

DUCHENNE AND BECKER MUSCULAR DYSTROPHY

Center for Disease Control and Prevention

Birth Defects, Developmental Disabilities, Disability and Health - Within the total provided, the following amounts are provided for the specified activities above the comparable amount for fiscal year 2004: \$1,500,000 to expand surveillance and epidemiological efforts of Duchenne and Becker muscular dystrophies.

National Heart, Lung, and Blood Institute

Duchenne Muscular Dystrophy- The Committee encourages NHLBI, in collaboration with the Office of Rare Diseases, to enhance the research and related activities surrounding pulmonary and cardiac complications associated with Duchenne muscular dystrophy. The NHLBI is expected to report to the Committee on its approach to this issue not later than January 15, 2005.

National Institute of Neurological Disorders and Stroke

Duchenne Muscular Dystrophy- The Committee urges NINDS, in collaboration with NIAMS and NICHD, to dedicate additional resources to develop a standard of care and improve treatments, accelerate clinical trials and translational research for patients with DMD. The Committee strongly encourages the funding of three additional Centers of Excellence and doubling of the funding for existing Centers of Excellence by the end of fiscal year 2005. In addition, the Committee notes that the NIH Interagency Coordinating Committee for muscular dystrophy has missed its 1-year deadline to submit a plan for conducting and supporting research and education on muscular dystrophy through NIH in accordance with the MD CARE Act of 2001. The Committee strongly urges the Coordinating Committee to finalize and submit this plan, as well as include representation from the Department of Defense on the panel, as soon as possible.

The Committee was disappointed with the July 2004 'Muscular Dystrophy Research and Education Plan for the National Institutes of Health'. The Plan developed by the MD Interagency Coordinating Committee, provides a summary of research goals, but lacks sufficient detail into baseline research efforts, funding, and technical progress. In compliance with the provision of the Muscular Dystrophy Care Act of 2001 (Public Law 107-84, 'MD Care

Act'), the report from the Coordinating Committee should be sufficient to support science and research objectives; thus providing an investment strategy and technical roadmap. In addition, the Committee notes that in accordance with the MD Care Act, the Department of Health and Human Services was to establish an Interagency Coordinating Committee whose responsibility was the development a plan on research and education on Muscular Dystrophy. The Committee is also disappointed with the speed at which the Act is being implemented. The Act became law in 2001 and the coordinating Committee did not convene until July 2003. Therefore, the Committee requests that, not later than February 1, 2005, the NIH shall provide an submit an additional report which should contain a plan which establishes research goals; the Institute or Center responsible for that research, the amount of funding spent in fiscal year 2000 through fiscal year 2004 and the amount of funds that NIH will devote to this research in fiscal year 2005 and 2006; key technical risk areas; integration efforts between NIH Institutes and Centers; and all opportunities for transition of technology to industry; translational research; major milestones and clinical trial opportunities.

Duchene Muscular Dystrophy Translational Research- The Committee commends the Institute for its enhanced efficiency in the grant review cycle to fund translational research for Spinal Muscular Atrophy [SMA]. The Committee strongly endorses this accelerated research approach and urges the Institute to evaluate the merits of this review model to fund translational research for Duchenne Muscular Dystrophy and report back to Congress no later than April 2005. The Committee believes the SMA model enhances the dialogue between reviewers and scientists, facilitating modifications of grant proposals that are in the best interest of the patients.

In addition, the Committee strongly urges the Institute to increase funding for basic and translation DMD research by aggressively soliciting grant applications. The Committee encourages the Institute to employ existing mechanisms such as a standing program announcement, websites, publications, workshops and conferences to promote awareness of funding opportunities. In addition, the Committee encourages the NIH to further develop opportunities for upcoming clinical trials and to help make available all ongoing patient care options. The NIH is encouraged to collaborate with other Departments, Federal agencies and Patient Groups to develop formal programs that increase public and professional awareness of the disease. The Committee requests that NIH report back to the Committee, no later than

April 2005 with a progress report on all aspects of DMD translational research.

National Institute Of Arthritis And Musculoskeletal And Skin Diseases

Duchenne Muscular Dystrophy [DMD]- The Committee urges NIAMS, in collaboration with NINDS, to dedicate additional resources to improve treatments for patients with DMD.

Office of the Director

Duchenne Muscular Dystrophy- The Committee strongly encourages the funding of three additional Centers of Excellence and to double the funding for existing centers of excellence by the end of fiscal year 2005. In addition, the Committee notes that the NIH Interagency Coordinating Committee for muscular dystrophy has missed its 1-year deadline to submit a plan for conducting and supporting research and education on muscular dystrophy through NIH in accordance with the MD CARE Act of 2001. The Committee strongly urges the Coordinating Committee to finalize and submit this plan, as well as include representation from the Department of Defense on the panel, as soon as possible.

In addition, the Committee strongly urges the NIH's to devote additional resources to DMD translational research and clinical trials. This funding is to be separate from the Centers of Excellence program. Based on the severity of this disorder, its high incidence in children, and the prospect of important new treatments, the Committee strongly urges OD, through NIAMS and NINDS, to follow the accelerated model for funding DMD translational research that was used for motor neuron diseases, including ALS and SMA. The Committee strongly endorses that plan and urges the OD to equip the program and the personnel charged with executing the plan with appropriate authoritative and financial resources to maximize the chances of success.

Finally, the Committee encourages the OD to further develop opportunities for upcoming clinical trials and to help make available all existing and emerging patient care options. The Institutes are encouraged to work with other Federal agencies to develop formal programs that increase public and professional awareness of the disease.

Health Costs, Quality, and Outcomes

Duchenne Muscular Dystrophy--The Committee urges AHRQ to study and develop recommendations on the need for standards of care for individuals with Duchenne muscular dystrophy, allowing for input from external entities including parent advocacy programs. In addition, the Committee encourages AHRQ to conduct a workshop on standards of care for the muscular dystrophies and coordinate this activity with national advocacy organizations dedicated to this condition.