

THE  
2ND DECADE



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May 23, 2017

Dr. Janet Woodcock  
Director,  
Center for Drug Evaluation and Research

Dr. William Dunn  
Director,  
Office of Drug Evaluation 1 – Division of Neurology Products

Dr. Theresa Mullin  
Director,  
Office of Strategic Programs, Center for Drug Evaluation and Research

Dr. Jonathan Goldsmith,  
Associate Director, Rare Disease Program, Office of New Drugs

Food and Drug Administration

*via digital transmission*

Dear Drs. Woodcock, Dunn and Mullin:

The Myotonic Dystrophy Foundation is very pleased to submit a *Voice of the Patient Report* generated from our Externally-Led Patient-Focused Drug Development meeting held in September 2016 in Beltsville, MD. It is our hope that you will forward this document to all appropriate FDA officials for use in regulatory decision-making, and include it in the public database of externally-submitted Voice of the Patient Reports under PDUFA VI. We are particularly interested in ensuring that this Voice of the Patient Report is shared with the Division of Neurology Products and the Rare Disease Program.

As the first Externally-Led Patient-Focused Drug Development meeting approved by the FDA, we appreciated the attention and support your offices provided to this effort. We are particularly grateful for Drs. Woodcock, Dunn and Goldsmith's attendance at the meeting, and the informative and insightful presentations from Dr. Woodcock and Goldsmith. We believe this meeting demonstrated the significant unmet medical need people living with myotonic dystrophy experience and the critical need for meaningful therapies, as well as the sincere commitment the FDA has made to supporting and assisting patient advocacy groups in bringing the patient voice to drug development and review. Thank you again for your ongoing support and enthusiasm.

The Myotonic Dystrophy Externally-Led Patient-Focused Drug Development Meeting was held on September 15<sup>th</sup>, 2016 at the Sheraton College Park in Beltsville, MD. More than 200 people from families living with myotonic dystrophy, as well as academic professionals and industry representatives attended, as did nine representatives from the FDA. The meeting was livestreamed, and included input and live polling from over 100 remote participants as well as those in the room. A 30-day comment period allowed additional community members to take part. This report includes all input captured during the meeting and the comment period.

Panelists and meeting participants provided moving and illuminating descriptions of the daily and progressive struggles they encounter living with myotonic dystrophy or as caregivers of people living with this disease, and what they would find meaningful in a DM therapy or treatment. Video recordings of the meeting are available here: <http://www.myotonic.org/patient-focused-drug-development-meeting-part-1-2016-mdf-annual-conference> (part 1) and here: <http://www.myotonic.org/patient-focused-drug-development-meeting-part-2-2016-mdf-annual-conference> (part 2). A very short film, *Challenges of DM*, which provides additional insights from people living with DM, which was screened at the meeting and is available here: <http://www.myotonic.org/challenges-dm>.

This information will provide important additional data and insights to the FDA, industry and other stakeholders seeking to understand the myotonic dystrophy disease burden and develop potential therapies for this population. We respectfully request that this report be circulated to all appropriate FDA divisions and individuals and included in the public database for externally-led PFDD meetings.

Thank you again, for your insights, support and participation in this meeting. We believe the Patient-Focused Drug Development program and other patient-focused efforts the FDA has initiated have brought critically important insights and information to the drug development and review process in the U.S., and we deeply appreciate your efforts to shepherd and support these initiatives. I look forward to your comments and feedback. Please feel free to contact me with any questions or comments.

Sincere regards,

A handwritten signature in black ink, appearing to be 'Molly White', with a long horizontal line extending to the right.

Molly White  
Chief Executive Officer