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EMA's Clinical Trials Raw Data project - Update on proof-of-concept pilot

Industry Stakeholder Platform on the Operation of the Centralised Procedure for human medicines
24 November 2022

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EMA - Data Analytics and Methods Task Force

Outline of presentation

Proof-of-concept raw data pilot update

- Background and mandate
- Timelines and scope
- Integration of pilot with assessment process
- First PoC pilot procedure

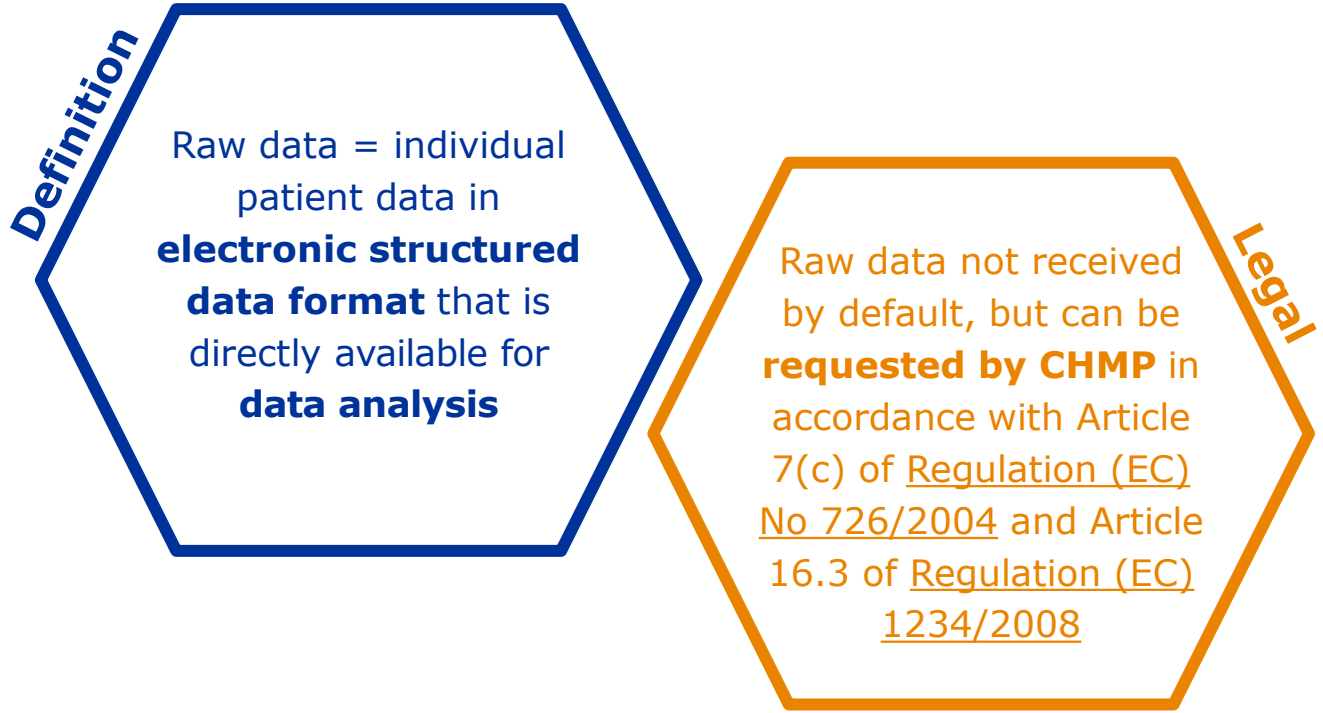
Engagement with Industry

- Industry Focus Group on Raw Data

Next steps

Definition of 'Raw Data' and current legal basis

Now: Individual patient data in PDF format already routinely submitted by companies, but format does not directly enable data analysis.



Mandate of the raw data pilot

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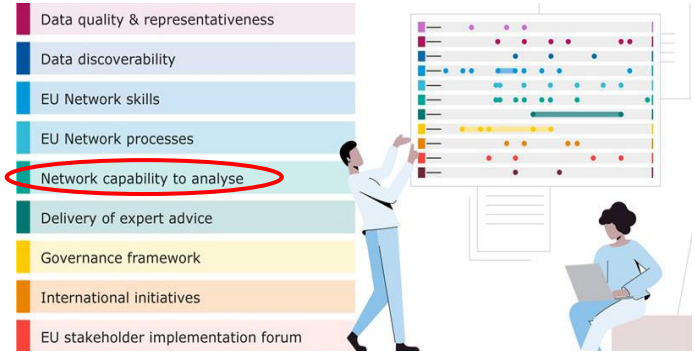
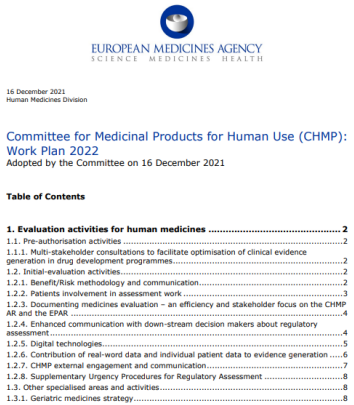
Big Data Steering Group work plans

A proof-of-concept pilot will be performed to clarify the benefits and practicalities of access to individual patient data […]. Learnings from the pilot will help the EU medicines regulatory network to make an informed decision on the place of raw data in regulatory decision-making.



CHMP work plans (2021 & 2022)

Proceed with proof-of-concept pilots of analysis and visualisation of raw data from MA dossiers to support the assessment and learn of the practicalities and benefits of such an approach.



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16 December 2021
Human Medicines Division

Committee for Medicinal Products for Human Use (CHMP): Work Plan 2022
Adopted by the Committee on 16 December 2021

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Proof-of-concept pilot – summary

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- **Timeline:** Approx. **10 regulatory procedures over two years** from September 2022 (interim report after 12 months).
- **Scope: Initial Marketing Authorisation Applications (iMAAs)** and **post-authorisation applications** (e.g., variations or extensions). No restrictions for clinical characteristics of dossier.
- **Participation:** Procedures will be selected based on **voluntary participation of CHMP Rapporteurs** and **assessment teams**. Eligible applicants or marketing authorisation holder (MAHs) will be asked to confirm their voluntary participation by signing a participation letter.
- **Data Standards:** Raw data to follow CDISC standards (SDTM, ADaM), data packages for other **international regulators** in general **accepted**
- **Usage:** Analyses that are considered relevant to the assessment will be **shared with the Applicant or MAH via the assessment report (AR)** and be used for decision-making by the CHMP. The Applicant or MAH will also be asked to **replicate these results via the LoQ/ LoOI/ RSI**.
- **Resources:** Three **resourcing scenarios for who is doing the analysis** are going to be explored: (1) the Rapporteurs' assessment team, (2) EMA or (3) EMA contractor (DKMA).

Integration of the pilot into the assessment process

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Duration of procedures will NOT change

- Regardless of the procedure (initial Marketing Authorisation Application, post-authorisation procedure), participation to the PoC pilot
  - will **not** impact the **legal time** for CHMP opinion;
  - is per se **not** expected to increase the **number of questions** to applicants or Marketing Authorisation Holders during the procedure

Communication channels will NOT change

- Communications about the procedure will **continue to be conveyed as usual** (i.e., through the existing procedural channels)
- In particular, use of pre-submission interactions, Assessment Reports, List of Questions, List of Outstanding Issues, Requests for Supplementary Information etc. as in non-PoC pilot procedures

First pilot procedure

EMA has included the first procedure in the pilot and successfully received the data

High level summary

- An Initial Marketing Authorisation Application in neurology
- Resourcing scenario (3) – EMA contractor (DKMA) will be applied
- EMA and Rapporteurs will act as joint controllers for the data

Initial learnings

- Identification of the procedure during the pre-submission interactions
- Pilot participation was proposed by the Applicant, Rapporteurs confirmed interest
- Interaction between Applicant and EMA to prepare official pilot participation
- Important aspects are published now in the Q&A for industry

Industry Focus Group on Raw Data

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-Strengthen collaboration with Industry

-Promote dialogue

-Opportunity for members to share their views on specific pilot's aspects

- Guidance for Industry
- Application of EMA's data transparency policy

Industry Focus Group on Raw Data membership

efpia

EuropaBio

medicines for europe
better access. better health.

EUCOPE

EFSPI

AESGP

Janssen

PHARMACEUTICAL COMPANIES OF Johnson & Johnson

Communication on the PoC pilot

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- Public communication via EMA's website in July 2022
  - Updated information on the pilot for Industry in October 2022
- Additional documents published
  - Questions & Answers document for applicants/MAHs (details & technical)
  - Pilot participation letter
  - Raw data submission cover letter template
  - Data Protection Notice, Records of data processing

Proof-of-concept pilot – next steps

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Additional information & next steps

- Consultation with industry via the IFG on pilot's outcomes
  - Applicants/MAHs participating in pilot will be asked for feedback on process
- Summary of PoC pilot learnings will be published after end of pilot respecting CCI
- Workshop with external stakeholders planned after end of pilot

How to take part in the raw data pilot?

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If you are interested in participating in the PoC pilot or would like more information, please contact rawdatapilot@ema.europa.eu

Any comments or questions?

TO KNOW MORE

- Priority VI in HMA-EMA Joint Big Data Taskforce Phase II report
- HMA-EMA joint Big Data Steering Group (BDSG) workplan 2022-2025
- Information about the raw data proof-of-concept pilot for industry