EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Key points from Introductory session – Setting the scene

Workshop on Haemophilia Registries
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Aim of Session:

To identify regulatory needs and discuss how well they are currently met, using Factor VIII and Factor IX products as an example

Discussion questions

What are the regulatory requirements and how well are they being met?

•Limits in clinical data availability at time of Marketing Authorisation for products used in rare diseases

•Gap between clinical data package leading to Marketing Authorisation, particularly with respect to PUPs, and long-term performance of the product.

What is the key data that regulators would like to see coming out of registries?

•Robust data to close the gap

•Ideally every patient in a registry, especially PUPs

•Key data:

- Patient characteristics

- longitudinal data covering product(s) used, treatment modalities, consumption of product, bleeding rates, adverse events

Key points: Summarising key points from discussion

- New products - possibly new safety issues (eg. PEG) of concern, long term follow up needed
- Inhibitors (especially low titre); improvement of comparability between different assays (ISTH work for harmonization reducing variability)
- Need assured quality of the data, enough data in a reasonable time frame
- Small sample size regarding to safety available pre-authorisation
- Post-authorisation: possibility of increasing sample size with registries and thus targeting whole hemophilia population. Registries should be better funded (e.g. CRAs to audit) to improve data collection (better quality?)
- Registry considered as an one possible way (among others) to collect data
- Independence of the registry is important. Objectives should be robust up front.

Key points: Summarising key points from discussion

- Issues of EMAs requirement with PTPs vs. PUPs at the moment. Are they appropriate? (PDCO, PRAC and CHMP discussion needed)
- PUPs with regard to paediatric regulation: is efficacy results enough? How are the risks for e.g. with regard to immunogenicity met with PUP studies?
- At the moment PUP-studies underpowered with respect to immunogenicity
- PASS-studies needed for long term safety issues (immunogenicity) and registries in addition for long term clinical benefit (QOL, joint bleeds etc.) How to ensure adequate data collection?
- Patients should not be excluded from registry because recruited in a clinical trial
- Adequate definition of a registry is vital (what questions are needed for robust data collection)