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EUROPEAN MEDICINES AGENCY

Leveraging the power of data for public and animal health

Presented by Peter Arlett

DGRA conference, 8 May 2025

Clinical evidence 2030

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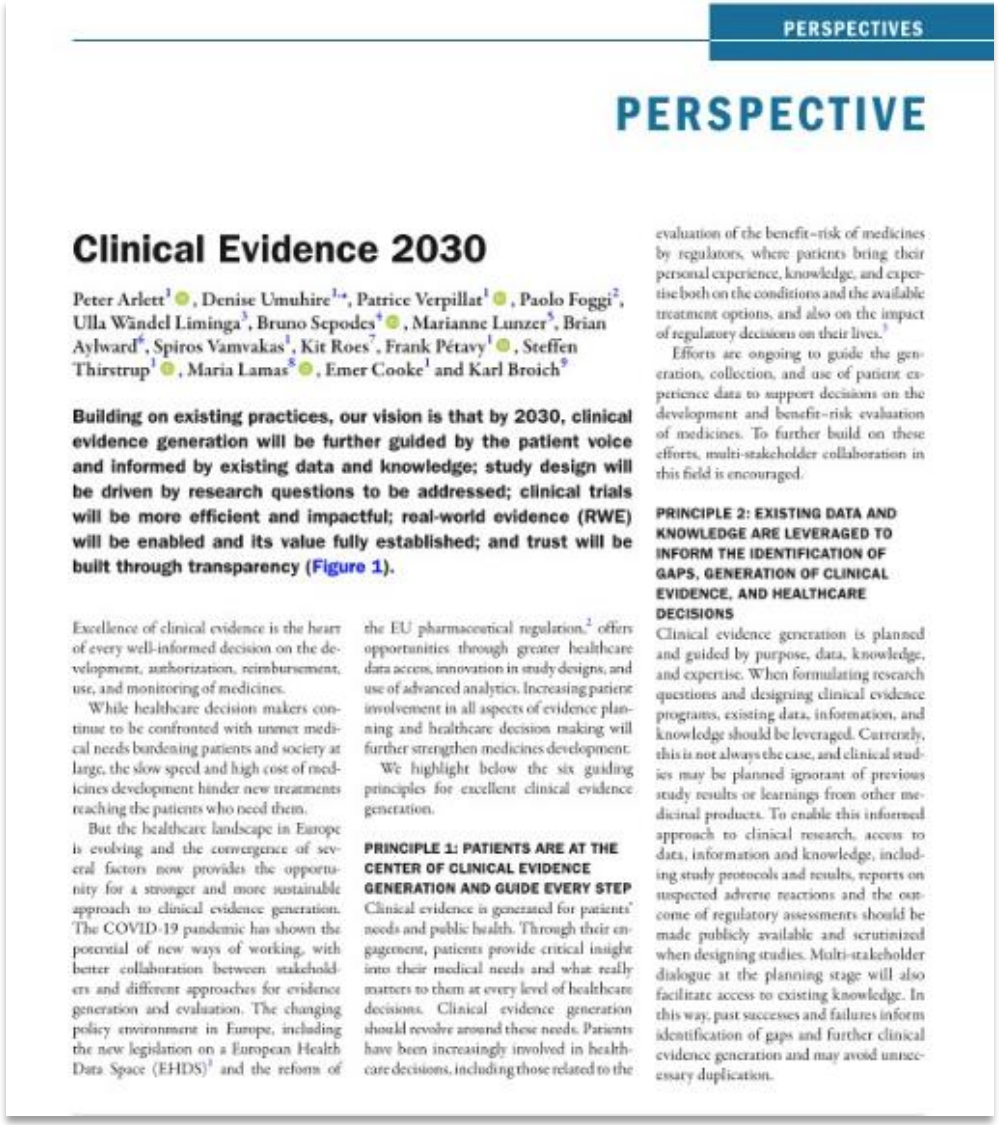
Clinical evidence: drivers for change

- Slow speed and high cost of product development
- Burden of unmet medical need
- Changing policy environment (AI, European Health Data Space, new pharma legislation)
- Opportunity of greater healthcare data access
- Opportunity of better study methods
- Opportunity of advanced analytics
- Pandemic shows new ways of working

Clinical evidence 2030: vision

- Patient voice guides every step of the way
- Evidence generation is planned and guided by data, knowledge and expertise
- Research question drives evidence choice and embraces spectrum of data and methods
- Clinical trials remain core but are smarter, better and faster
- Real world evidence is enabled, and its value established
- High transparency level underpins societal trust



PERSPECTIVES

PERSPECTIVE

Clinical Evidence 2030

Peter Arlett[1], Denise Umuhire[1,*], Patrice Verpillat[1], Paolo Foggi[2], Ulla Wändel Liminga[3], Bruno Sepodes[4], Marianne Lunzer[5], Brian Aylward[6], Spiros Vamvakas[1], Kit Roes[7], Frank Pétavy[1], Steffen Thirstrup[1], Maria Lamas[8], Emer Cooke[1] and Karl Broich[9]

Building on existing practices, our vision is that by 2030, clinical evidence generation will be further guided by the patient voice and informed by existing data and knowledge; study design will be driven by research questions to be addressed; clinical trials will be more efficient and impactful; real-world evidence (RWE) will be enabled and its value fully established; and trust will be built through transparency (Figure 1).

Excellence of clinical evidence is the heart of every well-informed decision on the development, authorization, reimbursement, use, and monitoring of medicines.

While healthcare decision makers continue to be confronted with unmet medical needs burdening patients and society at large, the slow speed and high cost of medicines development hinder new treatments reaching the patients who need them.

But the healthcare landscape in Europe is evolving and the convergence of several factors now provides the opportunity for a stronger and more sustainable approach to clinical evidence generation. The COVID-19 pandemic has shown the potential of new ways of working, with better collaboration between stakeholders and different approaches for evidence generation and evaluation. The changing policy environment in Europe, including the new legislation on a European Health Data Space (EHDS)[3] and the reform of the EU pharmaceutical regulation,[2] offers opportunities through greater healthcare data access, innovation in study designs, and use of advanced analytics. Increasing patient involvement in all aspects of evidence planning and healthcare decision making will further strengthen medicines development.

We highlight below the six guiding principles for excellent clinical evidence generation.

PRINCIPLE 1: PATIENTS ARE AT THE CENTER OF CLINICAL EVIDENCE GENERATION AND GUIDE EVERY STEP

Clinical evidence is generated for patients' needs and public health. Through their engagement, patients provide critical insight into their medical needs and what really matters to them at every level of healthcare decisions. Clinical evidence generation should revolve around these needs. Patients have been increasingly involved in healthcare decisions, including those related to the evaluation of the benefit–risk of medicines by regulators, where patients bring their personal experience, knowledge, and expertise both on the conditions and the available treatment options, and also on the impact of regulatory decisions on their lives.[3]

Efforts are ongoing to guide the generation, collection, and use of patient experience data to support decisions on the development and benefit–risk evaluation of medicines. To further build on these efforts, multi-stakeholder collaboration in this field is encouraged.

PRINCIPLE 2: EXISTING DATA AND KNOWLEDGE ARE LEVERAGED TO INFORM THE IDENTIFICATION OF GAPS, GENERATION OF CLINICAL EVIDENCE, AND HEALTHCARE DECISIONS

Clinical evidence generation is planned and guided by purpose, data, knowledge, and expertise. When formulating research questions and designing clinical evidence programs, existing data, information, and knowledge should be leveraged. Currently, this is not always the case, and clinical studies may be planned ignorant of previous study results or learnings from other medicinal products. To enable this informed approach to clinical research, access to data, information and knowledge, including study protocols and results, reports on suspected adverse reactions and the outcome of regulatory assessments should be made publicly available and scrutinized when designing studies. Multi-stakeholder dialogue at the planning stage will also facilitate access to existing knowledge. In this way, past successes and failures inform identification of gaps and further clinical evidence generation and may avoid unnecessary duplication.

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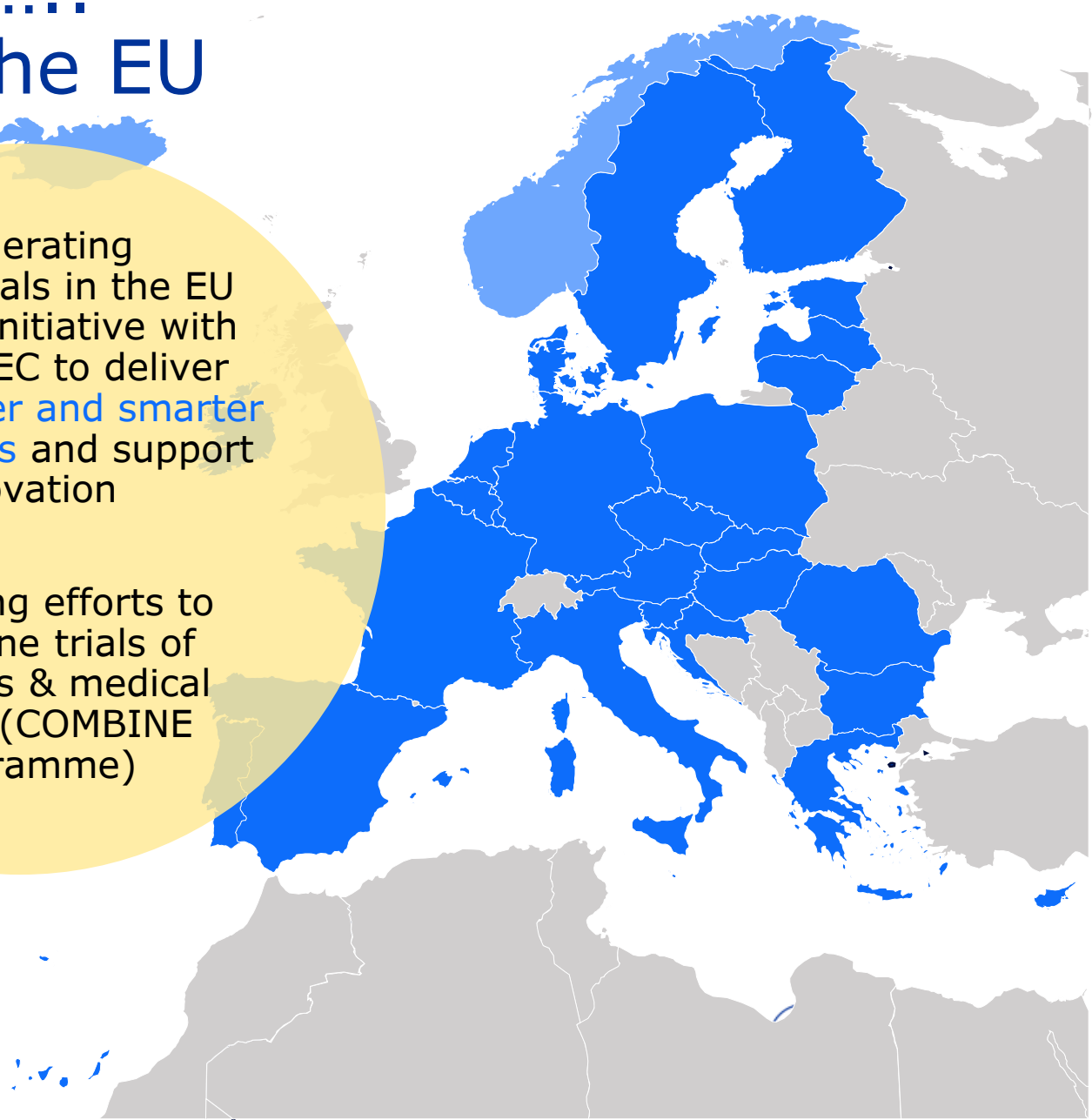
Not for today but important…..
Supporting clinical trials in the EU

Supporting the implementation of the Clinical Trials Regulation and further harmonisation (CTR Collaborate)

Maintaining and improving the Clinical Trials Information System (CTIS), the IT tool of the CTR

Accelerating Clinical Trials in the EU (ACT EU) initiative with HMA and EC to deliver better, faster and smarter clinical trials and support innovation

Supporting efforts to streamline trials of medicines & medical devices (COMBINE programme)



Data Strategy and Governance

Joint HMA-EMA Network Data Steering Group (NDSG)

Context - EMANS TO 2028

Theme 2: Leveraging data, digitalisation and artificial intelligence

Goal 1

- **Maximise the generation, interoperability, use and exchange of data to support EU decision-making**

Objectives:

- Embed the use of healthcare data from diverse populations in the network's processes, including in support of the EHDS implementation, and pilot the use of novel types of data (e.g. synthetic data, patient experience data or data for personalised medicine, e.g. genomic data)
- Ensure a high level of interoperability (notably through the use of Master data), standardisation and quality of data addressing potential biases and ethical considerations, and ensure that the network data assets are appropriately managed

Goal 2

- Leverage digitalisation, experimentation and innovation to deliver optimised regulatory processes

Goal 3

- **Realise the network vision on AI across all EMANS focus areas**

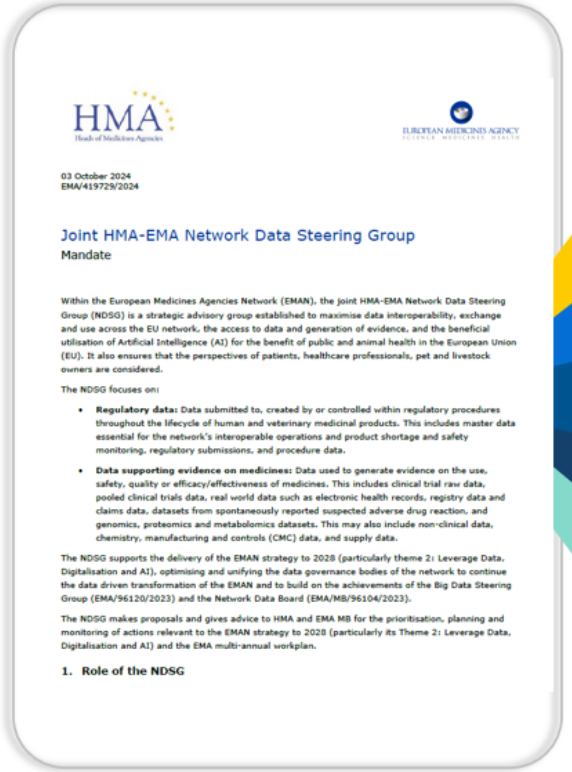
Objectives:

- Leverage experimentation and technological advances in AI to support the digital business transformation of the EU network
- Harness the potential of AI throughout the medicines' lifecycle

Network Data Steering Group mandate

NDSG is the strategic advisory group established to:

- maximise data use, exchange and interpretation,
- improve access to data and evidence generation,
- leverage use of artificial intelligence for better decision-making.



HMA
Heads of Medicines Agencies

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

03 October 2024
EMA/419729/2024

Joint HMA-EMA Network Data Steering Group
Mandate

Within the European Medicines Agencies Network (EMAN), the joint HMA-EMA Network Data Steering Group (NDSG) is a strategic advisory group established to maximise data interoperability, exchange and use across the EU network, the access to data and generation of evidence, and the beneficial utilisation of Artificial Intelligence (AI) for the benefit of public and animal health in the European Union (EU). It also ensures that the perspectives of patients, healthcare professionals, pet and livestock owners are considered.

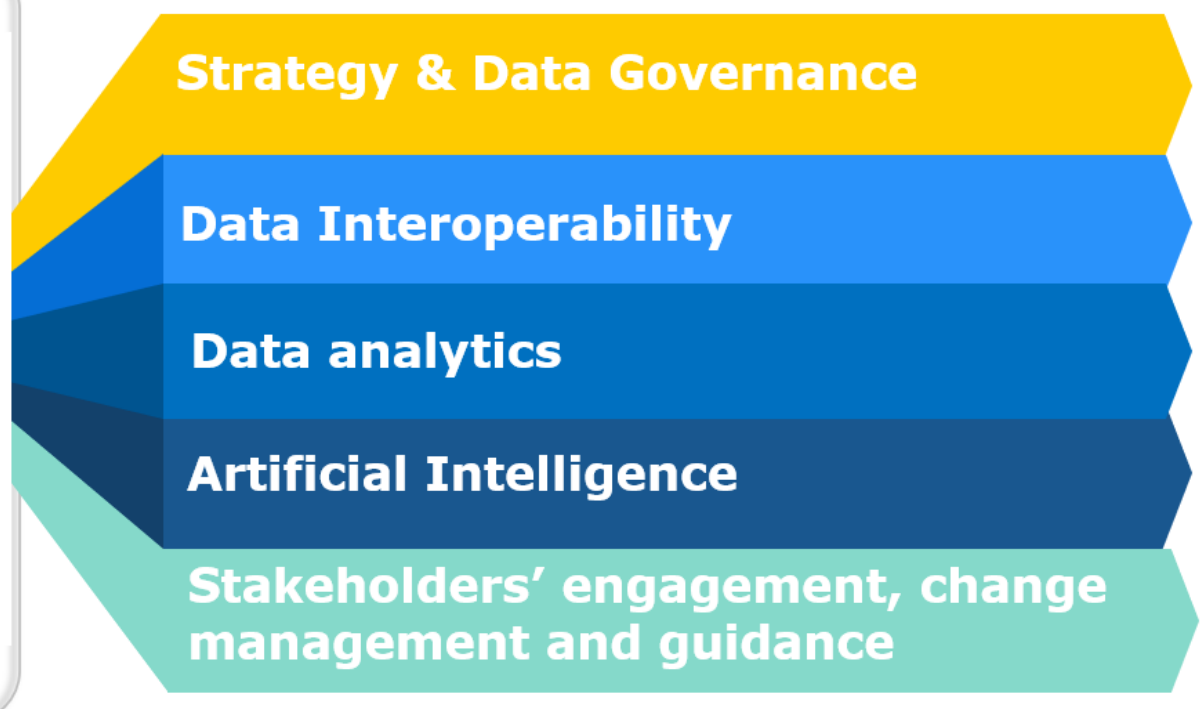
The NDSG focuses on:

- **Regulatory data:** Data submitted to, created by or controlled within regulatory procedures throughout the lifecycle of human and veterinary medicinal products. This includes master data essential for the network's interoperable operations and product shortage and safety monitoring, regulatory submissions, and procedure data.
- **Data supporting evidence on medicines:** Data used to generate evidence on the use, safety, quality or efficacy/effectiveness of medicines. This includes clinical trial raw data, pooled clinical trials data, real world data such as electronic health records, registry data and claims data, datasets from spontaneously reported suspected adverse drug reaction, and genomics, proteomics and metabolomics datasets. This may also include non-clinical data, chemistry, manufacturing and controls (CMC) data, and supply data.

The NDSG supports the delivery of the EMAN strategy to 2028 (particularly theme 2: Leverage Data, Digitalisation and AI), optimising and unifying the data governance bodies of the network to continue the data driven transformation of the EMAN and to build on the achievements of the Big Data Steering Group (EMA/96120/2023) and the Network Data Board (EMA/MB/96104/2023).

The NDSG makes proposals and gives advice to HMA and EMA MB for the prioritisation, planning and monitoring of actions relevant to the EMAN strategy to 2028 (particularly its Theme 2: Leverage Data, Digitalisation and AI) and the EMA multi-annual workplan.

1. Role of the NDSG



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‘Trusted medicines by unlocking the value of data’

Network Data Steering Group – unlocking value of data

Key priorities 2025

Strategy and governance	Support to New Pharma Legislation, EHDS2 and AI Act	EMRN data strategy, incl. data standardisation strategy, data quality and cataloguing	EMRN data analytics strategy, incl. strengthening network capabilities
Data analytics, interoperability and AI	Continue ongoing initiatives of DARWIN EU® and clinical study raw data	Medicines master data implementation - EHDS1 as priority use case	Artificial intelligence
Change management	Communication	Training and guidance	Stakeholder engagement

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 & 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

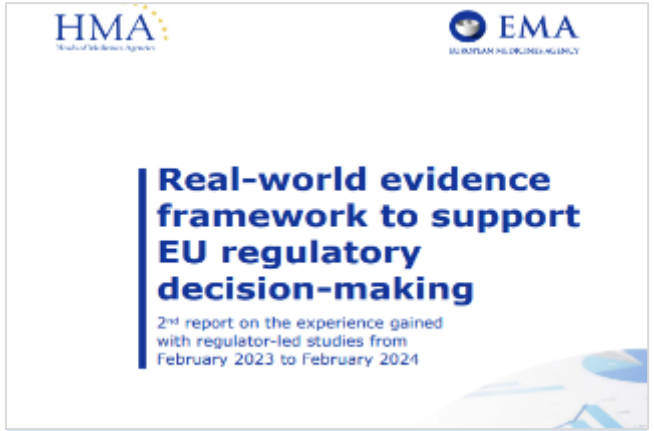
Guidance and international initiatives :

- Guidance
- International initiatives

Real-world evidence (RWE) integration into medicines regulatory decisions

Transformational journey

- Support planning and validity of studies
- Understand clinical context
- Investigate associations and impact of regulatory decisions



RWE framework

- Addresses research needs across full spectrum of medicinal product lifecycle
- >100 studies initiated since 2021
- ±100 NEW research topics assessed per year now

DARWIN EU®

- Primary pathway for generating RWE
- Beginning of 2025: 30 data partners from 16 European countries giving access to data from >180 million patients in Europe

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Use of clinical study data in medicine evaluation

Anticipated benefits

- Improved understanding of clinical study results to inform regulatory decision making
- Less questions to applicants
- Faster access to innovative, safe and effective medicines

Proof-of-concept pilot

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

Support potential implementation

- Pilot extended until NPL comes into force
- Explore IT solutions to cater for heterogeneous EMRN needs
- Guidance and training

PGx workshop report

- Report - Joint EC/HMA/EMA multi-stakeholder workshop on pharmacogenomics
- Includes 11 recommendations on PGx in medicines regulation and implementation into clinical practice:
  - For additional regulatory action
  - For facilitating the uptake of genomics by national healthcare systems
  - For leveraging genomic data linked to real-world data sources
  - For increasing the impact of EU-funded projects

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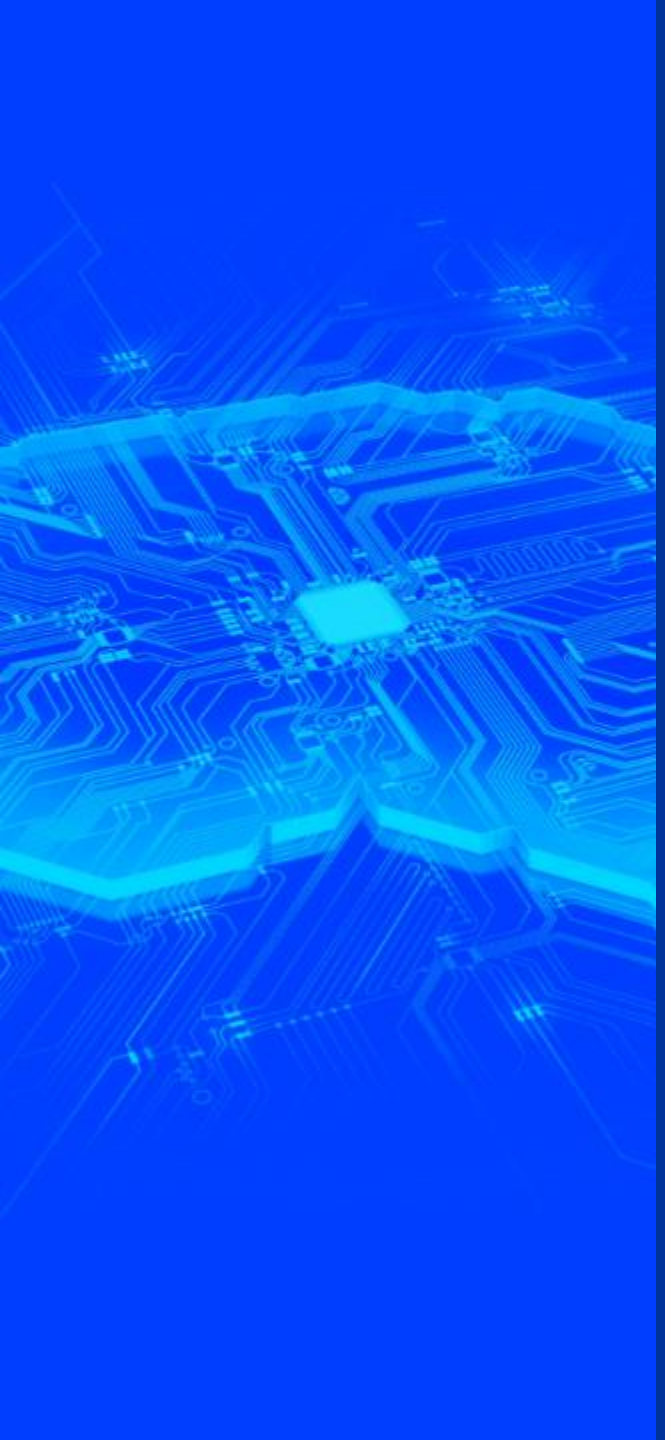

Artificial intelligence

EMRN experience to-date and looking ahead

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Enabling safe and responsible use of AI

Guidance and policy

- Reflection paper on use of AI
- Guiding principles on the use of LLMs

Tools and technologies

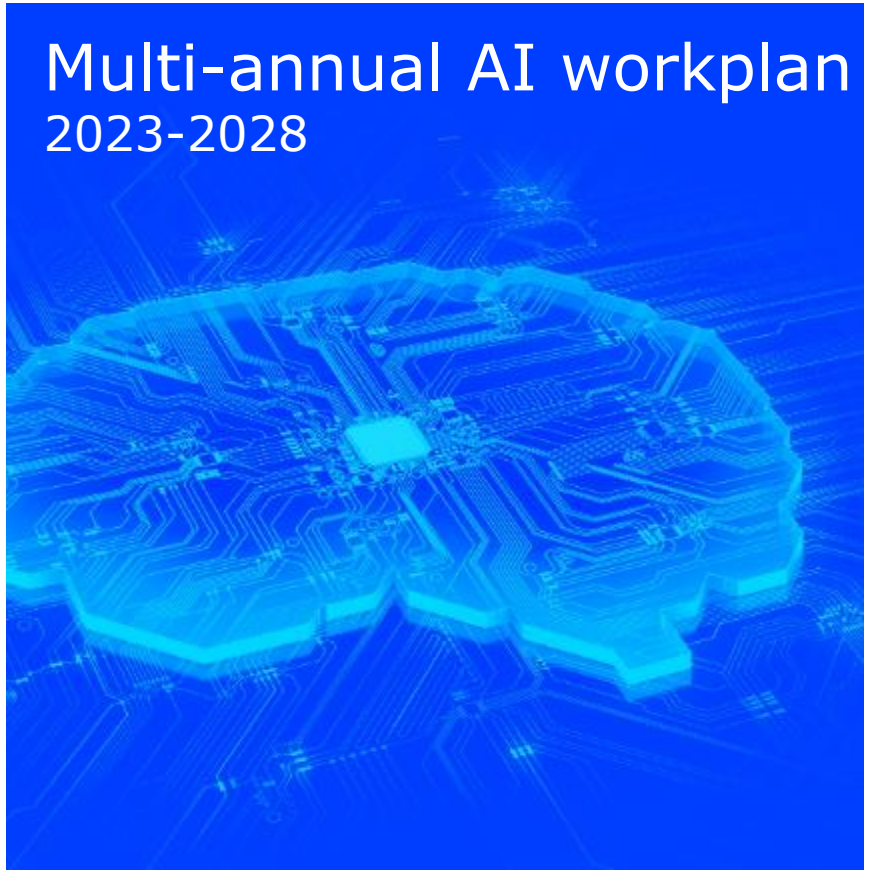
- Roll-out of knowledge mining tools
- Use cases to build Network knowledge mining roadmap

Collaboration & change management

- Public AI workshops
- Network masterclasses on AI

Experimentation

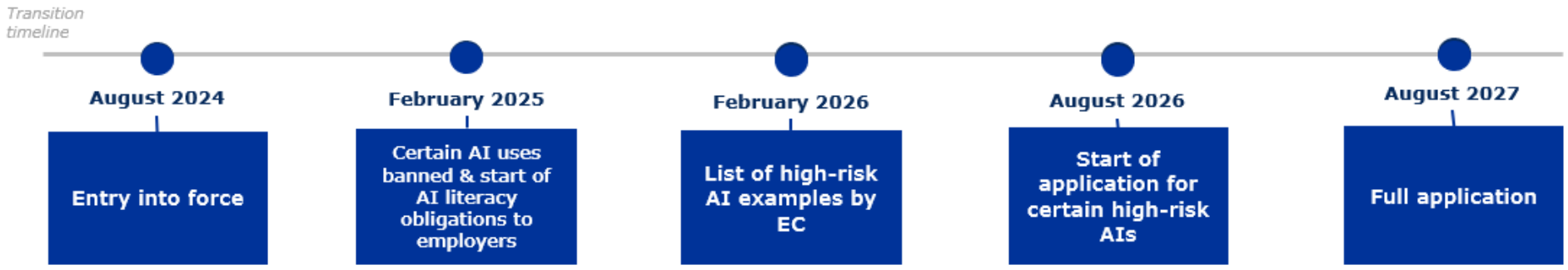
- Network AI technical deep dives
- Network AI experimentation



The EU AI Act

- Phased implementation.
- First measures are to catalogue tools and uses + roll out AI literacy for staff.
- **NDSG will support as important legal obligations apply**.

AI Act implementation timeline



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2024 Observatory: A snapshot of EMRN experience by 2024

- **Product related**
  - 7 Qualification procedures
  - 4 Scientific advices
  - 4 ITF meetings
  - 8 Portfolio and Technology meetings

    + Applicants' feedback following a call for insights on AI use in drug development
  - A review of peer-reviewed journals, preprints and EU-funded research
- **AI for personal productivity or automating processes and system**
  - 26 Tools reported (half by 6 National Agencies)

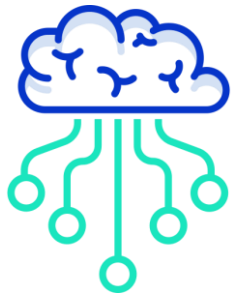
2024 AI Observatory

- Growing interest in discussing AI throughout the medicine life-cycle, with a preponderance in the pre-authorisation stage

Examples of AI applications in medicine life-cycle discussed within the EMRN	
Scope of application	**Publication related to specific AI use**
• Manufacturing process monitoring and control • Discovery of drug target • Imaging and histological analyses • Patient selection in clinical trial • Event adjudication • Predictions (of prognostic covariates, response or adverse event) • Digital Twins • Precision dosing • Pharmacovigilance (Adverse event detection and reporting) • Leveraging of RWE to complement clinical trials	• Preliminary QIG Considerations regarding Pharmaceutical Process Models • Statistical adjustment on deep learning prognosis covariates obtained from histological slides • Draft qualification opinion for artificial intelligence-based measurement of non-alcoholic steatohepatitis histology in liver biopsies to determine disease activity in NASH/MASH clinical trials

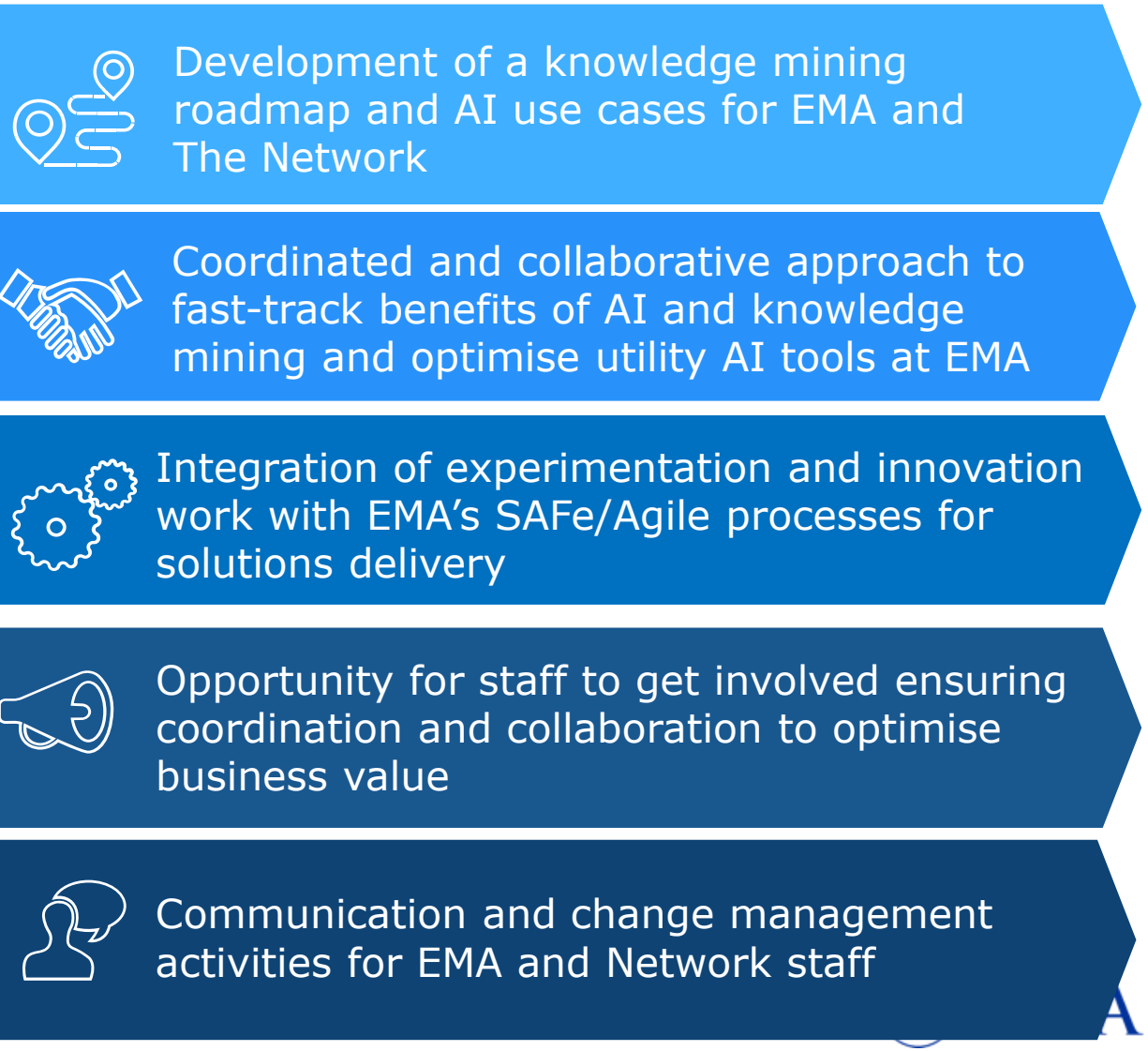
- In term of automating processes and enhancing systems or personal productivity, the Network is primarily developing AI Knowledge Mining capabilities tools.

Knowledge Mining roadmap



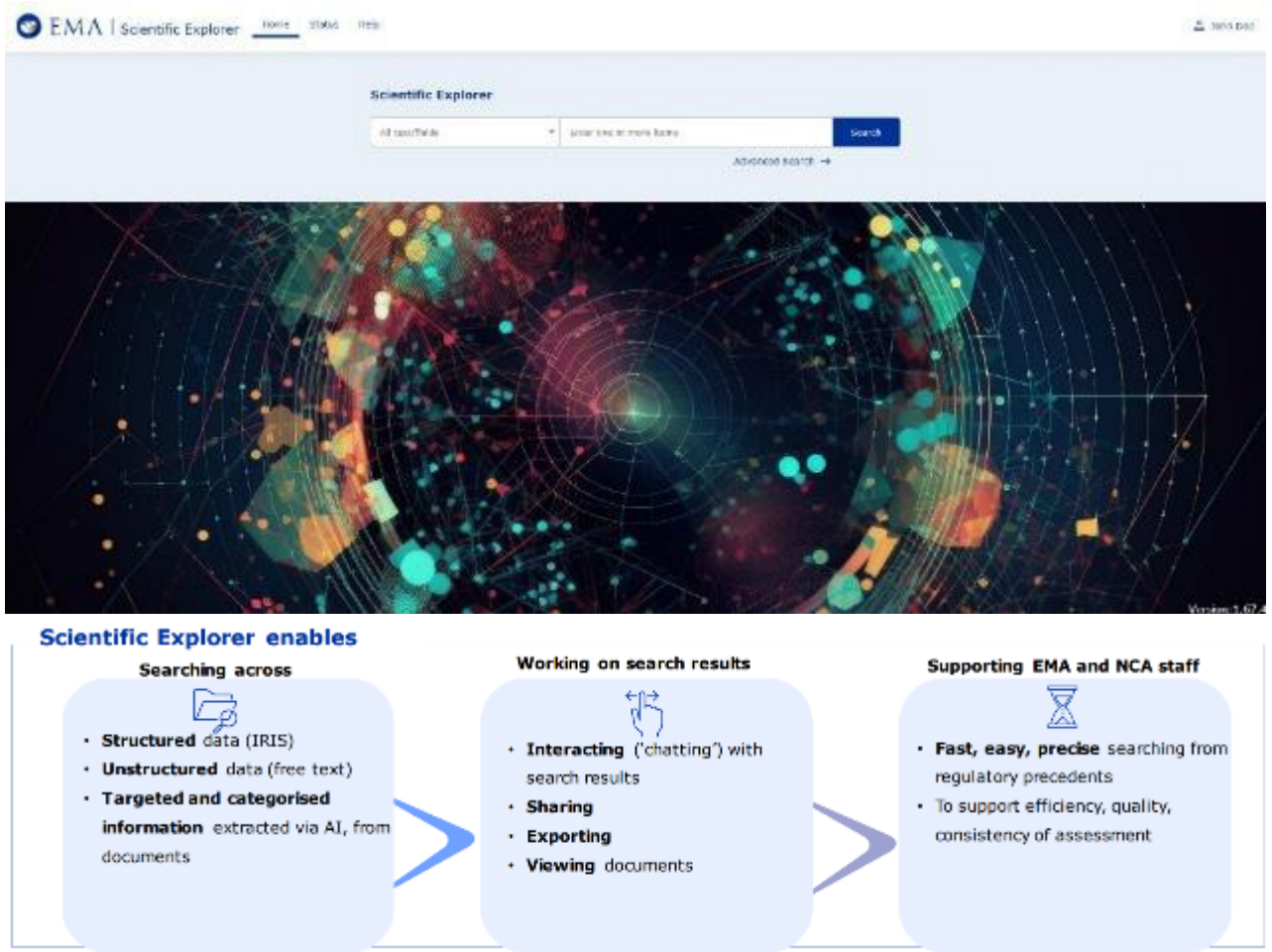
AI/Knowledge mining initiative

- Aims to develop **knowledge mining capabilities** by leveraging the information at Networks disposal utilising **AI**
- Supports EMA and the Network in **optimising efficiency and quality** in all aspects of their work for the benefit of human and animal health



AI-enabled knowledge mining tool for EU regulators: Scientific Explorer

- The AI-enabled knowledge mining tool called Scientific Explorer for EU regulators rolled out on 4th March 2024 .
- The tool enables easy, focused and precise search of regulatory scientific information from network's sources to support decision-making and simplify processes.
- The first release focuses on scientific advice procedures for human medicines.
- Available to the Network here.



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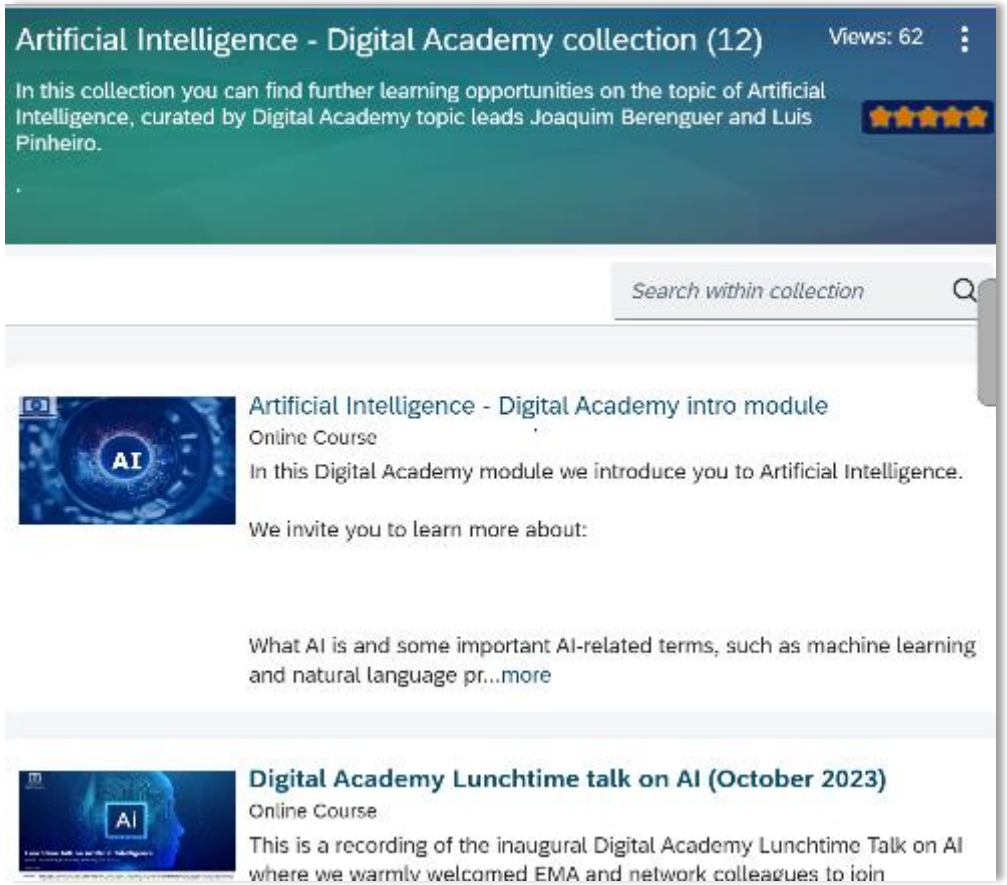
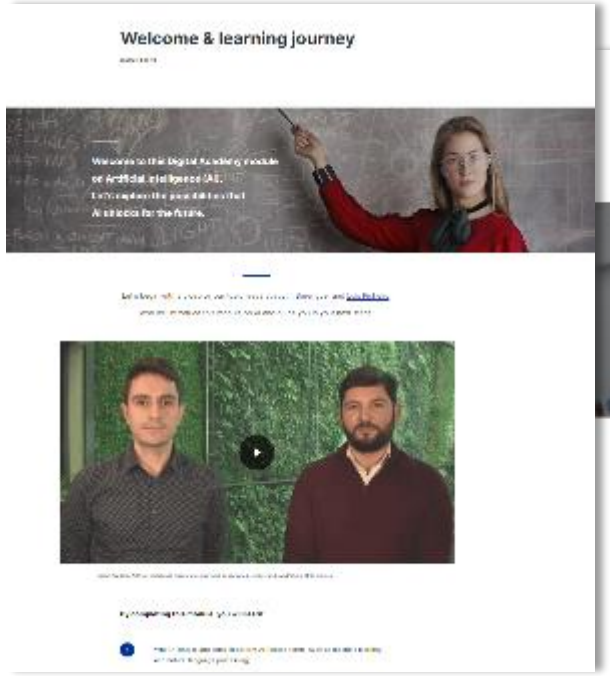
Digital Academy module and learning collection on AI



AI learning collection



AI Intro module

Upcoming:

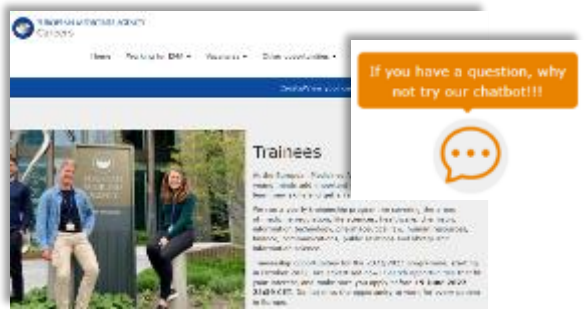
- ✓ Continuously expanding collection of learning resources on AI

Examples of digital innovation and experimentation at EMA

Exploit novel digital technologies	Apply process analytics, AI and automation	Identify digital solutions & horizon scanning	Redesign or optimise processes for digital solutions

Virtual reality

Chatbots

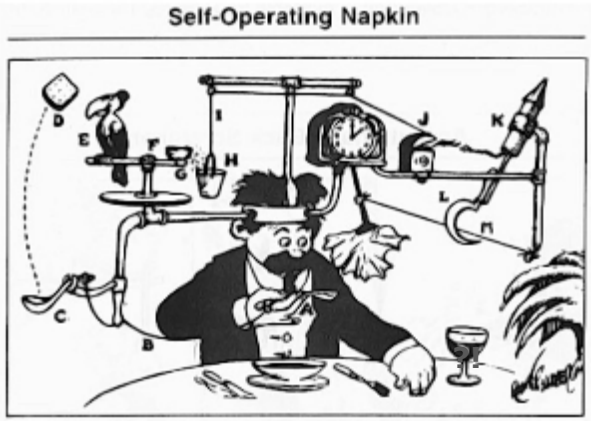


Document & Image processing

Digital business cards

Artificial intelligence & robotics

Horizon scanning



Streamline before automating

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Vielen Dank für Ihre Aufmerksamkeit

peter.arlett@ema.europa.eu

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