

FDA-ASCO: Geriatric Oncology Workshop

Pediatric Regulatory Landscape

Monday, November 6, 2017

Peter C. Adamson, M.D.
The Children's Oncology Group
Children's Hospital of Philadelphia

**CHILDREN'S
ONCOLOGY
GROUP**

Regulations & Incentives

- FDA
 - Best Pharmaceuticals for Children Act
 - Pediatric Research Equity Act
 - Creating Hope Act
- EMA
 - Paediatric Investigation Plan (PIP)

Regulations & Incentives

- **FDA**

- Best Pharmaceuticals for Children Act
- Pediatric Research Equity Act
- Creating Hope Act

- EMA

- Paediatric Investigation Plan (PIP)

Best Pharmaceuticals for Children Act (BPCA)

- Provides a financial **incentive** (additional 6-month exclusivity) to pharmaceutical companies to test drugs in pediatric patients

Pediatric Research Equity Act (PREA)

- Authorizes FDA to **require** a pediatric assessment of certain approved drug indications

Creating Hope Act

- Issues *Priority Review Vouchers* for first in children drug development in pediatric life-threatening, rare diseases
- Unlimited transferability of vouchers

Regulations & Incentives

- FDA
 - Best Pharmaceuticals for Children Act
 - Pediatric Research Equity Act
 - Creating Hope Act
- **EMA**
 - Paediatric Investigation Plan (PIP)

Paediatric Investigation Plan (PIP)

- Paediatric development is **mandatory** in the EU for new medicines

Paediatric Investigation Plan (PIP)

- A company's Paediatric Investigation Plan is discussed, modified, and agreed or refused by Paediatric Committee
- Followed by EMA Decision
- Binding on company

	US BPCA	US PREA	EU
Development	Optional	Mandatory if likely to be largely used and/or meaningful benefit	Mandatory Optional for off-patent
Instrument	Written Request	-	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 2	End of phase 1
Reward	6 months exclusivity	-	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes	Yes
Biologicals (most)	No	All	All
Orphan	Excluded	Excluded	Included
Scope	Not limited to adult indication	Limited to adult indication	Derived from adult indication
Decision	FDA	FDA	EMA Paediatric Committee

	US BPCA	US PREA	EU
Development	Optional	Mandatory if likely to be largely used and/or meaningful benefit	Mandatory Optional for off-patent
Instrument	Written Request	-	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 2	End of phase 1
Reward	6 months exclusivity	-	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes	Yes
Biologicals (most)	No	All	All
Orphan	Excluded	Excluded	Included
Scope	Not limited to adult indication	Limited to adult indication	Derived from adult indication
Decision	FDA	FDA	EMA Paediatric Committee

	US BPCA	EU
Development	Optional	Mandatory Optional for off-patent
Instrument	Written Request	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 1
Reward	6 months exclusivity	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes
Biologicals (most)	No	All
Orphan	Excluded	Included
Scope	Not limited to adult indication	Derived from adult indication
Decision	FDA	EMA Paediatric Committee

	US BPCA	EU
Development	Optional	Mandatory Optional for off-patent
Instrument	Written Request	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 1
Reward	6 months exclusivity	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes
Biologicals (most)	No	All
Orphan	Excluded	Included
Scope	Not limited to adult indication	Derived from adult indication
Decision	FDA	EMA Paediatric Committee

	US BPCA	EU
Development	Optional	Mandatory Optional for off-patent
Instrument	Written Request	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 1
Reward	6 months exclusivity	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes
Biologicals (most)	No	All
Orphan	Excluded	Included
Scope	Not limited to adult indication	Derived from adult indication
Decision	FDA	EMA Paediatric Committee

	US BPCA	EU
Development	Optional	Mandatory Optional for off-patent
Instrument	Written Request	Paediatric Investigation Plan
Timing	End of phase 2	End of phase 1
Reward	6 months exclusivity	6 months patent
Drugs (section 505)	Yes With exclusivity	Yes
Biologicals (most)	No	All
Orphan	Excluded	Included
Scope	Not limited to adult indication	Derived from adult indication
Decision	FDA	EMA Paediatric Committee

Regulations & Incentives

- FDA
 - Best Pharmaceuticals for Children Act
 - Pediatric Research Equity Act
 - Creating Hope Act
 - **Research to Accelerate Cures and Equity for Children (RACE) Act**

