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HMA/EMA Annual Data Forum 2025

Report of the HMA/EMA Annual Data Forum 2025 – 9 December 2025

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Introduction

The European medicines regulatory network ('the Network') continues to advance its efforts to harness the power of regulatory and health data, to drive innovation, support evidence-based decision-making, and ultimately benefit public and animal health.

Building on the last five years of progress under the Big Data Steering Group, this annual forum – now coordinated by the Network Data Steering Group – offers an opportunity to engage with regulators and stakeholders from across Europe and share progress and insights to co-create the future of data use in medicines regulation.

This year's forum took place on 9 December 2025 at the EMA building in Amsterdam and aimed to:

- strengthen collaboration by enabling stakeholders and partners to share their views, priorities and needs to inform the update of the HMA-EMA Network Data Steering Group workplan, aligned with the data-related activities of the Network Strategy to 2028;
- showcase progress in evidence generation, data interoperability, and the use and exchange of data across the EU network.
- foster dialogue through inspirational keynote speakers and discussion.

More than two hundred stakeholders and partners participated in the Forum and many hundreds followed the broadcast of the event online.

This report provides a high-level summary of the forum presentations as well as highlights from the comments and discussion with stakeholders.

Opening remarks

The forum was opened by EMA Executive Director Emer Cooke. She emphasized the Network transition from an information-based regulatory system to a data-driven approach that leverages diverse data sources and technologies including AI for faster and safer medicines regulation. She highlighted progress made already through critical initiatives such as DARWIN EU®, clinical trial data analysis pilots, and ongoing collaboration on AI-driven process automation, knowledge mining for decision support, as well as the expanded role of the Network Data Steering Group in data governance and interoperability. She reminded the audience how this forum aligns with the European Medicines Agencies Network Strategy to 2028 (EMANS 28), which prioritizes data, digitalization, and AI to build a connected medicines data ecosystem and prepare for future innovation.

The keynote, 'From Insight to Evidence: Strengthening the role of patient data in regulatory decision-making,' was delivered by Mencía de Lemus Belmonte (SMA Europe; Patient Representative on EMA's Committee for Advanced Therapies). She highlighted the critical role of patient data in shaping regulatory decisions and improving health outcomes. Drawing on her experience as a patient advocate, she illustrated how the evolution of data collection in spinal muscular atrophy — from fragmented registries to structured, patient-driven datasets — transformed drug development and access. She highlighted the initial challenges, such as registries designed for clinical classification rather than regulatory use, and explained how patient communities mobilized to create fit-for-purpose registries, validate patient-relevant outcomes, and influence trial protocols and marketing authorizations.

Mencía emphasised that meaningful patient input has enabled regulators to understand real-world needs, such as minimal functional gains and treatment feasibility, which were often overlooked in traditional endpoints. Despite the EU regulatory Network progress and efforts, she identified persistent gaps: lack of proactive engagement from regulators with registry holders, insufficient trust in patient-

generated data, and limited ambition in leveraging advanced technologies. She called for early and sustained collaboration between regulators and patient organizations, systematic integration of patient input in all stages of assessment, use of technologies to reduce burden and accelerate insights and take bold steps to use patient data. Her closing message was clear: more engagement, more patient data use and trust and transparency to underpin a data-driven regulatory future that truly addresses patient needs.

Session 1: Transformation to a data-driven regulatory system: from plan to practice

Session 1 translated the EMRN strategy into delivery and highlighted the key progress of the Network in delivering actions that drive innovation and support evidence-based decision-making.

Peter Arlett, presenting in his role as NDSG co-chair, highlighted eleven months of achievements of the NDSG 2025- 2028 workplan:

- The NDSG was established in early 2025, guided by its vision on data ('*Trusted medicines by unlocking the value of data*') to optimise the use and management of data in medicines regulation.
- the first EMRN Data Strategy providing common principles and strategic objectives for data governance, quality, interoperability and ethical use;
- the continued scale-up of DARWIN EU ® as a key enabler for generating evidence from real-world data (RWD) for regulatory decision-making — now exceeding one hundred initiated studies across thirty-one data partners in sixteen EU countries with access to ~180 million patient records. Key impactful studies have been delivered in 2025 in oncology, RSV disease epidemiology, paediatric disease natural history for autoimmune conditions, and supply/shortages analytics.
- exploring the potential of genetic information to improve treatments and patients care.
- the transition from AI promises to AI practices with safe and targeted application. Achievements include key Network collaboration on AI use case prioritization and delivery planning, an AI tools framework for safe collaboration, AI literacy plans via the EU Network Training Center, an AI observatory report, and public consultation on research priorities alongside international alignment with the US FDA on drafting AI principles and terminology;
- Key recommendations for harmonising the implementation and use of PMS medicinal product master data in the Network.
- and continued collaboration with stakeholders and partners and progress on experts' guidance and international harmonisations.

Discussions with stakeholders' representatives included the learnings and future priorities from regulators, pharmaceutical industry, healthcare professionals (HCP), heath technology assessment (HTA) bodies, veterinary representative and academia.

Regulators acknowledged the progress made through DARWIN EU, while emphasizing that minimizing bias and ensuring robust uncertainty quantification will be critical for future evidence generation. They highlighted new opportunities arising from upcoming pharmaceutical legislation, particularly in enabling access to raw clinical study data, and emphasised the need for pragmatic guardrails to harness the impressive potential of AI responsibly.

Industry commended EMA and the EMRN for significant progress in real-world evidence, particularly through DARWIN EU, and praised the strong leadership provided in data governance and AI. Industry emphasized the EU's potential to set a global example in AI regulatory policy and clinical trial reforms, advocating for an integrated approach to data-driven regulation that combines real-world data, patient experience, and digital innovation. Highlighting the importance of partnerships, they noted how DARWIN EU feasibility assessments fostered dialogue on optimizing data quality and linkage across Europe. Finally, industry emphasised that sustainable progress requires clear, globally aligned regulatory guidance and supportive policy levers to maintain Europe's competitiveness and leadership in clinical trials and innovation.

Health Care Professionals (HCPs) emphasised that collaboration is essential for successful projects, noting that technology and real-world evidence pipeline alone are insufficient without meaningful data. They emphasised the importance of clinically relevant data combined with transparent, sustainable organizational support and adequate funding. Finally, they highlighted that balancing quality data, skilled AI teams, and strong organizational structures is key to achieving impactful results.

The veterinary network perspective highlighted the sector's unique challenges such as limited resources, cost constraints, and the need for pragmatic approaches. Veterinary regulators emphasized the strength of the Union Product Database (UPD) for veterinary medicinal products, supported by EMA's data quality framework, which ensures structured, high-quality data exchange and reuse of product data for pharmacovigilance and antimicrobial monitoring. In terms of AI adoption, the veterinary field leverages practical solutions and strong community collaboration to achieve high impact with minimal investment. Looking ahead, they noted growing importance of data governance and reuse.

For HTA, the critical role of data was emphasized, advocating for early patient registry participation to build historical datasets. The representative praised DARWIN EU's progress. AI and new tools require robust data and sound methodology to avoid creating an illusion of progress, stressing their potential to answer current questions more efficiently. Education, collaboration, and steady advancement as essential for leveraging AI effectively in the future.

For Academia, legal compliance must be embedded in technological design, ensuring interoperability and AI deployment align with data governance requirements. To manage a regulatory transition period, flexible "transitional compliance architectures" are needed to meet current GDPR obligations while preparing for new regulations like the AI Act and European Health Data Space. Finally, there is a need to address the complex debate on anonymization and data minimization, noting recent EU court rulings and the need to balance privacy with sufficient data granularity for healthcare innovation.

Session 2: Opportunities in Data, Policy, and Ethics

Session 2 explored opportunities of upcoming European legislations for faster and better access to medicines, and ethical foundations needed to guide innovation and use of data responsibly.

The European Commission speakers provide an overview of the European Commission's legislative and strategic initiatives to enable a data-driven regulatory ecosystem. Key EU regulations discussed included:

- the European Health Data Space (EHDS), aims to empower individuals with access and control over their health data, enable secondary use for research and regulatory purposes, and create a single market for interoperable electronic health records systems.

- The AI Act was highlighted as the world's first comprehensive regulation on artificial intelligence, introducing a risk-based approach and fostering safe, effective deployment of AI in healthcare through projects like Compass AI and SHAIPED.
- The Health Technology Assessment (HTA) regulation, promotes a common framework and procedures for cooperation of Members States on health technologies, strengthens data requirement for joint clinical assessment by promoting standardized evidence generation and encourages cross-border data sharing and reuse to accelerate access to innovative technologies.
- The upcoming new EU pharmaceutical legislation will modernize regulatory processes with digital-by-default submissions, enabling the use of AI, the submission of raw data to support scientific assessment, more systematic use of real-world evidence to support regulatory decision-making, and a pathway towards electronic product information (ePI).
- Finally, the Medical Device Regulation reinforces the trends towards more evidence generation and strengthens traceability, post-market surveillance, and cybersecurity.

Collectively, these initiatives form a coherent strategy to modernize EU medicines regulation, foster innovation, and build a resilient, patient-centred health ecosystem across Europe.

Alessandro Blasimme (ETH Zürich University) explored how technological advances such as genome sequencing, pervasive data collection, and AI are transforming health data ethics and governance. The last 15 years have seen a transition from precision medicines to learning from common patterns of data, through AI. The expansion of data sources — from hospitals and registries to mobile apps and living environments — creates uncertainties around ownership, access, storage, and permissible uses, alongside risks of bias, discrimination, and privacy breaches. This transition has created the necessity to rethink the ways in which we conceptualize and understand the ethics of collecting using and reusing human data for purposes of healthcare.

To navigate these challenges, he proposed six guiding principles for data governance to navigate these uncertainties: adaptivity to evolving technologies, flexibility to adopt oversight mechanisms, continuous monitoring for new vulnerabilities, responsiveness to mitigate harms, reflexivity to scrutinize assumptions and biases in research practice and regulatory decision-making, and inclusiveness to involve diverse stakeholders, including lay publics and under-represented stakeholders.

Blasimme discussed then the three dimensions of data control: data governance, data access and data use. In this context, informed consent alone is insufficient in this complex ecosystem and participatory governance models, data portability, and shared oversight mechanisms are needed to empower individuals and foster trust. The EHDS can promote such principles and should integrate ethical best practices and possibly establish an ethics coordination board to ensure transparency and co-creation. Ultimately, he concluded that regulation must be complemented by collaborative innovation and value-driven governance to achieve responsible health data use and give value back to data subject in Europe.

Discussions with stakeholders' representatives included their view on how to exploit these new data, policy and ethics opportunities from regulators, pharmaceutical industry, Health Data Access Bodies representative and patients.

For regulators, this new landscape is an opportunity to continue to bring innovative and safe medicines to patients, with greater access to data representatives of all the EU population and with more support to innovation in Europe via the regulatory sandbox, promoting greater dialogue between developers and regulators. The success of these different pieces of legislations will depend on the dialogue

between these different regulatory frameworks - as medicines of the future will include a combination of medical device and technologies – and adaptability/agility of the EU regulatory network.

For Industry, Europe has the richest health data ecosystem in the world and the EHDS will help Europe to boost the use of data for innovation. Industry discussed what a future landscape will look like in which patients can have access to their standardised data 'library', helping Industry to design and complement clinical trial data and helping physicians and patient to compare their treatment outcomes.

For the representative of Health data Access Bodies, the future also looks optimistic. The community of practice on the health data access bodies is an example of European collaboration to support Members States for the implementation of the EHDS. Inspired by DARWIN EU ®, the future of data is federated, based on open common data standards, open common data model and federated data standards. This will ensure data is interoperable, allow to lower the cost of entry for research and enable the conduct of federated studies form different members states.

Patients applauded these new EU initiatives. For patients, the right treatment for the right patient at the right time is the goal.

Session 3: Building a Connected Medicines Data Ecosystem

Session 3 discussed the recently published first data strategy for EU medicines regulation, a major step in accelerating EU regulatory agencies use of data for impactful decisions and improved patient and animal health. The session also explored the use of medicinal product master data as one of the key pillars to enable data to drive better regulation.

Georg Neuwirther (AGES) presented the EMRN Data Strategy, emphasizing its vision of 'trusted medicines by unlocking the value of data'. It began with an interactive poll with stakeholders asking what data aspect matters the most for realising this vision on data. Trust, quality, reliability and interoperability were fed back as top priorities, reinforcing the need for a common strategy. Georg outlined why the strategy is needed and some of the key challenges working with data, such as fragmented processes, inconsistent standards, and manual, error-prone workflows, stressing the importance of collaboration across data creators, users, and decision-makers.

The strategy is built around core principles that include accessibility, security, ethical use, interoperability, and treating data as a strategic asset, with an emphasis on catalogue-based metadata and minimising free text to enable automation and multilingual exchange. Its shared objectives include: robust network data governance; fit-for-purpose data quality management; interoperability grounded in ISO IDMP and SPOR services (Substances, Product, Organisations, Referentials); catalogue/metadata management; workforce training and literacy; and turning data into analysis for decisions.

Illustrative testimonies from patients, clinicians, human and veterinary national regulatory agencies, CMDh, industry, national Electronic Health Record and EMA Network IT Portfolio echoed the strategy's importance for improving regulatory efficiency, accelerating patient access to medicines, enabling AI adoption, building trust, enhance collaboration, and create a future-ready, data-driven regulatory ecosystem across Europe.

Aimad Torqui (CBG-MEB) then focused on medicinal product master data (PMS) as a pillar for smarter regulation and transforming regulatory operations into a data-driven ecosystem aligned with the EU Network Strategy to 2028.

He emphasized the importance of product data management and consistency across the entire product life cycle—from development and clinical trials to regulatory business processes and commercialization—while reducing manual interventions and errors through automation.

The Network PMS recommendations published in May 2025 outline key principles for creating a unified, trusted repository of medicinal product master data, enabling single submissions, interoperability, and data validation across EU and national systems. In this context, he highlighted the key challenges of qualifying large volumes of legacy data, harmonizing PMS data with static national databases, and estimating the effort required for data quality improvements.

Complementary to the regulatory use cases, unlocking the value of PMS data is essential for the public to provide clearer information to patients, to enable safer, smoother and more consistent digital prescribing and dispensing across EU, and finally to the EHDS regulation and its objectives.

PMS should evolve from static archives to a living system supporting real-time decision-making and innovation. Regulating data on medicines will accelerate innovation, improve patient care, and strengthen trust in regulatory decisions.

Session 4: Evidence Generation for Regulatory Decision-Making: What if we were only just beginning?

Session 4 challenged participants to think beyond current frameworks for evidence generation and explored through a series of seven 'pitches' how innovative methods and new types of data could enhance medicines regulation and regulatory decision-making.

1. Patient-generated data and registries (François Houyez, EURORDIS) demonstrated real-time, representative insights on patients' needs, treatment outcomes and national healthcare and regulatory systems performance, reducing burden on patients while collecting meaningful endpoints for drug developers. This might be essential for future health crisis preparedness.
2. Innovative clinical trial designs (Katrien Oude Rengerink, CBG-MEB) combined the advantages of randomization with increased efficiency and reduced patient burden — provided data quality and operational realism are respected. They leverage registry-based/pragmatic elements, AI to support conduct and outcomes evaluation, augmentation with external controls and use of digital tools to capture endpoints data. More collaboration is needed amongst stakeholders.
3. Organoids (Janaki Raman, VPH Institute), three-dimensional cell cultures grown in controlled environments that enable drug screening, repurposing, and personalized drug selection. Key challenges remain, including ensuring reproducibility, achieving standardization, implementing automation, and managing high costs. Digital twins and synthetic data are based on the use of computational models and help integrate data from various sources across different scales. They offer personalised predictions to transform data into actionable insights to conduct in sillico clinical trial.
4. AI in clinical trials (Enkeleida Nikai, Sanofi/EUCOPE) is increasingly used to optimize patient recruitment, protocol design, data management, risk-based monitoring and outcome adjudication and predictive analysis. In non-interventional studies, AI enhances accuracy, scalability, and efficiency when dealing with complex observational data, particularly for

endpoint operationalization, confounder detection and target trial emulation to strengthen causal inference.

5. Digitalized clinical outcomes, behavioural/lifestyle and mobile health data (Ebony Dashiell-Aje, BioMarin) showed how they can unlock faster evidence generation, provide a more sensitive assessment of patients' benefits and give unprecedented visibility into real-world effectiveness and ultimately faster patient access. International regulators have recognized this shift by issuing frameworks, methodological guidance, and real-world case studies to design and validate technology-derived endpoints. Globally, sponsors are developing over five hundred digital endpoints for use in both interventional and non-interventional studies. Validated digital endpoints can increase sensitivity, efficiency, and representativeness across therapeutic areas. Digital health is no longer experimental; advancing science in real time now requires greater standardization, harmonization, and global collaboration.
6. Personalized medicines data, including omics (Professor Sir Munir Pirmohamed, University of Liverpool/MHRA), underscored the shift from population-based evidence to individualized treatment through advanced data and technologies. Pharmacogenomics is the leading approach for personalized drug response, but challenges remain in scaling its use across Europe. Emerging areas such as polygenic risk scores, proteomics, and multi-omics are generating a vast amount of data, requiring integration with clinical information through multimodal algorithms to improve patient outcomes. Regulatory acceptance will depend on evidence from randomized trials or, where impractical, robust observational studies supported by replication and real-world implementation within learning health systems. Ultimately, the convergence of omics with AI and digital tools is pivotal for enabling precision medicine and shaping future regulatory frameworks.
7. Modelling & Simulation (Flora Musuamba, FAMHP/EMA) emphasized modelling and simulation as a set of computational tools that transform diverse data into evidence for regulatory decisions. Modelling & Simulation applications includes mechanistic models for predicting drug efficacy and safety, biostatistical and epidemiological models for trial optimization, and extrapolation models for small populations. These approaches integrate clinical, non-clinical, and real-world data to improve decision-making. It is needed to combine these models with AI and digital technologies while ensuring rigorous evaluation to maintain reliability.

The session concluded with a shareholder vote on which innovative methods or new data types hold the greatest potential to strengthen future regulatory decision-making on medicines. The vote showed support across the different innovative approaches and the personalised medicine/omics pitch received the highest ranking. The plenary reflections emphasised that these approaches are complementary and should be integrated according to the research question, data maturity, and regulatory context.

Closing keynote

The closing keynote, 'Healthcare beyond human capabilities,' by Ignacio H. Medrano (Savana/Mendelian), explored the future of AI in healthcare, explaining how machine learning — based on pattern recognition and learning by repetition — has transformed medicine by enabling systems to outperform humans in tasks like image interpretation and diagnostics. He highlighted AI's growing role in classification and forecasting, with applications ranging from predicting breast cancer risk to anticipating Alzheimer's decades in advance, supported by large-scale health data initiatives such as the UK's predictive modelling of two hundred diseases. Medrano emphasised the importance of multimodal data integration — combining genomics, imaging, clinical notes, and real-world data — to unlock precision medicine and optimize trial design. While validation and data readiness remain key challenges, he noted that existing regulatory methodologies can accelerate adoption. Looking ahead,

he distinguished between discriminative AI for prediction and generative AI for autonomous decision-making, envisioning future "AI companions" that work alongside clinicians and regulators. His closing message emphasized that success depends not only on technology but on asking the right questions to harness AI's full potential.

Closing remarks

In summarising the day, Peter Arlett reflected the Forum's throughline:

- We need to hear the patient voice and leverage patient registries for better decision on Medicines.
- The patient perspective is key to scientific advice on the development of medicines.
- We are moving to data driven medicines regulation that supports a fast path from innovation to safe and effective medicines for patients.
- We need to continue to build on the achievements of DARWIN EU in unlocking the value of real-world data.
- We have a toolbox of EU legislation available to build EU leadership on data.
- Intelligent use of data can help get the right medicine to the right patient at the right time.
- We are starting to benefit from healthcare beyond human capabilities.

Participants were thanked for their contributions—on site and online—and invited to follow the work of the NDSG next year looking at the NDSG's upcoming update of its multi-annual workplan.