HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

EMA/PDCO Paediatric Formulation Working Group Experience

Brian Aylward MD

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Disclaimer and Declaration of Interests

- All opinions are my own, and cannot be considered to be the opinion of any Competent Authority or Regulatory Body

- No actual or potential interests to declare

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

The problem

- Medicines for children have historically not been appropriately made or tested in that population
- Several parallel legislative attempts to resolve this

- Paediatric Regulation 1901 of 2006
- Condition in adults, similar condition in children
- Paediatric Investigation Plan agreed with EMA

Paediatric Committee - PDCO

- 5 members/alternates appointed by CHMP
- 1 member/alternate from other EEA States
- 3 members/alternates representing HCPs
- 3 members/alternates representing patients

- Assessment supported by specialist Working Groups
  - Formulations, Non-Clinical, Modelling & Extrapolation, etc.

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Formulations Working Group

- Currently 12-15 national experts
- Pharmacy (industrial, academic and clinical)
- Regulatory
- Clinical

- Help the PDCO with the review of the formulation proposals of the applicant

Paediatric Formulation guidelines

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

London, 28 July 2006
EMEA/CHMP/PEG/194810/2005

COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE (CHMP)

REFLECTION PAPER: FORMULATIONS OF CHOICE FOR THE PAEDIATRIC POPULATION

Paediatric Formulation guidelines

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

1 August 2013
EMA/CHMP/QWP/805880/2012 Rev. 2
Committee for Medicinal Products for Human Use (CHMP)
Paediatric Committee (PDCO)

Guideline on pharmaceutical development of medicines for paediatric use

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Problem #1 – Industry

- Applicants to come with PIP proposals following completion of adult Phase 1
- Adult form already developed by this stage
- Paediatric development seen as an add-on
- Reformulation to account for paediatric needs time-consuming and costly
- Reluctance / resistance
- Investment vs Reward

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Problem #2 – Regulators

- Specific information often lacking or inadequate, esp. in younger age cohorts
- Uncertainties on how best to fill these gaps
- "Precautionary Principle" vs. innovation
- ? Inappropriately detailed assessment & requirements
- ? Lack of confidence that formulation will ultimately be developed

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Problem #3 – Innovation

- New chemical entities
- New "excipients"
- "Inactive" substance
- Anything which is not an active substance

- "Active" excipients – a contradiction?

My hopes for this meeting

- ; Earlier appreciation of a “whole life” paradigm by industry
- Greater acceptance of uncertainty by regulators
- Development of methods for addressing knowledge gaps

HPRA
An tÚdarás Rialála Táirgí Sláinte
Health Products Regulatory Authority

Thank you