IFAPP TODAY

The Global Newsletter on Pharmaceutical Medicine

INTERNATIONAL FEDERATION OF ASSOCIATIONS OF PHARMACEUTICAL PHYSICIANS AND PHARMACEUTICAL MEDICINE

THIS ISSUE:

IFAPP European Regional Meeting is Coming Soon

I am delighted to announce in our April IFAPP TODAY newsletter that the upcoming IFAPP European Regional Meeting takes place in Amsterdam on 29-30th June 2023, starting on Thursday, the 29th, at noon and ending on Friday, the 30th, early in the afternoon.

Our Dutch colleagues from NVFG, a historic and dynamic National Member Association (NMA) of IFAPP, with the support of the IFAPP secretariat in the Netherlands, will host and support us in having a productive and agreeable meeting. In anticipation of their great contribution to a successful event, I convey my warmest thanks to Dr Ellen Evelaar, NVFG President, Dr Eric Klaver, NVFG Delegate, and Caroline van Bruggen, IFAPP secretariat.

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My first regional meeting as IFAPP President will be the starting point of our roadmap to the IFAPP Pharmaceutical Medicine long-term strategy, where region Europe NMA Delegates are invited to join, engage, and contribute their thought leadership for our planned initiatives, innovative approaches, benefits for our members, and collaboration with our stakeholders.

IFAPP Board Members will have the privilege to listen and learn from the invited NMAs their key initiatives, challenges, needs and expectations from IFAPP.

We will be also launching at this meeting the IFAPP strategy for the next generation IFAPP leaders by focusing on how to elevate young and middle career colleagues from various Pharmaceutical Medicine sectors and across the globe to create a stronger, inclusive and diverse, multidisciplinary Pharmaceutical Medicine fit for the future.

I personally envisage leveraging new generations to thrive by adopting continuous learning, breakthrough challenges and changes, for their own growth and for a balanced IFAPP future-proof continuum.

Furthermore, we will address a 'stay up to date and raising of standards' approach with online educational activities and career development offerings by promoting networking with peers, working groups participation, young professionals' community projects, certification, awards, and fellowships.

As anticipated, the regular House of Delegates meeting will be part of this meeting with the out of Europe global regions' NMAs connecting virtually and adding the Asian, African, Australian and America's perspective to the dialogue.

IFAPP leadership aims to advance science, ethics and research through strategic contributions which support the Pharmaceutical Medicine knowledge that our Working Group Leaders will illustrate to receive valuable feedback from their NMA colleagues.

The IFAPP Board is excited to welcome our colleagues and stakeholders and enable direct interaction and engagement with all meeting participants for fostering Pharmaceutical Medicine excellence and professional diversity.

Agenda, venue, and more information will be released shortly by the IFAPP Secretary, Dr Anna Jurczynska, and the IFAPP Secretariat.

Our next Regional Meeting is planned for the Asian region, as online session and will be part of the, JAPhMed Japanese NMA Conference on the 29th of July, 2023.

Looking forward to extending and sharing with all regions NMA leading practices, innovative projects, next-generation capabilities, ethical, scientific, and technical standards in R&D, including artificial intelligence technologies challenges, so that we sense the momentum of future directions and areas of focus.

Dr Varvara (Barbara) Baroutsou
MD, PhD, GFMD, EMAUD
Consultant in Internal Medicine
Pharmaceutical Medicine Consultant Research & Experimental Development in Medical Sciences Expert
ELEFI President
IFAPP President

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Nikos Tsokanas – New IFAPP Treasurer

Hello! I am Nikos Tsokanas, and I am the new Treasurer on the Board of Directors for the International Federation of Associations of Pharmaceutical Physicians (IFAPP). I come from lovely Greece, and I am really happy to be part of this important organisation!

I hold a BSc (Hons) in Biomedical Sciences from the University of Brighton, United Kingdom, an MSc in Bioinformatics from the University of Exeter, United Kingdom, and a Master of Business Administration from the Hellenic Open University, Greece. In addition, I am a Qualified Lead Auditor and Lean Six Sigma Black Belt Certified.

Throughout the years, I have worked in different areas within the Pharmaceutical Industry. I started in a preclinical environment as a Scientist/Proteomics Analyst at GlaxoSmithKline, doing a lot of bioinformatics work and data mining, followed by a Clinical Development role at Roche Ltd, working as a Senior Clinical Researcher/Programmer, all in the UK. Returning to Greece I have worked in various positions mainly in the post-market clinical follow-up area of the pharma industry, I set up the Pharmacovigilance and Quality Departments in both a national (ALAPIS) and a multinational (Teva, local affiliate) company, helping both of them to fully comply with relevant regulations. Since 2015 I have been working for Bayer Hellas. I started in a Governance role heading Quality Systems for the Medical Department. The position evolved into an operations role with me heading the Medical Operations Excellence Team for the Medical Department initially for Greece and then for the full cluster (6 countries). I currently hold the position of a Medical Digital Lead for the Europe, Middle East and Africa region to help countries elevate their digital Health Care Professional (HCP) engagement capabilities from the medical perspective.

I contributed as *Secretary of Digital Information Governance & Information Technology Working Group (2016 - 2021)* in the **Research Quality Association** where I have been a member since 2014. I am also a member of the *Young Professionals Working Group* of the **International Federation of Associations of Pharmaceutical Physicians (IFAPP)** since 2021 and act as a Member of the Board of Directors of the **Hellenic Association of Pharmaceutical Medicine (EL.E.F.I.)** since 2020.

Looking forward to working with you all!

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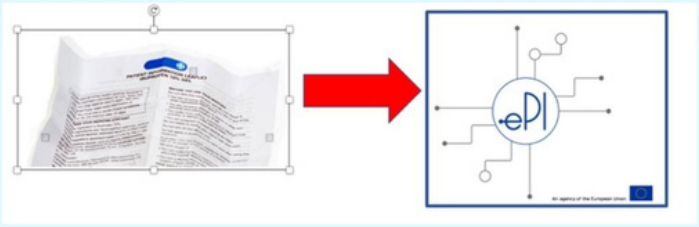
New offering of IFAPP's Continuous Professional Development Programme

The ECWG announces the next webinar:

From past to future



Key Principles for Electronic Product Information in the EU

20th April 2023 | 2-3 pm CET

Topic: The medicinal product information is regulated, scientifically validated information that assists healthcare professionals in prescribing and dispensing and informs patients and consumers about their medicine and its safe use.

The webinar will give information and updates and possibly more for the development of the electronic medicinal product information (ePI).

Register in advance for this webinar:

https://us02web.zoom.us/webinar/register/WN_0lysX9EaTk2AtDe8JjfYBg

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

Prof. Tatyana Benisheva, (Bulgaria)
MD, PhD, MDRA, MPH, DSci

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Dr Hasan Mahmood, BAPP President and IFAPP Communication WG Member

Dr. Hasan Mahmood is the founder President of the Bangladesh Association of Pharmaceutical Physicians (BAPP) with over 19 years of experience in the Pharmaceutical Industry, currently in a role in the training and development department of a pharmaceutical company.

In his earlier responsibility, he used to work in international marketing and explored several countries namely Libya, Nigeria, Philippines, Burkina Faso, Mali, Niger, Afghanistan, and was involved in the liaison with Myanmar, Cambodia, Sri Lanka and Vietnam distributors.

In addition to that, he is voluntarily involved in the International Federation of Association of Pharmaceutical Professionals (IFAPP) where his main responsibility is to maintain communication and keep their Facebook page current. He was also the creator of the IFAPP Facebook page.

With the mixed background of Learning and Development and International Business, he has a passion for the education sector. He is currently working to create awareness of pharmaceutical medicine education in Bangladesh.

Bangladesh: An Emerging Power in Pharmaceuticals

Pharmaceutical Industry Overview of Bangladesh

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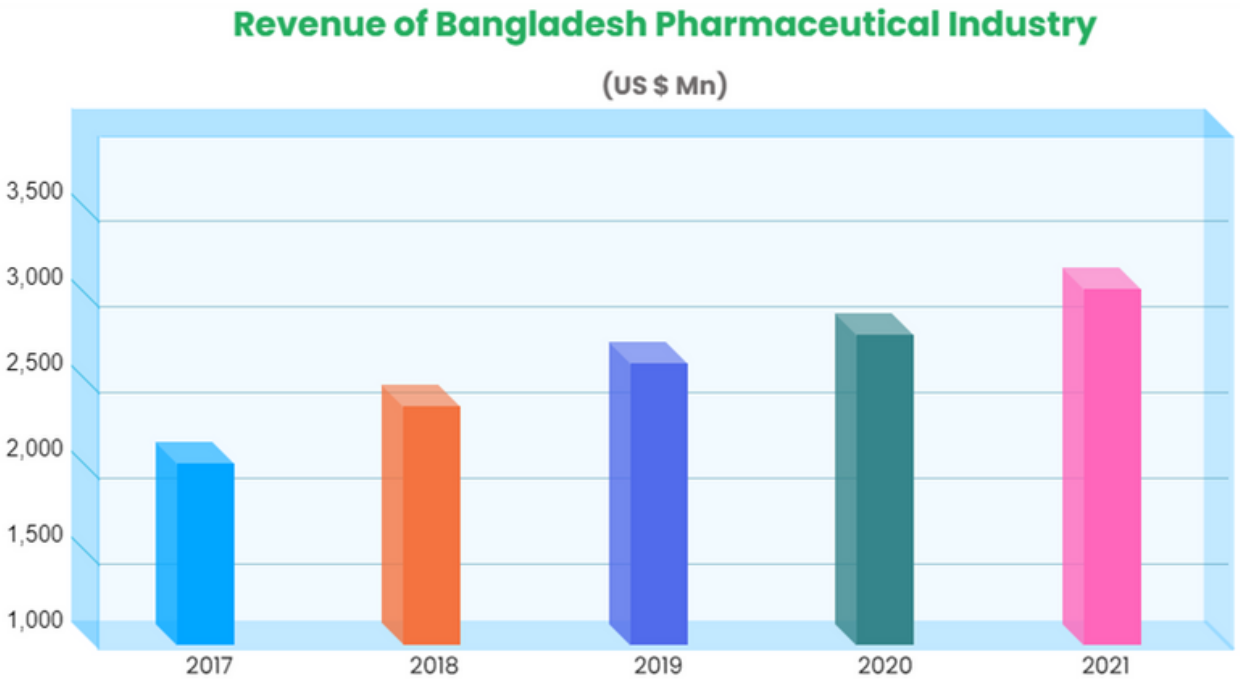
The Pharmaceutical Industry is one of the most developed technology sectors in Bangladesh. Manufacturers produce almost all types of medicines which includes high-tech products like insulin, hormones, anti-cancer products, etc. This sector provides 98 % of the total medicinal requirements of the local market. The industry also exports medicines to 150+ countries, including USA, UK, Canada, Australia, Germany, EU, etc. Pharmaceutical companies are expanding their business with the aim to expand the export market.

The Pharmaceutical sector of Bangladesh has been transforming and evolving since the early 1980s. The sector has grown from strength to strength over the last 5 decades. Since this is a technology- and knowledge-based sector, the journey was not an easy one for a Least Developed Country (LDC) faced with enormous economic challenges. Now, Bangladesh proudly stands alone as the only LDC that has a well-developed pharma sector.



*considering current currency exchange rate (1 US $= 85 BDT)

Source: MAT Q3, 2021

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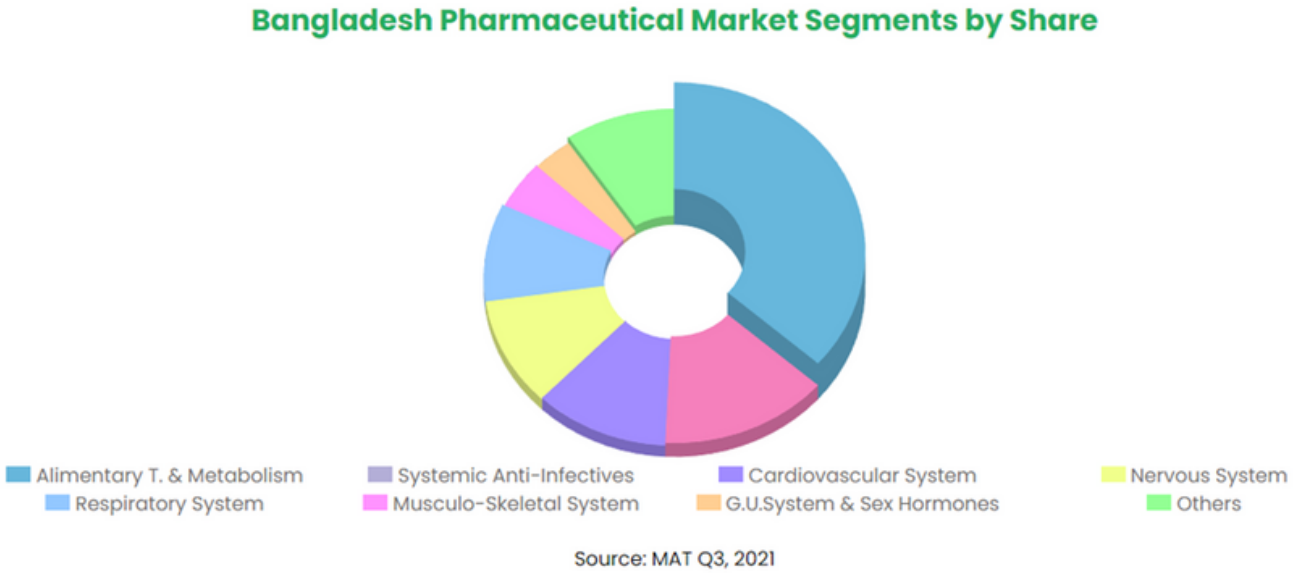

- Emerging generic drug hub in Asia
- Nearly self-sufficient; 98 % of demand met by local production.
- Total market size approx. $3.2 bn in 2021 with a growth rate of 10.72%
- Historically good market growth maintained (CAGR 8.93 % in last 4 years)
- Strong manufacturing base with skilled manpower
- Largest white-collar labour-intensive employment sector in Bangladesh
- 2nd highest contributor to national exchequer
- Registered allopathic pharmaceutical companies: 284, and functional around 213.
- All the top 10 companies are local, and they have approx. 70 % market share.
- Leading companies have nearly all major GMP accreditation like USFDA, UK MHRA, EU GMP, Health Canada, TGA Australia, ANVISA Brazil, GCC, etc.

Bangladesh Pharma has high capability in Specialised Pharmaceutical Products

The industry continues its strong research orientation in generic formulation development and has already proven its skills with successful development of specialised, high-tech formulations which are very difficult to imitate. Leading companies have focused on specialised dosage delivery systems to create strong differentiation and successfully developed metered-dose inhaler (MDI), dry powder inhaler (DPI), lyophilised injectables, sterile ophthalmics, prefilled syringes, oral thin films, multi-layer tablets, biological products, including insulin, vaccines etc.

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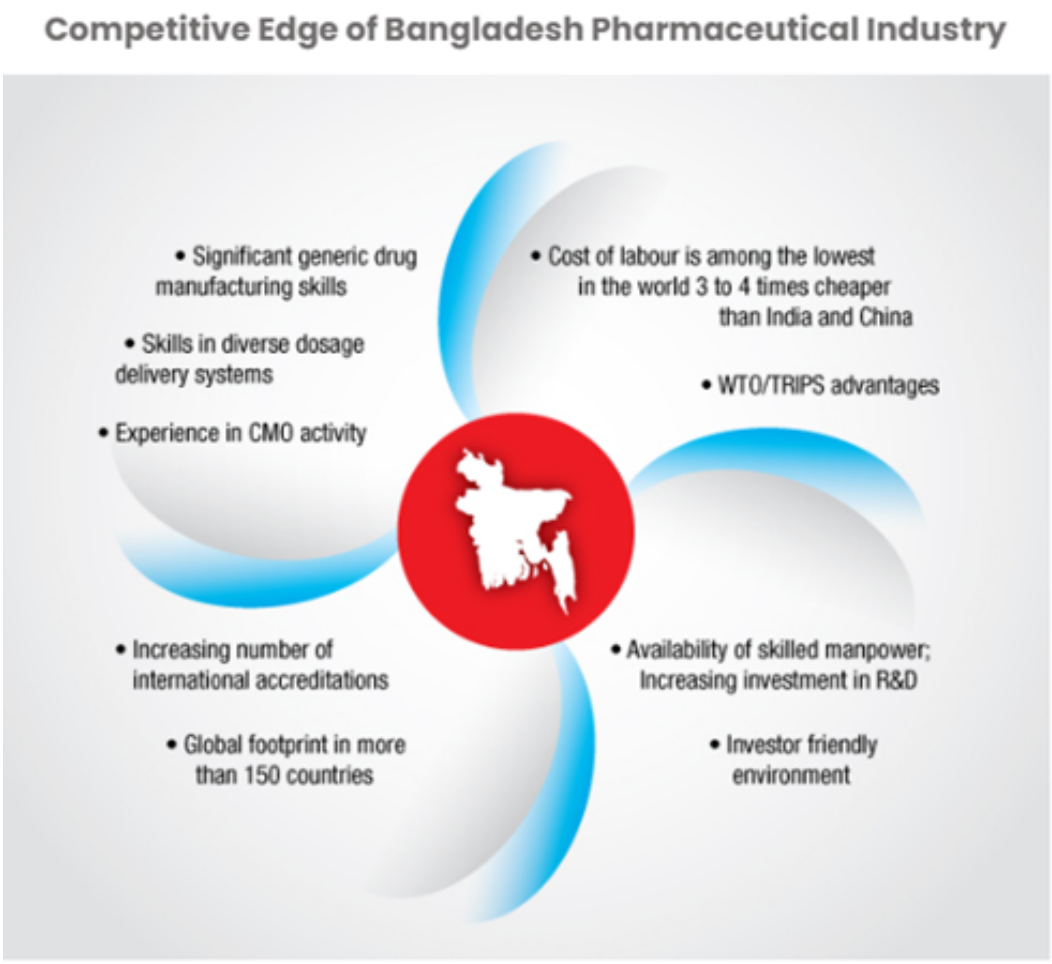
Prospect and Opportunities

Opportunities in the global generic market - Bangladesh is an emerging generic hub in Asia

The global generics market is valued at around US $1.3 trillion (tn) and is projected to grow $ 1.52 tn by 2023 (Ref.: IQVIA Market Prognosis, Sep 2018)

- Generic drugs will continue to grow maintaining healthy rates.
- Drugs worth more than US $ 200 billion coming off patent by 2025.
- Rising healthcare cost, particularly cost of medicines has become a global concern.
- Developed countries are increasingly promoting use of generic drugs.
- Multinational companies are increasingly outsourcing their production for cost containment.
- Bangladesh offers tremendous manufacturing cost advantages.
- Major generic hubs like India and China are losing cost advantages.
- Medicine price in Bangladesh is currently among the lowest in the world.
- Extended patent waiver from TRIPS.

Yes, you can get more from us if you are looking for CMOs.



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Pharmaceutical companies in Bangladesh are ideally positioned to provide high-quality contract manufacturing services to companies from developed countries. Bangladesh pharmaceutical companies have

- Expanded geographical footprint
- Many facilities are approved by global regulatory authorities.
- Cost advantages due to competitive white-collar labour and energy
- Proven track record with multinational companies (MNC)
- Expertise in diverse delivery systems, specialised high-tech products
- Investment-friendly environment

We are expanding our Global Footprint

- Export is growing fast, the current export earning is more than $ 188 mn.
- The Bangladesh Government has declared Pharmaceuticals as the “Thrust Sector”.
- Approximately 1,200 pharmaceutical products received registration for export over the last two years and are being exported to more than 150 countries including USA, UK, Canada, Australia, Germany, EU, etc.
- Leading companies have an increasing focus on highly regulated markets, including USA, UK, Canada, Australia, Germany, EU, etc.
- Sector has attracted huge attention from clients abroad.
- Leading companies have nearly all major GMP accreditation like USFDA, UK MHRA, EU GMP, Health Canada, TGA Australia, ANVISA Brazil, GCC etc.
- Few companies are now exporting medicine to the highly regulated countries, including USA, UK, Canada, Australia, Germany, Europe, etc.

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In upcoming years, the government of Bangladesh will play a major role in the rapid growth of the pharmaceutical market by providing favourable policies for easy drug approval, production and marketing of new products. The Government is focusing to reduce the dependence of Bangladesh over the import of raw material and establishment of API Park will act as a turning point for this purpose. The top 50 companies are setting their facilities at the active pharmaceuticals ingredient industrial park that will help in the production of patented and already opened active pharmaceuticals ingredients. It is expected that the development of the API Park will be completed by the next 2 years, which will reduce the expenditure related to the import of raw material.

All the associated factors are in favour of the rapid growth Bangladesh pharmaceutical market, and it is expected to grow with a CAGR of more than 12 % during the 2019-2025 period. The share of generic drugs is expected to surpass 85 % by 2025, which will further strengthen the dominance of local pharmaceutical companies in the market. In addition to this, the presence of leading multinational pharmaceutical companies is also increasing in Bangladesh, which is indicating towards the competitive landscape in upcoming years.

Bangladesh Association of Pharmaceutical Physicians (BAPP)

BAPP was founded in 2007 and got IFAPP membership status in 2011. Dr. Hasan Mahmood is the founder president. Currently, BAPP has about 100 members. Most of the members are working in the medico-marketing sector. BAPP is currently aiming to attract pharmaceutical medicine education in Bangladesh.

Reference:
www. bapi-bd.com

"Boosting Austria's Position as a Research Destination"

The Society for Pharmaceutical Medicine (GPMed) aims to strengthen Austria's position as a location for international clinical trials. GPMed President, Priv.-Doz. Dr. Johannes Pleiner-Duxneuner, discusses the measures being taken in a recent interview.

PHARMAustria: What are GPMed's current goals?
Priv.-Doz. Dr. Johannes Pleiner-Duxneuner: GPMed is a society that is primarily focused on clinical research. Thus far, we have been heavily involved in the operational aspects of research, particularly through our Clinical Operations Circle. However, the field of research is evolving fast, and we want to adapt to these changes by expanding into new areas. Two years ago, we established the Medical Affairs Circle within GPMed. This circle serves as a liaison between research departments in pharmaceutical companies and external research institutions.

Priv.-Doz. Dr. Johannes Pleiner-Duxneuner, President of GPMed; © Harald Eisenberger

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Additionally, we aim to stay on top of emerging trends and are currently exploring the growing role of patients in clinical research, research related to medical devices such as digital applications like DIGAS (digital health applications), as well as research on health data, including not just traditional study data but also hospital and social security data. Our most recently established Research Innovation Circle within GPMed is dedicated to this area of research.

In general, GPMed is committed to contributing to knowledge transfer in order to strengthen clinical research in Austria. As such, training and education are important topics for us.

GPMed defines itself as a scientific forum for physicians, scientists, and healthcare professionals from academia, industry, regulatory bodies, and other institutions. Why is the dialogue between these different players so important?

From GPMed's perspective, we believe that such networking is absolutely necessary for clinical research, which is a team sport, so to speak, that requires collaboration across many different stakeholders to deliver the best possible outcomes. Rather than individual companies, interested individuals from pharmaceutical companies and the academic community are members of GPMed. This diverse composition is crucial, as it allows for discussions between companies, regulatory bodies, universities, and institutions, which is essential for successful research. Despite differing perspectives, these groups can effectively collaborate through active communication. GPMed serves as a facilitator in this regard.

GPMed focuses on the field of Medical Affairs. What are the current topics and objectives in this area?

GPMed is a professional society consisting of three circles: Clinical Operations and Research Innovation, which were already mentioned, and the Medical Affairs Circle. In recent years, this area has become increasingly important, not only in pharmaceutical companies, where this circle acts as an interface to external research institutions and healthcare professionals but also in-house in close collaboration with sales and marketing departments. We believe that this field has been underrepresented in Austria, which is why we have launched a special training initiative in the Medical Affairs sector this year to strengthen this area. For example, we have partnered with the Medical University to offer a short course in this field.

How has medical research changed in recent years, and how will this area continue to change in the future? What role do digitalisation, artificial intelligence, and real-world data play in this?

Digitalisation has, of course, drastically changed clinical research. One important trend we have observed in recent years is the use of real-world data. Since the number of cases in clinical trials is constantly decreasing, traditional research is reaching its limitations and must rely on real-world data. This is because only 2-5% of patients are involved in clinical trials, while the remaining 95-98% are treated outside of trials. However, the evidence is based only on the 2-5% of patients who participated in the study. Therefore, it is logical to include real-world data in research.

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For this reason, in 2021 we formed a multi-stakeholder group to create a consensus on quality criteria for real-world data. The group consisted of people from ministries, authorities, the Austrian Health Agency, academic institutions, and the pharmaceutical industry, and their consensus was published in 2022 (Note: published in JMIR Med Inform 2022; 10(6):e34204. We are currently working on determining which data can be used for what purpose and how our quality criteria can be applied.

Digitalisation also plays an increasingly important role in traditional clinical research. For example, patient diaries are now digitalised. This is a topic that our Clinical Operations Circle is concerned with. Legal and regulatory aspects are also important in this area, and we are addressing these as well. Research on digital health apps has also increased significantly, and as mentioned earlier, this is an area that GPMed is also addressing. Artificial intelligence (AI) is also essential in all of these areas, as we cannot process the vast amount of data, we have today without it.

What is the state of Austria as a research location from the GPMed perspective? What measures are needed to strengthen Austria as a research location?

When discussing Austria as a research location, one must consider the situation in Europe as a research location. The trend is that pharmaceutical companies are moving their research away from Europe. Austria is not an exception to this trend and, as a small country, is affected by the overall development in Europe. There are many reasons for this, one of which is the very heterogeneous environment in Europe. Although we are trying to counteract this trend with appropriate regulations, for example, medical device research and, therefore, all research with apps, digital applications, and other software solutions, etc., are not uniformly regulated in Europe. In addition, Real-World Data research is not or is very differently regulated in Europe. There is also the hindrance caused by an over-interpretation of data protection in Europe, which also applies to Austria.

All of these are reasons why we have fewer clinical research projects in Austria and Europe, and urgent action is needed to address this. Therefore, it is of great importance to us at GPMed to raise our voices for the research location Austria. Additionally, we need to work on how research is perceived by the general public in Austria. We aim to create pride in research "made in Austria". Another significant step to strengthen Austria as a research location is to promote collaboration between academia and industry, making our country more attractive as a research location for the industry. Furthermore, we would like to see Austria - coordinated by BMSGPK (Austrian Federal Ministry of Social Affairs, Health, Care and Consumer Protection) and BMBWF (Federal Ministry of Education, Science, and Research) with the involvement of academia and industry - take a leading role in the European Health Data Space, which involves the exchange of European health data, instead of waiting to see what other countries are doing in this regard. This would also strengthen Austria as a research country.

Thank you for the conversation!

Original source: German.
PHARMAustria 1/2023, MedMedia Verlag,
https://www.medmedia.at/pharm-austria/oesterreich-als-forschungsstandort-staerken/

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MDR Extension Deadline: It's Set in Stone

L 80/24 EN Official Journal of the European Union 20.3.2023

REGULATION (EU) 2023/607 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 15 March 2023

amending Regulations (EU) 2017/745 and (EU) 2017/746 as regards the transitional provisions for certain medical devices and *in vitro* diagnostic medical devices

The European Parliament has just published the extension of the transition period in the Official Journal of the European Union for a full application of the Medical Device Regulation (MDR).

The European Parliament adopted a proposal by the European Commission to delay the transition period due to concerns about device supply, notified body capacity, and manufacturer preparedness.

Although notified bodies (NBs) have been ramping up capacity and the number of MDR-designated NBs keeps on growing, the capability to process applications is not endless. The amendment is actually not intended to give the industry more time, but to only give NBs a longer period to process the huge pile of applications that had amassed towards the end of the grace period. As such, any manufacturer that misunderstands this fact is making a critical mistake and will miss its launch window for extension, inevitably leading to a period of not being able to place devices on the market in the Union until the MDR CE mark has been obtained.

In fact, the application of the extended transition period is subject to several cumulative conditions to ensure that only safe devices for which manufacturers have already taken steps to transition to the MDR will benefit from the additional time.

As such, the transition period deadline is extended from 26 May 2024 to 31 December 2027 or 31 December 2028, depending on the risk class of the device. High-risk devices are subject to the shorter transition period ending in 2027; while low- and medium-risk devices, will have until the end of 2028 to complete a conformity assessment.

For class III custom-made implantable devices, the Commission created a new transition period until 26 May 2026, to give manufacturers more time to get their quality management systems (QMS) certified by a notified body. For such manufacturers, the new transition period will only apply if the company has registered with a notified body by 26 May 2024, and has signed a contract for the certification by 26 September 2024.

Regarding the deletion of the sell-off date provision, the Commission said the measure will reduce the risk of shortages by allowing devices and IVDs that were already on the market based on compliance with the former directives to remain on the market. The Commission noted that the provision would not allow devices with a limited shelf-life to be offered past expiration.

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Passage of the amendment may also have some unintended consequences since some companies have slowed their pace to comply with the regulation already expecting an extension. The best thing to do is to continue to push for completion and provide the notified body with timely dossiers for review.

First and foremost, it is important that manufacturers ensure that they are generating sufficient clinical data and try getting the product submission right the first time, so not to delay the process.

Since the amendment is a very technical document, the European Commission is already working on a Q&A article that they plan to present to stakeholders during March to help resolve any questions regarding interpretations.

As such, let's all work together - Member States, notified bodies and industry, to ensure a smooth transition to the new rules without further delay.

Link (English version)

Catarina Carrão, Science Writer at
Gouya Insights GmbH & Co. KG

Clinical Trials 2023: Results to Look Forward

Recently, Nature Medicine reporters C. Arnold and P. Webster asked leading researchers to name the most interesting clinical trials coming to an end in 2023.

Roger Albin, Professor of Neurology and co-director of the Movement Disorders Clinic at the University of Michigan Medical School, highlighted the Exenatide phase 3 clinical trial funded by the Michael J. Fox Foundation currently running at the University College of London. Exenatide is a medication used to treat Diabetes mellitus and is being repurposed as a potential disease-modifying treatment for Parkinson's disease. A previous double-blind placebo-controlled trial with 60 participants indicated that exenatide may be a "neuroprotective" drug. Triggering insulin-signaling pathways for neurodegenerative conditions has created a lot of buzz during the last couple of years, as such according to Prof. Albin "the community is looking for unequivocal results, whether positive or negative" with this study.

Next, Dr. Rober L. Coleman, Chief Scientific Officer at the U.S. Oncology Research, one of the largest networks dedicated to advancing high-quality evidence-based cancer care, emphasised the phase 3 MIRASOL trial in platinum-resistant ovarian cancer.

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This study from ImmunoGen Inc., a biotechnology pioneer in Antibody–Drug Conjugate (ADC) therapeutics is designed to compare the efficacy and safety of mirvetuximab soravtansine vs. investigator's choice chemotherapy. This new ADC was given accelerated approval by the FDA, as such it needs a confirmatory trial to validate the overall safety and efficacy of the novel compound.

Prof. Dr. Simone Spuler, head of the myology research group at the Experimental and Clinical Research Centre in Berlin, Germany, underlined the importance of the GenPHSats-bASKet phase 1/2 study.

This clinical trial will be fundamental to validate the safety of CRISP-Cas9 technology for the correction of genetic disorders. In this case, gene edited Primary Human Satellite cell derived muscle stem cells are being used to promote healthy muscle development in patients with Limb-Girdle Muscular Dystrophy (LGDM) – a group of rare progressive genetic disorders characterised by atrophy and weakness of the voluntary muscles of the hip and shoulders. Not only the safety of the technology is being evaluated, but also measurable clinical improvement. The regulatory agencies have an eye on the trial to make sure that a benefit is brought to the patient's life, considering the high risk of using such novel biotechnology for the treatment of LGDM.

Important is also the study highlighted by Dr. Olaf Valverde Mordt, the clinical project leader in Human African Trypanosomiasis (HAT, sleeping sickness) at the Drugs and Neglected Diseases initiative (DNDi) – a not-for profit organisation founded in 2003 for the discovery, development, and delivery of new treatments for neglected patients around the world. Currently, the only treatment available for stage II HAT is melarsoprol, an arsenic derivative that kills 5% of the patients - a very toxic drug. As such, the trial is evaluating whether fexinidazole is as effective in treating this devastating neuropsychiatric disorder, which is caused by trypanosome parasites transmitted by Tsetse flies' stings. This disease affects millions of people in sub-Saharan African countries, and all-oral safe medicines are much needed.

If you want to help, DNDi donations are accepted and tax-deductible: https://dndi.org/donate/

Original article: Arnold, C., Webster, P. 11 clinical trials that will shape medicine in 2023. Nat Med 28, 2444–2448 (2022).

Catarina Carrão, Science Writer at
Gouya Insights GmbH & Co. KG

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Webinar on the New EU Clinical Trials Regulation – Follow-up

The Webinar held on 1 and 2 March 2023 was a GREAT SUCCESS! It delivered first-hand information on all the aspects of clinical trials with medicinal products throughout the European Union to over 200 registered participants.

Professionals from the following countries attended the Webinar:

Austria, Belgium, Bulgaria, Czech Republic, France, Germany, Hungary, Ireland, Italy, Japan, Lithuania, The Netherlands, North Macedonia, Poland, Portugal, Romania, Sweden, Switzerland, Russia, Tanzania, United Arab Emirates, United Kingdom, and USA.

Some of the comments received from the attendees were:

“It was a very helpful Webinar. I would like to thank you and the organisation, and wish you a nice weekend.” (Austria)

„The training was great, we all benefited a lot from it.” (Hungary)

“Great training!!! Well structured, informative, and very useful for our daily work. I really learned a lot.” (Germany)

“It was perfectly organised and the content was very comprehensive.” (Bulgaria)

“Thank you very much for the amazing 2-day training, full of beneficial and essential information. Great experts and speakers shared with all of us a lot of important details, which have to be effectively digested and successfully implemented in practise. These slides and presentations will be a great support in our everyday challenges to CTR and CTIS adaptation. I am following the IFAPP website, and enjoyed reading the latest Newsletter from March, which included info from our EU CTR training days.” (Serbia)

“Thank you for this amazing training on the new CTR.” (Germany)

“Thanks so much for the interesting webinar.” (The Netherlands)

All the participants received a full set of slides presented during the Webinar and a personalised Certificate of Attendance (see next page).

Anna Jurczynska, PhD, MBA
IFAPP Board of Officer Secretary and AMIFE Delegate

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CERTIFICATE

Introduction to the New EU Clinical Trials Regulation

Virtual training on 1 & 2 March 2023

.....attended the above training course

Provider: International Federation of Pharmaceutical Physicians and Pharmaceutical Medicine (IFAPP)
Duration: 10 hours

Modules:

- Clinical trial aspects that will change under the new EU Clinical Trials Regulation
- The new "Single Dossier" in clinical development
- The Clinical Trials Information System "CTIS"-structure, functioning, requirements, training options
- The Coordinated Clinical Trial Authorisation procedure in theory and practice
- Safety data reporting and serious breaches handling under the Clinical Trials Regulation
- Advantages and hurdles of the new Regulation for sponsors of trials in different phases from within the EU and abroad
- CTIS Access and User Management
- (Re?-)Organisation of responsibilities and oversight for sponsor and vendors required to
- achieve clinical trial authorisation in an auditable quality environment
- Reporting obligations during and after the clinical trial: study management, pharmacovigilance, technical summary and lay summary of trial results
- IMP Management under the Clinical Trials Regulation from definitions to labelling
- Setting the standard for greater diversity in clinical trials
- Challenges in the transition period between former Clinical Trials Directive and the new Clinical Trials Regulation
- How should I prepare for the Clinical Trials Regulation in my work environment

Learning Outcomes: You will understand the new processes, procedures and obligations of parties involved for updating your regulatory knowledge and for the preparation and conduct of your clinical trials in the EU.

Moderators: Ingrid Klingmann, MD, PhD, FFPM, FBCPM, Dr Birka Lehmann MD PhD

Varvara (Barbara) Baroutsou
IFAPP President

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Clinical Research in Rare Diseases Could Accelerate the Authorisation of New Drugs by Public and Private Payers

From left to right: Cristiano Zerbini, MD, PhD (Clinical Research – São Paulo State University), Helio Osmo MD, MBA (President of Brazilian Society of Pharmaceutical Medicine), Elie Fiss (Clinical Research – ABC School of Medicine – Oswaldo Cruz German Hospital), Cristhiane Gennaro (CRO – IQVIA), Luciana Ferrara (CRO – AZIDUS)

Hélio Osmo, MD, MBA, President Brazilian Society of Pharmaceutical Medicine and Global Fellow in Medicines Development of IFAPP - International Federation of Associations of Pharmaceutical Physicians & Pharmaceutical Medicine.

On 31 March 2023 a roundtable discussion on Value Based Payment Models (VBPM) was held in São Paulo and discussed the potential of clinical research to generate data that meets the criteria considered valid for medicines to be accepted by public or private payers.

According to the Brazilian legislation, rare diseases are diseases that affect less than 65 individuals per 100,000 births, and in total there are about 8,000 of these diseases, and only 4 % of them are treatable. This is a cruel reality, which raises ethical, economic, and also social issues, since in Brazil alone there are about 13 million patients with some rare disease, which often drastically affects the quality of life of these people and their families. [1]

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Most rare diseases are genetic and remain throughout the patient's life, even if symptoms do not appear initially. Many of them appear early in life and their symptoms are very disabling, preventing the patient from leading an active and healthy life. About 30 % of children with these diseases die before their fifth birthday. [1]

To make matters worse, most of the time the diagnosis of a rare disease comes only after years of a pilgrimage from doctor to doctor, with many misdiagnoses and unnecessary treatments, which further impact the patient's health.

And even after the correct diagnosis, the vast majority of rare diseases have no adequate treatment. This happens for several reasons, but the main one is the rarity of the disease itself, which causes a series of difficulties in the process of research, development, and approval of new specific drugs.

Pharmaceutical industries and regulatory agencies - such as ANVISA, in Brazil's case - are more used to working with drugs for common diseases, with many patients, and have defined their processes and regulations according to the reality of these diseases. Thus, research and regulatory approvals follow paradigms in which thousands of patients are evaluated, which is not the case for rare diseases. This is the first challenge for a new drug to be approved. If this is not enough, most of the time payers do not consider the approved endpoints for the registration of a new drug and would like to consider other endpoints, with perspectives not evaluated in the studies. For this, other endpoints need to be evaluated.

What about rare disease patients, since their number is relatively small? There are fewer Pharma companies focusing on rare diseases and producing new drugs for these diseases. Research and drug development for these rare diseases are big challenges. Besides the small number of patients there is the need to have a clearer definition of the clinical outcome, in a timeframe where diseases have no cure, which is not feasible for neurodegenerative diseases, for example. Faced with this reality, the rare disease patient, who cannot wait to receive the most appropriate treatment available, is severely harmed and often does not even survive to wait for the approval of a new drug that could change the course of the disease.

One real advance that generates a positive expectation is Artificial Intelligence (AI) applied in favour of generating real-life data that can be applied in research. This data would come from early diagnosis, more concrete data on the natural history and evolution of the disease, definition of outcomes based on biomarkers and sharing of global data based on patient records. In addition, AI would help define new statistical models to define evidence.

In this regard there is a need to advance global patient data records, either through electronic medical records or through clinical research registries. Such real-world data would be extremely useful for designing protocols and outcomes that are plausible not only to drug approval agencies but also to payers. The registries will also be useful for validating the natural history of the disease and can be used as comparator controls. With this, fewer patients will be needed in the trials, accelerating their completion. AI will also be important for the evolution of statistical analyses. Many number crunching manoeuvres are currently used, which rely on methodologies that are not necessarily useful in rare diseases. Inevitably biases arise that hinder conclusions and create more doubt for payers.

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There are advances, but not at the necessary speed. The Brazilian Ministry of Health created the Brazilian Network for Clinical Research last year, but without the specific expertise in rare diseases. Therefore, it is necessary to call those responsible to talk, clarify the situation through meetings with the FDA and the EMA (European regulatory agency), so that decision-makers feel more secure.

Lately, companies had stopped bringing clinical research to Brazil due to the difficulties imposed by legislation, such as approval time, requirement to provide medication after the end of the study, among others. However, with COVID-19, due to the high number of infected people in Brazil, clinical research started to return to our country. This was also a reflection of a change in the legislation for ultra-rare diseases, which since 2017 started to require the supply of new drugs for only 5 years after the study, no longer indefinitely.

In light of all the above, one of the biggest challenges in our country today in relation to rare diseases is to create a standard of clinical research that is sanctioned by regulatory agencies, specific for rare diseases, so that the efficacy and safety of a new drug can be proven in a more appropriate way, and so that it can be demonstrated to the system that the product really delivers what it promises. These new scientific paradigms for rare diseases would allow agencies, such as ANVISA and CONITEC, to approve new drugs more quickly and with a commitment to data delivery during product development.

For this change to happen, there must be a union between patient organisations, rare disease companies and professionals specialised in clinical research, in the fight for specific legislation for the development of treatments for rare diseases that would allow, in the short term, the registration of a medicine that could save many lives. This is an evolution that needs to happen, so that it is not increasingly difficult to get new drugs for these diseases approved.

We are all trying to contribute to science and health. We need to join efforts and overcome these challenges so that the life of the rare disease patient is finally placed as a priority.

Reference [1]:
https://bvsms.saude.gov.br/bvs/publicacoes/doencas_raras_A_Z.pdf

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