International Harmonization of Expedited Programs: Challenges and Opportunities for Increasing Patient Access to Innovative Therapies
Duke-Margolis Center for Health Policy Conference Center • Washington, DC
June 7, 2017

8:30 a.m. Registration

9:00 a.m. Welcome and Introduction
Mark McClellan, Duke-Margolis Center for Health Policy
Greg Daniel, Duke-Margolis Center for Health Policy

9:15 a.m. Overview of existing expedited programs
Presentation: David Joy, US Food and Drug Administration

9:35 a.m. Session I: Stakeholder experience with expedited programs: process-related issues
Objective: Highlight the perspectives and experiences of key stakeholders, identify whether process oriented differences across regulatory agencies may complicate drug development or delay drug approval, and if so, discuss potential solutions for addressing these barriers. Moderated discussion will be opened to all participants in the room.

Moderator: Greg Daniel, Duke-Margolis Center for Health Policy

Panelists:
- Kay Holcombe, Biotechnology Innovation Organization
- Saima Khan, Pfizer
- Jarno Hoekman, Utrecht University
- Anne-Virginie Eggimann, bluebird bio, Inc.

Moderated Discussion

Potential Questions
- How and to what extent is a lack of harmonization across the expedited programs of FDA, EMA, and PMDA adversely affecting drug development?
- To what extent do differences in process requirements across the various regulatory agencies’ expedited programs lead to delays in approval (e.g. differences in timing of application and review timelines, eligibility criteria)?

10:45 a.m. Break
Session II: Stakeholder experience with expedited programs: scientific review and advice

Objective: The discussion of challenges and opportunities for international regulatory harmonization continues when participants highlight the perspectives and experiences with differences in evidentiary requirements across regulatory agencies that may lead to delays in drug approval. Participants will discuss potential solutions for addressing these barriers. Moderated discussion will be opened to all participants in the room.

Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Panelists:
- Jay Siegel, Johnson & Johnson
- Emil Kakkis, Ultragenyx
- Jennifer Dudinak, GlaxoSmithKline
- Mark Stewart, Friends of Cancer Research

Moderated Discussion

Potential Questions
- To what extent is lack of alignment across the agencies’ scientific advice leading to delays in approval? (e.g., differences in acceptable endpoints, trial design, number of trials required, safety population) Are there particular contexts in which better alignment is important?
- Are there additional issues related to the design or implementation of expedited pathways that may contribute to delays in patient access? (e.g., issues related to lack of harmonization or alignment between regulator and payer evidentiary needs, delays in emerging market regulatory approval)
- What are some possible solutions for addressing stakeholder concerns with these issues?

Closing Remarks

Mark McClellan, Duke-Margolis Center for Health Policy

Adjournment