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SCIENCE MEDICINES HEALTH

Update on Transparency Aspects of the Raw Data Pilot & CTIS Guidance

Plenary Session with updates from Health Authorities

PHUSE/FDA CSS 2023 – 20 September 2023

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Head of Documents access and Publication Department

An agency of the European Union

Mandate of the Raw Data pilot

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EMA MWP 3-year workplan (2022-2024)

To deliver an improved access to raw data (e.g. clinical or pharmacometrics), it is proposed to actively engage with the Network Advisory Group on Raw Data with members across committees and working parties to examine the practical aspects of patient level data visualisation and analysis, with an initial focus on clinical trial data.

HMA/EMA Big Data Steering Group work plan (2023-2025)

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 workplan (2023)

Proceed with 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.

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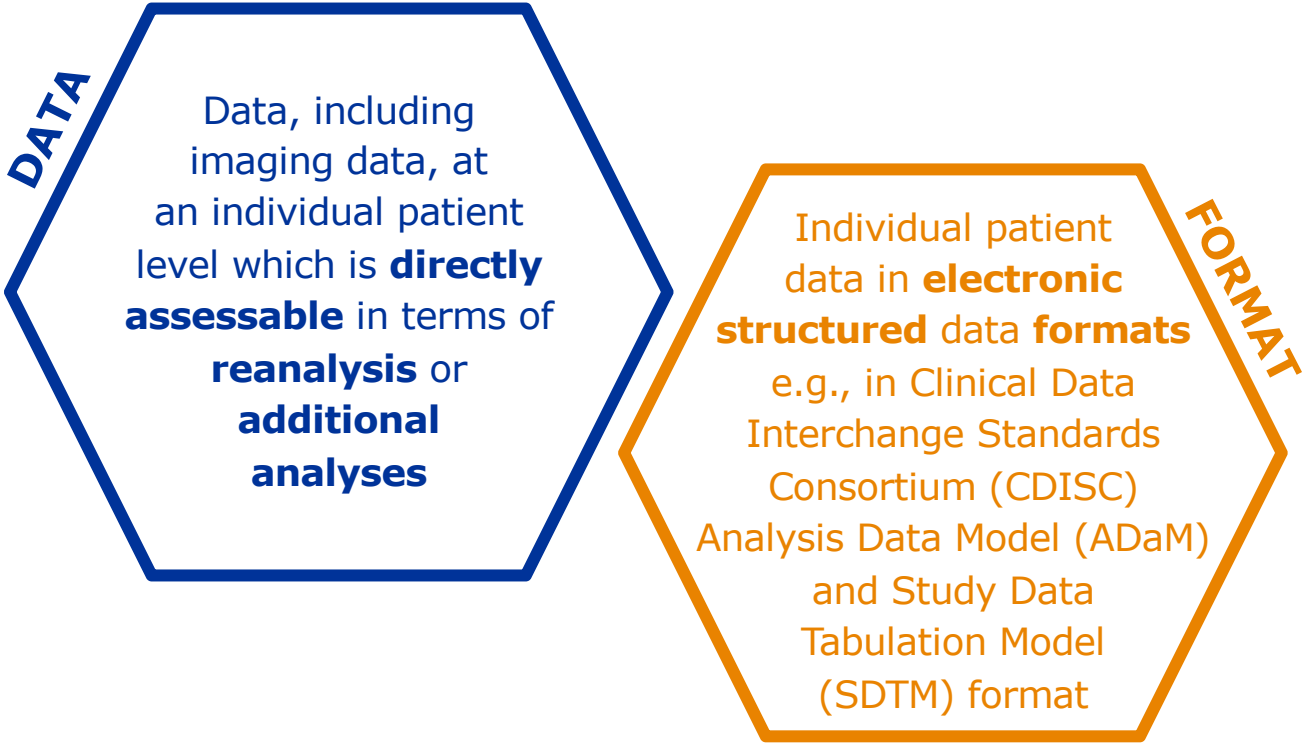
Definitions

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



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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,** in accordance with data protection legislation.
- **Perform a proof-of-concept pilot** in order establish the value of IPD and to build, step by step, capacity to analyse raw data.

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PoC pilot status update

5
Included in the pilot

1. iMAA (new active substance) - neurology
2. iMAA (biosimilar) – endocrinology
3. Type II variation – oncology
4. Type II variation – dermatology
5. iMAA (new active substance) – gastroenterology

Focus on **benefit-risk** incl. modelling and simulation (M&S) analysis

Excellent collaboration with industry for included pilot procedures

- Interaction between Applicant and EMA to prepare official pilot participation
- Identification of the procedure during the pre-submission interactions

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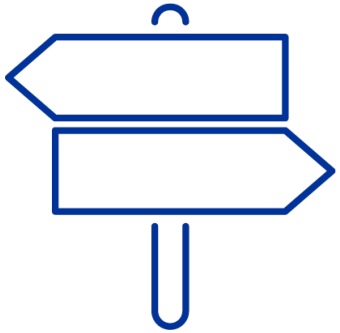
Important upcoming milestones

- **Interim pilot report expected by early 2024**
  - Interim learnings on **benefit to regulatory assessment and practicalities** (operating model, capacity & capability, technical demands)
  - **Feedback** to be sought from all stakeholders involved, including **Applicants/MAHs via surveys**
  - Report's **summary to be published on EMA's website** (bearing in mind that most pilot procedures will have ongoing assessments)



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Raw Data pilot and transparency principles

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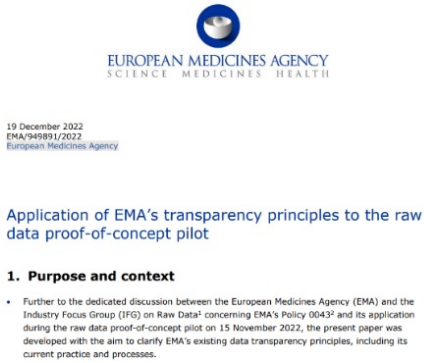
- **Policy 0043**: Access to documents Regulation 1049/2001 applies to RW PoC pilot
  - Access refused while regulatory procedure is ongoing
  - Third party will be consulted if data are requested
  - Commercially confidential information, if any, will be redacted
  - Data will be anonymised according to EUDPR



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19 December 2022
EMA/949891/2022
European Medicines Agency

Application of EMA's transparency principles to the raw data proof-of-concept pilot

1. Purpose and context

- Further to the dedicated discussion between the European Medicines Agency (EMA) and the Industry Focus Group (IFG) on Raw Data[1] concerning EMA's Policy 0043[2] and its application during the raw data proof-of-concept pilot on 15 November 2022, the present paper was developed with the aim to clarify EMA's existing data transparency principles, including its current practice and processes.

- **Policy 0070**: Individual patient data are part of phase 1 of Clinical Data Publication

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Clinical Trials Regulation

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Transparency is a pillar of the CT Regulation,
& is delivered through the searchable Clinical Trial Information System (**CTIS**) website*

Article 81(4) of Regulation (EU) No. 536/2014

EU database publicly accessible by default, with exceptions justified on any of the following grounds:

– Protection of personal data (PPD);

– Protection of commercially confidential information (CCI) in particular taking into account the MA status of the medicinal product, unless there is an overriding public interest in disclosure;

– Protecting confidential communication between MS in relation to the preparation of the assessment report;

– Ensuring effective supervision of the conduct of a clinical trial by MSs

* https://www.ema.europa.eu/en/human-regulatory/research-development/clinical-trials/clinical-trials-information-system

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Public consultation on Transparency rules

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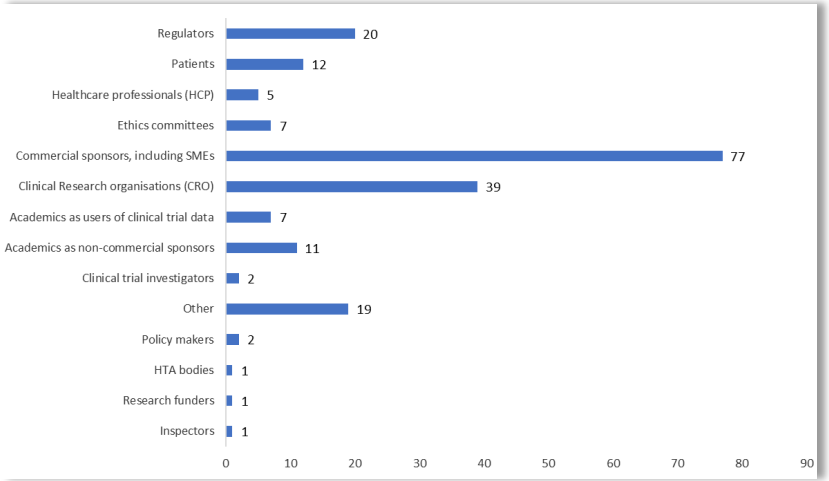
- Public consultation on CTIS Transparency rules from 3 May to 28 June 2023 to gather feedback from CTIS stakeholder community
- Affiliation (pre-defined options)



- Publication of structured data and documents considered critically important for publication
- Deferral functionalities in CTIS
- Redaction to protect PD and CCI

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Most important for publication

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Clinical trial design information, details on trial sponsors, details of the medicinal products used

Trial and recruitment status

Temporary halt, including safety related, urgent safety restrictions, serious breaches

Summary of results, Clinical study results

Protocol, subject information and informed consent form, recruitment arrangements

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Proposal for new transparency rules (1/2)

- Category 2 and 3 trials (vast majority of all trials)

  ↳ Publication of documents at the time of the first Member State decision* for:

  - ❑ Protocol, including synopsis and patients facing documents
  - ❑ Informed consent form and patient information sheet
  - ❑ Summary of medicinal product characteristics (SMPC)
  - ❑ Recruitment arrangements, advertising material

  ↳ Publication upon submission in CTIS for:

  - ❑ Final summary of results and layperson summary *(expected to be within 12 months from the end of trial in EU/EEA)*

*Authorised, authorised with conditions, not authorised

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Proposal for new transparency rules (2/2)

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- Category 1 trials in paediatric/part of PIP*:

  ↳ Publication of protocol and summary of results as soon as results are submitted in CTIS (expected within 6 months from the end of trial in EU/EEA)

- Category 1 trials in adults:

  ↳ Publication of protocol and summary of results 30 months after the end of trial in EU/EEA (submitted in CTIS secure domain 12 months after end of trial in the EU/EEA)

- Clinical Study Reports (CSR) submitted in a marketing authorisation application,

  ↳ Publication as soon as the CSR is submitted in CTIS (expected within 30 days from the end of the marketing authorisation procedure)

* Paediatric Investigational Plan

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Benefits for stakeholders

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Patients

- Access key data and documents as early as possible in the clinical trial lifecycle
- Identify published information of interest more easily
- Earlier access to information for patients will facilitate the start of trial and enrolment

Health Care Professionals

- Increase user friendliness in accessing clinical trials information
- Increased awareness on possible treatment options

Sponsors

- Reduced complexity and faster preparation of CTA dossier
- Process simplification and reduction the risk of inadvertent publication of CCI
- Simpler and earlier publication increases patients' engagement and trust

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Next steps

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01

Revised rules for endorsement by EMA MB

5 October 2023

02

Implementing the new rules in CTIS public portal

Q4 2023 – Q2 2024

03

Launch of revamped CTIS public portal & application of new transparency rules

2024

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Any questions?

Further information

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