EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3rd EMA/HMA Big Data Stakeholder Forum 2022

01 December 2022

Session 3: Big Data – data discoverability, skills, processes and capability –
Regulatory capability to analyse data – Clinical Trial Raw Data analysis in medicine evaluation

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Outline

Proof-of-concept raw data pilot update

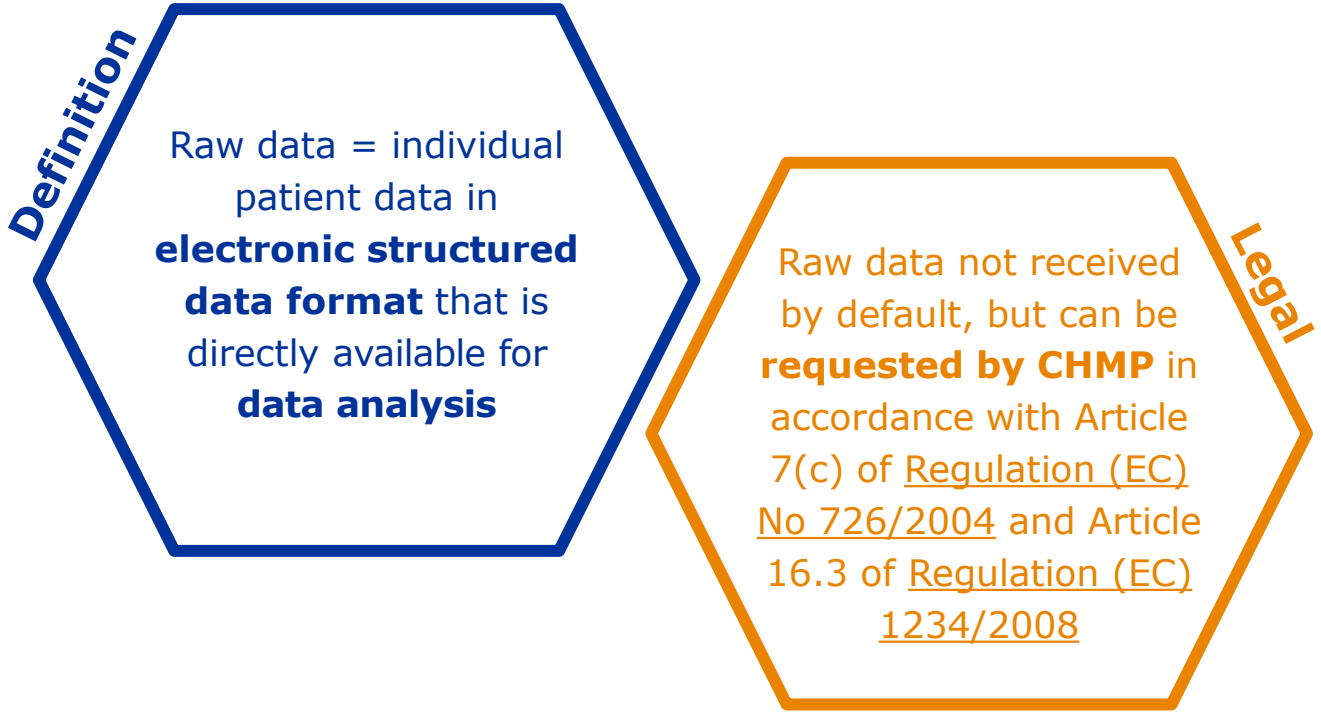
— Background and mandate
— What it entails?
— Pilot's design phase achievements
— Pilot's execution phase – selection of procedures

Next steps

— What to expect in 2023?

Definition of 'Raw Data' and current legal basis

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



Big Data Priority Recommendations

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.

Proof-of-concept pilot – what it entails?

- **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.
- **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.

PoC pilot preparatory phase – key achievements (1)

- Business consultancy support (funded by EMA)
  - **Pilot operating process (who, what, when, how)**

    Timelines, interactions, metrics for benefits realisation

  - Data protection impact assessment

- Competition for EMA contractor to support raw data analysis
  - Danish Medicines Agency (DKMA)

Design phase Q3 2021 – Q3 2022

PoC pilot preparatory phase – key achievements (2)

Design phase Q3 2021 – Q3 2022

- Stakeholder engagement
  - Network Advisory Group on Raw Data (AGRD)
  - Industry Focus Group on Raw Data (IFGRD)
  - Information sessions for assessors
- Guidance and templates
  - Internal and external guidance
- Communication planning
  - Communication plan with stakeholders
  - Identification of learning needs and proposed training approach

Stakeholders

Internal External

PoC pilot execution – selection of procedures

- Public communication via EMA's Big Data website (July 2022) marks the pilot's launch
  - Pilot's description for Industry and patients
  - Guidance for Industry
  - Data Protection documentation
  - Pilot participation documentation
- Selection of procedures – ongoing
  - Network interest
  - Industry interest

Raw Data for first pilot procedure now received

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

Execution phase Q3 2023 – Q3 2024

Stakeholders

Internal External

What's next for **2023**?

- Pilot's outcomes
  - Further consultation with Network and Industry in Q4 2022 - Q1 2023
- Use of raw data for the assessment to be informed by pilot learnings: when and how
  - Interim pilot report expected in Q3 2023
- Continue engagement with stakeholders
  - Raw Data dedicated groups (AGRD, IFGRD)
  - Presentations to WPs (e.g. PCWP/HCPWP) and Industry fora
  - Network community on Raw Data
  - Workshops/information sessions (focus on assessors)

Stakeholders

Internal External

Thank you

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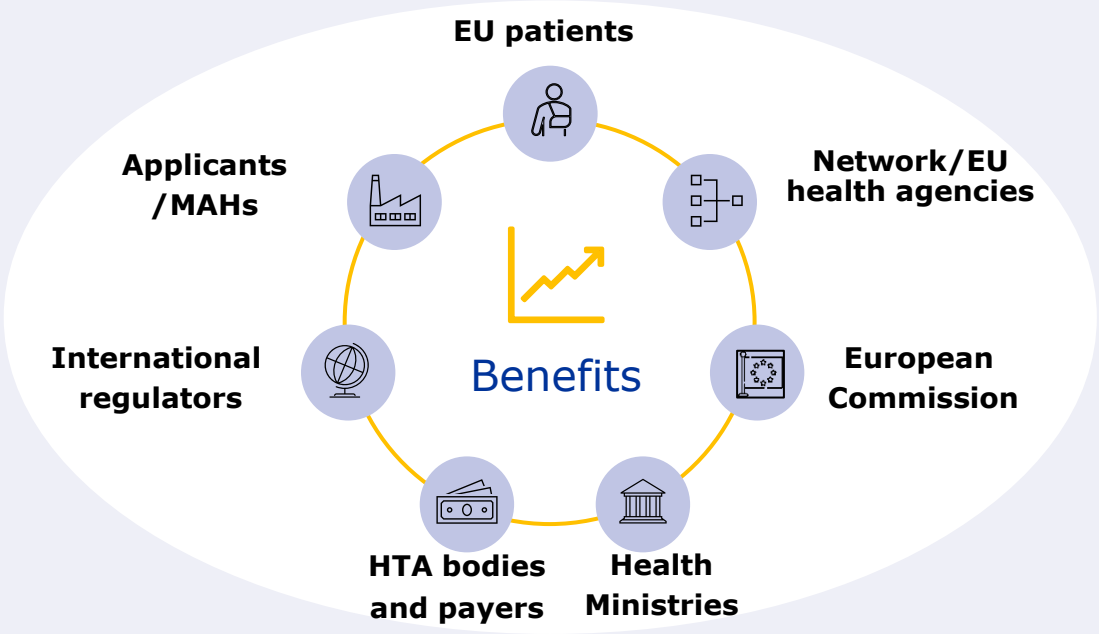
Potential benefits for various stakeholders



- **Faster access** to safe & effective medicines
- Improved **confidence** in regulatory decision-making
- Refined **product labelling**

- Better informed regulatory **decision-making**
- Improved **robustness** of assessment
- Improved identification of sites for **GCP inspection**
- Facilitation of **cross-product analyses**

- **Workload reduction**
- Earlier **authorisation**

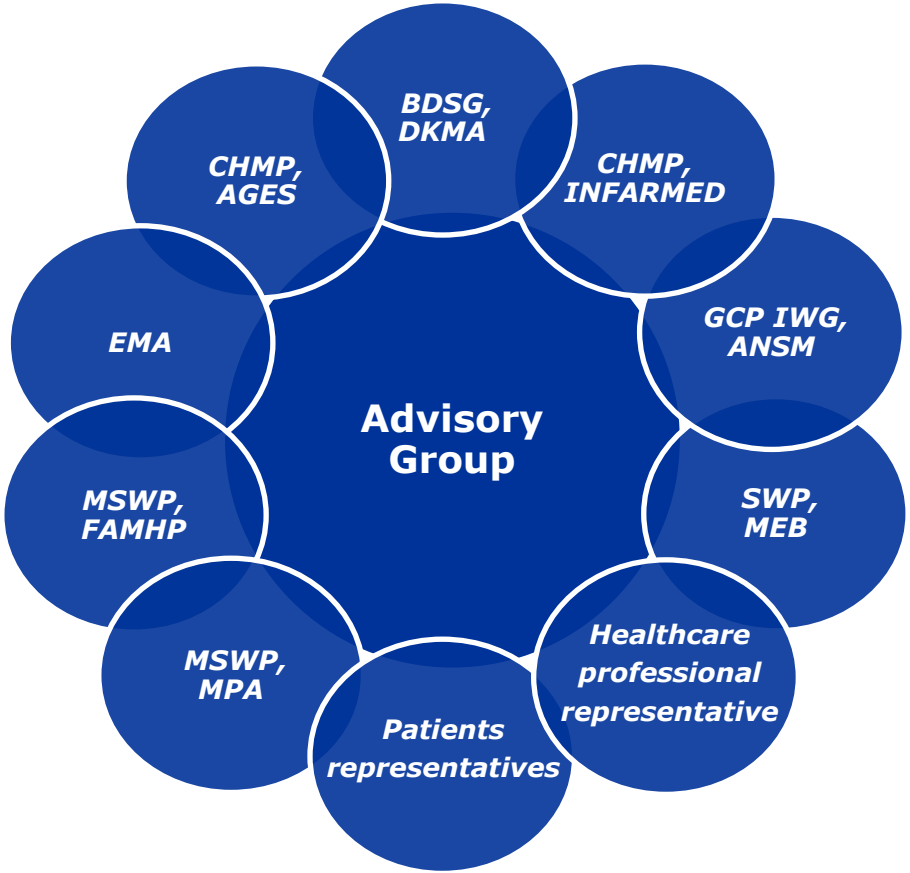
- Greater **collaboration**

- Increased support to **health policy**

- Better understanding of **drug safety profiles**
- Increased **transparency** & sharing / **understanding** of quantitative evidence

- Improved **collaboration** in response to health emergencies

Network – Advisory Group on Raw Data



New Industry Focus Group on Raw Data member

efpia

EuropaBio

medicines for europe
better access. better health.

EUCOPE

EFSPI

AESGP

Janssen

PHARMACEUTICAL COMPANIES OF Johnson & Johnson