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The Global Pharmaceutical Medicine Journal

INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS AND PHARMACEUTICAL MEDICINE

THIS ISSUE:

Bernhard Mraz Appointed New President of GPMed, the Austrian IFAPP Member Association

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Already during my studies in veterinary medicine, I discovered my passion for research. In the time between lectures and exercises, I volunteered on research projects and was able to gain a lot of laboratory experience in basic research. After completing my studies, I was immediately drawn to clinical research. I started my career as a CRA and project manager in a Contract Research Organisation (CRO), where I was able to supervise phase 1 to 4 studies in a wide variety of indications. Clinical research remained an important companion for me in my further professional career. What excites me most about it is that the current standard of care is challenged and that the boundaries of what is possible are constantly being pushed through regular small or even large successes. These successes ultimately lead to a continuous improvement in patient care.

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The basic prerequisite for this is the cooperative partnership of numerous important stakeholders. For me, GPMed is the ideal platform to work together not only to keep Austria as an attractive research location, but even to expand it further.

As newly elected president of the GPMed I am committed to continuing the journey we started together with our past president Johannes Pleiner-Duxneuner. Furthermore, I want to increase our value to our members and to expand into areas where we can contribute to our main objective – to strengthen Austria as an international location for the benefit of Austrian patients.

Mag. Bernhard Mraz, President GPMed, Head Medical Affairs Innovative Medicines at Novartis Pharma GmbH, Vienna, Austria

Ministries of Health and Agriculture Appoint New AGES Management Team: Johannes Pleiner-Duxneuner appointed as Technical Director, Anton Reinl's term as Commercial Director extended.

Vienna - The General Assembly of the Austrian Agency for Health and Food Safety (AGES) has unanimously appointed a new Executive Board: Priv.-Doz. Dr. Johannes Pleiner-Duxneuner, former President of GPMed, will take over the position of Technical Director as of 1 April 2024. The current commercial director, Dr Anton Reinl, has been reappointed for a further five years.

Dr Pleiner-Duxneuner, is a specialist in internal medicine with additional qualifications in clinical pharmacology, clinical research management and nutritional medicine. He has many years of professional experience in the medical field and industry, most recently as Head of Innovation to Business at Roche Austria GmbH.

"AGES plays an indispensable role in the fight against infectious diseases, counterfeit medicines, antibiotic resistance, residues in food, as well as in soil and seed testing, radiation and climate protection. I am delighted that Johannes Pleiner-Duxneuner will bring his expertise as an outstanding physician and his management experience to AGES. I am confident that, together with Anton Reinl, he will position AGES well for the future," said Austrian Health Minister Johannes Rauch.

Press Release: Bundesministerium für Soziales, Gesundheit, Pflege und Konsumentenschutz (BMSGPK). pressesprecher@sozialministerium.at; sozialministerium.at

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International Clinical Trials Day

International Clinical Trials Day, celebrated annually on 20 May, marks the pivotal moment in 1747 when Scottish physician James Lind began his groundbreaking study of treatments for scurvy. The day recognises the enormous contribution of clinical trials to advancing medical research and improving patient care worldwide.

Clinical trials have been instrumental in medical breakthroughs that have revolutionised healthcare. From the discovery of penicillin in 1928, which ushered in a new era in the treatment of infectious diseases, to the serendipitous findings around drugs such as sildenafil, first tested for hypertension and later famously used to treat erectile dysfunction. Such milestones underline the unanticipated benefits that clinical trials can bring.

Moreover, the importance of clinical trials extends beyond the discovery of new medicines; they also provide insights into additional uses for existing medicines. For example, aspirin, widely used for pain relief, has been shown to prevent blood clotting in small doses, helping to prevent heart attacks and strokes. Research is continuing to explore its potential to relieve symptoms in patients with depression. The phenomenon of drugs finding success in applications other than their original intent is a fascinating aspect of pharmaceutical development. Here are further notable examples:

Sildenafil (Viagra):

- Original Use: Developed to treat hypertension and angina pectoris.
- Serendipitous Use: During clinical trials, researchers noted that sildenafil had a marked effect on erectile dysfunction. This side effect was so significant that it redirected the drug's primary application to treating erectile dysfunction, making it one of the most famous cases of drug repurposing.

Minoxidil (Rogaine):

- Original Use: Introduced as an oral drug to treat high blood pressure.
- Serendipitous Use: Patients taking minoxidil started experiencing excessive hair growth (hypertrichosis) as a side effect. This led to the development of a topical formulation of minoxidil, which is now widely used as an over-the-counter remedy to promote hair regrowth and treat baldness.

Thalidomide:

- Original Use: Marketed in the 1950s as a sedative and used off-label to treat morning sickness in pregnant women.
- Serendipitous Use: After its initial market withdrawal due to severe teratogenic effects, researchers discovered that thalidomide is effective in treating leprosy and multiple myeloma. Its ability to modulate the immune system has led to a controlled, albeit cautious, reintroduction under strict regulations.

Bupropion (Wellbutrin/Zyban):

- Original Use: Developed and marketed as an antidepressant.
- Serendipitous Use: It was discovered that bupropion also helps in smoking cessation. This was an unexpected benefit noted during clinical trials when smokers reported decreased nicotine cravings while taking the drug. It is now prescribed as Zyban for smoking cessation, in addition to its use as Wellbutrin for depression.

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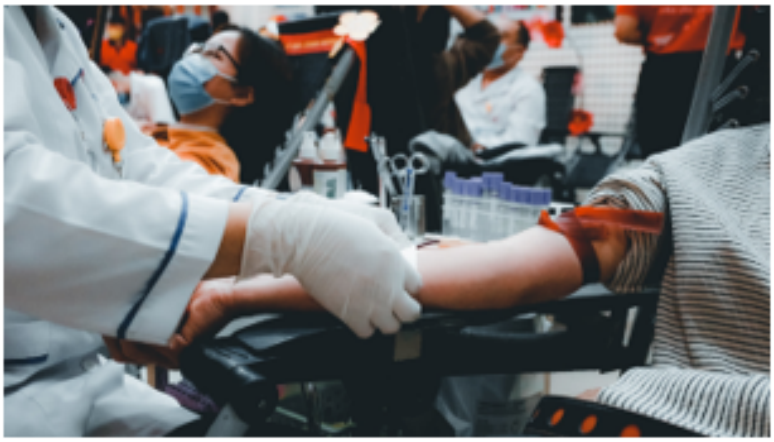
Finasteride (Propecia/Proscar):

- Original Use: Originally developed to treat benign prostatic hyperplasia (enlarged prostate).
- Serendipitous Use: During trials, it was found that finasteride also helped in promoting hair regrowth in male pattern baldness. This led to its approval as Propecia for the treatment of hair loss, in addition to its original use as Proscar for prostate enlargement.

In the field of aesthetic medicine, Botox was discovered to smooth wrinkles while being used to treat crossed eyes. This discovery has led to its use in other conditions such as migraines and Parkinson's disease. These examples show how clinical trials can lead to important dual-use applications of medicines, improving both health and quality of life.

The commitment of research teams and volunteers in clinical trials is vital. Each participant contributes to a vast pool of valuable data that helps understand new treatments and ensures safety and efficacy for future generations. On Clinical Trials Day, we recognise the efforts of those involved and reaffirm our commitment to advancing medical science for the benefit of society worldwide. Let's continue to support and invest in clinical trials, fostering innovation and ensuring that new treatments meet the diverse needs of patients everywhere.

Author: **PD Dr. Ghazaleh Gouya-Lechner**, founder and CEO Gouya Insights, Strategic Clinical Development Experts, board member GPMed, Austrian member association, and IFAPP.



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Bulgarian Association for Drug Information (BADI)

June 06, 2024 | Pharmaceutical Legislation - Update

ANNUAL CONFERENCE

Venue: University Hospital "St. Ekaterina" | Aula "Prof. Dr. Alexandar Chirkov", Sofia
Address: 52A Pencho Slaveikov blvd Sofia, Bulgaria

Working language: English
Format: in person

08:15 - 08:45 AM Registration & Welcome coffee
Main moderators: Prof. Tatyana Benisheva, DSci

08:45 - 09:15 AM Welcome and introduction to sessions
- *Ministry of health, Ministry of education and science.*

Welcome speeches
- *Prof. Dobriana Sidjimova, MU – Sofia*

09:15 - 10:00 AM European Pharmaceutical Legislation – Proposed changes by the EU - Parliament.
- *Prof. Dr. Barbara Sickmueller*

10:00 - 10:45 AM European Pharmaceutical Legislation - Focus on Drug Shortages, including Orphans & Paediatrics.
- *Dr. Christa Wirthumer-Hoche*

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Bulgarian Association for Drug Information (BADI)

10:45 - 11:00 AM *Discussion*

11:00 – 11:30 AM *Coffee break*

11:30 – 12:15 AM EU-CTR - transition period;
New approaches e.g. decentralized clinical trials;
- *Dr. Birka Lehmann*

12:15 - 12:45 PM EU-CTR – Clinical Trials Information System;
- *Prof. Tatyana Benisheva*

12:45 - 13:30 PM *Lunch break*

13:30 -14:15 PM Worksharing – Regulatory Update;
- *Radoslava Naydenova – Tunel*

14:15 - 15:00 PM Regulatory Strategies for Combination Products - Update and Future Perspective
- *Prof. Dr. Folker Spitzenberger*

15:00 - 15:45 PM Challenges of new MDR for the stakeholders
- *Ekaterina Genova, MD*

15:45 - 16:00 PM *Discussion & Closing speech*

Scan QR-code for registration

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Did You Know that RWD is a Valuable Source for Healthcare Research and Development, Policy, and Regulatory Decision-Making?

Despite global efforts for international collaboration to integrate real-world data (RWD) into regulatory decisions and fill knowledge gaps, some challenges remain.

On the initiative of the Research Innovation Circle of the Austrian IFAPP Member Association GPMed (Gesellschaft für Pharmazeutische Medizin), a study was carried out recently with the participation of numerous experts from the Austrian healthcare system, in which the Austrian RWD sources were evaluated using a multilateral enquiry approach, compared with already published criteria, and interviews were conducted with representative RWD sources. The paper titled “A national evaluation analysis and expert interview study of real-world data sources for research and healthcare decision-making” (1) was published recently in Scientific Reports 2024 by Mikl et al.

The article presents a comprehensive evaluation and expert interview study focusing on RWD for research and healthcare decision-making in Austria. The study outlines the challenges associated with accessing and using RWD sources and highlights the importance of addressing issues such as data silos, variable standardisation efforts, and governance issues. It emphasises the need for a national health data strategy and governance framework to inform researchers, policymakers, and decision-makers on the effective use of RWD in the health sector.

A key recommendation of the study is the establishment of a central repository of RWD to streamline access and ensure the quality of datasets used for research and decision-making.

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The authors advocate increased coordination, common data standards, and interoperability to maximise the benefits of RWD and avoid data silos. They also stress the importance of data exploration in linked datasets to capture the complexity of public and individual health issues.

The study highlights existing Austrian initiatives, such as sector-specific personal identifiers and the Austrian Microdata Centre, as positive examples of data use for research purposes. It calls for more ambitious and structured health data governance activities to facilitate a more comprehensive approach to data collection and use for future research and wider applications.

Finally, the findings underline the need for clear legal frameworks and improved data accessibility to generate added value from the use of RWD for individuals, society, and the health system. The objectives of the study are to provide an overview of available Austrian RWD sources for healthcare, to test and improve a quality checklist, and to discuss data quality aspects to improve the use of RWD for scientific and regulatory purposes.

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Summary by **PD Dr. Ghazaleh Gouya-Lechner**; founder and CEO Gouya Insights, Strategic Clinical Development Experts, and board member at GPMed and IFAPP.

1 Mikl, V., Baltic, D., Czypionka, T. et al. A national evaluation analysis and expert interview study of real-world data sources for research and healthcare decision-making. Sci Rep 14, 9751 (2024).
Foto credit: The Digital Artist on Pixabay

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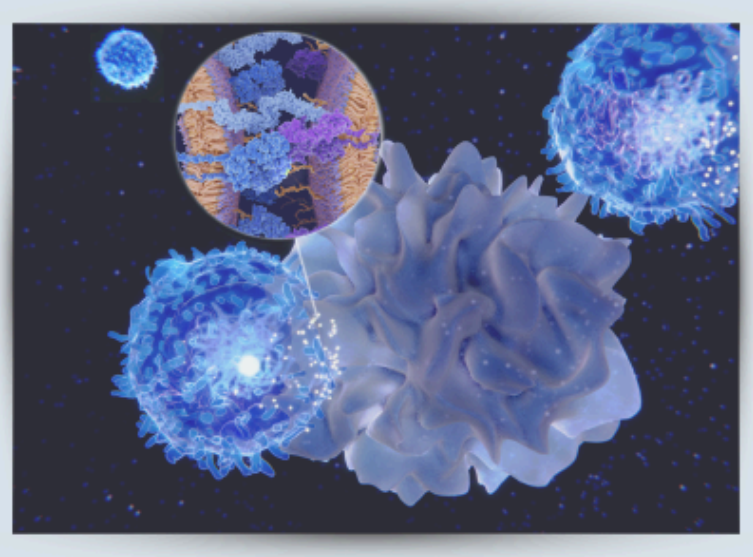
IFAPP WEBINAR

CAR T-Cell Therapy and Ethical Aspects (patients' involvement)



Wednesday, May 29, 2024

11:00 AM - 01:00 PM CEST

Time Schedule

05:00 - 07:00 AM EST
09:00 - 11:00 AM GMT
11:00 - 01:00 PM CEST
06:00 - 08:00 PM JST

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IFAPP WEBINAR

CAR T-Cell Therapy and Ethical Aspects (patients' involvement)

CAR T-cell therapy has demonstrated high response rates, particularly in certain types of blood cancers like acute lymphoblastic leukemia (ALL) and certain types of lymphoma. It has provided durable remissions in some patients who have not responded to other treatments. CAR T-cell therapy specifically targets cancer cells by recognising specific proteins (antigens) on their surface.

Besides the side effects like cytokine release syndrome (CRS) and neurotoxicity the most discussed topic is the access to treatment for patients. This in the light of the rarity of specialised centres and the expensive costs. This high cost can pose financial challenges for patients and healthcare systems, potentially limiting access to those who could benefit from the treatment.

Therefore, patients' involvement plays an important role in the ethical considerations in the CAR T-Cell therapy.

Register in advance for this webinar

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

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IFAPP Webinar - CTR Update Workshop | First Announcement

Only 6 months left for transition!

Introduction to New Developments in the CTR Framework

The "Clinical Trials Regulation", Regulation EU 536/2014, has been valid since 31 January 2022 and mandatory since 31 January 2023. The "Transition Period" will end on 30 January 2025.
Practical experience of competent authorities and sponsors have detected deficiencies and gaps when applying for authorisation, authorising, supervising and reporting clinical trials in CTIS, the overarching Clinical Trials Information System. This CTR Update Workshop will inform you about the latest developments from EMA and sponsor perspectives and give you an opportunity to discuss your questions and concerns.

Date: Thursday 27 June 2024
Time: 12:00 pm - 2:00 pm CEST
Moderator: Dr. Ingrid Klingmann, PharmaTrain

What has changed in CTIS (since March 2023) from an EMA perspective?
Oskia Bueno Zaragueta, EMA, Scientific Specialist
Data Analytics and Methods Task Force

Experience with working in CTIS, relevant changes and requirements for the sponsor
Nicole Woik, Biogen, Clinical Country and Site Lead

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Capacity Building in Medicines Regulation in Africa

In response to capacity and capability challenges within African National Regulatory Authorities (NRAs) for medicines and health products, experts and leaders from various NRAs in Africa convened in June 2020 to explore innovative ways to ensure Regulatory Sciences Capacity Development on the continent.

Among the interventions and strategies resolved up by the meeting, which was also attended by academics and scientists from various organisations, was the development and rollout of a capacity-building programme to address the critical skills gap observed in the evaluation of clinical sections of regulatory dossiers.

Following the convention, a major regulatory capacity-building initiative was established to address a long-term strategy, conceptual frameworks and implementation tactics for training and professional career development in African medicines regulatory agencies. This came in the form of a short course, “Dossier Assessment for Clinical Assessors within African Regulatory Agencies”. The course is accredited by the University of Witwatersrand in South Africa, and managed by the South Africa Health Products Regulatory Authority (SAHPRA), Medicines Control Agency of Zimbabwe, Ghana’s Food and Drugs Authority, Pharmacometrics Africa NPC, and the Fundisa African Academy of Medicines Development. As at April 2024, three cohorts of graduates have successfully completed this short course.

The objective of developing the 13-week course is not only to address the critical skills gaps in regulatory sciences on the African continent but also to address an essential part of patient safety. The course included perspectives from patient organisations like the National Osteoporosis Foundation of South Africa (NOFSA) and private sector players. Participants came from NRAs including Botswana’s Medicines Regulatory Authority, Ghana’s Food and Drugs Authority, Kenya’s Pharmacy and Poisons Board, Ethiopia’s Food and Drug Authority, SAHPRA, Rwanda’s Food and Drug Authority, Tanzania’s Medicines and Medical Devices Authority, Namibia’s Medicines Regulatory Council, and the Medicines Control Agency of Zimbabwe. Scientists not working for a regulatory agency have also been accepted into the programme.

The course is premised on addressing healthcare challenges and the critical skills gap in relation to medicines and health products regulation on the African continent. Graduates certainly acquire critical expertise in health products dossier assessment, which empowers African NRAs with new capacities and capabilities that will serve to ensure a robust regulatory ecosystem.

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The training programme has allowed for candid dialogue among NRAs, pharmaceutical industry stakeholders and academics in order to ultimately benefit patients with better therapeutic interventions, access and quality of care across the continent.

Feedback from students who have formed the three cohorts of graduates since its inception indicates the course has closed knowledge gaps in the medicine development process and the assessment of clinical data. It has also provided knowledge and technical expertise for the risk-benefit evaluation of medicines. These are skills that are crucial in ensuring the registration of safe, efficacious, and quality medicines for patients. The course has enhanced the evaluation competency of the clinical units within participating African NRAs and enabled the timeous evaluation of the clinical aspects of applications received for marketing authorisation.

References

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- Semete-Makokotlela, B., Mahlangu, G., Mukanga, D., Delese M.D., Stonier, P., Gwaza, L., Nkambule, P., Matsoso, P., Lehnert, R., Rosenkranz, B., Pillai, G. Needs driven talent and competency development for the next generation of regulatory scientists in Africa. Br. J. Clin. Pharmacol. 88: 579-586 (2021)
- Article: https://www.sahpra.org.za/news-and-updates/sahpra-invests-in-patient-safety-announcing-new-graduates/
- Report: A Primer for Clinical Assessors within African Regulatory Agencies

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2. **Prof. Colin Pillai**, CEO: Pharmacometrics Africa
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Source: SAHPRA

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THE FLAG

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