Summary and recommendations

ICDRA pre-meeting
on paediatric medicines
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Points Agreed

• Children and children's medicines are a priority
  – World Health Assembly, Millennium Development Goals, US/EU, others

• We know more today
  – Growth and development, PK, modelling techniques, faster computers…

• But there are still major evidence gaps
  – Some real
  – Some apparent, linked to lack of availability of information

• Some achievements
  – Regulatory requirements/incentives
  – Technologies for dosage forms and delivery devices

• Multi-stakeholder approach essential
To do – big picture

• More, and better, ethical research on medicines for children
  – Capacity – clinical trials, ethics committees
  – Standards / guidelines for trials
  – BUT no duplication
• Find out what we already know
  – Get the existing literature and data together, with a critical review approach
• Better dosage forms and delivery devices
  – Solid / flexible / suitable / affordable

• Efficient and effective registration
• All aspects from developing, to making them effectively available to children
To do – specifics (1)

• Coordination
  – Activities related to children’s medicines globally
  – A global observatory? institution? A Working party?
  – Mechanism to link regulators, industry, academics?

• Collaboration,
  – To avoid duplication and ‘reinvention of the wheel’, to reduce cost
  – Regulatory authority task force?
  – Inventory of existing products, technologies and documents available to all, with a review for global applicability?

• Research
  – More, better (ethical), but more clever
  – New specifications of methodology for trials with children?

• Transparency
  – Registration of trials, access to data, dose,
  – Information about licensing, prequalification,
To do - Specifics (2)

• Information / global
  – Manipulation of adult medicines for paediatric use
  – Extemporaneous formulations
  – Dose
  – *But how to manage the overload?*

• Global advocacy – need to engage paediatricians, pharmacists, carers, researchers, parents and consumers so that they require better medicines for their children
‘Picking up pumpkins’
(getting the very low-hanging fruits…)

• Zinc for diarrhoea
  – products needed, use
• Treatment (vaccines) for pneumonia
  – products and use
• Neonates
  – Sepsis: what drugs, what doses, what products?
• HIV, TB, Malaria
  – What doses, what products?
• Analgesics
  – What drugs, what dose, what products?
…and the (biological) pumpkins

Vaccines
• Safety
• Importance of standardising definitions – adverse reactions / events
• Strengthen post-marketing surveillance (including persistence of protection)

• Antivenoms availability
Which recommendations and which deliverables?
Common objectives

• Better medicines for children:
  – More and better research with children
  – More transparency and information easily accessible
  – Increased availability of and access to paediatric medicines with age-appropriate dosage forms and delivery devices
  – Increased safety, through in particular monitoring of adverse drug reactions
Member States/ICDRA

- To form an ICDRA paediatric working group to
  - Ensure global collaboration
    - Agree on global regulatory standards
    - Streamlining paediatric clinical trials
  - Implement efficient registration of children's medicines
    - Put children medicines as top priority
    - Fast track strategies: eg hybrid applications, mutual recognition, cooperative review, waivers, others…
  - To develop consolidated view/advice on dosage forms and delivery devices
    - Guideline on dosage forms
    - Manipulations, extemporaneous formulations
    - Increase knowledge on paediatric excipients
  - To devise mechanisms for ensuring transparency and exchange of information
    - On trials
    - on licensing
    - On children’s medicines (dose, adverse effects)
  - Improve information on safety of medicines used in children
    - Infrastructure for pharmacovigilance
Recommendations to WHO

• Convene the global paediatric working group of regulators
• Work with civil society to mobilise and empower consumers, parents, patients’ groups and health professionals to advocate for better medicines for children
• Develop strategies for addressing high priority needs with achievable results:
  – Zinc for diarrhoea, Pneumoniae treatment, Neonatal sepsis, HIV, TB, malaria treatments, Analgesics
• Establish drug development helpline to support new essential medicines for children
And Others

• For industry: to continue integrating paediatric dosage forms and delivery devices early in development of new medicines
• For industry: to continue integrating paediatric needs, including developing countries needs in the development of new vaccines (safety)
• For generic industry, to develop missing dosage forms of off-patent medicines (including necessary fixed-dose combinations)
• To health professionals to engage actively into sound, ethical research with children, avoiding duplication
Better Medicines for children!