EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Summary report of Big Data Steering Group and Industry Stakeholders meeting

30 May 2022 – co-chaired by Jesper Kjaer (DKMA) and Peter Arlett (EMA)

1. Welcome and opening remarks

The Co-chairs of Big Data Steering Group (BDSG) opened the first meeting with industry stakeholders and welcomed participants representing pharmaceutical (human and veterinary), medical devices and IT technologies industries noting that:

- This meeting was organised in the context of EMA's continuous endeavours to foster regular interactions with industry stakeholders on topics of common interest, and in this instance - Big Data.
- Such meetings are planned to be held biannually to receive industry's feedback on the BDSG workplan and its implementation activities.
- The timing of this meeting was important since the BDSG was in the process of reviewing its multi-annual workplan.
- Objectives of this meeting were to:
  - Gather industry's views on Big Data priorities and needs for the coming years in order to re-enforce exsisting Big Data recommendations actions and identify gaps for new ones;
  - Consider and feed industry suggestions received during the meeting into the BDSG workplan's 2025 finalisation.

2. Industry perspective on prioritised Big Data recommendations

Discussion on each Big Data action topic prioritised by industry comprised:

- A short recap on the status of Big Data priority recommendation given by the BDSG Co-chairs and, on the veterinary recommendation, by Ilaria Del Seppia;
- Consolidated industry perspective on priorities, needs and gaps concerning that Big Data priority recommendation, and

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- Questions and answers session to clarify points related to the Big Data priority recommendation discussed.

This section of the report focuses on capturing priorities and recommendations on Big Data actions expressed by industry.

2.1. Veterinary

Rick Clayton (AnimalhealthEurope) presented on the Veterinary Big Data strategy noting that:

- Short term focus of veterinary industry was to implement the VMP Regulation 2019/6 and, in this context, complete and optimise the implementation of the three IT systems.
- Completing this will be the first step towards embracing a new data culture, aligning data management activities and building analytical capabilities while critically assessing their added value to the veterinary sector.
- As a starting vision for veterinary Big Data:
  - The objectives have to be clear and use-case driven in terms of what benefits can be achieved for the veterinary sector given its limited resources;
  - Same basic infrastructure, common standards and principles for both veterinary and human domains should be considered. However, some adaptation will be required to cater for specific veterinary sector needs and, more importantly, to ensure cost-benefit.

The relevance of other Big Data priority recommendations actions to the veterinary sector will also need to be considered and highlighted in the new Big Data Steering Group workplan.

2.2. DARWIN EU

Almath Spooner (EFPIA) introduced industry's priorities for DARWIN EU highlighting the need to predict a study request via DARWIN EU and to provide transparency for sponsors about plans to conduct a study using DARWIN EU.

Based on these priorities, industry recommended:

- Establish a focus group to discuss the results of the RWE pilots and the transition from establishment/validation to execution of the DARWIN EU platform/network.
- Before a study is conducted, the applicant should have an opportunity to review the research question, the study design, data source and analytical methodology. This needs to be done in parallel with any ongoing assessment or procedure (MAA, variation, PIP review etc.) so that it does not delay the conclusion of that procedure.
- On study completion, the MAH or applicant should have the opportunity to review and respond to study findings without any impact on the timeline of any ongoing assessment/procedure. There should be provision for the applicant to review the study report before it is posted on public register.

2.3. Data quality and representativeness

Mareke Schoonen (EUCOPE) began industry's presentation by pointing out that data quality was highly dependent on the context and, therefore, quality requirements will vary based on a regulatory purpose that the real-world evidence intends to support.

In this regard, the industry offered following recommendations:

- Organise a workshop demonstrating the practical application of the Data Quality Framework through real-life use cases and explore what constitutes "fitness-for-purpose".
- Establish a forum for further discussion about key data quality topics requiring further attention including validation and verification of real-world data elements; approaches to data linkage across RWD sources; flexible approaches to Common Data Models; and clear mandate on the use of Artificial Intelligence to improve data quality.
- It will be critical to include data curators and providers in the discussions in order to ensure a common understanding of data quality expectations across stakeholders.
- Share insights into how data quality efforts are being integrated across different aspects of the Big Data workplan (e.g. with DARWIN EU or other aspects of pilot studies) and connected to similar global efforts.

2.4. International initiatives

Maren Koben (EuropaBio) expressed industry's appreciation for the EU regulators making significant steps towards the use of real-world data by welcoming:

- The development of the M14 General principles on plan, design, and analysis of pharmacoepidemiological studies that utilize real-world data for safety assessment of medicines as a first step towards harmonisation of real-world data studies, however, further ICH harmonisation activities are needed.
- Collaboration on RWE amongst ICMRA (International Coalition of Medicines Regulatory Authorities) members such as demonstrated during the COVID-19 pandemic.

Recommendations:

- Any future output from ICMRA should be positioned as a complement to future ICH guidance, a framework for which could be developed in an ICH Discussion Group. This could address topics such as data relevance, depth and quality as well as analytical methodology principles.
- Need for transparent communication of discussions with non-EU regulators and at international platforms such as ICMRA including publication of summaries of such interactions at EMA webpage and updates at relevant stakeholder meetings.
- Industry would also welcome transparency of non-proprietary discussions with the EMA-FDA RWE cluster and data protection application in EU Members States.
- Need for a Data Analytics software, sharing of experiences and learning in that area.

2.5. Governance framework

Giedre Kvedaraviciene (COCIR) continued building upon remarks expressed by industry earlier in the meeting, noting that:

- Promotion of co-creation and dialogue in necessary for the efficient innovation ecosystem in EU. Therefore, industry should be represented in all interactions with external stakeholders such as public sector, academia, patients and healthcare professionals.
- More transparency in all the work streams systematically will ensure all available competences are leveraged to support development of new methodologies and systems, without having to course

correct at a later timepoint. Early interaction and transparency will help the sector to move in the same direction simultaneously.

Industry recommendations related to the governance framework priority recommendation include:

- Use the established tool of focus groups to facilitate scientific and policy discourse with industry on key topics e.g., DARWIN EU, CHMP pilots on submission and analysis of Individual Patient-Level Data (IPLD).
- Improve the transparency of the BDSG with publication of agendas, minutes and key presentations/documents in addition to increasing engagement opportunities for industry including in planning of workshops and key activities.
- Establish clear pathways for interactions with the Methodologies Working Party when established and provide transparency around its establishment and operation.
- MAHs should have clear visibility of and opportunity to interact in regulator-initiated (pilot) activities related to their products.

2.6. Network capability to analyse

Karin Van Baelen (EFPIA) conveyed industry perspective on the CHMP pilots on Individual Patient-Level Data (IPLD) submission and analysis and in this respect expressed need for clarity on:

- How the IPLD analysis will be used in future to inform regulatory decision making as well as the scope of its use, and
- Use cases for secondary use of data.

Industry recommendations on the pilot included:

- Involve industry in the design and operation of the pilots in order to address challenges a *priori*;
- Have clear objectives, metrics and pre-defined measures of success;
- Focus on regulatory procedures that have already concluded with no impact on regulatory decision making;
- Post the pilot outcome on the EMA website, however without reference to specific products and with industry reviewing ahead of publication, with respect to IP/commercial confidentiality.
- Make publicly available pilot's use cases/ examples of different types of regulatory submissions supported by RWE to improve industry's understanding and learn about raw data processes

Industry also welcomed the development of an EMA reflection paper, however using it as a guideline would be premature. In this respect it was recommended considering development of a Q&A document.

2.7. Data discoverability

Virginia Acha (EuropaBio) outlined challenges faced by the industry what concerned data discoverability:

- Discoverability required coordination in view of work underway to establish the list and definitions of meta-data in Europe and similar work being explored by other organisations and other regions. International and across stakeholders (including industry) coordination on what metadata are developed and used was needed.

- Benefits of discoverability also required planning and efficiency: improved discoverability and characterisation of each data source according to FAIR principles did not address the further challenge of how to ensure resource planning and efficiency across the growing number of data sources globally. As new sources were introduced and existing sources developed, discoverability requirements are needed to manage these complexities and not add further challenges nor duplication.

Industry recommends:

- Engage industry stakeholders alongside others in the ongoing progress for data discoverability measures, promoting FAIR principles and the efforts underway to advance international coordination.
- When international alignment on core metadata and related requirements is achieved, seek to formalise these as international technical standards.
- Actively manage the risk of multiple metadata catalogues and registers of data sources globally, to avoid duplication and complexity that works against effective discoverability.
- Seek to proactively engage international regulators and data authorities to outline principles for coordination and planning in data sources and metadata catalogues to avoid inefficiencies and duplication of resources, where possible.

3. Other BDSG priority actions and horizon scanning

Concerning other BDSG priority actions industry identified the need for:

- Specific discussion on the real-world evidence use for non-prescription medicines including their use cases and data sources.
- Consideration about communication to the general public through the formal transparency measures and regulatory documents as evidence changes.

Other points beyond the BDSG workplan captured during the meeting include:

- Need for clarity on data residency;
- Emerging new data types e.g. mobile health data, pharmacogenomics and how to analyse them;
- Randomised Clinical Trials and how Big Data can help to optimise understanding of a disease burden to specific populations in the EU Member States;
- Need for clear expectations, transparency and guidance on the use of Artificial Intelligence and Deep Learning and, in particular, validity of results that are generated using these concepts.
- Use of new data sources for pregnant and lactating women. Provide scientific evidence to support guideline drafting e.g. collating what happened during pandemic in the context of pregnancy and lactation.
- Industry participation in the GravitateHealth project: findings might be useful to consider in the Big Data context.

4. Wrap-up and conclusions

The BDSG co-chairs thanked the meeting participants for their active participation and fruitful discussion and concluded the meeting by informing of next steps:

- Industry input received at this meeting will be considered by the BDSG in the new workplan discussion and the final workplan will be published on the EMA Big Data webpage in Q3 2022.
- Next meeting of the BDSG and industry stakeholders is planned for Q3 2022.