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Committee for Medicinal Products for Human Use (CHMP)

Consensus points - Workshop on Haemophilia Registries

1. Introduction

Independent, disease-based registries are a potential source of valuable safety and efficacy data on products. Disease-based registries enable data to be collected over a long period and allow comparison of products. Ideally, when a significant number of patients are entered, registries are able to minimise registration bias. Data on both previously treated patients (PTPs) and previously untreated patients (PUPs) can be collected, provided the different needs for follow-up are reflected.

EMA is currently embarking on a strategy for patient registries. This initiative provides an appropriate starting point to consider what regulators need from haemophilia registries and also how to maximise the benefit to public health that can be derived from data collected in registries. The aim is to get the best capture of data in the EU and build on existing expertise in this area.

It is proposed to work with stakeholders on this topic, commencing with this EMA workshop.

2. Purpose of the workshop

For new FVIII and FIX products, the Pharmacovigilance Risk Assessment Committee's (PRAC) recommendations include enrolment in registries (e.g. EUHASS, PedNet). The workshop will discuss:

- What is the key data that regulators would like to see coming out of these registries?
- Are existing registries providing the data that regulators are looking for?
- What is the rationale for different approaches in some cases (e.g. EUHASS, PedNet)?
- How to maximise the benefit to public health that can be derived from data collected in registries?
- It is desirable to have data from the different registries in a similar fashion so that data from different registries can be combined. How far is this achievable with the national registries?

The aim will be to identify strengths and weaknesses of registries from the perspective of providing safety and efficacy data on products, and to consider approaches/initiatives to strengthen this.

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3. Consensus points

- Collaboration and thinking together of all stakeholders and consider different needs (including HTA and patients)
- Regulators to identify in advance which questions they would wish to see addressed by registries
- Registries only one platform for defined studies. They will not answer all questions.
- Ideally every patient should be in a disease registry
- Patient identifier to avoid overlap between registries and reduce double-counting
- Agreement with regulators and other stakeholders on a minimum protocol or dataset (parameters, minimisation of bias, look at covariates / variables, adjust for confounding)
- Patients enrolled in clinical trials should remain in registries
- Review PUP approach in EMA guidance
- Link with initiatives of other rare disease registries as there will be common issues
- Harmonise national registries and promote/support more national registries and quality assurance

4. Way forward

- Establish a collaborative network involving all stakeholders
    - Regulators will have a key role
- Data sharing (anonymised) for EMA and national competent authorities should be possible, upon request