EU Proposed Pharmaceutical Legislation: *Forecasting impacts*

Richard Price

Head of Policy

European Cancer Organisation

About the European Cancer Organisation

42 Member Societies

21 Patient Advocacy Groups

Working together to build consensus and achieve improvement in cancer care

To reduce the burden of cancer, improve outcomes and the quality of care for cancer patients, ***through multidisciplinarity and multiprofessionalism***

european cancer ORGANISATION

Our Members

Member SOCIETY OF THE EUROPEAN CANCER ORGANISATION

ESPEN The European Society for Clinical Nutrition and Metabolism

EADV EUROPEAN ACADEMY OF DERMATOLOGY & VENEREOLOGY

European Society of Endocrinology

CIRSE

EAPC VZW

ECL Association of European Cancer Leagues

EANM EUROPEAN ASSOCIATION OF NUCLEAR MEDICINE

eau European Association of Urology

efc EUROPEAN FEDERATION FOR COLPOSCOPY

EHA

European Hereditary Tumour Group

ESSM European Society for Sexual Medicine

EONS EUROPEAN ONCOLOGY NURSING SOCIETY

EORTC European Organisation for Research and Treatment of Cancer
The future of cancer therapy

ERS EUROPEAN RESPIRATORY SOCIETY every breath counts

ESO European School of Oncology Learning to care

ESHI MT EUROPEAN SOCIETY FOR HYBRID, MOLECULAR AND TRANSLATIONAL IMAGING

esdo european society of digestive oncology

ESTRO

EFIC EUROPEAN PAIN FEDERATION

EHNS European Head & Neck Society

EUSOMA European Society of Breast Cancer Specialists

EUSOBI european society of breast imaging

ESCP European Society of COLOPROCTOLOGY

ESGO European Society of Gynaecological Oncology

ESOI European Society of Oncologic Imaging

UNITED EUROPEAN GASTROENTEROLOGY ueg

ESOP

European Society of Pathology

ESSRF

E·P·C European Pancreatic Club Founded 1965

EUROPEAN SOCIETY OF SKIN CANCER PREVENTION EUROSKIN

esso THE EUROPEAN SOCIETY OF SURGICAL ONCOLOGY

eaun

IPOS INTERNATIONAL PSYCHO-ONCOLOGY SOCIETY

SIOG INTERNATIONAL SOCIETY OF GERIATRIC ONCOLOGY

MASCC Multinational Association of Supportive Care in Cancer WWW.MASCC.ORG

EFOMP

ipvs International Papillomavirus Society

OECI ORGANISATION OF EUROPEAN CANCER INSTITUTES EUROPEAN ECONOMIC INTEREST GROUPING

EASL The Home of Hepatology

european cancer ORGANISATION

Our Patient Advisory Committee

Patient ADVOCACY GROUP OF EUROPEAN CANCER ORGANISATION

Patient Advocates are at the heart of the European Cancer Organisation, with 21 patient advocacy groups

ALAN
Childhood Cancer International EUROPE
DIGESTIVE CANCERS EUROPE
MPNE
EUROPA UOMO
EUROPA DONNA
EURORDIS RARE DISEASES EUROPE
BTA INTERNATIONAL BRAIN TUMOUR ALLIANCE
INCA International Neuroendocrine Cancer Alliance
LEUKEMIA PATIENT ADVOCATES FOUNDATION
Lung Cancer Europe
LYMPHOMA COALITION EUROPE
THE MDS ALLIANCE
ELPA European Liver Patients' Association
MPE Myeloma Patients Europe
PANCREATIC CANCER EUROPE
Sarcoma Patients EuroNet
THYROID CANCER ALLIANCE
World Bladder Cancer Patient COALITION
YOUTH CANCER EUROPE
IKCC International Kidney Cancer Coalition

The Patient Advisory Committee is involved in all policy-making and activities, including the Focused Topic Networks

Our strategic pillars of activity

Pillar 1: Policy and Advocacy

- On behalf of our Member Societies, we strive to be a leading organisation in the development of oncopolicy and advocacy at the European level
- On behalf of our Member Societies, we are available as a resource to European institutions to convene and facilitate across disciplines, professions and with patient advocates
- We advocate on key policy issues, speaking with one voice on behalf of our Member Societies and patient groups, to enhance the impact of their expertise and experience

Pillar 2: Convenor and Facilitator around Focused Topics

- We act as the "federation" of the European cancer community - amplifying the important work of our member societies and Patient Advisory Committee.
- We convene our Member Societies, patient groups and other stakeholders for discussion and exchange, building consensus and co-ordinating activities, including through our Focused Topic Networks.
- We believe in collaboration, bringing together those who share our Mission and Vision to promote multidisciplinarity and multi-professionals for the benefit of patients, as well as advocating across Europe for our agreed policies.

Background: A growing political will for legislation change

Council conclusions on strengthening the balance in the pharmaceutical systems in the European Union and its Member States

(2016/C 269/06)

THE COUNCIL OF THE EUROPEAN UNION,

1. RECALLS that under Article 168 of the Treaty on the Functioning of the European Union, a high level of human health protection shall be ensured in the definition and implementation of all Union policies and activities, that Union action, which shall complement national policies, shall be directed towards improving public health, that the Union shall encourage cooperation between the Member States in the field of public health and, if necessary, lend support to their action, and fully respect the responsibilities of the Member States for the organization and delivery of health services and medical care and allocation of the resources to them;

2. RECALLS that under Article 168(4)(c) of the Treaty on the Functioning of the European Union, the European Parliament and the Council can, in order to meet common safety concerns, adopt measures setting high standards of quality and safety for medicinal products and devices for medical use;

3. RECALLS that under Article 4(3) of the Treaty on European Union, the Union and the Member States shall assist each other in carrying out tasks which flow from the Treaties, pursuant to the principle of sincere cooperation;

4. RECALLS that under Article 5(2) of the Treaty on European Union, the Union shall act only within the limits of the competences conferred upon it by the Member States in the Treaties to attain the objectives set out therein and that competences not conferred upon the Union in the Treaties remain with the Member States;

5. RECALLS that under Article 3(1)(b) of the Treaty on the Functioning of the European Union, the Union has exclusive competence in relation to the competition rules necessary for the functioning of the internal market for medicinal products;

6. STRESSES that it is fully Member States' competence and responsibility to decide which medicinal products are reimbursed and at what price and that any voluntary cooperation on pricing and reimbursement between Member States should remain Member States driven;

European Parliament
2014-2019

TEXTS ADOPTED

P8_TA(2017)0061

Options for improving access to medicines

European Parliament resolution of 2 March 2017 on EU options for improving access to medicines (2016/2057(INI))

The European Parliament,

– having regard to its position of 6 February 2013 on the proposal for a directive of the European Parliament and of the Council relating to the transparency of measures regulating the prices of medicinal products for human use and their inclusion in the scope of public health insurance systems[1],

– having regard to Article 168 of the Treaty on the Functioning of the European Union (TFEU), which lays down that a high level of human health protection should be ensured in the definition and implementation of all Union policies and activities,

– having regard to the Commission REFIT evaluation of Council Regulation (EC) No 953/2003 to avoid trade diversion into the European Union of certain key medicines (SWD(2016)0125),

– having regard to the obligations set out in Article 81 of Directive 2001/83/EC for the maintenance of an appropriate and continued supply of medicinal products,

– having regard to the Commission's Inception Impact Assessment[2] on the strengthening of EU cooperation on Health Technology Assessment (HTA),

– having regard to the HTA Network Strategy for EU Cooperation on Health Technology Assessment of 29 October 2014[3],

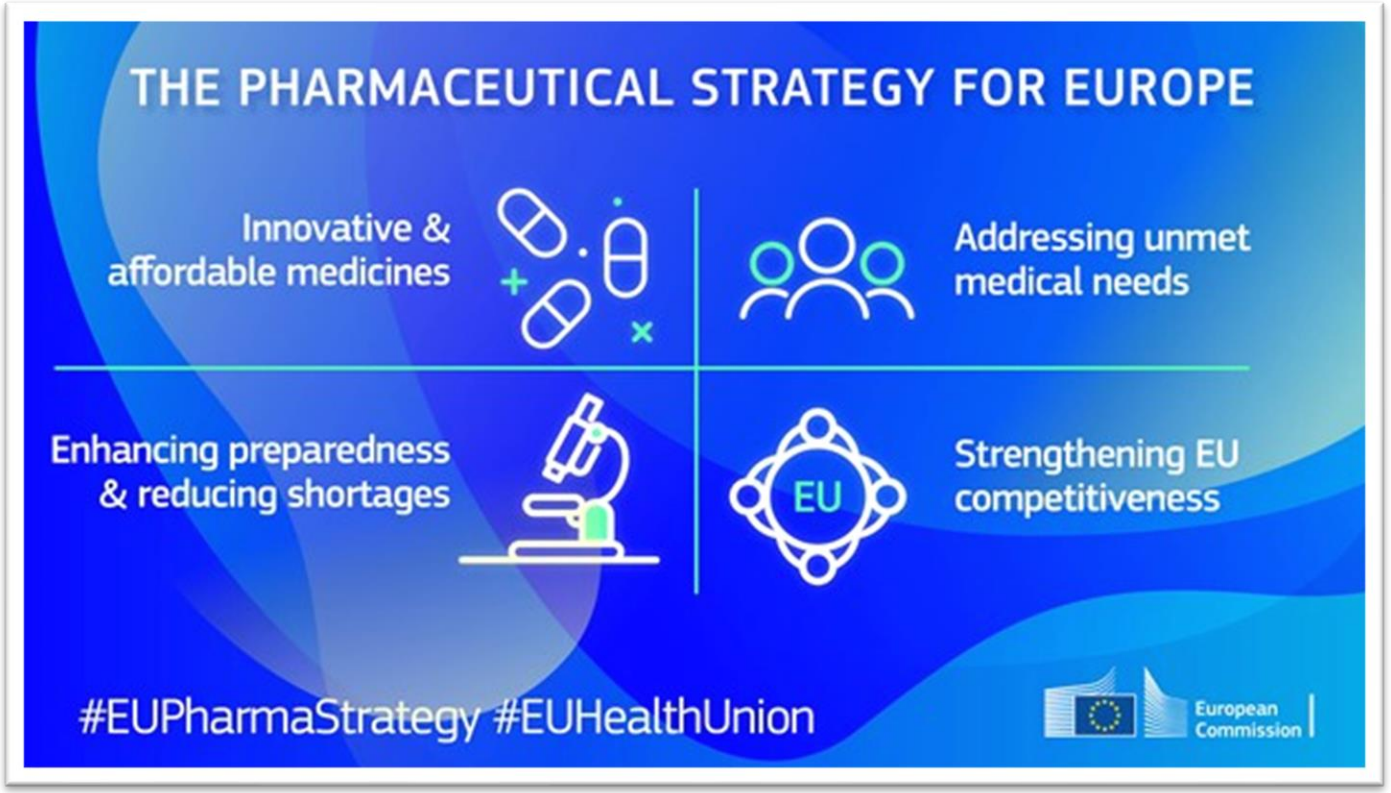
– having regard to the final report of the Commission's Pharmaceutical Sector Inquiry (SEC(2009)0952),

The Commission sets out its vision

November 2020

The Commission publishes its intention to pursue a comprehensive update of EU pharmaceutical regulation including:

- Examination of **changes to market exclusivity rules**
- Further **regulatory intervention to ameliorate medicines shortages**
- Use of pharmaceutical **legislation to help combat antimicrobial resistance**



PRESS RELEASE | 26 April 2023 | Brussels

European Health Union: Commission proposes pharmaceuticals reform for more accessible, affordable and innovative medicines

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Today, the Commission is proposing to revise the EU's pharmaceutical legislation - the largest reform in over 20 years - to make it more agile, flexible, and adapted to the needs of citizens and businesses across the EU. The revision will make **medicines more available, accessible and affordable**. It will support innovation and boost the **competitiveness** and attractiveness of **the EU pharmaceutical industry**, while promoting higher environmental standards. In addition to this reform, the Commission proposes a Council Recommendation to step up the **fight against antimicrobial resistance** (AMR).

The **challenges** this reform addresses are fundamental. Medicines authorised in the EU are still **not reaching patients quickly enough** and are not equally accessible in all Member States. There are significant gaps in addressing **unmet medical needs**, rare diseases and antimicrobial resistance (AMR). **High prices** for innovative treatments and **shortages** of medicines remain an important concern for patients and healthcare systems. In addition, to ensure that the EU remains an attractive place for investment and a world leader in the development of medicines, it needs to adapt its rules to the **digital transformation** and **new technologies**, whilst cutting red tape and simplifying procedures. Finally, the new rules need to address the **environmental impact** of medicine production in line with the objectives of the European Green Deal.

The revision includes proposals for a new Directive and a new Regulation, which revise and replace the existing pharmaceutical legislation, including the legislation on medicines for children and for rare diseases. It aims to achieve

What the proposals do

The proposed legislation will replace the following legislation:

- Directive 2001/83/EC, which is the primary EU law for human medicines.
- Regulation 726/2004, which establishes the EMA and governs the centralised procedure.
- Regulation 1901/2006 “Paediatric Regulation”.
- Regulation 141/2000/EC “Orphan Regulation”; and
- Regulation on advanced therapy medicinal products No 1394/2007 “ATMP Regulation”

Both the Paediatric and Orphan Regulation will be incorporated into the new Regulation and Directive.

Key features: Market exclusivity changes

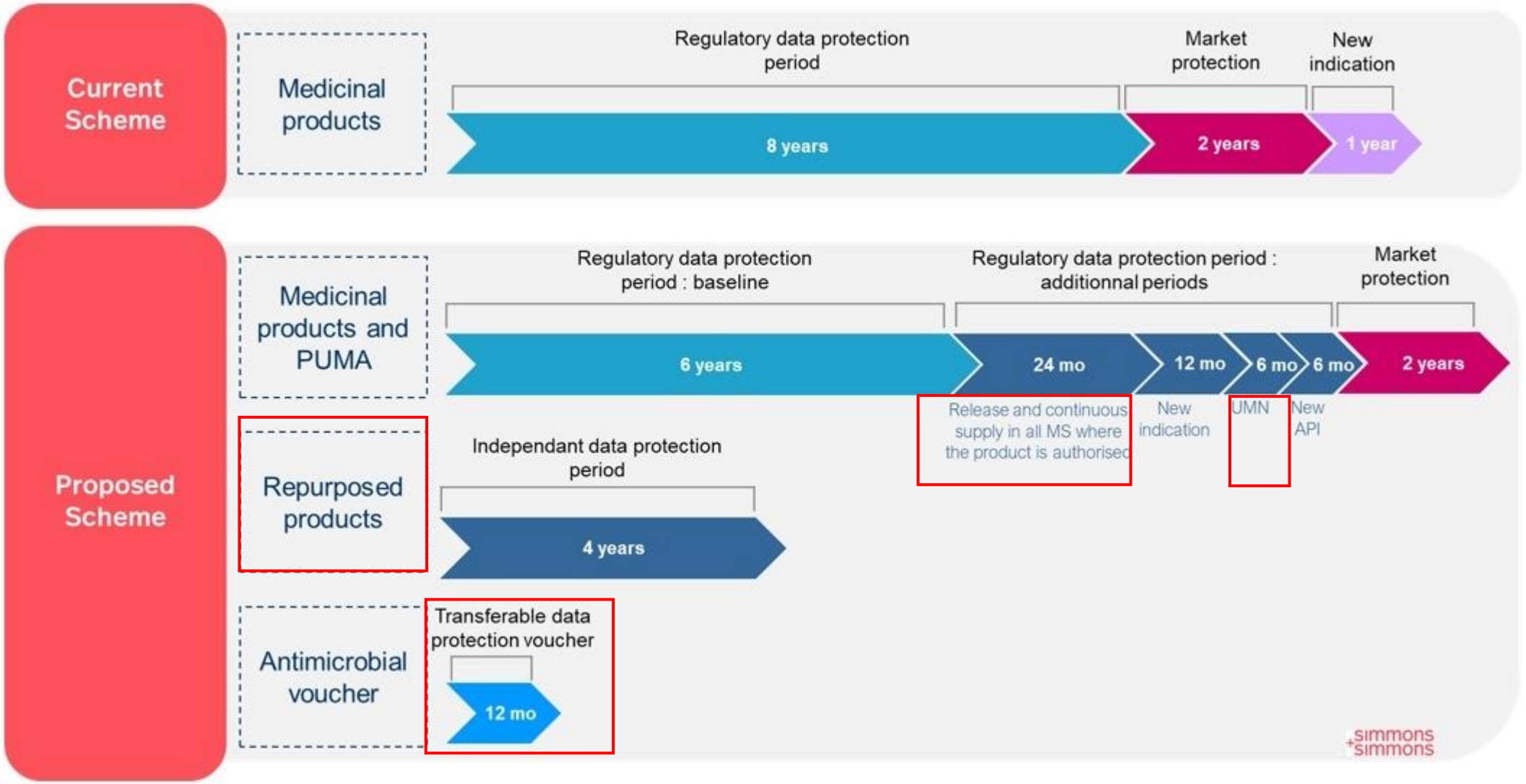


Image credit: Simmons & Simmons

Key features: Continuous supply prolongation

Article 82

Prolongation of the data protection period for medicinal products supplied in Member States

1. The prolongation of the data protection period referred to in Article 81(2), first subparagraph, point (a), shall only be granted to medicinal products if they are released and continuously supplied into the supply chain in a sufficient quantity and in the presentations necessary to cover the needs of the patients in the Member States in which the marketing authorisation is valid.

The prolongation referred to in the first subparagraph shall apply to medicinal products that have been granted a centralised marketing authorisation, as referred to in Article 5 or that have been granted a national marketing authorisation through the decentralised procedure, as referred to in Chapter III, Section 3.

2. To receive a prolongation referred to in Article 81(2), first subparagraph, point (a), the marketing authorisation holder shall apply for a variation of the relevant marketing authorisation.

The application for a variation shall be submitted between 34 and 36 months after the date when the initial marketing authorisation was granted, or for entities referred to in Article 81(2), first subparagraph, point (a), between 46 and 48 months, after that date.

The application for a variation shall contain documentation from the Member States in which the marketing authorisation is valid. Such documentation shall:

(a) confirm that the conditions set out in paragraph 1 have been satisfied in their territory; or

(b) waive the conditions set out in paragraph 1 in their territory for the purpose of the prolongation.

Key features: Unmet Medical need prolongation

Article 83

Medicinal products addressing an unmet medical need

1. A medicinal product shall be considered as addressing an unmet medical need if at least one of its therapeutic indications relates to a life threatening or severely debilitating disease and the following conditions are met:

   (a) there is no medicinal product authorised in the Union for such disease, or, where despite medicinal products being authorised for such disease in the Union, the disease is associated with a remaining high morbidity or mortality;

   (b) the use of the medicinal product results in a meaningful reduction in disease morbidity or mortality for the relevant patient population.

2. Designated orphan medicinal products referred to in Article 67 of [revised Regulation (EC) No 726/2004] shall be considered as addressing an unmet medical need.

3. Where the Agency adopts scientific guidelines for the application of this Article it shall consult the Commission and the authorities or bodies referred to in Article 162 of [revised Regulation (EC) No 726/2004].

Key features: Repurposing facilitation

Amendments introduced in the proposed Regulation

Article 48

Scientific opinion on data submitted from not-for-profit entities for repurposing of authorised medicinal products

1. An entity not engaged in an economic activity ('not-for-profit entity') may submit to the Agency or to a competent authority of the Member State substantive non-clinical or clinical evidence for a new therapeutic indication that is expected to fulfil an unmet medical need.

   The Agency may, at the request of a Member State, the Commission, or on its own initiative and on the basis of all available evidence make a scientific evaluation of the benefit-risk of the use of a medicinal product with a new therapeutic indication that concerns an unmet medical need.

   The opinion of the Agency shall be made publicly available and the competent authorities of the Member States shall be informed.

2. In cases where the opinion is favourable, marketing authorisation holders of the medicinal products concerned shall submit a variation to update the product information with the new therapeutic indication.

3. Article 81(2), point (c) of [revised Directive 2001/83/EC] shall not apply for variations under this Article.

Key features: Transferable exclusivity voucher for new antimicrobials

Article 40

Granting the right to a transferable data exclusivity voucher

1. Following a request by the applicant when applying for a marketing authorisation, the Commission may, by means of implementing acts, grant a transferable data exclusivity voucher to a 'priority antimicrobial' referred to in paragraph 3, under the conditions referred to in paragraph 4 based on a scientific assessment by the Agency.

2. The voucher referred to in paragraph 1 shall give the right to its holder to an additional 12 months of data protection for one authorised medicinal product.

3. An antimicrobial shall be considered 'priority antimicrobial' if preclinical and clinical data underpin a significant clinical benefit with respect to antimicrobial resistance and it has at least one of the following characteristics:

   (a) it represents a new class of antimicrobials;

   (b) its mechanism of action is distinctly different from that of any authorised antimicrobial in the Union;

   (c) it contains an active substance not previously authorised in a medicinal product in the Union that addresses a multi-drug resistant organism and serious or life threatening infection.

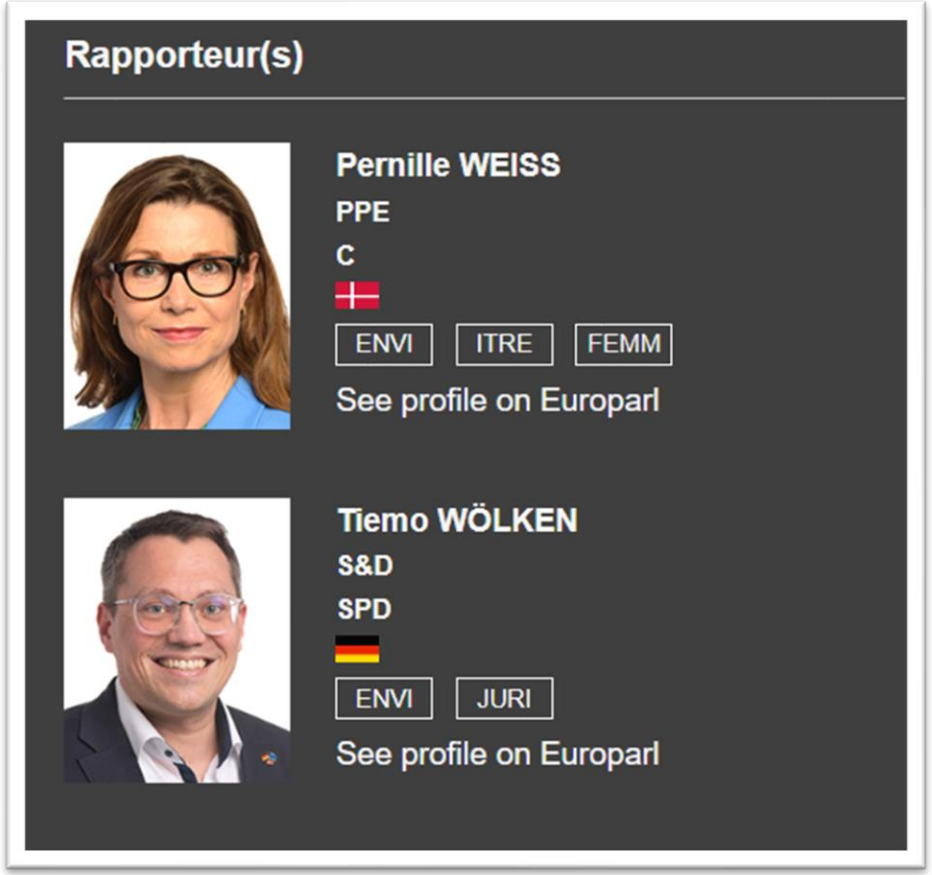
   In the scientific assessment of the criteria referred to in the first subparagraph, and in the case of antibiotics, the Agency shall take into account the 'WHO priority pathogens list for R&D of new antibiotics', or an equivalent list established at Union level.

Current scenario: with the Parliament and Council

Following the Commission proposal, the legislative proposals are now in the hands of **the co-legislators (the European Parliament and the Council).**

The lead Committee in the European Parliament for scrutinising the proposal is the **ENVI** (Environment and Public Health) Committee.

Pernille Weiss (EPP, Denmark) is the rapporteur on the Directive proposal, **Tiemo Wölken** (S&D, Germany) for Regulation proposal



Current scenario: Parliament deliberations to date

Pernille Weiss's draft report on the proposed Directive:

- Challenged the Commission's proposal to reduce the length of protection for clinical trial data from eight to six years, suggesting extending exclusivity to nine years without conditions, to provide certainty and incentives to drug developers.

Tiemo Wolken's draft report on the proposed Regulation:

- Deletes the inclusion of the transferable market exclusivity vouchers on the basis it is a non-transparent form of pricing
- Recommended the introduction of a new EU authority 'European Medicines Facility' (EMF) to drive research in areas less addressed by the private sector

Current scenario: Parliament deliberations to date

An ENVI vote on its positions is expected to take place on 11 March 2024, followed by a full plenary vote in April.

Ahead of 11 March meetings between political groups in the Parliament to achieve more alignment are ongoing

Should the file not be completed (i.e. agreed with the EU Council of Ministers) before the European Parliament elections in June 2024, the matter will return to the next Parliament as unfinished business from the previous Parliament, in a similar manner to the HTA regulation in 2019.

Current scenario: In the Council

Member State governments were unable to begin official scrutiny and deliberations on the Commission proposals until all relevant documents were translated into all languages.

This delay has meant serious consideration by Governments has only really commenced under the current Belgian Presidency of the EU Council of Ministers.

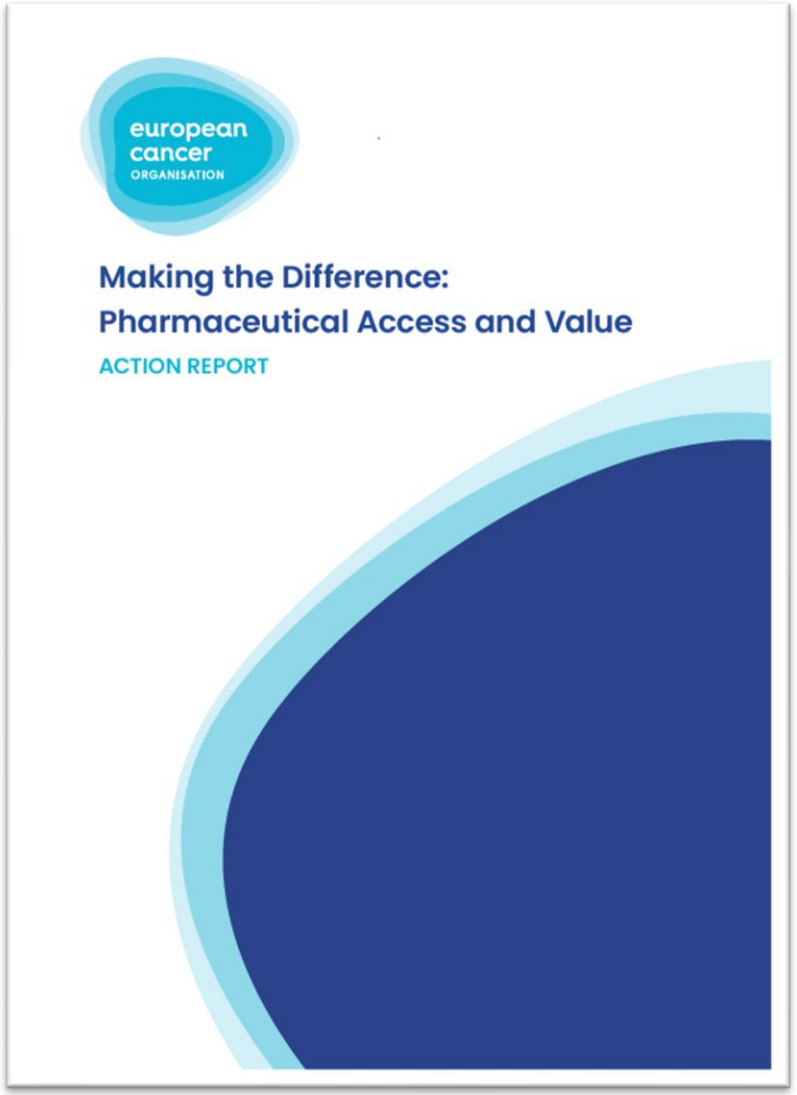
Alignment of countries on all matters considered to be unlikely before the European Parliament elections, but Belgian Health Minister hopeful some aspects may be agreed, including on medicine shortages.

ECO's perspective

The application of **a narrow a definition of 'unmet medical need' in EU pharmaceutical legislation could have unintended consequences**, such as disincentivising development of certain medicines.

Unmet medical needs in respect of cancer patients and survivors includes achieving safer treatments with reduced side effects of treatment. This can include acute and late toxicity and other side effects such as nausea, cognitive impairment and dysfunction, fatigue, pain and cachexia, as well endocrine, cardiac, metabolic and fertility issues.

As a core principle, any application of 'unmet medical need' requires **the confidence of patient communities** and needs to reflect what is most important to them.



TIME TO
ACCELERATE FOR EUROPE
TOGETHER AGAINST CANCER

www.europeancancer.org/manifesto