“The ALS Association has been the greatest organization to be affiliated with because they’ve got chapters around the country to help patients and their families. They do that through support groups and providing in-home care where people may not be able to afford the cost. And that gives a break to the loved ones, the family members that are left to take care of us when we can’t take care of ourselves.”

—John Russo
Co-Chair, ALS Focus Patient and Caregiver Advisory Committee
# TABLE OF CONTENTS

Our Case for Support ......................................................................................... vi
Our Impact ......................................................................................................... vii
Our Vision and Mission .................................................................................... viii

**Our Research**

The ALS Association Research Program

- The ALS Association Research Program ..................................................... 1-1
- Research ....................................................................................................... 1-4

Research Infographics

- Scientific Focus Areas .................................................................................. 1-12
- Current Projects ........................................................................................... 1-14
- Accelerating the Search for A Cure .............................................................. 1-15
- Research By the Numbers ........................................................................... 1-16
- Funded Research .......................................................................................... 1-17
- Advances in ALS Research .......................................................................... 1-18

Mission Initiatives

- The ALS Association’s ALS Roundtable Program ..................................... 1-20
- Thank You to Our 2020 Roundtable Sponsors ........................................... 1-22
- ALS Voice of The Patient Report ................................................................. 1-23
- ALS Focus Survey Program ....................................................................... 1-25
- Research Staff .............................................................................................. 1-28

**Our Care Network**

How We Work

- Summary ..................................................................................................... 2-1
- How We Work .............................................................................................. 2-2
- Chapter Membership ................................................................................... 2-4
How We Support Clinical Care

Supporting Clinical Care ................................................................. 2-11
Certified Centers ........................................................................... 2-14
Where We Support Care ............................................................... 2-15

Impact & Resources

Care Services Impact ................................................................. 2-20
Filling the Gap .............................................................................. 2-21
Education ..................................................................................... 2-22
Education ..................................................................................... 2-23
Assistive Technology ................................................................. 2-25
Children’s Programs and Resources ............................................. 2-27
The Jane Calmes ALS Scholarship Fund ......................................... 2-31
ALS Association Care Connection .............................................. 2-32
Care Services Staff ........................................................................ 2-33

Our Advocacy

Introduction To ALS Advocacy

Introduction .................................................................................. 3–1
History of ALS Advocacy ............................................................... 3–2
Timeline of Events ......................................................................... 3–3

Advocacy In Action

Advocacy Guiding Principles ......................................................... 3–5
How We Advocate ......................................................................... 3–6

Priorities, Impacts & Coalitions

Advocacy Impact ........................................................................... 3–10
How We Collaborate ...................................................................... 3–11
Advocacy Staff ............................................................................... 3–15

Appendix

Abbreviated Glossary of Scientific Terms ..................................... APP-2
OUR CASE FOR SUPPORT

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 accelerates 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 20,000 advocates helped protect and secure millions of dollars in funding for ALS research and enhanced access to treatments, including $40 million in the Department of Defense ALS Research Program and $10 million for the National ALS Registry. Our care services team served more than 20,000 people living with ALS, including 9,296 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.
OUR IMPACT

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

Healey Center establishes first platform trial

$3M Helped support the Healey Platform Trial to speed up drug development by reducing the cost of research by 30% and decreasing the trial time by 50%.

Starting a new effort to stop ALS as soon as possible

Starting a new effort to stop ALS even before people get sick by funding the first ever development of genetic testing and counseling guidelines for ALS to help with ALS and their families identify and manage their genetic risk and guide treatment decisions on emerging gene-based therapies. We are also partnering with CDC on a workshop to problem-solve and translate research about ALS causes into preventive approaches and treatments to reduce risk of ALS.

Bringing effective treatments closer to approval

Including AMX0035 from Amylyx Pharmaceuticals that was shown to extend life and slow disease progression, and continued progress on Tofersen, a genetic therapy for SOD1-ALS in late phase trials, along with progress on biomarker research.

Finding out what matters most to people with ALS and their caregivers

Since launching, over 1,450 people have participated in the ALS Focus. These surveys bring the perspectives and needs of people with ALS and caregivers to the forefront of program and policy decision making. To join, visit www.als.org/research/als-focus.

ADVOCACY

Boosted funding for the ALS Research Program at the Department of Defense (DOD) from $20 to $40 million in 2020 to support the creation of more clinical trials that will lead to the discovery of treatments and a cure.

Protected access to noninvasive ventilators (NIV) for Medicare beneficiaries with ALS by fighting the Centers for Medicare and Medicaid Services (CMS) decision to add NIV to the competitive bidding program.

Ended the SSDI 5-month waiting period for people living with ALS by spearheading successful efforts to pass and enact into law the bipartisan ALS Disability Insurance Access Act.

Ensured $10 million in continued funding for the National ALS Registry and Biorepository to identify genetic and environmental factors for ALS, provide support to researchers to find treatments and a cure, and promote access to clinical trials.

Supported over 630 meetings with members of Congress and over 500 calls to Congress during the 2020 Virtual Advocacy Fly-In and National ALS Virtual Advocacy Conference. Empowered more than 40,000 ALS advocates through 20 action alerts — resulting in over 85,000 advocacy messages to Congress.

CARE SERVICES

21,545 people living with ALS served through the chapter network in the past year

2,295 veterans received assistance through our nationwide chapter network

$4,885,387 in grants provided through our Certified Center Program

42,696 people viewed, downloaded, or ordered our educational materials, resource guides and medical information packets

25,831 attendees at support groups offered through our vast chapter network across the U.S.

9,296 people served through our Certified Treatment Centers of Excellence and Recognized Treatment Centers
OUR Vision
A WORLD WITHOUT ALS

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.
“We’ve made incredible progress in terms of understanding the biology behind the disease, and there is great hope for people living with ALS”

—Dr. Veronique Belzil, former Milton Safenowitz Research Fellow, Mayo Clinic Neuroscience Department, Jacksonville, FL
THE ALS ASSOCIATION RESEARCH PROGRAM

SUMMARY

Since 1985, The ALS Association has been at the forefront of ALS research. We pursue an agenda of effective and rigorous research to understand how ALS works and how it might be treated, and to improve the lives of people living with ALS today. Though we are the world's largest philanthropic funder, the scientific challenges posed by ALS are far bigger. We fund what we can, coordinate with other funders on the projects we cannot directly support and invest in the people and infrastructure needed to make ALS research efficient and effective.

FUNDING STRATEGY

Our direct funding is focused on high impact projects anywhere in the world. Our project selection approach is modeled after the National Institutes of Health. We receive structured applications from scientists, no matter where they are in the world, or if they work in academia or industry. Most often these applications are requested at specific times during the year, but we accept requests for funding at any time. Applications are scored and reviewed by outside experts selected for their knowledge of the topic proposed. Scores and the reviews are synthesized by Association scientific staff. Final funding decisions are made by the Research Committee of the Association’s Board of Trustees.

We also take into consideration ALS research that we do not fund, and how our funding can complement or accelerate these efforts. When projects are promising, but too large and expensive, we jointly fund a project with other ALS philanthropies. Sometimes those organizations approach us with funding ideas, and in other cases we identify projects and engage other funders for support. These projects are still reviewed by external experts and approved by our Research Committee. We have been fortunate to collaborate with just about every ALS research funder, including ALS Finding a Cure, Answer ALS, the Muscular Dystrophy Association, Project ALS, Target ALS, and more. We also collaborate with private companies in the ALS space. We have funded small biotech companies, as well as companion studies, such as biomarker studies to larger clinical trials through our Lawrence and Isabel Barnett Drug Development Program and Biomarker Program, respectively.

Our goal is to always ensure impactful work gets funded, even if we do not fund it ourselves. When we fund a project, we do so with the intention that other larger funders,
such as the federal government (like the NIH) or the private sector, will be able to carry the work forward. This strategy works. For example, an independent evaluation found our Ice Bucket Challenge research spending has served as a critical multiplier of funding from other sources. As a direct result of $40 million in Ice Bucket research funding we awarded, ALS scientists reported receiving an additional $122 million in follow-on grants from other funders.

**CAPACITY BUILDING**

We work to increase the capacity of ALS research as an enterprise. We fund multiple postdoctoral research fellowships (apprenticeships for new PhDs) every year through the Milton Safenowitz Postdoctoral Fellowship Program. We are proud that over 76% of our postdoctoral fellows go on to careers in ALS research, along with a large percentage of the remaining staying in the ALS space in industry or nonprofit jobs.

We also fund the infrastructure to make ALS research more efficient and impactful. These infrastructure projects include repositories of specimens, genetic information and electronic data that make it faster and cheaper for people to study ALS, and a smarter investment for private capital.

We pay particular attention to the infrastructure and capacity for clinical trials. We are the primary supporter of the Northeast ALS Consortium (NEALS), a network of clinical trial sites that facilitate almost all the ALS trials in the United States. We also are supporting the Healy Center ALS Platform Trial, which promises to speed ALS clinical trials. All these efforts move forward under the formal stewardship of our research program staff, external reviewers, and Research Committee.

**MISSION INTEGRATION TO ADVANCE RESEARCH**

The Association plays a critical role in research through our other mission areas as well. Our 70 plus Certified Treatment Centers of Excellence, overseen by our Care Services program, are critical settings for ALS human studies and clinical trials in their local communities.

There are many challenges in a person’s journey living with ALS, and there is a critical need for robust data to inform decisions of key opinion leaders to drive new programs and policies to improve their lives. We are using our ALS Focus survey platform to help inject empirical patient and caregiver preference data into these decisions. We are making these data available to the entire ALS community without charge, and interoperable with other ALS research tools through global unique identifiers for people with ALS and their caregivers. Our ALS Roundtable Program is geared toward identifying challenges and coming to creative solutions with leaders in the ALS community – in government, industry,
and most importantly, people with ALS and caregivers. This program has already identified the disconnect between FDA treatment approval standards compared to insurance coverage and payment standards.

Our Advocacy program supports the National ALS Registry for ALS research, which not only counts the number of people with ALS but collects data and funds scientists throughout the country to better understand the causes of ALS. We fight successfully for increased federal research resources for ALS, and the federal government spends more than $10 for every $1 we directly fund. We also recently fought to double the Department of Defense commitment to ALS research to $40 million. Our Advocacy and Research programs also collaborate on engagement with the FDA, which has led to many improvements in the conduct of clinical trials, including the new ALS Clinical Trial Guidelines released in 2019.

Our focus on the overall ALS research enterprise, and efforts to steward the ALS research community to greater capacity, efficiency, and collaboration, are working. In 2014, we were able to support 42 principal investigators, and by the end of 2018 that support had grown five-fold, to 237 principal investigators. In addition, the number of researchers collaborating on ALS-related scientific papers has nearly doubled since 2014.

Collaboration in the ALS space has already born fruit. Some of the recent discoveries in ALS research simply would not have been possible without the infrastructure and broad collaboration we have supported over time. We were able, for example, to fund multiple gene studies and databanks that pooled information and talent. More than 250 scientists around the world analyzed over 100,000 samples to identify a new genetic link to ALS, the KIF5A gene.

**MOVING FORWARD**

Our research strategy is robust and successful. There is an ever-growing portfolio of exciting drug targets and genes, clinical studies, scientists, and companies new to the ALS space. As the ALS clinical trial enterprise grows larger and faster, we will have to pay closer attention to preclinical safety and toxicity projects, to ensure there are enough promising candidates to test. And as these clinical studies bear fruit and practice starts to change, we will have to shift resources to study new treatments in real world settings, to ensure these new treatments are being used effectively. Some of these expanded areas of study might be disease modifying therapies in clinic. They may also be vital assistive technology and care giving practices that need data to drive both better service delivery and policy. And we will be supporting research to detect and treat ALS earlier and earlier in the disease process, in the hopes of preventing disability altogether. We are just getting started.
RESEARCH

Our Strategic Initiatives

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: [www.als.org/research/research-we-fund/partnerships-initiatives/strategic-initiatives](www.als.org/research/research-we-fund/partnerships-initiatives/strategic-initiatives)

**ALS PLATFORM TRIAL**

The ALS Association invested $3M over three years in the ALS platform trial to accelerate drug development.

This groundbreaking trial will test multiple therapies for efficacy at the same time, providing time and cost savings and increasing patient access to trials. The trial will take place at the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital in collaboration with the Barrow Neurological Institute and the Northeast ALS Consortium (NEALS). This investment builds on the Association’s long history of supporting research at Mass General – over $9 million just in the last 10 years.

Read the news:

- Learn more about the ALS Platform Trial here: [www.massgeneral.org/neurology/als/research/platform-trial](www.massgeneral.org/neurology/als/research/platform-trial)

**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 the 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 $835K 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.
**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 increase our understanding of the biology of ALS through “omics,” approaches to identify new targets for therapeutic development. 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 pre-clinical 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.

GENOME-SEQUENCING DATABASE

The Association contributed $3.3M to fund a centralized, cloud-based, genome-sequence database, including $100,000 from The ALS Association Georgia Chapter.

The first of its kind database will allow all researchers to share genomic data and conduct standardized analyses, leading to identification of new ALS drug targets. The Association’s investment will fund cloud storage, cloud computing, and the genomic sequencing of 3,000 people living with ALS.

The creation of a central repository was conceived by three researchers:

- John Landers, Ph.D., University of Massachusetts Medical School
- Jonathan Glass, M.D., Department of Neurology & Pathology, Emory University
- Bryan Traynor, M.D., Ph.D., National Institute on Aging, National Institutes of Health

Research Infographics
**SCIENTIFIC FOCUS AREAS**

The ALS Association Commitment

The ALS Association supports a wide breadth of specific fields of study that are critical to advancing ALS research. We are always on the lookout for the next cutting-edge field to invest in.

**BIOMARKERS**

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 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 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.
CURRENT PROJECTS
The ALS Association is Accelerating Progress Towards Treatments (as of February 2021)

162 Active Research Projects

In an effort to accelerate progress toward finding treatments and a cure for ALS, as of 02/01/2021, The ALS Association is currently funding 162 active research projects total. There are 134 within the United States.

Active Projects by Location

UNITED STATES

- Arizona – 4
- California – 14
- Colorado – 1
- Connecticut – 2
- Florida – 7
- Georgia – 2
- Illinois – 2
- Louisiana – 1
- Massachusetts – 28
- Maryland – 12
- Michigan – 4
- Minnesota – 3
- Missouri – 7
- North Carolina – 1
- New Hampshire – 1
- New York – 19
- Ohio – 2
- Oregon – 2
- Pennsylvania – 8
- Rhode Island – 2
- Tennessee – 2
- Texas – 4
- Virginia – 3
- Wisconsin – 3

WORLDWIDE

- Australia – 1
- Belgium – 1
- Canada – 5
- Chile – 2
- France – 1
- Finland – 1
- Germany – 2
- Ireland – 3
- Israel – 1
- Italy – 4
- Switzerland – 2
- United Kingdom – 5
ACCELERATING THE SEARCH FOR A CURE

The ALS Association is funding scientific projects across the research pipeline. We support a wide breadth of scientific focus areas – each is critical to advancing ALS research.

**Basic Research:** Understand causes of the disease and identify new therapeutic targets through gene sequencing, omics analyses, and epidemiological studies.

**Therapeutic Development:** Develop drugs and technologies against novel and genetic targets to test for preclinical efficacy and safety.

**Clinical Phases (I/II/III):** Test drugs for safety and efficacy in people with ALS. (Please note: The ALS Association does not fund phase III trials).

**Managing ALS:** Impact lives of people with ALS today through funding research in developing assistive technology, measuring patient/caretaker burden, and improving quality of life.

**Research Tools:** Generate, characterize, and distribute standardized tools and animal models for preclinical use by the research community.

**Biomarkers:** Discover, validate, and develop new ALS biomarkers. Biomarkers are needed to improve measurement of ALS diagnosis, prognosis, and a person’s response to treatment.

**Patient Cohorts:** Support infrastructure to run clinical trials, support registry and recruitment for clinical trials, and enable open source sharing of data and biospecimens for research.

**Community Engagement:** Integrate patient voice and perspective, and engage stakeholders across the community including other nonprofits, industry, investors, government, and regulators.

**Training & Education:** Support training through postdoctoral and clinical fellowships. Provide training for principal investigators, trial coordinators, and clinical sites for optimal clinical trials.
RESEARCH BY THE NUMBERS

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.

$113 MILLION

$113 MILLION

dedicated to research since the ALS Ice Bucket Challenge to advance treatments and a cure

162 ACTIVE RESEARCH PROJECTS ACROSS 13 COUNTRIES

2 CLINICIAN SCIENTIST DEVELOPMENT AWARDS IN 2020

3 potential new ALS antisense drugs and numerous other drugs on the horizon aimed to slow or stop the progression of ALS

9 new global strategic initiatives launched or supported

11 NEW ALS GENES (MATR3, CHCHD10, TUBA4A, TBK1, NEK1, C2orf2, ANXA11, TIA1, KIF5A, GLT8D1, and LGALSL) identified since the 2014 ALS Ice Bucket Challenge each representing new therapeutic targets

33+ new postdoctoral fellows chosen in 2020

35+ ALS drug trials and observational studies are actively enrolling*.

*as of 2/9/2021
FUNDED RESEARCH
The ALS Association Funded Research Across the Research Pipeline up to Phase 2 Clinical Trials

Thanks to our generous donors, The ALS Association awards various research projects throughout the year as part of its competitive 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 new 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.
- **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.
- **Managing ALS 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 research@alsa-national.org.
ADVANCES IN ALS RESEARCH

The ALS Association is the largest private funder of ALS research in the world. We advance ALS science by building research infrastructure and funding individual research projects and engaging the patient community in the search for treatments and a cure.

The ALS Association is proud to provide funding for these exciting innovations:

- **Healey Center establishes first platform trial**
  - $3M Helped support the Healey Platform Trial to speed up drug development by reducing the cost of research by 30% and decreasing the trial time by 50%.

- **Investing in infrastructure and data access accelerates ALS research.**
  - With over $1.2M for the PRO-ACT (Pooled Resource Open-Access ALS Clinical Trials) database which is the world’s largest ALS clinical data repository. Since its launch in 2012, over 900 researchers from 68 countries in both for-profit and non-profit sectors, have obtained access and analyzed these datasets resulting in 32 scientific publications.

- **Starting a new effort to stop ALS as soon as possible**
  - Starting a new effort to stop ALS even before people get sick by funding the first ever development of genetic testing and counseling guidelines for ALS to help with ALS and their families identify and manage their genetic risk and guide treatment decisions on emerging gene-based therapies. We are also partnering with CDC on a workshop to problem-solve and translate research about ALS causes into preventive approaches and treatments to reduce risk of ALS.

- **Continuing the search during a global pandemic**
  - By committing funding for 24 new projects spanning diverse areas of research such as biology, therapeutics, biomarkers, infrastructure, and clinical trials. The Association managed over 162 active projects with a multiyear commitment of over $55M and $113M committed to research from the ALS Ice Bucket Challenge.

- **Bringing effective treatments closer to approval**
  - Including AMX0035 from Amylyx Pharmaceuticals that was shown to extend life and slow disease progression, and continued progress on Tofersen, a genetic therapy for SOD1-ALS in late phase trials, along with progress on biomarker research.

- **Finding out what matters most to people with ALS and their caregivers**
  - Since launching, over 1,450 people have participated in the ALS Focus. These surveys bring the perspectives and needs of people with ALS and caregivers to the forefront of program and policy decision making. To join, visit www.als.org/research/als-focus.
Mission Initiatives
THE ALS ASSOCIATION’S ALS ROUNDTABLE PROGRAM

Since 2019, The ALS Association Roundtable Program has assembled members of the ALS community for candid, facilitated discussions that shape the Association’s strategic planning efforts and find solutions to improve ALS care, advocacy, and research.

PROGRAM OBJECTIVES

The Roundtable Program provides a forum for solution-based discussions with ALS stakeholders to accelerate the development of meaningful treatments for ALS and improve the lives of people with ALS and their caregivers. All Roundtable meetings include individuals with expertise and experience from a variety of sectors who reflect diverse points of view on ALS-related issues. The group identifies challenges faced by people with ALS and their caregivers and then recommends action that significantly impacts future care and treatment of the disease.

While these meetings began as in-person gatherings to build relationships and rapport among stakeholders, the COVID-19 pandemic prompted the most 2020 Roundtable meetings to go virtual. Though the format has changed, the level of engagement, participation and productivity remains exceptional, even allowing greater participation among members of the ALS community for whom travel is challenging.

BENEFITS OF PARTNERING IN ALS ROUNDTABLES

GLOBAL COLLABORATION

All members of ALS Roundtables benefit from this collaborative forum, which promotes understanding and builds partnerships with industry peers, clinician and scientific leaders, policy makers, regulatory officials, public and private insurance representatives, ALS Association leadership, and — most importantly — people with ALS and their caregivers.

DRIVING PRIORITY PROGRAMS

The Roundtable meetings are a unique opportunity to shape and inform program- and advocacy-based solutions to challenges faced by the ALS community.

INFORMING ALS RESEARCH AND POLICY AGENDAS

Roundtable meetings inform agendas and decision making on topics that are critical to
the ALS community, including clinical trial issues, regulatory and access evaluations, payment determinations for ALS therapies, and more.

PAST ROUNDTABLE TOPICS
- Setting ALS Association Strategic Priorities (spring 2019)
- Access to New Therapies (fall 2019)
- Reducing Time to Diagnosis (spring 2020)
- Reducing ALS Complications (fall 2020)

2020 PROGRAM OVERVIEW
The April 2020 Roundtable was focused on reducing time to diagnosis, a critical topic for the ALS community that brought together 69 attendees representing key ALS stakeholders. ALS diagnosis is complex and is primarily based on the physician’s interpretation of clinical symptoms and ruling out other diagnoses. People with ALS present first symptoms and progress in varied ways adding to the diagnosis complexity. During the meeting, participants mapped out key challenges, potential solutions, and near-term action steps the community can take to streamline and accelerate the process of arriving at an ALS diagnosis. As an outgrowth from the meeting, the Association formed two multi-stakeholder working groups: time to diagnosis with a focus on education and awareness for general neurologists and on genetic testing and counseling (see below).

The November 2020 Roundtable was centered around decreasing ALS complications for people with ALS, with the goal of identifying common ALS complications, focusing on those that may be preventable. We brainstormed on what can be done by the Association in collaboration with our community (especially with clinicians and care services) to reduce complications. Decreasing complications could potentially clear the way for more efficient clinical trials and improved quality of life and longer/better lives for people with ALS. The fall meeting also provided important updates from the Association regarding its strategic planning efforts and ongoing and future work to secure access to future therapies.

ALS ROUNDTABLE WORKING GROUPS
Stemming from past Roundtable meetings, three working groups were formed in 2020 to delve deeper into each topic and put solutions into action.

- The Increasing Clinical Trial Enrollment working group (formed as result of spring 2019 Roundtable) met this year. The first recommendation the group is pursuing is to increase enrollment with a system to match people with ALS to clinical trials.
- The Time to Diagnosis working group is focused on reducing time to ALS diagnosis by improving the education and resources for ALS diagnosis, with a focus on general neurologists. The group first aims to
develop a multi-stakeholder consensus statement about the benefits of early diagnosis.

- The Genetic Testing and Counseling working group is focused on making a measurable impact on genetic testing and counseling landscape for people with ALS and their families. So far, the group is working on developing a genetics glossary to emphasize consistent and clear terminology around ALS genetics. The Association is also supporting the development of Genetic Testing and Counseling Guidelines, which are aimed at improving genetic testing literacy among clinicians and genetic counselors in order to accelerate the genetic characterization of the ALS population.

**WHAT TO EXPECT**

Roundtable meetings focus on a clearly defined topic that is selected ahead of time by the Association staff and the Roundtable Advisory Panel. A professional moderator facilitates and guides the discussion. Everyone has the opportunity to be heard. After the Roundtable, an executive summary is distributed to participants. Sponsors are invited to participate in working groups to further delve into identified challenges to advance concrete solutions.

**THANK YOU TO OUR 2020 ROUNDTABLE SPONSORS**
ALS VOICE OF THE PATIENT REPORT

The ALS Voice of the Patient Report is a groundbreaking document that gives the FDA and other key stakeholders, including drug developers, health care providers, and insurance companies, data from people with ALS and caregivers about the everyday impact and burden the disease has on peoples’ daily lives, their experiences with currently available treatments, and their hopes for future ALS therapies. This information is also providing context of the disease burden to the FDA to inform the Agency’s review of new drug applications. This report documents the immense unmet medical need we all are working to confront. The content of the ALS Voice of the Patient Report was derived from the IMPACT ALS survey initiative that was conducted in December 2017. This survey was led by The ALS Association with our partners Cytokinetics, Biogen, Ionis Pharmaceuticals, regulatory experts, ALS clinical thought leaders, and, most importantly, patient and caregiver input.

The vision of the IMPACT ALS survey was to systematically gather and expand available data on the perspectives and experiences of people with ALS and caregivers. We surveyed both people living with ALS and their care partners. A total of 1,534 people participated. Of that, 813 were people with ALS, 74 were people assisting a person with ALS, and 647 were caregivers. People with ALS were represented from across the U.S., with representation across states comparable to the portion of the U.S. population in each state.

While the report’s findings will probably resonate with people living with ALS; what is important here is the systematic
Our Research

The vision of the IMPACT ALS survey was to systematically gather and expand available data on the perspectives and experiences of people with ALS and caregivers.

documentation of these findings and their formal submission to the FDA. This is the first time such data has been gathered to this extent across disease progression.

**KEY TAKEAWAYS OF THE ALS VOICE OF THE PATIENT REPORT:**

- Individuals with ALS and caregivers reported significant symptoms of fatigue and weakness, followed by speech problems, shortness of breath, difficulty sleeping, and pain.
- These progressive symptoms result in a loss of independence and led to less time spent at work or school, socializing, or traveling.
- People with ALS do not report high satisfaction with currently available treatments and reported that such treatments “somewhat help” with managing ALS and everyday impacts of disease. Breathing assistance devices were reported to be very inconvenient and burdensome, but necessary.
- With current treatments mainly focused on supportive care, people with ALS would like treatment options that stop disease progression, improve muscle weakness, and assist with breathing or respiratory function.
- The majority of people with ALS reported having fears about the future. The fears most frequently reported were related to dying, “leaving family too soon,” followed by “dying from respiratory failure.” The next most reported fear was “spending the family’s savings on medical care.”

The IMPACT ALS survey is the first of a series of patient and caregiver surveys through the ALS Focus survey platform initiative, which is a cross-sector collaboration to place the preferences of people with ALS and their caregivers in the center of treatment and policy development, through surveys and studies. ALS Focus will build from the IMPACT ALS survey and conduct robust research on patient and caregiver needs, preferences, and experiences to produce generalizable and open data for the entire ALS community.
ALS FOCUS SURVEY PROGRAM

Bringing the perspectives of people with ALS and their caregivers to the forefront of research, care, and advocacy.

ALS Focus is a patient- and caregiver-led survey program to understand the experiences and needs of people with ALS and their caregivers, putting their perspectives at the center of treatment and policy making. ALS Focus surveys collect data on the symptoms, burdens, and everyday impacts of ALS that people face throughout the disease journey so that the whole ALS community can benefit. All components of ALS Focus are built on a cross-sector collaboration between The ALS Association, people with ALS, caregivers, industry, academia, and the federal government.

WHAT ARE THE GOALS OF ALS FOCUS?

The overarching goal of ALS Focus is to conduct scientifically rigorous patient- and caregiver-focused research to improve their quality of life. Specifically, we aim to:

- Distribute surveys throughout the year to the ALS community.
- Create a collaborative setting that invites candid feedback and insights from patients and caregivers.
- Develop and validate tools such as clinical outcome assessments to measure what is most important to people with ALS and caregivers across the spectrum of disease and disease progression.
- Conduct benefit-risk research and other preference studies.
- Use ALS Focus measures and findings to inform:
  - Clinical trial design, clinical endpoints and scales, and regulatory decisions.
- Value-based reimbursement models for ALS therapies and products.
- Federal regulators and policy makers in government agencies such as the FDA and Centers for Medicare and Medicaid Services (CMS), as well as members of Congress.
- Clinical care, care services, and home health practices.

**WHY IS IT IMPORTANT TO PARTICIPATE IN ALS FOCUS?**

Scientifically rigorous efforts to collect experience and preference data from people with ALS and caregivers were largely non-existent before ALS Focus. Through this program, the Association is committed to bringing the voice of the ALS community to the forefront to inform how researchers, health care providers, policy makers, and payors make decisions about improving the lives of people with ALS and those who care for them. In addition, the FDA calls for rigorous input from patients and caregivers to inform drug development. ALS Focus data address this need.

Importantly, all de-identified data and findings from each ALS Focus survey are free and open to the entire ALS community after a six-month embargo period. ALS Focus assigns a neurological global unique identifier (NeuroGUID), a secure de-identified code, to each participant, making it possible to link experience and preference data from Focus with data from other ALS research studies that use NeuroGUIDs, such as the National ALS Registry and clinical trials.

**WHO CAN PARTICIPATE?**

**People living with ALS**

**Current or past caregivers of people with ALS**

**HOW LONG WILL IT TAKE?**

Once registered, each secure online survey can be completed in approximately 15-25 minutes.

**WHERE CAN I PARTICIPATE?**

- Access the survey at [als.org/ALSFocus](http://als.org/ALSFocus).
- Learn more here: [www.als.org/ALSFocus](http://www.als.org/ALSFocus) and for questions email: ALSFocus@alsa-national.org

*Participants must be at least 18 years old and reside in the United States. Survey instructions and questions are presented in English.

**ALS FOCUS ACHIEVEMENTS**

Since January 2020, ALS Focus achieved the following milestones:

- Secured funding for ALS Focus through multiple major corporate sponsorships.
- Built out a Focus survey platform and data repository housed at NeuroBANK™ through the Neurological Clinical Research Institute at Massachusetts General Hospital.
- Increased membership of the Patient and Caregiver Advisory Committee to 20, which
includes people with ALS and current and past caregivers across the country, all lending their unique perspectives and expertise.

- Since launching, over **1,450** people signed up to participate in ALS Focus.
- Conducted the first ALS Focus survey – Understanding Insurance Needs and Financial Burdens – along with an ongoing demographics survey. In total, **530** people with ALS, current caregivers and past caregivers participated in the demographics survey and **440** participated in the insurance survey.
- Expanded and updated a suite of communication materials to increase awareness and recruitment, including a dedicated Focus web page (www.als.org/ALS-Focus), social media, national emails, and turn-key information for distribution.
- Prepared the first survey dataset and analysis tools for Focus’ corporate sponsors to access before the six-month embargo period.
- Visualized and interpreted results from the first

survey to share through the Focus website and other communication materials. ([www.als.org/ALS-Focus/survey-results/](www.als.org/ALS-Focus/survey-results/))

- Conducted the second ALS Focus survey on what matters most to people with ALS. This survey uses the ALS Health Index Short Form developed by Chad Heatwole, MD, MS-CI at the University of Rochester through an Association grant. Over **675** people participated.
- Launched a new health status survey to gauge where Focus participants are in the disease journey.
- Upcoming ALS Focus surveys topics include Caregiver Burden and Risk Tolerance for People with ALS.
RESEARCH STAFF

NEIL THAKUR, PH.D., CHIEF MISSION OFFICER

Neil Thakur brings more than two decades of experience as a public health expert to the fight against ALS. He has led The ALS Association’s mission programs – research, care services, and advocacy – since 2018.

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 US 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.

KULDIP DAVE, PH.D., VICE PRESIDENT, RESEARCH

Dr. Kuldip Dave, Vice President, Research, joined The ALS Association in 2019. Reporting to Neil Thakur, he oversees the Association’s
Research Program. In this role, he develops and implements the broader research vision by setting annual priorities and department goals including research funding strategy, research programs, and strategic initiatives. He is also responsible for the operational, staffing, and budgetary oversight of the research program. He engages with various stakeholders including key opinion leaders from academia, government, industry, and other nonprofit organizations. He serves as a key communication lead for programmatic efforts by presenting the Association’s perspective and strategy to diverse audiences at workshops/conferences/forums and by developing content for written publications, blogs, social media, informational videos, perspective papers, and journals.

Dr. Dave received his bachelor’s degree in Biology from Rutgers University in New Brunswick, NJ. He received his Ph.D. degree in Pharmacology and Physiology from Drexel University College of Medicine in Philadelphia, Pa. He went on to do postdoctoral fellowship at a small biotechnology company in Pennsylvania where he was first exposed to the drug development process. He went to work for the pharmaceutical company Wyeth for two years before joining The Michael J. Fox Foundation for Parkinson’s research. At MJFF, he oversaw a $30M portfolio and led two priority therapeutic areas. After nine years at MJFF, he joined The ALS Association has the head of the Research program.

JILL YERSAK, PH.D., VICE PRESIDENT, MISSION STRATEGY

Dr. Jill Yersak, Vice President, Mission Strategy, joined The ALS Association in October 2015. Reporting to Neil Thakur, she helps oversee the strategy of projects and programs that span the Association’s mission – research, care, and advocacy. In this role, she leads mission programs including the ALS Roundtable Program and ALS Focus survey program. She is also responsible for communicating ALS research in an accessible way by developing and giving research presentations tailored to people living with ALS, their caregivers, and loved ones. She provides Association-wide support, at the national office and throughout the chapter network and in all departments, with research information needs. These include donor outreach and research development training. As a former part of the Research Communications team, she played an integral part in implementing the Association’s blog, 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 on to 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 at Brown University. During this time, she spearheaded a project to generate precise ALS C. elegans models (which are microscopic worms) and co-wrote a successfully funded ALS Association grant based on this project.

**SARAH PARVANTA, PH.D. MPH, DIRECTOR, ALS FOCUS**

Dr. Sarah Parvanta, ALS Focus Director, has a Ph.D. in communication from the University of Pennsylvania and a master of public health (MPH) from the University of North Carolina at Chapel Hill. Dr. Parvanta leads development and implementation of ALS Focus, a survey program to understand the experiences, preferences, and needs of people with ALS and their caregivers. She specializes in survey design, data analysis, and studies on health perspectives and quality of life. In her previous role as a research analyst at RTI International, she evaluated the impact of The ALS Association’s research program following the ALS Ice Bucket Challenge.

**ASHTON FERRARA, MPH, MANAGER, RESEARCH OPERATIONS**

Ashton Ferrara started with The ALS Association in September 2019. Reporting to Kuldip Dave, she is responsible for issuing research contracts and managing contract negotiations, tracking compliance with funded awards, supporting the development of new research programs and collaborating on impact measures of the research program and reporting outcomes. Ashton received her bachelor’s degree in Public Health from James Madison University in Harrisonburg, VA and a Master of Public Health (MPH) with a concentration in Health Promotion from Liberty University in Lynchburg, VA. Prior to joining the Association, Ashton worked as a Contract Administrator with Patient-Centered Outcomes Research Institute (PCORI), where she managed pre and post award activities for Healthcare Research, Research Infrastructure (PCORnet) and Task Order based Contracts.

**SELLAM BIRHANE, MBA, MANAGER, MISSION PROGRAMS**

Sellam Birhane is the Manager for Mission programs at the ALS Association. She is responsible for helping to coordinate ALS mission programs, including overseeing any
mission programs that emerge from the ALS Roundtable program. She is also responsible for the overall project management of new mission programs, tracking and executing against milestones and action items, as well as management and communications with working groups and committees. She also provides support to existing mission programs such as ALS Focus and ALS Roundtables.

Sellam received her bachelor’s degree from the University of Mary Washington (UMW) Fredericksburg, VA. She also graduated with a master’s degree in Business from UMW in 2018. While working on her MBA she worked for the Office of Student Activities and Engagement as a Graduate Assistant, mainly performing the duties of an office manager, event planner, and student mentor. She also served as a volunteer project manager for a health careers institute, creating a work breakdown structure and doing risk management for the client. After graduation she went on to work as a Business Analyst at CGI in Rosslyn, VA. In this capacity she assisted in risk management activities, user troubleshooting, requirements analysis, and basic programming. She also served as a Program Coordinator for The ALS Association on a temporary basis before becoming the Manager for Mission Programs.
“We would not be where we are today without the help of others. We immediately reached out to The ALS Association Evergreen Chapter and they have been immensely helpful in our journey.”

– Janetta Michelsen, Caregiver, Seattle, Washington
How We Work
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 have access to information they may 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.

The Association uses continuous feedback mechanisms and quality improvement processes to actively assess the needs of the communities we serve in order to address gaps in service delivery. This serves as the foundation for developing and delivering innovative services and for 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 500 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 among 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, policymakers, 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 a 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 new to the chapter, 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 the 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 planning 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 acknowledgment or retreat programs.
- Supporting access to The ALS Association Care Connection Program, which utilizes an online calendar to coordinate family needs with available family, friends, 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 referrals to professional service providers as needed.

DURABLE MEDICAL EQUIPMENT (DME) 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 AUGMENTATIVE & ALTERNATIVE COMMUNICATION PROGRAMS (AT/AAC)

- Providing education and information related to how ALS impacts communication and what types of interventions are available to
support augmentative or alternative communication.

- Initiating and cultivating relationships with augmentative/alternative communication (AAC) equipment vendors and speech-language pathologists to facilitate access to appropriate evaluation, selection and training in the use of communication equipment.
- Implementing an assistive technology (AT) Loan Program consistent with national assistive technology/augmentative/alternative communications (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 speech-generating device (SGD).
- Providing expanded program services including employing or contracting with a speech language pathologist (SLP) or assistive technology professional (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: durable medical equipment (DME), assistive technology/augmentative/alternative communications (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, physical therapist (PT), occupational therapist (OT), speech-language pathologist (SLP), and certified nursing assistant (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 modifications, 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, information 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
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 Parameters 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 an 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 73 Certified Treatment Centers of Excellence, 21 Recognized Treatment Centers, and 94 Affiliated Clinics.
- The Association (National Office and Chapters) provided $4,885,387 in grants to our Certified Centers and Recognized Treatment Centers ($4,525,981 to CTCEs and $359,406 to RTCs) in 2020.
- 9,296 people were served through our CTCEs and RTCs.
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 the 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 Dietitian
  - 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:
  - Clinical Trials (Interventional)
  - Clinical Studies (Observational, Biomarker Studies, Natural History)
  - Genetic Screening, Sequencing, or Counseling Research
  - Basic Research/Drug Discovery Research
  - Telemedicine/Telehealth Research
  - Assistive Technology/Device Development Research
  - Other ALS Specific, IRB Approved Research

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
CERTIFIED CENTERS
Certified Treatment Centers of Excellence (CTCE) and Recognized Treatment Centers (RTC)

<table>
<thead>
<tr>
<th>Requirements for the CTCE or RTC</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>
</tbody>
</table>

1. Full multidisciplinary team (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 Dietitian          | ✓ | ✓ |
   - Occupational Therapist        | ✓ | ✓ |
   - Physical Therapist            | ✓ | ✓ |
   - Pulmonologist or Respiratory Therapist (RT) (at clinic or on as-needed basis) | ✓ | ✓ |
   - Mental Health Professional (at clinic or as a referral) | ✓ | ✓ |

Active involvement in ALS-specific research (IRB-approved), to include at least one of the following:
- Clinical Trials (Interventional)
- Clinical Studies (Observational, Biomarker Studies, Natural History)
- Genetic Screening, Sequencing, or Counseling Research
- Basic Research/Drug Discovery Research
- Telemedicine/Telehealth Research
- Assistive Technology/Device Development Research
- Other ALS Specific, IRB Approved Research
WHERE WE SUPPORT CARE
Team Members (as of January 2021)

THE ALS ASSOCIATION CERTIFIED TREATMENT CENTERS OF EXCELLENCE℠

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 certified as a Center of Excellence, each clinic must:

- Adhere to 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

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 do not offer onsite ALS research.

OTHER ALS ASSOCIATION AFFILIATED CLINICS

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.
CERTIFIED TREATMENT CENTERS OF EXCELLENCE

To find a Certified Treatment Center in your area, visit http://www.als.org/community/centers-clinics/
## RECOGNIZED TREATMENT CENTERS

<table>
<thead>
<tr>
<th>Clinic Name</th>
<th>ST</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alabama Neurology Associates</td>
<td>AL</td>
</tr>
<tr>
<td>Kaiser Permanente Los Angeles Medical Center</td>
<td>CA</td>
</tr>
<tr>
<td>Kaiser San Rafael ALS Multidisciplinary Clinic</td>
<td>CA</td>
</tr>
<tr>
<td>Kaiser San Francisco ALS Multidisciplinary Clinic</td>
<td>CA</td>
</tr>
<tr>
<td>Kaiser Permanente South Bay Multidisciplinary ALS Clinic</td>
<td>CA</td>
</tr>
<tr>
<td>San Francisco VA</td>
<td>CA</td>
</tr>
<tr>
<td>Colorado Neurological Institute</td>
<td>CO</td>
</tr>
<tr>
<td>Iowa City VA Health Care System</td>
<td>IA</td>
</tr>
<tr>
<td>University of Louisville Physician’s ALS Clinic at Frazier Rehab</td>
<td>KY</td>
</tr>
<tr>
<td>Louisiana State University ALS Clinic</td>
<td>LA/MS</td>
</tr>
<tr>
<td>TidalHealth Peninsula Regional Medical Center</td>
<td>MD</td>
</tr>
<tr>
<td>Essentia Health Duluth ALS Clinic</td>
<td>MN</td>
</tr>
<tr>
<td>VA St. Louis Health Care System ALS Clinic</td>
<td>MO</td>
</tr>
<tr>
<td>Hackensack Meridian ALS Clinic at Jersey Shore University Medical Center</td>
<td>NJ</td>
</tr>
<tr>
<td>Lehigh Valley ALS Comprehensive Care Center</td>
<td>PA</td>
</tr>
<tr>
<td>Geisinger Medical Center ALS Clinic</td>
<td>PA</td>
</tr>
<tr>
<td>The Louise Wilcox ALS Center</td>
<td>RI</td>
</tr>
<tr>
<td>Greenville Health System</td>
<td>SC</td>
</tr>
<tr>
<td>Avera Brain and Spine Institute</td>
<td>SD</td>
</tr>
<tr>
<td>The ALS Clinic of the MidSouth</td>
<td>TN</td>
</tr>
<tr>
<td>Emory Bellard ALS Clinic</td>
<td>TX</td>
</tr>
</tbody>
</table>

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.
SUPPORT THROUGH EDUCATION

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 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 that 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.
Impact & Resources
CARE SERVICES IMPACT

People living 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.

21,545 people living with ALS served through the chapter network in the past year

9,296 people registered with our Chapter network & served through our Certified Treatment Centers of Excellence and Recognized Treatment Centers

12 CEU approved educational training sessions for healthcare providers

2,397 times our 12 monthly educational webinars were viewed live or on-demand

$470,000 in higher educational scholarships was awarded to 94 students through the Jane Calmes Scholarship Fund

2,295 veterans received assistance through our nationwide chapter network

12 videos addressing the role of the multidisciplinary team in respiratory care throughout the course of the disease

$4,885,387 in grants provided through our Certified Center Program

505,456 unique page views on the Care Services webpages on www.als.org

42,696 people viewed, downloaded, or ordered our educational materials including the Living with ALS and Families and ALS resource guides and medical information packets

25,831 attendees at support groups offered through our vast chapter network across the U.S.

190+ relationships with clinical partners, incorporating best practices as established by the American Academy of Neurology
FILLING THE GAP
In Education, Knowledge, and Understanding

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 this population. 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:

- 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.
- Informing caregivers about options to care for their loved ones, especially as the person living with ALS loses the ability to do so themselves.
- Educating medical professionals about ALS treatment best practices for those who have limited experience or who have never encountered the disease before.
- Creating public awareness of ALS with the general population to build support for the ALS Association’s many initiatives.
THE ALS ASSOCIATION’S LIVING WITH ALS RESOURCE GUIDES

The ALS Association’s Living with ALS Resource Guides are a module series that cover 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.

TRANSLATION OF RESOURCE GUIDES

The Hispanic, Spanish-speaking population in the United States reached 59 million people nationwide in 2017 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.
EDUCATION
Living with ALS: Medical Resources

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.

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.
EDUCATION

LIVING WITH ALS: CAREGIVERS RESOURCES

How-To Video Series for Caregivers

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 ones. 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.
ASSISTIVE TECHNOLOGY

Assistive Technology in the ALS Space

For some, ALS will completely take away their ability to communicate — verbally, written, bodily. 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.
CHILDREN’S PROGRAMS AND RESOURCES

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.

**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.

**FAMILIES AND ALS, CHILDREN’S AGE-BASED, AND AT-SCHOOL PROFESSIONAL RESOURCE GUIDES**

With no known cure and minimal known treatments, ALS can be a difficult subject to explain to children. These resource guides are 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.

Families and ALS — This guide was created primarily for families living with ALS, including parents, grandparents, siblings, and other family members. 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.

CHILDREN’S AGE-BASED GUIDES

Real Kids Talk About ALS: Feeling Normal, Sad, and Different - Graphic novel for youth, ages 7-12. Delivered in a graphic novel format, this book uses direct quotes from youth in the ALS research projects. These quotes help situate the learning style and experiences of children in ALS.

The ALS Experience: It’s Different and Hard — Choose your own adventure guide, middle school youth, ages 13-18. This book follows the stories of three youth, providing options for how they talked to people, who they talked to, and what was the outcome. The book allows other youth to choose who they might want to talk about ALS and their experiences. A companion online version has been developed.

School, Friends, Work, and ALS: A Young Adult Guide to Balancing Life with ALS — Older youth/young adult guide, ages 19-25. This book takes a more serious tone, again following several young adults as they manage their own lives with the care needed for their loved one with ALS, school and relationships. It is minimally illustrated and again uses actual stories and quotes.

At School: A Guide to Supporting Students Who Have Been Affected by ALS — These professionals recognize the limited information and the need to receive guidance in order to lessen the potential social isolation felt by kids in families with ALS — particularly in terms of how these issues affect school performance and attendance. This guide was created
to address the needs of school staff and personnel working with children impacted by ALS.

**ASSISTING WITH ACTIVITIES OF DAILY LIVING: DURABLE MEDICAL EQUIPMENT TRAINING**

Coping with ALS can be difficult for the many children 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.
THE JANE CALMES ALS SCHOLARSHIP FUND
Helping People Affected by ALS Continue Their Education

The ALS Association established the Jane Calmes ALS Scholarship fund to provide support for students affected by the financial hardship of living with ALS. Mark Calmes, vice chair of the ALS Association National Board of Trustees developed the fund in honor of his wife Jane, who lived with ALS for eight years until her passing in August 2017. This fund has allowed students to study a variety of majors, including medicine, biology, business and social services.

45 SCHOLARSHIPS WERE AWARDED IN 2019; 94 SCHOLARSHIPS WERE AWARDED IN 2020

“With the help from The Jane Calmes ALS Scholarship I will be able to finish my bachelor’s degree debt free and for that I am forever grateful.”

Chandler Brestel, studying healthcare management, University of Arkansas

In addition to the physical and emotional toll faced by people impacted by ALS, the financial strain of the disease can devastate a family’s ability to plan for future expenses. Families impacted by ALS often do not have the financial means to support the pursuit of college degrees or vocational certificates.

To learn more about the scholarship please visit www.als.org/get-involved/jane-calmes-als-scholarship-fund.

The Jane Calmes ALS Scholarship Fund is administered through Scholarship America.
ALS ASSOCIATION CARE CONNECTION

The Care Connection program is simple: it uses a private online calendar to bring together volunteers from the community — friends, neighbors and service groups — to provide help and support for people living with ALS and their families. For some families living with ALS, Care Connection is the only option to give loving caregivers a break from their day-to-day responsibilities.

Visit alsa.lotsahelpinghands.com/ to find tools that will help you organize a Care Connection Community for a family affected by ALS.

Step-by-step instructions assist a designated family Care Connection Coordinator with connecting the many family, friends, and community volunteers who want to help, with identified family needs.
CARE SERVICES STAFF

KIM MAGINNIS, SENIOR VICE PRESIDENT, CARE SERVICES

Kimberly Maginnis brings experience, energy, enthusiasm and an appreciation for respectful relationships to her role as Senior Vice President, Care Services, at The ALS Association. She has worked in the healthcare industry for more than three decades, and her resume includes positions at the Veterans Administration and Harvard Community Health Plan in Boston, Massachusetts.

Prior to joining The ALS Association, Maginnis worked at Inova Health System in Falls Church, VA for more than 20 years as senior director of Corporate Health Services, Urgent Care Centers and Employee Occupational Health. Some of her key accomplishments in this role include her developing successful, collaborative relationships with her colleagues in other departments; achieving income targets while managing risk and achieving strong operational controls; developing strategic business plans that yielded defined financial and operational successes.

Maginnis holds a B.S. in Hospital and Health Services Administration, Ithaca College, Ithaca, New York and an M.B.A. in Business Administration/Finance from American University, Washington, D.C.

LORI BANKER-HORNER, DIRECTOR, CLINICAL PROGRAMS

Lori has been working with the ALS Community for over 23 years. She began with the Southeast Wisconsin Chapter in 1997 and was instrumental in the growth of Care Services as the Chapter expanded to serving the entire state. The development of partnerships with ALS multidisciplinary clinics was key to that growth ensuring people living with ALS had access to the highest standard of care in the state. She served on several National ALS Association committees and task forces including the Care Services Committee and the Clinic Certification/Recertification Sub-team. Lori joined the National ALS Association in 2019 where she brings her experience to her role. In managing the National Certified Center Program, she works directly with both Chapters and ALS multidisciplinary clinics to obtaining and successfully maintaining designation as a Certified Treatment Center of Excellence or Recognized Treatment Center. She works closely with all mission areas including Research and Advocacy. Lori received her BA in Psychology and Adult Education from Alverno College. She has been a Licensed Practical Nurse for over 30 years serving in many areas of health care.
CYNTHIA KNOCHE, RRT, BBA, DIRECTOR, CHAPTER CARE SERVICES

Cynthia has served Association stakeholders since 2007 by providing consultation, guidance, and support to the Association’s nationwide network of chapters with respect to the wide range of care and support programs they deliver. Her clinical background as RRT, and management experience in the healthcare business arena support successful assessment and development of program services. Cynthia holds credentials as a Registered Respiratory Therapist with the National Board for Respiratory Care, is licensed as a RRT in Florida, and holds a Bachelor of Business Administration degree from the University of North Florida. Cynthia works closely with our advocacy mission team to support meaningful legislative and regulatory benefits for those living with ALS. In addition, Cynthia leads professional outreach efforts as an invited guest speaker, presenting to allied practice healthcare and service organizations on a state and nationwide level.

LESLIE RYAN, MSPT, DIRECTOR, EDUCATION AND PROFESSIONAL DEVELOPMENT PROGRAMS

Leslie joined the ALS Association in 2019. In her position, she oversees the development and implementation of educational materials and programs for people living with ALS, the community, and healthcare providers. She collaborates with partners across the association to ensure relevant and timely information is shared with the ALS community. Prior to joining the national office, Leslie worked at The ALS Association Rocky Mountain Chapter as the Care Services Director for 15 years. She was instrumental in building valuable care services programming as the chapter expanded its reach to cover 3 states and partnered with multidisciplinary clinics throughout the region. Leslie developed relationships with industry partners and helped to establish the high level of excellence the chapter is known for in the community. Serving on the national Care Services Committee and various task forces over the years gave her the opportunity to share her expertise throughout the association. She received her Bachelor of Science in Health Studies and a Master of Science in Physical Therapy from Boston University, and practiced clinical care in a variety of settings before transitioning to the nonprofit sector.
MIRIAM BRODKIN, MANAGER, RESOURCE CONNECTION

Miriam joined the ALS Association in 1997 after her mother passed away from ALS. It was important to her to help others with going through the same experience. In her role, she communicates with people living with ALS, their families, healthcare professionals, and the general public through email and phone inquiries. Miriam helps connect people to appropriate ALS Association chapter and community services and programs to help them address important-day-to-day decisions and to find solutions and options for managing the symptoms and journey of ALS. Miriam also helps people from other countries identify resources in their own communities.

ALISA BROWNLEE, ATP, CLIPP, CAPS, WSP, CLINICAL MANAGER, ASSISTIVE TECHNOLOGY SERVICES

Alisa is a 24 year staff member of the ALS Association working for both the Greater Philadelphia Chapter and the National Office. Alisa speaks, blogs, writes articles and uses social media to increase awareness of ALS and the use of assistive technology. Her specialty areas are communication, computer access, electronic aids for daily living and home modifications. She is a strong advocate for patient rights with elected officials, insurance companies, and other healthcare providers.

Alisa is 2000 graduate of the California State University Northridge with a Graduate Certificate in Assistive Technology. She has a Bachelor of Science Degree in Hospitality Management and a Bachelor of Arts Degree in History from East Stroudsburg University. She is a Certified Assistive Technology Professional (ATP) through the Rehabilitation Engineering and Assistive Technology Society of North America (RESNA) and a Certified Aging in Place Specialist (CAPS) through the National Association of Home Builders.

MARKEYA MARTIN COORDINATOR, MISSION AREAS

Markeya joined the ALS Association in 2018. In this role she assists with coordinating and planning for the three mission areas; Care Services, Advocacy/Public Policy, and Research. She also assists with organizing and overseeing major grants and programs provided. Prior to joining the national office, Markeya worked at LifeSpan Network in Columbia, Maryland as a grant coordinator. She received her Bachelor of Science in Community Health from Hofstra University.
“Our Advocacy

“It brought tears to my eyes when I saw the news about the bill. It was an honor to participate in ALS Advocacy Day.”

—Tabitha Wandell
ALS Advocate and Caregiver, Tennessee
Introduction To ALS Advocacy
INTRODUCTION

The ALS Association empowers advocates to take action on issues important to people living with ALS. Our goal is to educate policymakers and the public and to drive smart decisions related to research, treatments, and access to care that benefit people with ALS and their families.

We are the largest and most influential national advocacy organization in the United States focused solely on ALS. We are a nonpartisan organization committed to working across party lines to identify solutions for ALS. We empower and support people living with ALS to make a difference in the policy process to change laws, regulations, and policies.

Every day, policymakers at all levels of government make decisions that impact funding for ALS research and programs that serve people living with ALS. Our advocates educate and advocate with elected officials to make it an urgent priority to find cures and treatments and improve services for people living with ALS. The Association and advocates meet with members of Congress, call and write emails and letters to get their message across.

The ALS Association plans and directs strategic campaigns to achieve results:

- develop and promote legislation;
- develop and expand the number of congressional champions;
- lobby key members of Congress to improve services and increase research funding;
- engage Association chapters in lobbying their congressional delegations;
- mobilize a vibrant grassroots network; and
- partner with patient advocacy, physician and health professional organizations on cross-cutting issues such as pre-existing conditions.
HISTORY OF ALS ADVOCACY

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 through research, care services, public education, and public policy — giving help and hope to those affected by ALS.

We bring the ALS community together to speak with one voice and advocate on issues important to the community at the national level. This includes advocating before Congress, the White House, and with key federal agencies — including the National Institutes of Health (NIH), Department of Defense (DOD), the Centers for Disease Control and Prevention (CDC), the Centers for Medicare and Medicaid Services (CMS), the 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 approximately 40,000 advocates with the information, tools, and expertise needed to speak out and play an active role in the fight for treatments and a cure. In 2020, ALS chapters and advocates accomplished over 630 meetings with members of Congress. Many more meetings took place throughout the year in states and congressional districts. Throughout 2020, advocates mobilized through 20 action alerts that generated more than 85,000 messages to Congress.
TIMELINE OF EVENTS

In 2001, we successfully lobbied Congress to waive a 24-month wait for Medicare for people with ALS on Social Security Disability Insurance.

In 2003, we 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 (SSDI) and added ALS to the list of conditions that automatically qualify for presumptive disability payments under SSDI.

In 2006, we 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.

In 2007 we 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) at the Department of Defense (DOD) as part of a $5 million appropriation in the FY2007 Defense Appropriations Bill.

In 2008, we helped to 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.

We also led the fight to establish the National ALS Registry and Biorepository, which secured federal funding to design, build, and implement the Registry and Biorepository at the Centers for Disease Control and Prevention (CDC), the single largest ALS research project ever created.

In 2008 we helped to 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.

The ALS Association brought the ALS community together to create the first patient-focused guidance for ALS drug development ever submitted to the FDA. It has the potential to speed access, reduce costs, ensure more effective use of resources, and incentivize the pharma/biotech industry to enter the ALS market and develop new treatments for ALS.

We also worked with Congress to increase funding for the DOD ALSRP from $7.5 to $10 million, bringing the total funding for the ALSRP to nearly $80 million since the program’s inception in 2007.

In addition, we played a key role in securing a bill to protect access to customized wheelchairs and accessories that caused the Medicare program to reverse its decision to add these complex devices to the Competitive Bidding Program.

The ALS Association initiated and led the successful campaign to increase funding for the DOD ALSRP to $20 million — a $10 million increase — through direct lobbying, chapter advocacy, grassroots mobilization and engaging other ALS organizations.

The ALS Association spearheaded and co-led coalition efforts to protect access to noninvasive ventilators (NIV) in collaboration with physicians and clinicians. This included meetings with Medicare program officials and the introduction of the Safeguarding Medicare Access to Respiratory Therapy (SMART) Act of 2019.

In 2012 we 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.

In 2016 we 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.

In 2017, The ALS Association brought the ALS community together to create the first patient-focused guidance for ALS drug development ever submitted to the FDA. It has the potential to speed access, reduce costs, ensure more effective use of resources, and incentivize the pharma/biotech industry to enter the ALS market and develop new treatments for ALS.

We also worked with Congress to increase funding for the DOD ALSRP from $7.5 to $10 million, bringing the total funding for the ALSRP to nearly $80 million since the program’s inception in 2007.

In addition, we played a key role in securing a bill to protect access to customized wheelchairs and accessories that caused the Medicare program to reverse its decision to add these complex devices to the Competitive Bidding Program.

The ALS Association initiated and led the successful campaign to increase funding for the DOD ALSRP to $20 million — a $10 million increase — through direct lobbying, chapter advocacy, grassroots mobilization and engaging other ALS organizations.

The ALS Association spearheaded and co-led coalition efforts to protect access to noninvasive ventilators (NIV) in collaboration with physicians and clinicians. This included meetings with Medicare program officials and the introduction of the Safeguarding Medicare Access to Respiratory Therapy (SMART) Act of 2019.

In 2018, The ALS Association led the charge that secured $10 million in funding for the DOD ALSRP and $10 million for the CDC National ALS Registry and Biorepository.

We also secured the first-ever Senate “Dear Colleague” letter circulated for signature by senators and sent to the Appropriations Committee supporting funding for the DOD ALSRP and the CDC National ALS Registry and Biorepository.

In 2019, In 2020, we initiated and led the successful campaign to boost funding for the DOD ALSRP from $20 to $40 million.

We also ended the SSDI 5-month waiting period for people living with ALS by spearheading successful efforts to pass and enact into law the bipartisan ALS Disability Insurance Access Act.

In addition, we protected access to noninvasive ventilators (NIV) for Medicare beneficiaries with ALS by fighting the Medicare program’s decision to add NIV to the Competitive Bidding Program.
Advocacy In Action
ADVOCACY GUIDING PRINCIPLES

As legislative, regulatory, and other policy proposals are considered, they must be measured against the mission and values of The ALS Association: 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.

The ALS Association’s public policy priorities are grounded in our advocacy guiding principles which maintain that policy proposals must preserve and/or enhance health care, benefits, and services for those who have it, provide coverage to those who do not, and lower costs and improve quality for all. This must be achieved at the federal and state level and in both public and private settings. Research funding must be preserved and increased, people with ALS must not be discriminated against, and resources and support for caregivers must be increased.

PRINCIPLES

• **Protect and expand access to health care, benefits, and services.** People with ALS and their families must have health care that is accessible, affordable, adequate and understandable. The ALS Association is committed to fighting for people with ALS on every front to break down barriers to health care, benefits, and support services.

• **Preserve and increase support for ALS research.** Funding and support must be provided for research in all phases to discover new life-extending therapies and technologies for people with ALS. The ALS Association is dedicated to ensuring laws, regulations, and incentives relating to the therapy development process effectively and expeditiously promote new interventions and, ultimately, a cure for ALS.

• **Fight for people living with ALS on a wide range of issues.** People with ALS should be protected against discrimination in all settings, including in the workplace, when purchasing health insurance, or receiving veterans’ benefits. The ALS Association monitors legislation and regulation regarding health care, disability rights, taxation, and more to ensure the ALS community is represented.

• **Increase resources and support for families and caregivers of people with ALS.** Families and caregivers are the lifeline for people with ALS and must be recognized and supported. The ALS Association believes federal, state, and private programs must do more for families and caregivers in terms of programs, supports, and financial aid.
HOW WE ADVOCATE

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 over 40,000 grassroots advocates is mobilized regularly through action alerts to contact their elected officials on ALS priorities. These volunteers also come together for conferences and signature events throughout the year to discuss and learn about these priorities and advocate directly with lawmakers. Once someone enrolls as an advocate through our action center, they can engage at key moments 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. Coalitions allow us to leverage the combined expertise of the broader patient advocacy community to make sure the ALS perspective is accounted for when decisions are made. 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 ASSOCIATION’S CHAPTER NETWORK IN ADVOCACY

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 ALS 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 the ALS community’s priorities. Leaders at the chapter level also serve as our grassroots advocates — building the Association’s long-term relationships with members of Congress and their staff 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 an action center on our website that allows us to reach out to more than 40,000 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 email to their members of Congress.

ANNUAL ASSOCIATION CHAPTER FLY-IN

Every year, The ALS Association hosts an event where our chapter executives and advocate leaders get a chance to participate in a day of meetings and training 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 meet with lawmakers and their staff to discuss our priorities.

NATIONAL ALS ADVOCACY CONFERENCE

Every summer, the Association holds its largest public conference providing advocates from across the country an opportunity to come together to network, share their stories, and meet with members of Congress. People with ALS come with their family members and chapter staff to learn about ALS public policy priorities and what Congress needs to do. The conference also provides program sessions to update the community on the latest research and care services innovations. Almost 1,000 people attended this event virtually in 2020, including over 260 people with ALS — the most to ever participate.

OPPORTUNITIES FOR ADVOCACY AT SIGNATURE EVENTS

In addition to the Association Fly-In and National ALS Advocacy Conference, The Walk to Defeat ALS provides an excellent opportunity where chapters expand their roster of advocates by engaging a large audience of people who already have an interest in fighting ALS. Advocacy tents or tables serve as gathering spaces where chapters sign-up new advocates and reengage returning advocates. Chapters also 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 AND BIOREPOSITORY**

The ALS Association led the fight to establish the National ALS Registry and Biorepository 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 to begin identifying cases of ALS on a nationwide basis and collecting information urgently needed for ALS research – work that continues to this day.

The Registry and Biorepository are 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 a repository of data for scientists, and empowers patients to make invaluable contributions to a future freed from ALS. In January 2017, ATSDR launched the National ALS Biorepository as part of the Registry to enable researchers to request samples from both living and deceased (post-mortem) persons with ALS.

**ASSOCIATION COLLABORATION WITH FEDERAL AGENCIES**

The ALS Association has played a pivotal role in expanding the CDC’s investigations into ALS prevention and environmental risk factors, with the ultimate goal of translating CDC’s early stage research into a more definitive list of causes and preventing future cases of ALS.
Priorities, Impacts & Coalitions
ADVOCACY IMPACT

With the support of over 40,000 ALS advocates, we secure resources to support treatment and research for the entire ALS community. Our nationwide network of 39 chapters and two service areas are essential to our efforts at the federal, state and local levels in advancing 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 recent successes in ALS advocacy!

**Protected access to noninvasive ventilators (NIV) for Medicare beneficiaries with ALS** by fighting the Centers for Medicare and Medicaid Services (CMS) decision to add NIV to the competitive bidding program.

**Engaged the ALS community in a campaign to bring AMX0035 to people living with ALS as quickly as possible** by collecting over 50,000 signatures on a petition to FDA and Amylyx.

**Joined with other leading patient organizations in the fight to preserve protections for pre-existing conditions for people with ALS** under all insurance plans, including Medicare, Medicaid, and private insurance.

**Supported over 630 meetings with members of Congress and over 500 calls to Congress** during the 2020 Virtual Advocacy Fly-In and National ALS Virtual Advocacy Conference. **Empowered more than 40,000 ALS advocates** through 20 action alerts — resulting in over 85,000 advocacy messages to Congress.

**Ended the SSDI 5-month waiting period for people living with ALS** by spearheading successful efforts to pass and enact into law the bipartisan ALS Disability Insurance Access Act.

**Boosted funding for the ALS Research Program** at the Department of Defense (DOD) from $20 to $40 million in 2020 to support the creation of more clinical trials that will lead to the discovery of treatments and a cure.

**Ensured $10 million in continued funding for the National ALS Registry and Biorepository** to identify genetic and environmental factors for ALS, provide support to researchers to find treatments and a cure, and promote access to clinical trials.

**Supported increases in funding for the National Institutes of Health**, resulting in increased funding for ALS research from $83 million in fiscal year 2018 to $105 million in fiscal year 2019.
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 where there are shared concerns.

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 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 and cross-cutting issues that impact patients.

COLLABORATIONS WITH OTHER ALS ORGANIZATIONS

The ALS Association works in collaboration with a number of organizations, including the Muscular Dystrophy Association, I AM ALS, Team Gleason, and Les Turner ALS Foundation.

MEMBERSHIP ORGANIZATIONS

- **National Health Council (NHC):** 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 drugs, health insurance, device, biotechnology, and communications industries.
- **Consortium for Citizens with Disabilities (CCD):** CCD is the largest coalition of national organizations working together to advocate for federal public policy that ensures the self-determination, independence, empowerment, integration and inclusion of children and adults with disabilities in all aspects of society.

- **Defense Health Research Consortium (DHRC):** Comprised of more than two dozen diverse organizations, DHRC focuses on continued Congressional support for dozens of 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).

- **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.

- **Partnership to Protect Coverage:** A coalition of the nation’s leading patient advocacy organizations which have collectively opposed health care reform legislation that would negatively impact patients. The coalition led efforts on the repeal/replacement efforts on the Affordable Care Act (ACA) and favors a bipartisan approach to reforming the healthcare system.

- **Alliance for a Stronger FDA:** The Alliance for a Stronger FDA works to ensure annual appropriations that will adequately fund the FDA’s essential missions. A science-based and effective FDA supports biomedical innovations as well as approves safe and effective drugs and medical devices for people living with ALS and all Americans. The Alliance unites a broad and diverse spectrum of patient groups, consumer advocates, biomedical researcher, health professionals and companies.

**INFORMAL COALITIONS IN WHICH THE ALS ASSOCIATION PARTICIPATES:**

- **Independence Through Enhancement of Medicare and Medicaid (ITEM) Coalition:** The ITEM Coalition is devoted to raising awareness and building support for policies that will enhance access to assistive devices, technologies, and related services for people with disabilities and chronic conditions. The coalition is consumer-led and includes a diverse set of disability organizations, aging organizations, other consumer groups, voluntary health associations, and non-profit provider associations. The ALS Association is a member of ITEM’s steering committee.

- **Safeguarding Medicare Access to Respiratory Therapy (SMART) Coalition:** The coalition was formed to protect
access to non-invasive ventilators with support from respiratory therapists in the Medicare program. A majority of people living with ALS require access to ventilators at some point to maintain their quality of life. Working with national associations of respiratory physicians and therapists, the Association drafted and lobbied for immediate passage of Safeguarding Medicare Access to Respiratory Therapy (SMART) Act (H.R. 4945).

- **Ad Hoc Group for Medical Research**: Ad Hoc Group for Medical Research is a coalition of patient and voluntary health groups, medical and scientific societies, academic and research organizations, and industry that support enhancing the federal investment in the biomedical, behavioral, and population-based research conducted and supported by the NIH.

- **Medicare Access for Patients Rx (MAPRx)**: MAPRx is a coalition of patient, family caregiver and health professional organizations committed to safeguarding the well-being of patients with chronic diseases and disabilities who rely on Medicare Prescription Drug Coverage. MAPRx members represent every segment of Medicare beneficiaries and join together to advocate on their behalf and collaborate with national and state policymakers to ensure all beneficiaries have access to needed medications.

- **Alliance for Connected Care**: Alliance for Connected Care was formed to create a statutory and regulatory environment in which every provider in America is permitted to deliver and be adequately compensated for providing safe, high quality care using connected care (e.g. telemedicine and telehealth) at their discretion, regardless of care delivery location or technological modality. Members include leading companies from across the health care and technology spectrum, representing insurers, retail pharmacies, technology and telecommunications companies, and health care entrepreneurs. The Alliance is advised by a distinguished group of patient and provider organizations.
• **Partnership to Improve Patient Care (PIPC):** PIPC is at the forefront of applying principles of patient-centeredness to the nation’s health care system – from the generation of comparative clinical effectiveness research at the Patient-Centered Outcomes Research Institute (PCORI), to the translation of evidence into patient care in a manner that achieves value to the patient. Having driven the concepts of patient-centeredness and patient engagement in the conduct of research, PIPC looks forward to bringing the voices of patients and people with disabilities to the discussion of how to advance patient-centered principles throughout an evolving health care system.

• **National Coalition for Assistive & Rehab Technology (NCART):** NCART is a national organization of suppliers and manufacturers of Complex Rehab Technology (CRT) products and services used by individuals with significant disabilities and chronic medical conditions. NCART seeks to ensure these individuals have adequate access to CRT products and supporting services. In pursuit of that goal, NCART works with consumers, clinicians, and physicians along with federal, state and private policy makers to establish and protect appropriate coverage, coding, supplier standards, and funding policies.

• **Medical Expense Deduction (MedEx) Coalition:** MedEX is an AARP-convened coalition of more than 60 organizations that supports retention of the medical tax deduction that allows taxpayers to claim an itemized deduction for high out-of-pocket medical expenses.
ADVOCACY STAFF

KATHLEEN SHEEHAN, VICE PRESIDENT, PUBLIC POLICY

Kathleen 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, Kathleen 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 possesses expertise in public policy issues related to the development and approval of pharmaceuticals at the Food and Drug Administration.

ABRAM BIELIAUSKAS, ASSOCIATE DIRECTOR, GOVERNMENT AFFAIRS

Abram joined The ALS Association in 2017 and works with Kathleen Sheehan to lobby Capitol Hill on the Association’s various public policy priorities. Mr. Bieliauskas previously 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 US House and Senate. He is 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.
ASHLEY SMITH, ASSOCIATE DIRECTOR, GRASSROOTS ADVOCACY

Ashley designs and directs nonpartisan grassroots activities to advance The ALS Association’s priorities and mission. Prior to joining The ALS Association, Ashley led the grassroots efforts of the National League of Cities (NLC) by crafting advocacy strategies that supported NLC’s federal policy agenda and empowered city leaders to engage directly with federal elected officials. At M+R, she facilitated state- and federal-level advocacy activities for clients including the American Lung Association, the MacArthur Foundation, and the American Public Health Association. Ashley earned her BA in Political Science and American Studies from the University of Kansas and an MA in Government, with a Political Communications Concentration, from Johns Hopkins University.

ADAM BAKER, MANAGER, PUBLIC POLICY INITIATIVES

Adam serves as the liaison to the Center for Disease Control (CDC) and oversees all aspects of The ALS Association’s contract relating to the promotion of the National ALS Registry, as well as other issues concerning disease surveillance. He has served in this role since April 2018. Prior to his time at the Association, Adam worked for Senator Jon Tester and the Democratic Senatorial Campaign Committee. He earned his BA in English and Philosophy from Kenyon College.
ABBREVIATED GLOSSARY OF SCIENTIFIC TERMS

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.

D

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.

**E**

**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.

**F**

**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).

**G**

**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.

**N**

**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**: 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.

**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.

**Upper Motor Neurons**: Nerve cells (motor neurons) originating in the brain’s motor cortex and running through the spinal cord.

**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 glossary online, found at [http://www.alsa.org/research/our-approach/glossary.html](http://www.alsa.org/research/our-approach/glossary.html)