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The Global Pharmaceutical Medicine Journal

INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS AND PHARMACEUTICAL MEDICINE

THIS ISSUE INCLUDES:

When Exceptions Make the Rule: Compassionate and Named Patient Use in Austria

Insights from the GPMed Seminar of 27 March 2025, Vienna, Austria

On 27 March 2025, the Austrian Society for Pharmaceutical Medicine (GPMed) hosted a highly anticipated seminar on the use of medicinal products outside their approved indications. Held at the Auditorium Centre of the Vienna General Hospital (AKH), the event drew an interdisciplinary audience including physicians, pharmacists, regulators, and clinical researchers. With the theme “Use of Medicines Outside of Marketing Authorisation – What Is Permitted and When?”, the programme offered a comprehensive exploration of regulatory pathways such as Compassionate Use Programmes (CUP), Named Patient Use (NPU), and Off-label Use in Austria.

Understanding the Role of Authorisation in Clinical Practice

The seminar opened with remarks from Professor Dr Markus Zeitlinger, Vice President of GPMed, who welcomed attendees and underscored the importance of regulatory literacy in clinical decision-making.

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Magister Daniel D'Orlando, representing the Austrian Federal Ministry of Social Affairs, HealthCare and Consumer Protection, provided a detailed overview of the legal framework governing the use of medicines in Austria. He highlighted that, while marketing authorisation ensures quality, safety, and efficacy, legal provisions such as the EU Regulation (EC) No 726/2004 and the Austrian Medicines Act (AMG) also permit exceptions. These include clinical trials, compassionate use, named patient use, magistral preparations, and import regulations under the AWEG (approval for the use of an individual medicinal product in a single patient in Austria).

Of particular note was the clarification that NPU does not require prior approval from the authorities, placing the responsibility squarely on the treating physician. Off-label use, meanwhile, is not prohibited under Austrian law and may be practiced if supported by scientific evidence, though physicians assume expanded duties of patient information and legal liability.

Compassionate Use in Austria – A Regulatory Lifecycle

Dr Corina Spreitzer, Clinical Trials Assessor at the Austrian Medicines and Medical Devices Agency (AGES MEA), provided a deep dive into the regulatory framework surrounding CUP in Austria. Referencing Article 83 of Regulation (EC) No 726/2004 and §8a AMG, Dr Spreitzer outlined the circumstances under which CUP may be applied: for patients with life-threatening or seriously debilitating diseases lacking satisfactory authorised treatments.

Dr Spreitzer walked participants through the application process which includes treatment protocols, informed consent documents, and a multi-step review by AGES and emphasised that the European Medicines Agency (EMA) must be notified of all approved CUPs. Practical details such as communication of safety updates or programme modifications are facilitated via the dedicated email address: compassionate-use@basg.gv.at.

Her presentation underscored the utility of CUPs as a mechanism to bridge the gap between clinical need and regulatory timelines, enabling timely access to promising therapies.

Clinical Realities: Individual Treatments and Off-label Use at AKH Vienna

The afternoon's final session brought the regulatory concepts into practical focus. Professor Dr Gabriela Kornek, Medical Director at AKH Vienna, and Magister Martina Anditsch, Head of Pharmacy, presented an institutional framework for NPU and Off-label Use implementation. They described a personalised process designed to ensure medical appropriateness and economic sustainability. Applications may be submitted for first-time use at AKH, off-label use, high-cost therapies, or individual treatment trials, following defined submission criteria and involving review by the Evidence-based Treatment Methods Committee (GED) of the Vienna Healthcare Group (WIGEV).
Approval decisions incorporate expert evaluations from tumour boards or the Precision Medicine Board and may include the use of patient-reported outcome measures (PROMs). The evaluation of therapeutic response is conducted after 3 to 6 months through medical assessment and documentation.

GPMed
GESELLSCHAFT FÜR PHARMAZEUTISCHE MEDIZIN E.V.

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Real-world case studies ranging from salivary gland cancer to HIV (human immunodeficiency virus), organ transplantation, and cervical cancer illustrated the vital role of these pathways in enabling personalised care when standard options are exhausted.

A Balancing Act: Regulation and Flexibility

The GPMed seminar highlighted the critical balance between regulatory oversight and clinical flexibility. While marketing authorisation remains the gold standard for ensuring patient safety and therapeutic efficacy, exceptions such as CUP, NPU, and Off-label Use serve as essential tools for addressing unmet medical needs.

The event provided attendees with practical tools and legal clarity to navigate this complex landscape and reinforced the collaborative roles of regulatory agencies, clinicians, and hospital systems in safeguarding patient outcomes.

As regulatory frameworks evolve, especially under the EU's Pharmaceutical Strategy for Europe, continuous dialogue between stakeholders remains crucial. Events like this seminar not only build knowledge but also foster a shared responsibility for innovation, access, and care in Pharmaceutical Medicine.

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The Role of the Medical Science Liaison (MSL) in the Italian Pharmaceutical Industry

The Role

A Medical Science Liaison (MSL) is a field-based professional operating within the Medical Affairs division of the pharmaceutical industry. The MSL's primary role is to act as a liaison between the pharmaceutical company and the wider medical and scientific community. Internally, they connect the medical department with various stakeholders, including marketing, sales, market access, regulatory affairs and clinical research. The MSL plays a crucial role in raising awareness of clinical needs, supporting the development and dissemination of relevant scientific evidence, and introducing new therapeutic options.

In recent years, the MSL role has become increasingly widespread and is now recognised as a key player in the impartial, bidirectional exchange of medical-scientific data throughout the entire drug lifecycle from research and development to post-marketing and, in some cases, beyond to patent expiration.

The Survey

To better understand the current context and future perspectives of the MSL role in Italy, the SIMeF Clinical Research and Medical Affairs (RICMA) working group conducted an exploratory survey. The aim was to examine various facets of the role, including professional education, core competencies, and performance evaluation metrics.

The survey was web-based and distributed via a public link shared on LinkedIn. Inclusion criteria were strictly limited to professionals currently employed in Medical Affairs within the pharmaceutical sector.

A total of 103 responses were collected from individuals working in Medical Affairs departments across companies active in Italy. Notably, 97% of participants are employed by multinational pharmaceutical companies. Less than 1% each reported working for a national pharmaceutical company, a biotech company, or a food company producing medical nutrition products. No respondents reported employment within Contract Research Organisations (CROs).

Of the participants, 91% work as MSLs, while the remaining 9% have hybrid roles combining field-based and office-based responsibilities. In terms of educational background, most MSLs (44%) have degrees in STEM (Science, Technology, Engineering and Mathematics) subjects, followed by 14% who have degrees in Pharmacy or Pharmaceutical Chemistry and Technology (CTF). Only 9% of MSLs are physicians, which contrasts with data from the Accreditation Council for Medical Affairs (ACMA). Furthermore, 24% of respondents have completed postgraduate Master's programmes, highlighting the importance of advanced or specialised training in acquiring scientific and technical competencies. Nevertheless, the data show that academic education alone is not enough to fully prepare professionals for the MSL role.

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Another noteworthy finding is that 54% of respondents transitioned into the MSL role from commercial functions. This trend may reflect evolving corporate strategies focused on internal career development. However, in certain organisations, MSL roles are reserved exclusively for physicians who are specialised in the relevant therapeutic area.

In terms of professional experience, 38% of MSLs had been in the role for more than five years, 42% for between two and five years, and 20% for less than one year.

Performance assessment of MSLs commonly includes Key Performance Indicators (KPIs) or Medical Performance Index (MPI) measures, applied across both quantitative and qualitative dimensions. Among quantitative KPIs cited by more than 40% of respondents were: the number of hospital medical meetings (41%), the number of Key Opinion Leaders (KOLs) aligned with scientific messaging (43%), the percentage of days dedicated to scientific interactions (55%), the number of medical insights collected (59%), and the total number of scientific engagements (71%).

Regarding time spent on field-based activities, 14% of MSLs reported spending less than 50% of their time in the field, 44% reported up to 70%, and 42% spent more than 70% of their time engaged in on-site activities.

The most frequently applied qualitative KPIs were actionable insights (47%) and feedback from managers or internal stakeholders (67%). When asked to compare the importance of qualitative and quantitative KPIs in overall performance evaluations, 50% of respondents said they were both assessed equally, while only 21% said qualitative KPIs were given more weight.

Development and Future Challenges

Professional growth and development are essential pillars for consolidating the MSL role. The main challenges highlighted by survey participants were the need for ongoing scientific education and managing the communication of extensive clinical data. To address these challenges, 48% of respondents reported attending public speaking courses and 64% participated in soft skills programmes focusing on leadership, emotional intelligence and time management.

When asked to identify the top priorities for professional development, 69% selected Real World Evidence, 50% chose clinical research, 30% opted for the drug development and access pathway, and 33% emphasised the importance of enhancing digital skills for the future. These results underscore the increasingly multidimensional nature of the competencies required of MSLs, who must now be equipped with scientific expertise as well as advanced technological capabilities.

Indeed, MSLs are already integrating digital tools into their daily activities, including clinical data analysis and multichannel communication with stakeholders, often via virtual platforms for individual or group meetings. Leading companies are also developing and implementing artificial intelligence (AI) tools to optimise time management, particularly for routine or lower-impact tasks. Some innovative companies have introduced generative AI solutions for data summarisation, text translation, and critical analysis of scientific evidence.

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Finally, there is a growing demand for formal certification of MSL competencies. In fact, 44% of respondents expressed interest in obtaining certification upon course completion, while 33% would prefer a certificate of attendance. This indicates a clear trend towards formal recognition of professional development, underscoring the importance placed on continuous learning and career advancement.

This SIMeF survey is the first published investigation in Italy to include such a large sample of participants, providing valuable insights into the challenges and expectations faced by MSLs. It shows that the role is firmly established within the pharmaceutical industry, yet it is also evolving, particularly regarding the growing need to combine scientific expertise with digital and communication skills. A McKinsey analysis on the future of Medical Affairs by 2030 supports this trend, highlighting that, even as they spend significant time on digital platforms both during and outside working hours, healthcare professionals still primarily engage with Medical Affairs teams through traditional channels, such as MSLs. Despite the growing use of digital tools and evolving communication technologies, McKinsey predicts that MSLs will remain the primary point of contact for scientific engagement and discussion.

In conclusion, the findings reveal that pharmaceutical companies have a major opportunity to invest in the professional development of MSLs by equipping them with the necessary tools to meet future challenges and maintain their central role in the medical-scientific landscape.

Discussion

To our knowledge, this represents the largest survey conducted in Italy to date specifically focused on the role of the MSL, with over 100 participants offering a comprehensive snapshot of the profession across educational backgrounds, performance metrics, developmental needs, and future perspectives. The findings confirm that the MSL role is firmly established within the Italian pharmaceutical industry, primarily among multinational companies, and is evolving in response to increasingly complex scientific and strategic demands.

The use of KPIs or MPI to assess MSL activities is widespread, reflecting a balanced focus on both quantitative outcomes, such as the number of scientific interactions or insights collected, and qualitative contributions, including actionable insights and internal stakeholder feedback. This dual focus suggests that companies are moving towards a more comprehensive and impact-oriented evaluation framework.

In terms of educational background, while STEM graduates constitute the largest group, Pharmacy and Pharmaceutical Chemistry and Technology graduates clearly represent a foundational pillar of the profession in Italy. These professionals bring a strong blend of scientific rigour and applied pharmaceutical knowledge, making them particularly well-suited for the multidisciplinary nature of the MSL role. Interestingly, the relatively low proportion of medical doctors in MSL roles contrasts with global trends, indicating a broader acceptance in Italy of diverse academic pathways into Medical Affairs.

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Another notable trend is the career trajectory into the MSL role: over half of the respondents transitioned from commercial positions, highlighting a shift in corporate strategies aimed at fostering cross-functional growth and internal talent development. This trend, however, coexists with a more traditional approach in some companies, where MSL positions remain exclusive to physicians with specific therapeutic expertise.

The survey also identified several unmet needs, particularly in the areas of Real-World Evidence, clinical research, digital skills, and understanding of the full drug development-to-access process. These findings point to a clear demand for continuous professional development that goes beyond scientific expertise to include communication, leadership, and technical fluency in digital tools.

Lastly, the high level of interest in formal certification and advanced training programs reflects a growing recognition of the need to professionalise and standardise the MSL career path. As the strategic importance of Medical Affairs continues to grow within the pharmaceutical industry, companies have a unique opportunity to support the professional development of their MSLs, ensuring these key scientific ambassadors are prepared for the challenges of an increasingly dynamic healthcare environment.

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Clinical Investigator Certificate (CLIC) Course in Cape Town

The clinical development of novel (better) medicines that are safe, effective and of good quality has become increasingly complex because of new technologies, regulatory and legal requirements and the interdisciplinary and global nature of the pharmaceutical industry. This requires effective capacity strengthening in medicines development and regulation worldwide.

On the African continent, there is a critical skills gap in medicines development and regulatory sciences. Aligned with this need, Fundisa African Academy of Medicines Development (http://www.fundisa-academy.com/) has been established as non-profit organisation in Cape Town in 2014 with the mission to provide leadership and promote teaching and training in these disciplines in South Africa and other African countries. One of the flagship programmes is its Clinical Investigator Certificate (CLIC) course offered together with the Cape Town-based Tiervlei Trial Centre since 2015. This 5-day course which is GCP (1) and CPD (2) (clinical and ethics) accredited is presented face-to-face on the Nitida wine farm in Cape Town (with one exception in Johannesburg and a brief interruption during the pandemic).

The content is structured according to an internationally standardised syllabus established by PharmaTrain and the European Clinical Research Infrastructures Network (ECRIN). The target audience consists of investigators, site staff, regulatory and other scientists. Whereas the first two days address basic concepts (level 1), the second part presents details on planning, execution, evaluation and ethical considerations of clinical trials (level 2).

This year, the CLIC course is offered as a 2-day refresher (15-16 May 2025). The main focus is the 2025 revision of the ICH E6 GCP Guideline (R3). For the first time, this major GCP adaptation addresses requirements for data governance and computerised systems, for decentralised and pragmatic trials and for Real-World Data (RWD). The guideline emphasises novel approaches to clinical research, such as Quality by Design (QbD), Risk-Based Quality Management (RBQM), and the critical-to-quality (CTQ) factor concept. It must be read together with ICH Guideline E8 (R1) on general considerations for clinical trials. The Cape Town CLIC course also addresses local requirements, especially the 2024 Research Guidelines by the National Department of Health providing the South African context for core ethical knowledge, high-quality data, proper study preparation and conduct at investigational sites. Training on the updated 2024 Research Guidelines is mandatory for investigators in South Africa.

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Abbreviations:
1) Good Clinical Practice
2) Continuing Professional Development

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Challenges to Establishing Pharmaceutical Medicine as a Profession: Needs for Professional Identity and a Career Pathway: Role of Education and Training

Pharmaceutical Medicine
https://doi.org/10.1007/s40290-025-00559-w

CURRENT OPINION

Pharmaceutical Medicine (PM) was originally defined as a "medical/scientific discipline concerned with the discovery, development, evaluation, registration, monitoring and medical aspects of the commercialisation of medicines, for the benefit and health of the community" (1). Conceived as a medical specialty, the discipline encompasses several health-related professions and occupations, such as medicine, pharmacy, clinical pharmacology, drug safety and pharmacovigilance, pharmaceutical sciences, biology, health economics and others. The practice of the disciplines involved in Pharmaceutical Medicine/Medicines Development has evolved from an occupation to be accepted as a distinct profession, by meeting well established criteria. The lack of professional identity and a clear career path have been identified as key factors limiting the advancement of this profession.

To read the full article, please visit: https://rdcu.be/ejO4B

1) www.fpm.org.uk

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IFAPP WEBINAR

Date: 30th June 2025
Time schedule:
05.00-07.00 AM EST
10.00-12.00 AM GMT
12:00-02:00 PM CEST
07.00-09.00 PM JST

The speakers

Rebecca Stanbrook

BPharm (hons), MRPharmS, FFRPS, DipRQA, FRQA

ICH – GCP Revision

Gabriele Schwarz

a graduated pharmacist, joined the German Federal Institute for Drugs and Medical Devices (BfArM) in 2001

Good Clinical Practice (GCP) is the international scientific and ethical standard for the conduct of interventional clinical trials. The ICH E6 Guideline, published in the mid-1990s, established a harmonised understanding of GCP.

New trial designs, new technology and the greater use of different data sources required a comprehensive revision of the guideline. This seminar will familiarise participants with the key aspects of this revision.

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IFAPP WEBINAR

ICH – GCP Revision

Rebecca is the EFPIA Topic lead for ICH E6(R3) Expert Working Group, the group responsible to rewriting the Good Clinical Practice Guideline, the global standard for the conduct of clinical trials.

Rebecca has worked at a number of pharmaceutical companies in various roles across all aspects of the pharmaceutical industry and as a regulator at the Medicines and Healthcare products Regulatory Agency. To date she has over 30 years' experience in the industry or as a regulator.

Rebecca Stanbrook

Gabriele is currently BfArM's GCP Strategy Expert and represents the EU in the ICH E6(R3) Expert Working Group. For more than a decade and a half, until the end of 2022, Gabriele was Head of BfArM's GCP Inspection Unit and responsible for BfArM's GCP inspection activities, particularly in the context of international pre-approval inspections coordinated by the Europe Medicines Agency.

Over the years, she has contributed to the development of a considerable number of European and international guidelines, such the OECD 'Recommendation on Clinical Trial Governance', the ICH E6(R2) and (R3) Guideline on 'Good Clinical Practice' and the ICH E19 Guideline on a 'Selective Approach to Safety Data Collection in Specific Late-stage Pre-approval or Post-approval Trials'.

Gabriele Schwarz

Follow-up workshop

This webinar will be followed by a virtual workshop on "Practical challenges of implementing ICH-GCP(R3) in your clinical trials" on 18th September 2025 from 10:30 am CEST to 3:00 pm CEST. The speakers will be Ingrid Klingmann, MD, PhD, PharmaTrain, Belgium, and Elisabeth Reus, Swiss Tropical and Public Health Institute, Switzerland.

REGISTER IN ADVANCE FOR THIS WEBINAR

AFTER REGISTERING, YOU WILL RECEIVE A CONFIRMATION EMAIL CONTAINING INFORMATION ABOUT JOINING THE WEBINAR.

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International Conference of Pharmaceutical Medicine: ICPM 2025 Summary Report

The IFAPP's flagship biennial event, the International Conference of Pharmaceutical Medicine, ICPM 2025, co-hosted by the Dutch national member association of the IFAPP, the NVFG (Nederlandse Vereniging voor Farmaceutische Geneeskunde), and held in Amsterdam from 9 to 11 April, focused on the theme "Purpose for Future in Pharmaceutical Medicine" (1,2,3).

The conference brought together academics, clinicians, consultants, ethicists, experts, industry innovators, patient organisations, regulators, scientists and Pharmaceutical Medicine professionals from IFAPP and NVFG, the Faculty of Pharmaceutical Medicine (FPM) and other organisations such as the World Medical Association (WMA) and CIOMS (see below).

This year's conference featured dynamic keynote speeches, provocative plenary sessions, ethical panel discussions with international leaders sharing their ideas to catalyse change and transform education in Pharmaceutical Medicine (PM) for physicians, patients, researchers, scientists and regulators to create a brighter future for better global health. A poster reception, the IFAPP Fellows ceremony and networking during the breaks in the historic West-Indisch Huis venue generated enthusiasm, joy and bold ideas about the continuum of professional education and future opportunities for career growth.

The content of the sessions explored high priority areas including:

PM and Innovative Therapies

PM encompasses medicines, vaccines, medical devices, diagnostics and other cutting-edge health technologies. Every sector in medicine globally is dependent on Pharmaceutical Medicine, i.e. prevention, diagnosis, treatment and safety. The drivers for change in PM are Genomics and Personalised Medicine, Artificial Intelligence (AI) and Large Language Models (LLMs), Advanced Therapeutics (Cell and Gene Therapy, Messenger Ribonucleic Acid (mRNA) Technology), Digital Health, Wearable Technology, Automation and Robotics in Drug Development (4).

PM and Public Health

Public Health is a scientific profession with public purpose. "Let's keep Pharmaceutical Medicine purpose-driven and public-health powered. Pharmaceutical Medicine becomes meaningful when we connect it to health systems, people, and public goods" said Dr Karine van 't Land, Chair KAMG-Organisation for Public Health in the Netherlands (https://www.kamg.nl/) in the closing remark of her keynote lecture.

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Pharmaceutical Medicine

PM is a recognised, board-certified medical specialty in four countries in Europe (UK, Ireland, Switzerland, and Belgium) with the exceptional case of Belgium in that Healixia, the Belgian National Member Association of IFAPP, succeeded to achieve a recognition of the combination of PM and Clinical Pharmacology as a unique specialty (5).

PM and Professional Identity

Professional competencies and capabilities in medicines development are gained through continuous education and certification, based on the PharmaTrain Syllabus (6).

Clinical Trials in Europe

France, Spain, the UK, Germany, Italy, Poland, the Netherlands, Belgium, Denmark and the Czech Republic are among the top 10 countries on the continent in terms of the number of clinical trials initiated in Europe since the start of the COVID-19 pandemic compared to the previous five years. The decline in trials across Europe can be attributed to the impact of the General Data Protection Regulation, the European Clinical Trials Regulation and the COVID-19 pandemic.

PharmaTrain Syllabus 2024 V3.0

Pharmaceutical Medicine/Medicines Development Science

Jointly developed by:
PharmaTrain Federation; Faculty of Pharmaceutical Medicine; International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine.

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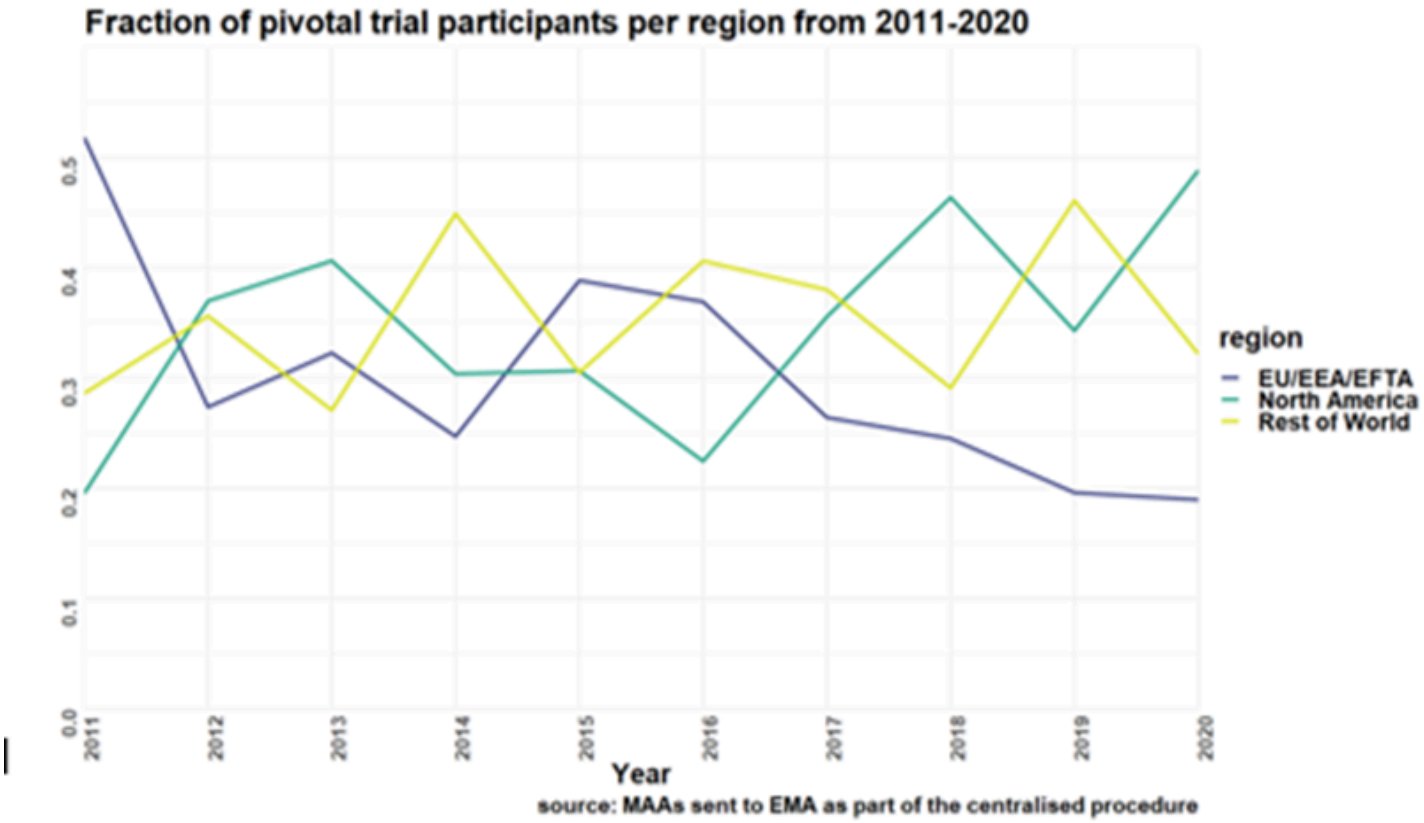

Accelerating Clinical Trials in the European Union, ACT EU

A joint initiative of the European Commission, the Heads of Medicines Agencies, and the European Medicines Agency (EMA) was launched in 2022 to build on the momentum of the implementation of the Clinical Trials Regulation (CTR). The vision of ACT EU is to have better, faster and smarter clinical trials in the EU, creating a favourable environment for clinical research. Despite the ACT EU platform of multi-stakeholder cooperation, the EMA is not moving fast enough to remain competitive and should speed up the harmonisation of Part 2 of a clinical trial application, remove national administrative barriers (such as Clinical Trial Application, pricing negotiations, etc.), increase support to academic research and focus on patients, technological and process innovation.

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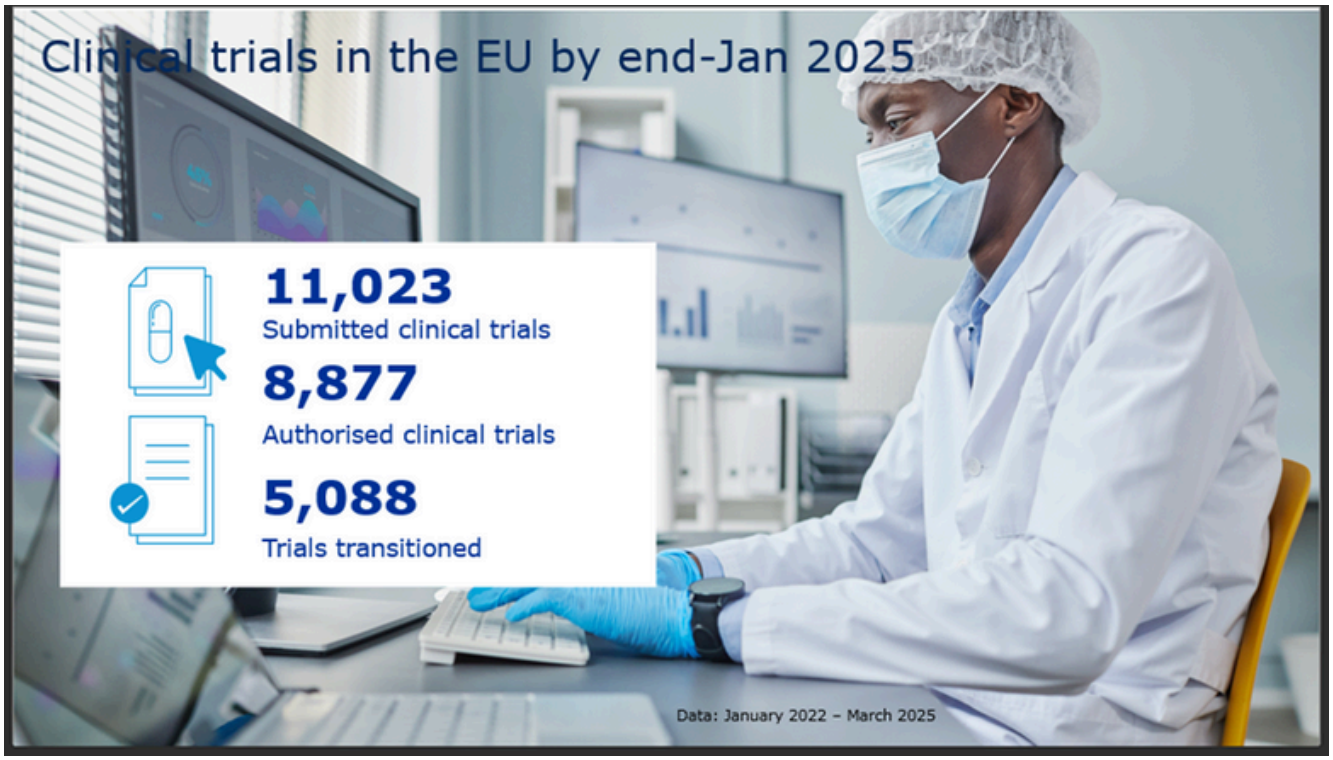

Source Act EU classified as public by the European Medicines Agency.

Regulatory Trends

Discussions led by EMA officials highlighted the latest trends and changes in regulatory guidelines, submission processes, and developments, especially relevant given the EMA's location in Amsterdam. The EMA and the European Medicines Research Network commitment (EMRN) has been set to a maximum of 210-day review time, the highest scientific and regulatory standards, constant improvements within the legal framework, also to support innovation, and act in the interest of patients and transparency (7).

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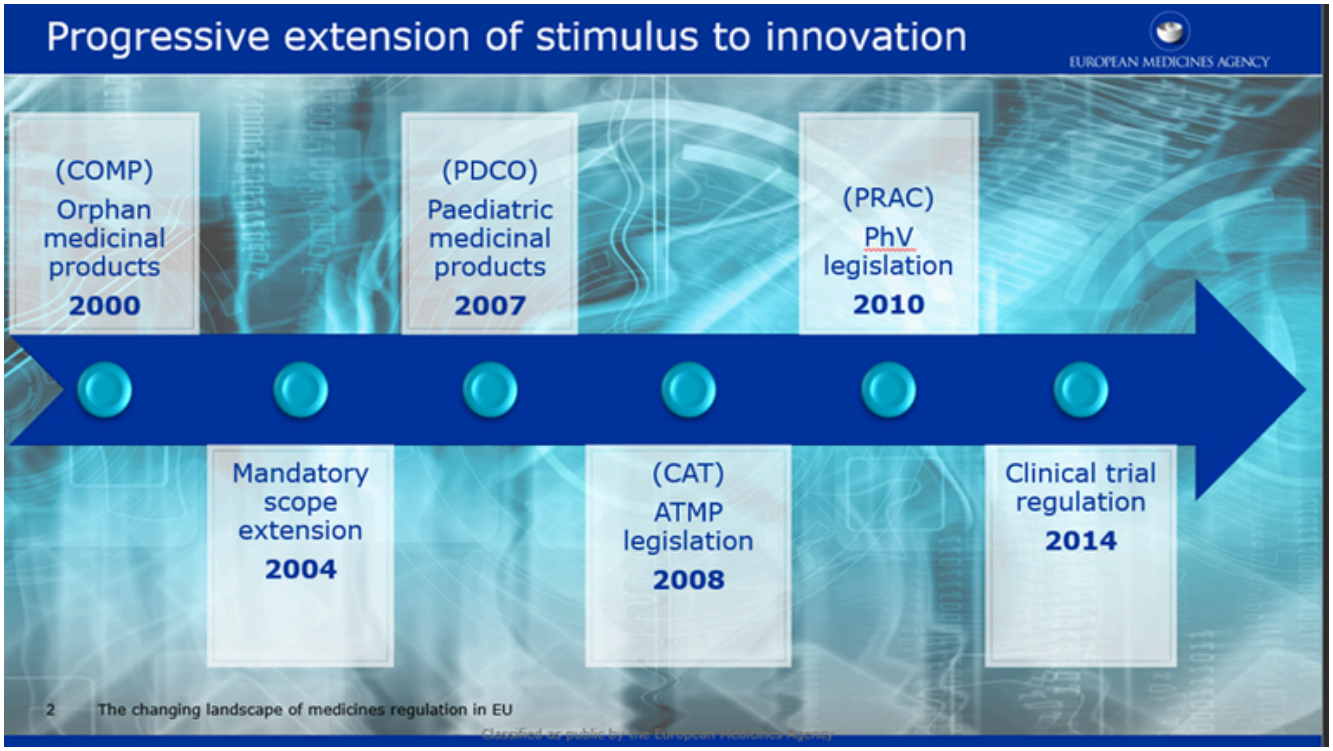

Source slide classified as public by the European Medicines Agency

It is the EMA's task to develop legislation of medicines regulation, but the increasing complexity makes the need for revision imperative. The COVID-19 pandemic demonstrated the need for better preparation and urgent action. The European Commission's proposal for a revised pharmaceutical legislation is an ambitious effort to future-proof the EU regulatory system.

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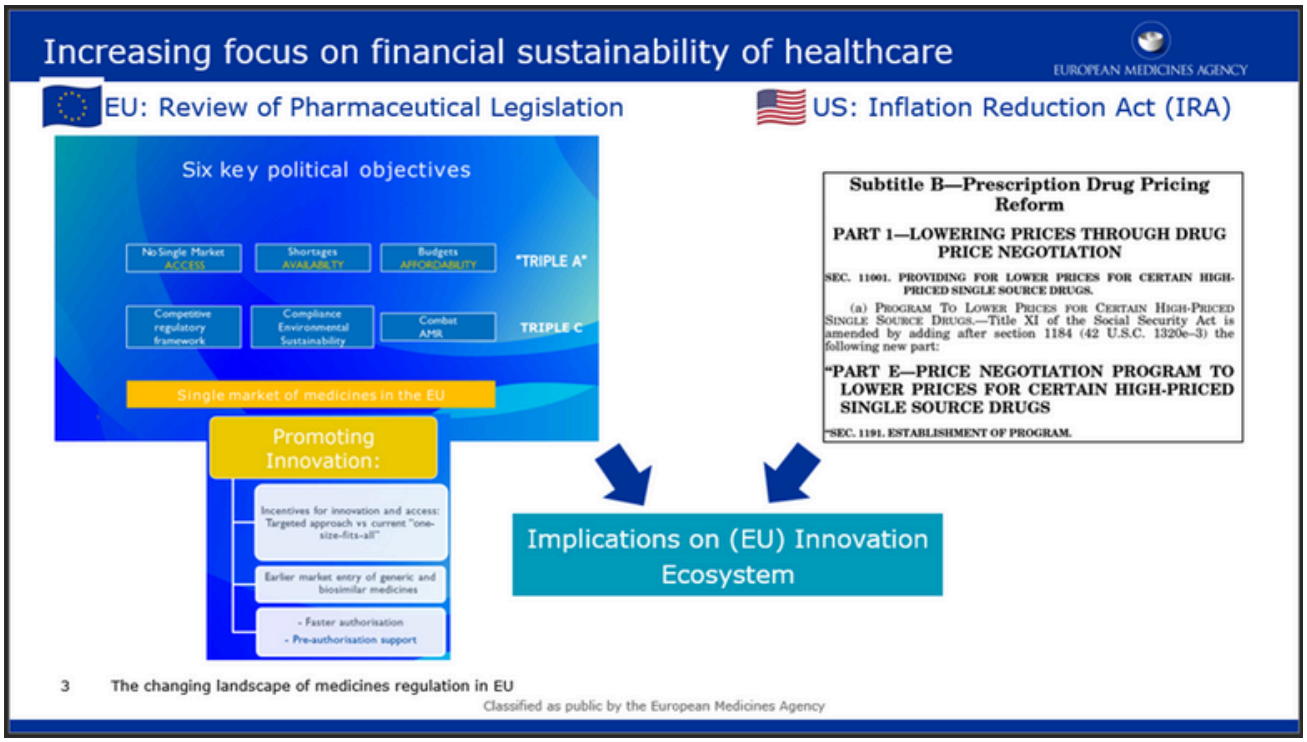

Source slide classified as public by the European Medicines Agency

The EMA pursues the opportunity to reshape the regulation of medicines in the European Union by:
- making the EU regulatory framework fit for new, innovative medicines,
- supporting better access to medicines for patients,
- addressing major public health challenges of the future, such as antimicrobial resistance,
- helping to realise the opportunities of the EMA's Regulatory Science Strategy and the EMA's Network Strategy for 2025 (e.g. digitalisation, network sustainability, supporting innovation and availability, addressing environmental concerns).

New Medicines: A Regulatory Affair and a Regulatory Science Opportunity

Regulatory science plays a critical role in public health by bridging the gap between scientific research and the practical application of that research in regulatory decision-making. Stakeholders have an important role to play, from identifying problems to applying regulatory science solutions and advancing regulatory practice. Regulatory science research is the ecosystem for change. Competitiveness is becoming an increasingly visible driver. Regulators and policy makers seek to ensure that the framework is conducive to research, development and manufacturing of medicines (7).

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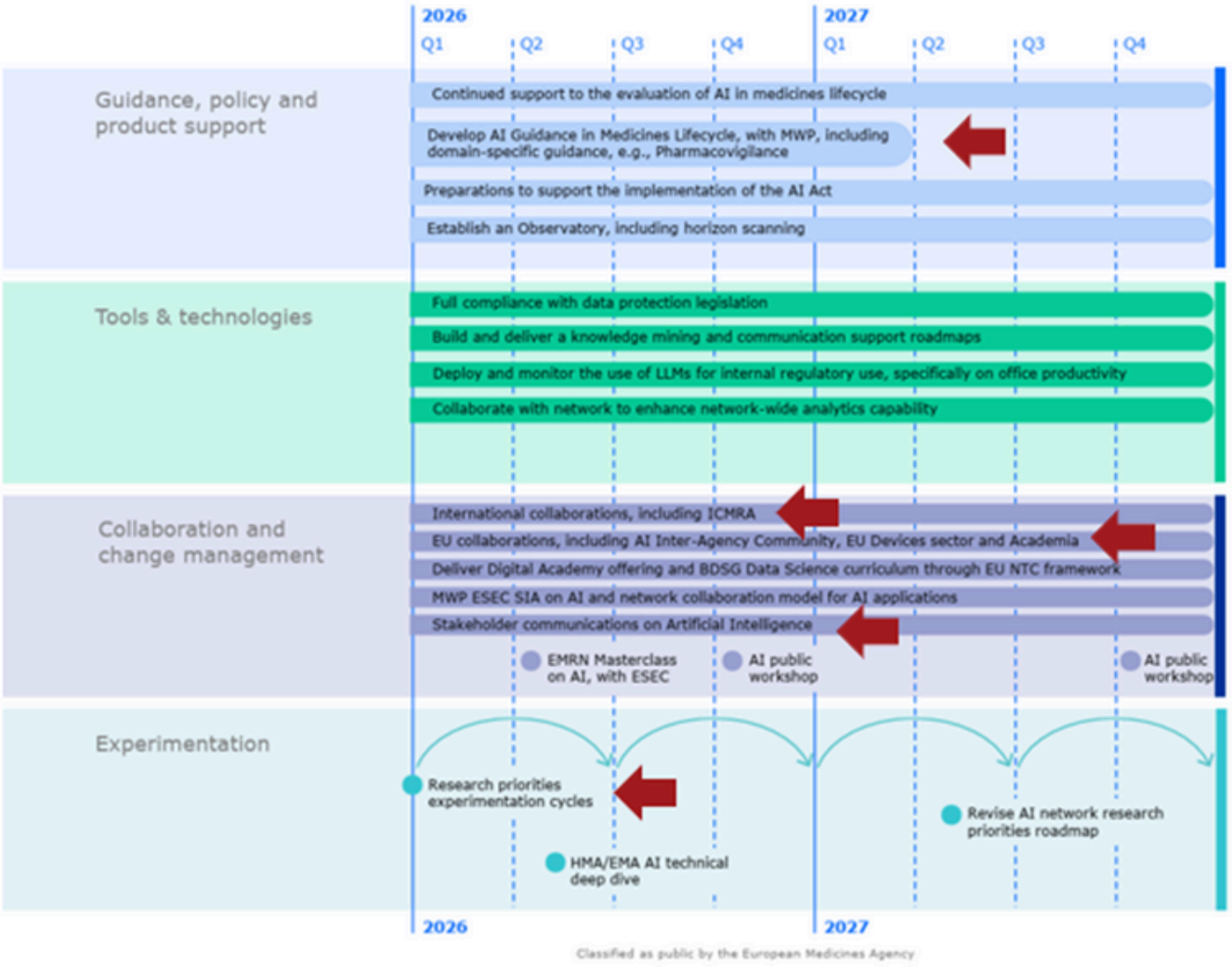

Ethical Considerations

Ethical issues in pharmaceutical development and patient care were a major topic at ICPM 2025, with discussions on how to balance innovation with patient safety and ethical standards under the scope of the current 10th revision of the Declaration of Helsinki for medical research and the Declaration of Taipei for biobanks. Sustained investment in Research Ethics Committees (RECs) is essential for ethical research in the healthcare landscape.

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A call for continued patient collaboration and commitment in ethical research for patients in line with the Helsinki Declaration revision of 2024 which calls for the inclusion of vulnerable people and recommends community engagement with meaningful participation, requires educated patients' and citizens' participation and peer supporters to bring forward patient voices. Patients should help shape the future and co-build ethical genomic medicines together with researchers. Vulnerability should not be perceived as weakness; it is an ethical lens in the 2024 revision of the Declaration of Helsinki and a step towards equity and inclusion. It is a call to action to promote dignity, respect and hope in every clinical trial (8, 9).

Healthy Volunteers

The VolREthics initiative has led to the inclusion of the rights of healthy volunteers in the Global Ethics Charter to protect them from the risks of harm and exploitation (10). It comprises:

1. laws and regulations that specifically protect healthy volunteers as research participants,
2. assurance that their participation in research is ethical and scientifically necessary,
3. adequate representation throughout the research process,
4. transparency about clinical trials in which they are involved,
5. adequate research ethics oversight,
6. adequate trial site and investigator oversight,
7. protection from physical harm,
8. adequate attention paid to their well-being,
9. adequate protection from potential long-term harm,
10. protection from the risks of over-volunteering,
11. recruitment through fair and respectful practices,
12. relevant study information to provide genuine informed consent,
13. fair financial compensation for their participation,
14. post-trial compensation for research-related injury,
15. adequate processes for confidential reporting of concerns.

CIOMS Guidelines

These guidelines, prepared by CIOMS Working Groups (WGs), are linked to both regulatory science and PM, and often give more detailed insights and explanations into topics that are subjects of regulation that facilitate implementation and good practices. CIOMS reports are not regulatory standards, but consensus reports written by leading subject experts, usually from academia, industry, and regulators. CIOMS's recent new reports include: Serious Cutaneous Adverse Drug Reactions (SCAR), Real World Data/Evidence in Regulatory Decision Making, and the CIOMS Cumulative Glossary with a Focus on Pharmacovigilance. CIOMS WGs are transparent, and their meeting minutes are public information (see WG section at https://cioms.ch/) (11).

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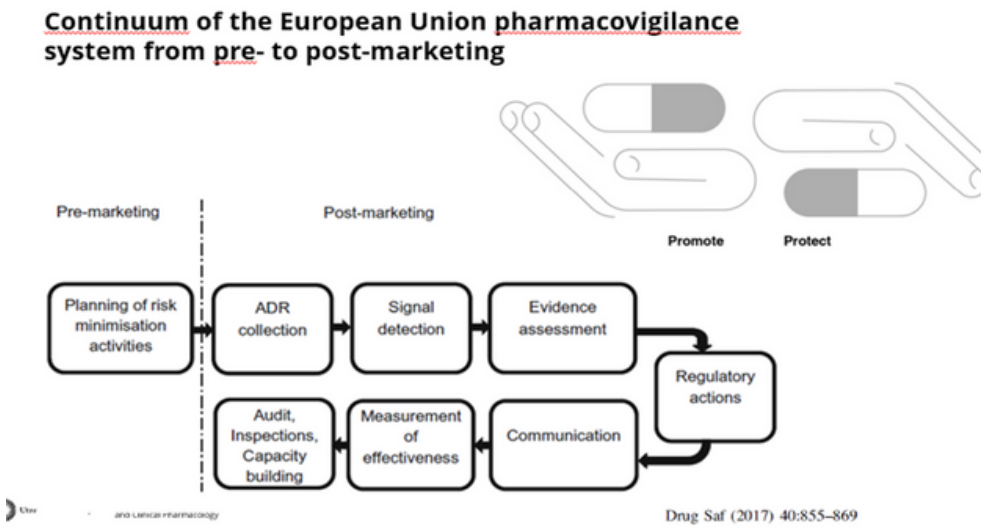
Patient Safety and Pharmacovigilance

Utrecht University — Pharmacoepidemiology and Clinical Pharmacology

Many changes globally aim to facilitate better understanding of regulatory interventions. They are needed to expand regulatory assessments beyond the intended effects of the medicinal compounds. Challenges arise when assessing the (un)intended impact of regulatory interventions. The potential is to learn from other disciplines and to improve while it is important to monitor the unintended impact.

Beyond impact studies this particular session focused on the value and importance of thinking beyond the single medication, including effects on the system, costs, caretakers, and the patients themselves. A lot can be learnt from implementation science that would allow for the design of better interventions in the future (12a).



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Pharmacogenetic (PGx) for preventing ADRs
Personalised Medicine

Analysis of genomic DNA to explain / predict the response of a patient to drug therapy

PGx applications

Drug	Clinically relevant enzymes	
	With dosing advice	Without dosing advice
5-FU **	DPYD	
abacavir **	HLA-B*5701	
acenocoumarol	VKORC1	CYP2C9
allopurinol	HLA-B*5801, ABCG2	
amitriptyline	CYP2D6	CYP2C19
aripiprazol	CYP2D6	CYP3A4
articaine	BChE	
atazanavir	UGT1A1	CYP3A4
atomoxetine	CYP2D6	CYP2C19
atorvastatine	SLCO1B1	CYP3A4
azathioprine **	TPMT, NUDT15	
brexpiprazol	CYP2D6	CYP3A4
capecitabine **	DPYD	
carbamazepine	HLA-B*1502, B*1511, HLA-A*3101	CYP3A4
celecoxib	CYP2C9	
citalopram	CYP2C19	CYP2D6 , CYP3A4
clomipramine	CYP2C19, CYP2D6	
clopidogrel **	CYP2C19 (a)	
codeine	CYP2D6 (a)	CYP3A4
doxepine	CYP2D6	
efavirenz	CYP2B6	CYP3A4
eliglustat **	CYP2D6	CYP3A4
escitalopram	CYP2C19	CYP2D6, CYP3A4
fenprocoumon	VKORC1	CYP2C9
fenytoine **	CYP2C9**, HLA-B*1502	CYP2C19
flecainide	CYP2D6	
flucloxacilline	HLA-B*5701	
flucytosine	DPYD	
haloperidol	CYP2D6	CYP3A4
imipramine	CYP2C19, CYP2D6	
irinotecan **	UGT1A1	CYP3A4
lamotrigine	HLA-B*1502	UGT1A4
lansoprazol	CYP2C19	CYP3A4

Drug	Clinically relevant enzymes	
	With dosing advice	Without dosing advice
mavacamten**	CYP2C19	CYP3A4
mercaptopurine **	TPMT, NUDT15	
metoprolol	CYP2D6	
mivacurium	BChE	
nortriptyline	CYP2D6	CYP1A2, CYP2C19
omeprazol	CYP2C19	CYP3A4
oxcarbazepine	HLA-B*1502	
pantoprazol	CYP2C19	
paroxetine	CYP2D6	
pimozide	CYP2D6	CYP1A2, CYP3A4
propafenon	CYP2D6	CYP1A2, CYP3A4
quetiapine	CYP3A4	
risperidon	CYP2D6	
rosuvastatine	SLCO1B1	ABCG2
sertraline	CYP2C19	CYP2D6, CYP2C9, CYP2B6, CYP3A4
simvastatine	SLCO1B1	CYP3A4 (a)
siponimod**	CYP2C9	CYP3A4
suxamethonium	BChE	
tacrolimus	CYP3A5	CYP3A4
tamoxifen	CYP2D6 (a)	CYP3A4 (a)
tegafur **	DPYD	CYP2A6
tioguanine **	TPMT, NUDT15	
tramadol	CYP2D6 (a)	CYP3A4, CYP2B6
venlafaxine	CYP2D6	CYP3A4, CYP2C19
voriconazol	CYP2C19	CYP3A4, CYP2C9
warfarine	CYP2C9, VKORC1	
zuclopentixol	CYP2D6	

DPWG (actionable):

60 drugs, 18 genes

ABCC2	HLA-A*3101
ABCG2	HLA-B*1502
BChE	HLA-B*1511
CYP2B6	HLA-B*5701
CYP2C9	HLA-B*5801
CYP2C19	NUDT15
CYP2D6	SLCO1B1
CYP3A4	TPMT
CYP3A5	UGT1A1
DPYD	VKORC1

Erasmus MC

www.farmacogenetica.nl/tabellen

(12b)

Medical Affairs 2.0

The medical department in a modern pharmaceutical company is the Medical Affairs department which generates and communicates data that help healthcare professionals, payers, policy makers and others to make informed decisions to ensure the best use of products for the benefit of the patients. Over the past decade, the Medical Affairs department has evolved from an operational function to a strategic one, alongside the Research and Development (R&D) and commercialisation departments by informing R&D strategies on the critical path from R&D to clinical practice.

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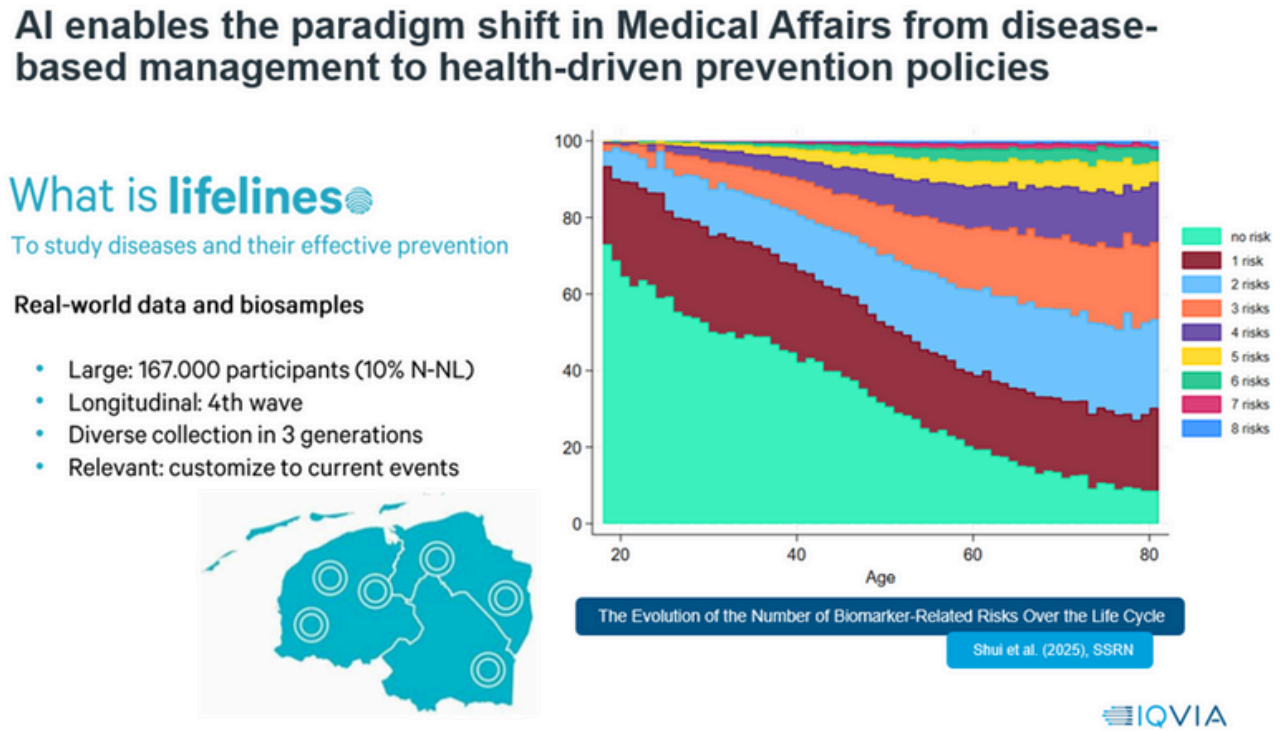
Medical affairs activities such as scientific exchange, symposia, continuing medical education (CME), webinars, medical information, advisory boards and value evidence generation can improve patient care and outcomes. Medical Affairs omni-channel engagement will increase relevance to Medical Affairs stakeholders. A goals-driven rather than tactics-driven approach enables accountability of Medical Affairs professionals (13).



(14)

Health Technology Assessment in the EU

The EU's ASCERTAIN project aims to improve access to innovative health technologies by developing affordable pricing models, cost-effectiveness assessments and reimbursement strategies, including precision cancer medicine, cell and gene therapy and medical devices, to reduce uncertainty for stakeholders, reward innovation and accelerate patient access (15).

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Real World Data (RWD) and More-EUROPA

Decision-making by regulators and health technology assessment (HTA) bodies continues to be primarily iinfluenced by randomised clinical trials (RCTs). RWD and evidence derived from them (Real World Evidence) can impact development at different stages of the drug lifecycle, not just during evaluation. Data quality and preplanning are key aspects. More-EUROPA focuses on disease registries and particularly on:

- curated data sets – proven data collection,
- data linkage and Natural Language Processing NLP to generate more data rich sets,
- activities centred around complementing trial datasets:
- effect estimates in subpopulations, effect modification/outcomes estimations,
- early (to late) stage drug development, e.g., trial design,
- external controls,
- registries as platform for trials,
- evaluate critical steps in designing, executing and evaluating R-RCTs (Randomized Registry-Based Controlled Trials)),
- activities around implementation: Long term surveys and guidance development (16).

The Futures of Healthcare: Signals from Today to 2035

Healthcare 2035, based on an analysis of publications, interviews with industry experts and foresight methodology, show that the future is already taking shape: By 2035, we can expect to see:

- whole genome sequencing as routine first-line diagnostics,
- AI-driven drug discovery that reduces timelines from 10 years to months,
- decentralised, digital-first clinical trials increasing diversity and patient-centricity,
- Real-world Evidence becoming the standard for regulatory approval,
- smart implants and AI-powered patients support personalising adherence and care.

The future is not a distant goal, it is emerging now through the signals seen today (17).

Collaboration and Networking

The conference highlighted the importance of collaboration between researchers, academics, clinical practitioners, patient advocates and biomedical professionals in industry to drive progress in Pharmaceutical Medicine.

ICPM 2025 oral presentations and posters

They will be published separately in the next issues of IFAPP TODAY.

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Summary
ICPM 2025 was an inspiring as well as a future-driven and purposeful conference due to its content, community networking and participation of renowned academics of international organisations, World Medical Association (WMA) and CIOMS officials, with industry leaders from US, EU, Japan, also of EFPIA (18) representatives, EMA regulators, patient and public associations, NVFG and IFAPP professionals, experts, leaders and fellows.

Please accept my warmest thanks to the members of the Organising Committee, Programme Committee, Scientific Committee, the board members of both organisations (IFAPP and NVFG), moderators, speakers, colleagues, participants and our organising partner Caroline van Bruggen, Business 2gether, for their important contributions.

Author:
Dr Varvara Baroutsou, IFAPP immediate past President

References:

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2) IFAPP
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4) Faculty of Pharmaceutical Medicine https://www.fpm.org.uk/
5) Healixia
6) PharmTrain Syllabus
7) European Medicines Agency: classified public slides
8) WMA Declaration of Helsinki
9) WMA Declaration of Taipei
10) VolREthics Initiative: Healthy Research Volunteers and Ethics
11) CIOMS
12a) Marieke De Bruin PhD, Professor of Drug Regulatory Science, Utrecht University, Pharmacoepidemiologyand& Clincal Pharmacology
12b) Pharamcogenetics .Do you have your passport? ProfessorDr Ron van Schaik, International Expertcenter Pharmacogenetics, Dept. Clinical Chemistry, Erasmus MC Rotterdam,The Netherlands
13) Björn Oddens M.D. Ph.D. SVP, Head Value and Implementation Organization Merck & Co, USA
14)The evolution of the numbers of biomarkers - Related risks over the lifecycle. Shul et al. 2025 SSRN.
15) Health Technology Assessment
16) Peter GM Mol, University of Groningen, University Medical Center Groningen, EMA CHMP member, Dutch Medicines Evaluation Board
17) linkedin.com/in/marklightowler
18) European Federation of Pharmaceutical Industries and Associations

Disclaimer: This report was made based on personal notes during ICPM 2025 and from slides presented by the speakers

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ICPM 2025

THE FLAG

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