



Public Meeting on Non-Tuberculous Mycobacterial (NTM) Lung Infections Patient-Focused Drug Development



October 15, 2015

8:00 – 9:00 am	Registration
9:00 – 9:05 am	Welcome Soujanya Giambone, MBA <i>Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA</i>
9:05 – 9:10 am	Opening Remarks John Farley, MD MPH <i>Deputy Director, Office of Antimicrobial Products (OAP), CDER, FDA</i>
9:10 – 9:20 am	Overview of FDA’s Patient-Focused Drug Development Initiative Theresa Mullin, PhD <i>Director, OSP, CDER, FDA</i>
9:20 – 9:30 am	An Overview of NTM Infections and Available Treatment Hala Shamsuddin, MD <i>Medical officer, Division of Anti-infective Products (DAIP), CDER, FDA</i>
9:30 – 9:35 am	Overview of Discussion Format Soujanya Giambone <i>OSP, CDER, FDA</i>
9:35 – 10:05 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.
10:05 – 10:45 am	Large-Group Facilitated Discussion on Topic 1 Patients and patient representatives in the audience are invited to add to the dialogue.
10:45 – 11:00 am	Break
11:00 – 11:30 am	Panel #2 Comments on Topic 2 Topic 2: Patient perspectives on current approaches to treating NTM infections. A panel of patients and patient advocates will provide comments to start the discussion.
11:30 – 12:15 pm	Large-Group Facilitated Discussion on Topic 2 Patients and patient representatives in the audience are invited to add to the dialogue.
12:15 – 1:00 pm	Lunch

Session 2: Scientific Discussion

Current Treatment of NTM Lung Infections

1:00-1:20 pm **The Epidemiology and Natural History of NTM Lung Infections**
Kenneth Olivier, MD

1:20 – 1:40 pm **Treatment Guidelines for NTM Lung Infections**
David Griffith, MD

Designing Clinical Trials for New Drugs to Treat NTM Lung Infections

1:40 – 1:55 pm **Review Considerations for New Drugs in the United States**
Hala Shamsuddin, MD

1:55 – 2:15 pm **The Road from Patient Focused Drug Development Meetings to Clinical Trial Endpoints**
Selena Daniels, PharmD and Alexandra Quittner, Ph.D.

2:15 – 2:35 pm **Challenges in the Design of Clinical Trials for NTM Lung Infections**
Anne O'Donnell, MD

2:35 – 2:45 pm **Questions**

2:45 – 3:00 pm **Break**

3:00 – 4:30 pm **Panel Discussion**
*Moderator: Sumathi Nambiar, MD MPH
CDER/FDA*

Clinical Trial Considerations

1. *Eligible population: CF vs. non-CF, Rx naïve vs. Rx experienced, MAC vs. other NTM (especially M. abscessus)*
2. *Use of Control: active or placebo: Add-on therapy vs. new regimen, how to choose optimized background regimen if there is no correlation between the results of susceptibility testing and clinical activity*
3. *Trial endpoints*
 - a. *Microbiologic endpoint:*
 - *Effect of inhaled therapies?*
 - *Sputum Conversion and Clearance: definitions, timing and durability – how many consecutive cultures define sputum conversion to negative? Sputum clearance?*
 - *How many months after sputum clearance is a “cure” declared?*
 - *Correlation of microbiologic endpoints with clinical outcomes*
 - b. *Patient Reported Outcome endpoint: which PRO, when to assess, effect of other concomitant interventions for underlying lung disease*
 - c. *Assessments of exercise tolerance: 6MWT: what change is clinically meaningful, effect of other interventions for underlying lung disease*
 - d. *Other endpoints*
4. *Trial feasibility: frequency of visits, frequency of labs, available treatment centers, length of study, possible need for equipment (IV or inhaled therapies)*

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. 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 cough, increased sputum production, shortness of breath, difficulty breathing, chest pain)
2. 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, walking/running, exercising, etc.)
 - a. How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days? (Examples may include limitations on the ability to undertake physically strenuous activities, restrictions on the ability to travel, inability to sleep, lack of appetite, fatigue, etc.)
3. How has your condition and its symptoms changed over time?
 - a. Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?
4. What worries you most about your condition?

Topic 2: Patients' perspectives on current approaches to treating NTM lung infections

1. What are you currently doing to help treat your condition or its symptoms? (Examples may include prescription medicines, over-the-counter products, nebulizers, and other therapies including non-drug therapies)
 - a. What specific symptoms do your treatments address?
 - b. How has your treatment regimen changed over time, and why?
2. How well does your current treatment regimen treat the most significant symptoms of your disease?
 - a. How well do these treatments stop or slow the progression of your disease?
 - b. How well do these therapies improve your ability to do specific activities that are important to you in your daily life?
 - c. How well have these treatments worked for you as your condition has changed over time?
3. 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, need for multiple medications, need for injections, going to the hospital for treatment, etc.)
4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?