Bringing the Patient Voice to CNS Targeting Drug Development in DM

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Purpose of this session?

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

- ✓ What it is like to live with CNS-related impacts of DM

- ✓ How complex and varied these impacts are

- ✓ Your concerns about CNS-related symptoms and progression

- ✓ How you currently manage CNS-related symptoms

- ✓ What meaningful treatments look like

Why are we focused on CNS-related symptoms of DM?

- On September 15, 2016, MDF hosted the first ever externally-led Patient-Focused Drug Development meeting
  - Well over 200 community members, industry professionals, academic researchers and FDA representatives attended the meeting live and via the live stream
  - Focus was to improve the FDA's ability to assess the benefits and risks of future therapies for DM, and the Agency's ability to provide real benefit to patients
  - May 23, 2017, MDF submitted Voice of the Patient report to FDA
- The PFDD meeting focused on all symptoms and burdens related to DM
- Researchers and MDF noted that there could be a more robust discussion of CNS-related symptoms of DM

Why should you participate?

- Opportunity to have <u>your voice</u> heard

- Researchers and companies responsible for studying treatments for DM need to know what is important to you related to your CSN-related symptoms

- Your input can lead to better treatments and faster development of new drugs that treat the CNS-related symptoms of DM

- Specifically, today your input will aid in the selection and development of new CNS-related endpoints for clinical trials in DM

What does the patient voice have to do with drug development and selection of endpoints in clinical trials?

FDA Benefit-Risk Decisions for New Drugs

- To adequately assess benefits and risks, FDA must understand the context in which a potential therapy will be used
- Two relevant categories of patient experience:
  - The disease and its impact on patients' daily lives (what is the severity of the condition?)
  - Patients' perspectives on the adequacy of available therapies (what is the current state of the treatment armamentarium?)
- This helps FDA understand the types of benefit that matter most to patients

Use in Benefit-Risk Decision-Making

- Structured Benefit Risk Assessment Framework

Decision Factor	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition	**Summary of evidence:**	**Conclusions (implications for decision):**
Unmet Medical Need	**Summary of evidence:**	**Conclusions (implications for decision):**
Benefit	**Summary of evidence:**	**Conclusions (implications for decision):**
Risk	**Summary of evidence:**	**Conclusions (implications for decision):**
Risk Management	**Summary of evidence:**	**Conclusions (implications for decision):**
Benefit-Risk Summary and Assessment		

Questions that inform benefit-risk decisions

Burdens of CNS-related symptoms of DM

✓ Of all the CNS-related symptoms that you experience because of DM, which 1-3 symptoms have the most significant impact on your life?

✓ Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your CNS-related symptoms?

✓ How do your CNS-related symptoms and their negative impacts affect your daily life on the best days? On the worst days?

✓ How have your condition and its symptoms changed over time?

✓ What worries you most about your CNS-related symptoms?

Approaches to treatment of CNS-related symptoms of DM

✓ What are you currently doing to help treat CNS-related symptoms? How well do these things control your condition?

✓ Assuming there is no complete cure for your CNS-related symptoms, what specific things would you look for in an ideal treatment for your condition?

Role of the Patient Voice in Endpoint Selection

- Clinical outcome assessments (COAs) are a certain type of endpoint that measures of how a patient feels or functions
- Can be:
  - Measure of performance
  - Clinician-reported
  - Observer-reported
  - Patient-reported
- Does not include:
  - Survival
  - Biomarkers
- Used in adequate and well-controlled studies to support FDA approval

Role of the Patient Voice in Endpoint Selection

- How do we know what the right concept of interest to measure?

- What opportunities are there to measure a concept of interest in patients' daily life?

- Do we know what result on a measure is considered clinically meaningful?

Overview of Session Agenda

- Clinical overview of CNS-related impacts of DM by phenotype (Dr. John Day)
- Panel of DM patients and caregivers living with CNS-related symptoms
  - Purpose: to set the foundation for the broader audience discussion
  - Panelists reflect a diverse range of experiences: age of onset, level of progression, key symptoms etc.
- Moderated discussion with audience participants after panel
- Summary remarks and initial thoughts on endpoint selection and development (Dr. Gersham Dent)
- Closing remarks

Discussion Questions

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Tips for Effective Participation

- Remember the purpose of this session:
- If you have something important to share, relate it to the most appropriate discussion question
- 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 to be heard about this important topic
- You can always send in additional comments to MDF after the meeting