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Why Your Participation in the National ALS Registry Matters



Paul Mehta, MD
Principal Investigator
National ALS Registry

**The ALS Learning Series is made possible because of the
Gilbert & Jacqueline Fern Foundation and our industry partner.**



Mitsubishi Tanabe Pharma
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About Us

Leader in comprehensive ALS care and research.

Founded in 1977, the Les Turner ALS Foundation is one of the longest-serving independent ALS groups in the country.

We are committed to providing the most comprehensive care and support to people living with ALS and their families so they can confidently navigate the disease, and advance scientific research for the prevention, treatment and cure of ALS.

Support Services for people with ALS, families and caregivers

*Care visits by ALS support services
coordinators*

Support Group Meetings

Education materials and programs

*Access to medical equipment and
communication devices*

*Need-based grant programs and
community resources*

*In-service education for community
care*



Lois Insolia ALS Clinic

We Offer

- Access to enrollment in clinical trials and dedicated clinical trial coordinators
- Chicago's first and largest multidisciplinary ALS Clinic, with the highest number of neurologists and dedicated pulmonologists
- Multidisciplinary care that brings together an experienced team of neuromuscular specialists in one clinic to provide comprehensive support



We know making decisions about ALS care can be overwhelming.

We're here to help!



My ALS Decision Tool™ can help you choose ALS care that's in line with your needs and values. This interactive tool explains ALS treatment options and includes reflection questions to help you decide what's right for you.

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**MY ALS
DECISION
TOOL**

Looking for in-depth information about ALS symptoms and care options?

Les Turner ALS Foundation has you covered. We've created guides about key topics like nutrition, communication, mobility, and more.



ALS LEARNING SERIES

Our online ALS Learning Series aims to empower the ALS community through the latest information and insights. Educational webinars and interactive Q&A's covering a diverse array of topics, from nutrition to respiratory care, are offered monthly.

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Dedicated National ALS Registry Associate



Cara Gallagher, MA, LCPC

Brad Dusek's Story



- <https://www.youtube.com/watch?v=m8N0odx7GYg>

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National ALS Registry: *Learn How You Can Join the Fight Against ALS*

Paul Mehta, MD
Principal Investigator, National ALS Registry

2023 Les Turner ALS Foundation Webinar
January 19, 2023



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**National Center for Environmental Health
Agency for Toxic Substances and Disease Registry**



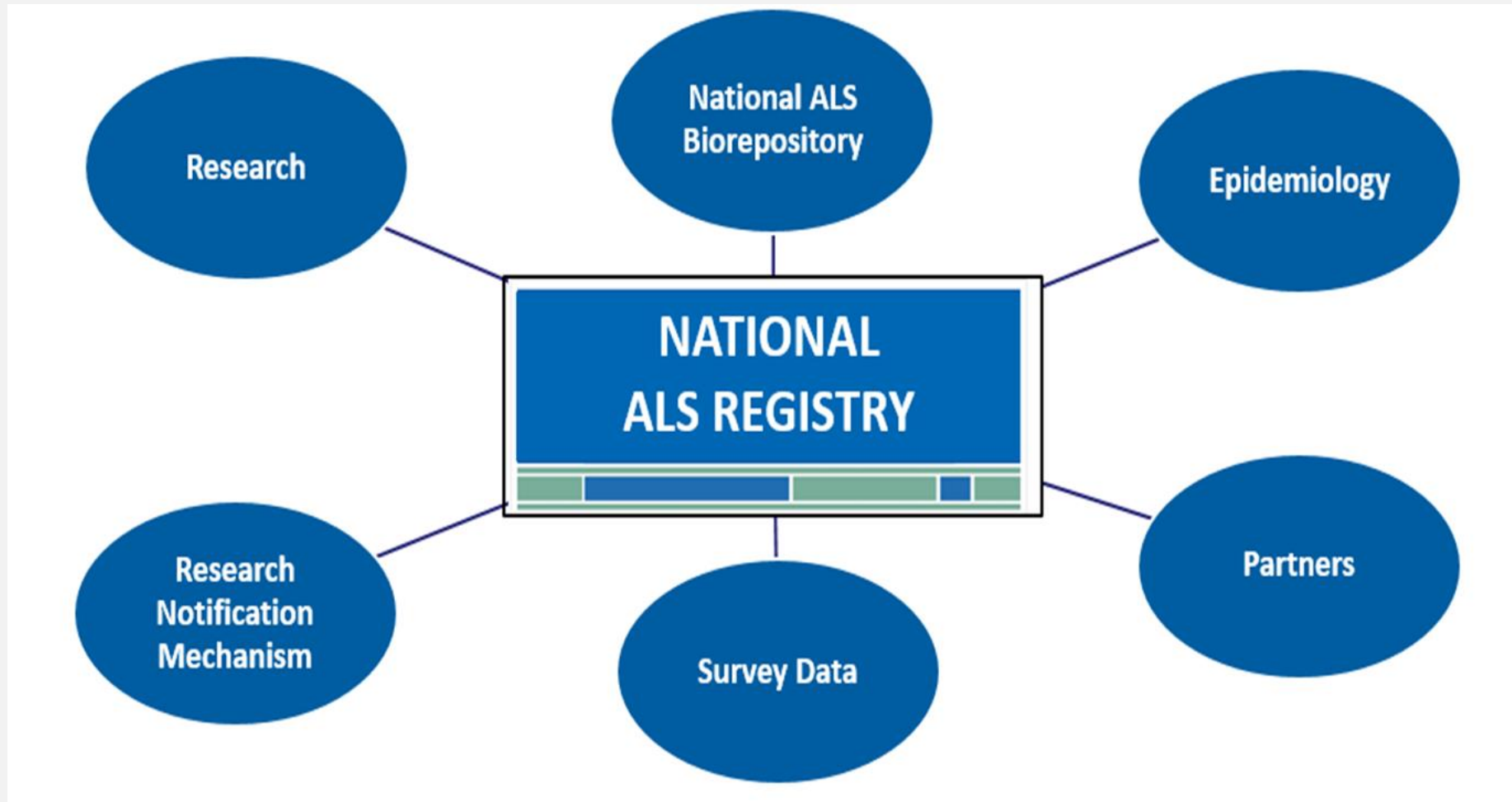
Disclosures

- None
- The findings and conclusions in this presentation have not been formally disseminated by the Centers for Disease Control and Prevention/the Agency for Toxic Substances and Disease Registry and should not be construed to represent any agency determination or policy.

Outline of Presentation

- **How you can join the fight against ALS:**
 - Importance of the National ALS Registry: Why should I join?
 - National ALS Biorepository: What is it and why is it important? How can I join?
 - Participate in clinical trials and epidemiological studies
 - Why take risk factor surveys?
- **What is the Registry doing to find the causes and risk factors for ALS?**
 - Reporting on who gets ALS (demographics) and the number of cases in the United States
 - Funding research
 - Publishing research
- **Conclusions/Questions**

Registry Does More Than Just Count ALS Cases

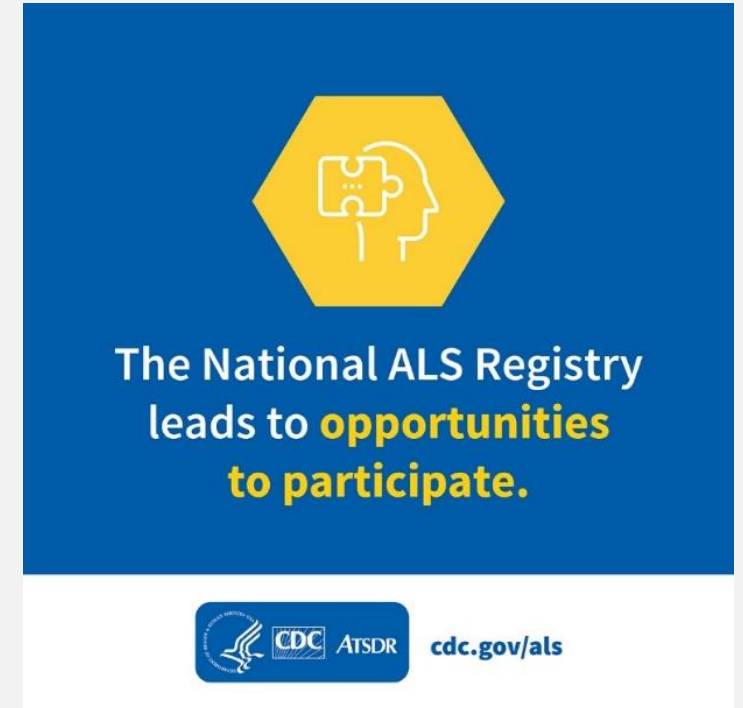


ALS Research Counts on You

Why is the National ALS Registry Important?

Why Should Patients Join?

- There are more unknowns than knowns about ALS
- Allows people with ALS to fight back and help defeat the disease
- **Goals of the Registry:**
 - Seeks to find out what causes (etiology) ALS
 - Seeks to find out the risk factors
 - Reports on the number of ALS cases in the United States (incidence and prevalence) – attempts to identify every person with ALS in U.S.
- **What we do:**
 - ✓ Collecting and analyzing data
 - ✓ Funding external research
 - ✓ Connecting people living with ALS with clinical trials and research studies
 - ✓ Collecting biospecimens



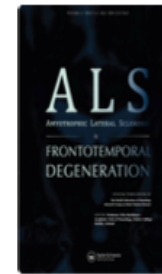
How many cases of ALS are there in the United States?

- **Background – ALS is not a reportable disease in the U.S.**
 - What does this mean?
- **Challenges**
 - ALS is not reported to state health officials (exception is Massachusetts)
 - ALS cases from private insurances are currently not captured by the Registry
 - Registry is a surveillance system and no system can capture all the cases
- **Registry captures between 80-85% of cases in the U.S.**
 - Always looking to improve case-ascertainment

One case of ALS is one too many...

How many cases of ALS are there in the United States?

- **New method used to estimate the number of missing cases in the U.S.**
 - This is a first.
- **Revised estimates are as follows:**
 - Close to 32K (31,843) cases as of 2017
 - Rate of 9.9 per 100,000 U.S. population
- **Estimate we are missing 14,043 cases**
 - Missing patients receive care outside CMS/VHA/VBA*
 - Private insurances (e.g., PPOs, HMOs)**



Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration



ISSN: (Print) (Online) Journal homepage: <https://www.tandfonline.com/loi/iafd20>

Prevalence of amyotrophic lateral sclerosis in the United States using established and novel methodologies, 2017

Paul Mehta, Jaime Raymond, Reshma Punjani, Moon Han, Theodore Larson, Wendy Kaye, Lorene M. Nelson, Barbara Topol, Oleg Muravov, Corina Genson & D. Kevin Horton

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To link to this article: <https://doi.org/10.1080/21678421.2022.2059380>

*CMS: Centers for Medicare and Medicaid Services

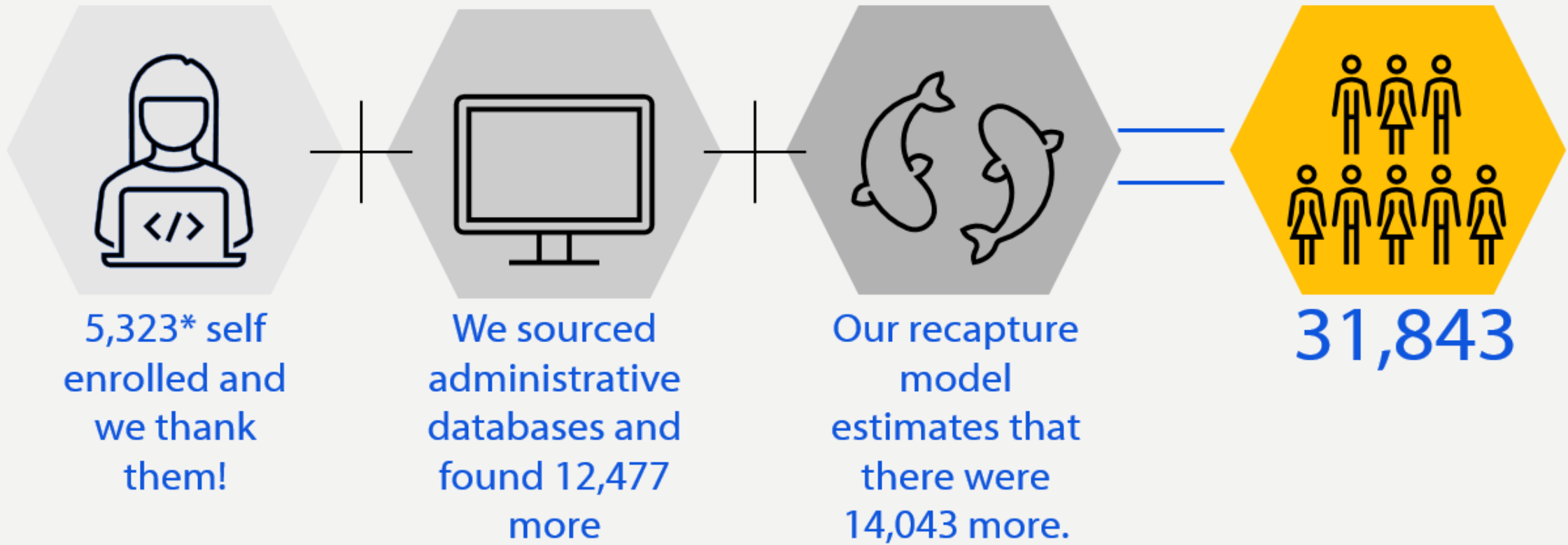
*VHA: Veterans Health Administration

*VBA: Veterans Benefits Administration

**PPO: Preferred Provider Organization

**HMO: Health Maintenance Organization

How We Came Up With 31,843 cases



*Approximately 5% of the total number of definite cases are in both the web portal and administrative databases.

Why Take the Risk Factor Surveys?

- **Helps researchers find clues about ALS:**

- Occupations: are certain occupations at higher risk for ALS?
- Military service: why are veterans at a greater risk of getting ALS?
- Physical activity, smoking and drinking: are these risk factors?
- Residential history and residential pesticide use: are these risk factors?
- Traumatic brain injuries: are these risk factors?
- Disease progression: how is ALS progressing (fast or slow) and what is affected?
- Open ended: patient perspectives on disease cause

Take surveys in any order (caregiver help is always appreciated)

HELPS US SOLVE THE MYSTERIES BEHIND ALS

Why is the National ALS Biorepository Important?

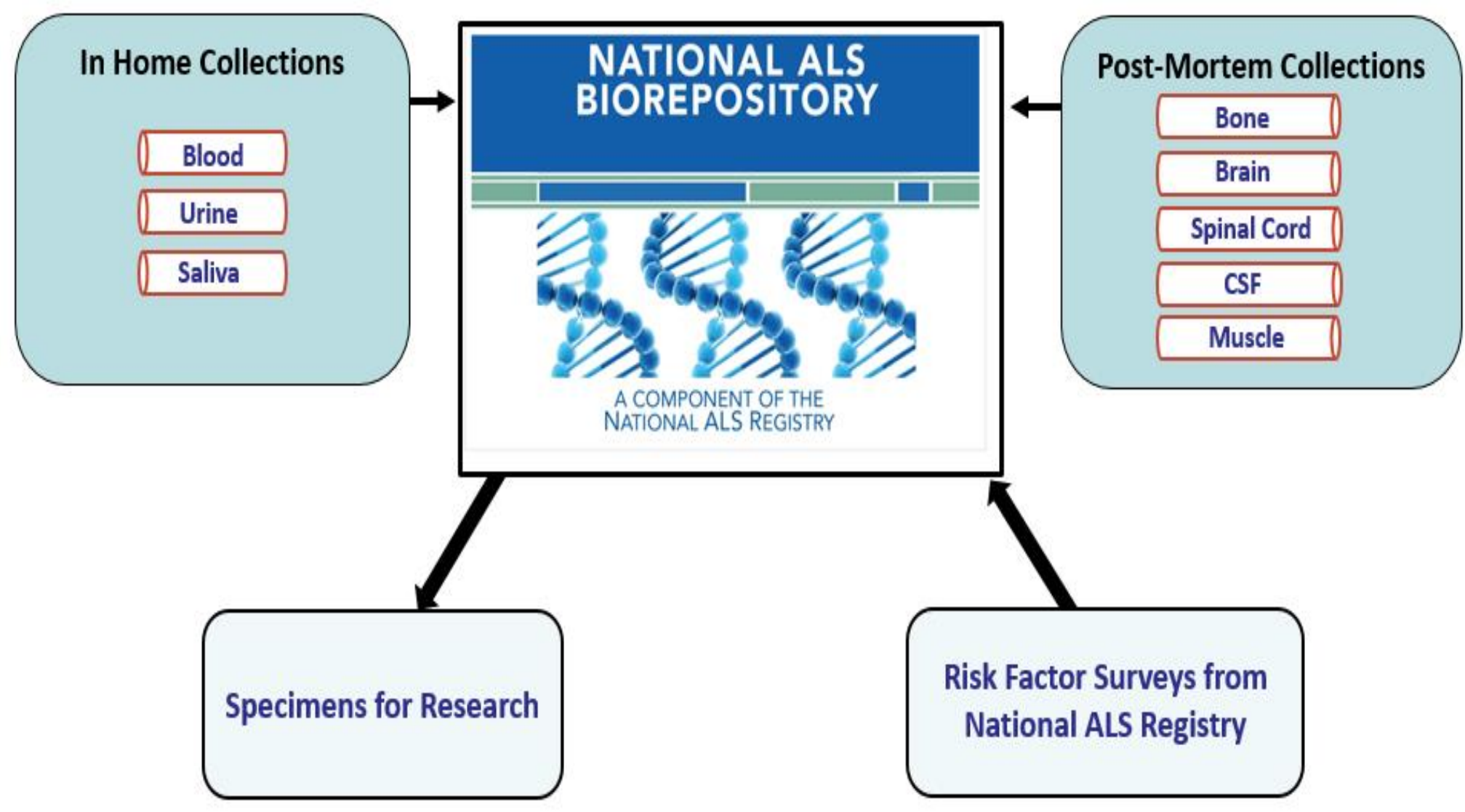
Help researchers in the area of ALS:

- **Genetics:**
 - Identification of genes such as SOD1 and C9orf72 and others
- **Biomarker identification: indicator of a biological state or condition**
 - Help us to examine disease progression (how fast or slowly ALS progresses)
 - See if certain drugs will work or not work
 - Possibly identify if a simple blood test could catch ALS early
- **Environmental factors (analyzing patient's blood and saliva):**
 - Heavy metals (lead, arsenic, and others)
 - Persistent organic pollutants (e.g., pesticides, insecticides, PCBs, DDT): tough to degrade/breakdown

Details on the National ALS Biorepository

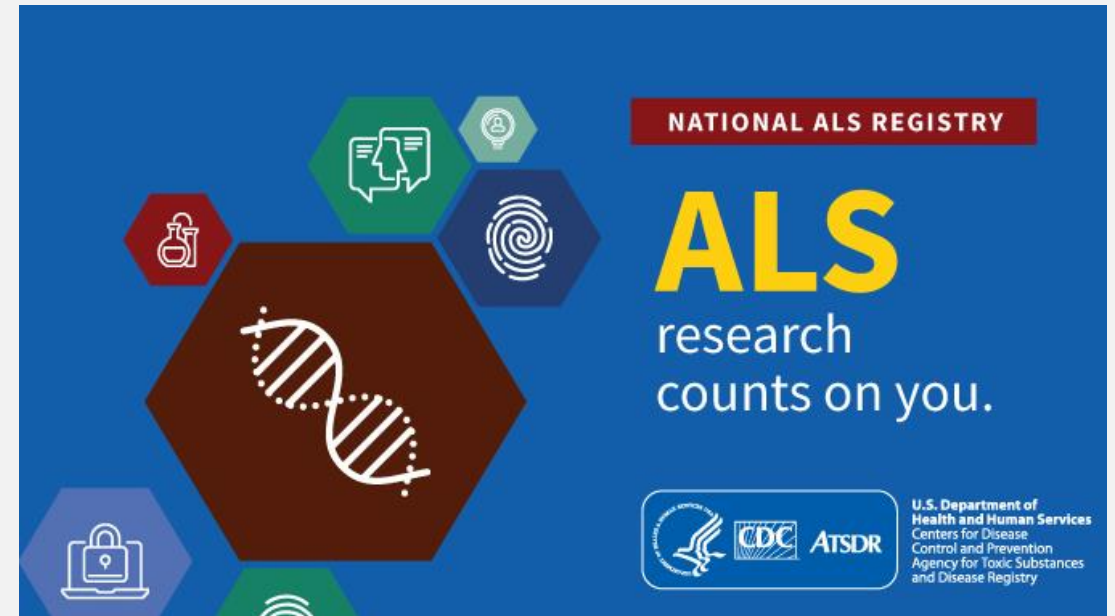
- **Biorepository is significantly different from others**
 - Extensive risk factor survey data linked with biosamples
 - Nationally representative, that is, beyond referral centers
 - User-friendly to person with ALS, that is, phlebotomists come to your home to collect samples
 - Specimens collected specifically for biorepository, that is, we do not use leftover study samples to constitute biorepository
 - Pre/post-mortem samples kept in one central biorepository
- **No charge for patients - user friendly - we come to your home**
- **Largest collection of pristine ALS samples for research, e.g., genetics, biomarkers, disease progression.**

National ALS Biorepository Overview



We Support Patient Participation in Clinical Trials and Epidemiological Studies

- Patient recruitment for research can be difficult and challenging
- Approx. 95% of Registry PALS want to participate in research
- Registry links PALS with scientists who are recruiting for research (e.g., clinical trials, studies)
- Domestic and international researchers are using the tool for recruitment purposes
- Over 70 institutions have used it



Highlighted Notifications Using the Registry

- **Notable Multi-site Clinical Trials:**
 - Amylyx Pharmaceuticals (*Paganoni*):
 - AMX0035, slows disease progression and improves muscle strength
 - Mitsubishi Tanabe Pharma (*Multiple*):
 - Efficacy and Safety Study of Oral Edaravone Administered in Subjects With ALS
 - Biogen (*Garafalo*):
 - Atlas Study, tofersen administered to those with a history of SOD1
 - Healy Platform Trial (*Cudkowicz*):
 - Test the efficacy of multiple investigational products

Registry Funds Research

- **ATSDR is funding extramural research to learn more about ALS etiology and risk factors**
- **24 research studies have been funded to date**
- **Information gleaned also will help ATSDR prioritize topics for future risk factor surveys**
- **Funded 2 awards in September 2022**
- **New funding opportunity announcement in 2023**



Areas of Research Funded

- **Studies range from:**

- Identifying environmental risk factors such as cyanobacteria (harmful algal blooms)
- Analyzing pesticides and pollutants Identifying biomarkers and genes
- Evaluating the human virome (400 viruses) for antibodies
- Genetics: why certain populations have lower rates of ALS than others
- Military service and the risk for ALS
- Evaluating traumatic brain injuries and chronic neuroinflammation
- Oxidative stress and environmental risk factors
- Evaluating the gut microbiome
- Conducting whole genome sequencing of Biorepository samples

Current and Past Funded Institutions

Northwestern University <i>(Siddique)</i>	Harvard University <i>(Weisskopf & Ascherio)</i>
Columbia University <i>(Schneider & Mitsumoto)</i>	Dartmouth College <i>(Stommel)</i>
University of Michigan <i>(Feldman & Murdock)</i>	University of Pittsburgh <i>(Talbot)</i>
Trinity College <i>(Hardiman)</i>	Karolinska Institute <i>(Fang)</i>
Stanford University <i>(Nelson)</i>	University of Missouri <i>(Song)</i>
University of Miami <i>(Benatar)</i>	National Institutes of Health <i>(Traynor)</i>

Impact of the National ALS Registry

- **Largest database of ALS patients in the United States for research.**
- **Collaborating with pharmaceutical companies and academia to provide recruitment assistance for clinical trials and epidemiological studies.**
- **National ALS Biorepository is advancing ALS research on biomarkers, genetics, and environmental exposures.**
 - User friendly and free system for patients to participate in research.
- **Funding research grants for leading academic institutions in order to learn more about risk factors and possible etiologies.**
- **Determining national epidemiological trends such as how many cases of ALS in the United States**
- **Looking to solve the who, the what, and why of ALS.**

In Conclusion

National ALS Registry and the Les Turner ALS Foundation are vital partners with the same goal of curing ALS.



Comments/Questions

www.cdc.gov/als



The National ALS Registry: Get The Facts

The National Amyotrophic Lateral Sclerosis (ALS) Registry enables persons with ALS to fight back and help defeat ALS (Lou Gehrig's Disease). By signing up, being counted, and answering brief questions about your disease, you can help researchers find answers to critical questions.

Learn more at www.cdc.gov/als or (800) 232-4636



Who can sign-up?

Anyone with ALS

What do I need?

- A computer with an internet connection
- An email address



What if I need help?

Caregivers and others can help you in person or even over the phone



No computer? Don't worry! A family member, caregiver, or friend with a computer can help you. You can also contact:



What kind of information is collected?

- Basic demographics (e.g., age, sex, height, weight)
- Military history
- Physical activity
- Family history



Do I need to update my information?

YES! Every six months – you'll get an email reminder

Will my information be private?

- YES! Only approved registry scientists can see it, NOT employers or insurers
- You CANNOT be looked up in the registry by name

YOU JOINING

More information for research

A better understanding of ALS

The chance to help create a better future for persons with ALS

For more information, contact NCEH/ATSDR
1-800-CDC-INFO (232-4636)

TTY: 1-888-232-6348

www.atsdr.cdc.gov

www.cdc.gov

Follow us on Twitter @CDCEnvironment

The findings and conclusions in this report are those of the authors and do not necessarily represent the official position of the Centers for Disease Control and Prevention and the Agency for Toxic Substances and Disease Registry.





Q&A

Please type your questions
in the Q&A box

www.lesturnerals.org



Upcoming Webinar:

**ALS & Relationships, Sex
and Intimacy**

February 23rd, 2023, 12:00 CT

alslearningseries.org



Rachael Marsden, RN

**MND Nurse Consultant & Care Centre
Coordinator for the Oxford MND Care &
Research Centre**

Oxford, United Kingdom



**Thank you for your participation in
today's webinar!**

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