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)
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