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The Global Pharmaceutical Medicine Journal

INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS AND PHARMACEUTICAL MEDICINE

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Announcement of the 2024 IFAPP Fellowship Awardees

We are delighted to announce the recipients of the 2024 IFAPP Fellowship Awards. These awards recognise outstanding contributions and achievements in the field of Pharmaceutical Medicine. This year's awardees have demonstrated exceptional dedication, innovation, and leadership in their respective fields.

We would like to thank all professionals and experts in Pharmaceutical Medicine, who applied for the IFAPP Fellowship Awards, and we are excited to name the awarded professionals.

Award Categories and Recipients:
Note: The names of the awardees are announced in alphabetical order of the first name.

Scientific Leadership in Pharmaceutical Medicine:
For over 15 years of exemplary service and leadership in advancing Pharmaceutical Medicine.

- Akiyoshi Uchiyama, Chairman/President, Medical Corporation Asbo, Tokyo Asbo Clinic, Japan
- Arturo Lopez Gil, Vice President & Cluster Medical Director, Spain, Portugal, Israel, Spain

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- Carla M. Visseren-Grul, M.D., Vice President, Oncology Global Clinical Development, Loxo@Lilly, Eli Lilly and Company, The Netherlands
- Edward Stewart Geary, Global Safety Officer, General Manager, Eisai, Japan
- Grigorios Rombopoulos, Chief Scientific Officer, Novartis Hellas SACI, Athens, Greece
- Hiroshi Ando, Associate Director, Medical Affairs Strategy Lead, Genmab K.K., Tokyo, Japan
- Kazuya Iwamoto, Senior Medical Director, Fortrea (Formerly Covance and Labcorp), Japan
- Kihito Takahashi, VP, Head of R&D/Medical Affairs & CMO of Swedish Orphan Biovitrium (SOBI), Japan
- Krishan Thiru, Head, Country Medical, Australia & New Zealand, Pfizer Inc., Australia
- Lisa Stagi, Patient Safety Country Cluster Lead, Roche International Pharmacovigilance, Italy
- Maria Katsara, Medical Manager Greece, Cyprus, Malta, Recordati Rare Diseases, Greece
- Michiko Tomiyasu, Medical Affairs (MA) Dept., MSL Excellence Lead, Alexion Pharma G.K., Japan
- Paola Trogu, Senior Director Country Head Italy and Greece - Site Management & Monitoring, Astra Zeneca, Italy
- Sandor Kerper-Fronius, Semmelweis University, Medical Faculty, Department of Pharmacology and Pharmacotherapy, Professor of Clinical Pharmacology, Hungary

- Takeshi S. Komatani, TAKASHIMA International Patent Office, Patent Attorney Visiting Professor at Kobe University and Doshisha University, Japan
- Taku Seriu, Executive Director, Otsuka Pharmaceutical Company; Consultant, Japan
- Toshifumi Sugitani, Syneos Health, Director, Real World Evidence, Japan
- Xavier Marfà Pons, Head of Pharmacovigilance Sanofi Spain & Portugal, Barcelona, Spain

Scientific Excellence in Pharmaceutical Medicine:
For significant contributions and excellence in Pharmaceutical Medicine over the past 10-15 years.

- Fumiyo Aoki, Team Manager, Compliance & Management, AbbVie GK, Japan
- Hirokazu Murakami, Medical Science Liaison Manager, Merck Biopharma, Japan
- Kouki Nakamura, MSL lead, Senior Manager, Inflammation and Immunology, Medical Affairs, Pfizer Japan Inc., Japan
- Mario Spione, Medical Lead Oncology and Hematology, Servier Italia, Italy
- Ryu Suzuki, Clinical Development Strategy & Project Management Group, Nippon Kayaku Co., Ltd., Japan
- Yoichi Inoue, Senior Director in Immunology, Pulmonary Hyper-tension and Neuroscience, Janssen Pharma KK, Japan

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Rising Star in Pharmaceutical Medicine:
For remarkable achievements and potential in Pharmaceutical Medicine with over 3 years of experience.

- Marisa Le Donne, Clinical Research Associate, European Cardiovascular Research Center – CERC responsible for Italy - UK – Asia, Italy
- Masanori Yagi, Senior Medical Science Liaison/MSL Lead, Medical Affairs, Moderna Japan Co., Ltd., Japan

We congratulate all the 2024 IFAPP awardees for continuing to strive for ethical, innovative and scientific leadership and for contributing to global medical research for better human health and social value worldwide.

As illustrated on the pictures the ceremony for the Japanese awardees took place on 26 July 2024 at the occasion of the annual JAPhMed Congress.
They received a pen which has a carved sign with both IFAPP and JAPhMed as a memento.

Note: The awardees' names and titles are published with their permission.

Authors: Varvara (Barbara) Baroutsou, IFAPP President **and Anna Jurczynska,** IFAPP Secretary

Pharmacovigilance and Patient Safety: Strengthening Global Efforts to Reduce Diagnostic Errors on World Patient Safety Day

An accurate and timely medical diagnosis is essential for safeguarding patient safety and delivering appropriate, effective preventative or interventional treatment. Errors may arise at any stage of the diagnostic process, leading to potentially serious repercussions. Delayed, inaccurate, or missed diagnoses can extend the duration of illness, potentially resulting in disability or, in some cases, fatal outcomes, as well as increasing overall healthcare costs and burden on healthcare systems (WHO, 2024).

In recognition of the critical role of accurate diagnosis in health, this year's World Patient Safety Day on 17 September, organised by the World Health Organization (WHO), is focused on improving diagnosis for patient safety, employing the slogan "Get it right, make it safe!" (WHO, 2024).

The diagnostic process involves many iterative steps, including the patient's initial presentation history taking and examination, diagnostic testing, discussion, and communication of results, collaboration and coordination on the final diagnosis, and treatment plan follow-up and re-evaluation (WHO, 2024). Errors can occur at any point. In the context of diagnostic testing (labs, assessments, imaging, etc), errors can be classified according to point of occurrence in the process, from pre-analytical to post-analytical stages. Pre-analytical errors involve issues before the actual analysis, such as incorrect sample collection, handling, or labelling, which can compromise the integrity of the specimen. Analytical errors arise during the testing phase itself, including technical inaccuracies, human errors, or equipment

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malfunctions. Post-analytical errors occur after the analysis, where misinterpretation of results, delayed reporting, or failure to communicate findings properly can lead to incorrect clinical decisions and adversely impact patient outcomes. To further mitigate diagnostic errors and enhance patient safety, healthcare systems have implemented robust quality control procedures, comprehensive quality assurance programmes, and stringent accreditation standards for laboratories and educational certifications (Plebani, 2007). The remarkable progress in analytical techniques, laboratory instrumentation, and automation over the past 50 years has dramatically improved analytical accuracy, leading to a substantial reduction in overall error rates, particularly in the analytical phase (Plebani, 2007).

However, despite these advances, according to the data from WHO, diagnostic errors account for 16% of preventable patient harm and are common in all healthcare settings (WHO, 2024). A range of solutions are available to address diagnostic errors. The WHO recommends that policymakers and healthcare leaders should foster positive workplace environments and provide quality diagnostic tools. In addition, health workers should be encouraged to continuously develop their skills and address unconscious bias in judgement (WHO, 2024). Patients should be supported to be actively engaged throughout their diagnostic journey (WHO, 2024). The WHO further states that correct and timely diagnosis requires collaboration among patients, families, caregivers, health workers, healthcare leaders and policymakers, noting all stakeholders must be engaged in shaping the diagnostic process and empowered to voice any concerns (WHO, 2024)

Challenges in the Diagnosis of Adverse Drug Reactions

In the realm of Pharmaceutical Medicine (PM), the diagnosis of adverse drug reactions (ADRs) remains a critical concern. ADRs are a significant health concern and rank among the leading causes of death globally, estimated to be between the fourth and sixth most frequent cause of death worldwide, alongside major causes of mortality such as heart disease, cancer, and stroke (Louet and Pitts, 2023). In addition, the financial burden of ADRs is extremely high, estimated at 30.1 billion US dollars in the United States and 79 billion Euro in the European Union (Louet and Pitts, 2023). In the United States, ADRs account for 3 to 7% of all hospitalisations (Marsh, 2023). ADRs are also reported to occur in 10 to 20% of hospitalised patients, of which approximately up to 20% are classified as severe, underscoring the critical need for vigilant safety monitoring and preventative management (Marsh, 2023).

The diagnosis of ADRs is often challenging and complex. ADRs often mimic “natural illness” with their own disease course and there are few specific diagnostics tests to confirm a symptom or illness is due to an ADR. As such, diagnostic uncertainty often surrounds most ADRs, and the association between an ADR and a given therapeutic is often assessed on a scale (relatedness), such as unrelated, unlikely related, possibly related, probably related, or related. Moreover, the use of widespread polypharmacy which may interplay and potentiate adverse effects, as well as the use of novel advanced therapeutics, such as immunotherapies and cell and gene therapies have further complicated the diagnosis of ADRs.

Global Safety Advancements in Pharmacovigilance

New techniques and technologies are helping to advance the field of pharmacovigilance (PV) to improve patient safety worldwide. In recent years, the development of artificial intelligence (AI) has rapidly expanded. AI can significantly enhance diagnosis in PV by rapidly analysing large datasets to identify risk patterns, predicting adverse drug reactions, and assisting in early detection of potential safety issues, ultimately improving patient outcomes and ensuring more precise and timely interventions. Moreover, at the patient-level, AI may facilitate real-time

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monitoring, analysing laboratory and imaging data, and applying predictive models to improve available formulas for computed tests or to predict outcomes (Padoan and Plebani, 2022).

Pharmacogenetic testing is an innovative diagnostic screening approach that tailors medication choices and dosages to an individual's genetic profile, thereby offering the potential to optimise therapeutic outcomes and minimise ADRs by predicting how a patient is likely to metabolise and respond to specific medications. This allows for prevention of potential ADRs via selection of an alternative treatment choice or enable identification of patients who require careful dosing and enhanced monitoring throughout treatment. A characteristic example is the use of testing for Dihydropyridine Dehydrogenase Deficiency (DPD) in patients scheduled to receive anticancer chemotherapy with 5-fluorouracil (5-FU) or its oral prodrug capecitabine. It is estimated that 2 million people receive 5-FU or its prodrug annually (Diasio and Offer, 2022), with data reporting that up to 30% of patients receiving 5-FU agents experience severe toxicity requiring hospitalisation and with up to 1% suffering fatal toxicity (Fidai et al., 2018). Identification through pharmacogenetic testing of patients with DPD complete (<1% of the population) or partial (up to 8% of the population) deficiency, an enzyme essential for metabolism of 5-FU, can enable risk mitigation, through use of an alternative medication or through dose adjustment/reduction and careful monitoring of side effects (EMA, 2020). In 2020, the EMA recommended DPD genetic testing and provided 5-FU dosing guidance in accordance with these results (EMA, 2020). However, in many countries, including the United States, such testing is not required prior to 5-FU administration. As such, the PV and broader PM community must continue to advocate for use of such pharmacogenetic screening tests that can significantly enhance patient safety.

The use of novel biomarkers in PV extends beyond pharmacogenetic testing. Novel safety biomarkers are constantly being discovered, tested, and validated, and then subsequently employed in clinical development programmes. Moreover, novel and conventional biomarkers can be combined into routine drug safety monitoring and diagnostic algorithms. As a recent example, IFAPP PV Working Group member Dr Brandon Henry and his colleagues published an article in the journal Clinical Chemistry and Laboratory Medicine, in which they developed a biomarker-guided approach for safety monitoring of drug-induced muscle injury and rhabdomyolysis in clinical practice and new drug trials (Ostrowski et al., 2023). Such techniques can enable more rapid identification of diagnostically challenging ADRs, as well as guide de-challenge/re-challenge of the suspected causative agent to aid in reducing diagnostic uncertainty and improving clinical outcomes.

Advancements in diagnostics and technologies in PV have become increasingly critical in the long-term follow-up of cell and gene therapies, particularly in monitoring for genotoxicity. Given the potential for delayed adverse effects, such as insertional mutagenesis or other genetic alterations, regulatory frameworks now mandate rigorous safety monitoring for a period of 5 to 15 years post-administration. This extended surveillance period is essential for detecting late-onset toxicities, ensuring that any long-term risks are identified and managed appropriately to safeguard patient health.

Conclusion

Advancements in pharmacovigilance, such as AI, pharmacogenetics, and novel biomarkers, are pivotal in enhancing patient safety. As we recognise World Patient Safety Day, it is a reminder of the ongoing commitment needed to improve diagnostic accuracy and ensure the safe administration of therapies. The IFAPP PV Working Group invites you to get involved and share your thoughts on other critical aspects of improving diagnosis for patient safety. Together, we can drive meaningful progress in safeguarding patients worldwide.

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Authors:
On behalf of the IFAPP Pharmacovigilance Working Group

Brandon Michael Henry, MD, FACSc, FAPCR

Monika Boos, MD, PhD, LLM

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IPPOSI & the Irish ENP – Putting the Patient Voice at the Centre of Health Policy, Care, Research, and Innovation in Ireland

Founded in 2005 by a group of visionary patient representatives, the Irish Platform for Patients Organisations, Science & Industry (IPPOSI) is a membership organisation open to all groups with an interest in healthcare and research and development, Patient Representatives, Academic, Science or the Healthcare Industry. Members share an interest in solving challenges in three priority areas: access to medicine and innovation; health information and public and patient involvement.

IPPOSI's work is co-funded by a Department of Health grant and from its industry membership fees and supplemented by grants from both public & private sources at the national & international levels. It focuses on three core activities:

- EDUCATION to provide its members with the knowledge they need to engage with important issues;
- ADVOCACY to coordinate joint actions by its members to call for patient-centred solutions;
- INFORMATION disseminated to its members to share national and EU developments.

IPPOSI is a sustaining partner of the European Patients' Academy in Therapeutic Innovation (EUPATI) which has established National Platforms (ENPs) in several countries across Europe and worldwide.

National Platforms bring patient, academic and industry partners together to discuss patient education and patient involvement in medicines R&D as well as the broader health innovation ecosystem. Working together, National Platforms raise awareness about the vital role of patients, and members identify challenges and opportunities for joint action.

The Irish ENP is supported by IPPOSI. In fact, all IPPOSI members form part of the Irish ENP, a sub-committee of the IPPOSI Board. The ENP comprises patients and carers and their representatives, including fellows from the EUPATI Patient Expert Training Programme and graduates of the IPPOSI Patient Education Programme. Membership is equally open to academia and industry representatives, government health departments, regulatory bodies, policy makers, healthcare professionals, as well as medical journalists. Through regular formal and informal communications, members explore national patient education and training; PPI opportunities and challenges, topical policy issues and, most importantly, support each other through shared experience.

"I learned so much from all the other graduates' life journeys and have been so fortunate to make a lifelong network of supportive friends in which I learn from still every day."

-Stacey Grealis, IPPOSI Graduate, Patient Insight Partner UCD

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IPPOSI and the Irish ENP are especially focused on education and capacity-building initiatives that provide patients with the knowledge and skills they need to feel confident in their advocacy and to take a more active role in collaborating with other health stakeholders. A biannual 11-month Patient Education Programme covers the entire lifecycle of medicines research and development (R&D) from the design and execution of Clinical Trials to Regulatory Processes to Health Technology Assessment (HTA). The Programme enables and empowers the patient community in Ireland by:

- providing an understanding of the health innovation system and the places for the patient's voice in it;
- teaching patients how to better collaborate with the scientific, medical, and healthcare communities;
- developing patients' confidence and knowledge to speak more effectively on behalf of themselves and those they represent;
- facilitating a network of like-minded patients, carers, and their advocates.

"I would recommend the IPPOSI Patient Education Programme to caregivers, patients, nurses, anybody who has a role in patient care. The course has helped me both in my professional and personal life so far... Now that I have finished the programme, I hope to continue with advocacy work."

-Claire Murray, Programme Graduate

A Patient Capacity Building Programme aims to build Irish patient advocates' capacity, confidence, and leadership skills by providing access to inclusive training materials and expert advice. Distinct from the biannual Patient Education Programme, this skills-based training comprises a series of online events including workshops on substantive topics, technical training, and information sessions, delivered by external experts with the support of IPPOSI staff. It aims to help patient advocates engage in a variety of topics related to broad priority areas of Access to Innovation, Health Information and Public Patient Involvement. Past trainings have covered digital storytelling, impact measurement, Patient Involvement at Regulatory agencies; How to find, read & use scientific literature; Making a patient submission to the National Centre for Pharmaco-economics. The 2024 programme includes: e-Advocacy - How To Develop A Strong Digital Identity on Social Media; Understanding & Influencing Decision makers; Presentation Skills; and more.

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"I found participating in the IPPOSI patient capacity building programme to be a worthwhile experience, which has instilled tremendous confidence in my patient advocacy endeavours. During the programme I learned from some of the best and got to make many valuable connections with like-minded advocates."

-Barry McGrath, EUPATI Fellow, HS Ireland

For more information about IPPOSI and the Irish ENP please go to https://ipposi.ie/

Authors:
Dr. Caroline Whelan, Education & Outreach Coordinator, Irish Platform for Patient Organisations, Science & Industry
Dr. Derick Mitchell, CEO, Irish Platform for Patient Organisations, Science & Industry

Bridging Regulatory Gaps: The Path to Global Harmonisation in Cell and Gene Therapy

The rapid advancement of cell and gene therapy (CAGT) technologies promises to revolutionise medicine, particularly for the treatment of rare genetic disorders. In the five years between 2017 and 2022, the United States Food and Drug Administration (FDA) approved a total of five gene therapies for treatment of rare genetic diseases (FDA, 2024). The pace dramatically increased in 2023, a breakthrough year of approvals in the United States, in which five gene therapies alone were approved, including the first gene editing therapy utilising CRISPR/Cas9 (FDA, 2024). The pace has continued in 2024, with three new gene approvals in the first half of the year (FDA, 2024).

In 2019, the FDA predicted it would approve 10-20 therapies per year starting in 2025, a reality which is reflected by the overall CAGT pipeline (Hunt, 2024). Through 2023, there were over 2,562 drug developers and 1,800 clinical trials in the CAGT space, amounting to over 11.7 billion USD in total investment (Hunt, 2024). CAGT growth has been driven by several factors, including advancements in molecular biology, access to genetic and genomic data, a demand for personalised medicine, increased investment in research and development, and most importantly,

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clinical success. In fact, orphan gene therapies are 3.5 times more likely to be approved entering phase I trials than an average drug (Hunt, 2024). Finally, the platform nature of CAGT, which allows developers to quickly pivot a therapeutic platform from one target indication to another, will enable for rapid expansion of the CAGT pipeline and facilitate potential treatment thousands of rare single gene inherited disorders.

Yet despite the success, high start-up costs and heterogeneity in global regulatory frameworks continue to create barriers that slow down the development, approval, and distribution process. This is particularly critical in the development of CAGT for rare diseases, often with very small populations, in which the cost to bring a life-saving therapy to market is especially high, thus lacking commercial viability and not attracting investment. For such indications with an extremely low incidence, global trials are often required to reach sufficient enrolment. Compounding these scale issues, sponsors must navigate a complex web of differing regulatory schemes, which can delay development timelines and increase costs.

The lack of CAGTs regulatory uniformity, with varying requirements and guidelines across different countries, limits the global accessibility to new therapies. In established markets like the US, Europe, Japan, and China, CAGT regulations vary widely, impacting drug classifications, complicating approval pathways, and increasing the complexity and cost of manufacturing and preclinical testing requirements. Emerging markets often lack the regulatory infrastructure and experience to efficiently manage CAGT approvals, creating additional barriers for global access. As such, harmonisation of regulatory requirements is essential to streamline the approval process, reduce development costs, and ensure that life-saving therapies reach patients in need, including those in low-resource settings.

Regulatory bodies are recognising the importance of creating more unified frameworks to facilitate the global development and distribution of CAGTs. To that end, new initiatives have been launched aimed at beginning to shift the global regulatory landscape towards harmonisation.

In 2023, the International Council for Harmonisation (ICH) launched the Cell and Gene Therapies Discussion Group, aimed to develop a global forum and a strategic roadmap for harmonisation by discussing scientific and technical issues related to CAGT development (ICH, 2023). However, as noted by Peter Marks, director of the FDA's Center for Biologics Evaluation and Research, at a panel discussion at the 2024 American Society of Gene & Cell Therapy (ASGCT) annual meeting, formal ICH guidance for CAGT is not yet feasible due to the need for greater convergence. He advocates for a more unified global regulatory framework, particularly for rare diseases, and envisions achieving sufficient convergence in the next five to ten years to reduce regulatory burdens, akin to the current global standards for vaccines (Slabodkin, 2024).

Recently, the FDA announced a pilot programme to explore concurrent review of gene therapy applications, called the Collaboration on Gene Therapies Global Pilot (CoGenT Global). This programme, in collaboration with WHO and ICH member countries, which includes the European Union (EU), Japan, Canada, and Switzerland, aims to boost investment, development, and accessibilities of gene therapies worldwide by utilising the collective global patient population to attract significant investment, and simplifying the review process through coordinated efforts and pooled resources, which will minimise costly, time-consuming, and repetitive regulatory submissions and reviews (Lu and Abbott, 2024).

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The European Medicines Agency (EMA) has included CoGenT Global in discussions for its Committee for Advanced Therapies, highlighting the focus on integrating global perspectives in regulatory decisions (EMA, 2024).

CoGentT Global is modelled after the success of the FDA's Oncology Center for Excellence's Project Orbis, launched in 2019, which allows for simultaneous submission and review of oncology products by international partners. Project Orbis's impressive results, with a significant increase in applications and hundreds of regulatory actions since its inception, highlight the potential effectiveness and promise of the CoGenT Global programme (Lu and Abbott, 2024).

Streamlined approval processes would reduce complexity and costs for sponsors seeking approval in multiple countries and facilitate faster access to innovative therapies for patients. However, regulatory frameworks must also be specially adapted to accommodate the unique challenges of emerging markets. Capacity building and training programmes are essential to support regulatory oversight in low- and middle-income countries. Ensuring equitable access to advanced therapies requires a concerted effort to address the high costs of manufacturing and distribution, as well as local infrastructure required for administration and patient care.

While the global regulatory landscape evolves, several unknowns will continue to present challenges to a unified framework. For example, the long-term durability of clinical responses observed remains yet to be determined, as is the risk for long-term adverse effects, such genotoxicity and oncogenicity, requiring prolonged follow-up times of up to 15 years post-treatment. Moreover, high reimbursements costs remain a major roadblock to accessibility, even in highly developed countries, with therapies often costings in the millions of dollars (USD) for a single one-time treatment.

Ongoing efforts towards harmonisation are critical to unlocking the full potential of CAGTs and ensuring global patient access. Continued collaboration among regulatory bodies, industry stakeholders, and international organisations is essential. As this process moves forwards, the pharmaceutical medicine community must be advocates bringing the patient voice to the process and utilising our hands on real-world experience with these therapies to provide critical feedback essential to maintaining a patient-centric approach. Harmonising regulatory frameworks will not only streamline the development process but also enhance global access to life-saving therapies.

Author:
Brandon Michael Henry, MD, FACSc, FAPCR,
Member of the IFAPP External Affairs Working Group

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About this webinar

In an evolving ecosystem of decision making, close interaction between regulators and health technology assessment bodies is paramount to enable patient access to important new medicines and hence for the benefit of public health. This aims to generate evidence relevant for regulators, HTA bodies and other stakeholders thereby re-shaping and focusing medicine development programmes.

Furthermore, acknowledging the dependencies and remits, collaboration can also facilitate sequential decision-making by sharing information in the context of the respective assessments. The webinar will focus on the newly evolving ecosystem in Europe, which provides unique opportunities for cooperation between the European Medicines Agency and the HTA Coordination group.

Agenda

Welcome: ***Varvara Baroutsou***, IFAPP President

- Introduction - 5 mins (***Birka Lehmann***)
- The new HTA Regulation in Europe – 20 mins (***Anne Willemsen***)
- Evolving collaboration at the HTA/regulatory interface – 20 mins (***Michael Berntgen***)
- Q&A – 15 mins

Register in advance for this webinar

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

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Austrian Living Document on Secondary Use of Real-World Data (RWD) in Health Research

GPMed
GESELLSCHAFT FÜR PHARMAZEUTISCHE MEDIZIN E.V.

The secondary use of Real-World Data (RWD) in health research is rapidly gaining traction, with the European Medicines Agency (EMA) catalogue already listing over 2,900 RWD studies (1). However, the evolving EU legal framework for health data use (e.g., Data Act, Data Governance Act, AI Act, EHDS Regulation, Clinical Trial Regulation, NIS2 Directive, Medical Device Regulation) presents a few challenges owing to its complexity, and it is difficult to really define the leeway for conducting research with RWD.

A group of Austrian experts, coordinated by the GPMed (Austrian Society for Pharmaceutical Medicine), met to analyse and discuss these challenges regarding secondary use of RWD in health research. These discussions resulted in a first orientation paper as "living document" reflecting the input and insights from the Austrian expert panel, representing various stakeholders from both health and legal institutions. It is intended to serve as a supporting material and to offer initial thoughts around the planning, set-up and implementation of medical or health economic outcomes research (HEOR) projects that require the secondary use of RWD in Austria. Hence, this living document is meant to assist anyone planning research projects that require RWD to answer their research questions.

RWD definition, relevance in health research and available sources in Austria

To assemble this working document, the GPMed coordinated an expert group that aligned with published definitions from health authorities (e.g., EMA and FDA) as well as local expert groups (2). They continue that RWD and the resulting Real-World Evidence (RWE) may complement findings from randomised clinical trials (RCTs) and generate supportive evidence when RCTs are not feasible or not representative. Examples for RWE utilisation include epidemiological studies and health economic evaluations. However, RWD have important limitations, including, but not limited to, data quality challenges, bias and confounding. Fit-for-purpose data, meticulous planning and a study design that fits both the research question and the available data are therefore crucial for addressing research questions (2).

If readers want to know what RWD sources are available in Austria, the authors refer to the following articles and reports:

- A national evaluation analysis and expert interview study of real-world data sources for research and healthcare decision-making (3)
- Registers in Austria and their use for improving healthcare, AIHTA project report no. 157 (4)
- Foundational paper on the creation of the Austrian health data space, Working Group on Digitalisation and Registers of the Supreme Health Council (5)
- Health Data in Austria – Report 2022, Gesundheit Österreich GmbH (6)

Boosting research by utilising RWD

In summary, the key to leveraging research with RWD starts with a clearly defined research question, as the study type and data required depend on it. Thus, invest adequate time in crafting the research question to ensure a high-quality study protocol. The document contains key questions that shall guide readers through common questions related to using RWD, thereby serving as a “checklist” to easily identify information that might be needed, in addition to practical suggestions.

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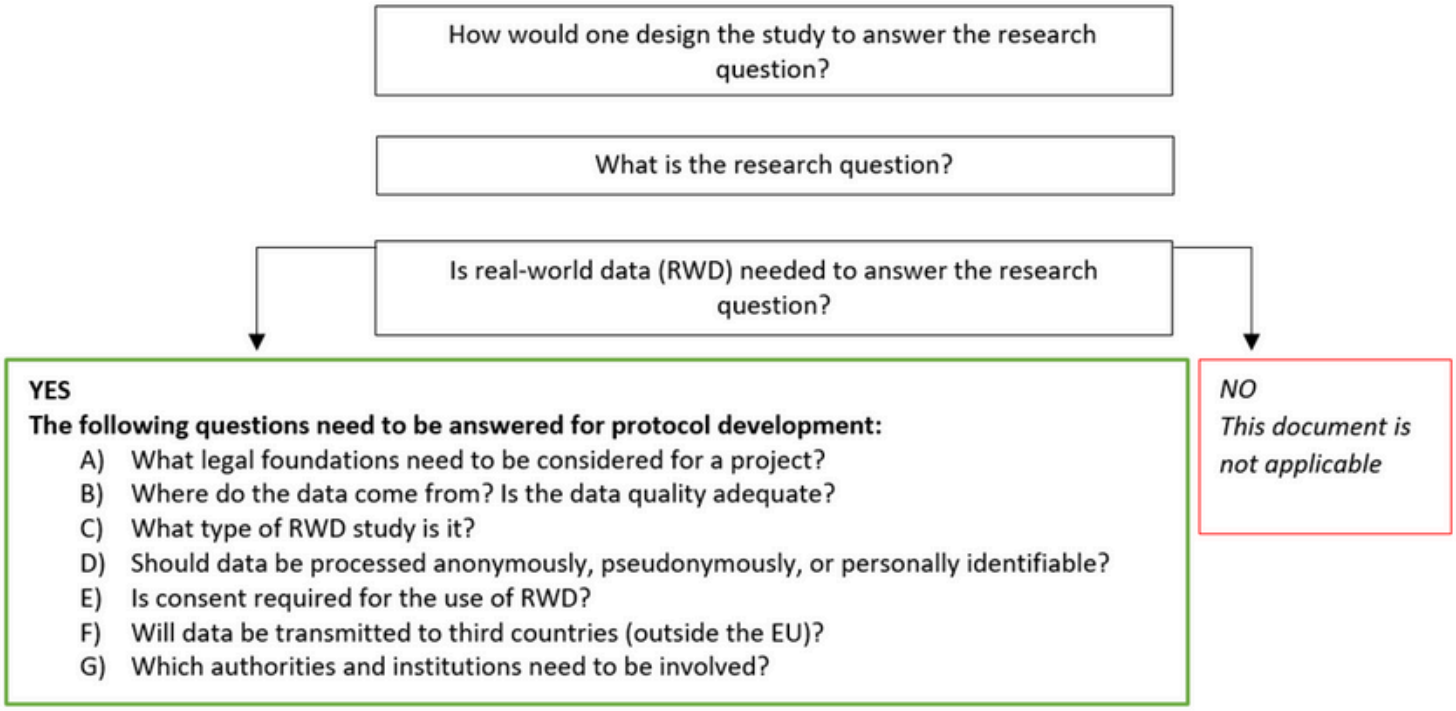

Diagram 1: Important questions on the applicability and use of the living document.

The living document elaborates on each of the questions and the laws and regulation within the Austrian healthcare ecosystem. It is available in German only and can be downloaded here: https://www.gpmed.at/gpmed-orientierungshilfe-forschen-mit-real-world-data-rwd/

Importantly, this living document offers advice and suggestions as how to proceed and contents can change over time. It was created to the best of the experts' knowledge representing the “status quo”. Critically, it also reflects the authors views and is neither to be understood as a guideline nor as a legal basis. The authors want to emphasise that it is essential that all legal and regulatory requirements, as well as the feasibility of implementation for each and every research project must be individually examined, weighed, and coordinated with the relevant authorities upfront.

Contributing experts (in alphabetical order): Baltic Dejan (GPMed, Amgen), Peter Fraisl (GPMed, Novartis), Ghazaleh Gouya (GPMed, Gouya Insights), Josef Haas (Ethics Committee of the Medical University of Graz), Florian Modler (GPMed, Roche), Veronika Mikl (GPMed, Roche), Žiga Škorjanc (University of Vienna, Legal Department), Tanja Stamm (GPMed, Ludwig Boltzmann Institute for Outcome Research, Medical University of Vienna), Stefan Strasser (BASG - Federal Office for Safety in HealthCare / AGES MEA - Austrian Medicines and Medical Devices Agency), Markus Zeitlinger (GPMed, Medical University of Vienna).

Author of summary and contact for inquiries: GPMed Research Innovation Circle, Veronika Mikl, veronika.mikl@gpmed.at.

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29th Annual Swiss Symposium in Pharmaceutical Medicine 2024

27th November 2024
Musikschule, Florhofgasse 6, Zurich

Special Populations in Drug Development and Clinical Care: Across the Entire Life Span from Paediatrics to Geriatrics

Chairs: Antonio Amodeo, CTC, Zurich, and representative from industry

The 29th Annual Swiss Symposium in Pharmaceutical Medicine will be organised by the Swiss Society of Pharmaceutical Medicine (SGPM) in partnership with and scientific support from the European Center of Pharmaceutical Medicine (ECPM).

With a keen focus on Vulnerable Populations, the symposium promises an insightful keynote address elucidating the Ethical Aspects inherent in addressing their unique healthcare needs. Delving deeper, sessions will delve into the intricacies of Developmental Pharmacology and Pharmacometrics, offering strategies to advance Neonatal and Paediatric Drug Development, alongside comprehensive discussions on Clinical Trials in Children, featuring insights from Swissmedic, the Swiss Agency for Therapeutic Products.

The focus of this symposium lies on Vulnerable Populations and Ethical Aspects of such populations will be discussed in a key-note speech following the welcome addresses. There will be presentations on Developmental Pharmacology and Pharmacometrics to Facilitate Neonatal and Paediatric Drug Development as well as on Clinical Trials in Children. Swissmedic, the Swiss Agency for Therapeutic Products, has been requested to provide a speaker for a talk on Regulatory Aspects of Clinical Trials in Children.

Gender Pharmacology will be addressed as well as a Case Study about the Use in Multiple Sclerosis during Pregnancy and a presentation on the Inclusion of Geriatric Populations in Clinical Trials.

Galenical Considerations and Application Systems for Paediatricand Geriatric Patients will be presented and also Nutrigenomics; Interventions for the Ageing Population.The final talk will be on Legal Considerations of Clinical Testing in Vulnerable Populations.

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Vienna School of Clinical Research (VSCR) is a leading provider of Capacity Building Programmes for International Health Policy Makers

VSCR and its faculty are increasingly engaged in Capacity Building Programmes for public and institutional health policy makers/payers from various geographies:

In 2024 a large number of policy makers and payers from **GCC countries** (United Arab Emirates, Qatar, Kuwait, Oman, Bahrain) successfully have accomplished **VSCR´s Diploma Programmes in Health Outcome Research (HEOR) and HTA Technology.** This programme was executed in cooperation with **EHES** (Emirates Health Economic Society) and **I&S** (Ideas and Solutions, Hungary).

Currently a large number of health policy makers/payers from **Sub-Saharan Africa (SSA)** including countries such as Ghana, Nigeria, Ivory Coast, Cameroon, Senegal, Namibia, Botswana, Kenya, Rwanda have started VSCR's HEOR Diploma programme. This SSA programme will continue until the end of 2025.

Selected **European stakeholders** attended successfully VSCR´s HEOR Diploma programmes in 2023 and 2024.

The programmes are designed and led by **Prof. Lieven Annemans**, MSc, PhD, Senior Professor of Health Economics, Ghent University, Belgium and **Prof. Heinrich Klech**, MD, PhD, Managing Director VSCR. and **Margarete Schreiner-Karner**, MSc, Operational Manager VSCR.

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Author: Margarete Schreiner-Karner,
MSc, Operational Manager VSCR

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IFAPP Secretariat - Leidsestraatweg 41d - 3443 BP Woerden - The Netherlands
Chamber of Commerce 30224375 – VAT number NL817747321B02
Phone: (+31) 6 2291 1039 – e-mail: secretariat@ifapp.org – website: www.ifapp.org

IFAPP Communication Working Group
Ghazaleh Gouya-Lechner (Chair), Varvara Baroutsou, Francesco Butti, Brigitte Franke-Bray, Anna Jurczynska, Rita Lobatto, Hasan Mahmood, Kotone Matsuyama, Helio Osmo, Joanne Ramsey, Alexandra Reis Stoffel and Johanna Schenk (IFAPP TODAY Editor-in-chief).

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