European Haemophilia Consortium (EHC)
Rue de la Loi 28, 1040 Brussels, Belgium
Email: office@ehc.eu; Website: www.ehc.eu

Public Policy Review

Periodic review of the European public policy landscape

2022

AISBL European Haemophilia Consortium

No. 887.106.966; VAT: BE 0 887.106.966; Bank KBC: IBAN: BE80 7360 0157 2777; BIC: KREDBEBB

The current edition covers legislative initiatives of 2022.
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. On rare diseases

1.1. UN Resolution on persons living with rare diseases

As readers may recall, in December 2021, the United Nations (UN) adopted the first-ever resolution on persons with rare diseases, *Addressing the challenges of persons living with a rare disease and their families*. This adoption marks an important milestone in global recognition of the needs of the rare disease community. It represents a formal commitment of the UN Member States (MSs), including the 27 EU MSs, to support rare disease policies, promote, and protect the rights of everyone living with a rare disease and their families.

The UN MSs have acknowledged the barriers and challenges that the 30 million people living with a rare disease in Europe face daily. By adopting the resolution, they have paved the way for future common umbrella legislation on rare disease patient needs in Europe. In fact, currently, there is no such legislation that would serve as a framework to ensure the coordination of national efforts to implement person-centred and gender-sensitive national plans and strategies for rare diseases with measurable objectives.

As readers may also recall, in 2021, EURORDIS proposed *Europe's Action Plan for Rare Diseases,* which emerged as the main recommendation of its *Rare 2030 study* and can potentially take the form of an EU Commission Communication and/or Council Recommendation.

The resolution is a powerful advocacy element that can support advocacy efforts both at national and European levels.

On 28 February 2023, EURORDIS will celebrate Rare Disease Day to raise awareness of people living with rare diseases. The EHC traditionally joins the celebrations and will this year focus on the extremely rare cohort of its community.

Access the resolution here.
Read more about what the resolution means for the rare disease community here.
Access the information page on Rare Disease Day here.

1.2. EU Revision of the legislation on medicines for rare diseases and children

The highly anticipated revision of the *Orphan Medicinal Products and Paediatric Regulations* in the coming months will hopefully shed light on how the EU should improve social protection for people with rare diseases and raise awareness of their situation and needs. The revision was set up in 2020 when the European Commission (EC) announced in its pharmaceutical strategy that, over the coming years, it would launch a revision of the EU's legislation on medicines for rare diseases and children:

- Regulation (EC) No 141/2000 on orphan medicinal products ('Orphan Drugs Regulation');
- Regulation (EC) No 1901/2006 on medicinal products for paediatric use ('Paediatric Regulation').

Generally, both the Commission and Members of the European Parliament (MEPs) agree that the Orphan Drugs Regulation has had some success in boosting the development of medicinal products for rare diseases. However, according to the Commission's evaluation of the regulation, the medical needs of patients with rare diseases are not sufficiently met. At the same time, the affordability of orphan medicines is becoming a growing challenge for EU healthcare systems, adding to that unequal access to medicines and insufficient incentives for developing new orphan medicines.

This revision will address these and other shortcomings in the functioning of the existing framework detected during a recent evaluation. It will also aim at supporting the development of products in areas of high unmet needs for patients and ensure their timely access. At the same time, it aims to ensure that the legislation is fit to keep pace with technological and scientific development. Finally, it hopes to streamline and simplify existing procedures linked to the evaluation and authorisation of medicines for rare diseases and for children, thus, reducing the burden both for companies and regulators.

Next steps:
The revision has been postponed several times already. It is expected to be adopted in the coming months and to come together with other key legislative actions in the EU's pharmaceutical strategy, including a revision of the general pharmaceutical legislation and legislation on paediatric medicines.

Rare disease community voice:
In November 2022, EURORDIS presented its proposal on the revision of the *Orphan Medicinal Products and Paediatric Regulations*. This proposal represents a contribution from both EURORDIS and its members, and offers concrete recommendations based on community expectations. The document is the result of progressive collective work since 2018 through EC's public consultations, evaluations, events, and conferences.

Read the EURORDIS proposal here.

2. European Health Union

2.1. Regulation revising the mandate of the European Medicines Agency

As readers may recall, as part of the European Health Union package, on 25 January 2022, the Council adopted the *Regulation revising the mandate of the European Medicines Agency* (EMA). The main purpose of this regulation is to allow the EMA to closely monitor and mitigate the risk shortages of critical medicines and medical devices during major events and public health emergencies, and to facilitate faster approval of medicines that could treat or prevent a disease causing a public health crisis.

The EMA will also provide scientific advice on medicines that may have the potential to treat, prevent or diagnose the diseases causing such crises, coordinate studies to monitor the effectiveness and safety of medicinal products. It will also coordinate clinical trials for

medicinal products intended to treat, prevent or diagnose diseases related to such public health crises.

The EHC will continue to monitor the implementation of this regulation.

Read more here.

2.2. EU Global Health Strategy

On 30 November 2022, the Commission adopted a new *EU Global Health Strategy* as the external dimension of the European Health Union to improve global health security and deliver better health for all in a changing world by going 'back to basics':

1. Achieving better health and well-being through promoting drivers of good health, addressing root causes of ill health such as poverty, social inequalities, climate change and environmental degradation, food security, conflict, and other humanitarian crises, and by paying particular attention to women, girls and people in vulnerable situations;
2. Advancing universal health coverage by establishing a more equitable primary healthcare system, ensuring better capacity to meet health emergency needs, leveraging digitalisation and research, and addressing workforce imbalances; and
3. Combatting health threats, including pandemics.

As a response to the emerging 'new global health order', the Strategy aims to expand the EU's international partnerships on health based on co-ownership and co-responsibility from its partners. Internally, leveraging the 'Team Europe' approach, the Strategy aims to ensure close cooperation between Member States.

Read more here.

2.3. European Centre for Disease Prevention and Control (ECDC) extended mandate endorsed by the European Parliament

As previously reported on 11 November 2020, as part of building a European Health Union, the EC adopted a legislative proposal to extend the mandate of the European Centre for Disease Prevention and Control (ECDC).

In October 2022, the extended mandate was adopted with 542 votes in favour, 43 against, and nine abstentions. It will allow the ECDC to adopt a stronger role in supporting EU MSs in the prevention and control of infectious disease threats, and to improve European preparedness and response ahead of future public health challenges.

Importantly for the rare congenital bleeding disorders community, the ECDC will be responsible for building a network of EU reference laboratories and a network for substances of human origin, and together with the EDQM will develop the safety standards.

Read more here.

3. EU4Health

EU4Health, with a budget of €5.3 billion, is the fourth and largest of the EU health programmes. 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 website of the European Health and Digital Executive Agency (HaDEA) here.

In November 2022, the EC adopted the 2023 annual work programme of EU4Health, which consists of four overarching strands:

- Crisis preparedness;
- Health promotion & disease prevention;
- Health systems & healthcare workforce; and
- Digital.

Cancer is considered a fifth cross-cutting strand.

The work programme will also address:

- Health-related urgencies in relation to the COVID-19 pandemic and Russia's war with Ukraine;
- Mental health;
- Global health;
- The developments in digital health and medicinal products; and
- Actions to improve the uptake of cancer screening.

Read more here.

The total budget of the 2023 work programme is €735.8 million. Of the total budget above, €428 million will be in the form of grants, €176 million will be in the form of procurement (both under direct management), and €131 million will be under indirect management. Approximately €9 million will be allocated to Operating Grants.

€493 million of the total will be dedicated to major actions supporting the Commission's Directorate-General for Health and Food Safety (DG SANTE) key priorities:

1) EU resilience to cross-border health threats, including strengthening national surveillance systems and EU reference laboratories – €105.1 million;
2) EHDS for the primary and secondary use of data – €26 million;
3) *Pharmaceutical Strategy for Europe* and implementation of health legislations, such as medical devices and health technology assessment legislation – €21.3 million.

Read more here.

4. Regulation on standards of quality and safety for substances of human origin (SoHO) first in 20 years review

As readers may recall, in July 2021, the EC 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. It builds upon the existing legal framework, which came into force in 2002 for blood and in 2004 for tissues and cells, containing parallel provisions for donor selection, quality and safety management, and oversight. The proposal repeals the directives and replaces them with a single regulation that will be equally applicable to all MSs.

The legislation will facilitate the cross-border circulation of SoHO-based therapies as well as cross-border cooperation between public health authorities while ensuring the same harmonised high-level standards of quality and safety for all SoHO. Nonetheless, this regulation leaves MSs the opportunity to add more requirements, in particular, to ensure alignment with national healthcare systems.

What has changed?

- The proposal extends the rules of safety and quality to all substances of human origin, including blood, tissues, cells, as well as human breast milk or microbiota.
- The safety standards will mostly be developed by scientific bodies, namely the European Centre for Disease Prevention and Control (ECDC) and the European Directorate for the Quality of Medicines and Healthcare (EDQM).
- Any entity conducting activities that affect SoHO will have to register with their competent authority and report their annual activity data.
- The proposal includes the establishment of a SoHO Coordination Board (SCB) as an advisory body that will support MSs in the implementation of the regulation.

As for the blood donation compensation, the proposal allows MSs to choose how to thank donors for their donations. However, there are still areas which remain uncovered by the current legislation, namely, the shortage of plasma in Europe. The EU's dependency on plasma imports from the US leaves Europe vulnerable to potential supply disruptions. Currently, it heavily depends on imports — mostly from paid donors in the US — to meet the needs of around 300.000 people who require plasma-derived medicinal products (PDMPs). As medicinal products, PDMPs can potentially be further included in the general European pharmaceutical strategy on shortage prevention.

What it means for the community:
For some patients with rare congenital bleeding disorders, PDPMs remain their primary source of treatment. It is critical for these patients to have timely access to safe and efficacious medicines. As the proposal impacts the collection of plasma for the manufacturing of such medicines, it is of direct relevance to patients as end-users of those therapies.

Next steps:
The proposal put forward by the EC will now be discussed in parallel by the Council and the European Parliament. Once the final text is agreed upon and adopted, it will come into force

albeit with a two-year transition period before most provisions apply and a three-year period for some particular provisions.

Read more here and here.

5. Pharmaceutical legislation

5.1. Revision of the EU's general legislation on medicines for human use

In 2022, as a response to the lessons-learned from the COVID-19 pandemic, the European Commission intended to reform the EU's 20-year-old *general legislation on medicines for human use* to improve access to affordable medicines across the EU and ensure their availability, foster research and innovation, and reduce the administrative burden of regulatory procedures. However, the EC failed to meet the original deadline and is now aiming to publish the draft update on 1 March 2023. The proposed revision of the EU rules for medicines for children and rare diseases is planned for the same date.

According to EURORDIS, "Challenges in pricing and access to therapies show how current models are not sustainable. Major difficulties remain in access to the right diagnosis and treatments, which are often too costly for patients. This further exacerbates existing discrepancies and inequalities between Member States. A treatment that is unavailable to the patient who needs it loses its value. Scientific advances are happening fast and the regulatory framework should be agile yet rigorous, providing the highest standards for the implementation of clinical trials and distribution of medicines."

In addition to the revision of the pharmaceutical legislation, in December 2022 the EC also proposed to revise the entire EMA fee structure, update and simplify the legislation on fees charged by the EMA in relation to marketing authorisation of medicines, which includes renumeration to national authorities involved in the assessment.

Read more here.

5.2. Accelerating Clinical Trials in the EU (ACT EU)

As readers may be aware, in January 2022 *Accelerating Clinical Trials in the EU (ACT EU)* was launched; this is an EU clinical trials transformation initiative that will contribute to delivering the European medicines agencies network strategy to 2025, and the Commission Pharmaceutical Strategy to better tackle the challenges clinical trials face, such as the absence of EU impactful multi-state trials, a disharmony of regulatory requirements between Member States, resulting in slower trial authorisations, and high expenses to conduct trials.

The objectives of this initiative are to:

- Optimise the EU environment for clinical research in Europe;
- Strengthen clinical trials that deliver decision-making evidence for unmet medical needs, rare diseases, and vaccines and therapeutics for public health crises and

pandemics, ensuring support for HTA bodies as well as for academic and small-and-medium-enterprise (SME) sponsors;
- Heighten the impact of European clinical trials through coordinated scientific advice;
- Engage all stakeholders to proactively deliver inclusive patient-oriented medicines development and delivery across populations;
- Ensure a clear and unified European position on clinical trials in strategic matters at the international level; and
- Build capacity in all aspects of drug development and regulatory science through, amongst others, research collaboration and training with academia.

ACT EU will be co-led by the EC, the Heads of Medicines Agencies (HMA) and the EMA.

Read the full proposal here.

6. Health Technology Assessment

The *Regulation on Health Technology Assessment* (HTA), which entered into force in January 2022, introduced a permanent framework for joint work between EU Member States.

This work includes joint scientific consultations, the identification of emerging health technologies, and voluntary cooperation, as well as work on joint clinical assessments, and helps tackle a lack of transparency in methods in assessing said technology, thereby saving national HTA bodies and industry from duplicating their efforts.

Therefore, the legislation aims to ensure greater transparency and patient engagement in the assessment process by providing a permanent framework, allowing vital and innovative health technologies to be more widely available, and establishing common rules in order to meet the objectives of the Pharmaceutical Strategy for Europe.

While the implemention work started last year, the legislation does not become immediately applicable; a three-year transitional period has been set up. Furthermore, even though all medicines registered by the EMA will have to go through a joint clinical assessment, the management of health services, incl. pricing and reimbursement decisions, will remain with national governments. Last but not least, consultations do not have a legally binding effect.

It is important to note that patient-care organisations are meant to play a crucial role in the implementation as patients and patient experts will become more involved in the entire process. Not only will the Regulation facilitate access to innovative medicines, it will also address unmet medical needs.

Read more here.

7. European Health Data Space

In May 2022, the Commission launched the *European Health Data Space* (EHDS) as part of the European Health Union with the aim to empower citizens to control and utilise their health

data in their home country or in 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 fully comply with the EU's high data protection standards.

The EHDS is therefore built on three main pillars:

1) Strong system of data governance and rules for data exchange;
2) Data quality; and
3) Strong infrastructure and interoperability.

One of the most important elements of the EHDS is that it empowers citizens with full control over their data to obtain better healthcare across the EU, incl. immediate and easy access to their data in electronic form, free of charge, and the possibility to easily share these data with other health professionals in and across Member States to improve healthcare delivery.

Not only will citizens be in full control of their data, they will also be able to add information, rectify wrong data, restrict access to others, and obtain information on how their data are used and for which purpose.

As for the role of Member States, they will ensure that patient summaries, ePrescriptions, images and image reports, laboratory results, and discharge reports are issued and accepted in a common European format.

Furthermore, interoperability and security will become mandatory requirements, and all Member States have to appoint digital health authorities so that patients can share their data across borders via the cross-border digital infrastructure called MyHealth@EU.

The second and equally important aim of the EHDS is to improve the use of health data for research, innovation, and policymaking by creating a strong legal framework for said purposes. However, access to such data is neither automatic nor provided: researchers, companies, or institutions will first have to acquire a permit, which will only be granted if the requested data is used for specific purposes in closed, secure environments, and without revealing the identity of the individual.

Lastly, the new decentralised EU-infrastructure for secondary use (HealthData@EU) will be set up to support cross-border projects.

According to EURORDIS, "Overall, it promises to advance research, diagnosis, treatment and care for people with rare diseases through increased and meaningful data sharing."

In her remarks on May 3rd, Commissioner for Health and Food Safety, Stella Kyriakides, stated that the EHDS "will allow those for example with rare diseases to access doctors in other Member States. We have seen the value the European Reference Networks have brought to the treatment and care of patients with rare diseases. The European Health Data Space will help to further pool data, always in a safe way."

8. Cross-Border Healthcare Directive 2022 assessment

As readers may be aware, in May 2022, the EC published its third triennial assessment report as required by the *Directive 2011/24/EU on patients' rights in cross-border healthcare*. This directive, first adopted in 2021, sets out the conditions under which a patient may travel to another EU country to receive medical care and reimbursement. It covers healthcare costs, as well as the prescription and delivery of medications and medical devices.

The 2022 report acknowledges that the directive has facilitated access to cross-border care to some extent and that the European Reference Networks bring a real EU-added value. Nonetheless, the report also reveals the limited success of the directive to-date, as exemplified by the small number of EU citizens aware of and making use of possibilities offered by the directive, the disproportionate administrative burden, and continued uncertainty about costs abroad and reimbursement.

As a matter of fact, 11 years after the adoption of the directive, persisting barriers - such as long approval times, fragmented payment and reimbursement processes, and difficult access to clinical trials - limit access to excellence of care and innovative therapies by rare disease patients.

The EHC closely monitors cross-border healthcare and will keep its community informed about further developments.

Read more here.

9. Women's health

In December 2022, the European Public Health Alliance (EPHA) published the article "Gender inequalities and discrimination in rare diseases: a double threat to women's health and wellbeing". According to the EPHA, in rare diseases "gender inequalities are coupled with the rarity, complexity, degenerative and often life-threatening characteristics inherent to the definition of these conditions".

For women, in comparison with men, later diagnosis, symptom management, uptake, and care often result in rapid health deterioration, negatively impacting their quality of life, socioeconomic status, and mental health.

Furthermore, as more women tend to be caretakers of people living with a rare disease, this often intensive and time-consuming care means a higher risk of unemployment, vulnerability in the labour market, social exclusion, limited time for paid work and other responsibilities, incl. their own wellbeing.

"Further research is needed to identify and address the roots of discrimination, in economics and especially health. It is only through a comprehensive approach, encompassing all aspects of gender inequalities, that the EU can achieve the promotion of equality between men and women and become a true global leader in gender equality", says the EPHA.

10. Liver health

Some readers may be aware that the MEP Friends of the Liver Group (hereafter 'the Group'), coordinated by the European Association for the Study of the Liver (EASL), was launched on 15 March 2022. The group, originally initiated in 2015, was relaunched with new MEPs. Although only MEPs may serve on this structure, the group looks forward to collaborating with diverse stakeholders. The five core priorities of the group are:

1) Prevention, early detection, and care of liver cancer;
2) Alcohol consumption and healthy diet;
3) Non-alcoholic fatty liver disease (NAFLD);
4) Viral hepatitis; and
5) The fight against stigma.

One key area of action is the surveillance of non-communicable diseases (NCDs) in Europe. MEP Radan Kanev (ENVI Committee, Bulgaria, and EPP shadow reporter on the legislative proposal to extend the mandate of the ECDC) brought up the surveillance of NCDs at the EU level. This initiative is urging the EU to put in place systems to monitor NCDs and consolidate scientific knowledge. Such a monitoring system could be achieved by extending the ECDC's mandate to have it include NCDs.

The EHC is also directly engaged with EASL and its Foundation and will continue to monitor this work.

Read more here.

11. Mental and physical health

11.1. Healthier Together – EU Non-Communicable Diseases (NCDs) Initiative

Readers may recall that in December 2021, the EC launched *The Healthier Together – EU Non-Communicable Diseases (NCDs) Initiative* with the aim to support EU countries reduce the burden of NCDs by identifying and implementing effective policies and actions, whilst improving citizens' health and well-being and reducing health inequalities, as part of a strong Health Union. In 2022, €156 million, i.e., 20% of the annual EU4Health budget was dedicated to health promotion and disease prevention.

The initiative covers the 2022-2027 period and includes not only mental health and neurological disorders but also health determinants, cardiovascular diseases, diabetes, and chronic respiratory diseases.

The four priority areas are as follows:

- Supporting favourable conditions for mental health and increasing resilience, implementing mental-health-in-all policies;
- Promoting mental well-being and preventing mental health disorders;
- Improving timely and equitable access to high quality mental health services; and

- Protecting rights, enhancing social inclusion, and tackling stigma associated with mental health problems.

Read more here.

11.2. A Comprehensive Approach to Mental Health

In September 2022, in her yearly State of the Union speech, Commission President Ursula von der Leyen promised an EU mental health strategy for June 2023 under the name *"A Comprehensive Approach to Mental Health"* which will be an entirely new initiative. While no further details were disclosed, the strategy will most likely cover research, the availability of mental health professionals, and a setting up of a European Year of Mental Health. Mental health is also among the EHC's priorities and will therefore be futher monitored.

Read more here.

12. Social care

In September 2022, the EC presented the *European Care Strategy for Caregivers and Care Receivers* to ensure quality, affordable, and accessible care services across the EU and improve the situation for both care receivers and care providers, whether professionally or informally, incl. early childhood education and care, long-term care, fair working conditions, training, and improved work-life balance for care staff.

Increasing access to high-quality, timely, comprehensive, and affordable care services means that people who, as a result of old age, illness, and/or disability depend on help for everyday activities, can live with autonomy and dignity.

The Strategy would also increase the offer and mix of professional long-term care services (homecare, community-based care, and residential care), close territorial gaps in the access to long-term care, roll out accessible digital solutions in the provision of care services, and support informal carers, who are often women and relatives of care receivers, through training, counselling, psychological, and financial support. Lastly, it will mobilise adequate and sustainable funding for long-term care, including by using EU funds.

As for early childhood education and care, the Commission is proposing that Member States revise the targets to enhance women's labour market participation so that by 2030 at least:

- 50% of children below the age of three are in early childhood education and care; and
- 96% of children between the ages of three and the starting age for compulsory primary education are in early childhood education and care.

Read more here.

13. Looking forward

13.1. European Commission's 2023 work programme

On 18 October 2022, the Commission presented its *2023 work programme*, which sets out its plans for the coming year. In the field of health, the Commission mostly focuses on the medicines ecosystem.

The Commission will continue working on most of the initiatives mentioned in this review in the course of the year. In the second quarter of 2023, the Commission will publish a patent licensing package, including a framework for standard essential patent licensing and clear rules for compulsory patent licensing. Both initiatives will affect intellectual property rights around medicines and vaccines and ultimately patient access.

A compulsory licence, if issued by a government, authorises a third party to use a patented invention without the consent of the patent holder. The view is that in crises of health (such as the COVID-19 pandemic), environmental, nuclear or industrial emergencies, compulsory licensing is crucial for maximising access to medical technology and intellectual property (IP) as well as cross-border production and distribution of vaccines or other products.

The general objective is to create a less fragmented and better-suited compulsory licensing system for EU-wide crises of a health, environmental, nuclear or industrial nature. However, the legislation faces many critics. The pharmaceutical industry, represented by the European Federation of Pharmaceutical Industries and Associations (EFPIA), has officially expressed its concern about the decision, noting that it is a backward step in the industry's collective ability to tackle the COVID-19 pandemic and future global health threats.

In 2023, the Commission will further focus on the European Health Data Space (EHDS), the creation of which was recommended by the Conference on the Future of Europe as a key pillar of the European Health Union. A swift adoption and implementation of this initiative would strengthen the quality and continuity of healthcare and ensure citizens' rights in relation to their health data.

The Commission will respond to another proposal from the Conference on the Future of Europe with a comprehensive approach to mental health, a major societal issue brought into extra focus during the pandemic. The Commission will publish its approach in the second quarter of 2023. It will also continue to build a Union of Equality through a flagship initiative for the rights of persons with disabilities, proposing a European disability card ensuring the mutual recognition of disability status across all Member States. This legislative initiative, which includes an impact assessment, is scheduled for the fourth quarter of 2023.

In addition, by the end of the year, the Commission will have revised its framework for medicines. Announced in the pharmaceutical strategy, this initiative aims to revise the rules setting out the procedures for post-authorisation changes to a marketing authorisation for medicines for human use. The purpose of this non-legislative initiative is to make the lifecycle management of medicines more efficient.

Read more here.