EFPIA position on CHMP work programme: Evaluation of risk benefit Scientific Advisory Groups

Julian Thompson on behalf of EFPIA

February 2009

OBJECTIVES:

To share EFPIA perspectives on the evaluation of risk:benefit

To share EFPIA experiences of Scientific Advice Groups

To engage in dialogue in the Q&A session

EFPIA position on Benefit-Risk balance Assessment 1

- Recognize that improved benefit-risk evaluation process would be beneficial
  - Pre-approval pre-filing
  - Regulatory review/Approval stage
  - Post-approval as patient exposure increases
  - Facilitate transparency of regulatory evaluations
  - Enable the patient:physician relationship
- General agreement with conclusions in March 2008 CHMP reflection paper and support development of better methodology and tools
  - Accept these tools are not available today
  - Encourage development/validation of these tools and will actively contribute
  - Note the importance of the IMI project for developing new methods for benefit-risk analysis
  - Note similar projects underway (e.g. PhRMA) and seek opportunities to partner

efpia

EFPIA position on Benefit-Risk balance Assessment 2

- Benefit-risk evaluation should be a combination of a defined methodology and transparent subjective judgement
  - A product should always be assessed on both risk and benefit
  - Need to ensure real/identified risks are managed (regulatory action) and establish an adequate threshold of probability for potential risks
- Proposals:
  - Collaboration with PhRMA/other projects to be optimised (single approach)
  - Consider a single qualitative benefit-risk model which captures qualitative and quantitative efficacy and safety data, complemented with a toolbox of quantitative methods
  - Work closely with the CHMP and regulators

EFPIA Experience with Scientific Advice Groups (SAG) 1

- Mandate: SAGs are established to provide an independent recommendation on scientific/technical matters related to products under evaluation (pre/post authorisation) by the CHMP or any other scientific issue relevant to the work of the Committee
- SAG experience presented here from multiple companies across different disease areas across the entire product lifecycle

EFPIA Experience with Scientific Advice Groups (SAG) 2

- **What works well?**
- Meetings are generally well organized
- There is a clear process
- CHMP/Rapporteurs share the issues to be addressed in advance
- Appropriate questions posed to the SAG that focus on key issues
- Open and detailed scientific dialogue within the SAG
- Rapporteurs generally very supportive of the process and reviewing presentations / providing comment and providing perspective on their key issues for the meeting
- SAG outcomes inform CHMP

EFPIA Experience with Scientific Advice Groups (SAG) 3

- **What does not work so well?**
- For certain indications SAG does not always have enough representatives with expertise of very rare conditions and experience in national differences in clinical practice
- Difficulty in identifying experts also applies in areas where SAG may not have been convened before and experts should have experience in specific clinical field (rather than generalist in therapeutic area).
- Conflict of interest considerations may preclude inclusion of key experts
- All experts providing input into the SAG should be present in person at the meeting (rather than provide input in advance/by phone)
- Meetings can have variable attendance and meeting efficiency depends on the chair
- Examples where debrief provided after the meeting did not correlate with the written minutes of the meeting (and were provided later than expected)

EFPIA Experience with Scientific Advice Groups (SAG) 4

- **What could be done differently in future?**
- Rapporteur to share their presentation with the applicant in advance (this presentation is provided in closed forum to the SAG without the applicant present - hence applicant is not aware of the background/context to questions as they arise during their presentation/discussion)
- Allow sufficient time to get the right panel together
- The selection of SAG experts is key: Rapporteurs should seek experts to facilitate open balanced and independent review
- Consider use of a patient organization representative on the SAG
- Consider expanding core beyond 9 members
- Consider more waivers (with transparency) to ensure key experts are not excluded

EFPIA Experience with Scientific Advice Groups (SAG) 5

- **Conclusions**
- SAGs inform scientific debate and answer questions on key issues
- Valuable where specialised expertise is required and/or a perspective on clinical relevance is needed
- Areas for improvement
  - Ensuring sufficient and appropriately qualified experts attend the meeting
  - Transparency
  - Meeting conduct consistency