National ALS Registry Update

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Background on The National ALS Registry

- Launched by the Agency For Toxic Substances and Disease Registry (ATSDR), a branch of the CDC in 2010 after the ALS Registry Act was signed into law in 2008.

- National ALS Registry is the only population-based Registry for ALS in the entire US.

- Main goals of the Registry are to:
  - Describe the incidence and prevalence of ALS
    - Incidence: the occurrence, rate or frequency of a disease
    - Prevalence: the percent of a population that is affected with a particular disease at a given time
  - Describe the demographics of those with ALS
  - Examine risk factors for the disease
Registry Methodology

- National Databases*
  - Apply Algorithm**
    - Non ALS Patients
    - Potential ALS Patients
    - True ALS Patient

- Web Portal Registration
  - Validation Questions
    - True ALS Patients
      - Risk Factor Survey
    - Non ALS Patients
Recruits patients for research

Collects specimens

Funds external research

Provides data/specimens
Risk Factor Survey Modules

- Demographics
- Military history
- Occupational history
- Smoking & alcohol use
- Physical activity
- Family history of ALS & neurological disease
- Disease progression
- Clinical data
- Open ended question

- Lifetime residential history
- Home pesticides use
- Lifetime occupational history
- Caffeine use
- Reproductive history (females only)
- Hobbies involving toxicant exposures
- Trauma history
- Health insurance status
Research Notification Tool

- Enables those **who have enrolled** in the Registry to be notified about research projects, including clinical trials, in which they are eligible to participate.

- Over 95% of those enrolled in the Registry have elected to be notified.

- Over 80,000 emails have been sent to Registry-enrolled individuals living with ALS.

- To date, the Registry has assisted in recruitment for 34 research studies and clinical trials (international and domestic).
Patient enrolls in ALS Registry

Patient consents to be notified during enrollment process

Researcher submits proposal to ATSDR

If approved, Registry queried by ATSDR for those meeting study criteria

Proposal goes to committee for approval

ATSDR reviews proposal for completeness

ATSDR notifies eligible patients via email with recruitment material and contact info

Patient contacts researcher to take part in study*

Patients & Researchers Connected!!
Examples of research studies that have used Research Notification Tool

<table>
<thead>
<tr>
<th>Study Name (n=34)</th>
<th>Institution</th>
<th>Investigator</th>
</tr>
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<tbody>
<tr>
<td>Risk Factor Analysis in ALS</td>
<td>Medical University of SC</td>
<td>David Stickler, MD</td>
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<tr>
<td>Phase II/III Trial of Arimoclomol in SOD1+ Familial ALS</td>
<td>University of Miami</td>
<td>Michael Benatar, MD, PhD</td>
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<td>Mindfulness, psychological well-being, and physical degeneration in people with ALS</td>
<td>Harvard University</td>
<td>Ellen Langer, PhD</td>
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<tr>
<td>Spatial Analysis of ALS in Florida, Ohio, New Hampshire, and Vermont</td>
<td>Dartmouth-Hitchcock Medical Center</td>
<td>Elijah Stommel, MD, PhD</td>
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<tr>
<td><strong>Mexiletine treatment of muscle cramps in ALS</strong></td>
<td>University of California, Davis</td>
<td>Björn Oskarsson, MD</td>
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<tr>
<td>Epidemiologic Risk Factors &amp; Genetics of ALS</td>
<td>University of Michigan</td>
<td>Eva Feldman, MD, PhD</td>
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<tr>
<td><strong>Exp. Treatment of Bulbar Dysfunction in ALS</strong></td>
<td>Center for Neurologic Study</td>
<td>Richard Smith, MD</td>
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<tr>
<td>The Natural History and Biomarkers of C9ORF72 ALS and Frontotemporal Dementia (FTD)</td>
<td>National Institutes of Health/NINDS</td>
<td>Mary Kay Floeter, MD, PhD</td>
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<tr>
<td>Developing a Satellite ALS Center at a Remote Site Incorporating Regional Resources &amp; Telemedicine</td>
<td>University of Kentucky</td>
<td>Edward Kasarskis, MD, PhD</td>
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<tr>
<td>Evaluating Ibudilast MN 166 in subjects with ALS</td>
<td>Carolinas Neuromuscular AL Center</td>
<td>Benjamin Rix Brooks, MD</td>
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National ALS Biorepository

• Launched in January, 2017.

• Collects:
  – Blood, urine, saliva, fingernail clippings, postmortem collection.
  – Phlebotomist will come to person w/ ALS’ house free of charge to collect samples.

• Allows people with ALS to donate specimens that are linked with their survey module data, which can then be shared with researchers.

• Collecting specimens allows for more data, which means more information for researchers.

• Anyone in the US, who is enrolled in the Registry, can participate.
The National Amyotrophic Lateral Sclerosis (ALS) Biorepository

The National ALS Biorepository (Biorepository) is part of the National ALS Registry. It will include samples from persons with ALS (PALS) who are enrolled in the National ALS Registry.

**Biorepository**

The term biorepository usually refers to a facility that collects and stores samples of biological material. These samples could include blood, urine, tissues, cells, DNA, and proteins. Some medical data may also be stored along with a written consent form.

**The Importance of this Biorepository**

The National ALS Biorepository differs from other biorepositories because it does not limit who can take part to a specific area, study, or clinic. The Biorepository samples can be linked with the data on risk factors collected by the Registry. Samples from other studies have been used to find new genes associated with the risk for getting ALS. This Biorepository will also add to the number of samples available for research.

**How to Take Part**

You must be enrolled in the National ALS Registry to take part in the Biorepository. Only PALS who indicate an interest in the Biorepository will get an information packet. After you agree to get more information, you will need to provide your mailing address and phone number. The Biorepository will try to include as many PALS who want to take part, as possible.

**How It Works**

**WHAT MAY BE COLLECTED**

- **In-home** – blood, saliva, urine, hair, or fingernail clippings
- **Postmortem** – brain, spinal cord, cerebral spinal fluid (CSF), pieces of muscle, bone, and skin

**HOW IT’S USED**

Samples collected will be used for future ALS research. Researchers need to apply and have their research approved by ATSDR.

**ALREADY ENROLLED?**

Log into your registry account and update your account.

**NOT ENROLLED?**

Visit the registry website, www.cdc.gov/als, and create an account.

**LEARN MORE AT WWW.CDC.GOV/ALSBIOREPOSITORY OR CALL (855)-874-6912**
Enrolling Information

• People with ALS who are interested in enrolling can visit www.cdc.gov/als to enroll.

• The time it takes to fill out the survey modules varies
  – Have the ability to stop, save and return
  – Can complete 1 module at a time
Extramural Research Funding

- ATSDR funds extramural research to learn more about ALS
- 13 research studies funded to date
- Info learned from these studies will help ATSDR prioritize topics for future risk factor surveys
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<th>Study Name (n=13)</th>
<th>Institution</th>
<th>Investigator</th>
<th>Funding Period</th>
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<tr>
<td>Epidemiology of ALS</td>
<td>Harvard University</td>
<td>Marc Weisskopf, PhD, ScD</td>
<td>2012 – 2013</td>
</tr>
<tr>
<td>Large-scale genome-wide association study of ALS</td>
<td>National Institutes of Health</td>
<td>Bryan Traynor, MD, PhD</td>
<td>2012 - 2013</td>
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<tr>
<td>Gene-environment interactions in ALS</td>
<td>Northwestern Univ.</td>
<td>Teepu Siddique, MD</td>
<td>2012 – 2013</td>
</tr>
<tr>
<td>A Prospective Comprehensive Epidemiologic Study in a Large Cohort in The National ALS Registry: A Step to Identify ALS Risk Factors</td>
<td>Columbia University Medical Center</td>
<td>Hiroshi Mitsumoto, MD, DSc</td>
<td>2013 – 2017</td>
</tr>
<tr>
<td>Identification and Validation of ALS Environmental Risk Factors</td>
<td>University of Michigan</td>
<td>Eva Feldman, MD, PhD</td>
<td>2013 – 2017</td>
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<tr>
<td>Ecologic Study to Evaluate Spatial Relationships between ALS and Potential Environmental Risk Factors</td>
<td>Dartmouth College</td>
<td>Elijah W. Stommel, MD, PhD</td>
<td>2014 – 2016</td>
</tr>
<tr>
<td>Prospective study of biomarkers and risk factors for ALS incidence and progression</td>
<td>Harvard School of Public Health</td>
<td>Alberto Ascherio, MD, DrPH</td>
<td>2014 – 2015</td>
</tr>
<tr>
<td>Case-Control Studies Nested in National ALS Registry to Evaluate Environmental Risks</td>
<td>Columbia University Medical Center</td>
<td>Hiroshi Mitsumoto, MD, DSc</td>
<td>2015 - 2018</td>
</tr>
<tr>
<td>Antecedent Medical Conditions and Medications: Associations with the Risk and Prognosis ALS</td>
<td>Stanford University</td>
<td>Lorene Nelson, PhD</td>
<td>2015 - 2018</td>
</tr>
<tr>
<td>ALS Risk in Latin Americans- A population based case control comparative study with 3 European population based cohorts</td>
<td>Trinity College – Dublin, Ireland</td>
<td>Orla Hardiman, MD, PhD</td>
<td>2016 - 2018</td>
</tr>
<tr>
<td>A Population-Based Ohio ALS Repository and a Case-Control Study of ALS Risk Factors</td>
<td>Dartmouth College</td>
<td>Elijah Stommel, MD, PhD</td>
<td>2016 - 2018</td>
</tr>
<tr>
<td>Environmental risk factors and gene-environment interactions in ALS risk and progression</td>
<td>University of Miami</td>
<td>Michael Benatar, MD, PhD</td>
<td>2016 – 2018</td>
</tr>
<tr>
<td>Identification and Characterization of Potential Environmental Risk Factors for ALS Using the ATSDR ALS Registry Cases and a Control Population</td>
<td>University of Pittsburgh at Pittsburgh</td>
<td>Evelyn O. Talbott, DrPH</td>
<td>2017-2020</td>
</tr>
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Registry Section of ALS Association website

Impact

• More people enrolled = more information on the disease.

• Will help impact/inform funding decisions for research.

• Ability to track and trend potential causes and contributing factors of the disease.

• Learn more about ALS and what causes it.
2nd report on the Registry data

The report represents the 2nd ever population-based estimate of the number of people living with ALS in the United States and included information collected during the ALS Registry’s operation from January 1, 2012 – December 31, 2013.

• In 2013, 15,908 persons were identified as definite ALS,

• Estimated ALS prevalence rates:
  – 2013: 5.0 cases of ALS per 100,000 persons

• The disease was more common among males, whites, non-Hispanics, and people ages 60 – 69
Next Steps

• New report will be released by the CDC before the end of 2017; will have updated information on the data collected.

• Registry Best Practices Meeting – December 14th
  – Chapter representatives and other stakeholders will convene to discuss successes and challenges of Registry promotion
  – Address the challenges and identify potential solutions

• Continue working to promote and facilitate this program.
Questions? Comments?

• Thank you!

• Contact me with further questions and comments – lstanford@alsa-national.org