Endpoints for Drug Development in Heart Failure
Friday, July 26, 2019 Great Room, White Oak Campus, FDA

Agenda

The U.S. Food & Drug Administration (FDA) is convening experts in heart failure research and clinical trials from the regulatory, academic, and drug development communities to discuss issues related to endpoints in heart failure drug development. Following a recently published draft guidance, "Treatment for Heart Failure: Endpoints for Drug Development", FDA is specifically interested in soliciting feedback regarding four high-priority topics:

1. Identify endpoints related to symptoms or physical function of clinical importance, including an approach to quantifying hospitalization;
2. Understanding when the nature, magnitude and clinical importance of an endpoint may justify deferral or omission of outcomes studies;
3. Identify the risk of mortality that should be ruled out in outcome studies and whether the acceptable upper bound should be influenced by a drug's demonstrated benefit and risk;
4. Discuss the pros and cons of capturing all-cause events vs. cause-specific events, and the need for adjudication of events.

7:45 am **Registration opens**

8:45 am **Welcome and Overview, Background (5 mins)**
Ellis Unger

8:50 am **Opening Remarks (5 mins)**
Norman Stockbridge

8:55 am **HFSA/FDA Survey Results (5 mins)**
Mona Fiuzat

9:00 am **HFSA Statement (5 mins)**
Randall Starling, HFSA President

9:05 am **<u>Part I: Endpoints related to symptoms or physical function of clinical importance in HF (75 mins)</u>**
Moderator: JoAnn Lindenfeld

QOL, Symptoms, PRO's (15 mins)
Eldrin Lewis

Functional Endpoints: 6MW, Accelerometer, New Technologies (15 mins)
Bill Abraham
Quantifying Hospitalization? (15 mins)
John Teerlink

Reactants and Panel Discussion (30 mins):

- *Panelist – Mary N. Walsh*
- *Panelist – Paul Heidenreich*
- *Panelist – Patient– Skype in Reiss Tatum*
- *Panelist – Patient – Skype in Cynthia Chauhan*
- *Panelist – Biykem Bozkurt*
- *Panelist – Ellis Unger*

10:25 am **Break (15m)**

10:45 am **<u>Part II: Mortality and Outcomes (85 mins)</u>**
Moderator: Christopher O'Connor

When would deferral or omission of outcomes studies be justified? (20 mins)
Scott Solomon

What is the risk of mortality that should be ruled out? (20 mins)
John McMurray

Reactants and Panel Discussion (45 mins):

- *Panelist - Michael Felker*
- *Panelist - Javed Butler*
- *Panelist – Lynne Stevenson*
- *Panelist – Patient – Rhonda Monroe*
- *Panelist - Norman Stockbridge*

12:05 pm **Lunch (60 mins)**

1:10 pm **<u>Part III: All-cause vs. cause-specific events, Adjudication (60 mins)</u>**
Moderator: Clyde Yancy

Should all-cause or cause-specific events be used? (15 mins)
Christopher O'Connor

Should the FDA require adjudication of cause-specific events? (15 mins)
Michael Bristow

Reactants and Panel Discussion (30 mins)

- *Panelist – JoAnn Lindenfeld*
- *Panelist – Peter Carson*
- *Panelist – John Teerlink*
- *Panelist – Robert Temple*

2:15 pm **Open Audience Feedback (1 hr. 45 mins)**
Moderators: Christopher O'Connor, JoAnn Lindenfeld, Clyde Yancy

The audience is invited to share remarks and outstanding questions regarding the day's discussion and FDA's draft guidance "Treatment of Heart Failure: Endpoints for Drug Development".

4:00 pm **Closing Remarks and Adjournment**