

FDA Update

Chronic Fatigue Syndrome Advisory Committee

JUNE 20-21, 2018

Introducing myself

- Associate Director for Regulatory Science for the Office of Drug Evaluation II
 - Division of Pulmonary, Allergy and Rheumatology Products

- Main Focus
 - External Engagement
 - Guidances
 - Research

Fostering Drug Development in ME/CFS

- CFSAC Clinical Trials and Treatment Working Group
- Drug Development Activity
- Challenges for Drug Development
- ME/CFS Guidance
- Clinical Trial Endpoints

Fostering Drug Development in ME/CFS: Drug Development Activity

- 8 molecules or molecule combinations currently under development
- Discussions with 3 commercial sponsors about proposed drug development programs

Fostering Drug Development in ME/CFS: Challenges

- Underlying mechanisms poorly understood
- Variability in nature and severity of symptoms
- Defining patient population
- Selecting efficacy endpoints

Fostering Drug Development in ME/CFS: Guidance for Industry

- Patient population:
 - Any of the defined diagnostic criteria set acceptable
 - Provide justification
 - Define whether studied patients represent the general ME/CFS population or a subset

- Efficacy endpoints:
 - Patient-reported symptoms using well-defined and reliable PRO instruments.
 - These can include any scientifically supported and logical combination of symptoms that are common in CFS/ME.

Fostering Drug Development in ME/CFS: Guidance for Industry

- Draft published in 2014
- FDA received comments from stakeholders
- Currently addressing or incorporating comments to the draft guidance
- Expect to publish final guidance this year

Fostering Drug Development in ME/CFS: Clinical Trial Endpoints

- PROMIS fatigue item bank
 - PROMIS Network Center of the American Institutes for Research
 - Initial package submitted
 - FDA facilitated collaboration of CDC and PROMIS investigators to further examine the CDC's ME/CFS patient cohort data
- Expecting submission this year
- Reaching out to investigator to help move this project forward

Thank you!
