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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 second 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. DG COMM publishes 2023 State of Health Preparedness Report

Two newly released reports by the EU delve into the strides made in fortifying health systems against crises and identifying areas that demand further attention. The 2023 State of Health Preparedness report outlines the EU's measures in bolstering its readiness against cross-border health threats, spotlighting initiatives such as the Regulation on serious cross-border threats to health and upgraded structures such as the EU Health Security Committee. It emphasises vaccination's pivotal role in disease prevention, stresses the urgency of addressing antimicrobial resistance (AMR), and underscores the need for a comprehensive approach to health threats such as animal diseases and climate change.

Meanwhile, the State of Health in the EU: Synthesis report 2023 evaluates the performance of health systems across the EU, spotlighting mental health challenges exacerbated by the pandemic. It advocates for reforms encompassing destigmatization, prevention, treatment, and reintegration, along with efforts to tackle health inequalities and unmet medical needs. Additionally, it emphasises sustained investment in health, the importance of robust health data, and initiatives such as the European Health Data Space to empower citizens with better access and control over their health information.

Both reports underscore the EU's commitment to fortifying health systems, implementing reforms, and leveraging comprehensive data analysis to drive informed policymaking and enhance the EU's health resilience.

1.2. Pharmaceutical legislation

As readers may recall, in 2023, the European Commission proposed a comprehensive reform of the EU's pharmaceutical legislation, the largest in over 20 years, with the aim of making medicines more accessible, affordable, and environmentally sustainable while fostering innovation and competitiveness in the EU pharmaceutical industry.

In October 2023, the European Alliance for Transformative Therapies (TRANSFORM), with the EHC among its signatories, updated its Position on the Pharmaceutical Package with a new position on unmet medical needs, recognising urgent unmet healthcare needs in the EU and supporting legislative measures to introduce innovative therapies. TRANSFORM emphasises the complexity of these issues, advocating for ongoing multi-stakeholder engagement to address social, economic, and medical factors contributing to these needs.

Furthermore, TRANSFORM calls for mandatory early dialogues involving various stakeholders within existing European Medicines Agency (EMA) and Health Technology Assessment (HTA) structures. These discussions aim to refine understandings of unmet needs in specific disease areas over time, aligning with evolving evidence and justifications for addressing these gaps in healthcare.

Read the full position paper here.

In November 2023, the European Parliament Environment, Public Health and Food Safety (ENVI) Committee held a debate on revisions to the EU's general pharmaceutical legislation. EURORDIS-Rare Diseases Europe welcomed the European Parliament's discussion, however, they cautiously supported MEPs' draft reports, emphasising the need to balance healthcare innovation for rare diseases with improved patient access to medicines and to involve patients throughout the medicinal life-cycle for a patient-focused healthcare system.

They stressed the importance of addressing high unmet medical needs within the rare disease community and supported amendments for medicine accessibility, favouring common procurement and swift access through PRIME. EURORDIS commended the plan for a European Action Plan for Rare Diseases, aiming to bridge healthcare gaps for the 30 million EU citizens affected by rare diseases. They pledged to collaborate with MEPs to ensure the legislative revisions reflect the needs of the rare disease community and showcase the EU's commitment to these individuals.

Read more here.

1.3. European Health Data Space (EHDS)

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 allow cross-border access to patients' electronic records so that general practitioners (GPs), hospitals, and medical practitioners can see a patient's medical data throughout the EU.

For patients, the EHDS would make it possible, for example, for a Lithuanian tourist to pick up a prescription in a French pharmacy, or for doctors to access the health information of an Italian patient undergoing treatment in Belgium. This so-called 'primary use' of data will mean a patient's data such as medical images, lab results or health summaries added to a European portal called MyHealth@EU. Medical professionals and the patients themselves will be able to access the data (in the relevant European language) through this platform.

However, there is another dimension of health data which so far made it difficult for the EU institutions to find a common ground - the 'secondary use' of the data by researchers and pharma companies. The 'secondary use' means that the hospitals and doctors forward patients' medical records — anonymised or pseudonymised — to a centralised national data space, which can then be accessed for research purposes.

Next steps:
The Belgian presidency has set the first inter-institutional discussion for January 30, with further talks planned on February 20 and March 7.

The most disputes will be around whether patients have to opt in or out of the entire system. Under the Commission's text, there is no mechanism foreseen. The Parliament's position gives countries the right to allow patients to object to the registration and storage of their health data in the EHDS. The Council meanwhile wants to give more say to countries over whether they choose to let their own patients block sharing of primary health care data for either cross-border or internal-country use, as well as for secondary purposes.

If and when the agreement is achieved, the system still needs to be set up and running. Some countries, like Finland and France, already have a fairly digitalised system in place, while other less digitally advanced countries have a lot of work to do, so they have pushed for a long implementation period (some nine years after the regulation is adopted).

Read the European Commission proposal here.

Advancing health data systems in Europe with the European Commission and WHO/Europe
In December 2023, the European Commission and WHO/Europe have entered a €12 million partnership aimed at strengthening health information systems and improving health data governance and interoperability across the WHO European Region, spanning 53 countries and nearly 1 billion inhabitants. Over four years, this collaboration seeks to increase the utilisation of health data by healthcare providers, policymakers, and patients. The project's focus is on elevating the quality and compatibility of health information systems through capacity-building activities and targeted assistance, intending to address existing gaps and foster expertise within supported countries' health data governance and capabilities.

The partnership with WHO/Europe aims to accelerate European integration by aligning health data governance and technical standards across EU and non-EU European countries with EU standards, emphasising citizens' and health professionals' access to health data.

The project will foster cooperation among participating countries, the European Commission, WHO/Europe, and external stakeholders, establishing the Health Information Network. It aligns with the EHDS framework and proposed regulation, contributing significantly to digital transformation in the healthcare sector while supporting various health strategies and programmes at regional and global levels. The initiative aims to enhance interoperability and data consistency within health information systems, a critical aspect for their cost-effectiveness and impact on public health. It is part of the EU4HEALTH programme, specifically addressing health data governance and interoperability to protect against cross-border health threats in the EU and neighbouring regions.

Read more here.

TEHDAS insights

TEHDAS, a collaborative effort aiming to shape the European Health Data Space (EHDS), has concluded, offering key insights and recommendations to the European Commission. Their report emphasises the necessity of secure, high-quality health data accessibility, mediated through a specialized body, and stresses stakeholder engagement for EHDS implementation readiness. It looks forward to TEHDAS2, emphasising the significance of these recommendations in future EHDS development.

Concurrently, EHDS2 Pilot, funded by the EU4Health program, is constructing an EHDS prototype over two years. It focuses on facilitating health data utilisation across EU Member States for research purposes and establishing standards for data quality, security, and transfer. Moreover, a newly released report defines a data quality framework for EHDS, proposing 13 recommendations including enhancing data management maturity, integrating the quality framework throughout the data lifecycle, and advocating for data holders to publish comprehensive dataset descriptions to ensure quality and utility alignment.

Read more here.

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

On 14 December 2023, after months of negotiations the European Council, the European Parliament, and the European Commission reached a provisional political agreement on the EU Regulation on Substances of Human Origin (SoHO).

As readers may recall, the European Commission adopted a Proposal for SoHO intended for human application in July 2022. 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 had seen several delays.

The text agreed by the three co-legislators broadens the scope of SoHO to also include human breast milk and intestinal microbiota. It also aims to future-proof the EU's legislation by covering other SoHO that may be applied to humans in the future and by allowing more flexible future updates.

The proposed regulation covers a wide range of activities from registration and testing of donors, collection, and processing to human application and clinical outcome monitoring of substances of human origin through:

- setting up an EU-level SoHO coordination board supporting member states in the implementation of the regulation;
- introducing common EU-wide procedures for the authorisation and assessment of SoHO preparations;
- requiring member states to designate a SoHO national authority and other competent authorities to authorise SoHO preparations and ensure independent and transparent oversight of SoHO-related activities;
- setting out additional authorisation and inspection requirements for establishments that both process and store, release, import or export substances of human origin;
- establishing a new common IT platform, the EU SoHO platform, to register and exchange information on related activities.

Voluntary and unpaid donations

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. Thus, 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.

Under the provisional agreement, donations of SoHO should be voluntary and unpaid as a matter of principle, and donors must not be provided with financial incentives to donate. Living donors may receive compensation or reimbursement as appropriate in line with national legislation.

Response to emergencies

The draft regulation also provides for a rapid alert system to cope with serious incidents or reactions that are likely to pose a risk for recipients or donors. Member States should also make reasonable efforts to ensure the sufficient, adequate and resilient supply of critical SoHO in their countries, including by drawing up national emergency plans, including measures to respond to critical shortages.

For the EHC 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.

Last year, 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 final text is still being checked at the technical level with a vote in the ENVI committee scheduled for February 14. Following the publication of the final text, it will then be formally adopted by the European Parliament and the Council, which will apply 3 years after its adoption.

Once adopted and implemented in all Member States, the Regulation will replace the rules for safety and quality set out in two Directives (2002/98/EC, for blood and blood components, and 2004/23/EC, for tissues and cells), and their implementing acts.

Read more here.

Read Press Releases of the co-legislators here: Click here for Council's PR; click here for Parliament's PR; click here for Commission's PR.

1.5. EURORDIS advocates for ERNs

In October 2023, EURORDIS-Rare Diseases Europe and representatives from 24 European Reference Networks (ERNs) wrote to the leaders of the European Commission, European Parliament and EU Member States to advocate for the renewal of key support for these networks. These ERNs connect clinical experts across Europe to collaborate on the diagnosis, treatment, and management of rare diseases, addressing the challenges posed by these conditions.

Due to the often limited patient populations and complexities of these conditions, accessing specialised expertise within individual nations can be challenging, leading to inadequate healthcare and social support for affected individuals. Launched in 2017, ERNs aim to bridge these gaps by connecting healthcare professionals across borders, allowing resource pooling, data sharing, and best practice development.

With 24 ERNs dedicated to specific rare disease categories, they bring together over 1,600 specialised healthcare units from 27 EU countries and Norway. These networks have achieved milestones, such as pooling expertise during COVID-19, facilitating cross-border expert panels, creating clinical practice guidelines, establishing patient registries, and fostering research initiatives.

The open letter highlights key achievements and proposes recommendations for the future, emphasising the need for sustained financial support, geographic coverage improvement, expanded disease coverage, enhanced collaboration, integration into national health systems, patient monitoring, and recognition of volunteer contributions. Overall, the rare disease community urges EU institutions and Member States to strengthen ERNs, making them a cornerstone of the European Health Union and ensuring equitable access to specialised healthcare for those with rare or complex conditions.

Read more here.

Not to miss

The EHC partnered with the ERN EuroBloodNet in 2023 to launch a big educational programme on von Willebrand Disease (VWD) on February 1, 2024. The launch of the programme is timed to correspond with the European VWD Awareness Day.

This programme is aimed to distinguish VWD from haemophilia and focuses on the particularities of this bleeding disorder. The programme also aims to discuss very topical areas related to VWD among patients and healthcare professionals in order to give visibility to the challenges of those living with this rare disease, including quality of life, access to treatments and care, medical services available in Europe, diagnosis and treatments options, and the overall patient journey through life. A further objective of this programme is to disseminate up-to-date knowledge among interested haematologists, gynaecologists, internists, paediatricians, nurses, or other healthcare providers and patient organisations in the field of VWD. Ultimately, the aim of this approach is to support better patient care.

The programme will consist of 13 sessions moderated by a duo of an expert physician or/and nurse, a psychologist, and a patient representative, who host the session together. The healthcare professional introduces the topic and shares clinical knowledge, and the patient representative ensures that the information provided is contextualised and accessible to patients and their families. The programme also highlights the key crucial concepts, encouraging a dialogue between patients and experts, and seeking clarification of these for the webinar audience.

Read more here.

2. Mental and physical health

2.1. European Commission publishes Eurobarometer on mental health

The Europen Commission published its Flash Eurobarometer on mental health in October 2023. The survey highlights the public's perception of mental health's importance compared to physical health, with 89% recognising its equal significance. However, less than half of respondents believe that individuals with mental health issues receive comparable care to those with physical ailments. The survey also underscores the significant impact of recent global events on mental health, with living conditions and financial stability emerging as key determinants in maintaining good mental well-being, alongside social interaction and physical activity.

In acknowledgement of the importance of mental health, Health Commissioner Stella Kyriakides spearheaded a significant conference addressing this crucial issue on World Mental Health Day on 10 October. The gathering convened representatives from EU institutions, national governments, European organisations, and various stakeholders. Throughout the event, participants shared personal accounts, exchanged insights, and discussed effective

strategies for promoting mental well-being and preventing challenges. Central to the discussions were considerations on ensuring equitable access to mental health resources for everyone, emphasising parity in care between mental and physical health concerns.

2.2. European Parliament adopts recommendations on prioritising mental health

The European Parliament has adopted recommendations on mental health, emphasising the need for a comprehensive EU Mental Health Strategy, building upon the recent European Commission's communication. With an overwhelming vote of 482 to 94, Members of the European Parliament (MEPs) have backed the report led by Sara Cerdas MEP under the Committee on Environment, Public Health and Food Safety.

These recommendations highlight the necessity for a long-term, integrated approach to mental health within EU and national policies. The report stresses the importance of addressing mental health promotion, particularly for vulnerable groups, combatting discrimination and stigma, and enhancing access to mental health services. It underlines the intricate interplay between socio-economic, environmental, and biological factors in shaping mental health, advocating for comprehensive policies across sectors.

Rapporteur Sara Cerdas emphasises the crucial link between mental and physical health, calling for robust EU action in prevention, support, treatment, and resilience for mental health. This report's adoption aligns with the European Commission's recent communication, laying out 20 initiatives aimed at prevention, quality mental healthcare access, and societal reintegration post-recovery.

Read the report here.

3. Health Technology Assessment (HTA)

The EUnetHTA 21 Consortium concluded its two-year operations, marking achievements such as joint consultations for medicines, training sessions for stakeholders, and recommendations for optimising assessment reports. Despite its closure, efforts continue toward implementing the Regulation on HTA, led by the HTA Coordination Group.

Over the past few years, the EMA and the EUnetHTA 21 consortium have collaborated on various initiatives to prepare the EU for the Regulation on HTA. This collaboration, starting in 2010, aimed to enhance clinical research and efficiency in generating evidence for both regulatory authorities and HTA bodies. While EUnetHTA 21 ceased operations in September 2023, their joint efforts have been incorporated into European law. The transition involves a

new framework for cooperation, culminating in the Regulation's implementation in January 2025, focusing on joint clinical assessments, consultations, and the identification of emerging health technologies. During the transition until 2025, a parallel advisory system involving both EMA and HTA bodies has been established.

Achievements highlighted joint scientific consultations, discussions on evidence needs for advanced therapy medicinal products, training for patient and healthcare professional engagement, and recommendations for optimising assessment reports. The Regulation aims to enhance the availability of innovative medicines, ensure resource efficiency, and elevate the quality of health technology assessment in the EU while fostering collaboration, transparency, and inclusivity among stakeholders.

Preparations are ongoing at EMA to align with the Regulation, focusing on defining evidence plans, harmonising evidence assessment, and involving various experts in decision-making processes over the next 15 months.

Read more here.

For the EHC community:
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, in December 2023, the EHC held an **online educational course on Health Economics in Europe**.

The programme of this course was created in collaboration with the experts from HCD Economics and others and consisted of four modules with additional interactive materials to improve personal and NMO knowledge. The participants of the course also had an opportunity to ask questions directly to the content experts during two live Q&A sessions. The Course was highly evaluated by participants with an overage score of "very good - excellent". We invite all EHC NMO representatives to take part in the next course edition in 2024.

Read more here.

4. EU Critical Medicines Act

On December 12, 2023, the European Commission, the Heads of Medicines Agencies (HMA) and EMA published the first version of the Union list of critical medicines. It contains more than 200 active substances of medicines for human use considered critical for healthcare systems across the EU/EEA, for which continuity of supply is a priority and shortages should be avoided. The European medicines regulatory network will prioritise critical medicines for EU-wide actions to strengthen their supply chain.

The publication of the Union list will not impact existing or to-be-established national lists of critical medicines. However, it will support the network's efforts in drawing up national lists where these do not yet exist. In addition, it will support and expedite the EC's analysis of the supply chain of critical medicines to determine potential vulnerabilities. A medicine is considered critical if it is used in serious diseases and cannot be easily replaced by other medicines, for example, in case of a shortage. It is included in the Union list of critical medicines if it meets certain criteria, including being critical in more than one-third of EU/EEA countries.

The medicines, critical for the EHC community can be found in the section 'B - Blood and blood forming organs'.

Next steps:

Following the publication of the list, the European Commission will soon launch a 'critical medicines alliance' to address the problem of drug shortages. Now the Health Emergency Preparedness and Response Authority (HERA) is whittling it down to a shortlist of medicines with the most vulnerable supply chains. HERA's report on these medicines is also expected in April 2024.

Also coming up: a common approach to medicines stockpiling, as well as new guidance on medicines procurement practices.

Access the list here.

Critical Medicines Alliance may be launched in April 2024

The Belgian presidency hopes to launch an alliance of public, private, and civil society partners in April to tackle the drug shortages in Europe. It is hoped that the Critical Medicines Alliance will be launched at an informal gathering of health ministers on April 23-24.

The alliance is one of the new measures designed to bring greater security to EU countries' medicines supplies. One of its first tasks will be to identify the most vulnerable medicines, which could benefit from extra measures to shore up supply. That could include making demand more predictable, encouraging diversification of suppliers, EU stockpiling, and boosting manufacturing through EU projects.

5. EMA publishes the final version of the Data Quality Framework for EU medicines regulation

On December 12, 2023, the European Medicines Agency (EMA), in collaboration with the Heads of Medicines Agencies (HMA) and the Joint Action Towards the European Health Data Space (TEHDAS), published the final version of the Data Quality Framework (DQF) for EU medicines regulation.

The document provides a set of definitions, principles, and guidelines that can be applied to current and novel data sets to characterise, assess, and assure data quality for regulatory decision-making.

There are 5 main dimensions of quality outlined in the Data Quality framework: Reliability, Extensiveness, Coherence, Relevance, and Timeliness.

The EMA will collaborate with stakeholders to apply the framework's principles and create practical guidelines for evaluating the quality of data. The initial focus will be on real-world data and adverse drug reactions.

Many examples provided in the framework relate to real-world data, including within clinical trials to supplement trial-specific data collection. However, the scope of the framework extends to a broad range of regulatory activities and their respective data types, including bioanalytical omics data, spontaneous adverse event reporting data, chemical and manufacturing control data, and more.

The publication is intended to be a general resource from which more focused recommendations can be derived for specific regulatory domains with specified metrics and checks. The EMA will collaborate with stakeholders to apply the framework's principles and create practical guidelines for evaluating the quality of data with an initial focus on real-world data and adverse drug reactions.

Access the document here.

6. Women's Health

The Women's Health Interest Group, hosted by MEP Tilly Metz, was launched in October 2023. The launch event featured discussions among stakeholders such as MEPs, health advocates, NGOs, researchers, and more, shedding light on women's health inequities, highlighting progress made in Member States, and addressing the work yet to be done.

The European Institute of Women's Health (EIWH) recognised the urgency of prioritising women's health, establishing the Interest Group ahead of the European elections. Its goal is to shape an EU Strategy for Women's Health and advocate for gender equity in health across Europe, and aims to integrate women's health into the European Parliament's work by uniting diverse MEPs to ensure quality healthcare for all women in Europe.

The event emphasised the need for gender equity across healthcare, spanning research, technology assessment, policy development, and strategies to improve healthcare equity. The discussions also referenced the 2024 Women's Health Manifesto, with the EHC among its signatories, for further insights.

7. Social care

The European Commission has proposed the creation of a European Disability Card and an improved European Parking Card to facilitate the movement of individuals with disabilities across the EU. The European Disability Card will act as proof of disability status, granting equal access to special conditions and preferential treatments in various settings like public transport, cultural events, museums, leisure and sports centres, and more. These benefits may include free entry, reduced tariffs, priority access, personal assistance, and mobility aids. The card will complement national disability cards already issued by individual countries.

Additionally, the enhanced European Parking Card will ensure access to designated parking spaces and facilities for persons with disabilities throughout EU countries, replacing existing national parking cards. The proposal was put forth in September 2023, and negotiations between the EU Council and European Parliament are underway. Once a legal act is passed, EU governments will have 2.5 years to implement these cards for their nationals. This initiative aligns with the EU's Strategy for the Rights of Persons with Disabilities 2021-2030, building upon the EU disability card pilot project and Council Recommendation 98/376.

Read more here.

EURORDIS welcomes the European Commission's EU Disability Card proposal, aiming to grant equal service access to individuals with disabilities across EU countries. While supportive, they emphasise the critical need for robust implementation and monitoring to ensure the card's effectiveness. EURORDIS applauds the separation of the Disability Card from the Parking Card but urges policymakers to broaden its scope to include areas such as EU mobility programs. They advocate for improved national disability assessments and EU-wide standards to prevent the exclusion of any citizen with disabilities from societal participation initiatives, emphasising equal rights for all in the EU.

Read more here.

8. Looking forward

8.1. Belgian Presidency of the Council of the European Union

On 1 January 2024, Belgium took over the Presidency of the Council of the European Union. Till June 30, it will ensure the continuity of the EU agenda, legislative processes, and cooperation among member states.

Under the Belgian presidency, health will revolve around three overarching themes: preparedness, care, and protection. The presidency will emphasise the importance of strengthening the EU's resilience to future health threats by reinforcing crisis management, supporting healthcare systems, and improving the security of medicines supply.

Taking a cross-cutting approach, the presidency will highlight the significance of health in all policies: among others, mental wellbeing at work, equitable access to health care and health products, global health and research and development for pandemic preparedness will be topics addressed in different formations of the Council under the Belgian presidency.

Find the full programme here.