PACKARD CENTER

ALS Research at Johns Hopkins

2024 Impact Report

www.packardcenter.org

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Dear Friends,

The network of the Robert Packard Center for ALS Research at Johns Hopkins, currently encompassing nearly 200 leading scientists and clinicians from 61 institutions across nine countries, is dedicated to advancing the understanding of ALS and related neurodegenerative diseases to develop effective therapies and biomarkers for individuals affected by ALS. We do this via our unrelenting focus on our mission: To empower collaborative and breakthrough research that advances our fundamental understanding of ALS by engaging and supporting the global research community.

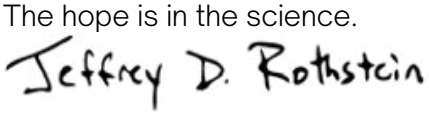
Over the past two and a half decades, the Packard Center has contributed to most fundamental and critical breakthroughs in ALS research, including the development of FDA-approved treatments and the data and models that are the foundation for future therapy discovery. As a community, we have been ecstatic to witness the immense progress in the field, with the recent success of Qalsody as a gamechanger for SOD1 families. But we know the work is not done. There are many more people affected with ALS that deserve effective treatment options. With the trend toward decreased federal funding for biomedical research, the Packard Center is essential to sustaining the momentum gained in ALS research.

For the past 24 years, the Packard Center has grown from a handful of ALS researchers into a global force, in aggregate of over 500 scientists and clinicians, a self-supporting entity that has propelled the understanding of ALS to unprecedented heights. Our scientists and research partners from across the globe work tirelessly on promising fronts to unravel the biology underlying ALS via synergistic and collaborative research. The foundational principle of the Packard Center is that the best therapies will ultimately come from understanding the root causes of the disease. The Packard Center remains steadfast in its dual role as a catalyst for groundbreaking advancements and a convener of the global ALS research community.

Over the next decade and beyond, the Packard Center will continue to innovate biomedical research for ALS and is intent on embracing AI and machine learning approaches to advance our collective understanding of the cell biology of ALS. We will continue to support the global ALS research community via our catalyst grant program, annual symposium, and monthly investigator meetings. In addition, we are standing up new partnerships and launching strategic initiatives that serve to empower our collective commitment to the early-stage, foundational research that is needed to guide future therapies and biomarkers for people living with ALS.

We believe that great innovation comes from great collaboration. This includes collaborations with you – our supporters. It is critical to achieving our vision to significantly alter the course of ALS giving those diagnosed the opportunity to live long and healthy lives.

The hope is in the science.



Jeffrey D. Rothstein, MD, PhD
Executive Director and Founder

Christine Vande Velde, PhD
Scientific Director

About the Packard Center

The Robert Packard Center for ALS Research at Johns Hopkins is an international consortium of researchers committed to facilitating collaboration and advancing our understanding of ALS. The Center has established an aggressive, multidisciplinary approach to unlock the disease through a unique model for inter-institutional cooperation, engaging leaders of the global scientific community to accelerate research of the highest quality. Through cutting-edge science, education, and robust partnerships, Packard researchers work together to advance discovery and move fundamental research towards treatments for ALS.

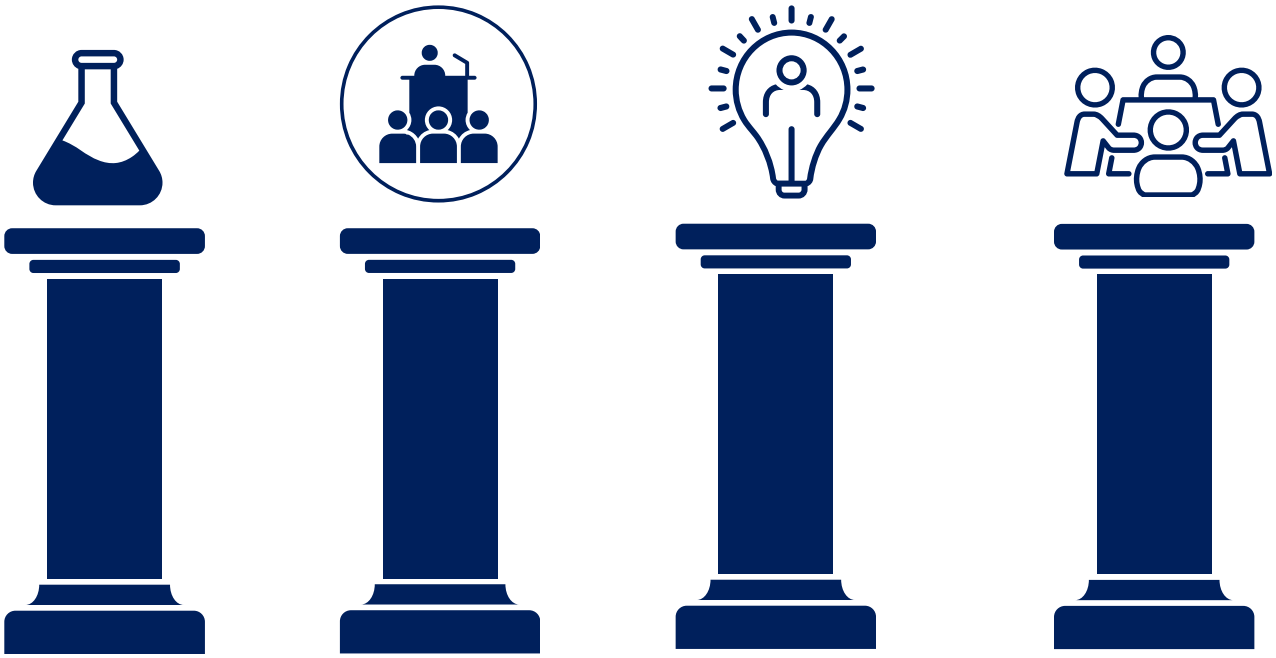
Vision

To significantly alter the course of ALS giving those diagnosed the opportunity to live long and healthy lives.

Mission

To empower collaborative and breakthrough research that advances our fundamental understanding of ALS by engaging and supporting the global research community.

Four Pillars of the Packard Center



Funding Research Grants

Annual Research Symposium

Strategic Initiatives, *including support for the next generation of researchers*

Monthly Collaborative Investigator Meetings

"For 25 years, the Packard Center has been driving the field's understanding of the fundamental biology that underlies ALS. Through the efforts of many collaborative investigators and their teams, we have seen outstanding advances in the development of new models with which to study the disease, the exploration of innovative biomarkers with which to track disease progression, and foundational early stage work to advance novel therapeutic approaches for people living with ALS."

Christine Vande Velde, PhD
Packard Center Scientific Director

The Hope is in the Science

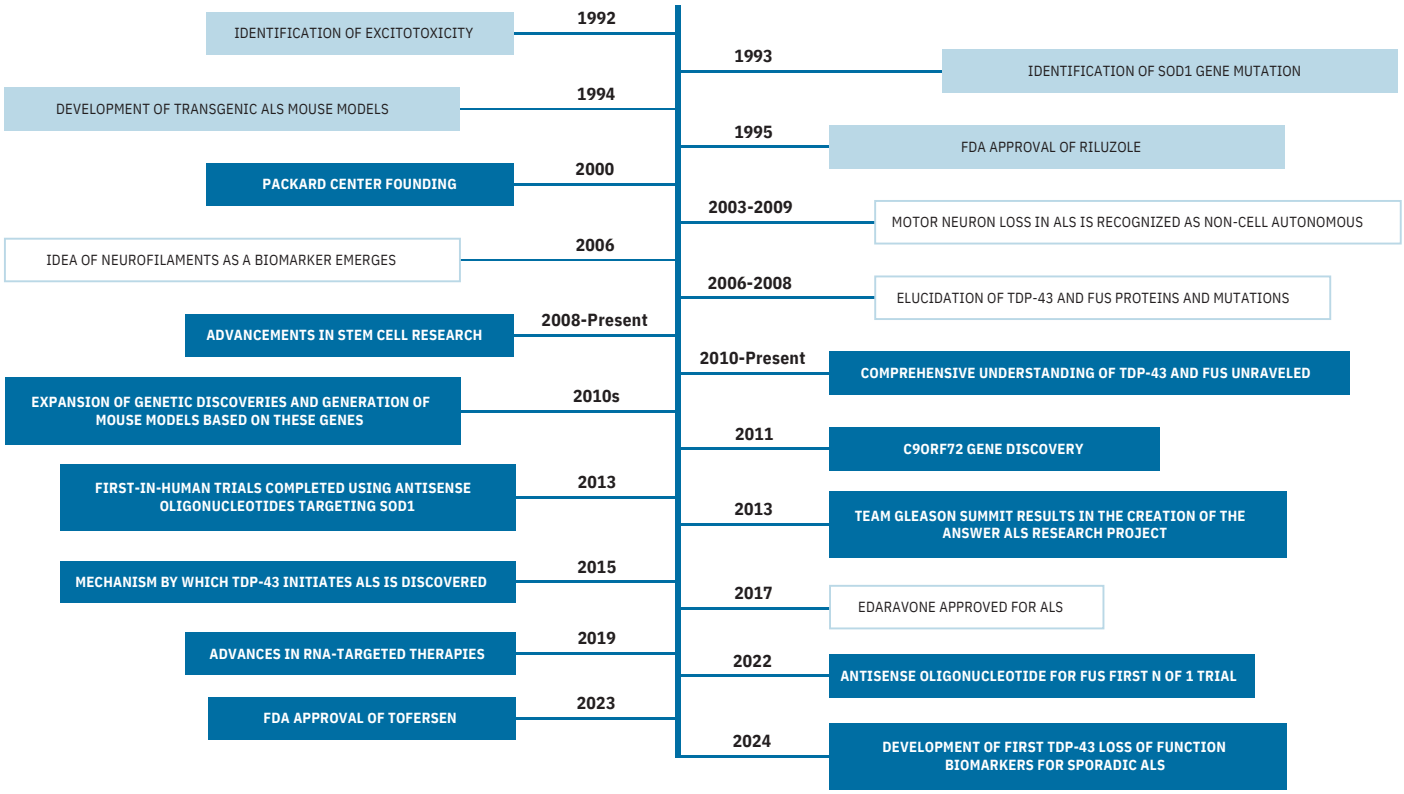
ALS has been studied for more than 150 years, yet most of the progress we've made in understanding and treating the disease has only materialized in the last 30 years. The advances of each successive decade have built on those of the previous in both frequency and impact.

Since 2000, the Packard Center has been a leading player in advancing ALS research and collaborative, investigator-led initiatives. Over the past 24 years, the Center has funded research that spans:

- **179 Total Projects**
- **300 Researchers & Trainees at Work**
- **61 Institutions**
- **9 Countries**
- **24 Years of Progress**

Significant Milestones in ALS Research

Over the past two and a half decades, the Packard Center has contributed to many critical breakthroughs in ALS research. It continues to catalyze the highest quality of research to advance our understanding of ALS. Over the next decade and beyond, the Packard Center will champion advances in science from cellular biology to AI and machine learning, with the belief that great innovation comes from great collaboration.



Discoveries affiliated with the Packard Center are highlighted in light blue. Advancements driven by Packard Center investigators are highlighted in dark blue.

"The Packard Center has been very important for my research program. When I first joined this relatively small group, my interest was mostly in axonal degeneration and PNS diseases. But I quickly expanded to what I do now, likely because I found the Packard Center environment and atmosphere intellectually stimulating, challenging, and supportive."

Jonathan Glass, MD
Emory University

Scientific Advisory Board

The Packard Center Scientific Advisory Board (SAB) is tasked with providing strategic advice to the Center leadership with respect to the research portfolio and other activities of the Packard Center to enable high impact ALS research. The SAB serves as a connection between the ALS research community and the Packard Center and to promote the mission of the Packard Center.

Sami Barmada, MD, PhD
University of Michigan

Lucie Bruijn, PhD - Emeritus
Novartis

Don W. Cleveland, PhD
University of California, San Diego

Christopher Donnelly, PhD
University of Pittsburgh

John Dunlop, PhD
Neumora

Aaron Gitler, PhD - Emeritus
Stanford University

Jonathan Glass, MD
Emory University School of Medicine

Lindsey Hayes, MD, PhD
Johns Hopkins University

Ahmet Hoke, MD, PhD
Johns Hopkins University

Evangelos Kiskinis, PhD
Northwestern University

Clotilde Lagier-Tourenne, MD, PhD
Mass. General Hospital/ Harvard Medical School

Thomas E. Lloyd, MD, PhD
Baylor College of Medicine

Nicholas Maragakis, MD
Johns Hopkins University

Timothy Miller, MD, PhD
Washington University School of Medicine in St. Louis

Piera Pasinelli, PhD
Thomas Jefferson University

Leonard Petrucelli, PhD
Mayo Clinic

Magdalini Polymenidou, PhD
University of Zurich

Laura Ranum, PhD
University of Florida College of Medicine

Rita Sattler, PhD
Barrow Neurological Institute

Sangram Sisodia, PhD - Emeritus
University of Chicago

Charlotte Sumner, MD
Johns Hopkins University

Leslie Thompson, PhD
University of California, Irvine

Davide Trotti, PhD
Thomas Jefferson University

Ludo Van Den Bosch, PhD
VIB-KU Leuven Center for Brain & Disease Research

Frank S. Walsh, PhD - Emeritus
Ossianix Inc.

Jiou Wang, MD, PhD
Johns Hopkins University

Michael E. Ward, MD, PhD
National Institutes of Health

Philip C. Wong, PhD
Johns Hopkins University

Daniela Zarnescu, PhD
Penn State College of Medicine

Packard Center Research Portfolio

The Packard Center funds innovative, high-risk ALS research often overlooked by traditional funders. Scientific leadership identifies researchers with novel approaches, requests that they present at a monthly Investigator meeting, and invites them to apply for a grant. Proposals are reviewed by the Science Advisory Board and Scientific Director, and, if approved, funding is awarded. Funded researchers are required to present at the Annual Packard Center ALS Research Symposium.

Bahareh Ajami, PhD (Oregon Health & Science University)

Microglia regulation of selective neuronal vulnerability associated with ALS
Motor neurons are the cells affected in ALS. In most cases, group of motor neurons controlling eye movements are spared. It is unclear why some motor neurons are selectively spared in ALS. Microglia, the resident immune cells of the brain, directly interact with motor neurons and have been implicated in ALS pathogenesis. This project will investigate the role of microglia in selective motor neuron death in ALS with the goal of providing a potential target for novel therapeutic strategies.

Daryl Bosco, PhD (Univ of Massachusetts Chan Medical School)

Investigating stress response mechanisms in human ALS-TDP neurons
The Bosco laboratory is studying stress as an upstream trigger of neuron death in ALS. These studies are based on observations that neurons in ALS patients become overactivated because of altered neuronal connections and metabolite imbalances within the central nervous system. However, the factors that cause ALS neurons to be more vulnerable to internal and external stressors are unclear. This research aims to understand the effects of stress on human neurons in healthy and disease contexts.

Claire Clelland, MD, PhD (University of California, San Francisco)

CRISPR gene therapy for C9orf72 FTD/ALS
This project aims to determine the percentage of edited cells and the timing of intervention that eliminate cellular markers of pathology in iPSCs and iPSC-derived neurons harboring a C9orf72 repeat expansion. The study will also work to determine the most efficient particle class to delivery CRISPR machinery in post-mitotic neurons in vitro using the team's novel screening platform. The potential impact is a universal therapeutic approach for all C9-carriers.

Don Cleveland, PhD (University of California, San Diego)

Dissecting C9ORF72 ALS/FTD and sporadic ALS using single cell spatial transcriptomics

ALS is a fatal neurodegenerative disease with no cure. In most instances, the cause of neurodegeneration remains unknown. To identify underlying disease mechanism for design of disease therapy, this project will use a newly developed, potentially transformative, imaging approach to determine in every cell of normal or ALS-derived human tissue sections the level of expression of more than a thousand selected genes, acquired ALS-associated DNA mutations, and accumulation of disease associated proteins.

Michael Coleman, PhD (University of Cambridge)
Ahmet Hoke, MD, PhD (Johns Hopkins University)

Compensatory effects preventing SARM1-dependent neuron and axon death

This research project focuses on a protein called SARM1, whose removal strongly protects nerve cells. Rare mutations that hyperactivate SARM1 are enriched in ALS, and additional evidence suggests its broader involvement in the disease, establishing it as an important drug target. The data indicate that natural mechanisms may compensate for active SARM1, enabling cells—and patients—to survive for several decades. The aim is to identify these compensatory mechanisms to enhance them for new therapies and to uncover modifiable risk factors that interact with SARM1, which could potentially be avoided.

Luc Dupuis, PhD (INSERM)

Consequences of NUP50 mutation or loss of function in amyotrophic lateral sclerosis

The team at INSERM has recently identified mutations in NUP50, that is one protein of the nuclear pore, in patients with ALS. The goal of this project is to understand how these mutations impact on the functioning of motor neurons and whether they could lead to motor neuron degeneration. To this aim, the team will generate novel human cell models and study at multiple levels the consequences of NUP50 mutations.

Adrian Issacs, PhD (University College London)

Lipid desaturase overexpression to ameliorate neurodegeneration in C9orf72 repeat mice

A mutation in a gene called C9orf72 is the most common genetic cause of ALS. In the team's initial work in fruit flies and in human nerve cells, they found that the C9orf72 mutation changes the levels of specific molecules called lipids and that restoring proper lipid levels can prevent nerve cell loss. The team now propose to test whether lipid restoration can also prevent nerve cell loss in a C9orf72 mouse model, a critical step in determining whether lipids are a viable therapeutic target for ALS.

Albert La Spada, MD, PhD (University of California, Irvine)
Sandrine Da Cruz, PhD (KU Leuven)

Alternative polyadenylation-driven subcellular RNA mislocalization in TDP-43 proteinopathies

TDP-43 is central to ALS pathogenesis and regulates where a mRNA transcript terminates by selecting the polyadenylation site. This results in RNAs with 3' untranslated regions (3'UTRs) of different lengths. Localization of RNAs to specific subcellular regions (e.g. axon) affects neuron function and is regulated by the 3'UTR. This project will test if TDP-43 dysfunction promotes RNA mislocalization due to "alternative polyadenylation", which could reveal novel pathways of ALS pathogenesis.

John Landers, PhD (Univ of Massachusetts Chan Medical School)

Development of Mutant Specific ASOs for TDP-43 and KIF5A

Gene-based therapeutics, including gene delivery and RNA interference (RNAi)-based therapies, have made tremendous advances over the past several years towards the development of therapeutics for ALS/FTD and numerous other diseases. The objective of this application is to develop potential therapeutics for ALS/FTD resulting from mutations in the TARDBP and KIF5A genes.

Payam Mohassel, MD, PhD (Johns Hopkins University)

Cellular and animal models of SPTLC1-related juvenile ALS

A new genetic form of ALS that stems from mutations that cause excessive activity of an enzyme called serine palmitoyltransferase has recently been discovered. This study seeks to create cellular and animal models of this new form of ALS. It also aims to understand how abnormalities in this enzyme cause motor neuron death. These studies will help to identify and test out candidate therapies in the future. ***This project was made possible through a joint grant with the Live Like Lou Foundation.***

Stephanie Moon, PhD (University of Michigan)

VCP-mediated regulation of neuronal RNA localization

This research focuses on how genetic causes of ALS disrupt the RNA biology of the stress response. In the human body, trillions of cells are programmed to adjust the amount and location of RNA molecules in response to stress. By utilizing microscopy and molecular biology approaches, the study seeks to understand how RNA regulation becomes impaired in brain cells under the stressful conditions associated with ALS.

Rita Sattler, PhD (Barrow Neurological Institute)

Loss of Sparcl1 in C9orf72 ALS/FTD-mediated neurodegeneration
The goal of this project is to explore defective communication between neurons in ALS/FTD. Neuronal connections, called synapses, are predominantly regulated by another important cell type of the brain, called astrocytes. While it has been shown that astrocytes are important in neuronal synapses formation during development, is not yet understood if and how astrocytes contribute to the synaptic dysfunction in ALS/FTD. Specifically, we will assess the role of astrocyte secreted protein Sparcl1.

James Shorter, PhD (University of Pennsylvania)

Pharmacological stimulation of Hsp70 disaggregases for ALS
There are no effective treatments for amyotrophic lateral sclerosis (ALS). In the vast majority of ALS cases, the nerves that control movement fail to stop the aberrant clumping and deactivation of a protein called TDP-43, which causes these nerves to die. Here, drugs that enhance ability of the nerves to declump and reactivate TDP-43 will be isolated. Ultimately, the project team envisions a therapeutic strategy whereby drugs stimulate the declumping and reactivation of TDP-43 in ALS.

Peter Tessier, MD, PhD (University of Michigan)

Toward bispecific antibodies for non-invasive monitoring of ALS disease progression: neuronal cell-surface target identification and pre-clinical testing
The project aims to develop multifunctional antibodies as imaging agents for non-invasively monitoring the progression of ALS. These antibodies can be administered into the bloodstream, pass through the blood-brain barrier, and selectively label neurons that are harmed during disease. This project will identify novel neuronal cell-surface biomarkers of ALS disease progression and evaluate first- generation multifunctional antibodies for monitoring disease progression in mouse models of ALS.

Jiou Wang, MD, PhD (Johns Hopkins University)

New Pathways of Protein Homeostasis in ALS
This study is aimed at elucidating a new mechanism of sensing misfolded proteins and regulating their synthesis related to ALS. The outcome could have important implications for understanding protein homeostasis as a common theme of the disease. The findings will not only provide novel insights into the pathogenic mechanisms in ALS but also identify new therapeutic targets for this devastating disease. **This project was made possible through a joint grant with the Muscular Dystrophy Association's Wings Over Wall Street.**

Sai Zhang, PhD (University of Florida)

Deciphering genetics of cellular heterogeneity in ALS
The research investigates how multiple cell types in the motor cortex and spinal cord contribute to ALS pathogenesis, with a specific focus on the unknown mechanisms by which genetic risk variants drive this cellular diversity. Using AI algorithms, the study will analyze extensive ALS genetic and single-cell data to identify critical variants, genes, and cell types that influence ALS risk and related phenotypes, such as age of onset and progression rate. This work aims to advance the understanding of ALS biology and guide the development of new therapies.

16 Research Grants Awarded

Total Amount Awarded: $1.2 Million

19% Awarded Internationally

2024 Highlights: Driving Progress

In 2024, the Robert Packard Center for ALS Research at Johns Hopkins made headlines with groundbreaking discoveries and impactful initiatives in the fight against ALS. The following pages showcase key stories that reflect our commitment to innovation, collaboration, and progress in ALS research.

Rothstein Elected to National Academy of Medicine

Jeffrey D. Rothstein, MD, PhD, Packard Center Founder and Executive Director, has been elected to the National Academy of Medicine (NAM), an independent organization of leading professionals from multiple scientific fields including health, medicine and the natural, social and behavioral sciences. NAM serves alongside the National Academy of Sciences and National Academy of Engineering to provide objective advice for the nation and international scientific communities.

Rothstein was elected along with fellow Hopkins colleague Christopher Chute, MD, PhD, a Bloomberg Distinguished Professor of Health Informatics. An announcement of 100 new members was made October 21, 2024.

Being elected to NAM is considered one of the highest honors in health and medicine. Current members elect new members based on their major contributions to advancements in medical science, health care and public health.

New Research Expands the Toolbox Available to Target C9orf72 Repeats

In a paper published in September 2024 in *The Journal of Clinical Investigation*, Packard Center investigators and members of the Center's Scientific Advisory Board, Lindsey Hayes, MD, PhD and Jiou Wang, MD, PhD showed important proof-of-principle experiments that C9orf72 expansions, which exhibit higher-order secondary structures that can be difficult to target, are amenable to genome editing using a newly engineered approach.

A C9orf72 repeat expansion is a genetic mutation where a hexanucleotide sequence (GGGGCC) is abnormally repeated hundreds or thousands of times in a non-coding region, compared to fewer than 30 repeats in healthy individuals. It is a key focus in ALS research.

Importantly, the new and improved approach demonstrated a positive correlation between the excision of the GGGGCC repeats that are characteristic of C9orf72 expansions, and reduced levels of dipeptide repeat (DPR) proteins. While the prevailing literature suggests that DPRs may not be a pathogenic driver of disease, they do represent an important biomarker by which to assess the GGGGCC repeat. This work, funded in part by the Packard Center, expands the toolbox available to target C9orf72 repeats that are causative of a subset of ALS and FTD.

Packard Center Hosts Baltimore's first ALS Clinical Research Learning Institute®

In September 2024, the Robert Packard Center for ALS Research hosted its first ALS Clinical Research Learning Institute® (CRLI) at Johns Hopkins, welcoming 22 people with lived ALS experience to Baltimore.

Organized by the Northeast ALS Consortium (NEALS), the ALS CRLI program provides comprehensive education on ALS clinical research, covering topics such as study design, data analysis, ethical considerations, and community engagement.

During the two-day Baltimore event, participants engaged with the Packard Center's scientific team, Hopkins' ALS clinical team, and clinicians from Temple University and Duke University to learn about:

- The ALS research pipeline
- Clinical trial challenges and opportunities
- Ethics and informed consent
- Current developments in ALS research
- The importance of pre-clinical research

Participants who complete the training become ALS Research Ambassadors, gaining opportunities to influence and enhance the ALS research process.

Packard Center's Annual ALS Research Symposium

This year, a record number of participants were in attendance for the 24th annual Packard Center Symposium. Nearly 300 were on-site in Baltimore, while an additional 100 participated online. The gathering allowed Packard grantees, affiliates, and other ALS researchers to share their latest results, fueling new avenues of investigation and fostering novel collaboration between teams.

This format—along with the monthly Packard Principal Investigator (PI) meetings—is by design. By sharing results before they are published, scientists can troubleshoot problems, gain new perspectives, and leverage others' work to advance their own findings. This encourages the scientific process to move faster, taking the field closer to effective therapies for ALS, and, perhaps one day, even a cure for the disease.

It's this end goal that continues to drive Packard scientists—to identify therapeutics that will improve quality of life and alter the course of ALS. The collaboration and research partnerships fostered at these annual symposiums are key strategies in driving these breakthroughs.

Packard Center Investigators Identify CHMP7 Modulators Using CRISPR

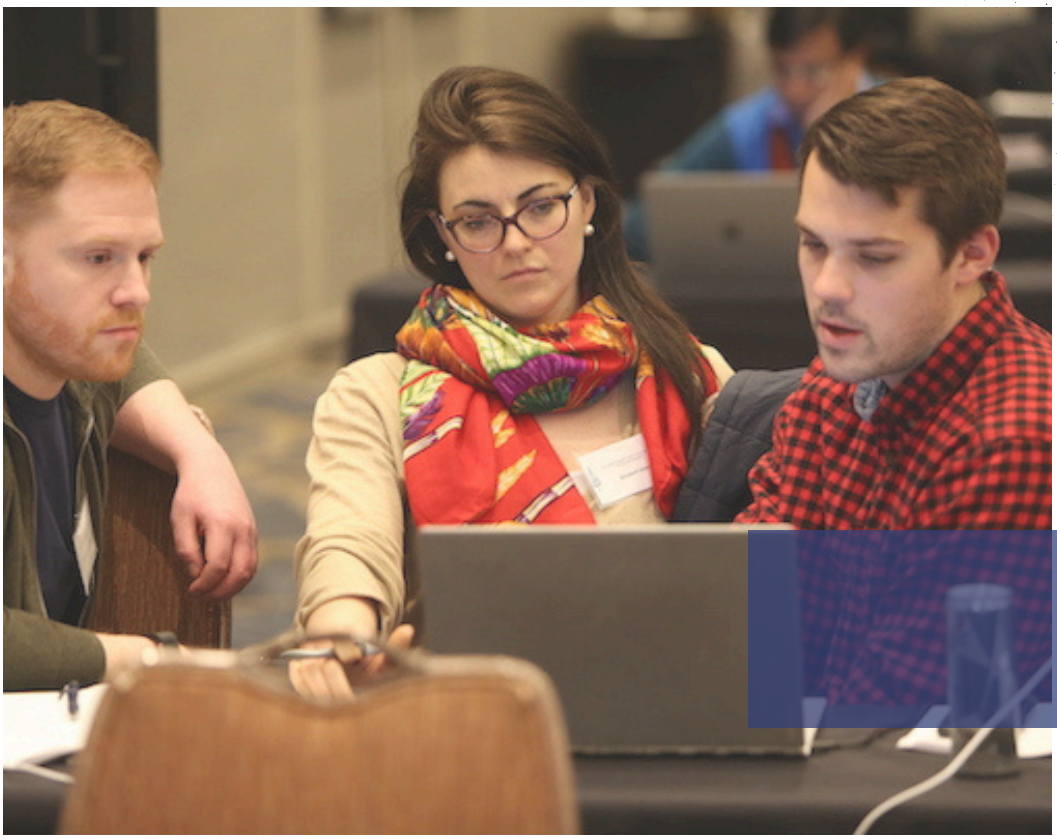
In a paper published in December 2024 in *Neuron*, Gene Yeo, PhD (University of California, San Diego), in collaboration with Packard Center founder, Dr. Jeffrey Rothstein and Packard Center investigator Dr. Alyssa Coyne used CRISPR based knockdown to identify novel factors that modulate the subcellular distribution of CHMP7. In doing so, they identified specific splicing complex proteins that when depleted can modulate the localization of CHMP7.

In addition, they found that CHMP7 itself is capable of binding to specific RNAs. Thus, collectively, this study supports altered RNA binding and processing as a modulator of protein localization in ALS, extending beyond RNA binding proteins themselves, to CHMP7, a protein involved in the homeostasis of the nuclear periphery.

Family Inspires a Community

In December 2024, Brian and Samara Jones shared their powerful family story with the Packard Center community, igniting a movement of generosity and hope. Shortly after their marriage in 2014, Samara was diagnosed with thyroid cancer, and Brian achieved a career milestone having been selected as President of Strayer University. Life took a challenging turn when Brian began experiencing symptoms that led to an ALS diagnosis just days after their first wedding anniversary, while Samara was six months pregnant with their first child, completing their family of five.

The Jones family shared a heartfelt call to action to support the Center's groundbreaking work in collaborative ALS research—which has become a vital source of hope for them—so future families like theirs will have a different journey with ALS. This inspired an extraordinary response from the community, securing a $125,000 calendar-year-end match generously offered by the Packard Center Board of Governors. The family's courage and determination exemplify the power of community and storytelling in advancing the fight against ALS, bringing us closer to a world without this devastating disease.



Collaborations Across the ALS Ecosystem: The Power of Partnership

At the Robert Packard Center for ALS Research at Johns Hopkins, collaboration remains central to advancing our mission of finding solutions to ALS. Our partnerships with other non-profit organizations amplify our impact and aid in accelerating progress.

ALS Network: Following our 2024 Annual Symposium, we partnered with the ALS Network to host a public-facing webinar designed to share the latest research developments with people living with ALS, families, and advocates. This initiative bridges the gap between scientific discovery and community understanding, ensuring stakeholders remain informed and engaged.

ALS Therapy Development Institute: Dr.Christine Vande Velde, Scientific Director of the Packard Center, participated in the NEALS Community Education Webinar titled “Bridging the Gap: The Crucial Role of Collaborative Pre-Clinical Research”. Held in February 2024, the event featured representatives from the ALS Therapy Development Institute and Project ALS, focusing on the vital role of the ALS community in shaping pre-clinical research and providing insights into how these partnerships drive innovation, accelerate discoveries, and ultimately pave the way for more effective treatments.

Answer ALS Foundation: The Neuromine data portal, supported by the Answer ALS Foundation and the Packard Center, represents a cutting-edge tool for advancing ALS research. By providing a centralized platform for vast datasets, Neuromine empowers scientists worldwide to uncover new insights and explore therapeutic targets with unprecedented precision. Neuromine contains over 150 trillion data points from 1,100 ALS participants with 900+ samples gene sequenced.

Hop On A Cure Foundation: The Packard Center partnered with the Hop On A Cure Foundation throughout 2024 to advance the fight against ALS. This collaboration was marked by Hop On A Cure’s establishment of a research endowment at the Center in February, providing critical support for innovative scientific initiatives. In December, the Packard Center received a transformative $300,000 grant, supporting four future critical research initiatives in the fight against ALS. Together, the organizations are committed to accelerating progress toward effective treatments and ultimately a cure for ALS.

Live Like Lou Foundation: In partnership with the Live Like Lou Foundation, the Packard Center co-funded a groundbreaking research grant, *Cellular and animal models of SPTLC1-related juvenile ALS*, that focuses on understanding how abnormalities in an enzyme called serine palmitoyltransferase cause motor neuron death. Led by Dr. Payam Mohassel, this study will help researchers identify and test out candidate therapies in the future, underscoring the importance of pooling resources to support innovative scientific inquiry that has the potential to transform patient outcomes.

Muscular Dystrophy Association: The Packard Center and the Muscular Dystrophy Association's Wings Over Wall Street jointly funded *New Pathways of Protein Homeostasis in ALS*, a research grant aimed at illuminating a new mechanism of sensing misfolded proteins and regulating their synthesis related to ALS. Awarded to Dr. Jiou Wang, this collaboration builds on the strengths of both organizations to address a critical gap in identifying new therapeutic targets, providing novel insights into the pathogenic mechanisms in ALS.

NEALS Consortium: In February 2024, Packard Center Scientific Director Dr. Christine Vande Velde joined representatives from Project ALS and ALS Therapy Development Institute for the NEALS Community Education Webinar, "Bridging the Gap: The Crucial Role of Collaborative Pre-Clinical Research," exploring the role of the ALS community in shaping pre-clinical research, and the transformative impact of interdisciplinary collaborations among leading research organizations. Building on the partnership with NEALS, the Packard Center co-hosted the Baltimore ALS Clinical Research Learning Institute (ALS-CRLI), providing vital training and discussions on the importance of fundamental research and best practices for ALS clinical trials. Following the ALS-CRLI, Dr. Vande Velde presented a talk on Preclinical ALS Research to the NEALS Patient Education and Advocacy (PEACe) Committee.

Project ALS: Looking to the future after the February 2024 successful webinar with Project ALS and ALS TDI, the Packard Center is again collaborating with Project ALS to organize a cross-disciplinary workshop on the Challenges of ALS Disease Modeling with iPSc (induced pluripotent stem cells). This workshop aims to foster dialogue among experts, spark innovative ideas, and advance our understanding of ALS disease pathways.

"The Packard Center has been an invaluable partner, and together, we are committed to advancing this critical area of research to deepen our understanding of the ALS disease process and accelerate the development of scientifically validated treatments for people living with ALS."

MARGOT SHANAHAN
Executive Director, Project ALS

Thank You to our Community Partners

We are grateful for the many partners who hosted an event to support ALS research. These events create awareness about ALS and raise needed funds for research. 2024 saw a number of events held nationwide, including*:

- **4th of July Lemonade Stand** (Ann Arbor, MI)
- **Charash Foundation/Carol Moeller Endowment Golf Tournament** (Wilmington, NC)
- **Cure ALS Golf Tournament** (Dallas, TX)
- **Delt Dunk 3v3 Basketball Tournament at Butler University** (Indianapolis, IN)
- **Get Tee'd Off Charity Golf Tournament** (Westminster, MD)
- **Gulls Way Campground JR Cropper ALS Memorial Event** (Dagsboro, DE)
- **Greater Baltimore Community Partners Golf Tournament** (White Hall, MD)
- **Jogger John's 5K and Cornhole Tournament** (Outer Banks, NC)
- **SEI/Strayer University Team Brian 5K** (Bethesda, MD)
- **Washington Suburban Sanitary Commission/Al's Pals Golf Tournament** (Bowie, MD)

**If your event is not listed here, please let us know so we can recognize your efforts.*

Philanthropy at Work

Did you know that the Packard Center is 100% funded through philanthropy?

The amount of research we can support is wholly dependent upon the donations we receive. Since 2000, gifts from individuals, foundations, and corporations have helped fund:

- 179 research projects at
- 61 institutions across 9 countries
- 24 Years of the annual Packard Center ALS Research Symposium

IN 2024, YOU HELPED MAKE OUR PROGRESS POSSIBLE

FUNDED 16 RESEARCH PROJECTS AT 12 INSTITUTIONS ACROSS 3 COUNTRIES

FUNDED $200,000+ IN SPONSORSHIPS FOR THE 24TH ANNUAL PACKARD CENTER ALS RESEARCH SYMPOSIUM

FUNDED 4 EXISTING AND ESTABLISHED 2 NEW NAMED ENDOWMENT RESEARCH FUNDS

FUNDED 2 EXISTING AND ESTABLISHED 1 NEW BEQUEST

Partnership Opportunities

THERE ARE VARIOUS WAYS TO SUPPORT THE PACKARD CENTER

RESEARCH PROJECT SUPPORT - FUNDING NOVEL RESEARCH AS QUICKLY AS POSSIBLE

Support of our research grant program ensures that we can fund the best research proposals.

Grants are funded in the amounts of $50,000-$100,000 per project.

NAMED ENDOWMENT & RESEARCH FUNDS - SUPPORTING THE NEXT GENERATION OF NEUROSCIENTISTS DEDICATED TO ALS

Gifts of all sizes help grow the Packard Center's permanent endowment and will help to secure a revenue stream for ALS research in perpetuity.

A $100,000 minimum contribution is required to establish a Named Research Endowment.

ANNUAL PACKARD CENTER SYMPOSIUM - BRINGING RESEARCHERS TOGETHER TO FOSTER GLOBAL COLLABORATION

The annual Packard Center Symposium is considered one of the most prestigious and scientifically stimulating scientific conferences in the field.

Sponsorship opportunities are available ($5,000 - $100,000).

PLANNED GIFTS - PROVIDING STABILITY FOR THE FUTURE

Donors may designate the Packard Center as a beneficiary of their will, life insurance policy and IRAs.

Opportunities are available to further our mission in alignment with broader philanthropic, financial, and tax objectives.

What impact would you like to make through your philanthropy?

We'd love to hear from you.

Please contact us if we may be of any assistance.

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To make a gift: www.packardcenter.org/give

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Meet the Team

JEFFREY ROTHSTEIN, MD, PHD
FOUNDER AND DIRECTOR

Packard Center founder Dr. Jeffrey Rothstein has served as the Center's Executive Director since its inception in 2000. A respected neuroscientist, Rothstein is credited as being one of the world's top ALS researchers, especially in the field of glutamate excitotoxicity. Dr. Rothstein is a Professor of Neurology and Neuroscience and a faculty member of the graduate program in cellular and molecular medicine at the Johns Hopkins University. He co-directs the Johns Hopkins Multidisciplinary ALS Clinic, one of the largest of its kind in the country. Dr. Rothstein serves as the Director of the Brain Science Institute. The Johns Hopkins Pedersen Brain Science Institute's translational neuroscience program which brings an innovative collaboration with industry and academic medicine in order to hasten treatments for neurodegenerative diseases.

CHRISTINE VANDE VELDE, PHD
SCIENTIFIC DIRECTOR

Dr. Christine Vande Velde is Full Professor in the Department of Neurosciences at the Université de Montréal and Université de Montréal Hospital Research Center (CRCHUM). Her research interests are centered on understanding the underlying pathological mechanisms that lead to the fatal neurodegenerative diseases amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). She obtained her Ph.D. in Biochemistry from the University of Manitoba and subsequently pursued post-doctoral studies at the University of California, San Diego/Ludwig Institute where she developed an expertise in ALS. Dr. Vande Velde serves on a number of grant review panels both nationally and internationally, has served as a Director of the Board of the ALS Society of Canada, and as Co-Chair of their Scientific and Medical Advisory Council. In her role at the Packard Center, she is responsible for charting the scientific direction of the Center.

Meet the Team

TARA LINCOLN, MS

DIRECTOR, OPERATIONS & ADMINISTRATION

Tara Lincoln oversees the operations of the Packard Center. She is responsible for guiding the strategic planning and implementation for the Center, leading the administrative team, and for the fiscal integrity of the Packard Center and its programs. She supports the Board of Governors and Answer ALS Advisory Board in reviewing overall program progress, sources of funding, public/external communications, and communications with donors.

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SUZANNE CONNELLY

DEPUTY DIRECTOR

Suzie Connelly has been with the Packard Center for 14 years. Starting as a volunteer, she now serves as the Center's Deputy Director, responsible for communications, the Packard Center website, and social media platforms. Suzie also works closely with the Center's Scientific Director on the Center's research grant program and planning and execution of the monthly investigator meetings and the annual research Symposium and collaborative workshops.

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MEG WHITEFORD, JD

SENIOR ASSOCIATE DIRECTOR OF DEVELOPMENT

Meg Whiteford is responsible for securing funds for Packard Center research grants. She seeks to build enduring relationships that result in the philanthropic support and advocacy for the Packard Center. Meg works to develop strategies to secure an annual stream of funding and to grow the Packard Center endowment. She engages directly with donors and the Center's Board of Governors and collaborates with the administrative team.

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TRAVIS SMITH

SENIOR DEVELOPMENT COORDINATOR

Travis Smith provides development-related administrative support to the Packard Center and helps manage the daily operations of the Packard Development team. He is responsible for gift processing and acknowledgment and reporting. He assists with the execution of events and manages third-party crowdfunding efforts to support the Packard Center. He works directly with the Senior Associate Director of Development.

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"Having witnessed and battled alongside two generations of family members fighting this disease, the Packard Center stands as a beacon of hope. The progress made over the past twenty five years in ALS research, with the Packard Center at the forefront of this effort, has been truly astounding, as well as gratifying for my family to be assisting in this journey."

SHELBY SAER
ALS Advocate, Co-Chair Packard Center Board of Governors

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ALS Research at Johns Hopkins