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Public Policy Review

Periodic review of the European public policy landscape

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AISBL European Haemophilia Consortium

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The current edition covers legislative initiatives of the first half of 2023.
It is designed to provide the readers with a retrospective overview.
All initiatives will be further monitored by the EHC.

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1. European Health Union

1.1. EU Global Health Strategy

The World Health Organization (WHO) and the European Commission have formed a digital health partnership to establish a global system based on the European Union's digital COVID-19 certification. This partnership will help facilitate global mobility and protect people worldwide from ongoing and future health threats, including pandemics. Additional use cases may include digitising the International Certificate of Vaccination or Prophylaxis.

The WHO Global Digital Health Certification Network (GDHCN) will be developed to provide open-source digital health tools based on equity, innovation, transparency, and data protection principles. The initiative aligns with the EU Global Health Strategy and the WHO Global Strategy on digital health and follows a previous agreement between the EU Commissioner and the WHO Director-General for strategic cooperation on global health issues.

The WHO's involvement aims to strengthen global health preparedness and facilitate the convergence of digital certificates worldwide without accessing any underlying personal data, which will remain the responsibility of governments.

Read more here.

1.2. European Parliament creates SANT Subcommittee

On 14 February 2023, the European Parliament approved the creation of the public health subcommittee, comprised of 30 full members and 30 substitute members. The subcommittee will deal with pharmaceutical and cosmetic products, the health aspects of bioterrorism, the European Medicines Agency (EMA), and the European Centre for Disease Prevention and Control.

MEP Bartosz Arłukowicz (European People's Party, Poland) was elected Chair during its constitutional meeting. Four Vice-Chairs were elected as well:

- First Vice-Chair: Tudor Ciuhodaru (Group of the Progressive Alliance of Socialists and Democrats, Romania)
- Second Vice-Chair: Irena Joveva (Renew Europe, Slovenia)
- Third Vice-Chair: Tilly Metz (Greens/European Free Alliance, Luxembourg)
- Fourth Vice-Chair: Joanna Kopcińska (European Conservatives and Reformists Group, Poland)

The first ordinary meeting of SANT took place on 20 April 2023, in Strasbourg.

Read more here.

2. EU4Health

EU4Health is the fourth largest of the EU health programmes with a budget of €5.3 billion. The programme provides funding to national authorities, health organisations, and other bodies through grants and public procurement to address the resilience of European healthcare systems, contributing to a healthier Europe.

All 'Current Prior Information Notices' and open tenders can be found on the European Health and Digital Executive Agency (HaDEA) website here.

The European Commission recently unveiled a comprehensive approach to mental health, allocating €1.23 billion in EU funding to 20 flagship initiatives. One of these flagship initiatives, number 15, seeks to provide training on mental health to health professionals in the EU. HaDEA has initiated a call for tenders with a budget of €9 million to implement the 'Capacity-building on mental health: multidisciplinary training programme and exchange programme for health professionals', aiming to train 2000 professionals across the EU by 2026.

Read more here.

3. Regulation on standards of quality and safety for substances of human origin (SoHO)

As readers may recall, in July 2022, the European Commission adopted a Proposal for a Regulation on standards of quality and safety for substances of human origin (SoHO) intended for human application. The proposal marked the first comprehensive blood legislation review in 20 years. Since then, the SoHO file has become the most disputable in the EU institutions and has seen several delays.

In July 2023, the European Parliament's Committee on Environment, Public Health and Food Safety (ENVI) adopted the report on SoHO, resulting in a compromise deal for donors, patients, and the EU health system.

The Commission's original proposal, presented last July, set a framework to provide donors and patients with a future-proof and harmonised system for transplants and donations, maintaining some limits on the supply side of these therapies. With 874 amendments presented at the beginning, the committee widely adopted most of the final amendments.

Some of the changes that were introduced include:

- the need to ensure the autonomy of the EU's supply of these substances;
- the need to create an EU list of critical SoHOs to monitor availability.

The most divisive problem since the start of the work on this Proposal was how to regulate voluntary unpaid donations (VUD) in the EU. Both the European Commission and the European Parliament agreed that donation of SoHOs should be founded on the principle of voluntary and unpaid donation, altruism of the donor and solidarity between donor and recipient, acknowledging VUD as a factor that contributes to high safety standards and therefore to the protection of human health. While all agree that donations should be VUD,

the discussion on compensation was the hottest – namely, how to ensure compensation does not become an incentive.

The Parliament, therefore, agreed to define 'compensation' as "making good of any quantifiable losses and reimbursement of expenses associated with a donation" and adopting the principle of 'financial neutrality of donation' – meaning that no financial gain or loss will be incurred by the donor as a result.

What it means for the community:
For some patients with rare congenital bleeding disorders, plasma-derived medicinal products (PDMPs) remain their primary source of treatment. These patients must have timely access to safe and efficacious medicines. As the proposal impacts the collection of plasma for manufacturing such medicines, it is of direct relevance to patients as end-users of those therapies.

The EHC took part in elaborating the Joint Stakeholders' Statement with other partners from the Platform of Plasma Protein Users (PLUS) consortium.

To access the Joint PLUS Stakeholders' Statement on the Commission proposal for a SoHO regulation, click here.

Next steps:
The report will be debated and voted on by the European Parliament in plenary in September 2023, making the SoHO revision one of the last health files to be discussed before the current EU legislative mandate ends in spring 2024.

Read more here and here.

4. Pharmaceutical legislation

The European Commission is proposing a comprehensive reform of the EU's pharmaceutical legislation, the largest in over 20 years, to make medicines more accessible, affordable, and environmentally sustainable while fostering innovation and competitiveness in the EU pharmaceutical industry. The reform seeks to address fundamental challenges, including the slow availability and unequal accessibility of medicines across Member States, unmet medical needs, high prices for innovative treatments, and shortages of medicines.

To achieve its objectives, the reform includes proposals for a new Directive and Regulation covering the entire lifecycle of medicines. Key elements of the proposal include better access to innovative and affordable medicines for patients, promoting innovation through an efficient regulatory framework, providing effective incentives for innovation, addressing shortages of medicines, and strengthening environmental protection.

Read more here.

In response to the European Commission's legislative proposals on the Pharmaceutical Package, in June 2023, The European Alliance for Transformative Therapies (TRANSFORM) published its Position on the Pharmaceutical Package, with the EHC among the signatories.

TRANSFORM is a multi-stakeholder Alliance in Europe that facilitates dialogue between MEPs, policy-makers, patient groups, medical experts, scientists, and industry actors to create evidence-based policy recommendations for safe and timely patient access to cell and gene therapies, ensuring healthcare system sustainability.

The TRANSFORM initiative, among others,

- urges the inclusion of targeted incentives in the Pharmaceutical Package to support the development of innovative therapies, making the EU a competitive hub for Advanced Therapy Medicinal Products (ATMPs) research and attracting investment;
- supports the inclusion of patient representatives in the regulatory process for medicinal products and calls for their involvement in decision-making to ensure faster access to therapies that improve patients' lives;
- express concerns about the unclear definition of "exceptional therapeutic advancement", which might exclude transformative therapies like ATMPs that have previously benefited from similar designations;
- calls for enhanced cross-border access, infrastructure development, and patient rights, while advocating for transparent reporting and harmonized rules for the use of products prepared under hospital exemption, emphasizing the need for consistent implementation and data collection;
- advocates for regulatory engagement with stakeholders, including patients and healthcare professionals, to establish pathways for using real-world data and emphasizes the importance of patient perspectives and ethical considerations in data collection and usage for evaluating new health technologies;
- advocates for complex discussions involving various stakeholders, including patient organizations, academics, clinicians, and industry, to collaboratively understand and refine strategies for addressing unmet needs in specific disease areas through compulsory early dialogues within existing regulatory structures.

Read the full Position here.

Concerning orphan medicines, the current Orphan Regulation (EC) No 141/2000 is being replaced, with the new provisions being incorporated into the general Regulation governing EU procedures for the authorisation and oversight of medicines. While the criteria for orphan designation remain largely unchanged, a key alteration is the inclusion of a requirement for a 'significant benefit' of a medicinal product to a substantial portion of the target population, making it more challenging to establish such a benefit.

The procedure for granting orphan designation is streamlined, shifting responsibility from the European Commission to the EMA through its Committee for Medicinal Products for Human Use (CHMP). The orphan market exclusivity period is reduced from ten to nine years, with potential extensions under strict conditions, including high unmet medical needs and timely supply across Member States. However, the revised regulations also weaken orphan market

exclusivity by allowing the submission and assessment of similar medicinal products, including generics and biosimilars, during the last two years of exclusivity.

The changes bring about both simplification and complexity. While the merger of orphan provisions into the general Regulation aims for a more cohesive approach, the new significant benefit requirement and stricter criteria for extending market exclusivity pose challenges for drug developers. The reduction in the market exclusivity period and the ability to assess similar medicines earlier signify a shift towards early competitor entry. Definitions of 'Unmet Medical Need' and 'High Unmet Medical Need' are tied to meaningful reductions in disease impact, potentially limiting the extension of protection periods. Consequently, the claimed possibility of up to 13 years of protection is uncertain due to stringent requirements and limitations compared to the previous regime.

Read the Global Compliance News analysis here.

Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe, responded to the European Commission's proposed legislative reforms positively, stating that many rare disease patients and their families would welcome the measures while also calling for greater clarity in certain aspects of the legislation, such as defining "meaningful reduction in disease morbidity or mortality" and identifying "significantly debilitating" medical conditions. Furthermore, he advocated for establishing a structured regulatory pathway for extremely rare diseases and conditions without therapeutic options and a need for clearer regulations on patient representatives' involvement in the Committee for Human Medicinal Products.

In addition to the proposed legislation, Le Cam stressed the importance of addressing delayed treatment access through policies, investments, and collaborations among Member States. To counter Europe's loss of ground to global competitors in the rare disease field, he proposed developing a comprehensive "European Action Plan for Rare Diseases", incorporating coordinated research infrastructure, investment, and access to ensure effective implementation of critical legislative measures like the Pharmaceutical Package in building the European Health Union.

Read the EURORDIS response here.

The European Patients' Forum (EPF) views the revision of the EU pharmaceutical legislation as a crucial opportunity to make the regulatory framework more patient-centric and ensure equitable access to medicines. While they acknowledge some positive aspects of the European Commission's proposal, EPF believes there are areas for improvement to fully achieve the legislation's objectives.

EPF advocates for a patient-led definition of 'unmet medical need', emphasising the importance of patient input in defining concepts such as 'added therapeutic value' to ensure medicines meet patients' specific needs. The organisation also urges EU institutions to create a more equitable system for patient access to medicines by striking the right balance between incentivizing research and development (R&D) of beneficial products and ensuring timely access for patients. Additionally, EPF highlights the need for patient involvement throughout

the regulatory process to enhance the quality of medicines and regulatory decisions, supporting innovation while prioritising patient safety.

Read the EPF response here.

5. Health Technology Assessment

EURORDIS-Rare Diseases Europe has initiated the European Capacity Building for Patients (EUCAPA) project under the EU4Health initiative. EUCAPA aims to train patients and their representatives in Health Technology Assessment (HTA), which involves evaluating the value of health technologies compared to others. By providing knowledge and skills, EUCAPA seeks to enable patients to participate in HTA decision-making processes and advocate for equal access to innovative treatments for rare diseases.

The project, lasting two years, will be carried out by a consortium comprising EURORDIS, the European Patients Forum (EPF), and UMIT TIROL. It will offer online and in-person training for patients to actively engage in HTA consultations and assessments as part of the new Regulation on HTA (EU) 2021/2282, which starts assessing health technologies in January 2025.

EUCAPA's initiative is crucial, given the increased role of patient experts in the HTA Regulation framework. By providing comprehensive training, the project equips patient advocates with the necessary expertise to meaningfully participate and share their lived experiences in assessing health technologies.

Read more here.

HTA plays an important role in the bleeding disorders landscape. The ability for an individual patient to input into horizon scanning, model inputs and design, procurement methods, and reporting is increasing with HTA agencies improving their engagement processes. The EHC aims to ensure access to education for patients and patient organisations on-demand for engaging in health technology assessments. As a result, the EHC has decided to create an **online educational course on Health Economics in Europe**.

The program will be online, consisting of sequential webinars with additional interactive materials to improve personal and NMO knowledge. The most significant benefit of the EHC Economics Workshops is the cross-fertilisation of discussion and ideas. The course would also provide two Q&A sessions with prominent experts to guide involvement in HTA at any level.

Read more here.

6. European Health Data Space

As readers may recall, in May 2022, the European Commission launched the *European Health Data Space* (EHDS) as part of the European Health Union to empower citizens to control and utilise their health data in their home country or other Member States, to foster a single market for digital health services and products, to offer a consistent, trustworthy, and efficient framework to use health data for research, innovation, policy-making, and regulatory activities, and lastly, to comply with the EU's high data protection standards fully.

Read the proposal here.

EURORDIS-Rare Diseases Europe, the EPF, and other not-for-profit organisations representing patients, healthcare professionals, hospital pharmacists, payers, and healthcare institutions are urging European policy-makers to adopt a more coherent, fair, and society-centred approach to the EHDS, emphasising that the interest of society should take precedence over profit when it comes to healthcare, research, and health data sensitivity, and urging European policy-makers to avoid supporting strengthened provisions for intellectual property (IP) rights and trade secrets, as data shared through the EHDS should be considered a public investment.

Furthermore, signatories of the *Joint Statement on the European Health Data Space Draft Proposal* believe that civil society representatives, including patients, consumers, citizens, healthcare professionals, payers, public health representatives, healthcare institutions, and digital rights advocates, should be incorporated into EHDS governance to ensure transparency and avoid conflicts of interest, and that sufficient time should be given to develop and implement the EHDS, respecting GDPR principles and ensuring robust cybersecurity to gain the trust of individuals and stakeholders.

Read the Joint Statement here.

7. Women's health

The European Institute of Women's Health (EIWH) is calling for an *EU Strategy for Women's Health* to address the longstanding issue of gender and health inequities. Sex and gender significantly impact health and well-being, affecting the risk of health problems, drug efficacy and adverse reactions, health-seeking behaviours, and interactions with healthcare services.

Women face underestimation of pain, longer diagnostic delays, and underrepresentation in clinical trials, leading to a higher risk of adverse drug reactions than men. Furthermore, many conditions specific to women lack sufficient research, funding, and attention and still carry stigma and taboo. Therefore, a more comprehensive and supportive approach is needed to empower and support women for a healthier and more equitable society.

The EIWH urges policymakers to integrate sex and gender into policies, programs, education, research, and data collection to address the gaps and unmet needs in women's health. An EU Strategy for Women's Health is proposed to drive gender equity in health in Europe, supported by a Women's Health Interest Group in the European Parliament. By investing in

women's health and well-being, society can combat inequities and ensure a healthier future for all genders.

Read the Manifesto here.

8. Liver health

As readers may remember, the MEP Friends of the Liver Group (hereafter 'the Group') was relaunched last year on March 15.

Whilst a lot has been achieved with the recognition of viral hepatitis B and C in Europe's Beating Cancer Plan and EU4Health funding, it is obvious that a lot still needs to be done to ensure that the EU collectively meets the WHO's 2030 elimination deadline. To achieve this, a Call to Action for the EU, European Commission, and EU Member States to lead on viral hepatitis elimination by 2030 was put together and endorsed by several MEP Friends of the Group.

On 25 April 2023, with the support of the ACHIEVE Coalition, The European Association for the Study of the Liver (EASL) organised a webinar to present the Call to Action. The event was hosted on the European Commission's Health Policy Platform. The recording is available *here*. This webinar aimed at presenting the MEP Friends of the Liver Call to Action's key objectives and receiving feedback from participants on how to turn them into actionable initiatives.

The EHC is also directly engaged with EASL and its Foundation. In the framework of World Haemophilia Week, the EHC held a webinar on Liver Health to mark World Liver Day 2023. The discussion 'On a Pathway Towards Healthy Ageing: Liver Health' featured Declan Noone (Ireland), Brian O'Mahony (Ireland), Clive Smith (United Kingdom), and Prof Vincenzo La Mura (Italy). The recording of the webinar is on the EHC *YouTube channel* and *EASL Campus*.

Community focus:

Liver disease remains one of Europe's leading causes of death for people with rare bleeding disorders. This is due to the long-term damage caused by hepatitis C virus (HCV) infection because of the treatment contamination in the 1980s and 1990s. Despite the HCV cure, people who have lived with this infection for decades remain at a higher risk for non-alcoholic fatty liver disease and require further monitoring and care.

Gene therapy in haemophilia has been recently marketed in Europe, bringing patients fresh hope for a better quality of life. However, the treatment involves the infusion of large amounts of viral vectors targeting the liver to deliver the modified gene, which may potentially impact liver health.

Read more about the Call to Action here.

9. Mental and physical health

In June 2023, the European Commission unveiled a new comprehensive approach to mental health, establishing it as a vital component of the European Health Union. This initiative includes 20 flagship initiatives and €1.23 billion in EU funding to prioritise people's mental well-being. Mental health problems have been a significant issue even before the COVID-19 pandemic, affecting 1 in 6 people in the EU, and the crises in recent years have exacerbated the situation, resulting in an estimated annual cost of €600 billion due to non-action.

The comprehensive approach focuses on three guiding principles: prevention, access to quality and affordable mental healthcare and treatment, and reintegration into society after recovery. The EU aims to address mental health across all policies, acknowledging the various risk factors contributing to mental health issues. To achieve this, concrete actions will be taken in areas such as promoting good mental health through prevention and early detection, investing in training and capacity building to enhance mental health support, ensuring good mental health at work through awareness-raising campaigns and addressing psycho-social risks, protecting children and vulnerable groups, and providing quality mental health support in humanitarian emergencies.

Read more here.

EURORDIS-Rare Diseases Europe welcomes the Comprehensive Approach to Mental Health but urges the European Commission to explicitly recognise and address the mental health challenges facing the rare disease community, as well as individuals with other physical health conditions. They emphasise the importance of turning the strategy into tangible actions, backed by sufficient resources, to ensure holistic care and support for affected individuals across Europe's communities.

Read the EURORDIS response here.

10. Social care

Long-term care service providers, social partners, education and training providers, along with support from the European Commission, have established a large-scale skills partnership for the long-term care sector. The goal of this partnership is to enhance career paths and improve the quality of care in the sector, making it more appealing. By 2030, they aim to train at least 60% of the long-term care workforce (approximately 3.8 million workers) annually, with a focus on developing digital skills and emphasizing person-centred care.

Europe's ageing population has led to an increased demand for long-term care, with over 6.3 million people currently employed in the sector. To maintain the current level of care coverage, an additional 1.6 million workers will be needed by 2050. To address labour shortages and ensure the sector attracts and retains workers, particularly since women constitute nearly 90% of the care workforce, improving skills and training is vital. The partnership will also facilitate the exchange of best practices and lessons learned with relevant authorities, education and training institutions, and the healthcare sector.

Read more here.

11. Looking forward

11.1. Spanish Presidency of the Council of the European Union

On 1 July 2023, Spain took over the presidency of the Council of the European Union. Till December 31, it will ensure the continuity of the EU agenda, legislative processes, and cooperation among member states.

In healthcare, the Presidency is committed to addressing the prevention of, and more effective response to, childhood obesity and the fight against HIV and stigma, two issues on which Spain has national plans. The Presidency also announced that it wants to tackle the approach to rare diseases and mental health problems.

With a comprehensive strategy encompassing safeguarding vulnerable groups, bolstering readiness, and response frameworks for health crises, and harmonising the European health agenda with global objectives, the Spanish presidency is fully committed to advancing a more robust and health-conscious Europe. The Spanish Presidency will also persist in its efforts towards the establishment of the EHDS, aimed at enhancing healthcare policies and research.

Find the full programme here.

Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe, welcomed this explicit prioritisation, as it signals progress towards a comprehensive EU strategy to address the unmet needs of those living with rare diseases. The organization hopes that the Spanish Presidency will bring the EU closer to implementing a European Action Plan for Rare Diseases.

The commitment to mental health as a health priority is also lauded, given that individuals with rare diseases often face challenges to their psychological well-being. The Spanish Presidency's emphasis on consolidating the EU's Social Pillar and advancing initiatives affecting vulnerable populations, including those with rare diseases, is seen as a step towards creating an inclusive and equitable society for all.

Read the EURORDIS response here.

11.2. Reflection paper on the use of artificial intelligence in the lifecycle of medicines

In July 2023, the European Medicines Agency (EMA) released a draft reflection paper focusing on the use of artificial intelligence (AI) in the development, regulation, and usage of human and veterinary medicines. The paper is open for public consultation and emphasises principles relevant to applying AI and machine learning (ML) throughout the entire lifecycle of medicines, from drug discovery to post-authorisation monitoring.

AI and ML tools have the potential to significantly support data acquisition, transformation, analysis, and interpretation across the medicinal product lifecycle. They can be employed in various stages, such as preclinical development, clinical trials, marketing-authorisation procedures, and post-authorisation activities like pharmacovigilance. However, the use of AI also poses challenges related to algorithm transparency, potential biases, technical failures, and ethical considerations.

The reflection paper underscores the importance of a human-centric approach to guide the development and deployment of AI and ML in the medical field. It stresses compliance with existing legal requirements, ethics, and respect for fundamental rights. Developers using AI/ML systems that could impact a medicine's benefit-risk balance are advised to seek early regulatory support through the qualification of innovative development methods or scientific advice.

Read more here.