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EMA/FDA/Health Canada Workshop on Paediatric PAH






Industry Perspective

Date: 12/06/2017 * **Version:** Final

Industry perspective on current landscape for paediatric PAH trials

Industry commitment

- More than 20 years after first PAH product available for treatment of adult patients, there are very few medications indicated for children, and none globally

- As leading pharmaceutical companies in the field of PAH, we recognize the importance of working to develop new therapeutic solutions for paediatric patients

Multiple challenges have resulted in a lack of approved pediatric PAH treatment options

- Multiple clinical trials **in a rare population** conducted at a limited number of qualified expert centers makes it very challenging to complete individual studies in a reasonable time
- **Lack of equipoise**: disconnect between clinical practice and clinical experiment
  - parents/investigators unwilling to randomize patients to placebo/SOC (standard of care) controlled trials when drugs have demonstrated efficacy in adults and are already being used in pPAH patients
- **Uncertainty about assumptions and methodology** (e.g., knowledge on appropriate endpoints, applicability of extrapolation) increases risk of inconclusive studies
- Level of evidence and interpretation of study data **differ between different regions** and stakeholders

Summary of Industry Perspective

1. Paediatric PAH patient numbers are very small – **each patient should contribute** to a conclusive study outcome
2. To support optimal trial conduct, **global regulatory harmonization** is key:
   - one achievable level of evidence required for approval
   - on endpoint(s), clarification, harmonization, and validation
   - practical approaches for extrapolation [of efficacy] from adult data, or from one paediatric population to the other, if applicable
3. *Goal*: streamline development, allow single studies meeting global (EU/US) requirements, **improve success in a reasonable time**