

2013 ALS Annual Meeting Summary Report Executive Summary

Much remains unknown about what causes ALS. The National ALS Registry was established in 2010 to describe the incidence and prevalence of ALS, describe the demographics of ALS patients, and examine the risk factors for the disease.

This report provides information presented at the 2013 Annual ALS Surveillance Meeting. Each year the Agency for Toxic Substances and Disease Registry (ATSDR) holds this meeting to update stakeholders on the progress of the National ALS Registry and to discuss strategies to further enhance the Registry.

Overview of the National ALS Registry

The ALS Registry Act, enacted as Public Law 110-373 in October 2008, directs CDC/ATSDR to establish and maintain the National ALS Registry. It is the only Congressionally mandated population-based registry for the U.S. ATSDR described the methodology used by the Registry. The Registry combines ALS data from existing national databases (i.e., Medicare, Medicaid, VA Health Administration, and the VA Benefits Administration) with information directly entered into a secure web portal by persons with ALS. In addition to registering, ALS patients are also asked to take brief online risk factor surveys. These surveys will help to answer questions about the potential risk factors for ALS.

An important aspect of the meeting is the discussion following each presentation. This discussion generates recommendations concerning Registry issues and suggestions for enhancing the Registry. ATSDR provided an overview of recommendations from the 2012 meeting and described the actions that have been taken regarding these recommendations.

ATSDR is also implementing several initiatives to strengthen the Registry including:

- the Research Notification System,
- the development of new risk factor surveys to be added to the Registry,
- the Biorepository Pilot Study,
- the State and Metropolitan-Based Surveillance Project, and
- new ATSDR supported ALS research funding opportunities.

Redesigned National ALS Registry Website Demonstration

An ATSDR consultant demonstrated how the Registry website was redesigned in 2013. Many of the changes resulted from suggestions offered during the 2012 annual meeting. The site has been reorganized for clearer and more direct access to the main topic areas. It has been streamlined to make it more useful and easier to navigate. Changes to specific web pages and the addition of new pages were also presented.

National Quantitative Data Findings

ATSDR described the data in the national databases used to populate the Registry. The criteria used in the algorithm developed to identify patients with ALS from the national databases were also explained. The algorithm was developed and tested for sensitivity and specificity through pilot projects conducted in Minnesota, Georgia, South Carolina, and an HMO consortium. The sensitivity was determined to be 0.87 and specificity was 0.85. Using this algorithm, a total of

36,610 cases of ALS were identified in the national databases for the period 2001–2009. These data were also presented by year, age, race, sex and geographic distribution.

Research Notification Mechanism and Research Committee Update

ATSDR described the Research Notification Mechanism and the development of the research committee. This is a system that connects ALS researchers directly with persons with ALS who are registered in the National ALS Registry and have indicated an interest in taking part in studies. ATSDR had received three applications. Applications were received from Harvard University, the Medical University of South Carolina, and jointly from the University of Miami and Massachusetts General Hospital.

National ALS Biorepository Pilot Study

An ATSDR consultant explained what a biorepository is, how they are being used in ALS research, and listed the existing ALS biorepositories. The goal of the National ALS Biorepository Pilot Study is to pilot methods for collecting and banking biological specimens from participants in the National ALS Registry. Information from this pilot study will be used to assess the potential for developing a comprehensive, national research resource associated with the National ALS Registry. The process for obtaining input into the draft ALS biorepository pilot study protocol was described.

Recruitment for the pilot study began in April 2013. To be eligible for the study, participants must be enrolled in the National ALS Registry. The specimens being collected include blood, urine, nail clippings, and hair clippings. Post-mortem collection is also being done. The status of specimen collection was described as of July, 2013 for the in-home study component and the post-mortem study component.

NIH-ATSDR ALS Risk Factor Research Update

Three research projects were presented. Each of these studies proposes to assess genetic and/or environmental risk factors of ALS. These projects were funded by ATSDR through a unique partnership with the National Institutes of Health (NIH). The projects include:

- *The Role of High Density Lipoprotein Particles in ALS,*
- *Environmental Risk Factors for ALS in a Representative Sample of the US Population,*
and
- *Large Genome-wide Association Study in ALS using the NeuroX Genotyping Platform.*

Registry Promotion and Outreach

ATSDR's marketing and promotion strategy for the National ALS Registry involves working with partners throughout the country. Activities include generating awareness of the Registry; encouraging persons with ALS (PALS) to self-register; and engaging persons and organizations that interact with PALS in order to reach the largest number of potential registry participants. The metrics associated with visits to the Registry and new projects and features were also described. Although the Registry continues to be promoted through traditional printed materials and the print media, this effort has been expanded significantly to include social media messaging and online ads.

ATSDR cannot release the data in the Registry until it has been shown to be representative of the ALS population in the US. However, in an effort to assist in promotion and outreach for the Registry, ATSDR is providing qualitative data. Comparison of these data identify states that are lagging below the national participation rate in registering persons with ALS. This information is being shared with its partners at the ALS Association (ALSA) and the Muscular Dystrophy Association (MDA) to assist them in targeting their outreach activities.

ALSA has entered into a contractual agreement with ATSDR to help market the Registry. ALSA described the work they are doing. This work includes conducting a listening tour of all their chapters, through which ALSA learned about rewarding activities, needs, and challenges. Based on this information, ALSA described the development of a comprehensive toolkit. This toolkit has been distributed to its chapters and affiliated clinics and centers across the country. ALSA also described how they are engaging in outreach to health professionals, researchers, veterans, elected state officials, and the general public. Another innovative tactic is ALSA's strategy of reaching out to inform the public about the Registry through Minor League Baseball.

The Muscular Dystrophy Association (MDA) described the many areas in which their organization is providing essential ALS services such as: MDA clinics and MDA/ALS centers, legislation and health policy, equipment, education, outreach, support to patients and family members, and publications. Also described was the tremendous research commitment MDA has dedicated to ALS and MDA's many efforts in information dissemination about the Registry.

[Metropolitan Area-Based ALS Surveillance](#)

The objective of the metropolitan area-based ALS surveillance is to use the data to evaluate the completeness of ATSDR's National ALS Registry. To try and assure complete ascertainment of persons with ALS, the project had to identify and ask every neurologist who had diagnosed or provided care to an ALS patient in the specified metropolitan area from January 1, 2009 to December 31, 2011 to report cases. Eight metropolitan areas participated: Atlanta, Baltimore, Chicago, Detroit, Las Vegas, Los Angeles, Philadelphia, and San Francisco. The methods were described for identifying and recruitment of providers, case ascertainment, quality assurance, and for selection of reported cases for case verification. Results were described for the number of cases reported, age, race, ethnicity, sex, metropolitan area and by practice type. The limitations and recommendations for use of this type of active surveillance for ALS were also described.

[American Academy of Neurology \(AAN\) Registry Task Force – ALS Performance Measures](#)

An update was presented on the AAN Registry Task Force's development of ALS performance measures. The goal is to approach this from an outcome oriented perspective. The reasons were described for developing the quality outcome measures. The subcommittee members involved in developing quality measures were listed, as well as, the other organizations, ALS centers, and other groups. The process for developing measures and the attributes of a good quality measure were described. The format for measures was also described and the time required for developing a measure was broken down into the estimated time for each phase of development. The AAN ALS Performance Measurement Set was presented and examples of measures were reviewed. This effort is particularly relevant to the Registry because it underscores the importance of the information being gathered by the Registry, such as defining the number of persons with ALS and determining how many people are being treated and where. These data

will be absolutely crucial to determine the number of people who may be impacted by any quality outcome measure in development.

[Les Turner ALS Foundation](#)

The Les Turner ALS Foundation described how the Chicago-based foundation was formed in 1977. An idea based on used books sales spawned the creation of the Mammoth Music Mart in 1978. This event continued for 25 years, providing funding to the foundation. Also described was how the foundation has grown over the years. Funds are being used to support ALS research at Northwestern University; the Les Turner/Lois Insolia ALS Center at Northwestern, and a wide variety of patient and family support programs. Ideas were discussed about how the Les Turner Foundation can help promote the National ALS Registry.

[PALS Perspective on the Registry](#)

There were three PALS at the meeting. Each of the PALS shared his perspective on how he is dealing with the disease. They talked about what they have lost and what they have gained, about what is important in their life, and what is not so important. They shared their thoughts on the value of the Registry. They pointed out where the Registry needs to work harder. They recognized the challenges, and they offered their suggestions on how to make it better. And they thanked everyone attending for their hard work on the front lines and behind the scenes.

[Mobile Service Locator Apps](#)

ATSDR's Geospatial Research, Analysis, and Service Program (GRASP), described the ALS Service Locator web application. This application was incorporated into the Registry web site in 2011. This mapping component uses geospatial analysis to locate the five nearest ALS clinics, ALSA chapters, and MDA offices based on the zip code entered by the user. An iPad app has also been developed and was released in September 2012. Also under development is an Android application.

[OMB Continuation Package](#)

An ATSDR consultant explained that the Office of Management and Budget (OMB) is responsible for among other things ensuring that the policies of the Paperwork Reduction Act (PRA) are complied with by federal agencies such as ATSDR. The primary reason for the PRA is to ensure that when citizens are requested by government to do activities, that the completion of these activities should have the smallest burden possible. OMB approval of the Registry must be requested every three years. The current OMB approval is due to expire July 31, 2013. The ATSDR consultant described what is involved in the lengthy application process. Also described were some of the changes that will apply for this renewal and their impact. The timeline for the renewal process, which began in September 2012, was presented. The original approval of the application included terms of clearance, which would not allow the dissemination of results until it was clear to OMB that the results were representative of the U.S. ALS population. It is anticipated that there will be terms of clearance included with the renewal application which will allow for dissemination of the results with a disclaimer describing the limitations of the results.

[Next Steps](#)

This session was intended to generate open-ended discussion. ATSDR opened the discussion with several questions related primarily to next steps in terms of releasing the Registry

information. Considerable discussion centered on the data elements that should be disseminated, the usefulness of including an online query tool with de-identified aggregate data, and the formats to be used to disseminate data.