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SCIENCE MEDICINES HEALTH

EMA's Raw Data project - Information on upcoming proof-of-concept pilot

Industry Stakeholder Platform on the Operation of the Centralised Procedure for human medicines
27 June 2022

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EMA - Data Analytics and Methods Task Force

Outline of presentation

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EMA's raw data project

— Background and mandate
— Proof-of-concept (PoC) pilot

Information on the proof-of-concept pilot

— Timelines and scope
— Integration of pilot with assessment process
— Terms of participation

Next steps

The timing is now…for raw data

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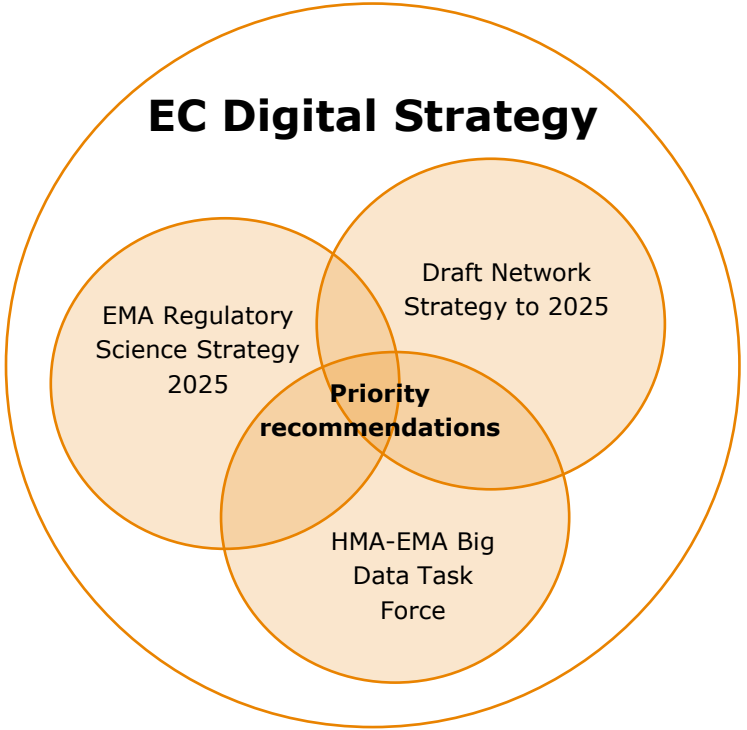
- Key initiatives referred to the Commission digital strategy **"EU health data space"** (EHDS):
  - **EU Network Strategy to 2025** (data & digital pillar)
  - **EMA Regulatory Science Strategy to 2025**
  - **Joint HMA EMA Big Data Task Force;** and
  - the resulting **Top-ten data recommendations**
- Synergic initiatives:
  - **Pharmaceutical strategy for Europe**
  - **European Health Union**
- **CHMP work plan** 2021 and 2022



Vision: innovate to turn data into decisions on medicines that create a healthier world

Big Data Task Force Priority Recommendations

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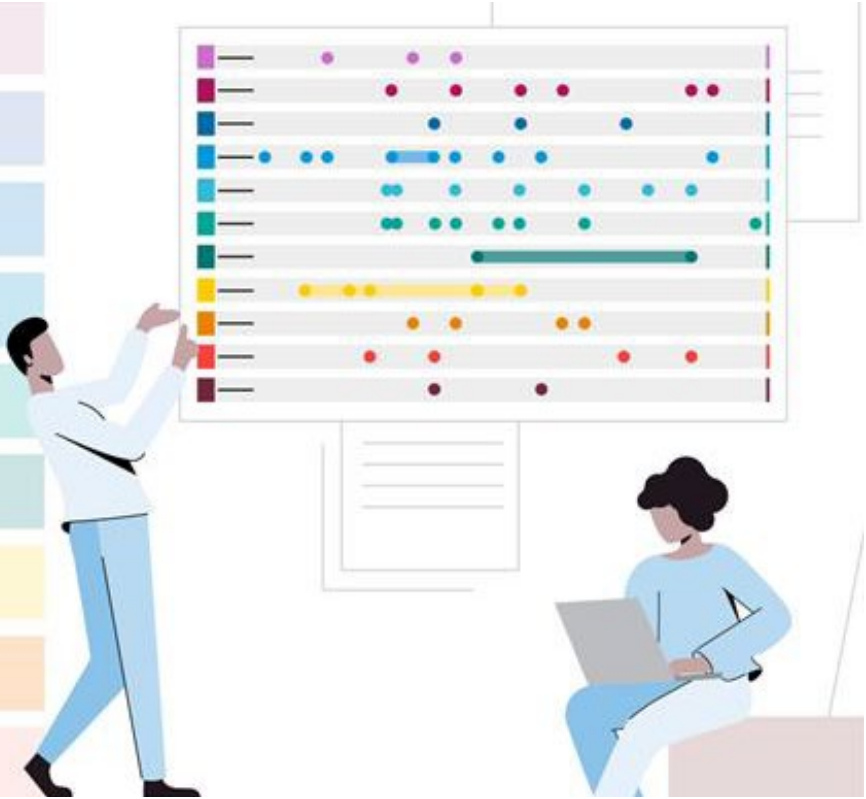
- Data quality & representativeness
- Data discoverability
- EU Network skills
- EU Network processes
- Network capability to analyse
- Delivery of expert advice
- Governance framework
- International initiatives
- EU stakeholder implementation forum



Definition of 'Raw Data'

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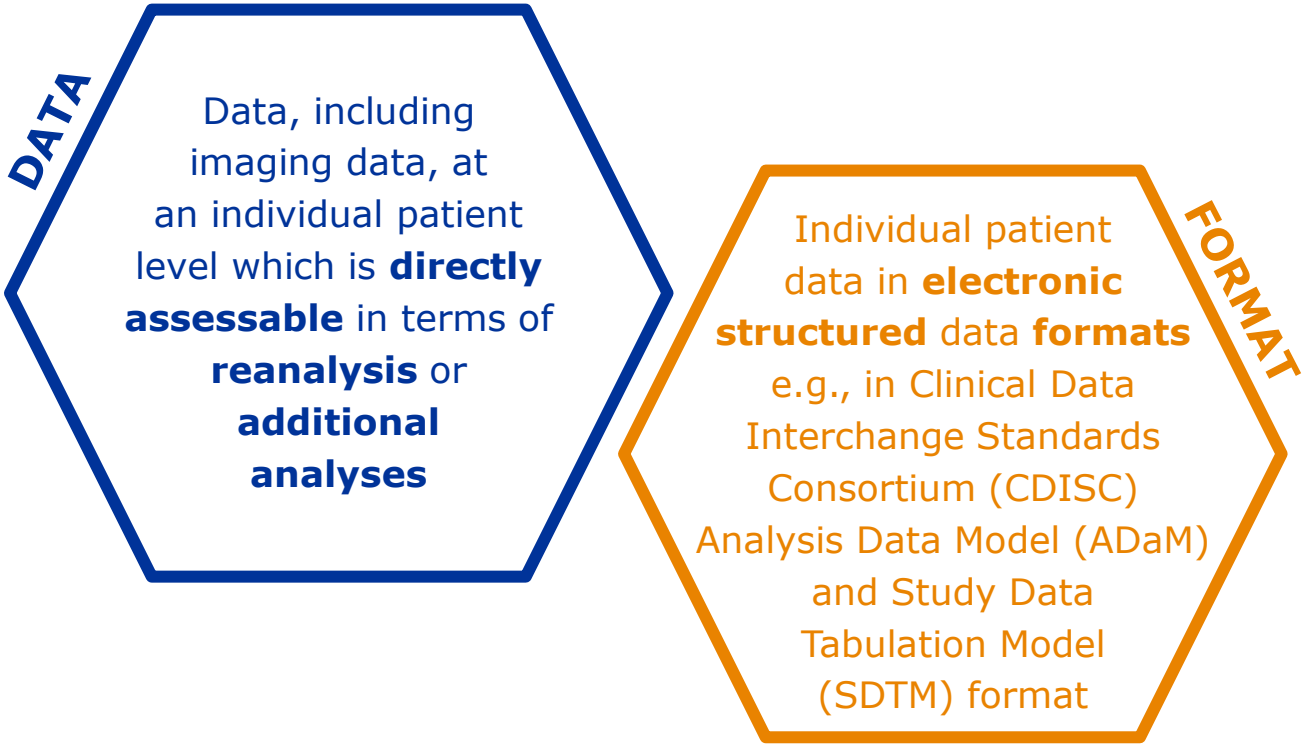
Raw data / Individual Patient Data (IPD) / Patient Level Data (PLD) / is **defined** as:



EMA's Raw Data project

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Aim

- **Determine the regulatory benefit of access to raw data** via **pilots of analysis of raw data** from clinical trials, before coming back with **recommendations to the Committee for Medicinal Products for Human Use** (CHMP).
- Ultimate aim is for **Network to understand and take informed decisions** on the place of analysis of **raw data for future regulatory submissions**.

How

- **Put in place procedures and safeguards to process clinical trial raw data**, also considering non-clinical data, in accordance with data protection legislation.
- **Establish an Advisory Group on Raw Data** identified in HMA-EMA Joint Big Data Taskforce Phase II report (multi-disciplinary group with members from CHMP, EMA Working Parties, patients' representatives)
- **Perform a proof-of-concept pilot** in order establish the value of IPD and to build, step by step, capacity to analyse raw data.
- **Foster stakeholders' engagement** through a communication plan.

Proof-of-concept pilot phases and timelines

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1 Q3 2021 – Q3 2022

Pilot design and preparation

- Consultation with various stakeholders incl. Regulatory Network and patient representatives

3 Q3 2024 (tbd)

Key decision point

- Recommendations to EMA, HMA, CHMP, BDSG on access to raw data during assessment

Execution of the pilot

- Planned **start in September 2022**; duration of up to 2 years
- Approx. 10 centralised procedures; step-wise approach
- Three resourcing scenarios
- Collect feedback from all stakeholders involved

Q3 2022 – Q3 2024 2

Scope and prioritisation for procedures for the PoC pilot

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- Centralised procedures: iMAAs and post-authorisation procedures (incl. extension applications and variations)
- No restrictions for clinical context of dossier; cover variety of procedures

- Analysis of clinical data; data standard similar to other international regulators
- Focus on benefit-risk assessment; selection of sites for GCP inspection

- Procedures at early stage, ideally after Rapporteurship assignment and before submission of dossier
- Date of submission starting September 2022

Assessment process including raw data analysis

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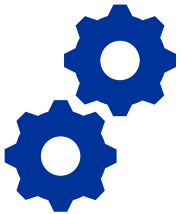
- For procedures chosen for PoC pilot, applicants/Marketing Authorisation Holders submit raw data in addition to regular dossier (how: Q&A will follow)
- During assessment, questions may arise which Rapporteurs want to answer via raw data analysis
- Raw data analysis happens during assessment phases, e.g. btw. Day 1 to Day 80 for iMAAs
- Rapporteurs include description of results in assessment report (AR)
- CHMP requests replication of analysis results from applicant e.g. via LoQ/LoOI/RSI



Key points of attention for industry on the PoC pilot

Voluntary participation by Rapporteurs & Applicant/MAH

Analyses to support assessment of dossier, no analyses across dossiers

Pilot participation will not cause delay to CHMP opinion

EMA will put in place procedures and safeguards in accordance with DP legislation (DPIA ongoing)

Transparent communication with Applicant (AR and LoQ/LoOI/RSI)

Communication on the PoC pilot

- Public communication planned for July 2022
- Additional documents that will be published before pilot starts
  - Questions & Answers document for applicants/MAHs (details & technical)
  - Data Protection Notice, Records of data processing
- Applicants/MAHs participating in pilot will be asked for feedback on process
- Summary of PoC pilot results will be published after end of pilot respecting CCI
- Workshop with external stakeholders planned after end of pilot

Upcoming proof-of-concept pilot – next steps

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Additional information & next steps

- Launching call for industry focus group that will be invited to share their views on specific PoC pilot aspects
- Possibility to present again at ISP in November 2022 as well as other fora
- For expressing interest in participating in the PoC pilot or questions please contact rawdatapilot@ema.europa.eu

Any comments or questions?

Back-up slides

Definition of 'raw data' / Individual Patient Data

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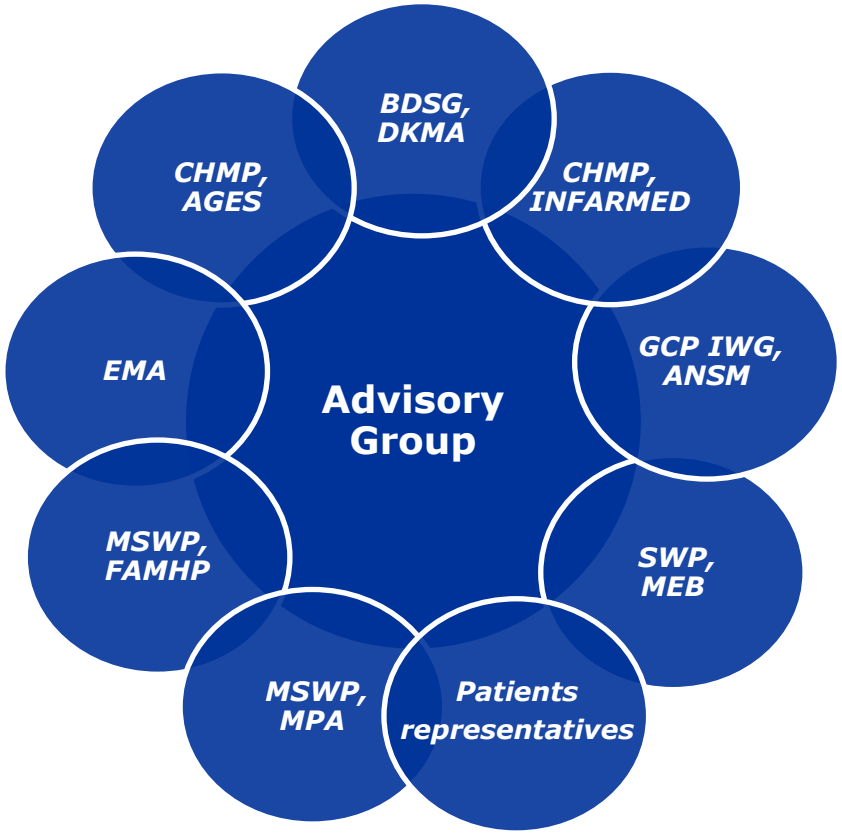
- Individual Patient Data (IPD) / raw data is **defined** as:
  - 'data, including imaging data, at an individual patient level which is directly assessable in terms of reanalysis or additional analyses'
  - 'individual patient data in electronic structured data formats, e.g. CDISC Study Data Tabulation Model (SDTM) or CDISC Analysis Data Model (ADaM)'

- Clinical trial data **already provided by marketing authorisation applicants and sponsors** in modules 4 & 5 of all MAA dossiers
  - EMA currently receives this data in the form of PDF listings; in a format that does not support data analysis
  - In contrast to PDF listings IPD / raw data is directly assessable in terms of reanalysis, additional analyses or visualisation

Network – Advisory Group on Raw Data



+ call for healthcare professional representative launched

Network – Experience from raw data pre-pilots

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- EMA performed retrospective review of previous assessment experiences with raw data, including experiences of other international regulatory agencies for whom raw data analysis forms part of the assessment process

- Until now pre-pilots by Regulatory Network (EMA- or NCA-led):
  - Link with CHMP work plan for 2021
  - Some pre-pilots supporting ongoing assessment
  - Some pre-pilots with retrospective analyses

- Now joining forces across Network for bigger proof-of-concept pilot

Raw Data submission

EFSPI/Phuse & EFPIA input for the discussion at the
8th Industry Stakeholder Platform

27. June 2022

Key points from industry - Requirements

- **Global** requirements on data structures and deliverables of raw data submission.
  - We could use and build on data standards, which are utilized by FDA, PMDA and Chinese authority. Changes should go through a global process like ICH
  - Everything else would be very costly for industry without add. benefit
- EMA to not move away from trust to check and control.
  - No required re-analysis, confirmation of correct programming etc. as this is not providing real benefit
- EMA open to discuss new ways of how do analyses, e.g. docker containers
  - This can lead to efficiency gains also in the interest of EMA

Key points from industry – Where we can help

- Industry can support pilots for fast take up of capabilities
  - Advise to start with retrospective pilots as these can be done fast and are sufficient to learn about variability of data structures across companies. Concurrent pilots should be second step
  - Help to identify a vignetta of pilots
- EFSPI, EFPIA, PHUSE can contribute to learnings defining process of raw data submission
  - Based on large experience with other health authorities
- Development of future tools and standards across industry to facilitate future raw data submission and analyses
  - Currently, industry experiments with new analysis methods (R-Shiny apps etc.) Could have strong impact on how EMA is using data
  - Other tools like submission data portal