Registry holder's perspective on the use of the Guideline for registry-based studies

I am Eoin McGrath @jacie_ebmt

History of EBMT & Registry

- Established in 1974
- >5,000 members in over 60 countries
- Registry - largest database of its kind
- Currently contains data on >709,000 HSCTs[1]
- Received >40,000 new HSCT registrations in 2019[1]
- Data from >550 centres / >50 countries[2]

1. EBMT annual report (2019; available at: https://www.ebmt.org).
2. https://www.rsnn.nl/sites/rsnn/files/181001_Jurgen_Kuball.pdf (accessed June 2019).

Why does EBMT have a registry?

Perform studies

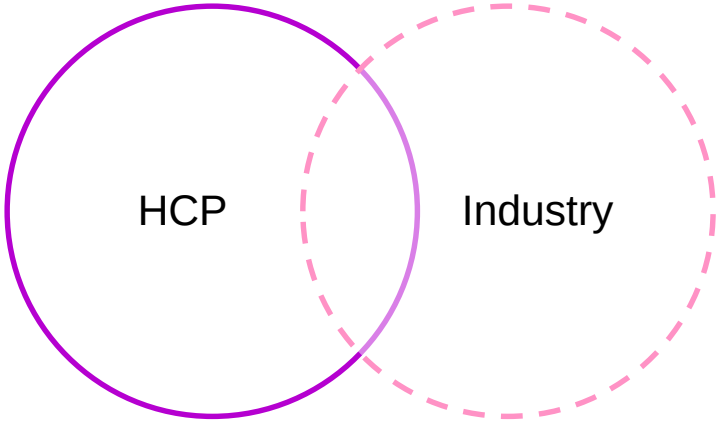
- e.g. in 2019, 138 EBMT publications

Assess trends

- e.g. Growth in haplo mismatched versus cord blood

Quality control

- e.g. Data completeness and benchmarking



EMA Qualification Opinion Feb 28 2019

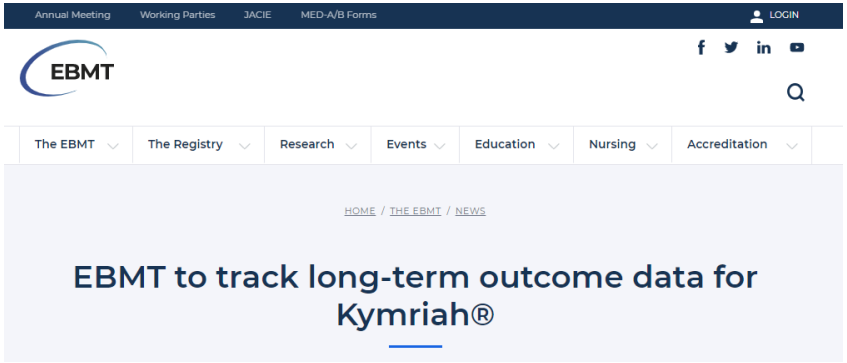
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

28 February 2019
EMA/CHMP/SAWP/792574/2018
Committee for Medicinal Products for Human Use (CHMP)

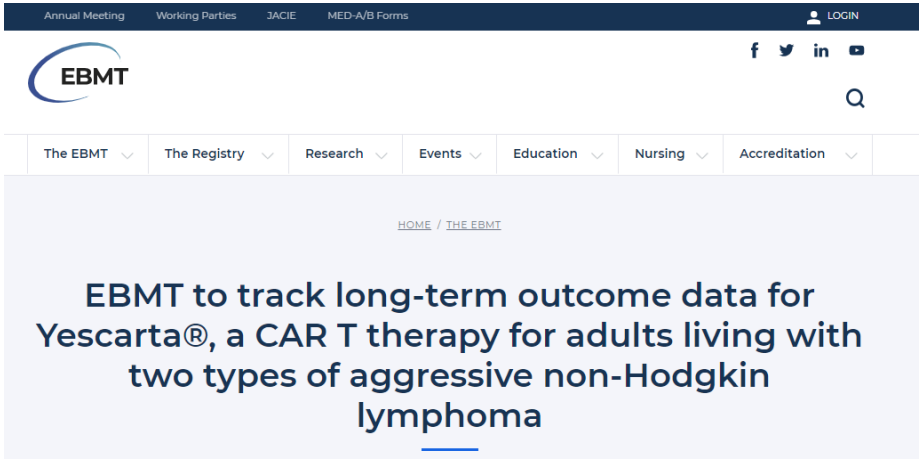
Qualification opinion on Cellular therapy module of the European Society for Blood & Marrow Transplantation (EBMT) Registry

"CHMP considers that the current status of the cellular therapy module of the EBMT registry (coverage, core dataset, governance, quality assurance approaches, and completeness of core variables), may allow its use as a data source for regulatory purposes in the context of the … studies concerning CAR-T cell therapies authorised for haematological malignancies"

PASS agreements



February 2020

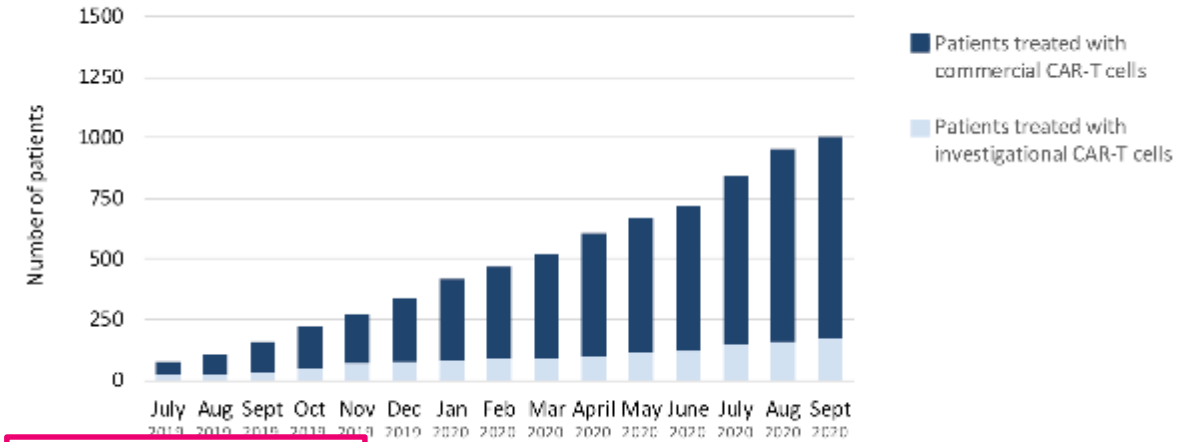


June 2020

European Society for Blood and Marrow Transplantation

Reported CAR-T patients

Number of CAR-T cell treated patients registered in the EBMT Registry

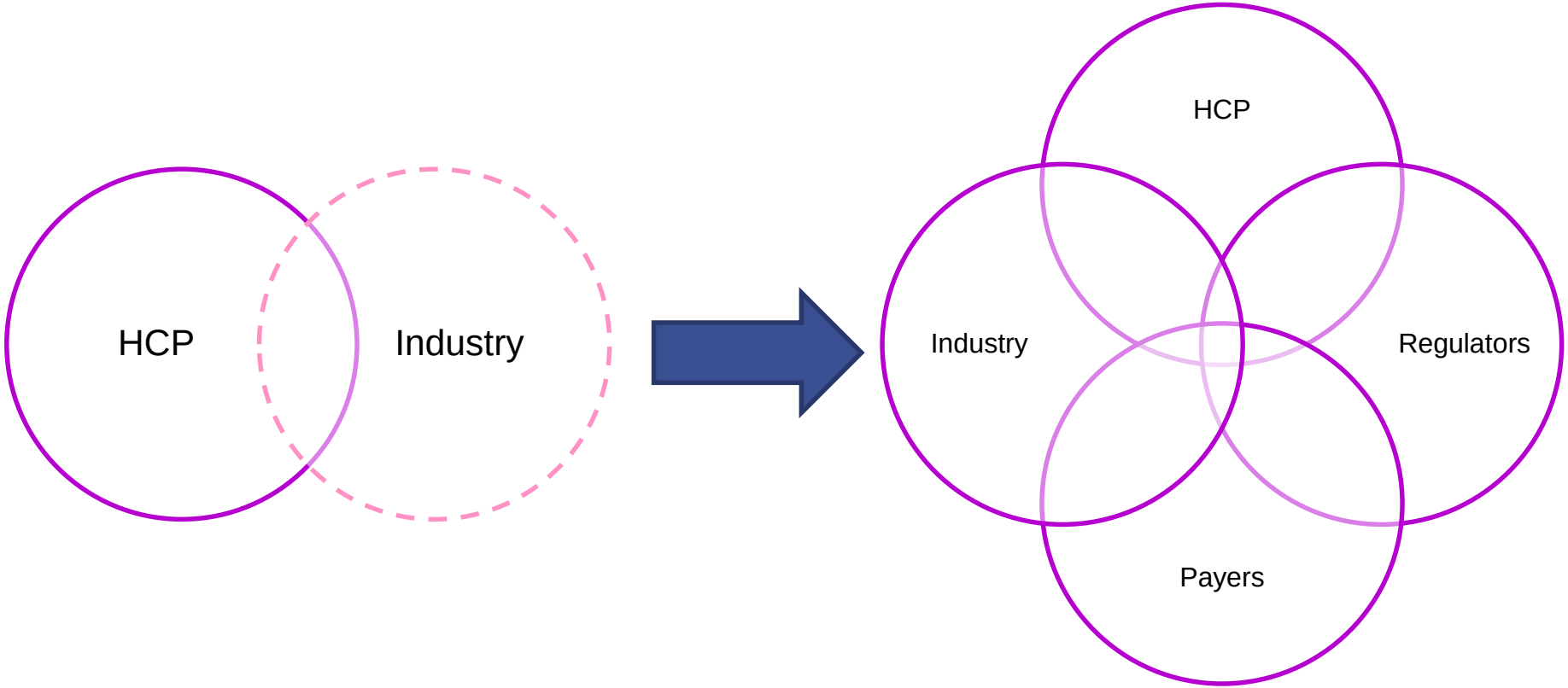


Source: EBMT Registry, September 2020

Countries reporting CAR-T cell treated patients to the EBMT Registry

Source: EBMT Registry, September 2020

EBMT adaptation to wider context, new stakeholders and different needs

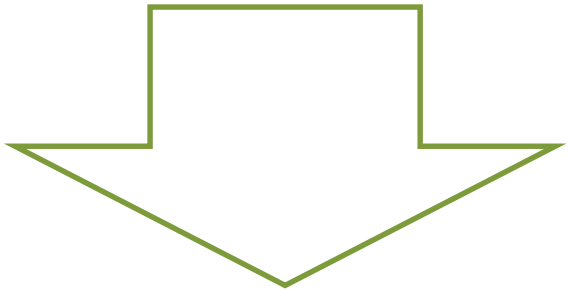


Registry-based studies: our challenges

- Use of Registry data for the conduct of PASS is new to authorities, Ethics Committees and hospital legal departments
- No harmonized European framework for the conduct of NIS-PASS based on secondary use of data. National and local interpretations are different.
- Variability of interpretation of GDPR between Member States
- Amended ICF required to allow sharing of pseudonymized data with MAHs. Sharing of anonymous data is virtually impossible due to:
  - Small patient numbers receiving cellular therapies
  - Personalised medicine - batch numbers are patient-specific
- Terminology
  - Understanding the difference ‘primary’, ‘secondary’, ‘purpose’, ‘use’ and ‘collection’

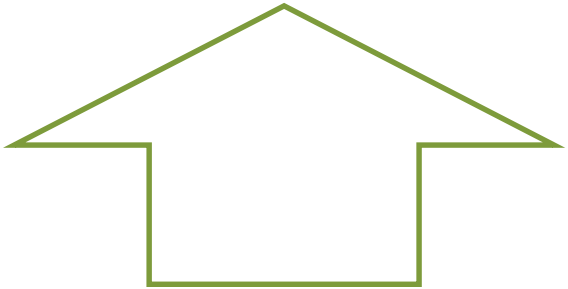
Implementation new ICF and study contracts complicated and cumbersome process

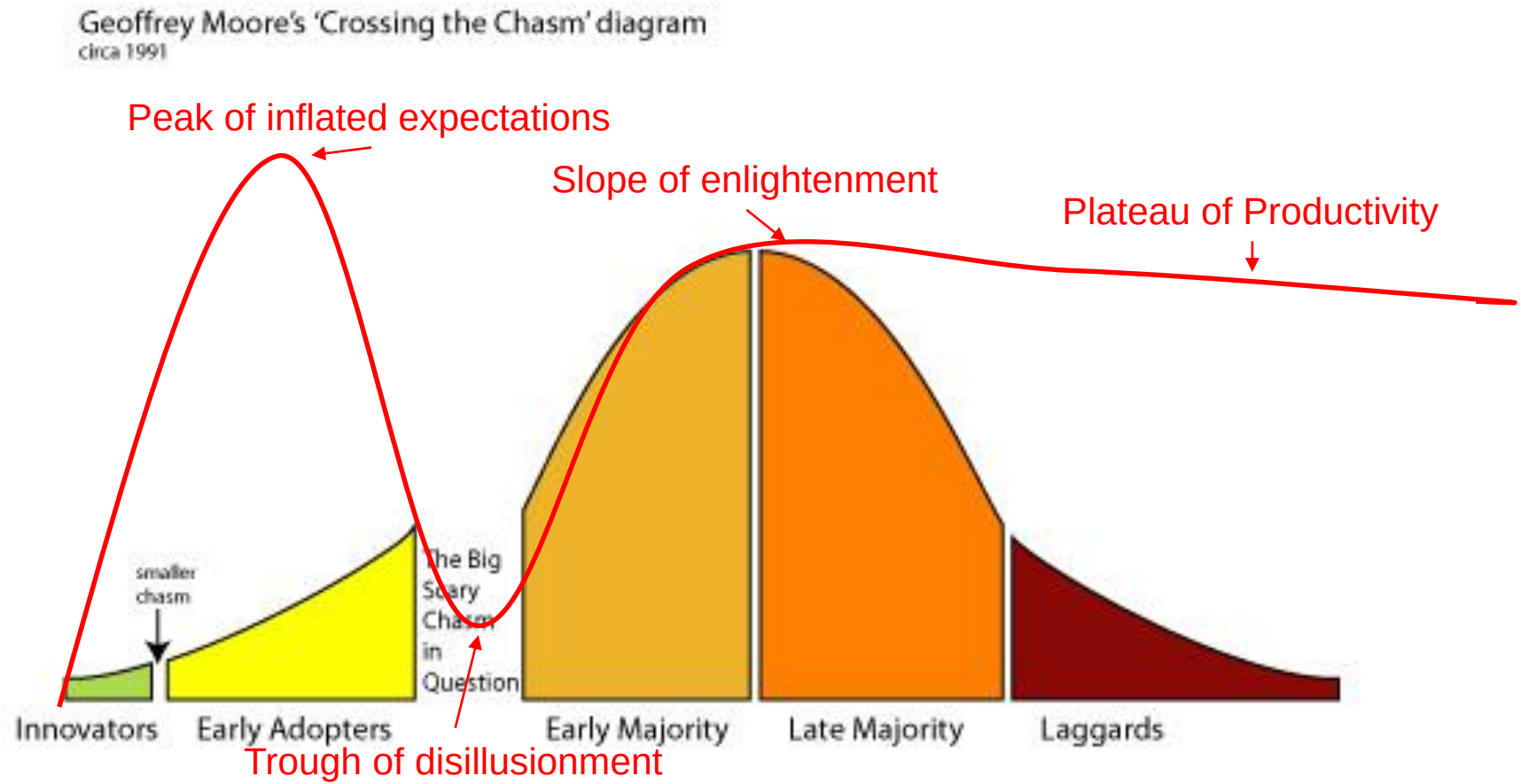
Overall impressions



- Reiterates role of registries for regulatory purposes
- Establishes expectations

- Written for MAH/MAA
- More guidance is needed for registry holders





https://medium.com/@invox_finance/why-blockchain-doesnt-want-to-end-up-like-the-microwave-73dc4a11d7f6
https://en.wikipedia.org/wiki/Crossing_the_Chasm

Multi-stakeholder Forum

All sessions will be held on Tuesday, March 16, throughout the day

The 2nd Multi-stakeholder Forum on Innovative Cellular Therapies is a forum designed to align regulatory science, practice and health economics with multiple points of view from both healthcare providers and users. It aims at fostering affordable access to innovative cellular therapies.

Simultaneous translation between Spanish and English TBC

EBMT European Society for Blood and Marrow Transplantation

Thanks to my colleagues Sofie Terwel, Marianne Mol and Iris Bargalló for their help with preparing the presentation



Any questions?

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