MYOTONIC DYSTROPHY FOUNDATION

Care and a Cure

2018

MDF ANNUAL CONFERENCE

September 14-15, 2018

Nashville, TN

LIVING WITH DM: PATIENTS REPORT ON CHANGES OVER TIME

Charles Thornton, MD: Moderator

UNIVERSITY of ROCHESTER

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

- Your experience and perspectives 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

Burden of Disease

Burden of Disease and the impact on daily living

Natural History

First-hand accounts of natural history and disease progression

Disease Impacts

What disease impacts matter most to patients to inform outcome measures

Available Therapies

If there are therapies available and if those therapies are meeting the patients' needs

Unmet Medical Need

The impact of unmet medical need-- an understanding of untreated aspects of the disease

Trial Endpoints

How well clinical trial endpoints align with outcomes that matter most to patients

Risk Tolerance

Patients' attitudes towards risk tolerance

Clinical Trial Burden

Are clinical trials causing unnecessary burden to the patient and impacting retention?

DM & Patient-Focused Drug Development

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

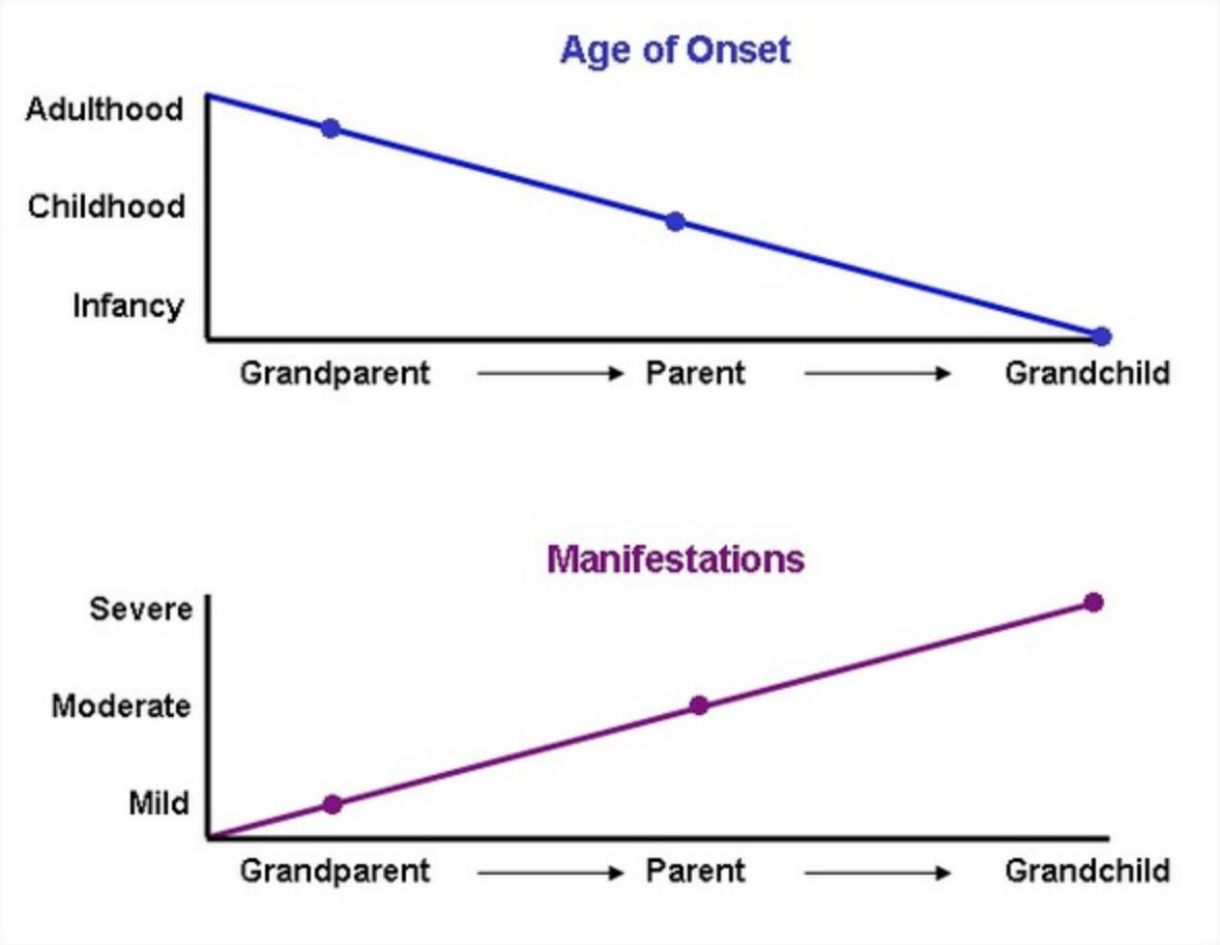
DM & Patient-Focused Drug Development

- 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

The Patient Voice & Progression

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