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infant
child & youth
research network

European network for pediatric research of the European Medicines Agency (Enpr-EMA)

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Outline

- The EU situation: Enpr-EMA
- Trials in Canada
- MICYRN

Pediatric Regulation as an instrument for European Pediatric research

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Legislated January 2007

Improve the health of children:

- increase high quality ethical research into medicines for children
- increase availability of authorized medicines for children
- increase information on medicines

Achieve the above:

- without unnecessary studies in children
- without delaying authorization for adults

Requirement to:

- develop a European network to coordinate studies

Enpr-EMA:
Key Operational Goals

- To link together 64 existing networks
- To liaise with Industry and other stakeholders
- To define consistent and transparent quality standards
- To harmonize clinical trial procedures
- To define strategies for resolving major challenges
- To facilitate recruitment because this is a problem
- To strengthen research in Europe on European children

Enpr-EMA:
Coordinating Group

- To be as diverse as possible (therapeutic areas; ages; activities; trials/network organizations)
- Self-assessment process to qualify (# trials; ethics; GCP...)
- Wanted to include a total of 20 networks; only 16/32 met minimum criteria
- National networks: UK, Scottish, Finnish, Netherlands, Itl, **MICYRN**
- Specialty networks: CA-BMBT (4), PRINTO, ECFS, GNN, UKPVG, PENTA, but no Cardio, Critical Care/Pain,

Secretariat of Enpr-EMA – provided by EMA

- To provide secretarial support
- To organize and host meetings of the network
- To coordinate exchange of information between network partners
- To coordinate exchange of and provide information to external partners and stakeholders

Enpr-EMA Coordinating Group: First Steps/next year

- Launch meeting held March, 2011 (London, UK)
- Nomination of Chair of CG: Peter Helms (Scottish National Network)
- Main Goal established: Communication between Industry, Regulators, Patient Organizations
- 1st Action point: webpage, resource database
- Agreement to determine 3 main topics to develop model Paediatric Investigation Plans (PIPs)

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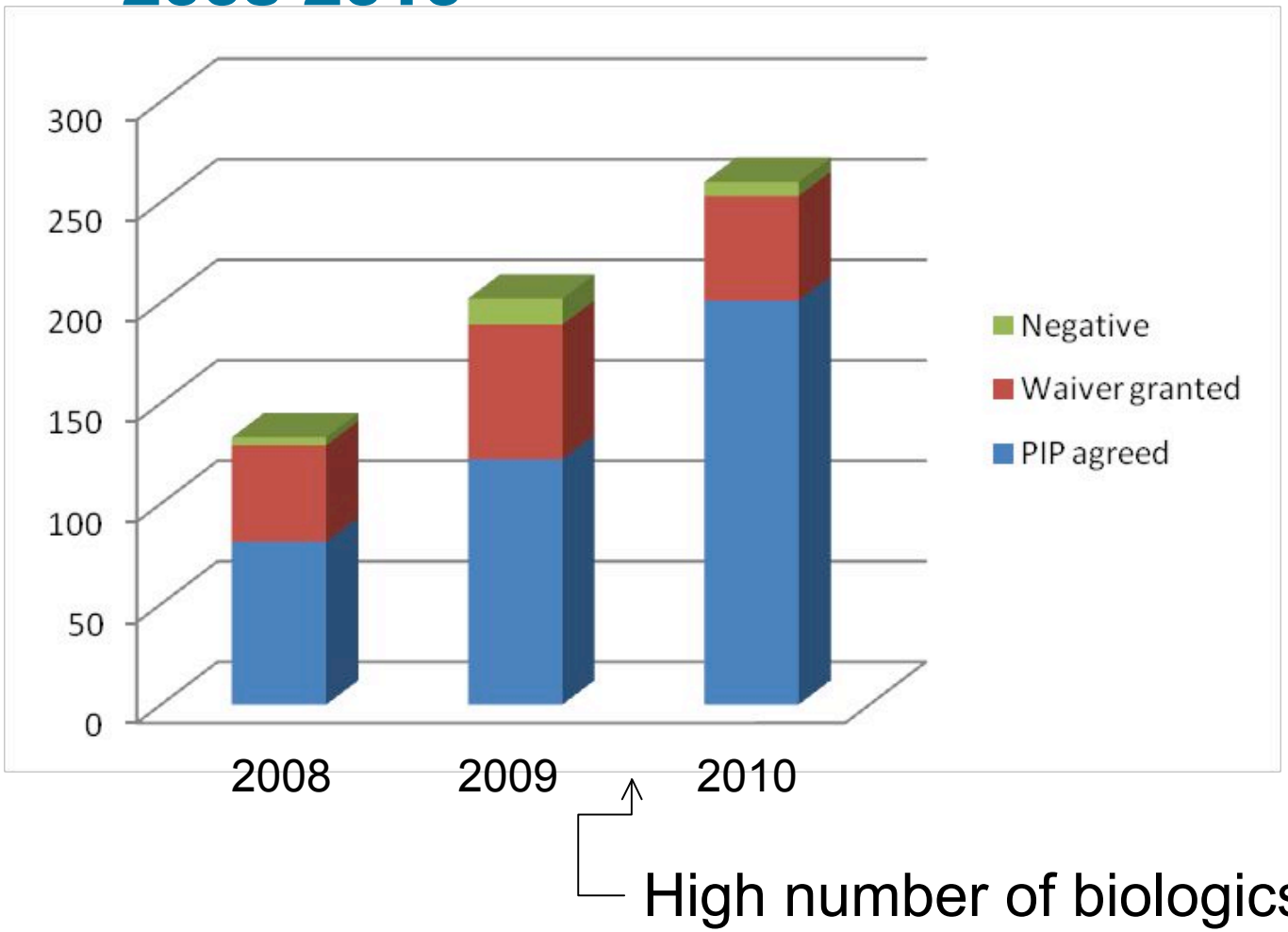
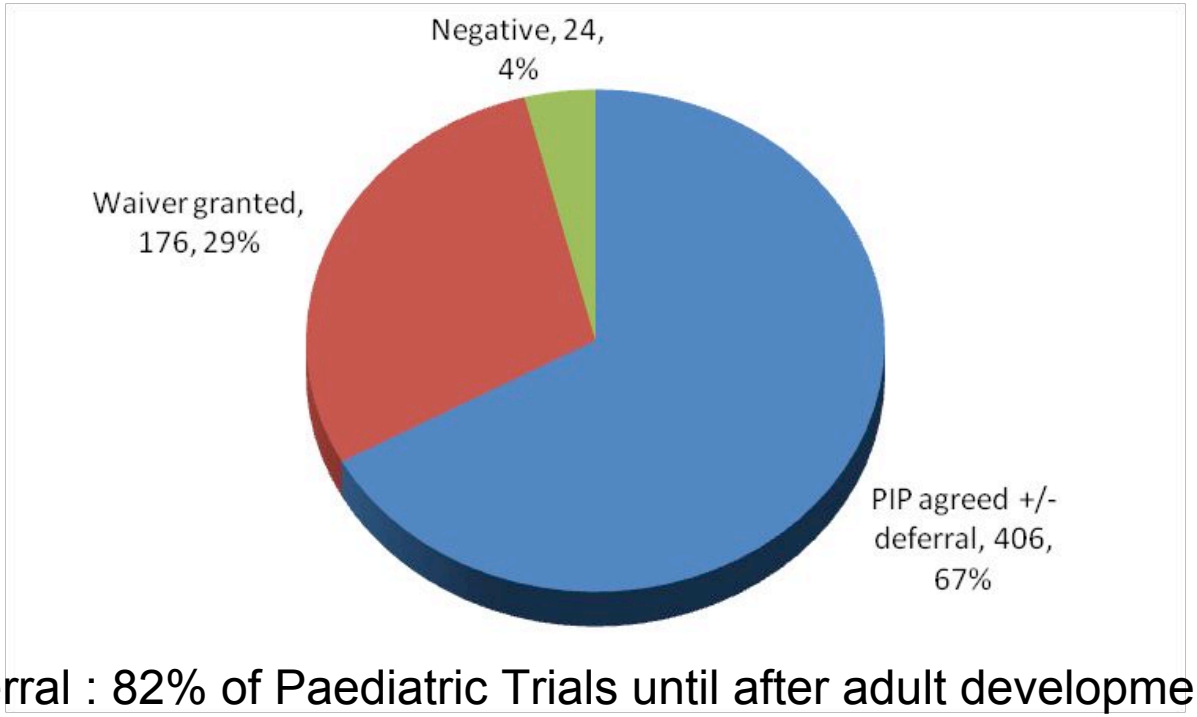
Paediatric Committee (PDCO) Opinions on PIP Applications 2008-2010



Outcome after PDCO Review: 82% Deferrals *to avoid delay in marketing authorization for adults*



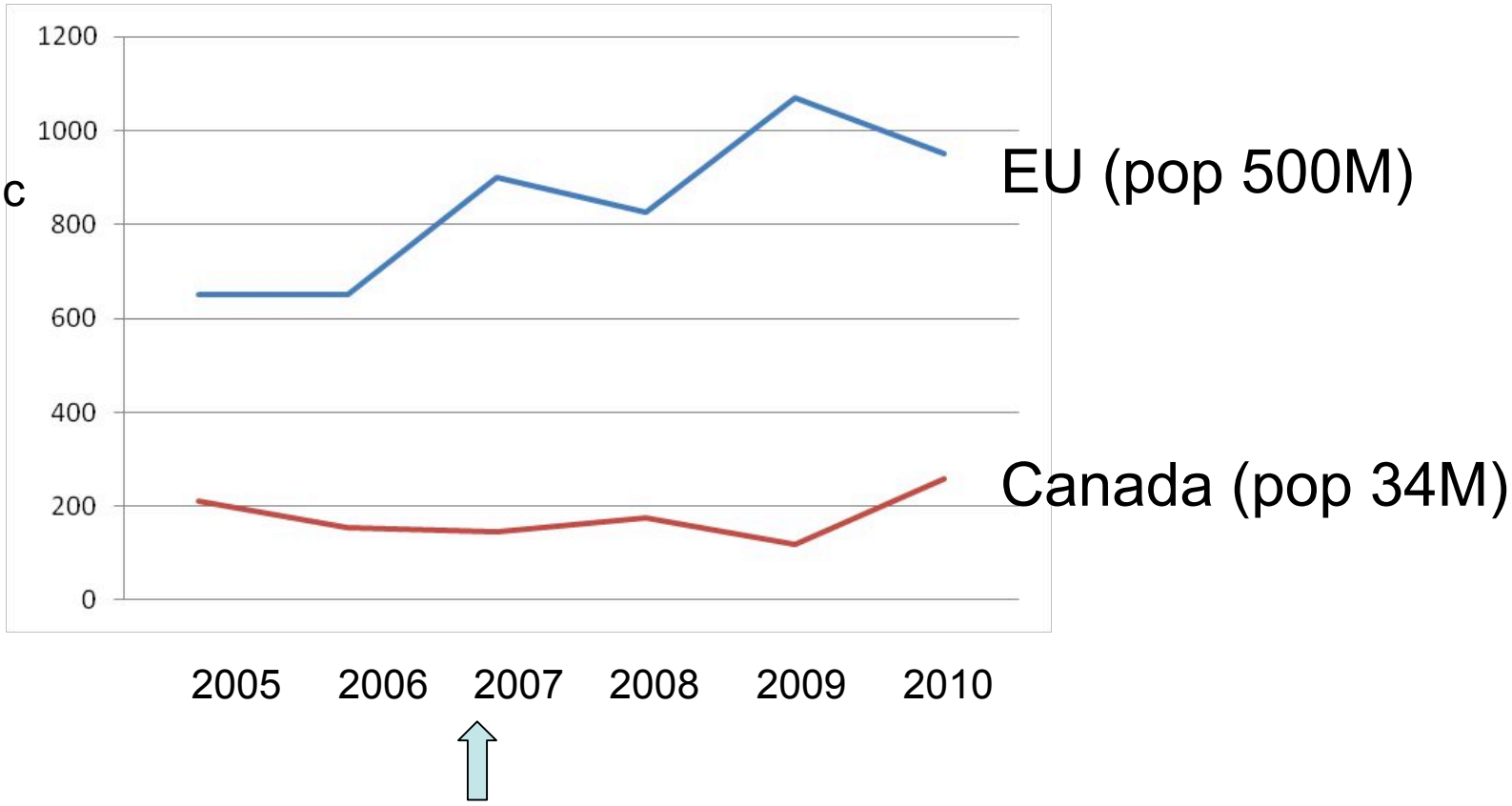
Deferral : 82% of Paediatric Trials until after adult development

- 91% for new products
- 64% for existing products
- deferral of 4-5 years, up to 8y from MAA in adults

Olski TM et al Three years of the Pediatric Regulation in Europe Eur J Clin Pharmacol 2011 (in press)

Effectiveness of the Regulation: Trials in Children?

Remains 9-10% relative to adult trials



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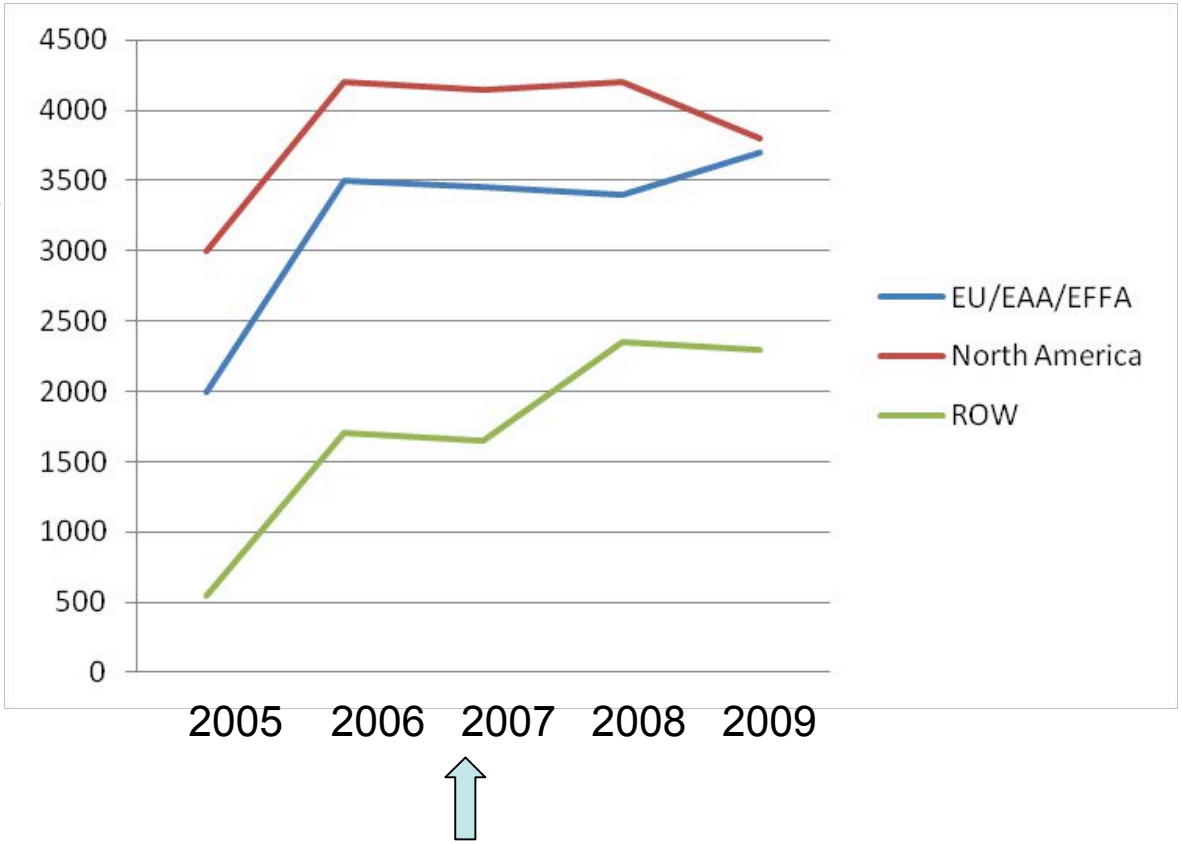
Effectiveness of the Regulation: Are Trials Moving?

Investigator Sites*



* sites involved in pivotal clinical trials submitted for marketing authorization approval to the EMA per region per year

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Canada

- World leader in pediatric pharmacology and development of trials methodology with contribution to international regulatory framework
- Biotech industry #2 in world (14.7B) and 80% of R&D directed to health products
- a Canadian child receives 4 prescriptions a year from a range of more than 1200 different drugs
- 75% of these drugs do not have dosing or safety data for use in children (90% for prematures)
- No legislation or incentives for Pediatric trials

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Canada:EU
Population comparisons

Population 0-14 years: 16%

Pediatric Trials in Canada 2005-2009

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ICTRP International Clinical Trials Registry Platform

114,495 registered clinical trials (August 16th, 2010)

↓

18,511 (16 %) trials in children

↓

13,420 pediatric trials registered 01/01/2005 – 31/12/2009

↓

1,036 listed Canada as a country of recruitment

→ Excluded Observational studies (n=85):
- establishing biobank or registry
- chart, outcome review
- survey of patient experience

→ Excluded Interventional studies (n=86) with:
- no projected subject number listed
- no lower age limit given and "adult" disease like Parkinson's or radical prostatectomy
- recruitment outside Canada
- not yet recruiting
- expanded drug access program
- study on medical student education

↓

865 Interventional clinical trials meeting criteria

http://www.who.int/ictrp/en/

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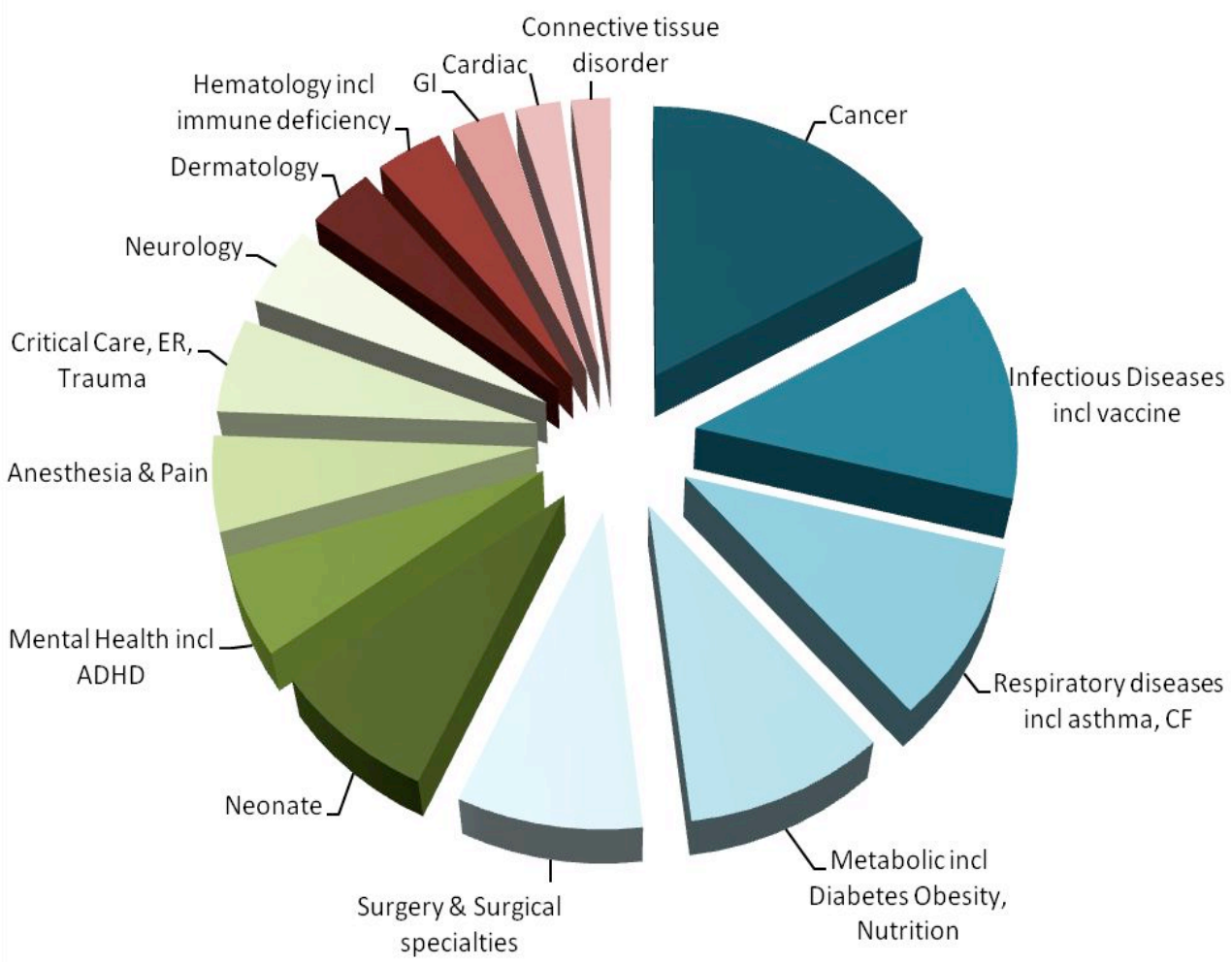
Health Conditions studied by more than 15 Pediatric Trials



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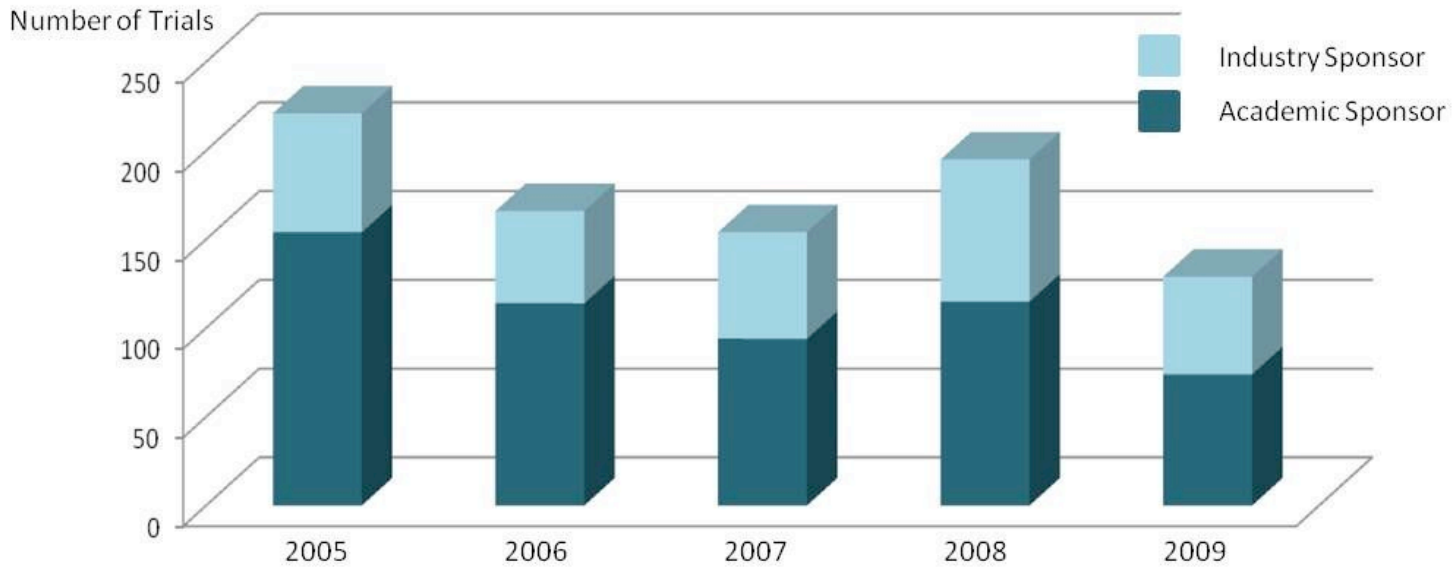
Non-Industry organizations are Primary Sponsor for 64% of Registered Pediatric Trials



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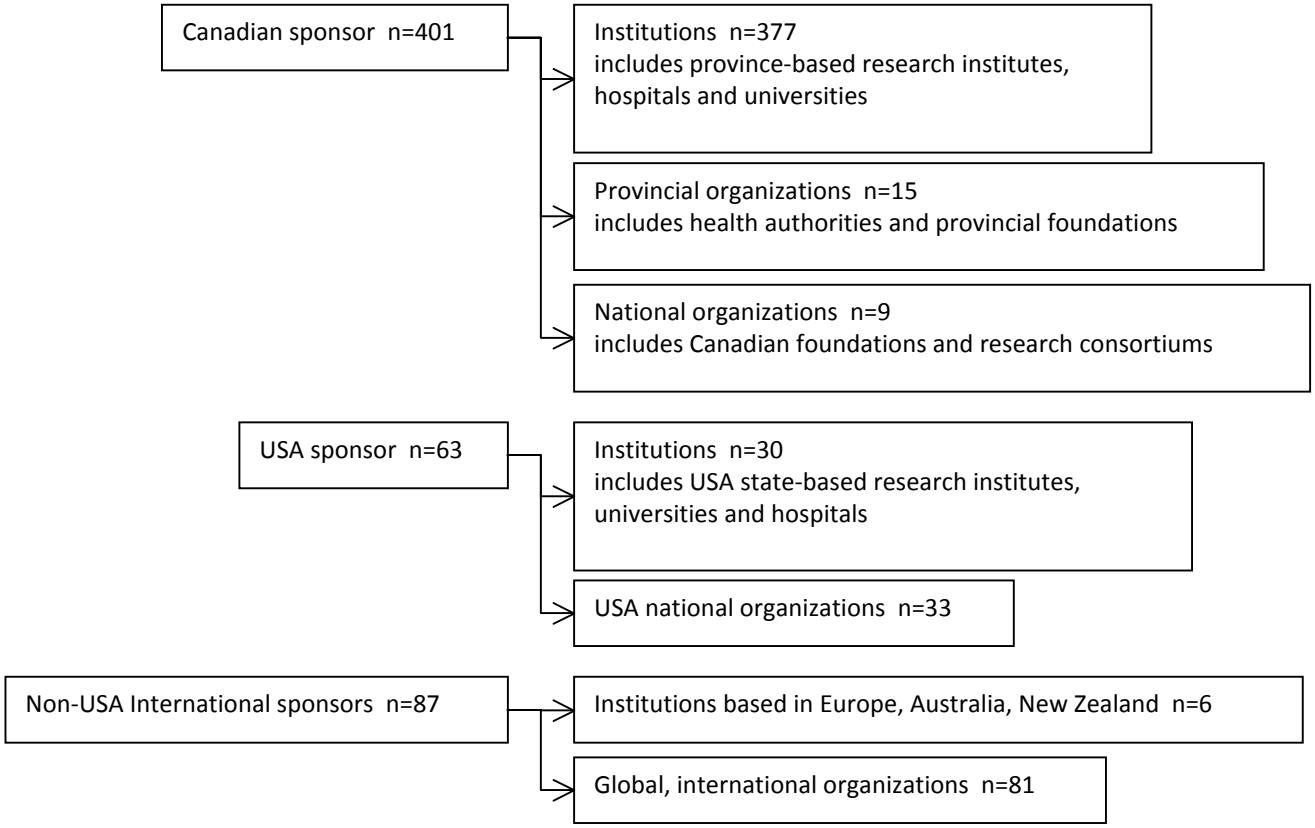
Academic Institutions are sponsors for 94% of non-Industry Registered Pediatric Trials



Research Activity in Canada: Registered Paediatric Trials

- Only 12% of non-industry trials included sites outside Canada
- Only 14% of industry trials limited to Canada
- 96 different companies conducted 314 trials (2005-2009)
- 54% of trials by 12 companies
- 51 companies did only 1 study

Multi-national nature of Pediatric Clinical Trials

Disorder	Company	Enrollment Target	Number of Countries listed for recruitment
Hemophilia	Bayer	139	25
Arthritis	Novartis	122	21
Influenza treatment	Hoffman LaRoche	250	19
Kidney disease	Sanofi Aventis	25	11
Asthma	Glaxo Smith Kline	300	11
Epilepsy	UCB Inc	357	11

- 25% of Industry trials listed an average of 12 countries to enroll ≤500 study subjects.

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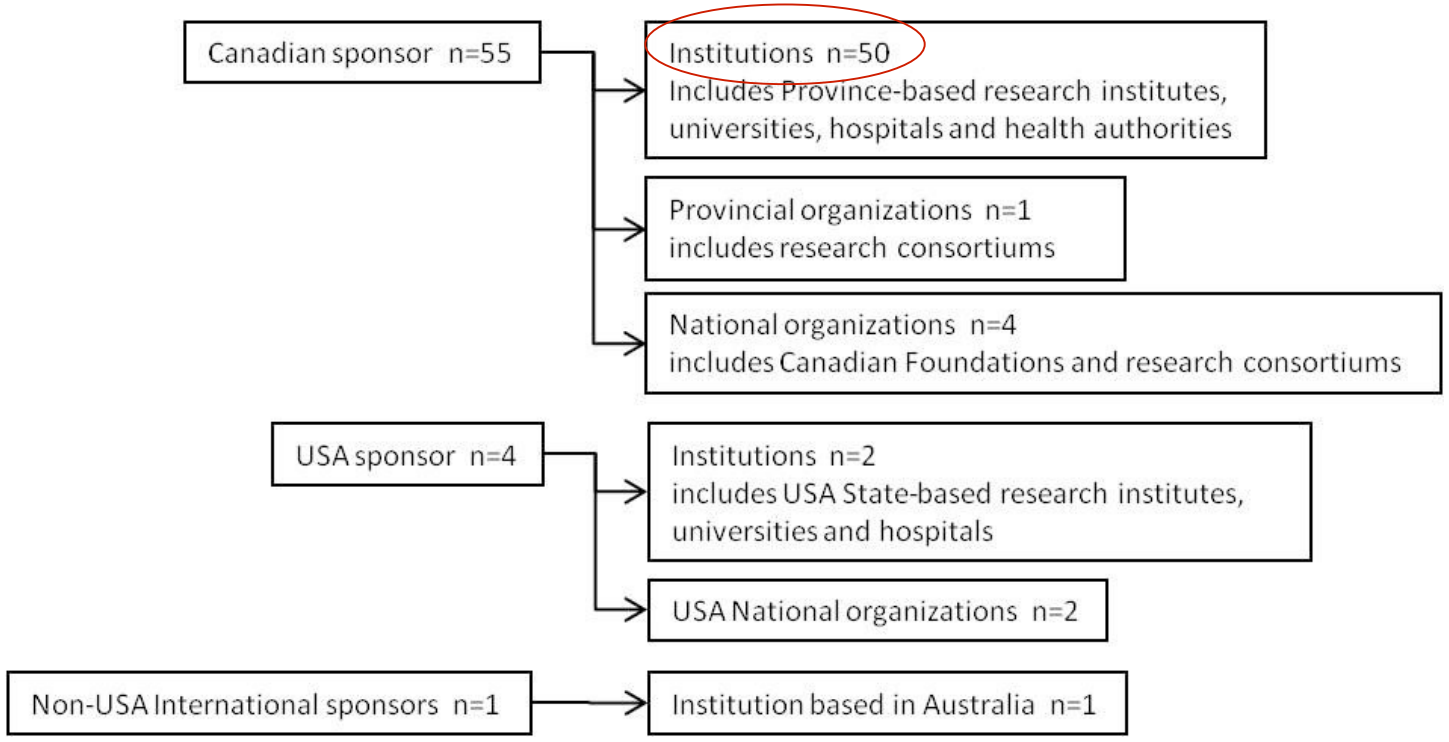
Maternal Trials in Canada 2005-2009

ICTRP International Clinical Trials Registry Platform

60 studies: 77% interventional, 23% observational



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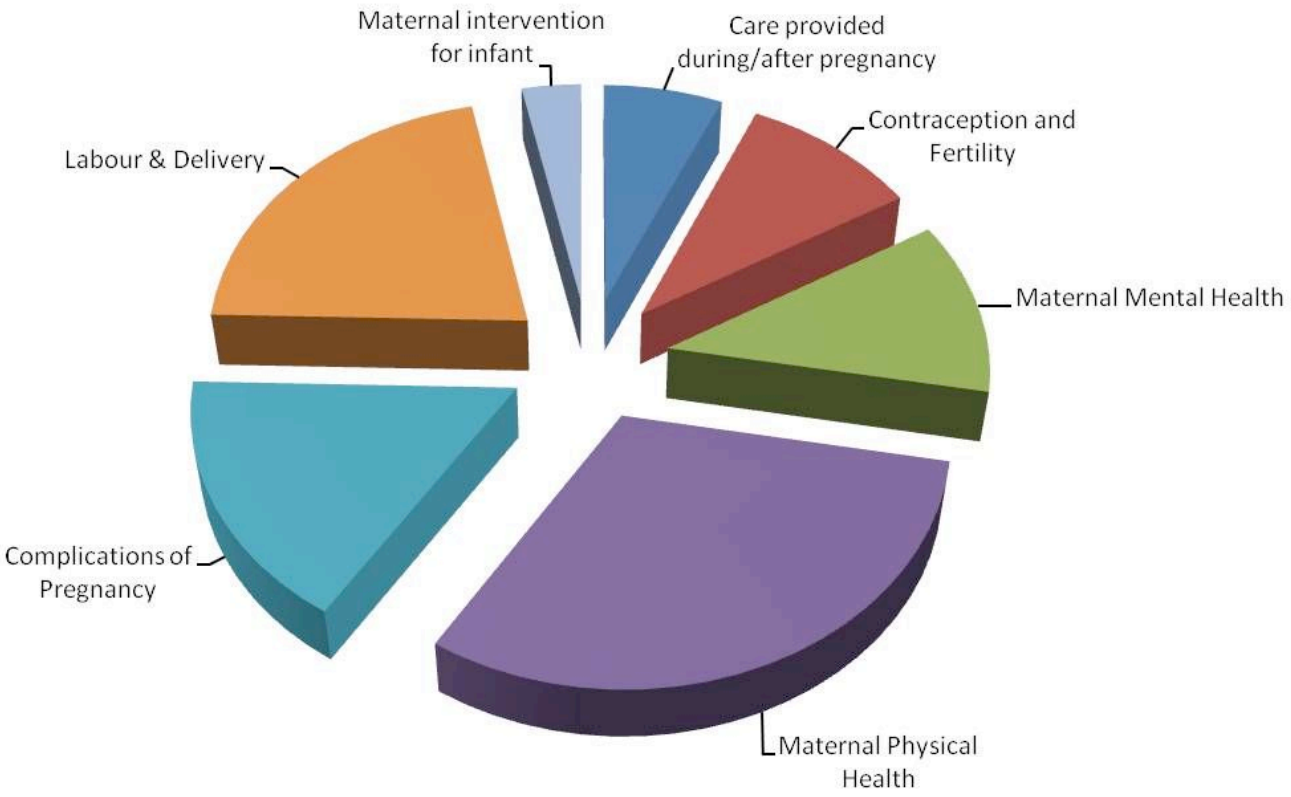
Maternal Trials in Canada
2005-2009



Maternal or Paediatric Trials in Canada

- 2nd to USA in total number of trials
- 3rd after Australia and Netherlands in number of trials/M births (maternal) or children (paediatric)
- Province-based academic organizations responsible for 94% of Canadian-sponsored trials

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MICYRN

- Now incorporated as federal nonprofit society
- Joins the 17 child – maternal research organizations in Canada
- Principal agenda to support multi-center collaborations
- Member interest in developing a national framework for multi-centre studies

Agenda for the Eastern Townships Workshop

- What works and what doesn't in doing Trials in Canada?
  - Methodologies, Ethics, New Frontiers
- How should we be engaging with patients, families and patient organizations?
- How should we be engaging with Industry?
- What do academic organizations require (reduce vulnerability) to support investigator-initiated studies? (DM, monitoring..)
- What can the academic organizations do to support networks?
- Determine how to qualify for CIHR-SPOR support as a (virtual) Support Unit or as a network of Support Units