Orphan Drug and Rare Disease Developments

Kendra Martello, Executive Director, Public Policy and Corporate Social Responsibility, Mallinckrodt Pharmaceuticals

Adora Ndu, Executive Director, Global Regulatory Policy, Research & Engagement, BioMarin Pharmaceutical Inc.

Julia (Julie) Tierney, Senior Policy Advisor for Strategic Planning and Legislation, CBER, FDA

Moderated by **Brian J. Malkin**, Counsel, Arent Fox LLP

FDLI Annual Conference: Orphan Drug and Rare Disease Developments

Julia C. Tierney, JD
Senior Policy Advisor for Strategic Planning & Legislation
Center for Biologics Evaluation & Research
U.S. Food and Drug Administration

May 3, 2018

Orphan Drug and Rare Disease Developments

- Orphan Drug Act and Products for Rare Diseases
- Attention on Products for Rare Diseases
- Clinical Superiority
- Cell & Gene Therapies
- Guidance Development
- Opportunities for International Cooperation

Orphan Drug Act and Products for Rare Diseases

- Enacted in 1983 to facilitate the development of drugs and biologics for rare diseases through series of incentives, including exclusivity and tax credits

- In 2018, the majority of drugs and biologics approved by FDA in 2018 were for rare diseases

- There are still many patients with rare diseases with no available therapy…

Recent Developments: Attention on Products for Rare Diseases

- GAO Report on Orphan Drug Designation Process (November 2018)
- Citizen Petition regarding cost recovery criteria for orphan designation
- GAO Study of Rare Pediatric Disease and other Priority Review Programs

Recent Developments: Clinical Superiority

- FDARA codified FDA's existing regulatory framework for clinical superiority into the statute
  - Clinical superiority framework had been challenged in lawsuits like *Depomed* and *Eagle*
- Designation based on plausible hypothesis of clinical superiority: Will notify sponsor the basis of designation
- Exclusivity based on demonstration of clinical superiority: Will publish summary of clinical superiority findings on FDA website

Recent Developments: Orphan Exclusivity for Cell & Gene Therapies

- Exponential growth of interest in cellular and gene therapies
- Decision regarding scope of orphan exclusivity for CAR-T products
- Orphan exclusivity considerations for collaborative development model for regenerative medicine therapies

Guidance Development

Recent Guidances

- Human Gene Therapy for Rare Diseases (Draft, July 2018)
- Rare Diseases: Early Drug Development and the Role of Pre-IND Meetings (Draft, Oct. 2018)
- Rare Diseases: Common Issues in Drug Development (Draft, Feb. 2019)
- Rare Diseases: Natural History Studies for Drug Development (Draft, Mar. 2019)
- Patient-Focused Drug Development Guidances

Additional Guidances Under Development:

- Additional Gene Therapy Guidances, including clinical guidances
- Bulleted Indication-Specific Guidances
- Tissue agnostic orphan designation in oncology
- Orphan drug designation considerations
- Rare pediatric disease priority review vouchers

Opportunities for International Cooperation

- Global development programs are increasingly common, particularly in rare diseases with limited patient populations

- Regular Cluster (Rare Disease, Pediatric, and Disease-specific) meetings between FDA, EMA, and other regulatory authorities provide a forum for discussion of:
  - General regulatory issues
  - Policy development
  - Product-specific issues

- International Council on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH)

- FDA acceptance of foreign clinical data (21 CFR 312.120)


FDA
U.S. FOOD & DRUG
ADMINISTRATION

Orphan Drug and Rare Disease Developments

Kendra Martello, Executive Director, Public Policy and Corporate Social Responsibility, Mallinckrodt Pharmaceuticals

Adora Ndu, Executive Director, Global Regulatory Policy, Research & Engagement, BioMarin Pharmaceutical Inc.

Julia (Julie) Tierney, Senior Policy Advisor for Strategic Planning and Legislation, CBER, FDA

Moderated by **Brian J. Malkin**, Counsel, Arent Fox LLP