Workshop on Haemophilia Registries
EMA, London 1-2 July 2015

Anneliese Hilger, Chair BPWP
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<th>Conflict</th>
<th>Disclosure - if conflict of interest exists</th>
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<td>Research Support</td>
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<td>Director, Officer, Employee</td>
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• Patient data base in the treatment center
• Regional registries or locally collaborating treatment centers that populate a local registry
• National Registry
• European and trans-regional registries and data collections
• International data collections and registries
• Industry-initiated data collection
• Clinical trials (investigator or Cy)
Registries...

- no overarching structure for how to manage, design, or host such data collection

- located at the Ministry of Health or organized by academia, patient organizations, companies

- could be set up by different stakeholders at different time-points and focus on different aspects of the disease, so that some countries end up with several national registries with no or minor interoperability.
What is known about these registries and data collections?

- Which parameters will be observed
- Which patients will be included
- What is the number of enrolled patients
- What is the outcome of the registry
- How complete is the data
- When will an analysis be performed and will it be published
- Who will have access
Does the current number of hemophilia registries improve patient safety and leads to a better research in the field of Haemophilia?

Participants

Steering Committee
Experts from CHMP, BPWP, PRAC, PDCO, and EMA
FDA and Health Canada
Patient Organisations
Health care provider organisations
Industry Organisation
Companies
National Registries
European Commission
Purpose

• Identification of Key data (regulatory perspective)
• Are existing registries providing the data that regulators are looking for?
• How to improve 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?
• Identification of strengths and weaknesses of registries

• **Consensus points**

• **Next Steps...**
Consensus points

- Collaboration of all stakeholders (including HTA and patients)
- Regulators to identify what they would wish to see from registries
- 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
• **Factor VIII**
  - Feb 2013, 86 PTP, >12yrs; Jan 2013 50 PTP <12yrs; Feb 2018, **25** PUP
  - Mar 2013, 10 PTP, >12yrs; Sep 2015 50 PTP <12yrs; Sep 2019, **100** PUP
  - June 2014, 10 PTP, >12yrs; Mar 2016 50 PTP <12yrs; Dec 2020, **50** PUP
  - Jan 2015, 8 PTP, >12yrs; Jun 2015 50 PTP <12yrs; May 2018, **50** PUP
  - Oct 2010, 50 PTP, >12yrs; Jul 2014, **20** PUP
  - Sep 2011, 17 PTP, >12yrs; Jan 2012 50 PTP <12yrs; Feb 2016, **50** PUP
  - Sep 2012, 18 PTP, >12yrs; Nov 2012 50 PTP <12yrs; Dec 2018, **50** PUP
  - Dec 2016 50 PTP <12yrs; June 2021, **100** PUP
  - Dec 2014 15 PTP >12yrs; Aug 2015 50 PTP <12yrs; Dec 2022, **50** PUP

• **Factor IX**
  - July 2012, 20 PTP, >12yrs; June 2015 20 PTP <12yrs; 2019, **40** PUP
  - July 2011, 24 PTP, >12yrs; June 2012, 12 PTP, >12yrs; Oct 2014, 50 PTP, >12yrs; Sep 2014 20 PTP <12yrs; June 2017, **20** PUP
  - June 2013, 15 PTP, >12yrs; Aug 2014, 20 PTP, <12yrs; Oct 2020, **20** PUP
  - Jan 2011, 50 PTP, >12yrs; Jul 2015, 20 PTP, <12yrs; Nov 2019, **40** PUP
International Society on Thrombosis and Haemostasis (ISTH)

The ISTH Congress in Toronto was notable for the progress reported on new developments in treatment of haemophilia. Longer-acting factor concentrates have now been on the market in the USA and Canada since last year while they await licencing in Europe. The scientific and standardisation committee sessions included discussion on real life experiences of clinicians with these products to date. There was a wide variation in viewpoints on several questions posed. In relation to which people with haemophilia (PWH) should be given prioritised access to these products, views ranged from children, those with poor venous access or poor compliance or adults who should be switching from on demand to prophylactic therapy. Several clinicians stated they would be reluctant to prescribe longer acting factor concentrates to previously untreated patients (PUPs) outside of clinical trials until more data is available. Updated information from the clinical trials for some of these products still under development were reported. .....
Pro’s and Con’s

Clinical Trials
- mandatory for every new Product
- same evaluation criteria
- enable comparison of products
- Regulators do have access to data

Registry
- voluntary
- data collection same criteria?
- comparison of products?
- Regulators do have no access to data
Not agreed upon...

- Data sharing (anonymised) for EMA and national competent authorities should be possible, upon request
• What is the consequence that some patients are registered in several registries and others in none?
• Does the growth of the number of registries come to the expense of the overall data quality?
• What needs to be done to transform registries into a powerful clinical research tool?
• How can data derived from registries be used for regulatory purposes?
• Who should have access to the data?
Way forward

• Start discussion with EMA groups
• Establish a collaborative network involving all stakeholders
• Regulators will have a key role
• Consensus points:

Thank you for your attention