EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# Key points from session 2 – Registries

Workshop on Haemophilia Registries
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# Aim of Session:

**On the basis of current experience, to consider how to maximise the benefit to public health that can be derived from data collected in registries.**

**Strengths and weaknesses of registries from the perspective of providing safety and efficacy data on products, and consideration of approaches / initiatives to strengthen this**

# Key points (Summary of discussion points)

•Are existing registries providing the data that regulators are looking for?

- Not exactly known, lack of transparency
- Depends also on quality of data
  - Most important: monitoring of the data
  - There should also be an international, at least European protocol how to monitor the patients, e.g. inhibitor testing every 3 ED until xx ED)

- Problem: no interface to other systems to pool and share data

# Key points

•What is the rationale for different approaches in some cases (e.g. EUHASS, PedNet)? Concomitant Surveillance and/or prospectively defined longitudinal data collection?

- Different approach comes from different questions that were asked and should be answered with the data: e.g., EUHASS is focussed on rare events
- PedNet has been developed to understand the etiology of immunogenicity in PUPS and to collect data on prophylaxis and long term outcome in a well-defined patients group that is followed for 20 years

# Key points

•It is desirable to have data from the different registries in a similar fashion so that data from different registries can be combined.

- yes!
- Important to get an informed consent in all countries from all patients (European solution possible?)
- PID from patients through center code and unique identifier (number could be stored at EMA or at an academic site)

•How far is this achievable with the national registries?

- Clear opinion: if at all, then it is only achievable with the national registries, but not easy to establish
- National registries that merge into a European umbrella registry
- Needs education of patients and centers to enlarge compliance
- Quality of the data must be guaranteed( Monitoring of baselines Mandatory)
- Collaboration with the industry must be improved, basic rules must be established for handling and analyzing the data, questions from an industry-perspective should be answered, too (Economic, consumption, … )

# Key points

- Is definition of a minimum data set the way forward (an ‘inter-operable model’).
- Absolutely
- “Minimum dataset” is not a short list – have to monitor confounders as well
- Different data sets for PUPs and PTPs needed
- It must be possible in national registries to link data from PUPs to their data when they become PTPs
- Perhaps start with end: fix the objectives, then go back to needed parameters, then find out, if they were collected in the registries
- Regulators should push for harmonization
- Agree on a generic protocol especially for the new products and make it mandatory

# Key points

•Are all patients included in clinical trials allowed to be included in registries?

- Large agreement from all stakeholders, but how exactly this can be managed must be discussed. More collaboration and cooperation is needed.
- It should be possible to not only state “investigational product” in the registry, because so you can’t even distinguish between long acting and “normal” products (industry to discuss the conditions and report)

•How to prevent overlapping of patient information from multiple sources (registries, clinical trials)?

- Unique patient identifiers!

# Other points

- Prelicensure no collection of PUP data on immunogenicity but only PK and efficacy
- Mandatory post licensure through RMP of at least 100 PUPS wiht comparable data to compare products through registrie such as PedNet
- Concern with the current PIP in the coming years 500 PUPS are needed
- No transparancy of these data and products can not be compared
- Independence of registries is important, funding needs to be improved
- Diseaese registries  are prefered to product registries! ( Xavier/ Bouvy)
- Facilate interaction through PIDs and research questions