22 November 2021
Data Analytics and Methods Task Force

Draft agenda: Learnings Initiative for Optimal Use of Big Data for Regulatory Purpose

30 November 2021

Virtual meeting, European Medicines Agency

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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

Objectives

Regulators need to continuously assess big data in combination with novel methodologies to take decisions on individual products that need to be based on comprehensive, valid and reliable evidence. Use of big data has however introduced new challenges and uncertainties around the quality of an increasing volume of complex data, new analytical approaches, new processes and the need for additional expertise and guidance. The Big Data Task Force Report – Phase 2 emphasised that assessment of big data requires continuous optimisation of current standards and specifications to provide all stakeholders with rules and guidance supporting evidence generation.

Building upon the experience of the submission of real-world evidence for regulatory purpose and the conclusions of previous workshops on data standardisation, metadata and artificial intelligence organised by the Agency, this webinar aims to:

- 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 consultation with stakeholders, including industry, the regulatory network, academia, healthcare professionals and patients.

The webinar recommendations will be fed back into internal processes, stakeholder consultations, support for individual product submission and ultimately guidelines for applicants and the regulatory network.

Agenda details

Tuesday, 30 November 2021

08:30 Connection to virtual room and technical checks

Introduction

09:00-09:15	**Welcome to the workshop and opening remarks** Peter Arlett, EMA	*15'*

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

Chair: Alar Irs, State Agency of Medicines, Estonia

09:15-09:30	**Lessons learned from the applications submitted to EMA for marketing authorisation and extension of indication, 2018-2019** 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** 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** 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:** Karin Van Baelen, Janssen, Belgium	15'
10:15-10:45	**Q&As**	30'
10:45-11:00	**Introduction to the breakout sessions** • Overview of recommendations from previous webinars on data standardisation, artificial intelligence and metadata • Running of breakout sessions Xavier Kurz, EMA	15'
11:00-11:15	**Break and connection to break out rooms**	

Breakout sessions: *(each breakout session will start with (*a*) short presentation(s) introducing the topic and questions to be addressed)*

11:15-12:45	**<u>Breakout session 1</u> Fitness of real-world data for regulatory purpose** What information should be made available on relevance, quality and generalisability of real-world data used in regulatory submissions? Is the feasibility analysis recommended in the CHMP Guideline on registry-based studies applicable to other data sources? Introductory presentations: John Concato, FDA; Kelly Plueschke, EMA Chair: Marjon Pasmooij, CBG-MEB, The Netherlands Rapporteur: Kelly Plueschke, EMA	90'
11:15-12:45	**<u>Breakout session 2</u> Platform for stakeholders' consultation** Using examples of existing platforms for collaboration, which mechanisms or fora could be put in place to support stakeholders' consultations on content, format and processes of submission of real-world data for regulatory purposes? Introductory presentations: Elizabeth Vroom, Duchenne Parent Project; Álmath Spooner, AbbVie; Marie-Helene Pinheiro, EMA Chair: Juan Garcia, EMA Rapporteur: Carla Jonker, EMA	90'
11:15-12:45	**<u>Breakout session 3</u> Process optimisation** How to optimise real-world data related processes for regulatory applications and decision-making? When should use of RWD be discussed with regulators? Should there be minimum requirements for the submission of big data for different use cases? Introductory presentations: Helga Gardarsdottir, Utrecht University; Solange Corriol Rohou, Astra Zeneca Chair: Inka Heikkinen, EuropaBio Rapporteur: Andrej Segec, EMA	90'
11:15-12:45	**<u>Breakout session 4</u> Training and expertise** Informed by the recommendations from the Artificial Intelligence workshop on need for skills and expertise, and the Big Data Task Force to introduce a training curriculum for big data, what additional training and expertise is needed to increase skills in development of study protocols, data analysis and regulatory assessment of protocols and study reports? How to achieve this goal? Introductory presentations: Gianmario Candore, EMA; Stefania Simou, EMA Chair: Marilena Vrana, European Heart Network, Brussels, Belgium Rapporteur: Valentijn de Jong, EMA	90'

11:15-12:45	**<u>Breakout session 5</u> Heterogeneity of results between data sources** Based on the experience of multi-centre studies carried out in the COVID-19 pandemic, how should heterogeneity between real-world evidence be analysed and interpreted for regulatory submission and decision-making? Could use of metadata characterising data sources be part of the solution? Introductory presentation: Catherine Cohet, EMA Chair: Daniel Prieto-Alhambra, CSM-NDORMS, University of Oxford, United Kingdom; Erasmus University Medical Centre, Rotterdam, The Netherlands Rapporteur: Alexandra Pacurariu, EMA	90′

12:45-13:45	**Lunch break**	

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′

15:50-16:00	**Coffee break**	

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** Patrick Ryan, Observational Health Data Sciences and Informatics (OHDSI), United-States	30’
16:30-16:50	Wrap-up and next steps Jesper Kjær, Danish Medicines Agency, Denmark	20’
16:50-17:00	**Concluding remarks** Peter Arlett, EMA	10’

17:00 End of the meeting