

Evaluation of the Paul D. Wellstone Muscular Dystrophy Research Centers

A report of the Wellstone Center Evaluation Working Group to the National Arthritis and Musculoskeletal and Skin Diseases Advisory Council

Executive Summary

NIH has funded the Paul D. Wellstone Muscular Dystrophy Research Centers since it established the program in 2003. Six active Centers—with support from the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), the National Institute of Neurological Disorders and Stroke (NINDS), the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), and the National Heart, Lung, and Blood Institute (NHLBI)—comprise the program.

Although NIH has refined the program over the past 15 years, NIH determined that a comprehensive review of the Wellstone Centers program was appropriate because of significant changes in the neuromuscular disease research landscape since 2003. Therefore, it undertook a formal evaluation to identify best practices for achieving the Wellstone Centers' goals of

- supporting impactful basic, preclinical translational, and clinical research in the muscular dystrophies (MDs) through synergistic projects;
- developing and broadly distributing resources that accelerate muscular dystrophy research;
- facilitating the training of the next generation of muscular dystrophy researchers and clinical scientists; and
- enabling connections with the patient community.

The NIH convened a Working Group of the NIAMS Advisory Council that reviewed data collected from various sources including Wellstone Center progress reports, NIH administrative data, information extracted from publications and Wellstone Center websites, interviews with principal investigators and other stakeholders, and a Request for Information that was advertised to the broad research and patient advocacy communities.

This report to the NIAMS Advisory Council presents the Working Group recommendations on potential enhancements to the Wellstone Centers program, as well as aspects that should be continued, in five areas: research portfolio, Center structure and implementation, community engagement, research resources, and training and career development. The recommendations described in the report are listed here.

Research Portfolio

- 1) Continue to support a Wellstone Centers program that covers a range of diseases, including the rarer forms of muscular dystrophy.
- 2) Expand the scope of the Wellstone Centers program to encompass muscle diseases with related pathophysiological mechanisms and similar clinical manifestations.
- 3) Maintain a balance between clinical and preclinical research.
- 4) Strongly encourage Wellstone Centers to pursue research activities to advance clinical trial readiness, including the development or leveraging of natural history studies and linkages to patient registries.

Center Structure and Implementation

- 1) Maintain flexibility regarding the extent to which individual Center personnel should be involved in multiple Center projects and shared resource cores.
- 2) Continue to require that each Center contain at least one clinical research project while allowing the Centers to have flexibility regarding the type of project.
- 3) Encourage Centers to seek out opportunities for interdisciplinary collaborations and to partner with external scientists with complementary expertise.
- 4) Work closely with Wellstone Center principal investigators (PIs) to plan the Wellstone Center biennial meetings so that they include non-Wellstone PIs and other muscular dystrophy stakeholders who could add expertise or perspective.

Patient Community Engagement

- 1) Urge Centers to listen to the concerns of the patient community and to clearly communicate the role of and opportunities for the patient community to collaborate with the Centers.
- 2) Encourage Centers to bring groups together to advance shared scientific interests.
- 3) Encourage Wellstone PIs to strengthen interactions with the broader muscular dystrophy patient advocacy stakeholder community on both a national and a local level.
- 4) Encourage Centers to work with patient groups to develop appropriate patient-reported outcomes (PROs).

Research Resources

- 1) Require Cores to maintain an active website that clearly lists resources, the process by which the resource can be obtained (e.g., a standard form), the process for how requests are prioritized, cost, and potential wait times.
- 2) Ensure that Centers make preclinical research resources from research cores and from individual projects broadly available.
- 3) Highlight the importance of sharing clinical research resources either through research resource cores or as part of a scientific project.

Training and Career Development

- 1) Continue to require that Wellstone training cores provide stipend and salary support to trainees.
- 2) Encourage Wellstone Centers to organize training activities that could be utilized by the entire muscular dystrophy scientific community.
- 3) Continue to encourage Wellstone Centers to leverage existing training resources and financial support through their home institutions.
- 4) Encourage Wellstone Centers to periodically convene meetings for trainees at the level of the Wellstone network.
- 5) Encourage Centers to expose non-clinical trainees at all levels (i.e., graduate students and postdoctoral fellows) to clinical aspects of muscular dystrophy research.
- 6) Encourage participation of clinical fellows, residents, and other clinicians on Wellstone projects and activities.