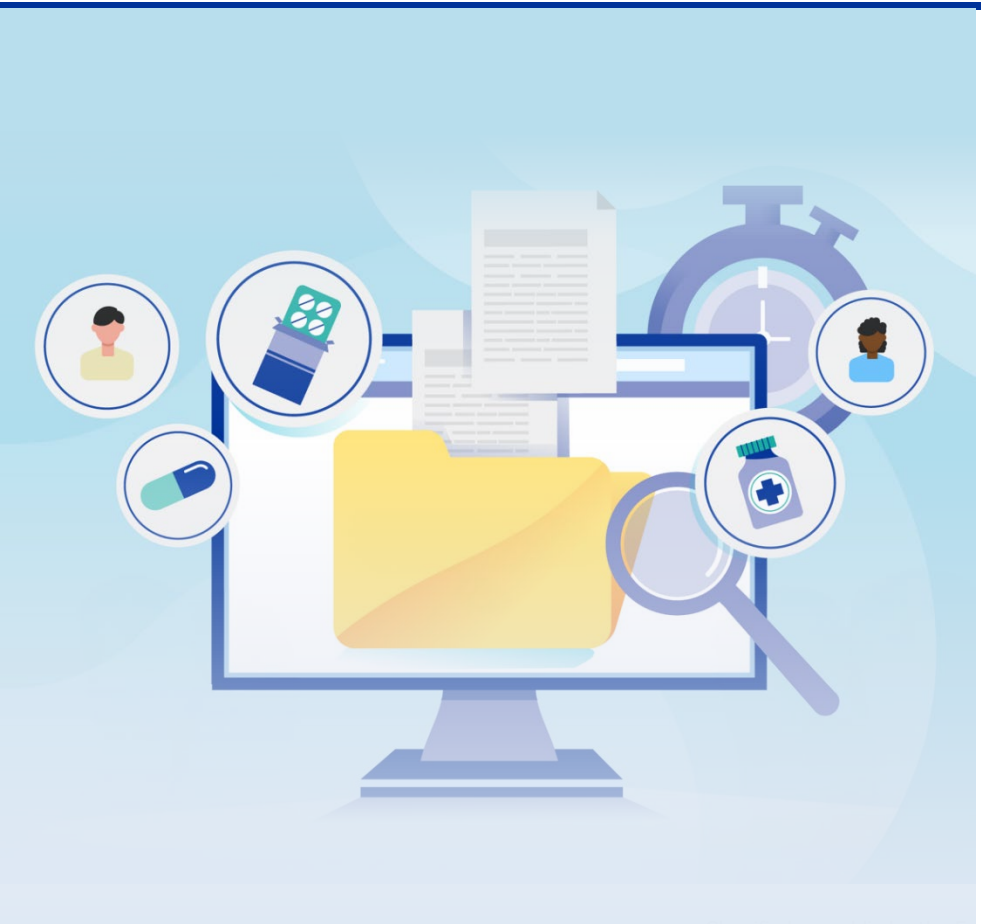
Clinical study data pilot – interim results and learnings

28 November 2024

Session 2 of the HMA/EMA Big Data Stakeholder Forum: Evidence generation to advance regulatory excellence, here and now

Presented by Florian Klinglmueller, Austrian Agency for Health and Food Safety
MWP, ETF, HMA-EMA Big Data Steering Group member



Content

1. **Pilot status update**
2. **Interim learnings**
3. **Recommendations and next steps**

Expected benefits of clinical study data access and analysis

EU patients & HCPs

- Faster access to innovative, safe and effective medicines
- Enhanced confidence in regulatory decision-making
- Refined product labelling/ targeting of subgroups within the recommended indications
- Facilitation of cross-product analyses

European Medicines Regulatory Network

- Enhanced understanding of clinical study results to inform regulatory decision making
- Fewer questions of data interpretation to the applicant/marketing authorisation holder (MAH)
- Facilitation of cross-product analyses
- Optimised use of inspection resources

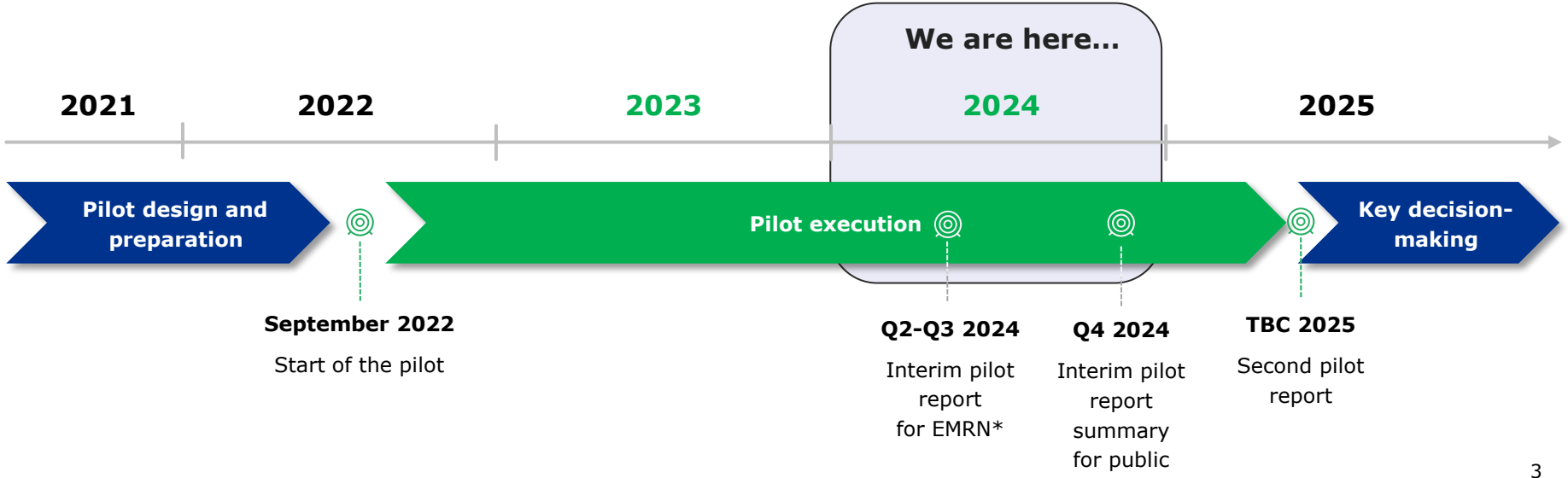
Applicants/MAHs

- Workload reduction due to fewer complex questions
- Shorter clock-stops
- Earlier authorisation

Clinical study data pilot phases and key timelines *(initial plan)*

Purpose of pilot

Determine the **benefits of early clinical study data access** (at time of submission) and analysis to support the scientific assessment of medicinal products; identify the **target operating model**, **capacity and capability requirements**, and **technical requirements** for receiving, validating, storing, managing and analysing clinical study data.



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, November 2024

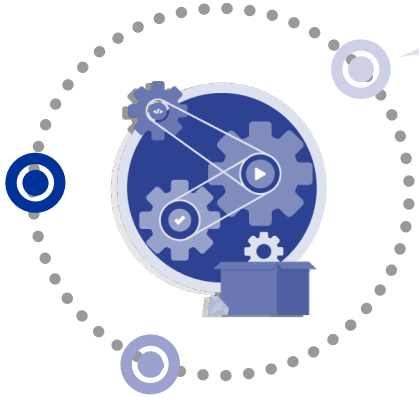
Procedure number	Therapeutic Area	Type of procedure
6	*Oncology* 	*iMAA-Biosimilar*
7	*Gastroenterology* 	*iMAA-Full MAA*
8	*Oncology* 	*iMAA-Full MAA*
9	*Oncology* 	*iMAA-Full MAA*

Interim pilot report

In **December 2023**, the cross-Agency's clinical study data pilot team conducted **surveys** to gather feedback from all pilot participants (e.g. Rapporteur teams, applicants/MAHs, etc.)

4 AREAS OF FEEDBACK

- Added value for assessment and decision making
- Capacity and capability
- Governance and processes
- Technical aspects



>60% RESPONSE RATE

60 different PARTICIPANTS

- CHMP Rapporteur/ Co-rapporteur
- Data analyst
- Rapporteur/Co-Rapporteur assessment team
- NCA staff involved in provision of member state comments informed by clinical study data analysis
- EMA pilot procedure coordinator
- EMA product lead
- EMA GCP officer
- GCP inspectors
- Applicant/MAH

Key preliminary learnings

Added value for assessment and decision making

- **Fewer questions to the applicant/MAH;** resolved with clinical study data analysis [potential to reduce overall assessment time]
- Improved **understanding of the information submitted** in the MAA dossier [potential for better opinion on indications and warnings]
- **Consensus** on methodological issues **amongst Rapporteurs** [potential to reduce outstanding issues during decision-making discussion]
- Potential to **optimise** the use of limited **inspection resources** [shorter time needed to plan and conduct inspections]

Capacity and capability

- **Additional EMRN expertise needed** in the field of **statistical programming, PK-PD modelling, biostatistics**, and clinical trial data standards [training]
- Conduct of tasks on clinical study data still **allowed assessment to be performed** according to timelines

Governance and processes

- **Most resourcing scenarios** tested successfully for analyses supporting the clinical efficacy and GCP routine inspection
- **Non-Rapporteur NCAs appear engaged,** with provision of Member State comments based on clinical study data analyses
- **Data package requirements** by other international regulators deemed suitable [no additional work for applicants]

Technical aspects

- **Data receipt, storage and analytics infrastructure** for EMRN will require optimisation to upscale
- **Choice of software under investigation** for all areas: clinical efficacy, PK-PD modelling, GCP [established off-the-shelf options available]

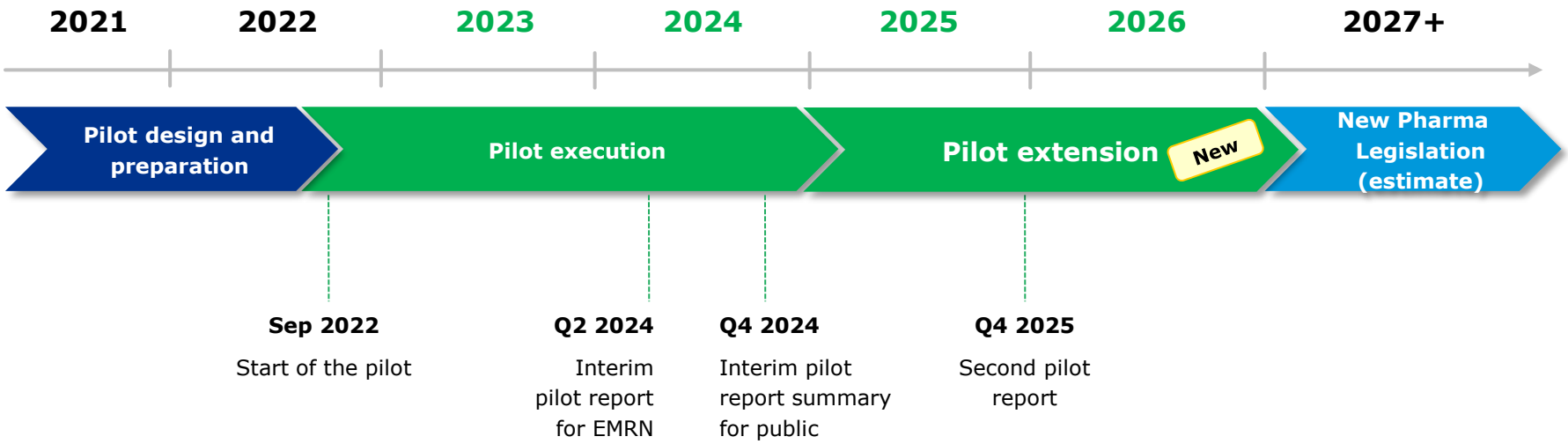
MWP ESEC Biostatistics SIA – Feedback

- Clinical trial data provide complementary approach to assessment
- Risks of data-driven assessment, how to remain neutral
- Diverse infrastructure solutions across EMRN with varying requirements
- Software maturity (e.g. to implement state-of-the-art analysis methods)
- Questions that can be resolved by analyses, may not be those that block approval
- Need to limit shift of workload from Applicant to Assessors
- Desire to collaborate and share (e.g. tools, templates, best practices)

Analyses should not be conducted routinely, but can eliminate questions, help phrase questions more precisely, improve learning and understanding

Extension of pilot

New



Selected recommendations for the second part of the pilot

Added value for assessment and decision making

- Continue **enrolling procedures for the pilot**

Capability and capacity

- Continue **raising awareness of stakeholders** regarding use of clinical study data analysis in regulatory decision-making by providing regular updates to the European Medicines Regulatory Network and public fora

Governance and processes

- **Update guidance to applicants/MAHs** for the second phase of the pilot, e.g. change in timelines, modes of participation
- **EMA to carry out analyses** in support of benefit-risk assessment

Technical aspects

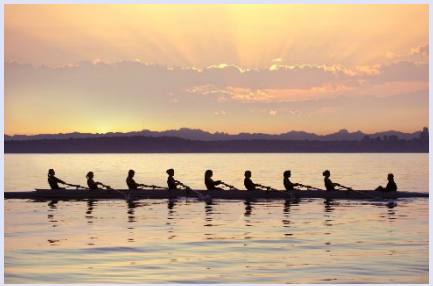
- Identify necessary upgrades of systems and process to **optimise applicants'/MAHs' eSubmission process**, e.g. the inclusion of the clinical study data package in eCTD structure
- **Strengthen exchange** with other international regulators on activities related to **data standardisation** and data governance, fostering **alignment regarding data submission requirements** and **electronic submission plans**

Discussion points

- **Interim pilot report** and updated pilot **guidance** available at EMA's Big Data webpage
- Open to receiving **new pilot proposals** from applicants/MAHs

  Please reach out to rawdatapilot@ema.europa.eu

- **Reflections** on interim learnings and recommendations
- Areas for **collaboration**
  - Data submission requirements
  - Guidance and training
  - Use of open-source software



Thank you for listening

Further information

See websites for contact details

Heads of Medicines Agencies www.hma.eu
European Medicines Agency www.ema.europa.eu

The European Medicines Agency is an agency of the European Union

Stay up to date

- Big Data webpage
- Big Data Highlights - **resubscribe** here
- EMA events
- Social media **@EMA_News**

Back-up slides

Network priority: clarify benefit of access to 'raw data'

Key objective

Explore the analysis of raw data from Marketing Authorisation (MA) dossiers to the assessment of initial marketing authorisation applications and selected post-authorisation procedures.

Activities in 2024

- Continue the proof-of-concept pilots of analysis and visualisation of raw data from MA dossiers to learn of the practicalities and benefits of such an approach
- Expand Network Community on Raw Data to regularly share developments on the raw data proof of-concept pilots and foster close collaboration across the Network into using raw data for regulatory decision-making
- Produce the proof-of-concept pilots' interim report to reflect on the pilot conduct

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

14 December 2023
EMA/573939/2023
Human Medicines Division

Committee for Medicinal Products for Human Use (CHMP): Work Plan 2024
Adopted by the Committee on 14 December 2023

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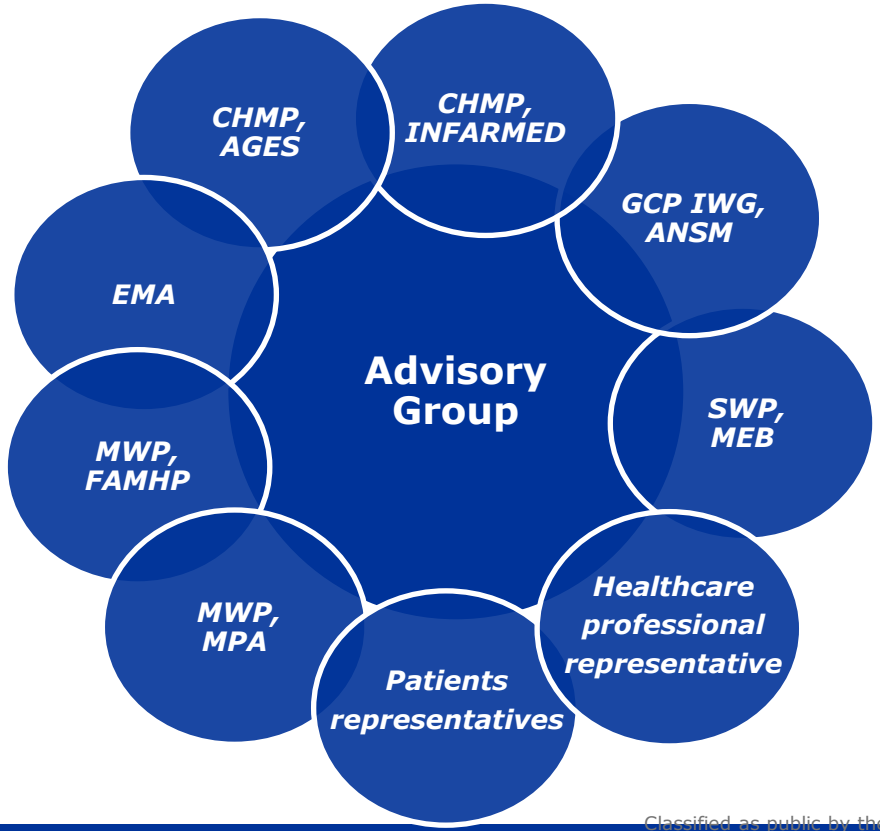
Network priority: clarify benefit of access to 'raw data'

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.

Network – Advisory Group on Raw Data



Industry Focus 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 data transparency policy
- Second pilot's phase and areas for collaboration

Industry Focus Group on Raw Data membership

efpia

EuropaBio

medicines for europe
better access. better health.

EUCOPE

EFSPI

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