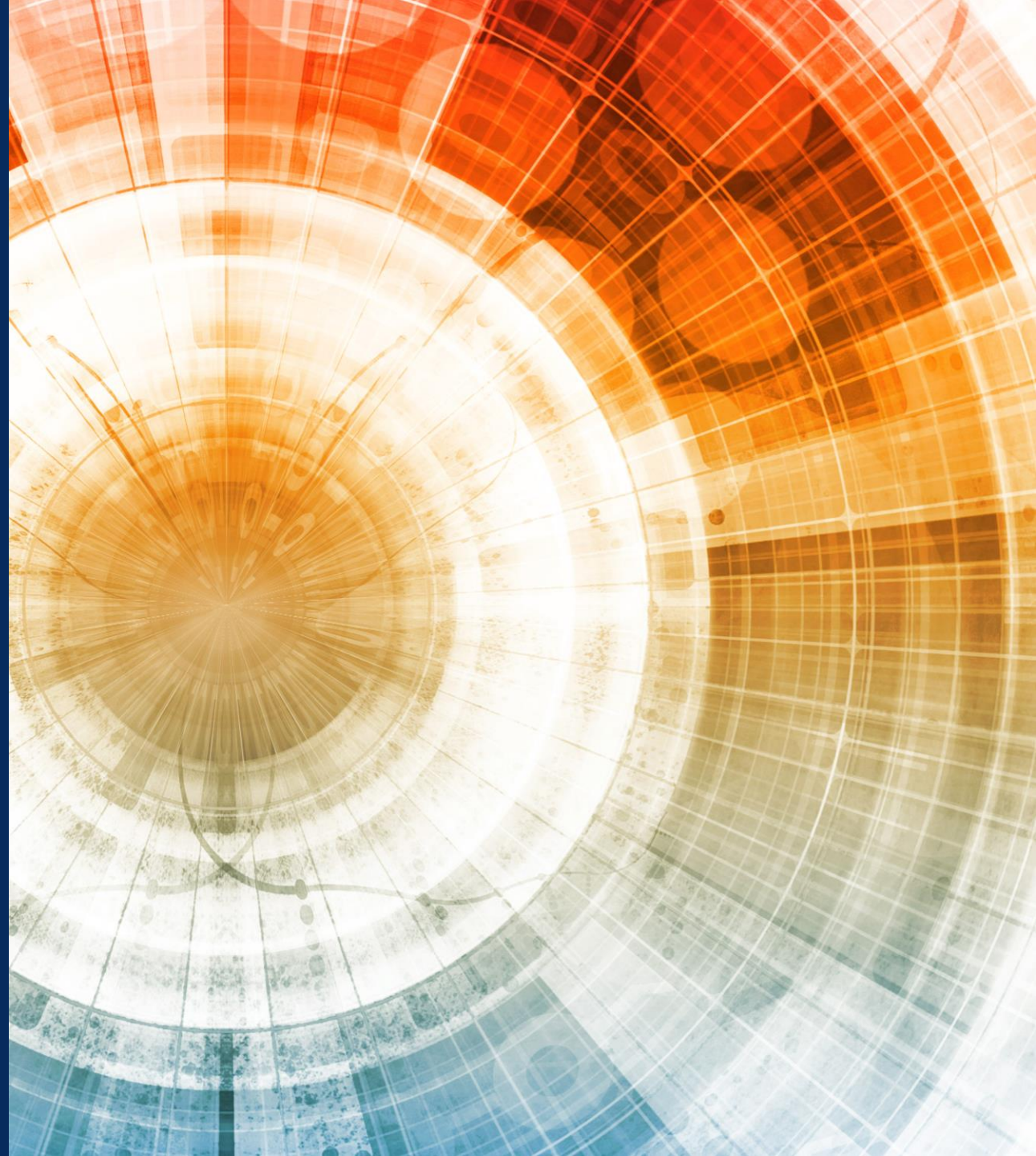
EMA
EUROPEAN MEDICINES AGENCY

Shaping the future of regulatory data in the AI era – An EMA perspective

CDISC EU Interchange 2026
May 21, 2026

Presented by Eftychia Eirini Psarelli

Data Analytics and Methods Task force, European Medicines Agency



Disclaimer

The presenter does not have any conflict of interests.

Meet the speaker

Eftychia Eirini Psarelli

Title: Scientific specialist, Clinical Study Data Lead

Clinical Trials Transformation, Data Analytics and Methods Task Force

Organisation: European Medicines Agency

Eftychia is a scientific specialist at EMA in the Clinical Trials Transformation Workstream of the Data Analytics and Methods Task Force, where she has been managing EMA's Clinical Study Data initiative, focusing on utilising individual patient-level clinical study data to generate evidence for better and more efficient regulatory decision making.
Prior to joining the EMA in July 2020, she spent 8 years as a Senior Statistician at the Liverpool Clinical Trials Centre within the University of Liverpool, UK, where she has gained strong analytical skills in the area of statistical programming and data curation.

Eftychia fosters EMA activities where data standards can have an added value, particularly for clinical trial data. She is also an observer in Europe's CDISC Coordinating Committee (E3C).

Technology delivers benefits

AI in Healthcare | regulatory drivers

Regulatory submissions

Regulate applications of AI in medicines development

Process Analytics

Improve **efficiency** by automating and digitalising processes

Healthcare Analytics

Structure information and increase insights into data to inform decision-making

AI in clinical trials | regulatory experience

Updated version in the making!

AI in clinical trials | regulatory experience

Common themes:

- Site and patient selection for a clinical trial
- Digital twins for covariate prognosis / clinical outcome prediction / external control arms
  -> to increase the precision in the analysis and decrease trial size
  -> for sponsor decisions about design and conduct of the trial
- Event adjudication, including:
  - Medical imaging and histological analyses
    - Possibly as primary endpoint (for example: Qualification Opinion of AIM-NASH)
  - Automated adverse event adjudication
  - (Remote) Digital endpoints, including wearables and video recordings (e.g. to assess mobility and gait)
- Safety monitoring, including case processing

AI in clinical trials | regulatory position

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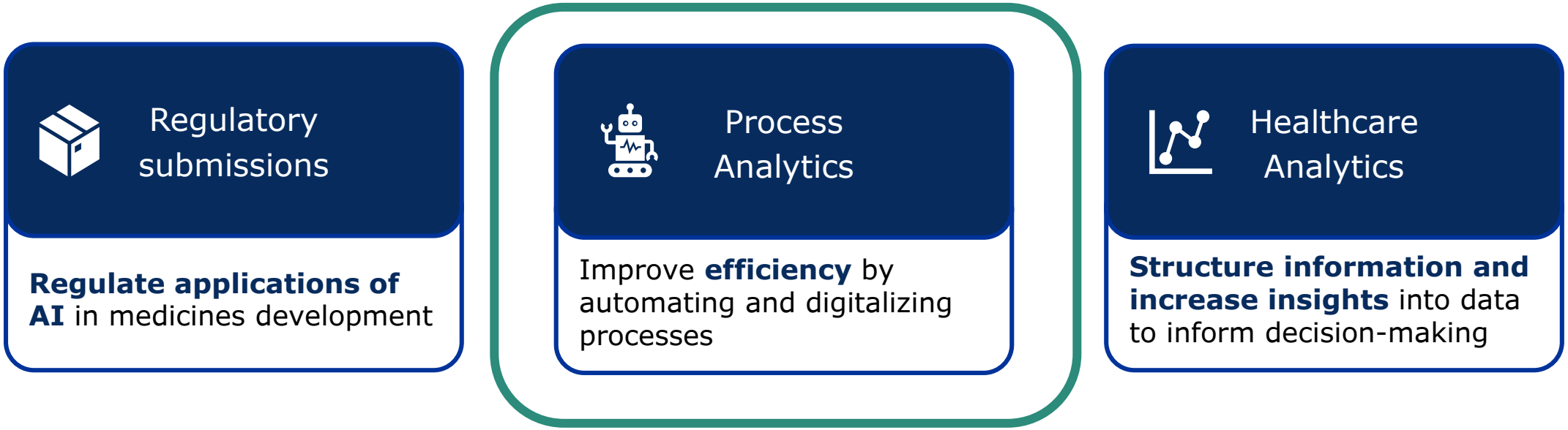
SCIENCE MEDICINES HEALTH

9 September 2024
EMA/CHMP/CVMP/83833/2023
Committee for Medicinal Products for Human Use (CHMP)
Committee for Medicinal Products for Veterinary Use (CVMP)

Reflection paper on the use of Artificial Intelligence (AI) in the medicinal product lifecycle

AI in Healthcare | Regulatory Drivers

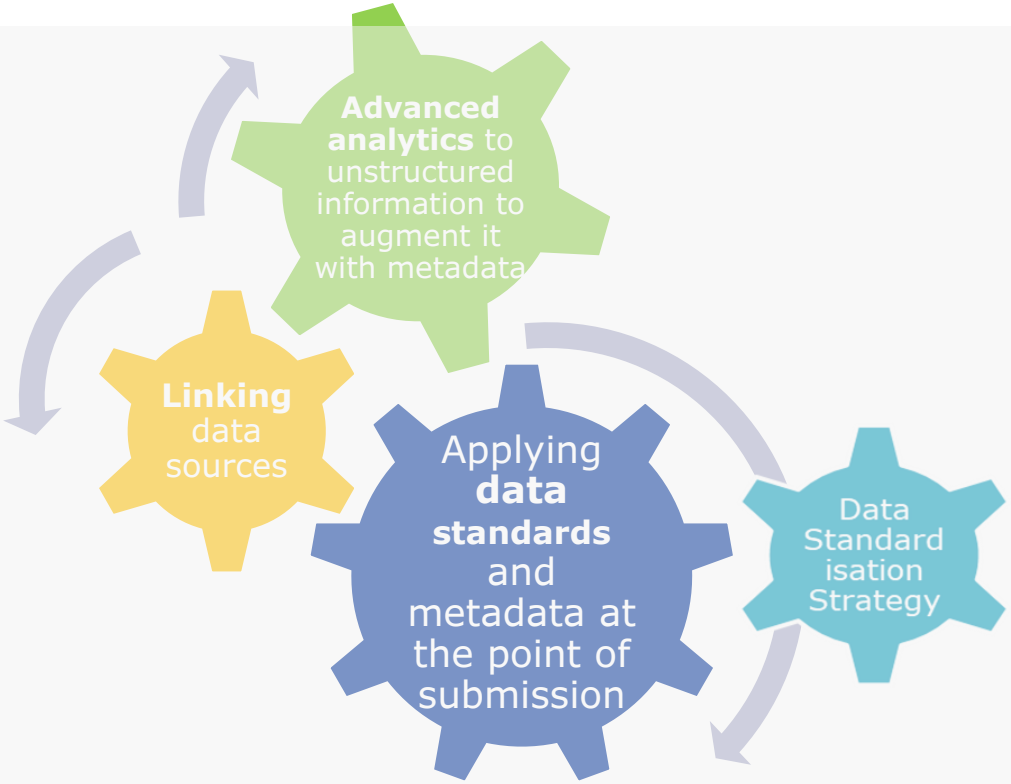


Where we came from

- An ambitious vision in 2021
  - But struggling with the insufficient power of available natural language processing tools

2021 project vision

The purpose of the project is to deliver effective generation of evidence in support of benefit/risk decision making from **data-driven interrogation of scientific information** within Lifecycle Regulatory Submissions.



- GPT4’s release in March 2023 turned things around !

Scientific advice letters - recap
Problem and solution

Scientific Advice Letters

Challenges Finding information

Searching

Scientific Explorer
Artificial Intelligence (AI) enabled search for EMRN

Data → Issue → Need → Solution

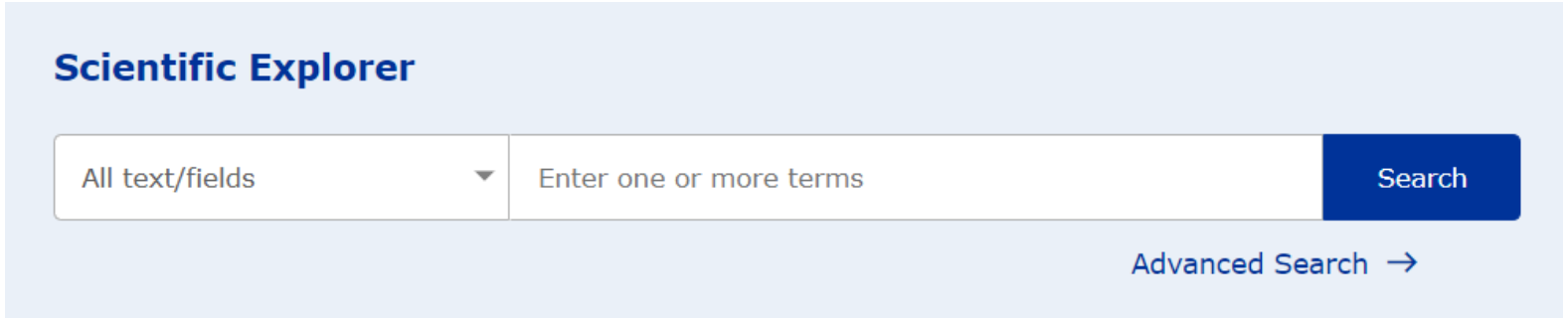
See regulatory precedents

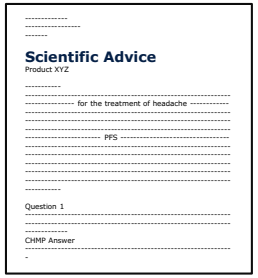
AI Pre-processing of document “indexing”;
Standardised, validated, reproducible

Supporting efficiency, consistency, quality; for EMA and for Network

Scientific Explorer Enables

Fast, easy, precise **searching** and **interacting** with regulatory precedents, to support efficiency, quality, consistency of assessment





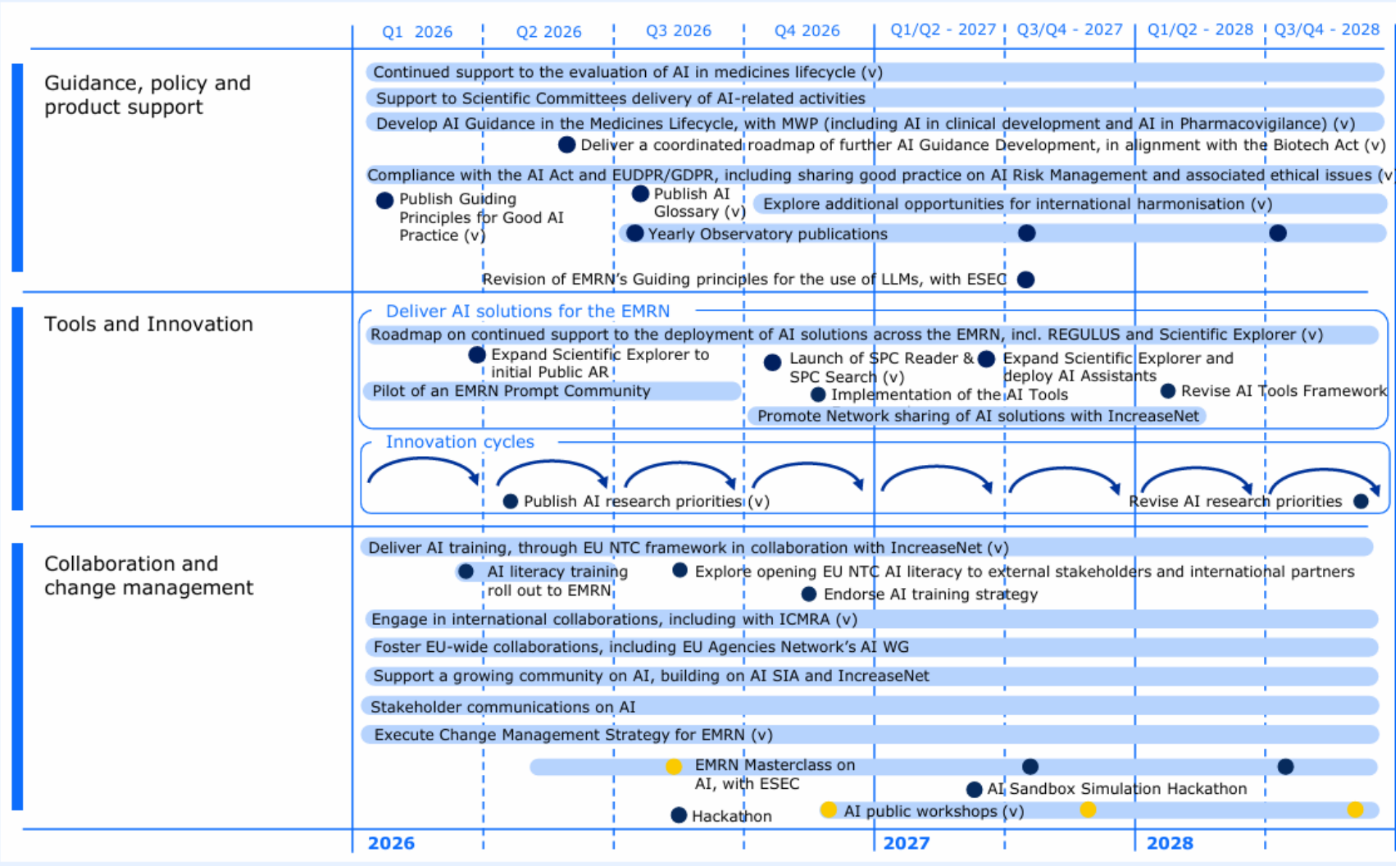
Across unstructured and **structured data**
(and we need to talk about standardised data…)

The first release focuses on **Scientific Advice** procedures on medicinal products for human use.

AI in clinical trials | AI workplan to 2028

NDSG workplan 2026-2028

Data and AI in medicines regulation



AI in clinical trials | planned regulatory activities

NDSG workplan 2026-2028

Data and AI in medicines regulation

- Glossary of AI terminology
- Workshop 'AI use in clinical trials'
- Guideline on AI in clinical development
- Annex to GCP Inspectors Working Group Guideline on computerised systems and electronic data in clinical trials

‘Trusted medicines by unlocking the value of data’

HMA-EMA Network data steering group’s vision

HMA-EMA Network Data Steering Group

NDSG workplan 2026-2028

Data and AI in medicines regulation

Workstreams

The NDSG workplan is organised in six workstreams:

Strategy and governance:

- Strategy
- Governance

Data analytics:

- Review of advanced or innovative methodologies and of new data types for evidence generation
- Real-world data, clinical study data, EudraVigilance data, non clinical data, genomic data

Artificial Intelligence :

- Guidance, policy and product support
- Tools and technologies
- Collaboration and change management
- Experimentation

Data interoperability:

- Data asset discovery, cataloguing and metadata management
- Data quality management
- Organisational and semantic interoperability

Stakeholder engagement and change management:

- Change management strategy
- Network skills and knowledge
- Stakeholder engagement and communication

Guidance and international initiatives :

- Guidance
- International initiatives

Additional initiatives & relevant regulation

- Development of a data standardisation framework
  - To facilitate **adoption and implementation of data standards** in the EU
- Definition of technical requirements for submission of **clinical study data**
  - To clarify **acceptable CDISC data standards, data formats** and any other data and IT-related requirements
- Ongoing **non-clinical (SEND) data** pilot
- Implementation of ICH M11 in clinical trial protocols
  - Following completion of ICH M11 (Step 5), to draft a **technical implementation guide** for the **submission of clinical trial protocols**

- **DARWIN EU®** - RWE use throughout medicinal product lifecycle
  - **Use of Common Data Model** (OMOP)
  - 40 data partners in 2026, access to data from over 250 million patients and approx. 110 studies delivered through DARWIN EU®
- **European Health Data Space (EHDS)**
  - Landmark EU regulation in force since 26 March 2025
  - Common legal, technical and governance framework for health data across all EU Member States
  - Primary and secondary use of data
  - Metadata standards, data catalogue requirements, interoperability rules in support of AI-enabled analytics under a trusted governance framework

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Thank you

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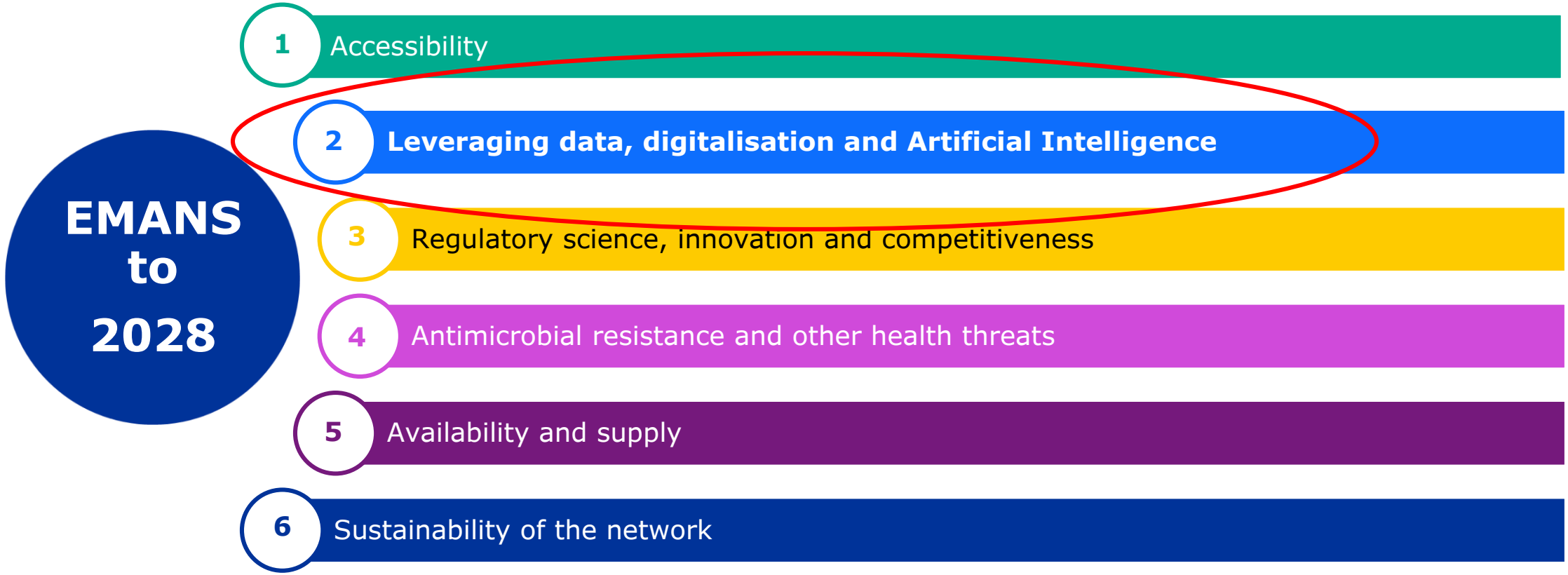
Follow us

Appendix

European medicines agencies network strategy (EMANS) to 2028: strategic focus areas



Useful links

- The European medicines agencies network strategy 2028
- NDSG workplan 2025-2028
- Artificial intelligence | European Medicines Agency (EMA)

2026 CDISC Europe Interchange
Regulatory Panel Session

PMDA
Making everyone's lives brighter together

PMDA Update

Yuki Ando
Principal Senior Scientist for Biostatistics
Pharmaceuticals and Medical Devices Agency

The views expressed on this presentation are those of the presenter and
do not necessarily reflect the official views of Pharmaceuticals and Medical Devices Agency.

Speaker

Yuki Ando, PhD

Title: Principal Senior Scientist for Biostatistics

Organization: Pharmaceuticals and Medical Devices Agency

Dr. Yuki Ando is a Principal Senior Scientist for Biostatistics of the Pharmaceuticals and Medical Devices Agency (PMDA), Japan. She is responsible for the biostatistics review and consultation in the new drug and device review offices in PMDA and is a leader of Biostatistics Reviewers who are the primary users of the electronic study data that are submitted with new drug applications. Additionally, she is supervising works in Office of Regulatory Science Coordination, the office which is responsible for receiving e-study data.

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Current Status of Electronic Data Submission in Japan

- It has been six years since the end of the transitional period for electronic data submission.
- PMDA has accumulated substantial experience in the use of clinical trial data for new drug review.
- All relevant information in English continues to be publicly available on the website:
  - https://www.pmda.go.jp/english/review-services/reviews/0002.html
- Since the 2024 Europe Interchange there have been no major revisions to notifications or PMDA Technical Conformance Guide.

Today, I would like to provide a few additional updates and perspectives

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Update of Data Standards Catalog (Mar 14, 2025)

PMDA Data Standards Catalog (2025-03-14) - Data Exchange Standards

Use	Data Exchange Standard	Supported Version(s)	Implementation Guide Version	Exchange Format	Date Support Begins (YYYY-MM-DD)	Date Support Ends (YYYY-MM-DD)	Notes
Clinical study datasets - Transport	SAS Transport (XPORT)	5	-	XPT	2016-10-01		
Clinical study datasets	SDTM	2.0	3.4	XPT	2025-04-01		
Clinical study datasets	SDTM	1.7	3.3	XPT	2023-04-01		
Clinical study datasets	SDTM	1.4	3.2	XPT	2016-10-01		
Clinical study datasets	SDTM	1.3	3.1.3	XPT	2016-10-01		
Clinical study datasets	SDTM	1.2	3.1.2 Amendment1	XPT	2016-10-01		
Clinical study datasets	SDTM	1.2	3.1.2	XPT	2016-10-01		
Clinical study datasets	ADaM	2.1	1.3	XPT	2024-04-01		
Clinical study datasets	ADaM	2.1	1.2	XPT	2024-04-01		
Clinical study datasets	ADaM	2.1	1.1	XPT	2022-01-01		
Clinical study datasets	ADaM	2.1	1.0	XPT	2016-10-01		
Clinical study data definition files	Define	2.1	-	XML	2024-04-01		
Clinical study data definition files	Define	2.0	-	XML	2016-10-01		
Clinical study data definition files	Define	1.0	-	XML	2016-10-01	2025-03-31	
Documents	PDF	1.4-1.7	-	PDF	2016-10-01		In principle, eCTD PDF specification should be referenced for details.

At present, the catalog provides comprehensive coverage of available (new) standard versions. Going forward, PMDA aims to make newly released versions of standards available without significant delay.

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Update on Operational Changes

- Based on accumulated experience in data receipt, as well as consideration of the burden on data submitters, PMDA has implemented several operational changes.
- We have confirmed that these changes have not affected the use of submitted data in new drug review process.
- Detailed information is provided in the section of ”Data Standards Catalog and Study Data Validation Rules” on the website.
  - As these updates are not covered in formal Technical Conformance Guide or other documents, they are communicated via the website.
  - While updates are not frequent, sponsors are encouraged to check the website periodically…

https://www.pmda.go.jp/english/review-services/reviews/0002.html

Version of PMDA Validation Rules

Data Standards Catalog and Study Data Validation Rules

- Data Standards Catalog (2025-03-14) [24.8KB]
- Study Data Validation Rules

Please note that when submitting electronic study data to the PMDA via the gateway system, the latest version(Version 6.0) of the validation rules is used. However, when applicants submit additional electronic study data after the application, the version of the validation rules at the time of the application will be used.

Although only one version of the validation rule is used for each single application via the gateway system, all versions of these rules — including those no longer open for acceptance — may be used for the validation and the explanation of the results for individual studies performed by an applicant prior to submission. In addition, different versions of the validation rules may be used for SDTM and ADaM datasets of the same study.

- Version 1.0 (2015-11-18) [82.0KB] Acceptable from Oct 1, 2016 to Mar 31, 2021 (application date)
- Version 2.0 (2019-09-27) [97.9KB] Acceptable from Apr 1, 2020 to Mar 31, 2023 (application date)
- Version 3.0 (2021-12-15) [103KB] Acceptable from Jan 1, 2022 to Mar 31, 2025 (application date)
- Version 4.0 (2023-02-28) [112KB] Acceptable from Apr 1, 2023 to Mar 31, 2026 (application date)
- Version 5.0 (2024-03-29) [124KB] Acceptable from Apr 1, 2024 to Mar 31, 2027 (application date)
- Version 6.0 (2025-03-14) [132KB] Acceptable from Apr 1, 2025 (application date) Latest version (current)

This item was recently introduced to prevent version selection errors by sponsors at the time of data submission.

This was already presented at the 2024 Interchange; however, it has been elaborated in conjunction with the addition above.

https://www.pmda.go.jp/english/review-services/reviews/0002.html

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Utilization of Real-World Data (RWD)

- At present, PMDA considers that CDISC standardization of clinical trial data conducted by industry in Japan has been implemented without major issues.
- On the other hand, challenges remain in:
  - Standardization of real-world data (RWD), which is expected to be increasingly utilized in the future
  - Implementation of CDISC standards in academia
- PMDA has issued “Points to consider for externally controlled trials (Early Consideration)”, reflecting the anticipated use of RWD as external controls

https://www.pmda.go.jp/files/000275337.pdf

- PMDA’s view on RWD standardization remains unchanged from FAQ 1-35, which summarizes expectations for registry data submissions. Sponsors are encouraged to proactively consult with PMDA when planning the use and submission of RWD.

https://www.pmda.go.jp/files/000274654.pdf

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Reference: FAQ 1-35 on Registry Data Submission

Q When registry data are utilized in documents of clinical data for a new drug application, what are the contents of electronic study data to be submitted?

A Even if registry data are utilized in document of clinical data, it is still important, as with the case of clinical studies, to submit data that provide the major evidence for the efficacy, safety, and dosage and administration for a new drug application in a format conforming to the CDISC standards basically.

On the other hand, the scope, contents and format of registry data to be submitted may require individual judgment, depending on the contents and purpose of registry data. Therefore, please consult with the PMDA in advance at an applicable consultation.

Regarding the submission format of data, if registry data are used at least for an evaluation of efficacy as an external control in a clinical study and an analysis that uses registry data corresponds to analyses subject to submission of analysis datasets as described in Section 4.1.1.3 of the technical conformance guide, please submit datasets used for the analysis in the ADaM format as part of the analysis datasets of the clinical study. If submission in the ADaM format is difficult because of the method of analysis or other technical reasons, please consult with the PMDA at a consultation on preparation of submission of electronic study data.

https://www.pmda.go.jp/files/000274654.pdf

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AI in Regulatory Context

- AI is rapidly evolving across the drug development and clinical research.
  - Data management, analysis, decision support, and documentation
- Potential benefits for regulatory review:
  - Improved efficiency such as supporting development of review documents and supporting analyses which are routinely implemented
  - Enhanced insight into complex designs and datasets
- However, AI is not a standalone solution and its value depends on how to use and the quality of underlying data
  - High-quality, structured data is essential for reliable regulatory decision-making and reproducible analyses.
  - CDISC standards provide a critical foundation including standardized datasets, consistent metadata, and traceability.

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PMDA's Internal Efforts on AI

- PMDA issued "Action Plan for the Use of AI in Operations at the PMDA (Sep 26, 2025)."
  https://www.pmda.go.jp/files/000277511.pdf
  1. Introduction of existing AI technology: Boosting the efficiency of daily operational process
     - PMDA will achieve higher efficiency in the administrative operations by introducing and utilizing AI technologies that are already available.
  2. Introduction of specialized AI technology for PMDA's key services: Technical verification and information gathering
  3. AI governance: Preparation of support / promotion framework and rules, implementation of measures to improve IT literacy in all PMDA staffs
- PMDA also announced the initiation of the use of generative AI in selected PMDA operations (announced April 2026, in Japanese only).

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Summary

- PMDA has accumulated substantial experience in the use of electronic study data in regulatory review.
- CDISC standards have been successfully implemented for clinical trial data submission in Japan.

- Ongoing efforts focus on addressing challenges related to other types of data, such as RWD
  - Standardization, improving data quality and usability
- AI is a promising enabler for future regulatory processes
  - However, its value depends on high-quality, standardized data

- Collaboration remains essential across multiple stakeholders
  - Regulatory authorities, industry (including coordination within global organizations), and standards organizations (e.g. CDISC)

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独立行政法人 医薬品医療機器総合機構
Pharmaceuticals and Medical Devices Agency

CDISC Europe Interchange 2026

Regulatory Panel Discussion

Ethan Chen, Director, FDA/OO/ODT

Dr. Y. Veronica Pei, Deputy Director, FDA/CDER/OND/ODES/DBIRBD

May 21, 2026

DISCLAIMER

The views and opinions presented here represent those of the speaker and should not be considered to represent advice or guidance on behalf of the Food and Drug Administration.

The opinions expressed in this presentation are not meant to imply any changes to guidance, or regulations. Official guidance and regulations will be announced via the official outlets

IMPACT OF DIGITAL TRANSFORMATION AND AI

The impact of digital transformation and AI to Electronic Submission to Electronic Submission:

- AI and emerging technologies are transforming regulatory frameworks
- This transformation creates both challenges and opportunities for regulatory data submissions

So, do we still need standardized data?

YES!

Because quality data is the foundation for AI-assisted, science-based decision making

CURRENT INITIATIVES

Policy and Guidance

FDA and EMA AI Guiding principles

Infrastructure and Technology modernization efforts to support interoperability

HALO, Elsa and MARS

AEMS

Continue with eCTD V4.0 adoption

Currently accepting eCTD v4.0 for new applications only, planning to implement forward compatibility, which will allow eCTD v3.2.2 applications to be transitioned to eCTD v4.0, in mid to late 2026.

HALO, ELSA AND MARS

- **HALO**: Harmonized AI and Lifecycle Operations for Data
- **Elsa**: FDA's enterprise Generative AI and Agentic AI capability
- **MARS**: Modernizing and Accelerating Regulatory Submissions

Data Platform

An enterprise data platform that aggregates sponsor submission and historical FDA reviews from all centers

AI Capabilities

The foundational AI engine powering intelligent, language-driven solutions across the agency.

WE ARE HERE

AI for Regulatory Review

Operationalizes and delivers mission-critical solutions for FDA programs and centers

Transform FDA

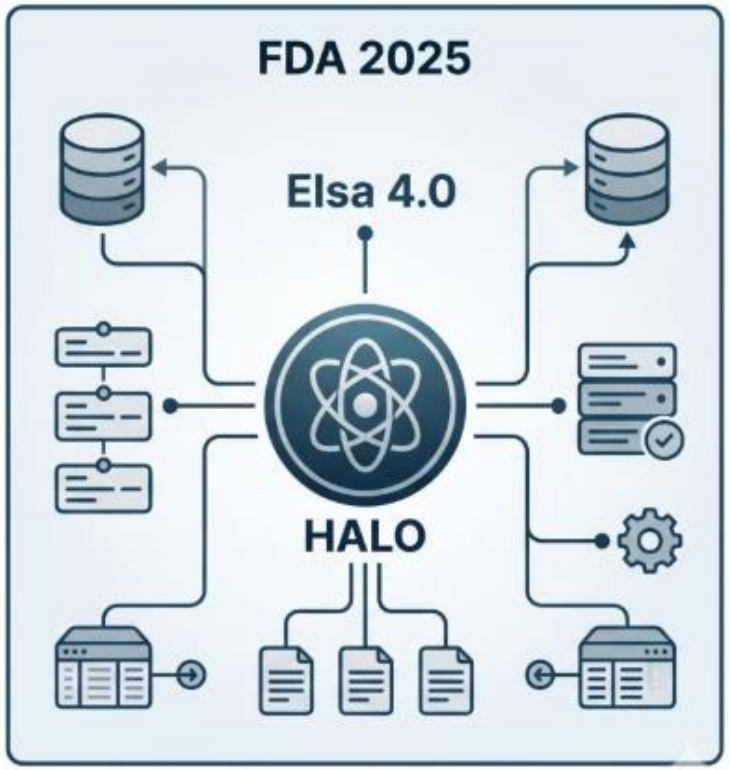
Revolutionize FDA operation to be the Gold Standard for the next decade

HALO + Elsa → MARS

WHAT WE ACHIEVED

- Harmonized AI and Lifecycle Operations for Data (HALO): consolidated and centralized more than 40 disparate data sources, systems and portals across all FDA centers into the HALO platform.
- Launched Elsa 4.0*, a significant upgrade to the agency's internal AI tool for scientific reviewers and staff. Elsa's new capabilities empowers staff to work more efficiently and avoid tedious tasks

The agency began integrating HALO and Elsa so that FDA staff can query data directly and build agentic workflows.



*FDA News Release: FDA Expands AI Capabilities and Completes Data Platform Consolidation (https://www.fda.gov/news-events/press-announcements/fda-expands-ai-capabilities-and-completes-data-platform-consolidation)

FDA ADVERSE EVENT MONITORING SYSTEM (AEMS)

- Major achievement in the agency's mission to modernize and provide transparency to the safety of regulated products.
- Combines adverse event reports submitted to FDA for drugs, biologics, vaccines, cosmetics, and animal food.
- In the months ahead, all remaining product centers will begin processing adverse event reports in AEMS.

COLLABORATIONS

FDA's collaboration between regulators, industry, and standards organizations:

- ICH: M2, M11 and other expert working groups
- Standard Development Organizations (CDISC, HL7, FHIR, ISO, etc.)
- Non-profit organizations (Reagan-Udall Foundation, TransCelerate, Gates Foundation, etc.)

FDA ICH ENGAGEMENT – M2

- Co-rapporteur for ICH M2 to facilitate electronic communication between pharmaceutical companies and regulators.
- In 2025, ICH M2 Expert Working Group (EWG) focused in below areas:
  - AI and Emerging Technologies Integration Initiatives
  - Standard Development Organization (SDO) Process Alignment and Harmonization
  - M11 Collaboration and Support
  - ICH Terminology Harmonization
- Moving forward in 2026, FDA will collaborate with M2 EWG to advance the following initiatives:
  - Update the outdated ICH M2 Concept Paper to strengthen the foundation for advancing electronic standards
  - Address the growing use of AI in regulatory data exchange

FDA ICH ENGAGEMENT – M11

- Rapporteur and Topic Lead for ICH M11: Clinical electronic Structured Harmonised Protocol (CeSHarP)
- In 2025, M11 EWG:
  - Finalized **3 Deliverables**: Guideline, Protocol Template, & Technical Specification
  - Collaborated with M2 and CDISC to finalize **M11 controlled terminology**
  - Collaborated with M2 and HL7 on development of M11 Technical Implementation Guide (**TIG**) for Fast Healthcare Interoperability Resources (FHIR)
  - Achieved **Step 4**: Adoption by the ICH Regulatory Members

ICH M11 NEXT STEPS

- Support regulatory party implementation
- Complete training modules
- Collaborate with M2 on publication of TIG for FHIR on M2 ESTRI page
- Participate in PRISM M11 Demonstration Use Case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