Chagas Disease Public Meeting on Patient-Focused Drug Development

April 28, 2015

8:00 – 9:00 am	**Registration**
9:00 – 9:05 am	**Welcome** Soujanya Giambone, MBA *Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA*
9:05 – 9:10 am	**Opening Remarks** John Farley, MD MPH *Deputy Director, Office of Antimicrobial Products (OAP), CDER, FDA*
9:10 – 9:20 am	**Overview of FDA's Patient-Focused Drug Development Initiative** Theresa Mullin, PhD *Director, OSP, CDER, FDA*
9:20 – 9:35 am	**An Overview of Chagas Disease and Available Treatment** Maria Allende, MD *Medical officer, Division of Anti-infective Products (DAIP), CDER, FDA*
9:35 – 9:40 am	**Overview of Discussion Format** Soujanya Giambone, MBA *OSP, CDER, FDA*
9:40 – 10:00 am	**Panel #1 Comments on Topic 1** Topic 1: Disease symptoms and daily impacts that matter most to patients. A panel of patients and patient advocates will provide comments to start the discussion.
10:00 – 10:30 am	**Panel Discussion on Topic 1**
10:30 – 10:40 am	**Break**
10:40 – 11:00 am	**Panel #2 Comments on Topic 2** Topic 2: Patient perspectives on current approaches to treating Chagas disease. A panel of patients and patient advocates will provide comments to start the discussion.
11:00 – 11:45 am	**Panel Discussion on Topic 2**
11:45 – 12:45 pm	**Lunch**

Session 2: Scientific Discussion

12:45– 1:05 pm **The Epidemiology and Natural History of Chagas Disease**
Caryn Bern, MD MPH
University of California, San Francisco

1:05 – 1:20 pm **Review Considerations for New Drugs in the United States**
Joe Toerner, MD MPH
CDER/FDA

1:20 – 1:50 pm **Recent, Ongoing, and Planned Clinical Trials for Chagas Disease**
Isabela Ribeiro, MD
Drugs for Neglected Diseases Initiative (DNDi), Geneva, Switzerland

1:50 – 2:30 pm **Panel Discussion**
Moderator: Sumathi Nambiar, MD MPH
CDER/FDA

Populations who could be enrolled in a clinical trial
What are the populations (e.g. stage of disease) for which a clinical trial could be feasible and acceptable?
Acceptable control groups
Are there any situations for which a placebo control would be acceptable?

2:30 – 2:45 pm **Break**

2:45 – 3:15 pm **Laboratory Monitoring Using Serology**
Louis Kirchhoff, MD, MPH
University of Iowa Carver College of Medicine, Iowa City, Iowa

Laboratory Monitoring Using PCR
Alejandro Schijman, PhD
Research Institute of Genetic Engineering and Molecular Biology, Buenos Aires, Argentina

3:15 – 4:30 pm **Panel Discussion**
Moderator: Sumathi Nambiar, MD MPH
CDER/FDA

Trial designs and trial endpoints
What are feasible and acceptable clinical trial designs?
What primary endpoint(s) would be appropriate for a clinical trial? What are the strengths and weaknesses of clinical outcome endpoints (For example, Is the clinical outcome endpoint well-defined and reliable? When should treatment benefit be assessed? How long would patients need to be followed?)
What are the strengths and weaknesses of the evidence that change in serology (sero-negative or reduction in titers), negative PCR, or other laboratory test result at a specified time point after treatment are predictive of later clinical outcome?

4:30 – 4:50 pm **Open Public Comment Session**

4:50 – 5:00 pm **Closing Remarks and Adjourn**

Discussion Questions (Morning Session):

Topic 1: Disease Symptoms and Daily Impacts That Matter Most to Patients

1) What **worries you most** about your condition?
2) Of all the symptoms that you experience because of your condition, which **1-3 symptoms** have the most significant impact on your life? (Examples may include irregular heartbeat, shortness of breath, difficulty swallowing, stomach pain or constipation)
3) 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 condition? (Examples of activities may include sleeping through the night, daily hygiene, driving, being a blood or organ donor, or for women in reproductive age concern about getting pregnant and transmitting the infection to your children, etc.)
4) How have your condition and its symptoms **changed over time**?
5) Do your symptoms come and go? If so, do you know of anything that makes your symptoms better or worse?

Topic 2: Patient Perspectives on Current Approaches to Treat Chagas Disease:

1) **What are you currently doing** to help treat your condition? (Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.)
   a) What specific symptoms do your treatments address?
   b) How has your treatment regimen changed over time, and why?
2) What are the most significant **downsides to your current treatments**, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, length of treatment, number of pills to take daily, going to the hospital for frequent check-up or treatment, restrictions on driving, potential consequences to your health and your child's health during pregnancy, etc.)
3) What specific things would you look for in an **ideal treatment** for your condition?

Docket Information

We encourage you to submit your written comments to the docket by June 29, 2015: http://www.regulations.gov/#!documentDetail;D=FDA-2014-N-1998-0001 or go to www.regulations.gov and search for: **Chagas disease patient-focused drug development.**