

Panels 1 and 2 Discussion Questions

The following questions delineate some of the gaps in our knowledge related to the design of clinical trials in NTM patients. We invite your thoughts on how to address these knowledge gaps, as well as, thoughts on ways to move forward to design sound clinical trials for NTM patients.

1. Balancing feasibility and need, what patient populations should be prioritized for study in clinical trials?
 - a. Treatment naïve vs. treatment refractory?
 - b. Patients with bronchiectatic nodular vs. fibrocavitary disease?
 - c. Patients with particular co-morbid conditions, such as, CF vs. non-CF?
 - d. Could all of these patient groups be enrolled in the same study or not?
2. What are the clinical symptoms, signs, and other measures that are important to patients and physicians that could be incorporated into outcome assessments in a clinical trial?
3. Assuming that the primary endpoint is designed to assess direct clinical benefit (how patients feel, function, survive), when should it be assessed?
 - a. On therapy vs. off therapy?
 - b. At 6 months, 12 months, 24 months after initiation of therapy?
 - c. Does the timing depend on the type of patient?
 - i. treatment naïve vs. refractory?
 - ii. bronchiectatic nodular vs. fibrocavitary disease?
 - iii. underlying co-morbid conditions (CF vs. non-CF)?
 - d. Should the assessment be based on a fixed timepoint or on a summary of the COA scores over time?
 - i. If based on a summary of COA scores, how frequently should assessments be made (e.g., daily, weekly, monthly, every 6 months, etc.)?