

# United States Senate

WASHINGTON, DC 20510

March 28, 2007

## HELP SUPPORT PATIENTS WITH DUCHENNE MD AND THEIR FAMILIES

Dear Colleague,

Duchenne Muscular Dystrophy (MD) is the world's top lethal genetic disease in children. Those affected typically survive only into their twenties.

Duchenne is the most common form of the more than 20 different forms of muscular dystrophy. It affects approximately one out of every 3,500 boys, with about 20,000 new cases each year. Becker MD is a less severe form of MD associated with Duchenne that affects about one out of every 18,000 children.

Recent support provided by Congress, however, has many in the Duchenne and Becker (DBMD) community feeling encouraged. Thanks to Congressional support, the National Institutes of Health (NIH) has been able to increase funding for DBMD. While DBMD barely registered on the nation's radar screen just a few years ago, today we are in the midst of a Phase 2 clinical trial for a drug that shows significant promise for DBMD patients.

Work at the Centers for Disease Control and Prevention (CDC) is equally promising. Thanks to Congressional support, CDC is continuing to track and record cases of DBMD, while also supporting important educational and outreach programs.

While progress is being made, we ask that you help us ensure that this work is continued by signing the attached letter to the Chair and Ranking Members of the Labor-HHS Appropriations Subcommittee requesting increased funding for MD activities at the NIH and the CDC. Specifically, the letter requests a \$2.5 million increase in activities at the CDC and increased funding at the NIH to ensure the continued support of the six MD Centers of Excellence and other research initiatives focused on DBMD.

We hope that you will join us in helping to advance critical DBMD research and public health programs by signing on to the attached letter. Please contact Priscilla Hanley with Senator Collins at 4-2523 or Eleanor Dehoney with Senator Brown at 4-2315 if you have any questions or would like to sign onto the letter.

Sincerely,



Susan M. Collins  
United States Senator



Sherrod Brown  
United States Senator

April 2007

Dear Chairman Harkin and Ranking Member Specter:

On behalf of all of the patients and families affected by Duchenne and Becker Muscular Dystrophy (DBMD), we are writing to thank you and the Labor, HHS and Education Subcommittee for your enthusiastic support in recent years and to urge your continued support for measures to develop safe and effective treatments for these conditions.

Duchenne is the most common form of the muscular dystrophies, affecting about one of every 3,500 boys. Becker is a less severe version that affects about one of every 18,000 children born each year. Duchenne is the world's most common lethal genetic disease among children, with patients typically just surviving into their twenties.

The DBMD community, however, is encouraged by recent progress and believes that we are getting closer to the development of a safe and effective treatment. Thanks in large part to previous support by your committee, the National Institutes of Health (NIH) has been able to increase funding for DBMD research and has ensured that this funding has been carefully coordinated and used efficiently. While DBMD barely registered on the nation's radar screen just a few years ago, today we are in the midst of a Phase 2 clinical trial for a drug that shows significant promise for our patients.

Your committee's past support has also enabled the Centers for Disease Control and Prevention (CDC) to track and record cases of DBMD and to develop programs to educate clinicians about early warning signs so patients can receive prompt and appropriate care. To help ensure that these important efforts can continue, we respectfully request that you provide the following in the Fiscal Year 2008 Labor, HHS, and Education Appropriations bill:

- A \$2.5 million increase in MD activities at the CDC. Of this amount, \$2.25 million should be dedicated to advance efforts to develop and launch an International DBMD Patient Registry, and \$250,000 would be used to continue the successful joint CDC/PPMD Education and Outreach initiative, bringing the total for this project to \$1 million.
- Increased funding at the NIH to ensure the continued support of the six MD Centers of Excellence and other research initiatives focused on DBMD;
- Report language, to be submitted via appropriate channels, to advance NIH's significant investment into MD research and to complete a joint CDC/Agency for Healthcare Research and Quality (AHRQ) project to issue a set of evidence-based care considerations to guide clinician care of patients with DBMD.

On behalf of all Americans affected by Duchenne and Becker Muscular Dystrophy, we thank you for your leadership and urge your continued support.

Sincerely,