Helping Jerry's Kids

3300 East Sunrise Drive, Tucson, AZ 85718-3299
Tel. (520) 529-2000 • Fax (520) 529-5300
www.mda.org • mda@mdausa.org

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Maryam I. Daneshvar
CDC Acting Reports Clearance Officer
Centers for Disease Control and Prevention
1600 Clifton Road
MS-D74
Atlanta, GA 30333
omb@cdc.gov

RE: Proposed Project: Registration of individuals with ALS in the National ALS Registry – New – Agency for Toxic Substances and Disease Registry (ATSDR), Coordinating Center for Environmental Health and Injury Prevention (CCEHIP), Centers for Disease Control and Prevention (74 FR 101, May 28, 2009). [60day-0923-09BR]

Dear Acting Reports Clearance Officer Daneshvar:

The Muscular Dystrophy Association submits the following comments on the above referenced rule. The Muscular Dystrophy Association ("MDA") is a national voluntary health association committed to funding cure-driven research and comprehensive medical services to Americans affected by more than 40 different neuromuscular diseases including Amyotrophic Lateral Sclerosis (ALS). MDA is the world's leading nonprofit ALS research and service organization directly dedicating more than $250 million dollars to ALS research and service programs since inception, $ 33 million of which is currently being dedicated to ALS this year. Through our 215 hospital-affiliated MDA clinics, 35 MDA/ALS Centers, 5 MDA ALS Clinical Research Network Centers, a far-reaching ALS service program implemented through more than 160 MDA offices in the U.S., and our unparalleled research program which currently funds more than 50 ALS research projects worldwide, MDA aims to be the source of education, outreach, support and hope to Americans affected by ALS.

The proposed project establishes a national ALS patient registry as authorized by S. 1382: ALS Registry Act, which amended the Public Health Service Act to provide for the establishment of an Amyotrophic Lateral Sclerosis (ALS) Registry. As outlined in the legislative language, the proposed registry will collect personal health information from the ALS patient community in an effort to provide a basis for further scientific studies into potential causes and treatments for ALS. The proposed registry aims to: (1) better describe the incidence and prevalence of ALS in the United States; (2) examine appropriate factors that might be associated with ALS; (3) better outline key demographic factors in ALS; and (4) better examine the connection between ALS and differential diagnoses that are often confused with, or thought to progress to, ALS.

Issue Impact of the ALS Community:

The Muscular Dystrophy Association has long recognized the importance of and need for nationalized patient registries for the diseases within our program and currently provides financial grant support to a number of disease-specific registries; including one ALS registry. Since 2003, MDA has provided $5.3 million in funding for infrastructure to support registry development and data collection. Such funding includes direct support for specific registries as well as support for clinic research networks that have a registry component and/or are conducting natural history studies, development of clinical trial outcome measures and genotype-phenotype correlations.

A national ALS registry will help combat the bottleneck in the therapy development and drug approval process. ALS is a rare disease which makes finding and enrolling people in clinical studies complicated. There is a lack of incentives for industry to develop drugs for small markets, with the cost of new drug development estimated at close to $1 billion. These costs are not generally scalable based on the size of the market for a given therapy. Progress is also slowed due to the lack of current natural history data for ALS and clinical endpoints, making it difficult to plan and power statistically meaningful studies. The conduct of studies is also complicated by lack of specificity in standards of care and significant diversity in the delivery of care, which introduces additional variability into already underpowered studies.

The impact of developing an efficient clinician-curated ALS registry would be felt both in advances in treatment and therapy development and improvements in clinical care. In addition, the registry would provide important data regarding the number of individuals currently living with ALS and the natural history of the disease.

Technical Recommendations for Design and Implementation of National ALS Registry:

We believe the ALS registry should utilize a single platform to manage data collection and reporting. Access to the registry through national ALS organizations' websites and clinic programs would ensure vast use of the registry by the ALS community. The diverse community of experts in the field of motor neuron disease allows the CDC to call on broad representation from the larger neuromuscular community to advise on design, development, implementation and data sharing through an advisory board and working groups. As specified in the legislative language, the advisory board and working groups should consist of experts from the ALS research and clinical community, representatives from national ALS patient advocacy groups, people with ALS, and representatives from other existing ALS registry advisory groups.

The advisory and working groups will need to address the fragmentation that inherently exists by having more than one registry collecting data for ALS and work to standardize the collection of clinical data among the registries. MDA recommends that these registries work with the CDC and advisory committees/working groups to develop core data elements and agree on a plan to integrate efforts.

For the CDC to achieve the goals of better describing the incidence and prevalence of ALS in the United States and to better examine the connection between ALS and differential diagnosis, it's vital the registry consists of either clinician-entered data or clinician-curated information. However, we feel patients should also be able to enter data directly into the registry, thereby providing a greater depth of information available for analysis and a wider participant/registrant reach. Advisory committees can be charged with developing standards and terminology for data collection, protocols for data curation, privacy, security and data sharing. A successful registry architecture will allow for tracking and reporting of individual and aggregate data over time, and be able to accommodate modules for additional studies such as genotype-phenotype correlations, natural histories, burden of disease, clinical trials endpoint development and validation, and phase 4 surveillance studies. The national ALS registry should also be built for compatibility with existing international ALS registries, thereby providing a global platform for ALS data obtainment and accelerated scientific discovery.

Critical to the success of the registry will be the availability of reports to study participants, clinicians, researchers, and industry. Patients should have access to view and print their personal records. Clinicians should have the ability to review data entered through their individual clinics and compare that information to aggregate data in the registry. As the registry matures and a critical body of data accumulates, researchers can leverage the analytic capability of the registry through the registry's web interface and through specific requests to the CDC to help them design their specific research studying based on the use of the registry system data.

In the absence of mandatory ALS reporting laws, the CDC will have to work closely with national ALS patient advocacy groups and national healthcare systems to maximize outreach and education efforts within the ALS patient community in order to solicit the participation of the majority of ALS patients in the United States. In addition to providing information about the registry's existence and importance via these organizations' national websites and publications, participant recruitment would be greatly expedited by the CDC's provision of electronic access to the ALS registry web portal at ALS clinical care centers supported by the major ALS organizations.

Conclusion:

While the Muscular Dystrophy Association strongly supports the CDC's efforts to develop a national ALS registry to accelerate the ALS community's quest for treatments, cures, and eventually prevention of ALS, we urge you to ensure that such a registry has clear goals, is designed through expert input and oversight, takes into consideration the experiences of existing registry projects, ensures compatibility with a global ALS scientific community and is implemented in partnership with national ALS patient advocacy organizations.

Thank you for the opportunity to submit these comments. We are available to discuss these issues with you further at your convenience. Please contact either me or MDA's Vice President – Advocacy, Annie Kennedy at 202-828-8560.

Sincerely,

Valerie Cwik, M.D.
Senior Vice President – Research & Medical Director
Muscular Dystrophy Association

cc: Annie Kennedy, Vice President - Advocacy