Regimen Y: A New Drug Regimen for Treatment of Newly Diagnosed Bronchiectatic Nodular Pulmonary MAC Disease

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Development of Antibacterial Drugs for the Treatment of Nontuberculous Mycobacterial Disease

RedHill Biopharma

FDA Public Workshop, April 8 2019

Points of Interest

- Known experience with prospective agents
  - Approved in same indication albeit different patient population
  - Clinical guidelines
- Defined patient population currently has no approved therapy even as SOC for ADD-ON therapy trials
- Orphan status with significant unmet need
- Clinical outcomes assessment as yet undefined and not validated
  - May take long time to demonstrate statistically
- Patient population needs FDA approved therapy

Path Forward

- Subpart H with accelerated approval based on sputum conversion at 6 months, safety & tolerability
- Follow up for durability of sputum conversion
  - Length of treatment for placebo
  - Possible re-randomization of responders
- Exploratory use of COA based on discussions with patient, KOL and FDA stakeholders
- Post marketing Phase 3 if necessary for full approval
  - Study design regarding clinical outcomes, length of observation based on experience of Phase 2 pivotal study