Tumor Panel I:
Rare and Pediatric Cancers

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Tumor Panel I: Rare and Pediatric Cancers

Moderator:

Mike Rice, MS, MBA, Senior Consultant, Defined Health

Panelists:

- *Peter C. Adamson, MD*, Chair, Children's Oncology Group, Alan R. Cohen Endowed Chair in Pediatrics, The Children's Hospital of Philadelphia
- *Meredith K. Chuk, MD*, Scientific Liaison for Sarcoma, Medical Officer, FDA
- *Carlos Rodriguez-Galindo, MD*, Associate Professor, Department of Pediatrics, Harvard Medical School; Medical Director, Pediatric Oncology Clinical Trials, Pediatric Oncology, Dana-Farber Cancer Institute; Director, Solid Tumor Program, Pediatric Oncology, Dana-Farber Cancer Institute
- *Peter Sandor, MD, MBA*, Vice President, Global Marketing Oncology, Amgen Inc.
- *Maoxia Zheng, PhD*, Global Development Team Leader, Pediatric Oncology, Genentech

Regulatory Overview: Pediatrics and Rare Diseases

Meredith K. Chuk, M.D.
Office of Hematology and Oncology Products, FDA

Pediatric Research Equity Act (PREA)

- Mandatory pediatric assessment for any change in:
  - indication, dosage form, active ingredient, regimen, or route of administration
- Indication specific
- Does not apply to drugs with indications with orphan designation
- Submission of Pediatric Study Plan (PSP)

Best Pharmaceuticals for Children Act (BPCA)

- Voluntary
- Not indication specific
- Extra 6 months marketing exclusivity for entire moiety
- Trials completed under Written Request from FDA

Orphan Drug Act

- Drugs for an indication which:
  - Affects less than 200,000 persons in the US OR
  - Affects more than 200,000 persons but there is no reasonable expectation that the cost of development will be recovered from US sales
- Benefits:
  - Potential for 7 year marketing exclusivity
  - Tax credits for qualified clinical trials
  - Grants
  - Waiver of user fee for application
  - Drug development assistance

FDA Guidances and Additional Information

- PSP Guidance
  http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM360507.pdf

- PREA Guidance
  http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DevelopmentResources/UCM077855.pdf

- Qualifying for Pediatric Exclusivity Guidance
  http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DevelopmentResources/UCM049924.pdf

- Rare Pediatric Disease Priority Review Vouchers Guidance
  http://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM423325.pdf

- Pediatric product development FDA website
  http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DevelopmentResources/ucm049867.htm

- Rare disease drug development FDA website
  http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/ucm2005525.htm