Overview of PIPs reviewed and paediatric trials proposed per therapeutic area

First meeting of the European Paediatric Network

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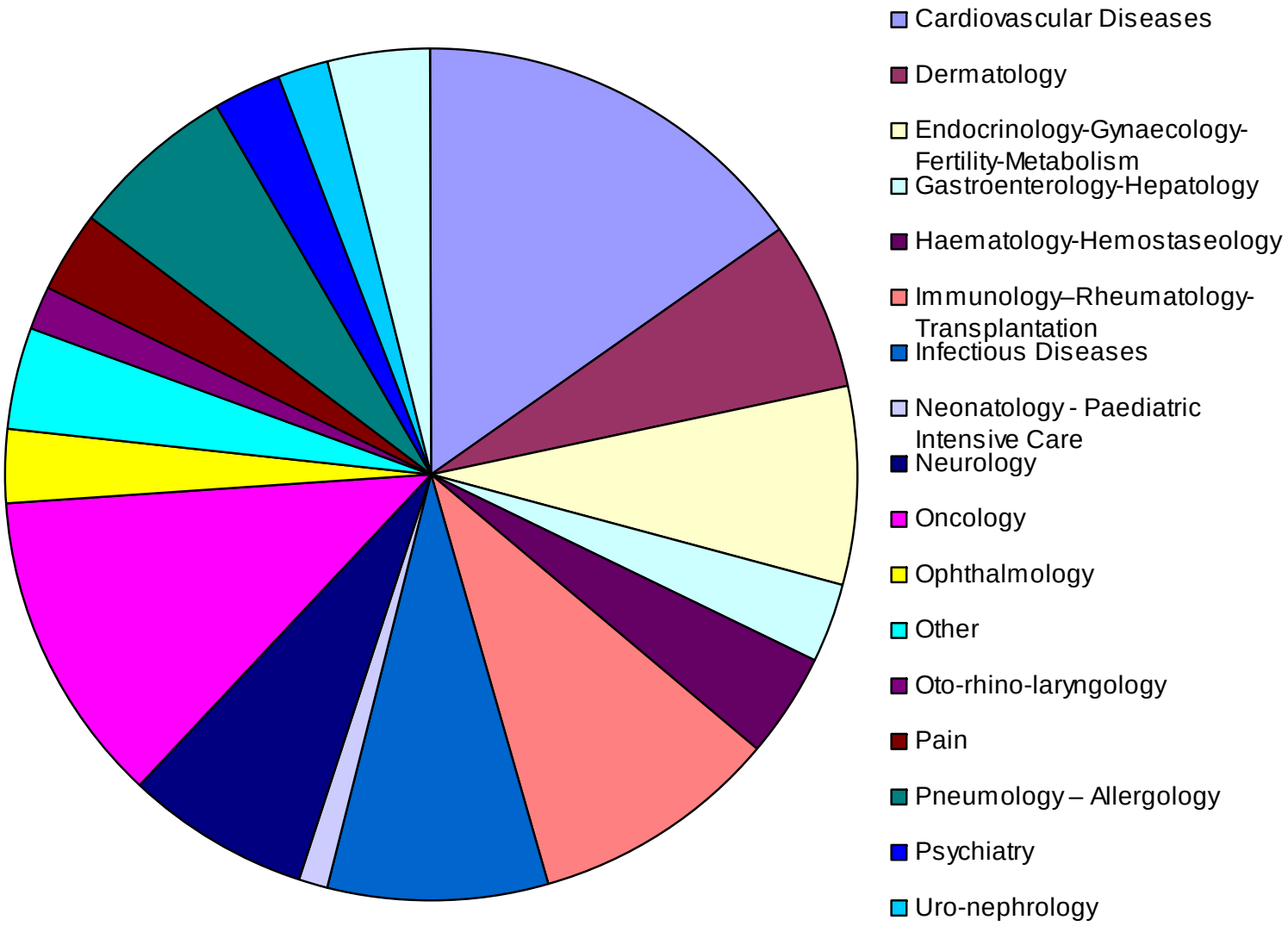
Ralf Herold and Paediatric Team

Overview of PIPs by therapeutic areas

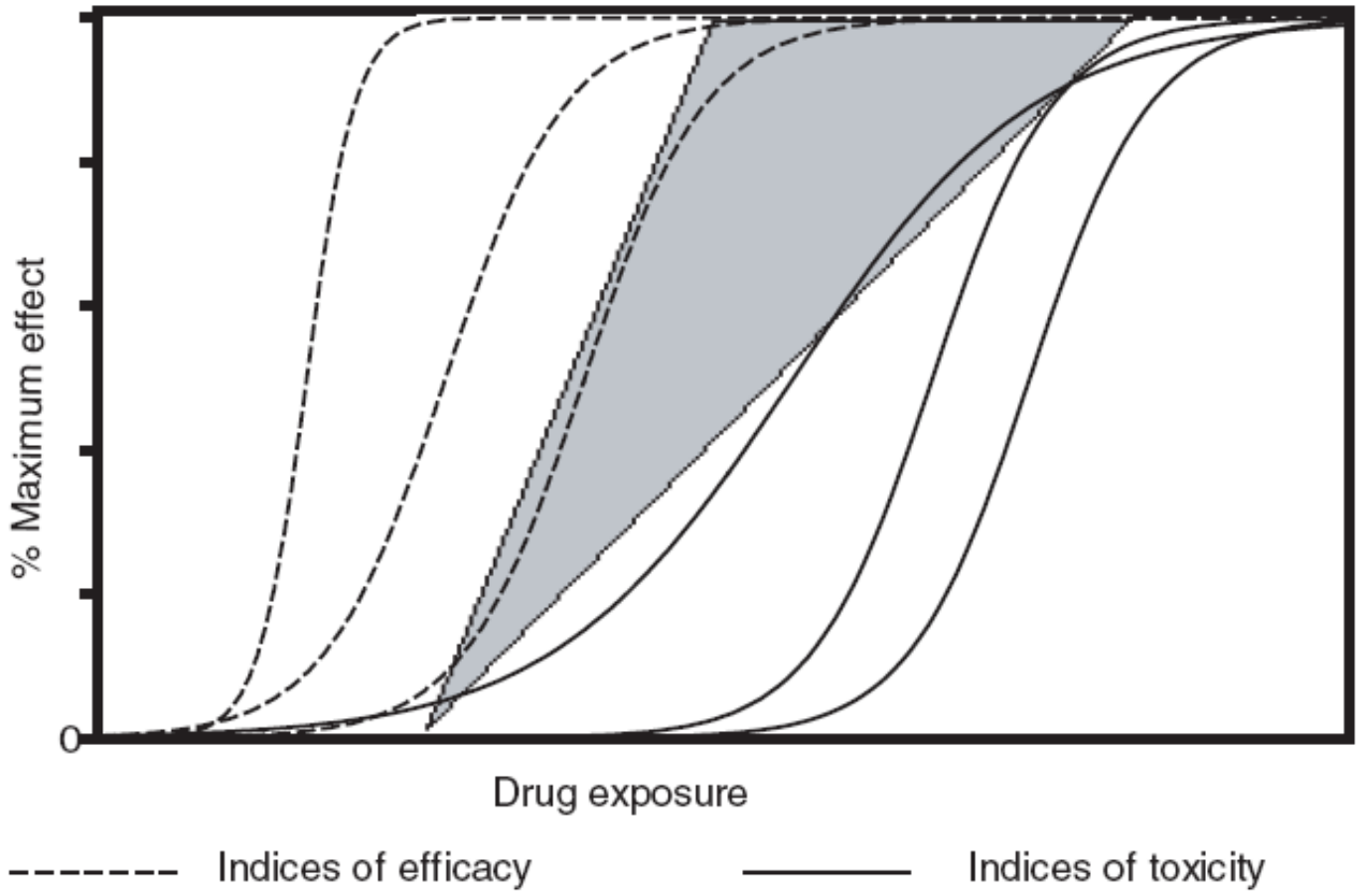
Areas covered by PIPs/waiver applications:	2007 (%)	2008 (%)	2009 (%)
Neurology	12	6	3
Uro-nephrology	-	3	3
Gastroenterology-hepatology	9	3	3
Pneumology-allergology	8	6	3
Infectious diseases	12	8	17
Cardiovascular diseases	12	14	3
Diagnostics	-	1	3
Endocrinology-gynaecology-fertility-metabolism	19	15	36
Neonatology-paediatric intensive care	-	1	0
Immunology-rheumatology-transplantation	5	6	3
Psychiatry	5	3	3
Pain	1	3	7
Haematology-haemostaseology	1	5	3
Otorhinolaryngology	-	1	0
Oncology	11	12	7
Dermatology	1	3	3
Vaccines	2	6	3
Ophthalmology	1	2	0
Anaesthesiology	-	1	0
Nutrition	1	1	0

PDCO press releases, http://www.emea.europa.eu/

PIPs cover all therapeutic areas



Inform(ed) paediatric trials

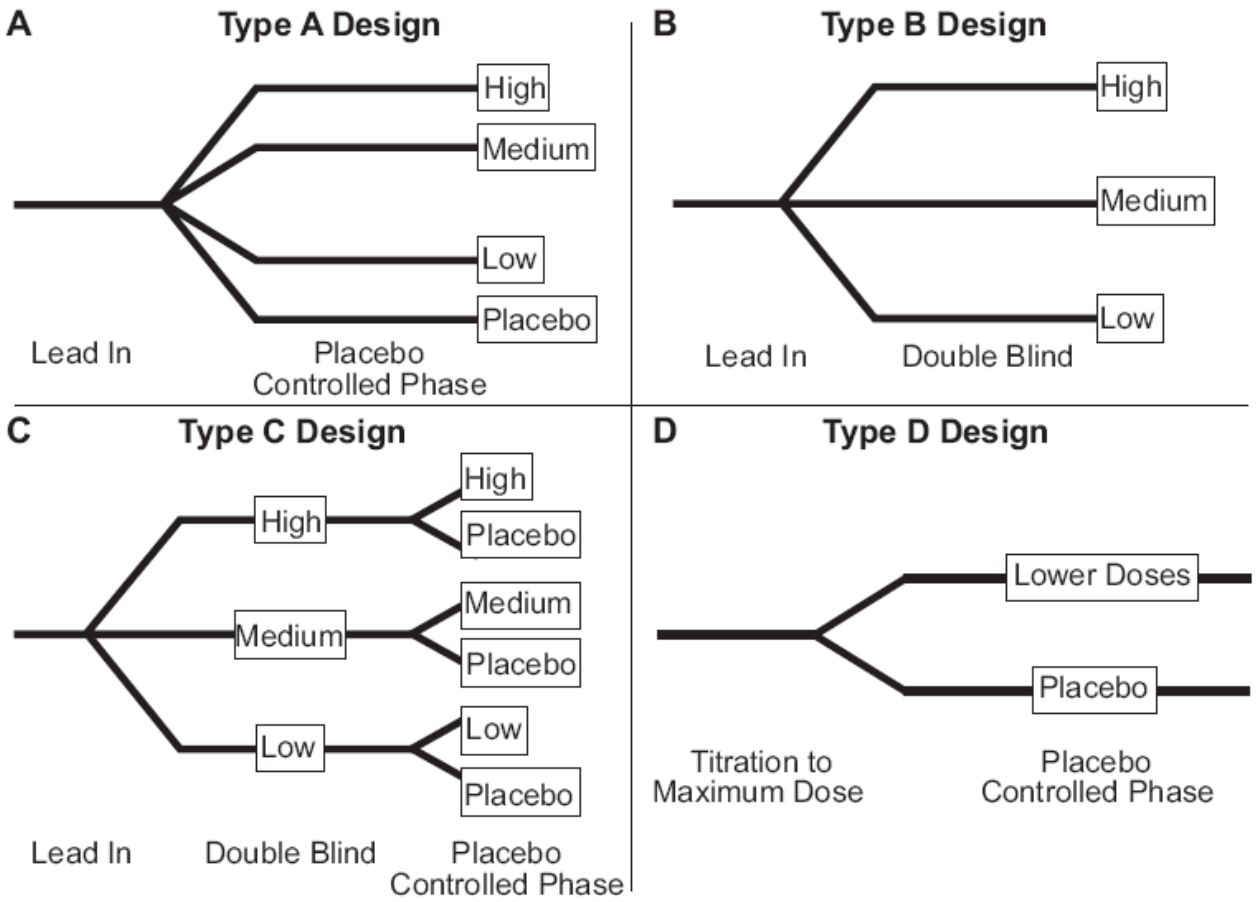


Barret 2007 Exp Op Drug Disc

Elements necessary in a PIP

- General paediatric development strategy
  - preclinical work → proof of concept / efficacy in adults → first in children → PK / PD, dose-finding → efficacy extrapolation or confirmation → long-term follow-up of safety and efficacy
- Age-appropriate formulation(s)
- Feasibility
- Clinical trial methodology robustness
- Ethical considerations

Some experience with some designs ...

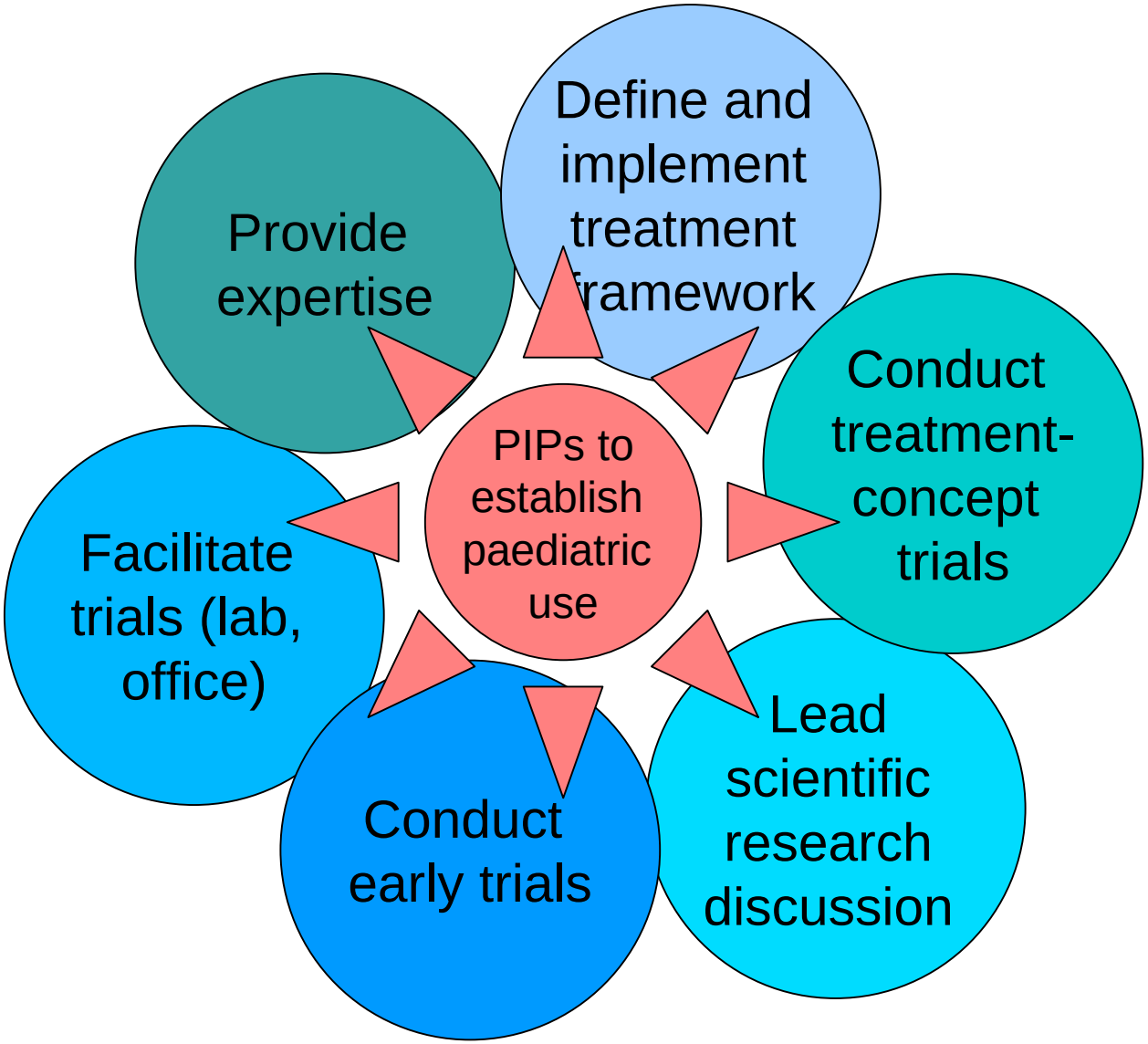


Safety: Smith 2008 Hypertension;
Trial failures: Benjamin 2008 Hypertension

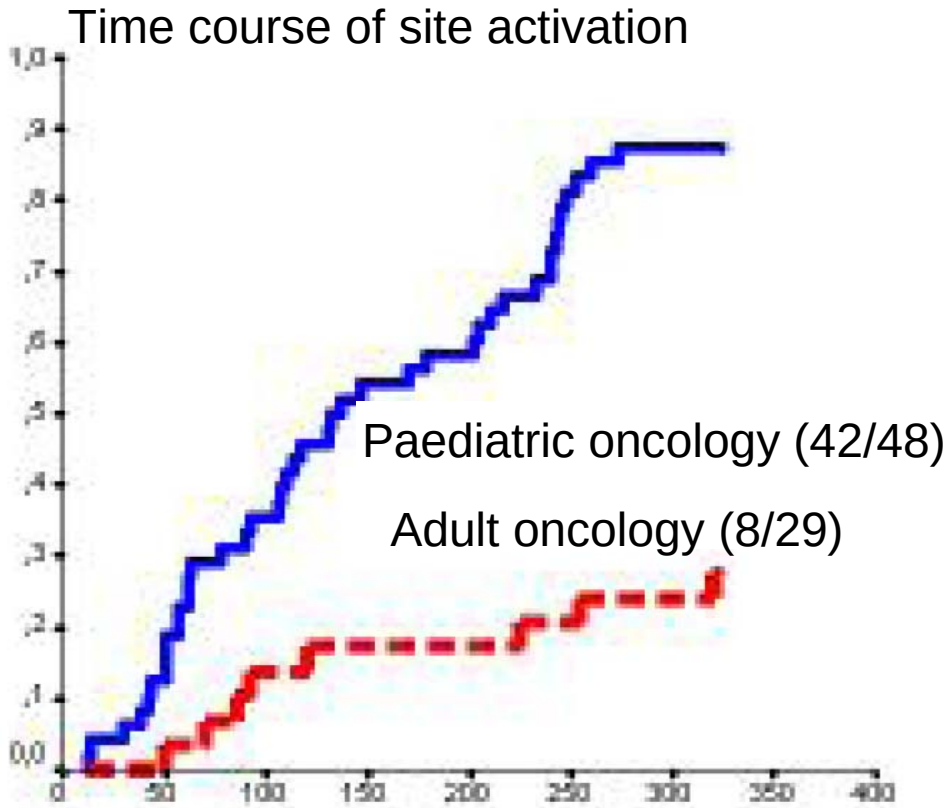
... many scientific questions to improve

- Trial design
  - Validated paediatric endpoints, markers for validation?
  - Ethical considerations: duration of placebo use; active comparator use?
  - Are special designs beyond small population guideline useful?
  - How address the need for dose-finding, effect estimation, model confirmation?
- Development design
  - One trial is almost never sufficient
  - Which trial types and strategy to cover all subsets with a need?
  - Trials may be part of study frameworks
  - Specific trials may interrelate with studies capturing safety
- Feasibility and acceptability
  - E.g., interaction with patients and parents
- Experts from the scientific community
  - Intensive involvement by the PDCO
  - Definition of the state-of-the-art medicine and research, managing biological diversity
  - Discussions of methodology and paediatric needs
- General guidance documents to be further developed

Diversity of roles of individual paediatric networks ...



... can contribute to high quality ethical and feasible research on medicinal products in children



Bielack 2006 (EURAMOS1)

Thank you -

- Paediatric Medicines website on http://www.emea.europa.eu/

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