



# IFAPP TODAY

The Global Pharmaceutical Medicine Journal



**INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS AND PHARMACEUTICAL MEDICINE**

**IFAPP**  
**The only international organisation for everyone involved in Pharmaceutical Medicine**



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## Brazil's Unique Opportunity to Become a Global Player in Clinical Research

Law 14.874/24, sanctioned by President Luiz Inácio Lula da Silva in May 2024, represents an important regulatory milestone for the clinical trial sector in Brazil, promoting transparency and legal predictability. Its main objective is to create a safer and more reliable environment for the development of clinical studies, bringing the country into line with international standards of ethics and innovation. Several entities and scientific associations have worked together to stimulate the creation of this law by the parliament. The SBMF (Brazilian Association of Pharmaceutical Medicine) took part in this group. Many politicians have understood the situation and, since 2015, have elaborated a law sine that was finally voted for in 2024.

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## Advantages Provided by Law 14.874/24

- With regard to transparency this law establishes clear guidelines for the conduct of research, strengthening the trust of patients, researchers and companies in the process.
- It also establishes legal predictability by creation of a solid regulatory environment, by reducing legal uncertainties and encouraging investment in the clinical trial sector in Brazil, and by facilitating the conduct of studies ensuring legal certainty for the companies and institutions involved. Rare disease patients face unique and urgent challenges when it comes to accessing effective treatments. For them, participating in clinical trials represents a vital opportunity, since the therapeutic options available on the market are often non-existent or insufficient. This context makes clinical trials a crucial path to hope and survival.

## Main Restrictions to Receiving Sponsored Studies in Brazil

The governance of clinical trials is a critical point in drug development. In addition to patient safety, data security and reliability, clinical trials need to have pragmatic deadlines and costs. In multi-centre studies, each site has a role which requires responsibilities that can affect the overall outcome of the study. Failure to foresee deadlines and costs can make the whole study unfeasible.

Law 14.874/24, which is still being regulated by the Ministry of Health, provides for approval deadlines through a committee (INEP - National Institute for Research Ethics) that will decentralise approvals to local Ethics Committees which will have autonomy and predetermined deadlines.

The costs cannot be unpredictable either. One point still to be resolved in the legislative environment is the sponsor's obligation to continue treating patients indefinitely. This happened because President Lula vetoed the article that set the maximum time limit at 5 years and the deadline was left open. This veto can be reversed by parliament and this decision should happen soon.

## Advantages for Rare Disease Patients in Clinical Trials

For many rare diseases, regulatory approval of new drugs can take more than 10 years, taking into account the research and the testing and approval phases. Participating in a clinical trial gives patients early access to experimental therapies that might otherwise be unavailable for decades.

Clinical trials targeting rare diseases generally seek solutions for conditions that have no effective treatments. This puts these patients at the centre of scientific advancement and increases the chances of finding a viable alternative.

Taking part in a clinical trial means access to specialised medical teams and continuous monitoring. This close monitoring is particularly beneficial for patients with complex and poorly understood diseases. The visibility and data generated by clinical trials in rare diseases can speed up the creation of specific regulations for these conditions, such as accelerated approval programmes or emergency authorisations.

## Why are Rare Disease Patients in a Hurry?

Many rare diseases have a progressive and degenerative course, leading to irreversible loss of function or even death in short periods of time. Every day without treatment can mean a worsening in quality of life or a reduction in life expectancy. More than 95% of rare diseases have no approved treatments. For these patients, experimental drugs are often the only hope.

The average development time for a drug is long, involving rigorous stages of pre-clinical research, clinical trials and regulatory review. These processes, while necessary to ensure safety and efficacy, are challenging for patients with rare conditions that require immediate solutions.



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For rare disease patients, time is a critical issue. Participation in clinical trials is not just an opportunity, but an urgent necessity. Creating a regulatory environment that prioritises these patients and speeds up drug development and approval processes is essential to transforming the reality of these individuals, giving them hope and quality of life.

**Author: Hélio Osmo MD MBA, Director Institucional SBMF (Associação Brasileira de Medicina Farmacêutica), Global Fellow IFAPP**



## Highlights of the MAPA-MAPS (1) Medical Affairs Summit 2024

Australasia's burgeoning pharmaceutical medical community continues to grow in size and influence within the medicines sector, with nearly 300 members of the Medical Affairs Professionals of Australasia (MAPA) recently convening in Sydney to discuss emerging trends offering the potential to enhance our profession, including Artificial Intelligence (AI) and Patient Centricity.

### Do More, With Less: How AI & Smart Content Management are Revolutionising Medical Affairs

Presenters:

- *Rozhin Asghari, Manage Access Program Medical Lead, Novartis*
- *Tristan Reid, Group Engagement Director, The Ward Marketing Group*
- *Jon Zdon, Medical Head Governance & Excellence, Novartis*

In a world with rapidly increasing content demands and regulatory complexity, medical affairs teams are looking for innovative ways to boost efficiency and engage healthcare professionals (HCPs) effectively. AI and smart content management solutions have the potential to enable medical affairs professionals to “do more, with less.” Jon Zdon highlighted how, though the role of medical affairs has evolved, with professionals now emerging as strategic leaders within their organisations, limited capacity and time make it challenging to fulfil these expanded responsibilities effectively, opening the door for innovative new technologies. Rozhin Asghari commented that leveraging AI can alleviate these constraints by improving efficiency and taking on some of the more “tedious” tasks.



From left to right: John Zdon, Tristan Reid and Rozhin Asghari

The speakers emphasised the importance of curiosity and proactive AI adoption, while also advising caution to ensure adherence to internal policies and guidelines. Tristan Reid addressed the challenges of content creation and navigating medical, legal and regulatory (MLR) reviews in an omnichannel landscape, where hyper-personalisation demands large-scale content production. He stressed the need to deliver relevant, targeted content to HCPs rather than overwhelming them with a continuous stream of emails, invitations, and materials.



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AI-powered tools, like digital assistants and content management platforms, offer a transformative solution to streamline workflows, automate tasks, and optimise content across various channels. Key takeaways were the potential for generative AI assistants, modular content, AI pre-checking, and digital asset management as tools to enhance efficiency, ensure compliance, and bolster HCP engagement. Tristan also provided a practical blueprint for adopting these technologies, walking attendees through assessing organisational needs, evaluating available technology, and applying effective strategies for implementation and change management.

Jon encouraged embracing AI as a partner. "AI won't take your job; a human using AI will." However, by incorporating these advancements rather than seeing them as a threat, medical affairs professionals can enhance their productivity, compliance, and strategic leadership within their organisations.

## Patient Affairs in Action: Moving from Concept to Practice

Presenters:

- *Kyren Lazarus, Senior MSL, AstraZeneca*
- *Sam Pearson, Patient Advocacy Lead, AbbVie*
- *Sara McLaughlin-Barrett, Head of Patient Affairs, MedWise Consulting*

The role of patient affairs within pharmaceutical medicine has experienced a strategic transformation from a reactive to a proactive role that integrates the patient voice throughout the pharmaceutical lifecycle. Sara McLaughlin-Barrett emphasised the need to combine patient affairs and patient advocacy through four pillars of patient centricity: understanding the patient journey, integrating care with the multidisciplinary team, identifying care gaps, and amplifying the patient voice. This shift from traditional patient affairs to a more engaged, patient-focused approach was highlighted with patient journey mapping explored, focusing on disease awareness, diagnosis, treatment, and post-treatment care.



Participants identified key touchpoints for medical affairs impact in mock journeys for cardiology, lung cancer, diabetes, and breast cancer. Key themes included educating patients and all multidisciplinary healthcare professionals involved on topics such as disease awareness, diagnostics, treatment, side effects, adherence, and lifestyle factors. Focus groups and patient advisory boards were recommended to identify gaps and foster shared decision making.

Other priorities included enhancing health literacy, supporting patient support systems, and using tools like lay summaries, audits, and case studies to strengthen

engagement. The importance of shared decision making and co-design between the whole multidisciplinary team, patients and their carers was highlighted as fundamental.

Sam Pearson shared some of her organisation's patient advocacy initiatives, discussing case studies on empathy exercises, patient advisory boards, and the importance of building long-term partnerships with advocacy groups. She highlighted the need to understand and address real patient needs and integrate patient-centred insights within the organisation, noting that "no one



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size fits all.” Kyren Lazarus discussed the potential of AI and digital solutions to enhance patient care, showcasing examples from clinical trials and diagnostic imaging. The importance of acting on patient insights to deliver on the promise of true patient centricity was emphasised. “Gathering insights will not bring about patient centricity. Actioning those insights will.”

**Authors: Ward 7 in partnership with MAPA**

1) Medical Affairs Professional Society

## Spotlight on Pharmaceutical Medicine in Ireland: APPI's Recent Achievements

The Association of Pharmaceutical Physicians of Ireland (APPI) has recently marked a series of successes concerning its unwavering commitment to advancing the field of Pharmaceutical Medicine and supporting the professional development of its members. The APPI provides a platform for professional networking and peer-to-peer learning in a vibrant community of professionals who can share insights, resources, and experiences. Pharmaceutical physicians play a critical role in clinical trials, drug safety, regulatory affairs, and ensuring that new treatments reach the market safely and effectively.

The APPI has also launched an exciting new LinkedIn social media campaign aimed at raising awareness of the critical role that pharmaceutical physicians play in the sector. LinkedIn offers the perfect platform for APPI to engage with professionals in the healthcare, research, and pharmaceutical industries. This campaign seeks to amplify the APPI's voice and build a network of thought leaders, innovators, and healthcare professionals committed to advancing medical affairs, patient-centred research, drug safety, and excellence in Pharmaceutical Medicine.

The campaign featured 'Member Spotlights' on profiles of APPI members who have made significant contributions to the pharmaceutical industry.



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One of our Spotlights included Dr. Onthatile Serehete biography:

## Introducing: *Dr Onthatile T. Serehete*, IFAPP National Member Representative for the Association of Pharmaceutical Physicians of Ireland, 2024



**Dr Onthatile Serehete, MBChB  
MSc (Oxon), Dip HIV Man,  
PGCert Int Reg Affairs**

**Senior Director, Global Patient  
Safety – Medical Reviewer  
Eli Lilly, Ireland**

Onthatile has over five years working as a Research Physician in Clinical Research Organizations (CRO) with a diverse background in global pharmaceutical and nutraceutical research trials. As a Medical Study Manager at Atlantia Clinical Trials since 2021 she has provided invaluable medical expertise, ensuring protocol compliance and patient safety monitoring across various clinical trials in different therapeutic areas.

In addition to her medical degree obtained from the University of the Free State in South Africa, Onthatile completed an MSc in International Health and Tropical Medicine from the University of Oxford in 2017.

Onthatile recently moved to Eli Lilly, Cork to take up a Senior Director role in the Global Patient Safety – Global Medical review division, providing Medical support for safety reporting which contributes to the global assessment of the safety of Lilly products and molecules.

As an International Federation of Pharmaceutical Physicians and Pharmaceutical Medicine (IFAPP) National Member representative for the APPI, her role is to be the Ireland delegate to join NMA meetings, engage, and contribute the APPI's thought leadership for IFAPP planned initiatives and innovative approaches, Onthatile is also a member of the IFAPP External Affairs Working Group (EAWG).



By showcasing real-world success stories in relation to careers and personal stories, we hope to inspire the next generation of pharmaceutical physicians. The APPI is thrilled to report a notable increase in membership and engagement following the launch of its new LinkedIn profile and social media campaign. Since the campaign's debut, the APPI has experienced a 50% increase in overall membership numbers. This impressive growth highlights the effectiveness of the APPI's digital outreach and its engagement with physicians in the pharmaceutical sector.

Recently, 'Pharmaceutical Medicine' was featured in the Royal College of Physicians Ireland, Institute of Medicine Hot Topics Webinar. Dr Bernard Vrijens, CEO at Advanced Analytical Research on Drug Exposure (AARDEX Group) and Professor of Biostatistics at Liège University, Belgium, presented on the topic "Beyond the Prescription: Enhancing Medication Adherence in Modern Healthcare". Dr Catherine O'Connor, Global Director in Clinical Development, spoke to "Paediatric Drug Development and its Challenges" and Dr Onthatile T. Serehete, Senior Director in Global Patient Safety, gave a talk



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on “Ensuring Patient Safety and Effective Medical Monitoring during the drug development process”. The webinar provided valuable insights into the evolving field of Pharmaceutical Medicine and its impact on patient care. The topics highlighted the crucial role that pharmaceutical physicians play in driving innovation in clinical trials, drug development, and public health.

The association also runs annual lectures designed to equip pharmaceutical physicians with the most up-to-date knowledge and this year Dr Brendan Boland presented on “Development of a medical device in Ireland’s dynamic start up environment in 2024”. Brendan, a member of the Irish College of General Practitioners, the Royal College of Physicians, Ireland, and a former Fellow of the prestigious BiInnovate Ireland, is a co-founder of Loci Orthopaedics. He is the mastermind behind the company’s overall commercial and clinical strategic successes and is also a named inventor on six granted US patents. His address to the APPI was insightful and inspiring. Indeed, this focus on continuous learning is crucial in an industry that is constantly evolving.

Finally, the Association is also pleased to announce the appointment of Dr Róisín Flynn and Dr Catherine O’Connor to the positions of Vice-chairperson and Secretary, respectively. Dr Anthony Chan and Dr Ceara Belviso will continue as Chair and Treasurer, and Dr. Emer O’Shaughnessy and Dr Anne Tobin will lead on the Education Committee. Dr Onthatile T. Serehete will continue as the APPI National Representative for The International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine. As the APPI continues to grow, these new nominations to the committee are poised to complement the existing leadership, are committed to supporting pharmaceutical physicians in the association and hope to inspire the next generation coming down the track.

**Author: Dr Catherine O’Connor**

Secretary of the Association of Pharmaceutical Physicians of Ireland

## Early Access to Investigational Treatments

### What Data Can Be Collected under Named Patient Use or Compassionate Use?



With medicinal products taking 10-15 years to reach the market, many patients and treating physicians seek early access to investigational drugs. Early access pathways provide urgently needed treatments to patients facing life-threatening or chronically debilitating conditions when all other options have failed, are unsuitable, or are unavailable (Figure 1). These programmes are especially critical for patients with rare diseases, who often have limited or no access to clinical trials. As one example, Novartis’ Zolgensma was used outside clinical trials to treat nearly 300 children with spinal muscular atrophy, a rare hereditary genetic condition, over a period of 5 years in 40 different countries, ensuring access to the drug while market application was under review (1).



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Another example is Gilead's remdesivir, originally developed to treat hepatitis C, which was used to treat Covid-19 before market approval (2). More recently, 173 glioblastoma patients received a personalised peptide vaccine outside a clinical trial in Germany (3), amid some criticism (4).

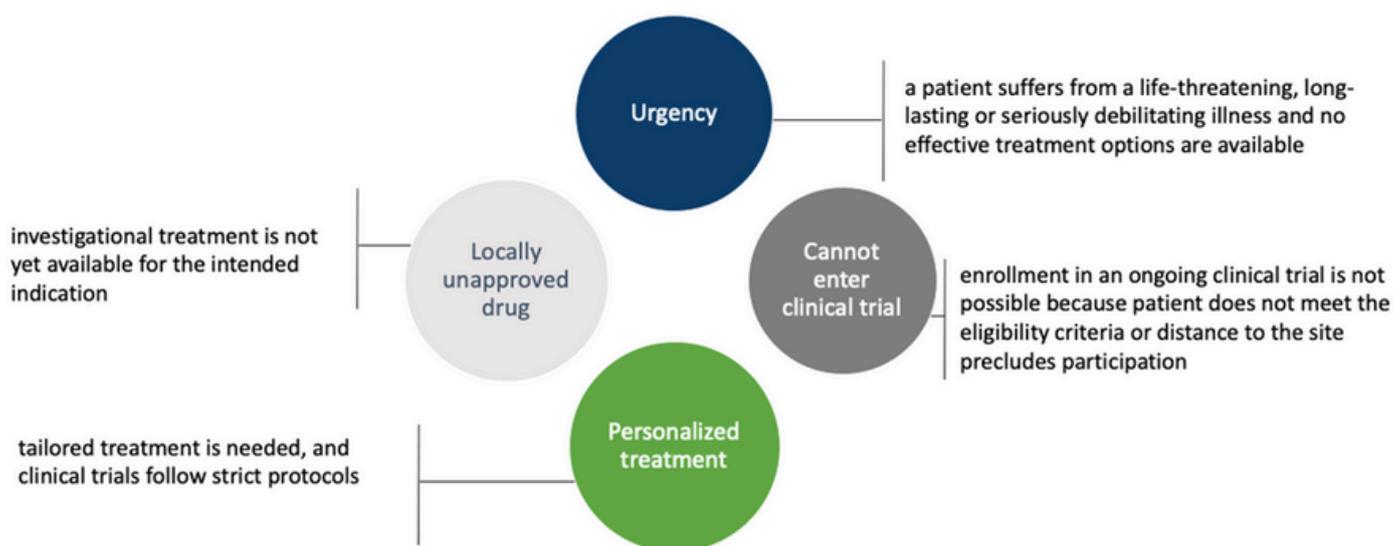


Figure 1: Factors driving access to investigational drugs outside clinical trials.

How is access to investigational drugs regulated? Unlike the harmonised framework governing their use in clinical trials, their use outside clinical trials reveals notable geographical disparities. In Europe, these programmes are typically categorised as named-patient use (for a specific patient) or compassionate use (for a group of patients). The regulatory agency EMA (5) is not directly involved in this process, leaving the decision to the member states. In the United States, the preferred terms are “expanded access for individual patients” and “expanded access for patient groups”. The FDA (6) coordinates both programmes. While named-patient use is initiated by the physician, compassionate use is often managed by the drug manufacturer. To illustrate the differences in the approval process in Europe, we cite the examples of the Netherlands/France and Austria/Germany. In the Netherlands and France, the treating physician must seek approval for named-patient use from national health authorities. This pathway requires that clinical data be available for review. The patient must be fully informed about the experimental nature of the drug and potential risks.

In Austria and Germany, by contrast, named-patient use (“Heilversuch”) does not require notification to or approval by the health authorities or the Institutional Review Board. The responsibility lies solely with the physician, who may, at times, treat a patient even before any phase 1 clinical trials have been conducted. As for compassionate use, the programme must be approved by the national agencies across Europe. However, not all countries benefit from an efficient scheme, and the disparity can result in a delay in access of more than three years depending on where the patients live (7). A list of compassionate use programmes approved in a country is often provided on the respective national agency’s webpages.



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While the primary goal is to offer patients access to treatment, there is a growing interest in gathering and analysing real-world data from these programmes for scientific publications (8) and regulatory submissions (9). A recent example where compassionate use data is being sought to complement clinical trial data in a FDA's review process is the case of Stealth Biotherapeutics' elamipretide. While the randomised clinical trial involving only 12 patients with Barth syndrome, an ultra-rare and life-threatening genetic disorder, did not meet its primary endpoint due to its small size and short duration, data gathered from patients treated under compassionate use showed encouraging results providing real-world evidence for the drug's effectiveness (10). Often, though, it is not clear to what extent companies can collect data during these early access programmes. A harmonised framework in Europe could streamline access and data collection, enhancing the impact of these programmes, while safeguarding patient safety. In the absence of such standardisation, biopharma professionals should proactively communicate with health authorities in the countries where the data is to be collected to understand what is possible and what not. Only by navigating these challenges effectively can the full potential of early access programmes be realised for patients, physicians, and the industry alike.

## Authors:

**Joana Enes**, PhD, Medical Writer & Scientific Advisor at Gouya Insights, member of GPMed (11)

**Kateryna Uspenska**, PhD, Senior Project Manager at Gouya Insights, member of the Young Professional Working Group at IFAPP, member of GPMed

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## Real-World Data and Real-World Evidence in Regulatory Decision Making

*Report of the Council for International Organizations of Medical Sciences (CIOMS) Working Group XIII (2024)*

### Introduction

Data from sources other than traditional randomised clinical trials are known as real-world data (RWD), and the evidence derived from the review and analysis of RWD is known as real-world evidence (RWE). RWD and RWE are used increasingly throughout the lifecycle of medicinal products to provide evidence about their effectiveness and safety. Recent regulatory guidance about RWE (1, 2, 3) and approvals based on the use of RWE to demonstrate beneficial effects of products have created an urgency to develop generally accepted processes that promote trust in the evidence-generation process. The CIOMS 2024 report (4) describes the use of RWE for decision making during the whole lifecycle of medicinal products, describes RWD and data sources, discusses key scientific considerations in the generation of RWE, and discusses ethical and legal issues in using RWD.

### Chapter 1: Real-world evidence for decision making during the product lifecycle

Several stakeholders use RWE to support their decision making, including medical product regulators, health technology assessment (HTA) organisations, healthcare payers, patients, health care professionals (HPCs), and pharmaceutical companies. Several medical product regulatory agencies have issued guidance on the key considerations for the use of RWE to support regulatory decisions. RWE can support decisions at several points in a medical product's lifecycle. For RWE to support decision making, sponsors, regulators, and HTAs should implement a transparent process of planning for reporting and evaluating RWE.

### Chapter 2: Sources of real-world data

The scope of RWD is broad, including health care data and federated systems of health care data, spontaneous adverse event reporting systems, ad-hoc data collection, as well as emerging sources such as mobile devices and biosensors. An important challenge in using novel devices to generate RWD is the need to assure the validity of the resulting data.

### Chapter 3: Real-world evidence for regulatory use: key considerations

RWD are often collected originally for reasons other than research. As a result, the fitness of specific RWD for specific research purposes needs to be assessed. The fitness of RWD depends on several factors including the research design that they will be used for. Chapter 3 outlines commonly used epidemiologic research designs and design elements and discusses considerations for the statistical analysis of RWD. It also summarises current best practices regarding study registration, transparent reporting, documentation and responsible communication, as well as the increasing focus on improving the reproducibility of studies using RWD.

### Chapter 4: Ethics and governance

Ethical and governance issues should be carefully considered when using RWD to generate RWE. These include not only privacy and data protection issues but also informed consent, as well as the efficacy-effectiveness gap between outcomes observed in randomised clinical trials (efficacy) and outcomes in real-world circumstances (effectiveness). One challenge is that while data protection laws protect the fundamental rights and interests of citizens in relation to the processing of their personal data, the laws can be too restrictive to use RWD for evaluating the effectiveness of medicinal products on citizens' health.



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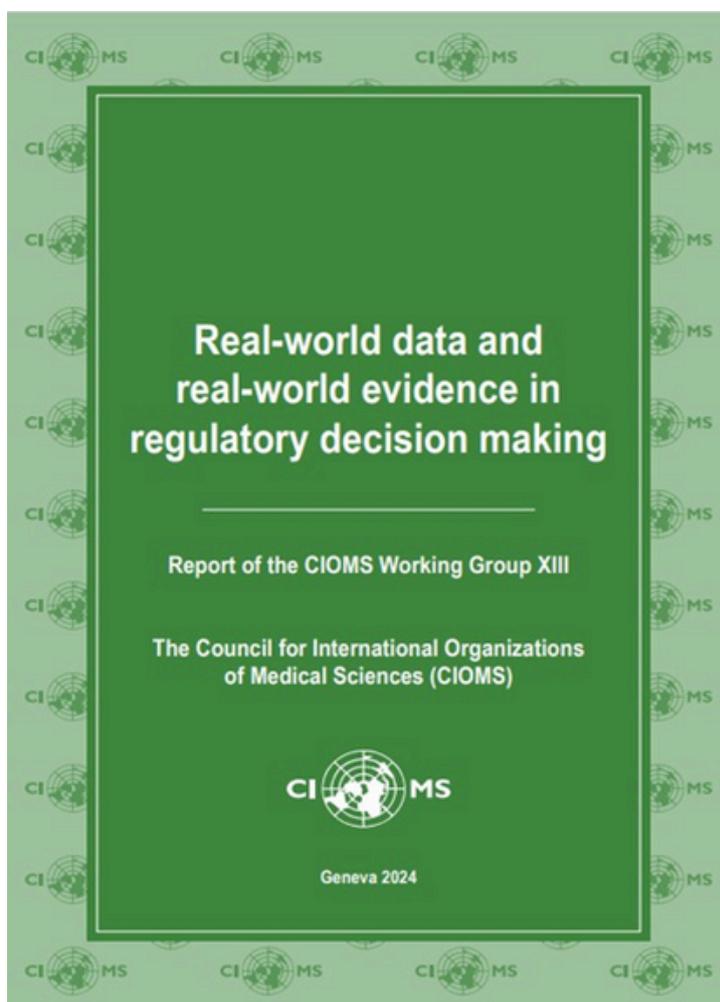
## Chapter 5: Conclusions and future directions

This report discusses the role of RWD/RWE in health-related regulatory decision making along the medicinal product's lifecycle and the needs of different stakeholders, available data sources, key scientific considerations, as well as the ethical and legal perspectives. More work remains to be done to globally harmonise practices and guidance for using RWD and RWE for regulatory decision making, thereby maximising the benefits they can bring to public health.

**Author: Dr Lembit Rägo**, Secretary-General, Council for International Organizations of Medical Sciences, Geneva, Switzerland

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3. US FDA. Real-world evidence. ([Website](#) accessed 16 May 2024.)
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## IFAPP WEBINAR

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### 2025

*The 2024 Revision of the Declaration of Helsinki: A Step Toward More Global Research Ethics*

**20 FEBRUARY 2025**

**1:00 - 2:00 PM CET**

### TIME SCHEDULE

07:00 - 08:00 AM EST  
12:00 - 01:00 PM GMT  
01:00 - 02:00 PM CET  
09:00 - 10:00 PM JST



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The DoH is often recognized as the constitution of research ethics. Its latest revision encompasses all the fundamental principles that emerged recently in global ethics, starting with the 2016 CIOMS (1) International Ethical Guidelines. It strives toward reinforcing the respect for and protection of human research participants and the quality and social value of research. More than ever it demonstrates the engagement of the researchers toward the highest ethical and scientific standards in the respect of human rights.

## SPEAKER



### Prof. Dr. iur Dominique Sprumont

Professor Dr. Dominique Sprumont is Professor of Health Law at the Institute of Health Law of the University of Neuchâtel, a WMA (2) academic partner and President of the Research Ethics Committee of Vaud, Switzerland ([www.cer-vd.ch](http://www.cer-vd.ch)), and past vice-director of the Swiss School of Public Health.

#### Abbreviations

- 1) CIOMS: Council for International Organizations of Medical Sciences
- 2) WMA: World Medical Association

[Register in advance for this webinar](#)

After registering, you will receive a confirmation email containing information about joining the webinar.



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## Summary of the Webinar of 23 January 2025: Empowering the Next Generation: A Conversation with eYPAGnet



Speakers were **Begonya Nafria**, eYPAGnet (1) Steering Committee member and Head of the Patient Engagement in Research at SJD Barcelona Children's Hospital, and **Ségolène Gaillard**, eYPAGnet Steering Committee member and PPI (2) expert of the Hospices Civils du Lyon

### Overview

Begonya Nafria and Ségolène Gaillard gave an excellent overview on the importance of collaboration with young people in the development of clinical trial design.

The eYPAGnet provides a platform for children and young people to have a voice across Europe, share their opinions, and apply their experience to a variety of issues in clinical trials such as relevant endpoints, protocol design, formulations, age-appropriate information, and patient tools.

The majority of eYPAGnet members include young people aged between 8-19 years who are patients, regular attendees at a hospital, and/or have an interest in science, healthcare, and children's rights.

The eYPAGnets are predominantly facilitated by a professional involved in a clinical research facility, children's hospital, or academic institution. The rationale is

- to improve the capacity of collaboration between children, young people, and stakeholders who participate in the research process and development of innovative drugs, and
- to promote the planning and development of clinical research initiatives for children at a European level.



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## eYPAGnet: Who we are

### Steering Committee Experts

**+ 50 years**  
of expertise in PPI

- **2006** First YPAG in the world
- **2012** Creation of eYPAGnet
- **2015** iCAN formed
- **2017** Recognition of EnprEMA
- **2018** Key partners in c4c



**Jenny Preston**  
Senior PPI Engagement Manager,  
Alder Hey Hospital, UK



**Begonya Nafria**  
Patient Engagement in Research Coordinator  
Barcelona Children's Hospital - Spain



**Pamela Dicks PhD**  
Manager Children's Research Network  
and Patient/Public Involvement Lead  
NHS Scotland



**Segolene Gaillard**  
Patient/Public Involvement Lead  
Rare disease network manager  
University Hospitals Lyon, France

### European Network of experts in PPI

01

Professionals embedded in **Children's Hospitals and Clinical Research Facilities**

02

Alongside clinicians and research nurses conducting paediatric trials in **all paediatric specialties** including rare diseases

03

Direct link with **patients and parents**  
Collaboration with **patients associations**

04

Experience in all **clinical trials** phases, longitudinal studies, **non-drug interventions** and **medical device research**



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## European Network of experts in PPI

05

Leading experts in **developing and delivering bespoke PPI plans** that meet the needs of the client and that are consistent with eYPAGnet core values

06

### Working with

- Parents and patients living with paediatric diseases
- YPAG's
- Patient Organisations
- National Paediatric Clinical Research Networks
- International Paediatric Clinical Research Networks: c4c-S
- Paediatric Hospital Services
- Paediatric Clinical Networks and Research Facilities
- Industry or academics partners

## eYPAGnet TODAY

### 14 countries

- Belgium
- Czech Republic
- Denmark
- Estonia
- Finland
- France
- Germany
- Ireland
- Italy
- Netherlands
- Poland
- Portugal
- Spain
- United Kingdom

### Founder teams

- Generation R
- Kids France
- ScotCRN
- Kids Barcelona



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## What are the challenges eYPAGnet?

### Challenges

- ▶ Acceptability of formulation
- ▶ Clinical trial protocol:
  - ▶ Study design
  - ▶ Study endpoints relevant for Patients
  - ▶ Quality of life scales and PROMs/PREMs adapted to the disease
  - ▶ Options of digitalization and decentralization of clinical studies
  - ▶ Etc.
- ▶ Informed assent/consent documents
- ▶ Strategies of recruitment

## What are we providing?

### Some of our services

#### Study specific support

- Co-design of clinical trial protocols
- PROMS, PREMS, QoL...
- Development and review of patient documentation...
- etc...

#### Education and training of key stakeholders

- Co-creation (with children and families) educational resources
- Development of training for key stakeholders

#### PPI advice and coordination of patient and public involvement activities

- Development of patient and public involvement plans
- Execution of PPI plans

#### Reporting and measuring the impact of involvement

- Evaluation and impact assessments of involvement activities
- Access to co-created reporting tools



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Where to find more and detailed information?

Respecting the rights of children  
and young people

Thank you!

[info@eypagnet.eu](mailto:info@eypagnet.eu)

**Author:** Birka Lehmann, MD PhD, GFMD, IFAPP Education and Certification Working Group Chair, Senior Expert Drug Regulatory Affairs

Slides provided by **Begonya Nafria**

Abbreviations

1) eYPAGnet: European Young Person's Advisory Group Network

2) PPI: Patient and public involvement



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## Don't Miss Out: ICPM 2025 is Approaching Fast! Amsterdam, 9-11 April 2025

Our flagship event, the International Conference on Pharmaceutical Medicine (ICPM 2025) with the theme "Purpose for future", is coming up soon! This is the perfect opportunity to meet (international) colleagues, network, and learn from renowned speakers. Check out the inspiring programme on our website: [www.icpm2025.com/programme](http://www.icpm2025.com/programme).

### And we have good news!

The Organizing Committee has decided to extend the Early Bird registration at a reduced rate until **15 February 2025**. Take advantage of this unique opportunity and register now at [www.icpm2025.com/registration/](http://www.icpm2025.com/registration/)

### A Special Milestone

During ICPM 2025, we will also celebrate the 50th anniversary of IFAPP! This is a unique chance to be part of a historic moment in Pharmaceutical Medicine.

### Programme

We are proud to announce that the programme is now complete! The Programme Committee has worked hard to put together an inspiring and impactful line-up of national and international speakers.

With thanks to Rudolf van Olden, Peter Bertens, Bart Scheerder, Ellen W. Evelaar, MD PhD, Eric Klaver, and team captain Henk Jan Out.

We look forward to welcoming you to Amsterdam!

21<sup>ST</sup> INTERNATIONAL CONFERENCE ON PHARMACEUTICAL MEDICINE 2025

Register now for **ICPM 2025**

**Early bird registration extended!**

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DAYS MORE...

Deadline is 15th February, 2025 | [www.icpm2025.com](http://www.icpm2025.com)

THEME: PURPOSE FOR FUTURE

9TH - 11TH APRIL, 2025

Koepelkerk Amsterdam

IFAPP NVFG



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## IFAPP WEBINAR

≡≡≡ 2025

*“Cardiovascular Gender  
Pharmacology”*

**20 MARCH 2025**

**12:00 - 1:00 PM CET**

### TIME SCHEDULE

07:00 - 08:00 AM EST  
11:00 - 12:00 AM GMT  
12.00 - 01:00 PM CET  
08:00 - 09:00 PM JST



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In this webinar, we will explore sex differences in cardiovascular pharmacology by examining specific examples of commonly used cardiovascular drugs. Following the presentation, an open discussion session will provide an opportunity to collectively draw conclusions and explore future directions in cardiovascular pharmacology. Participants are encouraged to contribute their expertise from other fields during the discussion session.

## SPEAKER

**Dr. Rubén Fuentes Artiles, M.D.**  
(University Clinic/Inselspital  
Berne, Switzerland)



*Dr Rubén Fuentes Artiles is a medical doctor with a specialisation in Sex- and Gender-specific Medicine (Certificate of Advanced Studies from the Universities of Zurich and Bern, Switzerland). He started his education as a cardiologist at the Limmattal Hospital and the Triemli Hospital in Zurich and is currently working as a Resident Physician at Bern University Hospital in the Department of Cardiology.*

[Register in advance for this webinar](#)

After registering, you will receive a confirmation email containing information about joining the webinar.



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## THE FLAG

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### **IFAPP Communication Working Group**

Ghazaleh Gouya-Lechner (Chair), Varvara Baroutsou, Francesco Butti, Brigitte Franke-Bray (Editor), Anna Jurczynska, Rita Lobatto, Hasan Mahmood, Kotone Matsuyama, Yasmin Nagaty (Editor), Helio Osmo, Joanne Ramsey, and Alexandra Reis Stoffel

IFAPP is the International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine.

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