world that are working toward closing the genetic gap to identify all possible ALS genes.

**IMPACT ON ALS**

Gene discovery represents opportunities for new therapeutic targets, thereby increasing the number of potential ALS therapies. Importantly, insights gained from studying genetic forms of ALS are likely to benefit those with sporadic ALS. For example, new model organisms based on newly identified genes are developed to better understand and discover novel disease pathways – information that can be tested and possibly applied to all ALS cases. These new genetic discoveries allow scientists to study disease in ways that would otherwise not have been possible. In addition, identified genetic mutations can be corrected using cutting-edge gene therapy that aims to slow or stop the progression of ALS. Antisense technology designed to correct the two most commonly inherited genes – SOD1 and C9orf72 – is in clinical and preclinical trial phases, respectively. New gene editing technology, such as CRISPR, may also add value in the ALS treatment landscape.

“The ALS Association is committed to supporting genome sequencing and the next frontier of gene discovery. The more genes we uncover, the more potential therapeutic targets we will have, leading us closer to our goal – to discover effective treatments and a cure for this devastating disease.”

– Dr. Neil Thakur, EVP Mission Strategy at The ALS Association
Project MinE is an international, large-scale research initiative dedicated to discovering genetic causes of ALS and to ultimately finding a cure. It was founded by Bernard Muller and Robbert Jan Stuit, both entrepreneurs and people living with ALS, who teamed up to change the genetic landscape of ALS. The ALS Association initially committed $1 million to fund the U.S. arm of this global initiative, which now includes 17 countries. Project MinE’s activities are rooted in the theory that genes are thought to contribute, directly or indirectly, to most cases of ALS. The goal is to sequence 15,000 people with ALS and 7,500 healthy people for a total of 22,500 genomes. Already, Project MinE has identified three new ALS genes – KIF5A, NEK1, and C21orf2 – discoveries that were published in in top scientific journals. For example, the KIF5A discovery involved a collaborative effort of 250 researchers that included Drs. Landers and Glass in the U.S. As of February 2019, Project MinE has achieved 47 percent of its goal, sequencing a total of 10,555 genomes and counting. There is still much more left to be done! Sequencing just one person’s genome costs approximately $2,000.

For more information on Project MinE, click [here](#).
For more information on Landers’s project, click [here](#).
For more information on genetics, click [here](#).
2018 ADVANCES IN ALS RESEARCH

A significant number of research innovations, advancements in drug development and clinical trials, new collaborations, and more, all aimed at finding treatments and a cure for ALS, accelerated the pace of discovery this year.

We’re excited to share some of 2018’s most newsworthy research headlines that gave us and people living with ALS hope this year! The ALS Association is proud to provide funding for these exciting innovations.

**Antisense Drugs Achieved Significant Progress**

- Biogen announced encouraging results of SOD1 (BIIB067) phase I trial and is moving to the next clinical phase with urgency.
- Biogen also initiated the C9orf72 (BIIB078) phase I trial this fall and the first patients were dosed.

**KIF5A ALS Gene Discovered**

A large collaborative effort involving over 250 researchers, led by Dr. John Landers (UMass Medical School) and Dr. Bryan Traynor (NIH), came together to achieve this exciting gene discovery, including organizations supported by the Association - Project MinE, Genomic Translation for ALS Care, Answer ALS, the Target ALS Postmortem Tissue Core, the New York Genome Center (NYGC) ALS Consortium, the CREAtE Consortium, the NIH, and others.

**Assistive Technology Advanced at Rapid Pace**

- Pison Technology, winner of our ALS Assistive Technology Challenge, is growing. Our $100,000 investment leveraged over $1.7 million in outside funding and their wearable muscle sensor technology is moving fast toward the market.
- Project Revoice was launched to help encourage people with ALS to voice bank and make it easier to use their synthesized voice.
- Telemedicine is on the rise to help people with ALS connect with their care teams, especially in rural areas, through the hard work of our chapter network and funded researchers.

**Drug Development Moved Compounds Towards the Clinic**

- A viral gene therapy targeting the ALS SOD1 gene was safe and effective in nonhuman primates, which paved the way for a human clinical trial.
- Anelixis Therapeutics moved its compound AT-1501 into clinical trials and dosed its first patient.
- Karyopharm Therapeutics announced that Biogen acquired compound KPT-350, with plans to advance it into clinical trials in the near future.

**FDA Drug Approved for ALS**

ITF Pharma, Inc. announced that Tiglutik*, the first and only thickened liquid form of riluzole, was approved by the FDA to treat ALS. This formulation should help individuals with swallowing difficulties.

*Not funded by The ALS Association

**Research Aimed at Helping People with ALS**

- Respiratory strength training showed safety and feasibility in an ALS case study and moves forward with hope to strengthen inspiratory and expiratory muscle function.
- Research to improve noninvasive ventilation for people with ALS is underway through the use of custom-printed 3D masks.

**State-of-the-Art Technology Reached Exciting Research Milestones**

- An organ-on-a-chip was developed to recreate human biology (blood vessels connected to motor neurons) on a microchip to uncover novel ALS biomarkers and therapeutic targets.
- Artificial intelligence was used to microscopically examine and analyze cells involved in ALS to better understand their disease characteristics.
- Specially engineered cells transplanted into the brain’s motor cortex protected motor neurons and delayed disease in animal models.
The ALS Association is committed to biomarker discovery, as their potential is immense. Identifying biomarkers is vital to improving diagnosis, following disease progression, tracking response to therapy, and make clinical trials more efficient. Our support of the TDP-43 Biomarker Grand Challenge Program is just one example.

ASSISTIVE TECHNOLOGY
The ALS Association is working to develop accessible, portable devices for people living with ALS, in order to help them maintain a high quality of life. The ALS Assistive Technology Challenge winners we announced in December 2016 are dedicated to achieving this!

ENVIRONMENTAL FACTORS
Multiple factors in one’s lifestyle and surroundings, such as smoking and military service, are the only known ALS risk factors. The ALS Association champions multiple efforts to better understand these risk factors and drive discovery of other factors that may contribute to ALS.

NATURAL HISTORY STUDIES
These studies are important to understanding the natural disease course of familial (inherited) ALS. The ALS Association is supporting several natural history studies of SOD1 and C9orf72 ALS, which are critical to helping inform patient care and clinical testing of new treatment approaches.

CLINICAL STUDIES
The ALS Association supports clinical management grants to improve the lives of people living with ALS and their caregivers, along with clinical trials to accelerate treatments through the drug development pipeline.

COGNITIVE STUDIES
There is a great deal of evidence that cognitive impairment is connected to ALS, such as overlap with Frontotemporal dementia (FTD). The ALS Association is committed to improving understanding of why and how this connection takes place.
GENETICS
The number of genes identified to cause familial ALS has multiplied since the discovery of SOD1. Many efforts are underway to identify more ALS genes and target them for therapy. The ALS Association continues to make significant investments in identifying new genes and has supported all the major ALS gene discoveries in history.

DISEASE MECHANISMS
ALS is a complicated disease involving multiple disease pathways. The ALS Association encourages research to discover novel pathways. Understanding how ALS disease works on many biological levels is necessary to identify potential therapeutic targets.

NANOTECHNOLOGY
There is growing interest in using nanotechnology as a delivery tool for ALS therapeutics, and we are on the cutting edge, funding this exciting technology.

DRUG DEVELOPMENT
The ALS Association is supporting development of several different treatment approaches, including small molecules, stem cells, and gene therapy. Our early support of antisense drugs in 2004 has paid off! Antisense therapies have already proven effective in spinal muscular atrophy (SMA), and are in trial targeting both SOD1 and C9orf72.

STEM CELLS
Stem cell technology is progressing rapidly, and The ALS Association is spearheading work on several critical fronts to advance this key research tool.

DISEASE MODELS
The ALS Association’s research portfolio supports a variety of model systems used for understanding disease pathways and testing promising compounds.

PRECISION MEDICINE
The ALS Association has helped establish and currently supports several partnerships and precision medicine programs to aid in the identification of new disease genes and targets for drug therapy.

Learn more on the Scientific Focus Area Page.
A
agonist
A drug that increases neurotransmitter activity by directly stimulating the nerve cell receptors.

ALS Functional Rating Scale – Revised (ALSFRS-R)
A survey of questions that assesses the impact of ALS on activities of daily living. It is often used as a primary outcome measure of ALS clinical trials.

amino acid
One of the 20 building blocks of protein.

antibody
A defense protein that binds to foreign molecules to allow their elimination.

antigen
A substance that is capable of causing the production of antibodies. Antigens may or may not lead to an allergic reaction.

antioxidant
A chemical compound or substance that inhibits oxidation.

assay
An investigative procedure (i.e., experiment) in the laboratory.

ataxia
Loss of balance.

atrophy
The progressive loss of muscle mass, or wasting, caused by reduction in the size or number of muscle cells. It is one of the later symptoms of ALS.

axon
The long, hairlike extension of a nerve cell that carries a message to the next nerve cell.

B
blood-brain barrier (BBB)
A protective barrier formed by the blood vessels and glia of the brain. It prevents some substances in the blood from entering brain tissue.

bradykinesia
Slowness of movement.

bulbar muscles
The muscles that control speech, chewing, and swallowing.

C
central nervous system (CNS)
The brain and spinal cord combined.

cerebrospinal fluid (CSF)
A clear fluid that covers and protects the brain and spinal cord.

chromosome
A visible carrier of the genetic information.

corticospinal tract
The bundle of nerves that reach from the motor area of the brain (see cortex) to the spinal cord, connecting to the nerves that go out to control the muscles.

CRISPR/Cas9
Genome editing technology that allows the permanent modification of genes within an organism. By delivering the Cas9 nuclease bound to a synthetic guide RNA into a cell, the cell's genome can be cut at the designed/desired location. This allows existing genes to either be removed or added in. CRISPR stands for Clustered Regulatory Interspaced Short Palindromic Repeats.
DNA
Deoxyribonucleic acid. Hereditary material that encodes genetic information.

dysarthria
Impaired speech and language due to weakness or stiffness in the muscles used for speaking.

dyskinesia
Abnormality or impairment of voluntary movement.

dysregulation; dysregulate
An impairment of a physiological regulatory mechanism; to cause a dysfunctional level of an activity or chemical in an organism by disrupting normal function.

dysphagia
Difficulty in swallowing.

dystonia
A slow movement or extended spasm in a group of muscles.

Electroencephalogram (EEG)
A method of recording the brain’s continuous electrical activity by means of electrodes attached to the scalp.

Embryonic stem cells
Embryonic stem cells are the “blank slates” of an organism, capable of developing into all types of tissue in the body.

Enzyme
A protein that acts as a catalyst in mediating and speeding a specific chemical reaction.

Excitotoxic
An agent that excites neurons which can, over time, lead to neuronal death.

Fasciculation
Small, involuntary, irregular, visible contractions of individual muscle fibers. Often seen in the legs, arms, and shoulders of persons with ALS. This is often described by people with ALS as “persistent rolling beneath the skin.”

Forced Vital Capacity (FVC)
The amount of air that can be forcibly exhaled from the lungs after taking the deepest breath possible. It is measured by a test called spirometry, a type of pulmonary function test. The percent force vital capacity is often used as criteria to participate in an ALS clinical trial.

Free radicals
Chemicals that are highly reactive and can oxidize other molecules (i.e., superoxide).

Gene
Genes are the basic biological units of heredity. They are composed of DNA.

Genome
All of the genetic information; all of the hereditary material possessed by an organism.

Genotype
The genetic makeup (i.e., DNA code) of an individual.

Glutamate
Glutamate is one of the most common amino acids found in nature. It is the main component of many proteins, and is present in most tissues. Glutamate is also produced in the body and plays an essential role in human metabolism. It is a primary excitatory neurotransmitter in the human CNS.
**H**

**hyperreflexia**
Excessive response of muscle reflexes when a normal stimulus is applied.

**hyporeflexia**
Weak or absent muscle response when a normal stimulus is applied.

**I**

**immune system**
A complex system that is responsible for distinguishing us from everything foreign to us and for protecting us against infections and foreign substances. The immune system works to seek and kill invaders.

**incidence**
The occurrence of new cases of a condition. The incidence rate describes the frequency with which cases are identified. Incidence is commonly measured in new cases per 1,000 (or 100,000) of population at risk per year.

**induced pluripotent stem cells (iPSCs)**
A type of pluripotent stem cell that can be generated directly from adult cells.

**inflammation**
The nonspecific immune response that occurs in reaction to any type of bodily injury. It is a stereotyped response that is identical whether the injurious agent is a pathogenic organism, foreign body, ischemia, physical trauma, ionizing radiation, electrical energy or extremes of temperature.

**inflammatory disease**
A disease that is characterized by activation of the immune system to abnormal levels that lead to disease.

**intrathecal**
Injection into the innermost membrane surrounding the central nervous system. Usually done by lumbar puncture.

**interventional trial**
Type of trial or clinical research study in which exposure to a potential therapy or drug is assigned and being tested. It is used to determine the effectiveness and safety of a potential treatment.

**investigator**
A person who carries out a scientific study. A researcher.

**in vitro**
In an artificial environment outside the living organism, such as in a dish or test tube in the laboratory.

**in vivo**
In a living organism, such as a mouse or human.

**L**

**lower motor neurons**
Nerve cells (motor neurons) originating in the spinal cord that connect to muscles, conduct signals to allow muscle movement.

**M**

**molecule**
The smallest unit of a substance that can exist alone and retain the character of that substance.

**motor neuron**
A neuron that conveys impulses initiating muscle contraction or glandular secretion.

**motor neuron disease (MND)**
A group of disorders in which motor nerve cells (neurons) in the spinal cord and brain stem deteriorate and die. ALS is the most common motor neuron disease.

**muscle atrophy**
Loss of muscle fiber volume characterized by a visible decrease in muscle size. This occurs because muscles no longer receive impulses or signals from nerve cells.

**mutation**
A permanent change, a structural alteration, in the DNA or RNA. Mutations can be caused by many factors, including environmental insults such as radiation and mutagenic chemicals. Mutations are sometimes attributed to random chance events.
**myelin**
A fatty substance that surrounds and insulates the axon of some nerve cells to help speed nerve transmission. It is important for proper function of the nervous system.

**nerves**
Bundles of fibers that use electrical and chemical signals to transmit sensory and motor information from one body part to another.

**nervous system**
The system of cells, tissues, and organs that regulates the body’s responses to internal and external stimuli. In vertebrates it consists of the brain, spinal cord, nerves, ganglia, and parts of the receptor and effector organs.

**neuron**
Neurons are the nerve cells which make up the central nervous system. They consist of a nucleus, a single axon which conveys electrical signals to other neurons and a host of dendrites which deliver incoming signals.

**neurodegenerative**
The progressive loss of the structure and function of the nervous system, especially neurons.

**neuroprotective**
If an agent provides protection to any part of the body’s nervous system, it is said to provide neuroprotection.

**neurotransmitters**
Chemical substances that carry impulses from one nerve cell to another; found in the space (synapse) that separates the transmitting neuron’s terminal (axon) from the receiving neuron’s terminal (dendrite).

**observational study**
Type of trial in which enrolled participants are observed. Outcome measures (i.e. measures of strength or function) may be part of the observation. No treatment/drug is given. It is often used to learn about trends of symptoms, the course of disease, and can include biomarker studies.

**oxidative stress**
Accumulation of destructive molecules called free radicals can lead to motor neuron death. Free radicals damage components of the cells’ membranes, proteins or genetic material by “oxidizing” them – the same chemical reaction that causes iron to rust.

**phenotype**
The observable characteristics of an individual resulting from the expression of genes. This may be directly observable (eye color) or apparent only with specific tests (blood type). Some phenotypes, such as the blood groups, are completely determined by heredity, while others are readily altered by environmental agents.

**pluripotent stem cells**
Human pluripotent stem cells are a unique scientific and medical resource. They can develop into most of the specialized cells and tissues of the body, such as muscle cells, nerve cells, liver cells, and blood cells. They are self-renewing, making them readily available for research and, potentially, for treatment purposes. Scientists derive these unique cells from human embryos, from fetal tissue, or from adult tissue (in the case of induced pluripotent stem cells (iPSCs)).
positron emission tomography (PET) scan
A computer-based imaging technique that provides a picture of the brain’s activity rather than its structure. The technique detects levels of injected glucose labeled with a radioactive tracer.

potassium channel
A type of ion channel that forms potassium-selective pores that span the cell membrane, thereby helping transport potassium across the cell membrane. They are found in most cell types and control a variety of cell functions.

precision medicine
A tailoring of medical treatment to the individual characteristics of each person, while taking into account individual variability in genes, environment, and lifestyle for each person. In precision medicine programs, researchers aim to learn as much as possible from each unique person living with ALS.

protein
Proteins are large molecules required for the structure, function, and regulation of the body’s cells, tissues, and organs. Each protein has unique functions. Proteins are essential components of muscles, skin, bones, and the body as a whole. Protein is also one of the three types of nutrients used as energy sources by the body.

proteomics
The study and identification of the proteins produced by the genetic instructions carried by a cell.

protocol
A precise and detailed plan for the study of a biomedical problem or for a regimen of an experimental therapy.

qualitative
Relating to measuring or measurement of the quality of something, such as its size, appearance, etc.

quantitative
Relating to measuring or measurement of the quantity (amount) of something.

RNA
Ribonucleic acid. The primary function of RNA is to act as a messenger carrying instructions from DNA for controlling protein synthesis within a cell.

sclerosis
A hardening within the nervous system, especially of the brain and spinal cord, resulting from degeneration of nervous elements such as the myelin sheath.

sialorrhea
Drooling.

spinal cord
Part of the central nervous system extending from the base of the skull from the brain stem through the vertebrae of the spinal column. It carries information from the body’s nerves to the brain and signals from the brain to the body.

stem cells
Cells that can differentiate into many different cell types when subjected to the right biochemical signals. Stem cells are a promising new therapeutic approach to treating central nervous system disorders. The most versatile stem cells, called pluripotent stem cells, are present in the first days after an egg is fertilized by sperm. Researchers believe they can coax stem cells to become whatever tissues patients need. Stem cells come from embryos, bone marrow, and umbilical cords. View the stem cell glossary to learn more.

stratify
To arrange or classify.

superoxide dismutase
An enzyme that destroys superoxide, which is a highly reactive form of oxygen. With ALS, 20 percent of the total population of patients have mutations in the gene for copper/zinc superoxide dismutase type SOD1. SOD1 normally breaks down free radicals, but mutant SOD1 is unable to perform this function.

synapse
1.3.7
A tiny gap between the ends of nerve fibers across which nerve impulses pass from one neuron to another; at the synapse, an impulse causes the release of a neurotransmitter, which diffuses across the gap and triggers an electrical impulse in the next neuron.  

**synergistic**  
Interaction or cooperation between two or more substances or organizations to produce a greater combined effect. 

**T**  
**toxicity**  
The extent, quality or degree of being poisonous.  

**transgenic**  
An organism whose sperm or egg contains genetic material originally derived from an organism other than the parents or in addition to the parental genetic material.  

**translational research**  
Studies that apply findings from basic science discovered in the lab to relevant disease therapies that enhance patient well-being.  

**trophic factor**  
One of a class of proteins that help keep cells healthy.  

**U**  
**upper motor neurons**  
Nerve cells (motor neurons) originating in the brain’s motor cortex and running through the spinal cord.  

**V**  
**vector**  
The agent used (by researchers) to carry new genes into cells. Plasmids currently are the vectors of choice, though viruses and other bacteria are increasingly being used for this purpose.  

For more ALS vocabulary, visit The ALS Association online, found at:  
http://www.alsa.org/research/our-approach/glossary.html
Neil Thakur became Executive Vice President of Mission Strategy in May 2018. He leads the advocacy, care services, and research programs for the Association.

Prior to joining the Association, Neil served in the National Institutes of Health (NIH) Office of the Director, where he supported NIH governance and helped make NIH research more open and less burdensome. He managed the world’s largest policy to make biomedical research papers publicly accessible and co-chaired the White House taskforce that lead to the requirement that all federal science agencies adopt similar policies. He also spent a year on detail to the U.S. Senate Special Committee on Aging, raising awareness about quality issues in long-term health care, particularly around Alzheimer’s care and pharmaceuticals.

Prior to his time at NIH, Neil worked with health systems in many capacities. He was Assistant Director of Health Services Research and Development at the Department of Veterans Affairs (VA), leading an evaluation service for the VA health system and represented the VA research service in setting clinical performance measures. In his post-doctoral fellowship, he studied the interactions between jails, Medicaid and behavioral health care, and how changes in health financing impacted people’s utilization of these systems. During graduate school, he worked throughout the Connecticut behavioral health system, helping to implement managed care and health information systems, and raise tens of millions of dollars in competitive grants.

Neil won many awards for his government service, including several NIH Director’s Awards, and the Secretary for Health and Human Services’ award for Meritorious Service, the second highest award that the Secretary can bestow. He holds a Ph.D. in Health Policy from Yale University School of Public Health and completed a NIMH postdoctoral fellowship in mental health services research at the Cecil G. Sheps Center for Health Services Research at the University of North Carolina at Chapel Hill. He lives in Maryland with his wife, Jen.
Dr. Jill Yersak, Director, Mission Strategy, joined The ALS Association in 2015. Reporting to Neil Thakur, she helps oversee the strategy of projects and programs in the Association’s mission – research, care, and advocacy. She is also responsible for communicating ALS research in an accessible way by developing and maintaining research information tailored to people living with ALS, their caregivers, and loved ones. She supports both ALS Association National and chapters in all departments with research information needs, including donor outreach. As a part of the Research Communications team, she played a large part in implementing The ALS Association’s research website redesign and the mission toolkit.

Dr. Yersak received her bachelor’s degree in Biology at Ursinus College in Collegeville, Pa. After college, she served as a research technician at the Children’s Hospital of Philadelphia in the department of Human Genetics and Molecular Biology, focused on a pediatric genetic disease called 22q11.2 Deletion Syndrome. She then went onto complete her Ph.D. at Thomas Jefferson University in Philadelphia, with a focus on a neurodegenerative disease called Kennedy’s Disease. Dr. Yersak then moved to Providence, R.I. to complete her postdoctoral fellowship under the mentorship of Dr. Anne Hart in the Neuroscience department. During this time, she spearheaded a project to generate precise ALS C. elegans models (which are microscopic worms), co-wrote a successfully-funded ALS Association grant based on this project, and mentored several graduate and undergraduate students. Dr. Yersak then went on to work at her alma mater, Thomas Jefferson University, as coordinator of the Postbaccalaureate Pre-Professional Program in the Graduate School of Biomedical Sciences, where she helped manage the program, in addition to counseling students in medical school and career placement.

Dr. Yersak is dedicated to mentorship, outreach, and advocacy in her community. She served on the Board for the Association for Women in Science in Philadelphia, where she championed young women to join and gain success in the STEM (science, technology, engineering, and mathematics) fields. During her time as a postdoctoral fellow, Dr. Yersak volunteered at the local ALS Multidisciplinary Clinic where she worked closely with people living with ALS and their caregivers, along with the multidisciplinary team, and local R.I. chapter. There she launched a National ALS Registry Program in R.I., where she significantly increased Registry enrollment. In 2014, she received the ALS Leadership Award presented by The ALS Association R.I. Chapter for her service to the chapter to raise ALS awareness.
SUMMARY

The ALS Association empowers people affected by ALS by supporting increased access to clinical care and support services on a nationwide basis. This includes a network of chapter-based professionals delivering a robust portfolio of care and support programs designed to enhance quality of life, a suite of multidisciplinary clinical programs where diagnosed persons can access expert clinical care, and comprehensive community and professional educational programs offering tools that support families, health care practitioners, and volunteers in the delivery of care and support.

OUR HISTORY

From its inception, The ALS Association has been the only nationwide nonprofit organization fighting ALS on every front, delivering a consistent set of core programs and services to families living with ALS. The early years focused on in-person volunteer information and support services. The portfolio of services has seen significant growth in recent years, including the initiation of a formal multidisciplinary Clinical Program of Excellence. Chapters expanded information, education, and support services delivered by professionally-prepared staff with expertise in all phases of the ALS journey. Strategic outreach and collaboration with medical providers, allied health professionals, service support organizations, and vendors have resulted in The ALS Association becoming the leading organization in program delivery.

OUR APPROACH TO CARE SERVICES

An ALS diagnosis impacts the individual diagnosed, as well as their family and loved ones. Thus, the Association’s core care services and programs have been developed to specifically address common needs in the ALS community.

• Access to consistent care, treatment, and services
• Resource and support services that enhance quality of life
• Quality care and support leveraged by collaboration with partners
As a result of our commitment to clinical care and education, families get the information they need to develop a care plan that will result in enhanced quality of life and optimal personal and family outcomes. Surveys, focused conversations, and advisory groups — which include diagnosed persons, caregivers, families, and care teams — help the Association identify unmet needs. Regular analysis of models of delivery, usage, and outcomes helps the Association refine its programs, services, and processes to identify priorities and maximize positive outcomes.

**HOW WE WORK**

The Association uses continuous feedback loops and quality improvement processes to actively assess the needs of the communities we serve in order to identify gaps in service delivery. This serves as the foundation for developing and delivering innovative services and to increasing access to care services.

- Designing, implementing, and monitoring certification and other clinical programs that support consistency and quality in services provided through Certified Treatment Centers of Excellence, Recognized Treatment Centers, affiliated providers, and other delivery models — all based on established best practices and standards of care,
- Building upon our educational resource libraries — including written publications, videos, web pages, and other media — for the provision of consistent information for our communities.
- Engaging in quality improvement reviews and training that supports an organizational culture that is attractive to a highly motivated and competent workforce serving our community, including professional and knowledge development, management, team building, professionalism, interpersonal communication, and work-life balance.
- Hosting a biennial National Clinical Conference to engage more than 420 health care professionals who work on ALS and other motor neuron diseases to discuss best practices and guidelines, multidisciplinary team care and coordination, ALS-relevant programs and services, research and technology updates, and other topics critical to the field.
- Hosting monthly educational webinars presented by experts for people living with ALS, their caregivers and family members, chapter staff, and professionals who work with people affected by ALS.
The Association continues to strengthen relationships internal to the Association, while acknowledging the interdependencies between chapter network, Care Services, Research, Public Policy, and national office support departments. The Association assumes a lead role in developing collaborative relationships and partnerships with the communities we serve, including medical providers, other like-minded organizations, researchers, policy makers, regulatory agencies, and others to strengthen services provided and initiatives undertaken.

The ALS Association supports a wide breadth of specific fields of study that are critical to advancing ALS research. Active participation of people living with ALS in these studies is paramount to their success and provides the foundation for continued work in areas that are of keen interest and show promising positive impact on people as they live with this disease.

The Association’s Clinical Management Grant Program supports clinical management studies in order to improve care for people with ALS. The program focuses on the full spectrum of clinical management, including gaps in the delivery of care, the development of telemedicine, assistive technology, and mental health care for people living with ALS and their caregivers.
CHAPTER MEMBERSHIP

PROGRAMS AND SERVICES OFFERED THROUGH OUR CHAPTER NETWORK
(SPECIFIC PROGRAMS MAY VARY BY CHAPTER)

Cumulative Number of People Registered with Each Chapter
INFORMATION AND RESOURCE REFERRALS

- Maintaining a physical, electronic, and video inventory of educational and informational materials related to ALS, including diagnosis, prognosis, treatments, research and clinical care options, common associated challenges, typically accessed equipment and services, insurance and benefit information, quality of life issues, and associated potential solution options.

- Offering a packet of information for people newly diagnosed, containing information critical to those newly diagnosed with ALS, including the value of multidisciplinary clinical care, the value of developing a strategic care plan, and the benefits available through accessing chapter programs and services.

- Producing a medical information packet to be given to first response professionals and medical providers in urgent and emergent medical situations.

- Providing referrals to local, regional, and national resources including appropriate medical care in the clinic, home, or hospice environment; access to medical equipment and health care services; insurance and benefit options; veteran’s services; and psychosocial services.

EDUCATION PROGRAMS

- Hosting programs that meet the various educational needs of stakeholders, including diagnosed persons, caregivers, families, medical professionals, and community health care providers.

- Providing in-service educational programming to community health care agencies or providers as part of a strategically planned professional outreach program, such as local home health or hospice organizations.

- Presenting relevant information regarding ALS and chapter resources to the general community as part of a strategically-planned community awareness program (for example, partnering with a local Rotary Club).

- Participating in state, regional, and national health care professional conferences as subject matter experts, or as an exhibitor or platform presenter (for example, at a State SLP Conference, Hospice Association, the Paralyzed Veterans of America Annual Summit, or an American Academy of Neurology Meeting).

CHAPTER CLINICAL LIAISON PROGRAMS

- Creating partnerships and coordinating care and services between The ALS Association chapter and local ALS Association Certified Treatment Centers of Excellence™, ALS Association Recognized Treatment Centers™, or Affiliated Clinics.

- Facilitating chapter care services staff attending ALS clinics to provide support services to patients, families, and the local ALS community.

- Providing financial or ‘in-kind’ support to ALS Association Certified Treatment Centers of Excellence™ and ALS Association Recognized Treatment Centers™.

- Cultivating relationships with community health care practitioners including speech language pathologists, physical therapists, occupational therapists, respiratory therapists, social workers, and home health, hospice, and other health care providers.
SUPPORT PROGRAMS

• Coordinating and facilitating support group meetings to provide education, information, and networking opportunities for diagnosed persons, caregivers, and families.

• Developing targeted support or program opportunities focused on specific needs of constituents, including caregivers, children, or homebound persons.

• Providing navigation assistance as diagnosed persons and their families transition from private insurance to public insurance programs, applying for disability benefits, or accessing other community, regional, and nationwide resources.

• Supporting grant funding to offset the cost of professional counseling services.

• Facilitating understanding of various care modalities, including treatment, palliative care, and hospice care.

• Supporting family end-of-life care plan with a focus on respecting care determination; personal, cultural, and religious values; and beliefs as practiced by individuals and families.

CAREGIVER PROGRAMS

• Providing expanded services to address unique needs of ALS family caregivers, including caregiver-only support or resource groups.

• Facilitating caregiver self-care and caregiver instructional education programs.

• Hosting caregiver acknowledgement or retreat programs.

• Supporting access to The ALS Association Care Connection Program, which utilizes an online calendar to coordinate family needs with available family, friend, or community volunteers.
• Supporting grant funding to offset the cost of caregiver respite services.
• Participating in National Family Caregiver Month activities.
• Offering a Bereavement Engagement Program, respectfully acknowledging a loss and reaching out to survivors on a time-specified basis to offer encouragement and support.

CARE MANAGEMENT GUIDANCE AND CONSULTATIONS
• Providing routine communication with diagnosed persons and families regarding progression-appropriate information, and anticipated challenges and needs.
• Providing guidance to support diagnosed persons and families in accessing health care services and making informed health care choices based on knowledge of ALS, health care resources, insurance, financial, personal, and community resources.
• Providing navigation assistance to families investigating options and applying for resource benefits.
• Providing appropriate referrals to professional service providers as appropriate.

DURABLE MEDICAL EQUIPMENT (DME) LOAN PROGRAMS
• Facilitating independence and quality of life for people living with ALS by providing information and education related to the benefits of durable medical equipment (DME).
• Providing appropriate referrals to qualified DME providers.
• Implementing a DME Loan Program consistent with national DME Loan program policies enabling persons with no or limited resources timely access to appropriate DME equipment on loan.
• Initiating and cultivating relationships with qualified DME providers interested in providing service excellence to the ALS community, including collaborating with an identified vendor to support operation of DME Loan Program.

ASSISTIVE TECHNOLOGY AND ALTERNATIVE AUGMENTED COMMUNICATION PROGRAMS (AT/AAC)
• Providing education and information related to how ALS impacts communication and what types of interventions are available to support augmented or alternative communication.
• Initiating and cultivating relationships with AAC equipment vendors and speech language pathologists to facilitate access to appropriate evaluation, and selection and training in the use of communication equipment.
• Implementing an AT/AAC Loan Program consistent with national AT/AAC Loan program policies enabling persons with no or limited resources timely access to appropriate DME equipment on loan, such as an iPad or SGD.
• Providing expanded program services including employing or contracting with a SLP or ATP to deliver comprehensive AT/AAC support services.
HOME VISIT PROGRAMS

• Providing personalized in-home or virtual home visits to consult with families in the comfort of their own homes regarding the disease, typical progression, anticipated common challenges, available resources, and guidance in developing a personalized strategic health care plan.

• Developing collaborative relationships with existing community health care home-visit providers, such as state or county health services, home health, Visiting Nurse Association, and hospice to maximize positive outcomes of service delivery.

GENERAL GRANT PROGRAMS

• Mitigating the enormous financial costs associated with addressing the challenges associated with maintaining quality of life during an ALS journey by providing grant funds to offset out-of-pocket expenses.

• Developing a general or specific grant funding program which may include the following eligible expenses: DME, AT/AAC, caregiver respite, transportation, home modification, medical or health care copays, or skilled or non-skilled home health care.

SPECIFIC GRANT PROGRAMS

• Cultivating relationships with qualified agencies dedicated to providing excellent in-home health services at a preferred contracted rate, including nursing, PT, OT, SLP, and CNA services. Support services via grant funding as available.

• Initiating and cultivating relationships with qualified transportation organizations able to provide appropriate transportation to clinics and to medical, chapter, and quality-of-life activities. Support services via grant funding as available.

• Initiating and cultivating relationships with organizations qualified to provide appropriate home modification, enabling diagnosed persons to more safely access and navigate their home environment. Support services via grant funding as available.
YOUTH PROGRAMMING

- Recognizing unique needs of ALS families that consist of children and youth and expanding other general services — including education, support groups, conferences, and retreats — and programs to include and address children and youth, subject to age-appropriate communications and activities.

- Providing targeted educational and informational projects for children and youth, such as backpacks, welcoming packets, children’s newsletter, family days, picnics, and youth camps.

- Collaborating with recognized children and youth content experts to develop youth-focused conferences or supported activities designed to provide age-appropriate information related to ALS progression and care, along with empowering networking opportunities, such as DME Youth Conference and ALS Youth Bowling Day.

VOLUNTEER PROGRAMS

- Developing a professionally-directed program that connects community volunteers to individual families living with ALS.

- Collaborating with existing community organizations to increase awareness of unmet volunteer needs of families experiencing a journey with ALS.

- Recruiting and training appropriate volunteers to provide direct, non-medical support for families.
HOW WE SUPPORT CLINICAL CARE

“There is a difference between curing and healing. Unfortunately, at this time, there isn’t a cure for ALS. We do, however, believe we can heal the patient. This can be done by addressing the fact that ALS is not just a physical disease, but an emotional, spiritual, and psychological disease as well. The multiple aspects of ALS are best addressed in the setting of a multidisciplinary clinic that acknowledges the unique challenges that ALS patients and their families face.”

– Scott D. Miller, MD, The ALS Association Recognized Treatment Center at Kaiser Permanente South Bay Medical Center

A person living with ALS and their loved ones face a variety of challenges along the journey. As the disease progresses and new symptoms and difficulties arise, the number of people involved in providing care increases as well. Clear communication among the care team and coordination of care and services becomes vital. The Association’s Certified Treatment Centers of Excellence (CTCE) and Recognized Treatment Centers (RTC) provide compassionate care in a supportive, family-oriented atmosphere. Centers that achieve either of these designations meet program requirements and follow recommended best practices as outlined in the American Academy of Neurology Practice Parameter and collaborate with their local Association chapter to offer care and support to people living with ALS and their families. People with ALS can maintain independence longer and experience improved quality of life when provided with options for symptom management, assistive technology, adaptive equipment, education, care services, and emotional support.

- The ALS Association’s Clinical Programs currently consists of 62 Certified Treatment Centers of Excellence, 21 Recognized Treatment Centers, and 64 Affiliated Clinics.
- The Association (National Office and Chapters) provided $3,332,946 in grants to our Certified Centers and Recognized Treatment Centers ($3,172,914 to CTCEs and $160,032 to RTCs) in 2017.
- 9,000+ people were served through our CTCEs and RTCs.

“ALS multidisciplinary care has been shown to result in higher quality of life than the more fragmented, traditional patient care. The integrated care at ALS centers is a model for the multidisciplinary approach, and studies have supported the value that patients perceive in such care.”

– Zachary Simmons, MD, The ALS Association Certified Treatment Center of Excellence at Penn State Hershey Medical Center
THE ALS ASSOCIATION CERTIFIED TREATMENT CENTERS OF EXCELLENCE

The ALS Association’s nationwide network of Certified Treatment Centers of Excellence™ provides evidence-based, multidisciplinary ALS care and services in a supportive atmosphere with an emphasis on hope and quality of life. To become certified as a Center of Excellence, an ALS clinic must meet clinical care and treatment standards, and all program requirements and processes to participate in ALS-related research, and successfully complete a comprehensive site review.

Criteria and Requirements

- Active relationship with, and support from, the local ALS Association chapter
- Organized ALS clinic monthly or more frequently as needed
- Clinic medical director, well-qualified in the field of neuromuscular neurology with a commitment to ALS
- Existing multidisciplinary or interdisciplinary ALS clinic, which should have been established for a minimum of one year with a substantial track record of institutional support before certification application process begins
- Availability of neurological diagnostic tools and other necessary medical services, including Gastroenterology or Interventional Radiology and Pulmonology
- Established caseload of people living with ALS (recommended minimum average of 50) and a pattern of new people with ALS joining the clinic
- Collaboration with other ALS Association Treatment Centers, for example in academic pursuits, educational pursuits, or IRB-related projects
- Full multidisciplinary team, which includes licensed and certified professionals present in clinic on ALS clinic days, including but not limited to:
  - ALS/Neuromuscular Neurologist
  - Nursing professional
  - ALS Association chapter liaison
  - Social Worker – MSW preferred
  - Speech Language Pathologist
  - Registered Dietician
  - Occupational Therapist
  - Physical Therapist
  - Pulmonologist or Respiratory Therapist (RT) *

- Available in the medical center and able to see the person(s) living with ALS or family during the clinic visit for unanticipated needs. Professional is not required to be physically present the entire clinic.
• If absent, another team member must be capable of performing required testing and assigned to conduct the pulmonary assessment and pulmonary function tests (PFTs).
• When known respiratory needs exist, the Pulmonologist or RT must be present during the clinic to see the person(s) with ALS with the identified needs.

• Mental Health Professional*
  • Ph.D. in psychology or psychiatrist available in the medical center and able to see the person(s) with ALS or family during clinic for unanticipated psychological or psychiatric needs. Doesn’t need to be present for the entire clinic.
  • If absent, another team member, such as a Master’s prepared nurse or social worker, must be capable of performing required consultations, evaluations, recommendations, and referrals.
  • If known psychological or psychiatric needs exist, there must be a timely plan for a psychologist or psychiatrist to meet with the person(s) or family to address issues.

• Active involvement in ALS-specific research (IRB-approved), to include at least one of the following:
  • Basic science or bench research
  • Translational research
  • Clinical trials
  • Clinical research studies
  • Genetics
  • Other IRB-approved ALS-specific projects

THE ALS ASSOCIATION RECOGNIZED TREATMENT CENTERS

The ALS Association Recognized Treatment Centers have the same high-quality approach to multidisciplinary care as The ALS Association Certified Treatment Centers of Excellence and provide services through a multidisciplinary care team. These centers, however, do not directly participate in ALS research.
## Requirements for the CTCE or RTC

<table>
<thead>
<tr>
<th>Requirement</th>
<th>CTCE</th>
<th>RTC</th>
</tr>
</thead>
<tbody>
<tr>
<td>Active relationship with, and support from, the local ALS Association chapter</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Organized ALS clinic monthly or more frequently as needed</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Clinic medical director, well-qualified in the field of neuromuscular neurology with a commitment to ALS</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Existing multi/interdisciplinary ALS clinic established for a minimum of one year, with a substantial track record of institutional support</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Availability of neurological diagnostic tools and other necessary medical services (to include Gastroenterology and/or Interventional Radiology and Pulmonology)</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Established caseload of people living with ALS (recommended minimum average of 50) and a pattern of new people with ALS joining the clinic</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Collaboration with other ALS Association Treatment Centers (e.g. in academic pursuits, educational pursuits, IRB-related projects)</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>1. Full multidisciplinary team (licensed and certified professionals present in clinic on ALS clinic days, including but not limited to):</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• ALS/Neuromuscular Neurologist</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Nursing professional</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• ALS Association chapter liaison</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Social Worker – MSW preferred</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Speech Language Pathologist</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Registered Dietician</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Occupational Therapist</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Physical Therapist</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Pulmonologist or Respiratory Therapist (RT) (at clinic or on as-needed basis)</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>• Mental Health Professional (at clinic or as a referral)</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Active involvement in ALS-specific research (IRB-approved), to include at least one of the following:</td>
<td></td>
<td>✔️</td>
</tr>
<tr>
<td>• Basic science/bench research</td>
<td>✔️</td>
<td></td>
</tr>
<tr>
<td>• Translational research</td>
<td>✔️</td>
<td></td>
</tr>
<tr>
<td>• Clinical trials</td>
<td>✔️</td>
<td></td>
</tr>
<tr>
<td>• Clinical research studies</td>
<td>✔️</td>
<td></td>
</tr>
<tr>
<td>• Genetics</td>
<td>✔️</td>
<td></td>
</tr>
<tr>
<td>• Other IRB-approved ALS-specific projects</td>
<td>✔️</td>
<td></td>
</tr>
</tbody>
</table>
The ALS Association’s nation-wide network of Certified Treatment Centers of Excellence™ provides evidence-based, multidisciplinary ALS care and services in a supportive atmosphere with an emphasis on hope and quality of life.

To become a certified as a Center of Excellence, each clinic must:
• Meet The ALS Association’s clinical care and treatment standards, based on AAN Practice Parameters
• Participate in ALS-related research
• Successfully complete a comprehensive site review

The ALS Association Recognized Treatment Centers have the same high-quality approach to multidisciplinary care as The ALS Association Certified Treatment Centers of Excellence™, however, they may not offer onsite research or opportunities to participate in clinical trials.

It is important to note that other models of care are necessary to meet the needs of all people living with ALS. These include, but are not limited to, neurology group practices and solo practitioners across the country. The ALS Association chapters may provide educational and other support to these practitioners and their patients in their local community. However, these models of care are not an official part of The ALS Association Certified Center Program.
TEAM MEMBERS

The ALS Association’s Centers have full multidisciplinary teams of ALS specialists at the clinic who will work collaboratively with the patient and their family. The ALS Association Centers are designed to provide a regular, thorough, and interdisciplinary evaluation; answers to questions; and potential solutions to problems. Typically, a clinic visit involves a full morning or afternoon and occurs every three months.

SUPPORTING EDUCATIONAL PRIORITIES

An ALS diagnosis can immediately leave people feeling distraught and struggling to cope. Our educational priorities focus on providing critical information to people living with ALS, their families and caregivers, and medical professionals — who may or may not, have prior experience with ALS.

- Providing educational programs and materials in a of myriad of strategic forms, including face-to-face, workshops, symposiums, print, digital, and video.
- Making sure content is accessible regardless of where a person lives and their access to available technology.
- Involving our intended audiences during the needs assessments, development and design of materials and programs, and identifying appropriate methods of sharing.
- Supporting the development and delivery of quality learning resources and opportunities for multiple age groups (including children), for various educational settings, respecting reading abilities, and in at least two languages.
- Developing educational resources that are relevant.
- Creating resources that can be repurposed locally, nationally, and internationally.
- Informing caregivers about options to care for their loved ones, especially as the person living with ALS loses the ability to do so themselves.
- Providing information on disease progression, symptoms and their management, and considerations for decision-making can help prepare individuals and families for addressing situations during the course of the disease.
- Educating medical professionals about ALS treatment best practices for those who have limited experience or who have never encountered the disease before.
- Identifying opportunities to collaborate with other professionals and like-minded organizations to reduce redundancy, increase efficiency, and build stronger unity including assessment and evaluation of programs and materials against a given set of key indicators to determine their effectiveness as part of ongoing quality improvement activities.
People with ALS come first in everything we do. The ALS Association is dedicated to providing people with ALS and their families and friends with the critical information, support, and resources necessary to live a full life and better meet daily challenges.

20,101 people living with ALS served through the chapter network in the past year.

Nearly 30,000 people viewed, downloaded, or ordered our educational materials including the Living with ALS and Families and ALS resource guides and medical information packets.

12 new Living with ALS resource guides released in digital and print formats and translated into Spanish to help educate about challenges that people living with ALS may experience.

538,441 unique page views on the Care Services webpages on www.alsa.org.

538,441

8,975 people served through our Certified Treatment Centers of Excellence and Recognized Treatment Centers.

1,717 times our 9 monthly educational webinars were viewed live or on-demand.

7 grants provided to support youth programs, including camps to provide emotional and educational support for children who have family members living with ALS, and an innovative pilot program to teach youths how to properly use durable medical equipment to assist with a loved one’s daily activities.

290 health care professionals participated in the first two webinars of a four-part webinar series designed for speech-language pathologists.

290

$4,069,784 in grants provided through our Certified Center Program.

25,751 attendees at support groups offered through our vast chapter network across the U.S.

25,751

Assistance provided to over 2,400 veterans through our nationwide chapter network.

2,400+

1,717 times our 9 monthly educational webinars were viewed live or on-demand.

1,717

130+ relationships with clinical partners, incorporating best practices as established by the American Academy of Neurology.

130+
THE NEED
An ALS diagnosis can immediately leave people feeling helpless and struggling to cope. There is a great need for new and innovative educational materials to support these people. As ALS is a progressive disease, information is needed at every stage of the disease to help people with ALS, families, and their caregivers understand what to expect and how to cope. Furthermore, education materials for medical professionals, who may not have prior experience with ALS have been found to be extremely useful in promoting the quality of care that people with ALS receive.

THE ALS ASSOCIATION’S ROLE
The ALS Association is the leading organization in the ALS community, committed to finding treatments and a cure, as well as providing support and educational materials for people living with ALS. Our educational mission priorities include:

1. Commitment to being the number one resource for people living with ALS by providing educational programs and materials in myriad forms, including print, digital, and video.
2. Informing caregivers about options to care for their loved ones, especially as the person living with ALS loses the ability to do so themselves.
3. Educating medical professionals about ALS treatment best practices for those who have limited experience or who have never encountered the disease before.
4. Creating public awareness of ALS with the general population to build support for the ALS Association’s many initiatives.

YOUR ROLE
From print to our digital online library of educational materials, The ALS Association is looking to partner with you to make important educational materials available.

Some opportunities for partnership include:
- Content and co-branding on print and digital materials. Our online educational resources were accessed over 430,000 times in the previous year and we have a wide-reaching audience throughout the chapter network, with approximately 14,000 people with ALS and their families receiving services at any given time.
- Building public goodwill through media campaigns that demonstrate how your company supports an important cause.
- Demonstrating to your employees that your company cares about the public causes that matter to them: the social piece of business.
THE ALS ASSOCIATION’S LIVING WITH ALS RESOURCE GUIDES

The ALS Association’s Living with ALS Resource Guides are a module series that covers the progression of ALS and the many issues and considerations surrounding the disease, from diagnosis to end-of-life. These guides cover rapidly expanding information and research in the clinical management of ALS and were designed to inform and educate people about ALS in a comprehensive and easily understood format. The resource guides address the most common challenges, concerns, and issues facing people living with ALS. They are extremely popular among the ALS community due to the breadth of subjects covered in each.

The guides cover the following topic areas:

- What is ALS? An Introductory Resource Guide for Living with ALS
- After the ALS Diagnosis: Coping with the “New Normal”
- Changes in Thinking and Behavior in ALS
- Living with ALS: Planning and Making Decisions
- Understanding Insurance and Benefits When You Have ALS
- Managing Symptoms of ALS
- Functioning When Mobility is Affected by ALS
- Adjusting to Swallowing Changes and Nutritional Management in ALS
- Changes in Speech and Communication Solutions
- Adapting to Changes in Breathing When You Have ALS
- Approaching End of Life in ALS

It is anticipated that the guides will be accessed online over 16,000 times over the next year. The printed resources are available through The Association’s order portal and throughout the ALS Association chapter network, resulting in thousands of printed guides being delivered to those in need.

The ALS Association is seeking $120,000 to print 2,000 copies of all resource guides for distribution to people with ALS, caregivers and professionals around the country.

TRANSLATION OF RESOURCE GUIDES

The Hispanic, Spanish-speaking population in the United States reached 57 million people nationwide in 2015 and is continuing to grow annually. Based on known prevalence rates, there are an estimated 3,400 Spanish-speaking people with ALS, although, due to underreporting, this number is likely higher. The ALS Association recently translated all Living with ALS Resource Guides into Spanish to ensure that this population can access information about ALS.

The ALS Association is seeking $60,000 to print 1,000 copies of each Spanish-translated resource guide for distribution to people with ALS, caregivers and professionals around the country.
COLLECTION OF MEDICAL RECORDS: MEDICAL INFORMATION PACKET
When providing emergency care, health professionals like paramedics and emergency room staff will have many questions about a person’s medical condition. In these stressful situations, it is often helpful to have an organized set of information, which can help inform medical providers about specific issues and considerations.

The Medical Information Packet has been developed to serve as a tool to inform medical providers caring for people with ALS, as well as provide insurance and family information, in a centralized location. The sheets may be used as a packet or as individual pages, based on one’s preference and need.

The ALS Association is seeking $9,000 to support the costs of printing 5,000 copies of the Information Packets for distribution to our chapter network across the country.

IN CASE OF EMERGENCY: KEY MEDICAL INFORMATION CARD
The Key Medical Information Card has been developed to include those fundamentally important key considerations that are necessary to know in an emergency situation. It can be printed and folded to fit easily in a wallet, pocket, car glove compartment, etc.

The ALS Association is seeking $6,000 to support the costs to print 5,000 cards for distribution to our chapter network across the country.

CENTRALIZED STORAGE OF MATERIALS: CUSTOM BINDERS
With the recent release of the new medical information packet mentioned above, and the creation of the key medical information card, The ALS Association aims to create a custom binder that retains the Medical Information Packet and the Key Medical Information Card.

The ALS Association is seeking $50,000 to produce 5,000 copies of the binders for distribution to people living with ALS nationwide through our chapter network.

GOING DIGITAL: ON THE GO MEDICAL INFORMATION-PHONE APPLICATION
One of the most exciting initiatives for the upcoming year is the creation of Android and Apple mobile phone applications that will combine the functions of the medical information packet and key medical information card. They will include critical emergency room information, such as necessary patient information like blood type, preexisting conditions, insurance information, important contacts, insurance details, and any medications that the person with ALS may be taking.

The ALS Association is seeking $100,000 to fund the development of these applications.
‘H O W T O ’ V I D E O S E R I E S F O R C A R E G I V E R S
ALS not only affects those diagnosed with the disease, but also those who care for them. While the person living with ALS has to adjust their life to live with the disease, their family members, or other caregivers, also have to make significant changes in their lives to ensure proper care for the person living with ALS. In conjunction with the caregiver printed materials, The ALS Association has as a programmatic priority to create a ‘How To’ Video Series. Often, caregivers lack the basic medical training and education to assist with the growing medical needs of their loved one. The ‘How To’ Video Series will focus on the subject matters relevant to meeting the daily needs of a person living with ALS, through presentations from subject matter experts. The goal of the video series is to ensure that caregivers are confidently able to provide the best care. Videos will be based on information contained in the Living with ALS Resource Guides.

The ALS Association is seeking $120,000 to enable development of storyboard, subject matter expert participation, and production of three videos in the upcoming year. Initial priority subjects include “Functioning When Mobility is Affected by ALS,” “Adjusting to Swallowing Changes and Nutritional Management in ALS,” and “Changes in Speech and Communication Solutions”.

ASSISTIVE TECHNOLOGY IN THE ALS SPACE

For some, ALS will completely take away their ability to communicate — verbally, written, bodily. While ALS destroys a person’s physical capabilities, the majority of individuals maintain their cognitive abilities. Without this ability to communicate, ALS can leave a person feeling trapped in their own body. This situation is particularly significant as people with ALS are often homebound due to the difficulties of leaving their residence.

Technology continues to play a key role in enabling ALS patients to maintain a level of independence. As a prime example, assistive speech technologies allow patients to communicate when they lose their ability to speak. Computer access (via a communication device or adapted computer, also known as augmentative and alternative communication (AAC) devices) may be their only link to the outside world; their only way to stay in touch with family and friends. This vastly improves their quality of life, as it provides them with the ability to gather information for decision-making, and offers opportunities for a variety of interactions.

THE ALS ASSOCIATION’S ROLE

Often, ALS patients have difficulty in using their devices and may have limited access to their clinic speech-language pathologist between clinic visits. One of the challenges The ALS Association faces is that, too often, Care Services chapter staff are neither trained in how to use these devices nor able to instruct patients on how to best use them. As a result, the person may have the technology available, but is unable to fully utilize its capabilities. There is a critical need for specialists who have the expertise and knowledge within the assistive technology spectrum. This includes both high-tech and low-tech assistive speech devices. A trained specialist is able to conduct more structured outreach to people living with ALS and family members regarding options for alternative forms of communication and can provide ongoing support.

One of The ALS Association’s goals is to hire Technology Integration Specialists (TIS) to support Association chapters. As The Association builds its technology offerings, the implementation plan requires the hiring of TIS’s to support multiple chapter service areas.

The TIS’s primary goal is to build up a chapter’s capacity to support technological advancements, including augmentative technology, for people living with ALS in their geographic region. The TIS will assist in developing strategies focused on engaging staff, constituents, and people living with ALS on utilizing technological opportunities that benefit the ALS community as a whole. This specialist will also work in conjunction with Speech Language Pathologist (SLP) in ALS certified centers and clinics, vendors, distributors, and other organizations providing communication devices throughout their service area. This will be critical to ensuring the creation of a culture in which appropriate resources and solutions are identified, planned for, sustained, and used safely and effectively.

The ALS Association is seeking $100,000 to hire each TIS. The final number of specialists will be based on geographical distribution.
YOUR ROLE
The ALS Association is asking for your help in helping people with ALS to live a fuller, less isolated, life. By choosing to fund this initiative, you will have a direct positive impact on so many in the ALS community. These programs are imperative to allowing people living with ALS the opportunity to be as independent as possible. Through your sponsorship, you will have a role in helping to improve quality of life for people living with ALS.

Your benefits:
- Promotion of your own brand’s assistive technology on The ALS Association’s many platforms.
- Branding on The ALS Association’s website or at Walks to Defeat ALS.
- Personalized social media campaigns highlighting your support.
THE NEED

Coping with ALS can be especially difficult for the children of a parent or other close family member living with ALS, as children do not have the same intellect and emotional maturity as adults. And, in most cases, they are not given the same age-appropriate educational resources to help them understand. This is a widespread concern, as a significant number of people living with ALS report having a child who is directly impacted by the disease.

Children often don’t understand what is happening to a loved one with ALS, leaving them feeling scared of the unknown and restricted in how they may be able to spend their time, such as feeling responsible for helping to care for the diagnosed loved one, rather than socializing with friends. The need for them to provide direct caregiving (sometimes in intimate situations, such as bathing a parent), combined with the need for them to provide emotional support to adults and siblings, can lead to feelings of isolation from peers, depression, and decreases in self-worth and self-esteem.

THE ALS ASSOCIATION’S ROLE

The ALS Association understands these young caregivers receive little of the attention given to their adult counterparts, leaving them a fragmented and relatively unknown caregiving population. In many instances, the isolation experienced by young caregivers restricts them from reaching out for assistance.

The ALS Association continues to develop resources and programs that support efforts to educate and assist children who have a loved one with ALS. Children handle trying times differently than adults and The ALS Association aims to ensure that they receive age and developmentally appropriate services.

YOUR ROLE

The ALS Association is asking for your help in achieving its mission priority of helping families live with ALS. By helping children directly impacted, you can enable positive experiences and increased knowledge, which will not only provide guidance during their journey with ALS, but will also have a lasting impact on their lives as they mature to adulthood.

YOUR BENEFITS

Through our abundance of resources, there are a number of opportunities from which to choose a program that will positively impact your brand. Some opportunities for partnership include:

- Content and co-branding on one or more of our family-oriented resource guides
- Build public goodwill by supporting a much loved, but underserved, demographic group
- Demonstrate to your employees that you care about the causes that matter most to them
CAMPS SPECIFICALLY DESIGNED FOR CHILDREN AFFECTED BY ALS

Children impacted by ALS often have trouble connecting with their peers who have not experienced similar circumstances. The ALS Association collaborates with different camp programs throughout the US that provide specialized programs where children can interact and connect with other children impacted by ALS while participating in organized group encounters. Youths attending such camp programs all have something in common (the significant impact of ALS on their lives). They can build new friendships and establish contacts, participate in sharing sessions, feel less alone in their challenges, and have an ongoing communication plan.

Each multi-day camp requires a budget of approximately $40,000, which includes expenses associated with venue, food, lodging, transportation for youths, activities, and professional staff. No child is required to pay for attending.

The ALS Association is seeking $20,000 to support five camps, which will be held in strategically identified locations across the United States. Enrollment at each camp is expected to be approximately 30 youths, 10 chaperones, and 10 professional staff.

FAMILIES AND ALS RESOURCE GUIDE

With no known cure and minimal known treatments, ALS can be a difficult subject to explain to children. The resource guide is the result of many years of clinical social work practice and formal research with families, children, and youths affected by neurological illness. While much attention is given to the person living with ALS and their adult family member/caregivers, children are often voiceless, despite experiencing much of the same shock, sadness, and grief as their adult counterparts.

The Families and ALS guide was created primarily for families living with ALS, including parents, grandparents, siblings, and other family members. It is also intended to support school-based and health care professionals who work with families affected by ALS. The goal of the Families and ALS resource guide is to facilitate discussions surrounding ALS, so family members can begin answering many of the questions that will be asked. This resource also offers guidelines for professionals assisting with families affected by ALS. A key theme throughout the guide is communication — how to do it, maintain it, and include it in everyday life.

The ALS Association is seeking $10,000 to fund a 2,000-unit run of this guide for distribution to children, families and professionals around the country. No family is charged for these resources.
ASSISTING WITH ACTIVITIES OF DAILY LIVING: DURABLE MEDICAL EQUIPMENT TRAINING

Coping with ALS can be difficult for the many children and youth of a family member living with ALS, given differences in emotional maturity and lack of age-appropriate educational and supportive resources. This is a widespread concern, as a significant number of people living with ALS report having a child who is impacted by the disease. Moreover, children are tasked with the physical care of a person living with ALS, yet had little to no training or support, leading to anxiety and concern in their lack of skill. In a study of young caregivers for ALS, 68 percent had no training or education in providing care, despite having to handle an average of 11 tasks, including complicated assistive devices. These children expressed fear of harming their family member with ALS, yet had no one to talk with, underscoring their anxiety and concern in their lack of skill.

THE ALS ASSOCIATION’S ROLE

In order to assist with the education and training of youths in caregiving including the correct use of durable medical equipment, the ALS Association will create a specific program in conjunction with Dr. Melinda S. Kavanaugh, PhD, from the University of Wisconsin, who has conducted significant research on young caregivers and children who are impacted by a loved one’s ALS diagnosis. This project will result in the creation of the manuals that will provide guidance, skill development, and support for children and youth caregivers who provide care to their loved ones living with ALS. This project specifically addresses our mission priority to provide persons with ALS and their family caregivers access to high quality consistent and compassionate support services. By working across Association chapters, this project strengthens the ability of the chapters to provide targeted, evidence-based, and rigorously tested caregiver educational programs, which will be made available to the ALS community nationwide.

Some opportunities for partnership include:

- Content and co-branding on all manuals that are created by this project.
- Building public goodwill through media campaigns that demonstrate how your company supports an important cause.
- Demonstrating to your employees that your company cares about the public causes that matter to them; the social piece of business.
ADVOCACY

ALS ASSOCIATION
INTRODUCTION TO ALS ADVOCACY
The ALS Association empowers advocates to talk about issues important to people living with ALS. Our goal is to educate policymakers and the public, and to drive toward smart decisions about ALS — related research, treatment, and access to care.

Federal and state governments play a vital role in ALS research, drug development, and the ability of people with ALS to get the health care they need. Every day, policymakers at all levels of government make decisions that could affect funding for ALS research and programs that serve people living with ALS. Our advocates educate elected officials about the urgent need to find a cure and treatments for ALS through meetings with members of Congress, writing letters, sending emails, or making phone calls. They encourage lawmakers to provide funding that supports care and encourage elected officials to consider ALS as they make policy decisions.

Our advocacy efforts extend beyond interacting with members of Congress. Our advocates also work to transform supporters, including members of Congress who are already supportive of ALS priorities, into even more vocal public health champions. Advocates can amplify their messages by engaging others and building greater public support through social media such as Facebook and Twitter. Social media helps amplify advocacy efforts by potentially reaching more people, in more places, faster than ever before.

- We are the largest and most influential national advocacy organization in the United States focused solely on ALS.
- We are committed to working across party lines to identify bipartisan solutions to problems.
- We empower and support people living with ALS to make a difference in the policy process to change laws, regulations and policies.
- We fight for increased funding for ALS Research and better access to Medicare, Social Security, and complex rehabilitation technology.
- We make sure that legislation, regulations, and other policy decisions reflect what is best for people living with ALS.
Over the past two decades, The ALS Association has been at the forefront of public policy efforts to better the lives of people with ALS. The ALS Association is the only national nonprofit organization fighting ALS on every front.

As the preeminent ALS organization, the Association leads the way in research, care services, public education, and public policy — giving help and hope to those affected by ALS. We bring our community together to speak with one voice and advocate on behalf of people living with ALS and for ALS research funding at the national level. We advocate before Congress, the White House, and with key federal agencies — including the National Institutes of Health (NIH), Department of Defense (DOD), Centers for Disease Control and Prevention (CDC), Centers for Medicare and Medicaid Services (CMS), Food and Drug Administration (FDA), Department of Veterans Affairs (VA), and the Social Security Administration (SSA).

Our chapters and advocates are essential to our success. The Association empowers a national grassroots network of more than 16,000 advocates with the information, tools, and expertise needed to speak out and play an active role in the fight for treatments and a cure. Between February and May of 2017 alone, ALS chapters and advocates accomplished over 770 meetings with members of Congress. Many more meetings took place throughout the year in states and congressional districts. During calendar year 2017, the National Office sent 11 Action Alerts to chapters and advocates who in turn generated more than 17,485 messages to Congress.
## HISTORY OF ALS ADVOCACY

### Timeline

<table>
<thead>
<tr>
<th>Year</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>2001</td>
<td>The ALS Association successfully lobbied Congress to waive the 24-month waiting period for Medicare coverage of people diagnosed with ALS.</td>
</tr>
<tr>
<td>2003</td>
<td>The ALS Association successfully prompted the Social Security Administration to publish new rules that made it easier for people living with ALS to qualify for Social Security Disability Insurance and added ALS to the list of conditions that automatically qualify for presumptive disability payments under Social Security Insurance.</td>
</tr>
<tr>
<td>2006</td>
<td>The ALS Association supported passage of the Lifespan Respite Care Act, which led to $15 million in funding per year for the kinds of respite care programs that are urgently needed by people with ALS.</td>
</tr>
<tr>
<td>2007</td>
<td>The ALS Association spearheaded efforts to establish a more focused, coordinated, and better-funded approach for studying ALS, which led to the creation of the ALS Research Program (ALSRP) as part of a $5 million appropriation in the FY2007 Defense Appropriations Bill.</td>
</tr>
<tr>
<td>2008</td>
<td>The ALS Association helped implement historic regulations at the Department of Veterans Affairs that designate ALS as a service connected disease, ensuring that veterans with ALS and their survivors have access to VA benefits, including health care and disability benefits.</td>
</tr>
<tr>
<td>2012</td>
<td>The ALS Association helped enact the FDA Safety and Innovation Act (FDASIA), which strengthened the Fast Track and Accelerated Approval processes, required FDA to partner more closely with patient organizations representing those with rare diseases, and provided additional flexibility to FDA to approve new treatments quicker through the use of biomarkers and other surrogate endpoints.</td>
</tr>
<tr>
<td>2013</td>
<td>The ALS Association partnered with FDA to convene the first-ever ALS specific public hearing and urged the FDA to partner with the ALS community to help expedite the drug development and approval process and bring new treatments to patients as soon as possible.</td>
</tr>
<tr>
<td>2016</td>
<td>The ALS Association helped push Congress to pass the 21st Century Cures Act, which included provisions that will improve the process of developing therapies targeting rare diseases and authorized $4.8 billion over 10 years for the NIH.</td>
</tr>
<tr>
<td>2017</td>
<td>The ALS Association brought the ALS community together to create the first patient-focused Guidance for ALS drug development that has been submitted to the FDA, a document that has the potential to speed access, reduce costs, help ensure resources are most effectively utilized and incentivize industry to enter the ALS market and develop new treatments for ALS.</td>
</tr>
<tr>
<td>2017</td>
<td>The ALS Association worked with Congress to authorize $10 million for the ALS Research Program in that year’s National Defense Authorization Act (NDAA), bringing total funding for the registry to nearly $80 million since the program’s inception in 2007.</td>
</tr>
<tr>
<td>2017</td>
<td>The ALS Association played a key role securing a bill that protects access to customized wheelchairs and accessories.</td>
</tr>
</tbody>
</table>
ADVOCACY GUIDANCE
The ALS Association submitted the first patient-focused guidance for ALS drug development to the Food and Drug Administration in 2017. The FDA has been engaged throughout the process of developing The ALS Association’s guidance and was committed to issuing its formal FDA guidance — FDA’s Draft Guidance on ALS Drug Development — that was released in February 2018.

The ALS Association community-led guidance is broader and more inclusive than that issued by the FDA’s Draft Guidance for two reasons:

1. It would give the agency a richer, more detailed understanding of the critical issues across the therapy development landscape in ALS.

2. It would build strategic focus and consensus among community stakeholders on as many key topics as possible.

The ALS Association Patient-Focused Guidance included over 100 participants, including industry, clinicians and researchers, the National Institutes of Health, the Centers for Disease Control and Prevention, and most importantly, people with ALS and their families.

The Guidance is designed to promote increased efficiency, predictability, and the speed of the drug development process, including clinical trials — leading to a more effective and earlier assessment of efficacy.

The ALS Guidance Document has the potential to:

- Speed access, reduce costs, help ensure resources are most effectively utilized, and incentivize industry to enter the ALS market and develop new treatments for ALS.

- Provide input to FDA on how to make the drug development process, including clinical trials, more efficient, predictable, and effective at assessing drug safety and efficacy.
In July 2018, an FDA Open Forum called, ALS Community Workshop: Developing Drugs for Treatment, Guidance for Industry, took place in Washington, D.C. where over 90 members of the pharmaceutical industry, clinical research experts, representatives from the FDA, and people with ALS and caregivers came together to provide targeted feedback and information to further inform the FDA’s Draft Guidance on ALS Drug Development. An FDA Workshop Report summarizing the recommendations that from the day were delivered to the FDA and are available to the public. The community is waiting for the FDA to release its revisions to its Draft Guidance.

The ALS Association has already seen tangible benefits from this work both in the research and development space, as well as in regulatory decision-making. In addition, The ALS Association has built on this foundational work for two initiatives:

**IMPACT ALS**
A patient preference study completed in late 2017 that gathered information regarding the perspectives of people with ALS and their caregivers in relation to disease burden, functional outcomes, and views on treatments. Over 1,534 people filled out the IMPACT ALS survey. The survey results were presented at multiple scientific conferences over the year. The Association also presented the results to the FDA in January 2019 to gather information on what the FDA wants included in the ALS Voice of the Patient Report, which is currently in draft form. A peer-reviewed publication is also in draft form to summarize and analyze the IMPACT ALS study results.

**ALS FOCUS**
A cross-sector collaboration to develop patient preference and experience data through surveys and studies. The vision of ALS Focus is to bring patient input into the center of ALS drug and product development, research and care to achieve a demonstrable impact on clinical trial design, regulatory review, coverage and payment, care services, and policy. It is a collaborative venture with industry, academia, and people with ALS and their caregivers to accelerate therapy development and bring a united voice to the ALS community. The initiative will produce generalizable and open data for the entire ALS community. In 2018, the Association raised funds to develop ALS Focus and plans to launch in 2019.
The Association works closely with volunteer leaders, ALS advocates, researchers, and partners on the federal and state level to advocate for policies that are supportive of and responsive to the needs of people living with ALS and their families.

Our network of approximately 16,000 grassroots advocates is mobilized on a regular basis through Action Alerts and comes together for conferences and signature events throughout the year to discuss and learn about public policy priorities, and advocate directly with lawmakers. Once someone becomes an advocate, they can be activated to contact their Senators and Representatives on important issues.

The Association values a collaborative approach to advocacy and belongs to several formal and informal coalitions, which allow us to benefit from and leverage the combined expertise of the patient advocacy community. Lawmakers have limited resources and time, and they rely on coalitions to provide policy solutions developed through consensus by diverse groups.

THE ROLE OF THE CHAPTER NETWORK IN ADVOCACY & PUBLIC POLICY

Our chapter network plays a vital role in our public policy efforts. Chapters serve as a resource for what people with ALS need and form the backbone of our long-term grassroots strategy.

While our grassroots advocacy grew as a result of the Ice Bucket Challenge, it is through our chapters that we expand our grassroots through their local connections and the work that they do on the ground, every day. This helps us continue to build our roster of grassroots advocates, who help amplify our message and make our story personal. Leaders at the chapter level also serve as our grasstops advocates — building the Association’s long-term relationships with members of Congress and their staffs, and bringing the local voice to Capitol Hill.

The ALS Association employs several tools as part of our grassroots advocacy strategy.

ACTION ALERTS AND CONTACTING CONGRESS

The Association maintains a database that allows us to reach out to thousands of advocates with updates on our public policy priorities and the latest developments from Capitol Hill, and to encourage them to contact their members of Congress and participate in the legislative process. In a matter of minutes, our advocates can send a personalized e-mail to their members of Congress.

ANNUAL FLY-IN

Every year, the ALS Association hosts a meeting in Washington, D.C., where our chapter executives and other leaders get a chance to participate in a day of meetings and trainings at the height of the appropriations process. Chapter leaders engage in discussion with national office staff and policy experts on the Association’s public policy priorities and travel to Congress for meetings with lawmakers and their staff to discuss our priorities, including continuing appropriations for ALS research at the Department of Defense and National Institutes of Health, as well as the ALS Registry at the CDC.
THE NATIONAL ALS ADVOCACY CONFERENCE

Every spring, the Association holds its largest annual conference in Washington, D.C., providing advocates from across the country an opportunity to come together to network, share their stories, and meet with members of Congress. More than 600 advocates — including as many as 120 people living with ALS — participate in this three-day conference to discuss the latest updates on research, compare notes on care services, and make an impact on public policy. Participants hear from national speakers and researchers leading the fight against ALS. They learn tips of the trade on how to meet with members of Congress, and take what they have learned and travel to Capitol Hill to meet directly with lawmakers and their staff.

OPPORTUNITIES FOR ADVOCACY AT SIGNATURE EVENTS

The Walk to Defeat ALS provides an opportunity for chapters to expand their roster of advocates and to participate in active advocacy with a large audience of people who already have an interest in fighting ALS. Advocacy tents or tables serve as gathering spaces where chapters can sign up new advocates or encourage existing advocates to sign letters to lawmakers. Chapters also have the opportunity to invite members of Congress to participate in the walk, which gives the member an opportunity to address the audience and to interact directly with people with ALS and their supporters and see how this disease impacts their community.

THE NATIONAL ALS REGISTRY

The ALS Association led the fight to establish the National ALS Registry at the CDC by working with Congress to enact the ALS Registry Act in 2008. The legislation provided the Agency for Toxic Substances and Disease Registry (ATSDR), a branch of the CDC, with the authorization and guidance necessary to create a National ALS Registry. The Association partnered with the CDC as it implemented the Act, and for the first time began identifying cases of ALS on a nationwide basis and collecting information urgently needed for ALS research.

The National ALS Registry is the single largest ALS research project ever undertaken. As the world’s largest population-based registry for people living with ALS, the Registry connects patients directly with clinical trials, fuels research as repository of data for scientists, and empowers patients to make invaluable contributions to a future freed from ALS. In January 2017, the ATSDR launched the National ALS Biorepository as an added component of the Registry, enabling researchers to request samples from both living and deceased (post-mortem) persons with ALS. The ALS Association has led efforts in Congress that have secured nearly $60 million in funding for the Registry since its creation.
PRIORITIES, IMPACT AND COALITIONS

Rep. Cathy McMorris Rogers (R-WA)
NEW CONGRESS

Educate, Advocate, Mobilize
The 116th United States Congress is scheduled to meet in Washington, D.C., from January 3, 2019, to January 3, 2021. In the 2018 midterm elections, the Democratic Party won the majority in the House of Representatives, while the Republican Party maintained control of the Senate. While there will be new members in both chambers, Democratic control of the House of Representatives will result in changes in leadership, committee chairs and committee assignments. The ALS Association will focus on educating, advocating and mobilizing the new Congress to achieve 2019 ALS Public Policy Priorities.

LEAD INITIATIVES

1. Waive the Five-Month Waiting Period for Social Security
The ALS Disability Insurance Access Act, to be introduced in the 116th Congress, will eliminate the five-month waiting period for Social Security Disability Insurance (SSDI). Under current law, people with ALS who qualify for SSDI must wait five months before receiving SSDI and access to Medicare. The legislation would eliminate the five-month waiting period for people with ALS.

2. Preserve and Increase Federal Resources for ALS Research
   A. National ALS Registry
      Provide a $10 million appropriation to continue the National ALS Registry and Biorepository at the Centers for Disease Control and Prevention. The Registry collects and analyzes data and directs a Bio Repository. It works in close collaboration with the Centers for Medicare and Medicaid Services, the Veteran’s Administration, the Department of Defense ALS research program and the National Institutes of Health (NIH). It is a critical driver of the search to find treatments and a cure because it connects researchers conducting clinical trials with people living with ALS and funds its own important research.

   B. Department of Defense
      Provide at least a $20 million appropriation to continue the ALS Research Program (ALSRP) at the Department of Defense (DOD). Research has repeatedly demonstrated that military veterans, regardless of branch or era of service, are approximately twice as likely to die from ALS than civilians. The ALSRP funded as a part of the Congressional Directed Medical Research Program at DOD, provides competitive grants that are an essential component of efforts to identify treatments and a cure for ALS.

   C. National Institutes of Health
      Continue ALS Research at the National Institute for Neurological Disorders and Stroke and other Institutes at the National Institutes of Health (NIH). In FY19, NIH spent approximately $77 million for ALS research, with National Institute of Neurological Disorders and Stroke (NINDS) making the largest investment and four other
NIH Institutes contributing the balance. The ALS Association will work in collaboration with other national organizations to advocate for $41.6 billion in funding for the NIH in 2019 (a $2.5 billion increase over 2018) so that NIH can maintain and increase this level of commitment to ALS research.

3. Access to Home Health Services
Achievable policy solutions are needed to improve access to home health services for people living with ALS. Federal government concerns about waste, fraud, and abuse of Medicare home health benefits for all seniors and misunderstandings about the scope of the benefit have significantly limited access for people living with ALS. In 2019, The ALS Association will continue to educate and advocate with the Administration and Congress on the home health needs of people with ALS as well as identify achievable ways to remove barriers through regulatory and legislative action.

4. Represent People Living with ALS on Access to Health Care and Medications
It is anticipated that both legislative and administrative proposal will be introduced that will impact access to health care and medications. The ALS Association will closely monitor and work with other patient advocacy groups to examine emerging proposals to determine their impact on people with ALS and their families.
We bring together the ALS community to speak with one voice to increase awareness, advocate for research funding and improved access to healthcare, and educate legislature at a federal level, impacting thousands of people with ALS and their families. Our 39 chapters and over 16,000 ALS Advocates are essential to our success. This advances our mission to discover treatments and a cure for ALS, and to serve, advocate for, and empower people affected by ALS to live their lives to the fullest.

Here are 2018’s BIGGEST successes in ALS advocacy!

**$10M NATIONAL ALS REGISTRY**
Led the charge to secure $10 million in appropriations for the National ALS Registry at Centers for Disease Control and Prevention to identify genetic and environmental factors for ALS, provide support to researchers to find treatments and a cure, and promote access to clinical trials.

**$10 MILLION**
Successfully protected the ALS Research Program at the Department of Defense (DOD), resulting in $10 million in appropriations and ensuring its place at the DOD.

**NIH $39.1B**
Joined with other leading patient organizations to secure $39.1 billion in funding for the National Institutes of Health (NIH) for FY2019. In 2018, NIH spent $83 million in FY2018 on ALS research.

**500 MEETINGS**
Supported nearly 500 meetings with Members of Congress at the Advocacy Fly-In and National ALS Advocacy Conference. Empowered more than 16,000 ALS Advocates with six action alerts – resulting in nearly 4,700 advocacy messages to Congress.

**Advocated with Centers for Medicare and Medicaid Services to remove barriers to Medicare home health for people living with ALS and initiated a multi-year effort to influence Congress and federal officials.**

Our nationwide chapters carry out our mission at both the state and community level and many have achieved successes in securing state-level services and support. Together, we provide an array of services to people living with ALS and their caregivers, work with our national network of ALS Certified Centers of Excellence, and support global ALS research efforts.
Kathleen Sheehan, Vice President, Public Policy, brings over 20 years of government relations experience on health care issues to the ALS Association. As an advocate and policy expert, she has extensive knowledge of patient concerns as well as the complexities of Medicare, Medicaid, private insurance, and pharmaceuticals. Throughout her career, Ms. Sheehan has championed access to care for patients, as well as increased appropriation for health science research at NIH and other federal agencies. Kathleen is a respected health care policy strategist known for her bipartisan approach and ability to create effective relationships across the political spectrum. Hallmarks of her career include vibrant engagement with chapters and volunteer leaders, as well as the development of effective collaborations with other national organizations. Kathleen has held leadership positions with the American College of Cardiology, the Visiting Nurse Associations of America, the Maryland Homecare Association, as well as national organizations focusing on substance abuse and mental health. Her leadership has resulted in numerous legislative victories, including securing a key provision in the Affordable Care Act and crafting key sections of reauthorization for the Substance Abuse and Mental Health Services Administration. Kathleen also possess expertise in public policy issues related to the development and approval of pharmaceuticals at the Food and Drug Administration.

Abram Bieliauskas, Manager, Government Affairs, recently joined The ALS Association and works with Kathleen Sheehan to lobby Capitol Hill on the Association’s various public policy priorities. Mr. Bieliauskas most recently served as Government Affairs and Advocacy Specialist at the Pancreatic Cancer Action Network, where he managed logistics for that organization’s Advocacy Day conference, oversaw a national grassroots network of advocates strategically located in key congressional districts, and advocated through influencing Congress to support increased cancer research funding. Abram also brings more than a year of Capitol Hill experience, having worked in both the U.S. House and Senate. He will be leveraging his appropriations experience to support CDC and ALSRP. Originally from Cincinnati, Ohio, Abram moved to Washington, DC, after graduating cum laude with a bachelor’s degree in Political Science Pre-Law from Ohio University.
HOW WE COLLABORATE

THE VALUE OF COALITIONS

When organizations join forces and work together to impact policy, they maximize access to policymakers and increase the likelihood of advocacy success. Members of Congress and administration officials, who have limited resources and time, rely on coalitions to provide policy solutions developed through consensus by diverse groups.

Belonging to a coalition in no way limits the ability of The ALS Association to act independently. It does amplify access to policymakers and the influence of The ALS Association in representing the concerns of people living with ALS. It also ensures that The ALS Association is at the table when consensus is developed and important decisions are made.

The ALS Association only belongs to coalitions that are nonpolitical and committed to working with Republicans and Democrats in a nonpartisan fashion. Participation by member organizations in any coalition activity is always voluntary. Decisions about whether to take a position on specific policy positions are made on a case-by-case basis — after a thoughtful consideration of the pros and cons.

Coalitions allow member organizations to benefit from combined expertise which, when leveraged, enables timely analysis and action. As new issues emerge, coalition efforts enable The ALS Association to join with others to examine complex issues and cross-cutting issues that impact patients.

PAID MEMBERSHIP ORGANIZATIONS

1. National Health Council (NHC)

The National Health Council (NHC) is the only organization that brings together all segments of the health community to provide a united voice for the more than 133 million people with chronic diseases and disabilities, and their family caregivers. Made up of more than 100 diverse, national health-related organizations and businesses, the NHC’s core membership includes the nation’s leading patient advocacy organizations, which control its governance and policy-making process. Other members include professional and membership associations; nonprofit organizations with an interest in health; and representatives from the pharmaceutical, generic drug, health insurance, device, biotechnology, and communications industries.

2. Research! America

Research! America is the nation’s largest not-for-profit public education and advocacy alliance working to ensure that research to improve health becomes a higher national priority. Relevant efforts focus on increasing funding for the National Institutes of Health, Centers for Disease Control and Prevention, and Food and Drug Administration.
3. Defense Health Research Consortium (DHRC)
Comprised of more than two dozen diverse organizations, the Defense Health Research Consortium (DHRC) focuses on continued Congressional support for several dozen medical research programs at the Department of Defense. These programs, funded annually in the Defense Appropriations Act, make up the Congressionally Directed Medical Research Programs (CDMRPs), which include the vital ALS Research Program (ALSRP).

4. National Organization for Rare Disorders (NORD)
A patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 260 patient organization members is committed to the identification, treatment, and cure of rare disorders through education, advocacy, research, and patient services. NORD provided essential leadership in the fight that maintained the Orphan Drug Tax Credit in the Tax Cuts and Jobs Act (P.L. 115-97), which was signed by President Trump in December 2017.

FREE AND INFORMAL COALITIONS OF WHICH THE ALS ASSOCIATION PARTICIPATES:

1. National Coalition for Assistive and Rehab Technology (NCART)
A national coalition of suppliers and manufacturers of Complex Rehab Technology (CRT) products and services used by individuals with significant disabilities and chronic medical conditions. NCART is focused on ensuring access to CRT and supportive services, and to establish and protect appropriate coverage, coding, supplier standards, and funding policies.

2. Leading Patient Advocacy Groups “Burrito” Coalition
A coalition of the nation’s leading patient advocacy organizations which have collectively opposed health care reform legislation that would negatively impact patients. Provided leadership on the repeal-replacement efforts on the Affordable Care Act (ACA), the coalition favors a bipartisan approach to reforming the health care system.

3. AARP Medical Expense Deduction Coalition
A coalition of more than 60 organizations opposing repeal of the medical expense tax deduction, a provision that could have major implications for households with extremely high health care costs. The coalition supports retention of the medical tax deduction so that taxpayers can continue to claim an itemized deduction for out-of-pocket medical expenses.