Introduction of Break-out Session 1

“Mature Products”

Luc De Schaepdrijver
Graham Bailey

Washington DC, 5 May 2010
Session on Mature Products

- Mature products in late development, registered or on the market (clinical data available)
  - 4 case studies: CNS, Metabolic, Anti-infective, Urology
  - Each case will be addressed by teams representing various roles e.g. industry toxicologist (T), regulator (R), parent/ethical voice (P) and expert group (E)
  - All teams will assume the role of T -> R -> P -> E
Objectives

• “A retrospective look at the data”

• Concentrate on key questions:
  – Have the juvenile studies been helpful for risk assessment?
  – Did they increase our knowledge base?
  – Looking retrospectively, what might you have done differently?

• Share experiences, prompt discussion within your group and get everyone engaged

• Get a better understanding of the viewpoints and expectations of the various functions / players

• Identify the “key topics” for the overall group discussion

• Have fun!! Get to know each other!
Datasets for cases

- General product information (indication, pediatric age group(s), treatment duration and regimen, ROA, ...)
- Pharmacology
- Preclinical pharmacokinetics and metabolism
- Preclinical toxicology
- Clinical safety (e.g. in pediatric target population)
- Other relevant information (e.g. regulatory interaction, labeling info, ...)
Methodology (1)

• All teams address the case studies in a fixed sequence:
  1. Toxicologist
  2. Regulator
  3. Parent (ethical voice)
  4. Expert Panel

• Sets of tailor made questions for first 3 rounds (T – R – P) available to provide a framework & facilitate the discussions. Make notes within your group of your discussions

• Round 4 ('mini-plenary session’ per case) is to review the answers of the first 3 rounds as “expert panel”. Try to come to a consensus opinion in collaboration with the rapporteur

• A rapporteur gives feedback to the whole meeting
Methodology (2)

- All 8 teams get to know all 4 cases
- Team composition is indicated in your meeting package
- Good mix of regulators, pharma industry and CRO’s from EU/US on each team
- Facilitators will help ‘drive’ exercise (keep you on time) and report back to the whole meeting
- Any questions PLEASE ASK !!
Introduction to Break-out Session 2

“Emerging Products”

Luc De Schaepdrijver
Graham Bailey

Washington DC, 6 May 2010
Session on emerging products

- Products in early development with preliminary clinical plan and initial regulatory responses
  - 4 case studies: Immunology, Oncology, CNS, Pain
  - Case studies will feed the overall group discussion
  - Each case will be addressed by teams consisting of a mix of the 3 roles (T – R – P)
  - Two rapporteurs designated to each case study will capture the opinions / responses to a set of questions
Objectives

• “What can we do better?”
• Concentrate on key questions:
  – When & how can juvenile studies be helpful for risk assessment?
  – Which improvements do we need to get there?
  – Other recommendations or suggestions?
• Stimulate group discussion and get everyone engaged
• Get a better understanding of the viewpoints and expectations of the various functions / players
• Identify the “key topics” for the overall group discussion
Datasets for cases

- Pediatric Investigational Plan incl. indication(s), pediatric age group(s), treatment duration and regimen, route of administration, proposed preclinical and clinical strategy
- Pharmacology
- Preclinical pharmacokinetics and metabolism
- Preclinical toxicology in adults
- Clinical data in adults?
- Regulatory interactions
- Other relevant information
Methodology

- All teams are composed of a mix of T-R-P and try to come to an expert opinion
- All 8 teams get to know all 4 cases
- Different team composition than yesterday! (see meeting package)
- Two rapporteurs per case facilitate the discussion and provide feedback to overall group
- If you need assistance please ask!
## Logistics

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Session 1 “Mature Products”
• Case 1 = Compound V : CNS
Georg Schmitt, Isabelle Leconte

• Case 2 = Compound X : Metabolic
Jeffrey Moffit, Karen Davis Bruno

• Case 3 = Compound Y : anti-infective
Kok Wah Hew, Beatriz Silva Lima

• Case 4 = Compound Z : urology
Kary Thompson, Ulla Wändel-Liminga

Session 2 “Emerging Products”
• Case 1 = Compound A : Immunology
Isabelle Leconte, Karen Davis Bruno

• Case 2 = Compound B : Onco
Beatriz Silva Lima, Kary Thompson

• Case 3 = Compound C : Pain
Georg Schmitt, Kok Wah Hew

• Case 4 = Compound D : biological – CNS
Ulla Wändel-Liminga, Jeffrey Moffit
BACK-UP SLIDES
OVERALL GROUP DISCUSSION

CONSENSUS BUILDING
Topics for Consensus Building

- When a juvenile animal toxicity study is warranted [assumes a data gap has been identified that can be addressed by a study] which points do you consider wrt following questions?
  - What are the minimal ages to start dosing for the different species and administration routes? (assuming no particular target organ)
    - Does a Day 10 rat = newborn child?
  - How long would you dose to cover all critical stages of organ system development? Is there a minimum length for such studies?
  - If there are organs of potential concern what criteria would you use to prioritize?
  - Are there endpoints, or organ systems, that should always be evaluated? Recovery needed (how long)?
  - Screening study design versus targeted study approach?
Topics for Consensus Building

- Which species and how does one decide?
- How do you select the high dose level?
- When, during the pediatric program, should the study be conducted?

- **What are the challenges for global pediatric development?**
  - Have differences in the current regional regulations been a hurdle? If yes, was this because of content and/or timing differences?