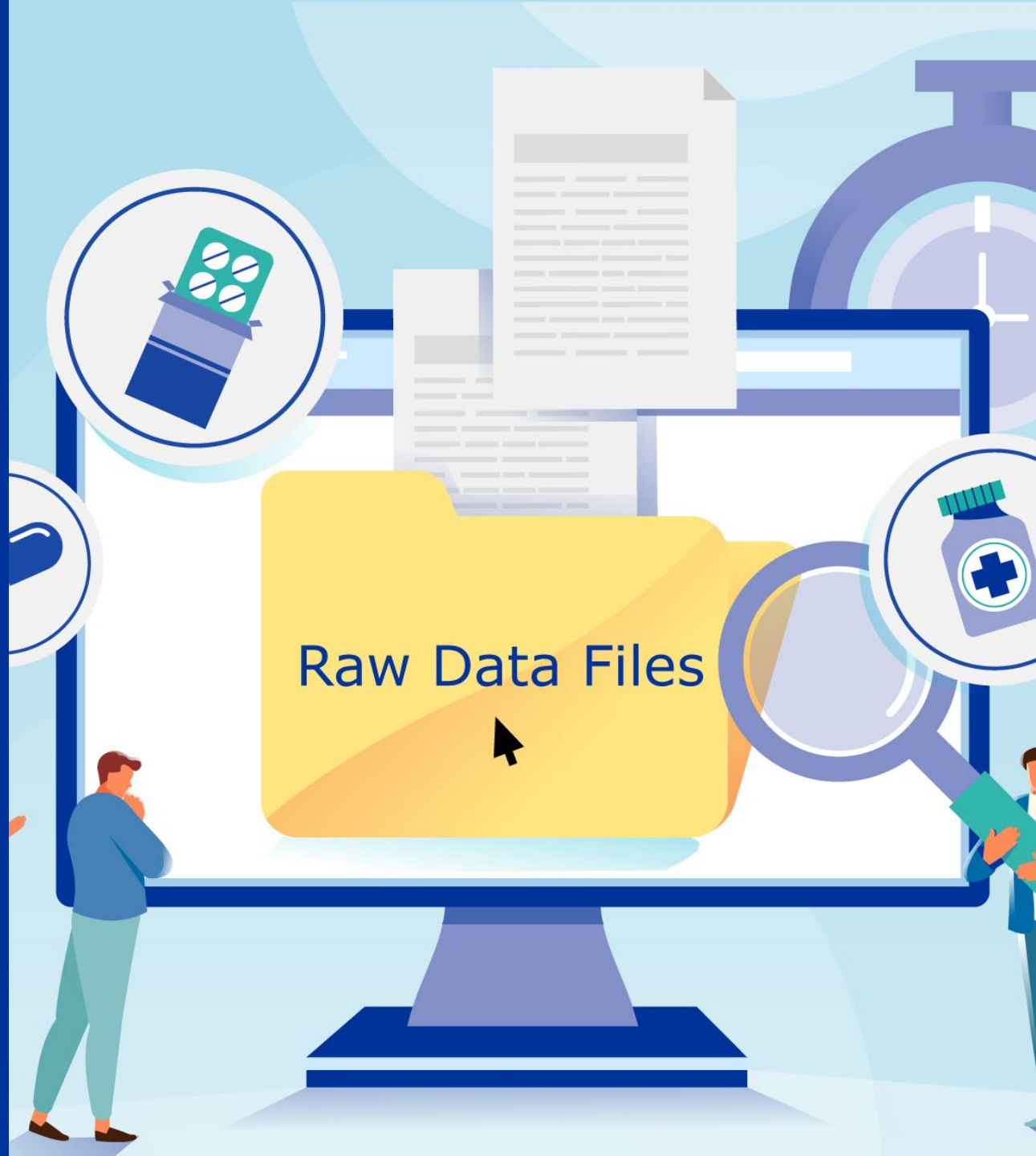
EMA
EUROPEAN MEDICINES AGENCY

Submission of clinical study data at EMA – follow up on the clinical study data pilot

Industry Stakeholder Platform on the operation of Centralised Procedure for human medicines



23 June 2025

Presented by Eftychia-Eirini Psarelli, Data Analytics and Methods Taskforce, EMA

Raw data are crucial because they provide significant additional information on clinical evidence. These datasets can be analysed to identify patterns and gain a deeper understanding, especially concerning smaller patient groups or rare conditions that may not be visible in aggregated clinical study data. The availability of raw data also potentially saves time in the regulatory process without compromising safety.

Christine Dehn, European Heart Network.

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EMA-CHMP clinical study data pilot when it was initially designed

- Timeline: Approx. **10 regulatory procedures over 2-3 years** from September 2022 (interim report after half-way point).
- Scope: **Initial marketing authorisation** and **post-authorisation applications** (e.g., variations or extensions). No restrictions for clinical characteristics of dossier.
- Participation: Procedures are based on **voluntary participation of CHMP Rapporteur teams** and **applicants/marketing authorisation holders (MAHs).**
- Usage: **Three analysis objectives** including Clinical Efficacy & Safety, Pharmacokinetic-Pharmacodynamic (PK-PD) and Good Clinical Practice (GCP) site selection.
- Resources: Three **resourcing scenarios for data analysis** are explored: (1) the Rapporteurs' assessment team, (2) EMA or (3) EMA contractor.

Procedures included in the pilot

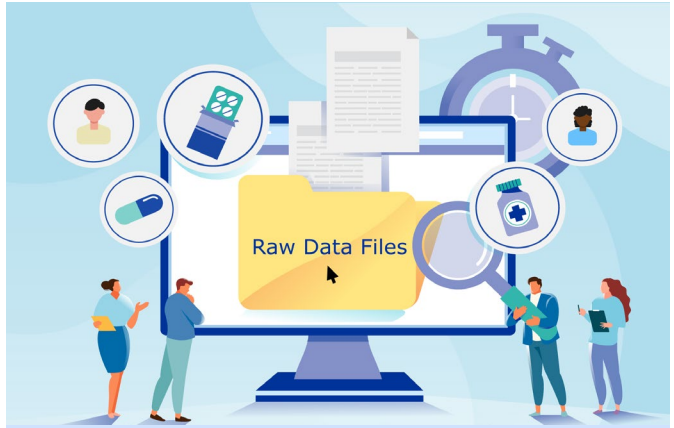
Pilot's half-way point *was reached by December 2023…*

Procedure number	Therapeutic Area	Type of procedure
1	*Neurology* 	*iMAA-Full MAA*
2	*Endocrinology* 	*iMAA-Biosimilar*
3	*Oncology* 	*Post auth. Type II variation*
4	*Dermatology* 	*Post auth. Type II variation*
5	*Gastroenterology* 	*iMAA-Full MAA*

...up to now, June 2025

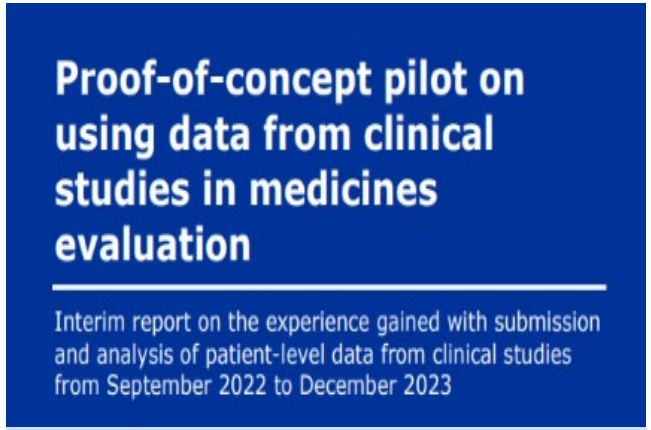
Procedure number	Therapeutic Area	Type of procedure
6	*Oncology* 	*iMAA-Biosimilar*
7	*Gastroenterology* 	*iMAA-Full MAA*
8	*Oncology* 	*iMAA-Full MAA*
9	*Immunology* 	*iMAA-Full MAA*
10	*Oncology* 	*iMAA-Full MAA*

Anticipated benefits and learnings made so far…



Anticipated benefits

- Improved understanding of clinical study results to inform regulatory decision making
- Less questions to applicants
- Optimised use of inspections



Proof-of-concept pilot

- Explore operating model, capacity & capability and technical requirements within EMRN
- 10 procedures included
- Interim pilot **learnings align with anticipated benefits**

Clinical study data pilot phases and timelines

Pilot's initial timeline

September 2022

Start of the pilot

Q3-Q4 2024

Interim pilot report

Q4 2025

Final pilot report

Pilot extension

New

2025 onwards

Pilot extension and preparatory work for potential implementation

New

Q4 2026

Follow-up pilot report

2028+

New EU Pharmaceutical Legislation comes into force (estimate)

Pilot’s extension focus areas

Intensify exploration of systematic use of clinical study data in support of regulatory assessment and decision-making

- Extent of **data interrogation** will vary – e.g. depending on dossier's quality
- Extent of **statistical analysis** will vary - e.g. for selected complex dossiers, or dossiers where applicant/MAH is not cooperating

Explore **IT solutions** in 2025/2026 (building on existing platforms):

- Clinical study data **receipt, storage and analytics infrastructure** for EMRN
- **Automated processes** for systematic data package characterisation incl. data validation

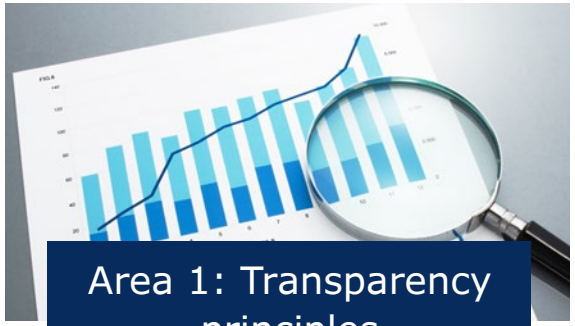
Change management activities to intensify:

- Awareness of stakeholders and foster **knowledge sharing** (leverage EMRN fora, workshops)
- Production/update of **process and data guidance**
- **Training**

Industry Fous Group on Raw Data – est. 2022

- Intensify collaboration with Industry
- Promote dialogue
- Opportunity for members to share their views on specific pilot's aspects
- Guidance for Industry
- Application of EMA's transparency policy
- **Second pilot's phase and areas for collaboration**

Proposed next steps to support collaboration between EMA/EMRN and Industry



Area 1: Transparency principles

Area 2: Standardised data analyses and visualisations

Area 3: IT/technical development including validation

Area 4: Data standards and submission requirements

Group's current membership

Group's rebranding

- Group's name to change into '***Industry group focused on clinical study data***'
- Broaden current membership → call to be launched for ***technical profiles***
- Explore creation of subgroups to support the four collaboration areas

Take home messages

- EMA-CHMP clinical study data pilot extended until further notice
  - Open to expressions of interest via rawdatapilot@ema.europa.eu

- Interaction with Industry to intensify
  - New calls for technical profiles to be launched in autumn 2025 via Industry group focusing on clinical study data
  - Feedback collection from pilot participants to continue
  - Calls for Industry subject matter experts to be launched in 2026 to support with IT development/epics (via Network Portfolio Board)

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Thank you

rawdatapilot@ema.europa.eu

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