Industry Perspective

Developing Drugs for Coccidioidomycosis

FDA public workshop, 5Aug2020

mayne pharma

Experience and Outlook

Lessons Learned from Ongoing Efforts: Study Conduct

- Enrolment: Challenging to recruit patients, even with multiple sites in California/Arizona
  - Many acute infections will be first treated by primary care physicians
  - By the time patients are referred to academic hospitals, many may fail study inclusion criteria (*e.g.*, prior therapy)
  - Very low patient numbers for disseminated disease
- Duration: fungal disease requires prolonged treatment, hence long evaluation timelines

Outlook: Demonstrating adequate clinical benefit

- Significant challenges in design, execution and affordability of multiple, large-scale RCTs
- Comparison of new agents *vs.* (or in addition to) standard of care in high-risk patient populations
- Non-inferiority trial designs
- Defining clinical benefit to patients in (often) chronic disease
- Risk assessment considers:
  - Time to complete studies (impacted by number of potential patients, and seasonality of disease)
  - Strength of evidence supporting potential superiority of novel agent
  - Cumulative Probability of Success

Outlook: Rare disease presents barriers to the commercial opportunity

- While incidence of Coccidioidomycosis is increasing in the US, low numbers of patients require treatment
- Infections are often seasonal, and vary year-to-year
  - This presents risk and uncertainty for any product utilization forecast
- The total opportunity is unlikely to meet the threshold for investment for many pharmaceutical companies

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