WELCOME!
September 19, 2022

Telling your Story: How Joining The CDC National ALS Registry Makes a Difference

Guest Speaker: Patricia Stanco, MHS
Associate Director, Mission Engagement
The ALS Association

The ALS Association
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Telling Your Story: How Joining the CDC National ALS Registry Makes A Difference

Patti Stanco, MHS
Assoc. Dir., Mission Engagement
Overview of the Registry
Why it matters and telling your story
How to enroll and updates on data
Summary of the Registry Annual Meeting
Questions
National ALS Registry

- Centers for Disease Control and Prevention/Agency for Toxic Substances and Disease Registry (CDC/ATSDR)

- The only national ALS Registry

- Passed by Congress in 2010; launched in 2012
GOALS OF THE REGISTRY:

Find out what causes ALS

Report on the number of ALS cases in the U.S.

Learn the risk factors of ALS

Report on the number of ALS cases in the U.S.
There is so much more we don’t know yet about my disease. I believe vigorous research into known and suspected ALS factors will help us all learn more.

- Ed, a person with ALS

cdc.gov/als
After the diagnosis…. now what?
After the diagnosis… now what?

"Why did this happen to me or my loved one?"
Where do people learn about the Registry?

- ALS Clinic or Neurologist
- Conferences
- Internet searches & websites
- Walk to Defeat ALS
- New registration packet
- Support group
- Care Services Staff
Answering risk factor surveys is a meaningful way to share your story.

I had back surgery and never fully recovered. Instead of getting stronger, I started getting weaker.

When I was growing up, I spent summers on my grandma and grandpa’s farm. My grandpa would take me fishing on a lake nearby that used to get full of algae.

Parkinson’s seems to run in my family and a cousin has Multiple Sclerosis – I wonder if that means anything.
The ALS Association Strategic Priorities

Ensure Access & Prevention
Ensure people with ALS have access to effective treatments, and cases of ALS are being prevented.

Empower & Engage
Empower people with ALS to engage with the world in the way they want.

Reduce Burdens
Reduce the physical, emotional, and financial burdens of living with ALS.

Ensure Support for ALL
Ensure ALL people with ALS and their caregivers receive high quality services that benefit them.

Create Accountability
Create a culture of accountability to ensure meaningful impacts for people with ALS.

OUR MANTRA:
Whatever It Takes
National ALS Registry - A Pathway for Greater Knowledge

Today’s Research → Tomorrow’s Answers

STEP 1
Collect & analyze ALS data

STEP 2
Observe changes in disease patterns over time

HOW
- Dedicated research
- Risk factor surveys
- National ALS Biorepository
- Clinical trials
- Partner collaborations

STEP 3
Identify common risk factors

STEP 4
Find the cause(s)
Reasons to Consider Joining the Registry

By completing the risk factor surveys people living with ALS can help direct researchers towards a treatment and a cure.

The Registry is the only national ALS research project. Anyone with an ALS diagnosis can participate from the comfort of their home.

Patients can share their thoughts about what factors they think may have contributed to their disease in the Registry. One of the risk factor surveys is an open comment box.

There is a biorepository connected to the Registry. When researchers use it for research the samples are connected to the risk factors data.
Components of the Registry

National ALS Registry

1. Risk Factor Surveys
   Up to 18 surveys in 5 main subjects. Understanding the risk factors of ALS will drive research towards better treatments, a cure, and even prevention.

2. Biorepository
   Currently collecting blood samples. Must enroll in Registry to participate. (Previously collected nail clippings, hair strands, saliva swabs, and post-mortem brain and spinal cord.)

3. Clinical Trial Notification
   Patients may opt in to receive notifications about clinical trials for which they may qualify. Personal data is not shared with researchers. Upon receiving the email, patients must reach out.

4. Environmental Research
   Air pollution
   Organic pollutants
   Pesticides and algae blooms
   Gene-environment interactions
   Biomarkers
   Environmental risks
   Comorbidities
RISK FACTOR SURVEYS

- Demographics and family history
- Disease onset
- Smoking, alcohol, and caffeine use
- Where you lived and worked
- Job history
- Military service
- Physical activity
- Hobbies
- Environmental exposures
- Pesticide use
- Head and neck injuries
RISK FACTOR SURVEYS

Open-ended question

I think [this factor] contributed to my diagnosis of ALS….
BIOREPOSITORY

- Requires enrollment in the Registry
- Step #1 Request a packet of information
- Step #2 Complete informed consent
- Step #3 Schedule in-home blood collection by a licensed phlebotomist
- About post-mortem ....
  - https://cdmrp.army.mil/alsrp/resources/Biorepositories_Postmortemtissues
FEDERAL RESEARCH

- Federal Research on ALS
  - NIH
  - DoD
  - CDC
- The CDC is the only federal agency to focus on etiology and risk factors
- To date, the Registry has funded 21 research projects
- More than half of the CDC’s Registry budget is allocated for research activities
- The ALS Association regularly include funding for the CDC and the Registry in their appropriations requests as part of their advocacy efforts
PASSWORDS & PRIVACY

• Only the last 5 digits of social security number required to enroll

• Choose when or if to change password:
  o Six months
  o Annually
  o Never

• Opt “in” for clinical trial alerts and newsletter
REMINDERS:

• To enroll in the Registry, you must have a diagnosis of ALS. People living with PLS or other related, non-ALS diseases are not eligible to enroll at this time.

• If you need assistance enrolling, it is okay to have a friend, family member, or caregiver help you.

• Sometimes people ask if a family member can enter information for a loved one who has passed away from ALS.
Hay muchas incógnitas sobre la ELA. Las personas que tienen ELA pueden ayudar a las generaciones futuras.

El Registro Nacional de ELA deja que puedan hacer un seguimiento a la enfermedad de una manera más precisa. El Registro Nacional de ELA abre un camino hacia los conocimientos.

El Registro Nacional de ELA

La Agencia para las Enfermedades del Sistema Nervioso (NINDS), que es un departamento del Departamento de Salud y Servicios Humanos, y su socio, la National Amyotrophic Lateral Sclerosis (ALS) Association, han desarrollado el Registro Nacional de ELA. El Registro Nacional de ELA acumula información sobre la enfermedad en un solo lugar y revisa la información para entender mejor el impacto de la enfermedad. Ellos pueden compartir esta información para ayudar a las personas que buscan tratamiento. Ellos pueden compartir esta información para ayudar a los investigadores que buscan entender la enfermedad y encontrar una cura.

Acerca del registro

La Agencia para las Enfermedades del Sistema Nervioso (NINDS), que es un departamento del Departamento de Salud y Servicios Humanos, y su socio, la National Amyotrophic Lateral Sclerosis (ALS) Association, han desarrollado el Registro Nacional de ELA. El Registro Nacional de ELA acumula información sobre la enfermedad en un solo lugar y revisa la información para entender mejor el impacto de la enfermedad. Ellos pueden compartir esta información para ayudar a las personas que buscan tratamiento. Ellos pueden compartir esta información para ayudar a los investigadores que buscan entender la enfermedad y encontrar una cura.

El Registro Nacional de ELA deja que puedan hacer un seguimiento a la enfermedad de una manera más precisa. El Registro Nacional de ELA abre un camino hacia los conocimientos.

Las personas que tienen ELA pueden ayudar a las generaciones futuras. Esto significa que los investigadores y los médicos pueden aprender más sobre la enfermedad. Esto significa que la enfermedad de la ELA puede ser tratada de manera más efectiva. Esto significa que la enfermedad de la ELA puede ser tratada de manera más efectiva.
Enrolling in the Registry

Step 1: Explore & Sign Up cdc.gov/als

- Voluntary
- Have an internet connection on a laptop or device
- Go to the Registry website
- Answer the screening questions
- Complete informed consent
- Create a user ID and password
Enrolling in the Registry

**Step 1: Explore & Sign Up cdc.gov/als**
- Voluntary
- Read materials and review website information
- Screening Questions
- Informed Consent
- Create a user ID and password

**Step 2: Participate**
- Ask a tech-savvy family member as project manager
- Tell your story!
- 18 surveys; 5 topics (RISK FACTOR SURVEYS)
- Consider the BIOREPOSITORY & opting in to receive CLINICAL TRIAL ALERTS
If a person living with ALS or caregiver needs to contact the Registry for confirmation of enrollment or technical assistance, please contact:

CDC National ALS Registry
Hours of Operation Monday–Friday 8 AM–5 PM EST

- **Phone** 877-442-9719
- **Email** Als@cdc.gov
- **Web form**
  https://wwwn.cdc.gov/ALS/ContactUS.aspx
CDC Website

www.cdc.gov/als

- Link to Spanish version
- Sign up
- Log in to complete surveys
National ALS Registry Dashboard

Estimated living persons/cases (≥18 years) with ALS in the United States in 2017 (Prevalence*)

- U.S. Prevalence (CY2017): 5.5-9.9 persons per 100,000 U.S. population
- U.S. Incidence (CY2016): 1.7 persons per 100,000 U.S. population
- U.S. Mortality (CY2011-2014): 1.7 persons per 100,000 U.S. population

Year 2017

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Estimated No. cases</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total</td>
<td>17,800 (lower bound) to 37,833 (upper bound)</td>
</tr>
<tr>
<td>Gender*</td>
<td></td>
</tr>
<tr>
<td>Male</td>
<td>11,024 (62.0)</td>
</tr>
<tr>
<td>Female</td>
<td>6,750 (37.9)</td>
</tr>
<tr>
<td>Unknown</td>
<td>18 (0.1)</td>
</tr>
<tr>
<td>Race*</td>
<td></td>
</tr>
<tr>
<td>White</td>
<td>12,928 (72.8)</td>
</tr>
<tr>
<td>Black</td>
<td>1,131 (6.4)</td>
</tr>
<tr>
<td>Other</td>
<td>973 (5.5)</td>
</tr>
<tr>
<td>Unknowns</td>
<td>2,747 (15.8)</td>
</tr>
</tbody>
</table>

Published Journal Articles: 96 publications

Research Study Recruitment (Clinical Trials and Epidemiological Studies)

- Completed Risk Factor Surveys by PALS: 101,853 surveys

Research Studies Using Registry Biorepository:
- 22 studies

Biorepository Promoters/Partners/Advisors:
- >100

Research Studies Using Registry Document Enrollment:
- 9 studies

New Report COMING SOON!
Highlights from the latest report:

- Cases collected from other databases = 12,477
- Estimate of missing cases = 14,043
- Self-enrolled = 5,323 (Thank you!)
- Total = 31,843
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

https://doi.org/10.1080/21678421.2022.2059380
PUBLISHED ONLINE:
15 April 2022
# Clinical Trial Notifications

## ACTIVE – ALS Research Notification for Clinical Trials and Studies

The following are approved ALS studies and clinical trials that have used the National ALS Registry for patient recruitment purposes. Email notifications have already been sent out to PALS meeting the specific study criteria.

**Active:** currently recruiting for the specified clinical trial or study
To view list of closed (recruiting has ended) clinical trials and studies, click [here](#).

To receive automatic notifications about the latest clinical trials and studies, please join the Registry, click [here](#).

This list will be updated as new research proposals are approved by ATSDR.

<table>
<thead>
<tr>
<th>No.</th>
<th>Study Name</th>
<th>Institution</th>
<th>Investigator</th>
<th>Research Notification Date*</th>
<th>Information Link</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Radiance@Eidaravone: Findings in Biomarkers From ALS</td>
<td>Mitsubishi Tanabe Pharma</td>
<td>James Berry, MD</td>
<td>New! JUL 2022</td>
<td>Click here <a href="#">here</a></td>
<td>Active</td>
</tr>
<tr>
<td>2</td>
<td>Feasibility of the BrainGate2 Neural Interface System in Persons with Tetraplegia</td>
<td>Massachusetts General Hospital</td>
<td>Leigh R. Hochberg, M.D., Ph.D.</td>
<td>New! JUN 2022</td>
<td>Email <a href="#">here</a></td>
<td>Active</td>
</tr>
<tr>
<td>3</td>
<td>Home-based Remote Digital Monitoring to Assess ALS progression</td>
<td>Emory University</td>
<td>Jonathan Glass, MD</td>
<td>New! JUN 2022</td>
<td>Click here <a href="#">here</a></td>
<td>Active</td>
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<tr>
<td>4</td>
<td>Healey ALS Platform Trial</td>
<td>Massachusetts General Hospital</td>
<td>Merit Cudkowicz, MD</td>
<td>New! MAY 2022</td>
<td>Click here <a href="#">here</a></td>
<td>Active</td>
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<tr>
<td>5</td>
<td>Rasch-built Outcome Measures to Improve ALS Clinical Trials</td>
<td>Emory University</td>
<td>Christina Fournier, MD</td>
<td>New! APR 2022</td>
<td>Click here <a href="#">here</a></td>
<td>Active</td>
</tr>
<tr>
<td>6</td>
<td>Efficacy and Safety Study of Oral Tazarotene Administered in Subjects With ALS</td>
<td>Mitsubishi Tanabe Pharma Development America, Inc.</td>
<td>Multiple</td>
<td>New! APR 2022</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>7</td>
<td>Phase 2b Study of TPN-101 in Patients with C9orf72 ALS-ETD (Amyotrophic Lateral Sclerosis and/or Frontotemporal Dementia)</td>
<td>Transgene Therapeutics</td>
<td>Merit Cudkowicz, MD</td>
<td>New! MAR 2022</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>8</td>
<td>COURAGE-ALS Phase 3 Clinical Trial</td>
<td>CytoKinetics, Inc.</td>
<td>Stacy A. Aujla, MD</td>
<td>New! FEB 2022</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>9</td>
<td>Microbiome in the Progression of ALS</td>
<td>Emory University</td>
<td>Wiki Herting, PhD</td>
<td>New! JAN 2022</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>10</td>
<td>Evaluation of IGNIS in RUS ALS Patients</td>
<td>Ionis Pharmaceuticals</td>
<td>Multiple</td>
<td>New! JAN 2022</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<td>11</td>
<td>COVID-19 ALS Registry</td>
<td>Athem Health</td>
<td>Levi Dascal, MD</td>
<td>DEC 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>12</td>
<td>ALS Focus Survey Program</td>
<td>ALS Association</td>
<td>Sarah Parina, PhD</td>
<td>DEC 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>13</td>
<td>Identify, Analyze, and Evaluate Potential Risk Factors for Amyotrophic Lateral Sclerosis (ALS)</td>
<td>Dartmouth-Hitchcock Medical Center</td>
<td>Eliah Stimmel, MD</td>
<td>OCT 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>14</td>
<td>The E-Health Application to Modify Oral Hydration and Measure Outcomes Remotely in ALS</td>
<td>Massachusetts General Hospital</td>
<td>Anne-Marie Wills, MD</td>
<td>MAY 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>15</td>
<td>ALS Quest: An online questionnaire for research into ALS</td>
<td>University of Sydney</td>
<td>Roger Pamphlett, MD</td>
<td>APR 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
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<tr>
<td>16</td>
<td>Answer ALS Companion App</td>
<td>Johns Hopkins University SOM</td>
<td>Jeffrey Rosenberg, MD</td>
<td>MAR 2021</td>
<td><a href="#">Click here</a></td>
<td>Active</td>
</tr>
</tbody>
</table>
ALS Registry Annual Meeting (Aug. 29-30, 2022)

• **NEED FOR BIOSAMPLES:** In a sample survey of ALS researchers, 71% of respondents anticipate needing biosamples in the next 6-12 months.

• **CURRENT RESEARCH:** Several presentations on studies looking at potential risk factors (overlap of EPA supersites and incidence of neurodegenerative disease).

• **OPEN COMMENT QUESTION IN SURVEYS:**
  - Comorbidities (example: autoimmune disease)
  - Medications (example: statins)
  - Immunizations
  - Military-specific exposures
  - Exercise and physical labor
  - Emotional trauma and stress

• **FEEDBACK: Suggestions from people living with ALS:**
  - Minimize the delay of collecting data
  - Work on improving diversity in the data collected
More ALS Data Helps Complete the Puzzle of ALS

5 data points vs 5000 data points
More ALS Data Helps Complete the Puzzle of ALS
More ALS Data Helps Complete the Puzzle of ALS

Answering risk factor surveys is a meaningful way to share your story.

- I had back surgery and never fully recovered. Instead of getting stronger, I started getting weaker.
- When I was growing up, I spent summers on my grandma and grandpa's farm. My grandpa would take me fishing on a lake nearby that used to get full of algae.
- Parkinson's seems to run in my family and a cousin has Multiple Sclerosis -- I wonder if that means anything.

New enrollee
More ALS Data Helps Complete the Puzzle of ALS

What is YOUR story?
Hear from ALS patients and why they’re joining the National ALS Registry to fight for their future and future generations. Your ALS experience could help change theirs.
Join the National ALS Registry today!

Visit www.cdc.gov/als
Questions?

Contact:
Patti Stanco
patricia.stanco@als.org
(202) 464-8045