EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# 7th Industry stakeholder platform on research and development support

23 November 2021

Presented by Peter Arlett
Data Analytics and Methods Task Force, EMA

An agency of the European Union

# Today's presentation

1. Big data Steering group workplan
2. Key achievements in 2021 and future highlights for 2022
3. Deep dive on 'RWE' priority recommendations
   - DARWIN EU
   - Complementary activities
4. Future collaborations

# Looking forward

*"By delivering the vision of a regulatory system able to integrate Big Data into its assessment and decision making, we can support the development of innovated medicines, deliver life-saving treatments to patients more quickly and optimise the safe and effective use of medicines through measurement of a products performance on the market."*

Big Data Task Force final report December 2019

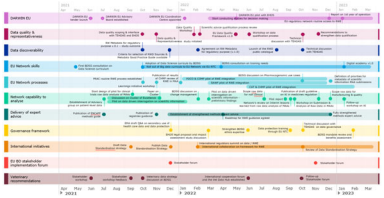
# Big Data: from recommendations to implementation

**Jan. 2020**

'Ten recommendations to unlock the potential of big data for public health in the EU'

**Sep. 2020**

Making best use of big data for public health: publication of the Big Data Steering Group workplan for 2020-21




1st Big data steering group meeting in May 2020

**May 2020**

Publication of BDSG workplan 2021/2023

**Aug. 2021**

# Big Data Steering Group: Recommendations

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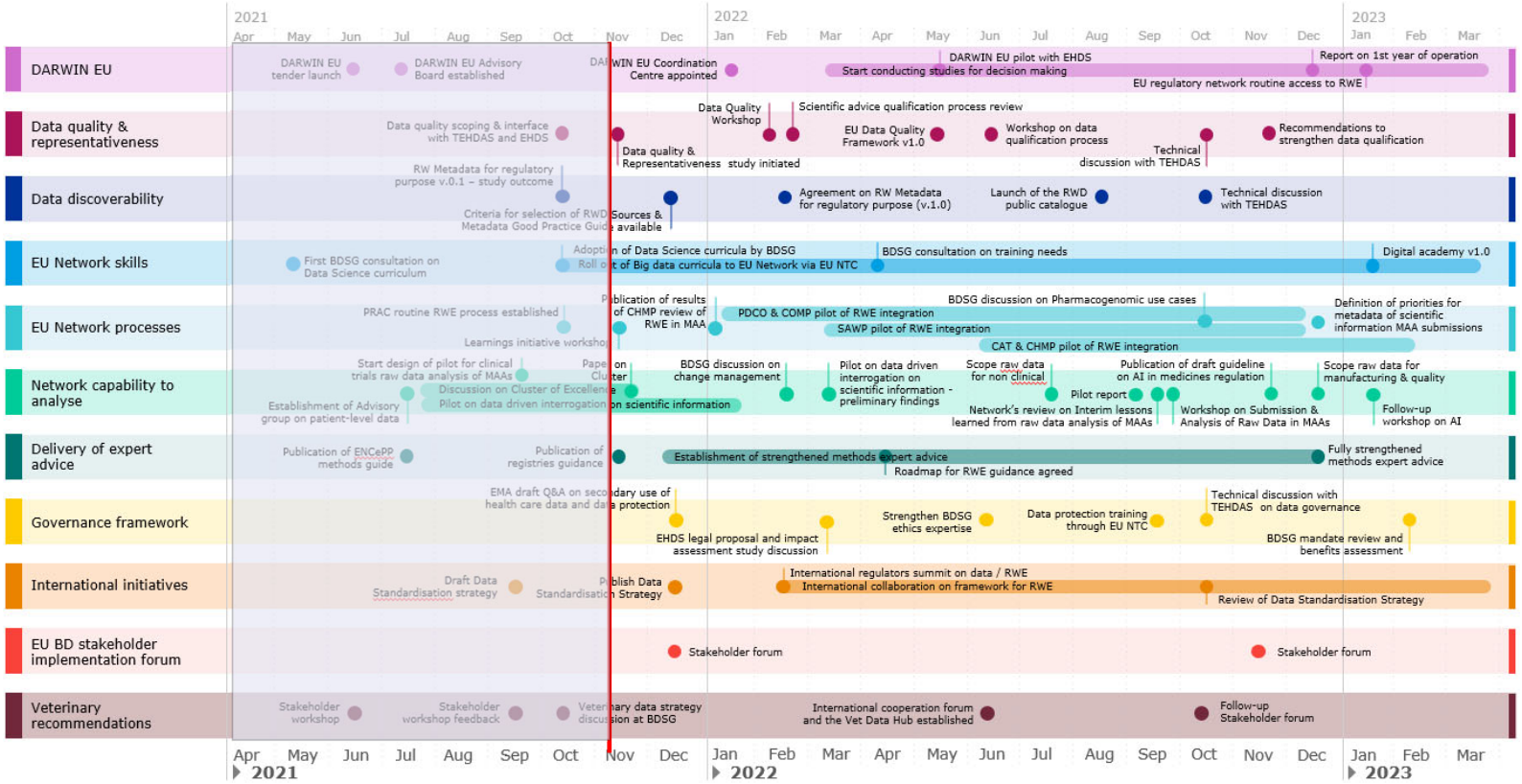
# HMA-EMA Joint Big Data Steering Group work plan




On track

# Key achievements in 2021 and future highlights for 2022

HMA Heads of Medicines Agencies

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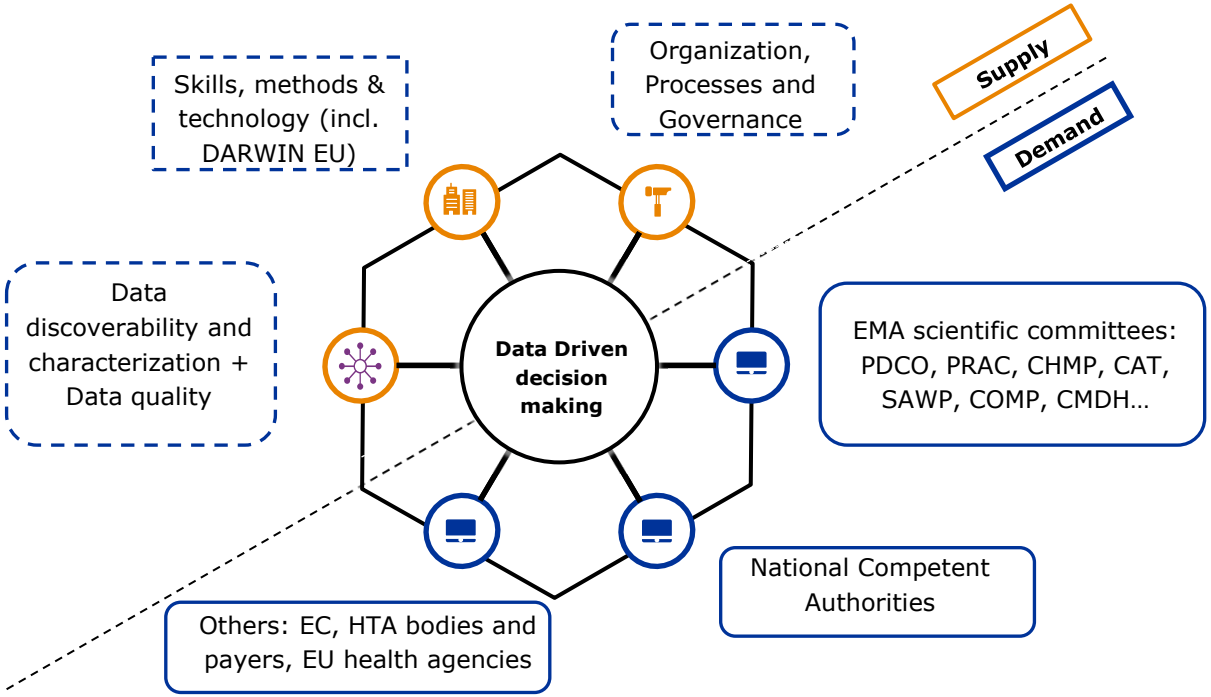
| | 2021 | 2022 |
|---|---|---|
| Next slides DARWIN EU | **Procurement launched** | **Coordination Centre appointed** – Support EHDS2 pilot – **Start conducting pilot/studies** for decision making |
| Next slides Data quality & representativeness | **Procurement launched** for academic consortium to deliver a data quality framework | **EU Data quality Framework v1.0 available -** Recommendations to **strengthen data qualification** – **2 workshops** on data quality and data qualification |
| Next slides Data discoverability | **Study of metadata for real-world data** | **Agreement on RW Metadata for regulatory purpose** (v.1.0) - **Launch of RWD public catalogue** |
| Next slides EU Network skills | **Data science curriculum** finalised – **Survey of skills** completed - **Training delivery outsourcing** initiated | **Roll out** of Big data curricula (Biostatistics, Pharmacoepidemiology, Data science) |
| Next slides EU Network processes | **RWE use cases developed** with PRAC, PDCO, COMP – **Learnings initiative workshop** November 2021 | **RWE integration pilots** (PDCO, COMP, SAWP, CAT, CHMP) |
| Network capability to analyse | Discussion on **Clusters of Excellence** - **AI workshop** **Pre-pilot on raw data analysis** completed | **Clusters of Excellence paper** - **Draft guideline on AI** in medicines regulation - **Workshop on Raw Data in MAAs** and **Pilot of raw data analysis** execution |
| Next slides Delivery of expert advice | **ENCEPP RWE methods guide** published **- RWE and advanced analytics expert advice** established | Publication of **registries guidance** - **Roadmap for RWE guidance agreed** |
| Governance framework | **BDSG recommendations on ethics advice** | **EHDS** legal proposal and impact assessment study supported – publish Q&A on **data protection** |
| International initiatives | **Data standardisation strategy** adopted Dec 2021 – Progress on **RWE Collaboration Roadmap** with FDA | International **regulators summit on real-world evidence** |
| EU BD stakeholder implementation forum | **Multi stakeholder forum Big Data - Dec. 2021** | Stakeholder forum |
| Veterinary recommendations | **Workshop on the** Veterinary **Data Strategy** | **International** cooperation **forum** and the **Vet Data Hub** established |

# Deep dive on 'RWE' priority recommendations




Vision to enable the generation and use of RWE in decision-making

# Data (and studies) discoverability, characterisation and quality

A catalogue of data sources (including registries) is being developed - Q4 2022 (TBC)

- The catalogue will be a newer and better version of the current ENCePP Resources Databases, focusing on data sources available in EU
- The catalogue will be searchable, and will include metadata describing the main characteristics of each data source
  - E.g. population size, demographics, type of care covered, diseases of interest covered,…

A catalogue for studies based on EU PAS Register will also be delivered - Q4 2022 (TBC)

- Useful to identify what studies have been done on a disease/product and which data sources have been used

First version of EU Data Quality Framework for big data used in the regulatory decision-making process is expected in 2022

# Coming in 2022: DARWIN EU®

## DARWIN EU is a federated network of data, expertise and services

### EU Medicines Regulatory Network

- **EMA** - provides leadership, setting standards, contracting studies, **overseeing**
- **European Medicines Regulatory Network -** including EMA scientific committees and working parties, national competent authorities (NCAs) and the European Commission: **request studies** via EMA

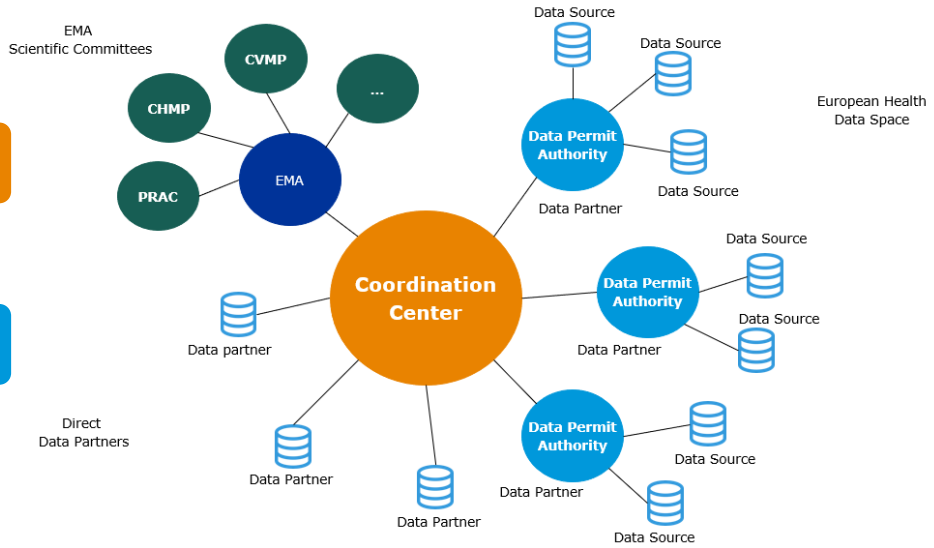
### The Coordination Centre

- **Establishes** and **maintains the network** (including onboard/maintain data sources), manage the **execution of scientific studies**

### Data Partners, incl. Data Permit Authorities

- **Partners** who have access to data, or who may request analyses in a data source and provide results to the Coordination Centre
- This includes **Data Permit Authorities** (DPAs), already existing or to be created as part for the European Health Data Space (EHDS)

Status:
- Coordination centre appointment expected early 2022
- First pilot/studies from 2022



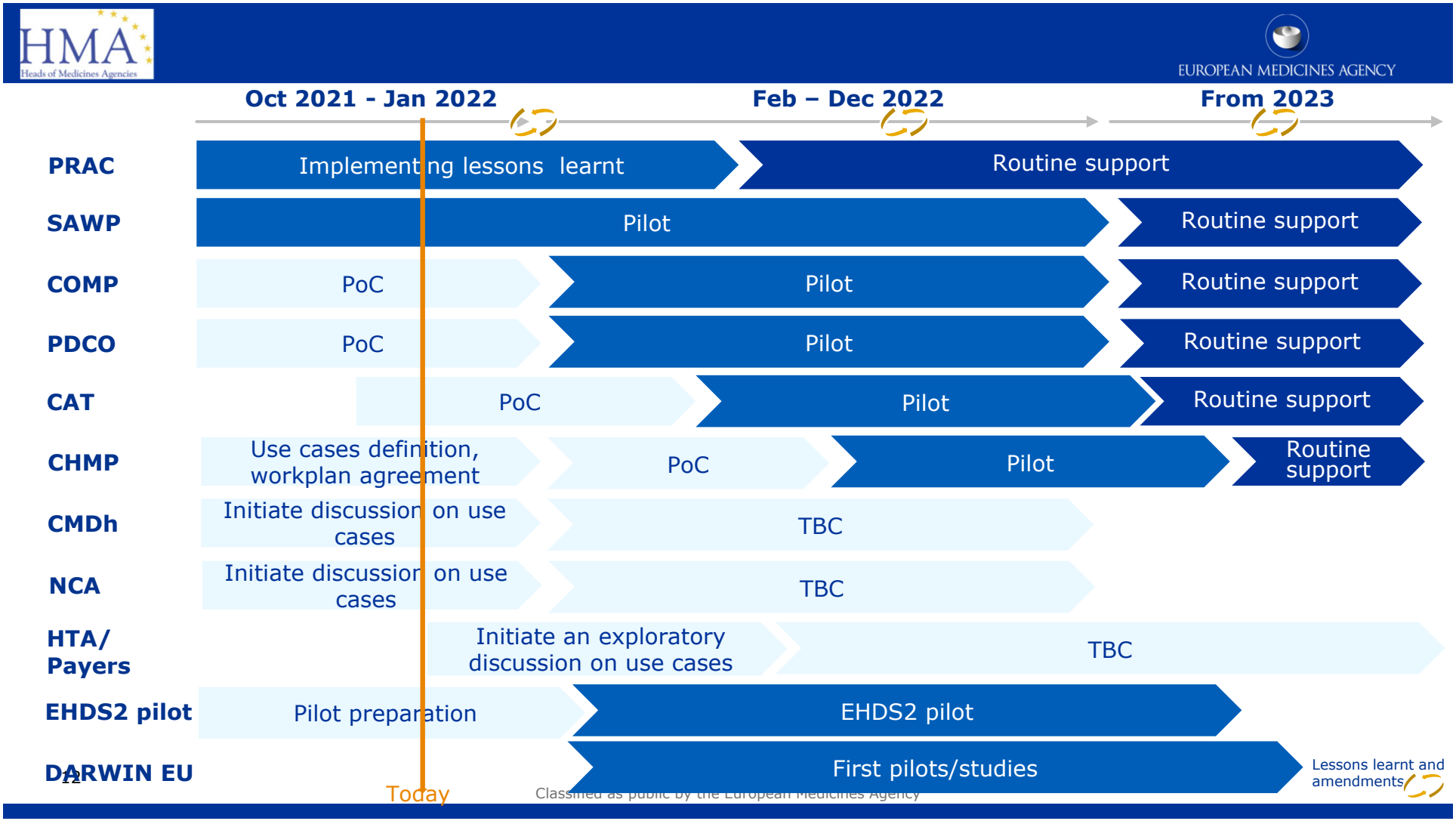
# Methodologies and capabilities

- Refine methodologies for the use of RWE:
  - Call published for the Horizon Europe Cluster 1 Health, submission from Oct. 21 to Apr 22 (HORIZON-HLTH-2022-TOOL-11-02: New methods for the effective use of real-world data and/or synthetic data in regulatory decision-making and/or in health technology assessment)
  - Evaluation of the impact of RWE in the decision-making process and to promote the provision of recommendations for best practice
- Big Data learning initiative workshop 30 November 2021:
  - track and learn from relevant Big Data applications through the product life-cycle and feed learnings to reflection papers and guidance.
- Developing training Data Science, Pharmacoepidemiology and Biostatistics
  - Curriculum agreed in 2021 and delivery and roll-out planned for 2022
- Creation of a Methodology Working Party with dedicated expertise in RWE

# Three potential use cases for the support to committees' decision-making

**From a regulatory perspective, RWE aims to support committees' decision-making in three main areas**

| | | | |
|---|---|---|---|
| **Use case objective** | **Support the planning & validity of applicant studies** | **Understand clinical context** | **Investigate associations and impact** |
| **Use case category** | Design and feasibility of planned studies | Disease epidemiology | Effectiveness and safety studies |
| | Representativeness and validity of Completed studies | Clinical management & drug utilisation | Impact of regulatory actions |



# Examples of benefits for industry

Industry will be consulted in the roll-out of RWE

Benefits of the RWE initiatives include

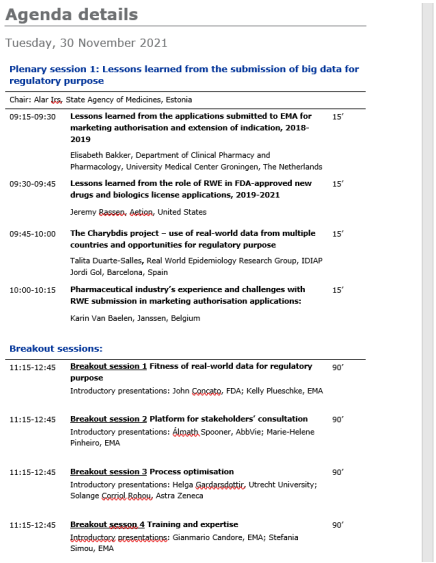
- Better evidence supports better decisions
  - Regulator advice supporting products in development
  - Regulator decision supporting authorization
  - Enabling an optimal use of the products
- Better RWE submissions, the ability to contextualise information provided, and better understanding of evidentiary value will result in a higher acceptance of RWE in marketing authorisation submissions
  - Reduce drug development costs
  - Time saved in developing new treatments
- Conduct of PASS for established, including generic, products
  - When legal obligations are placed on multiple companies having a marketing authorisation for a particular substance, studies conducted by the network could help avoiding duplication and reducing cost

# Future collaborations in 2022

| | |
|---|---|
| DARWIN EU | **Industry consulted on key aspects when needed (methods, processes and transparency)** |
| Data quality & representativeness | **Industry to be consulted on EU data quality framework (e.g. workshops participation, survey…)** |
| Data discoverability | **Industry to be consulted on metadata and catalogue of studies (e.g. workshops participation, survey…)** |
| EU Network skills | |
| EU Network processes | **Industry to be informed on RWE pilots/studies** |
| Network capability to analyse | **Industry to be informed pilot on raw data analysis** |
| Delivery of expert advice | **Industry to be informed/consulted on Registries guidance and Roadmap for RWE guidance** |
| Governance framework | **Q&A on data protection will be shared with Industry** |
| International initiatives | **Industry possible opportunities on roadmap on international collaboration on RWE** |
| EU BD stakeholder implementation forum | **Industry is invited to each Multi stakeholder forum Big Data** |
| Veterinary recommendations | |

# Learnings Initiative: deep dive on real-world evidence in regulatory decision-making – Webinar 30 November 2021

- Objectives:
  - learn from current experience of using real-world data for regulatory purpose
  - discuss important challenges related to optimal use of real-world data, including data relevance, submission processes and training needs
  - discuss means to support effective collaboration with stakeholders, including industry, the regulatory network, academia, HCPs and patients.
- Expected outcomes:
  - Recommendations to be fed back into processes, stakeholder consultations.
  - Ultimately learnings support individual product submission and guidelines.



**Agenda details**

Tuesday, 30 November 2021

**Plenary session 1: Lessons learned from the submission of big data for regulatory purpose**

Chair: Alar Irs, State Agency of Medicines, Estonia

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|---|---|---|
| 09:15-09:30 | **Lessons learned from the applications submitted to EMA for marketing authorisation and extension of indication, 2018-2019**<br>Elisabeth Bakker, Department of Clinical Pharmacy and Pharmacology, University Medical Center Groningen, The Netherlands | 15′ |
| 09:30-09:45 | **Lessons learned from the role of RWE in FDA-approved new drugs and biologics license applications, 2019-2021**<br>Jeremy Rassen, Aetion, United States | 15′ |
| 09:45-10:00 | **The Charybdis project – use of real-world data from multiple countries and opportunities for regulatory purpose**<br>Talita Duarte-Salles, Real World Epidemiology Research Group, IDIAP Jordi Gol, Barcelona, Spain | 15′ |
| 10:00-10:15 | **Pharmaceutical industry's experience and challenges with RWE submission in marketing authorisation applications:**<br>Karin Van Baelen, Janssen, Belgium | 15′ |

**Breakout sessions:**

| | | |
|---|---|---|
| 11:15-12:45 | **Breakout session 1 Fitness of real-world data for regulatory purpose**<br>Introductory presentations: John Concato, FDA; Kelly Plueschke, EMA | 90′ |
| 11:15-12:45 | **Breakout session 2 Platform for stakeholders' consultation**<br>Introductory presentations: Almath Spooner, AbbVie; Marie-Helene Pinheiro, EMA | 90′ |
| 11:15-12:45 | **Breakout session 3 Process optimisation**<br>Introductory presentations: Helga Gardarsdottir, Utrecht University; Solange Corriol Rohou, Astra Zeneca | 90′ |
| 11:15-12:45 | **Breakout session 4 Training and expertise**<br>Introductory presentations: Gianmario Candore, EMA; Stefania Simou, EMA | 90′ |
| 11:15-12:45 | **Breakout session 5 Heterogeneity of results between data sources**<br>Introductory presentation: Catherine Cohet, EMA | 90′ |

**Plenary session 2: Reports from breakout sessions**

Chair: Xavier Kurz, EMA

| | | |
|---|---|---|
| 13:45-14:10 | **Report from the breakout session 1: Fitness of real-world data for regulatory purpose** | 5′ |
| | Discussion | 20′ |
| 14:10-14:35 | **Report from the breakout session 2: Platform for stakeholders' consultation** | 5′ |
| | Discussion | 20′ |
| 14:35-15:00 | **Report from the breakout session 3: Process optimisation** | 5′ |
| | Discussion | 20′ |
| 15:00-15:25 | **Report from the breakout session 4: Training and expertise** | 5′ |
| | Discussion | 20′ |
| 15:25-15:50 | **Report from the breakout session 5: Heterogeneity of results between data sources** | 5′ |
| | Discussion | 20′ |

**Plenary session 3: Perspectives and next steps**

Chair: Jesper Kjær, Danish Medicines Agency, Denmark

| | | |
|---|---|---|
| 16:00-16:30 | **Analysis and interpretation of real-world data: a 5-year outlook**<br>Patrick Ryan, Observational Health Data Sciences and Informatics (OHDSI), United-States | 30′ |
| 16:30-16:50 | Wrap-up and next steps<br>Jesper Kjær, Danish Medicines Agency, Denmark | 20′ |
| 16:50-17:00 | **Concluding remarks**<br>Peter Arlett, EMA | 10′ |

**17:00** **End of the meeting**

# Multi stakeholder forum Big Data – 07 December 2021

- Objectives:
  - inform stakeholders on the delivery of the data pillar of the Network Strategy 2025 via the HMA-EMA Big Data Steering Group (BDSG) workplan.
  - listen to stakeholders' views and feedback.
  - discuss areas for collaboration.
- Expected outcomes:
  - recommendations to be fed back into BDSG workplan, change management and communication strategy

**Agenda details**

Tuesday, 7 December 2020

**08:30** Connection to virtual room and technical checks

**09:00** Welcome and introduction

Emer Cooke, *EMA* 5'

**09:05** Opening remarks

Karl Broich, *BfArM* 10'

Andrzej Rys, *EC* 10'

Marco Greco, *EPF* 10'

**09:35** Session 1: Report on implementation of the HMA-EMA Big Data Task Force priority recommendations

Chair: Jesper Kjær, *DKMA*

*Overview of 2021 deliverables and plan for 2022*
Peter Arlett, *EMA* 20'

*Veterinary recommendations*
Ilaria Del Seppia, *EMA* 15'

**10:10** Session 2: Big Data - enabling use and establishing value

Co-chairs: Peter Arlett, *EMA* and Jesper Kjær, *DKMA*

*DARWIN EU*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*Data quality & representativeness*
Speaker invited 5'

Panel discussion 25'
Discussants invited

**11:30** Session 2: Big Data - enabling use and establishing value (cont.)

*Data discoverability*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*Skills*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*Regulatory processes for data*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*Regulatory capability to analyse data*
Speaker invited 5'

Panel discussion 25'
Discussants invited

**13:30** Break

**14:30** Session 2: Big Data - enabling use and establishing value (cont.)

*Delivery of expert advice*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*Data governance*
Speaker invited 5'

Panel discussion 25'
Discussants invited

*International activities*
Speaker invited 5'

# Conclusion

- Progress on implementation of the Big Data recommendations continued in 2021 and plans for 2022 will enable to provide stakeholders with tangible deliverables.
- Implementation will require strong engagement and collaboration with stakeholders in 2022.

*"By delivering the vision of a regulatory system able to integrate Big Data into its assessment and decision making, we can support the development of innovated medicines, deliver life-saving treatments to patients more quickly and optimise the safe and effective use of medicines through measurement of a products performance on the market."*

Big Data Task Force final report December 2019

# Thank you

**Official address** Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
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