LIVING WITH DM: PATIENTS REPORT ON CHANGES OVER TIME

Charles Thornton, MD: Moderator
Session Objectives

For DM patients and their caregivers to help researchers and drug developers understand:

- The burden of living with a progressive disease
- How you currently manage disease change and progression – what works and what doesn’t
- What meaningful treatments that address progression would do
Why We Need Your Voice

- Your experience and perspective are important.
- DM is variable; drug developers and researchers need to hear from many people what disease changes and progression are like in DM.
- Your input will lead to better therapies and faster development of drugs that treat progression.
- Your input will help drug developers identify a clinical trial endpoint (measurement) that examines progression.
## Why We Need the Patient Voice

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<table>
<thead>
<tr>
<th>Burden of Disease</th>
<th>Natural History</th>
<th>Disease Impacts</th>
<th>Available Therapies</th>
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<tbody>
<tr>
<td>Burden of Disease and the impact on daily living</td>
<td>First-hand accounts of natural history and disease progression</td>
<td>What disease impacts matter most to patients to inform outcome measures</td>
<td>If there are therapies available and if those therapies are meeting the patients’ needs</td>
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<th>Unmet Medical Need</th>
<th>Trial Endpoints</th>
<th>Risk Tolerance</th>
<th>Clinical Trial Burden</th>
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<td>The impact of unmet medical need— an understanding of untreated aspects of the disease</td>
<td>How well clinical trial endpoints align with outcomes that matter most to patients</td>
<td>Patients’ attitudes towards risk tolerance</td>
<td>Are clinical trials causing unnecessary burden to the patient and impacting retention?</td>
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MDF has been educating the Food and Drug Administration (FDA) that reviews and approves new drugs for the US for several years:

• **2015** – **DM Regulatory Workshop** at 2015 MDF Annual Conference
  • Speakers from many FDA divisions
  • Over 80 attendees from academic research labs and industry
  • **OUTCOME**: Meeting report published in *Therapeutic Innovation and Regulatory Science*

• **2016** – **First Patient-Focused Drug Development (PFDD)** meeting on DM
  • 4 hours
  • Over 200 community members and research and industry professionals
  • Key speakers: people with DM talking about disease burden and what they want DM therapies to do
  • **OUTCOME**: Voice of the Patient Report published on FDA website
DM & Patient-Focused Drug Development

• 2017 – Patient-focused drug development meeting on CNS (brain-related) DM symptoms
  • Over 300 attendees: family members, researchers, industry members
  • Report from people living with DM on the impact and burden of CNS symptoms and what CNS-targeting DM therapies should do
  • OUTCOME: Formal report prepared; reviewed and posted to MDF website

• 2018 – Patient-focused drug development meeting on DM and disease changes and progression
  • Over 400 attendees: family members, researchers, industry members
  • Helping drug developers understand how DM changes over time, and how to develop a drug to target disease changes and progression
  • OUTCOME: Meeting report and publication
DM & DISEASE PROGRESSION

- Not well understood
- Not enough data
- Critical to design successful clinical trials
- Current barrier to industry engagement in drug development
- Important step: capturing patient perspectives on progression and progression triggers

Reference: National Institutes of Health
Our Panelists

- Judith Kroll – Living with DM2
- Lorraine Piechota – Living with DM2
- Saurabh Rai – Living with DM1
- Dean Sage – Living with DM1
- Kristen Vassallo – Living with DM1
Progression Session Questions

1. Describe a time when your disease took a turn for the worse - (life events, medical events, or other that led to disease changes)

2. Discuss triggers that you have experienced that have generated changes in the disease, such as:
   - A stressful event or situation
   - The flu or other illness
   - New or changed medications
   - Pregnancy, surgery, etc.

3. In managing your disease changes over time, what has worked and what hasn’t?

4. How would a clinically meaningful therapy affect the progression of your symptoms?
Audience Participation

Please:

- **State your name and your relationship to DM** (DM1, DM2, caregiver, other)
- **State the number of years you have been living with DM symptoms**
- **Answer One of the Following Questions:**
  - Describe a time disease symptoms have become worse
  - Describes triggers that have driven disease changes
  - Management strategies for disease changes (successful and unsuccessful)
  - If a treatment was available that stopped progression, what effects or changes would be most important to you?
Tips for Effective Participation

- If you have something important to share, relate it to one of the discussion questions.

- It is OK to reiterate a feeling/experience already voiced by someone that is similar to your own, but give it a personal or unique perspective.

- *Keep your comments concise and focused;* there are many voices that need to be heard here today.
THANK YOU!

- Your answers and insights are important to drug development and the review of potential therapies by the FDA.
- Your insights today will amplify the data we are collecting on disease progression (natural history).
- This information will be put into a report and published to help researchers and drug developers.
- If you have additional comments, contact MDF in the next 30 days: info@myotonic.org