

# Open Public Consultation on the revision of the general pharmaceutical legislation

Fields marked with \* are mandatory.

## Introduction

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On 25 November 2020, the Commission published a Communication on a Pharmaceutical Strategy for Europe.

The Pharmaceutical Strategy identifies flagship initiatives and other actions to ensure the delivery of tangible results. As part of the implementation of the strategy, the Commission is evaluating the general pharmaceutical legislation<sup>1</sup> and assessing the impacts of possible changes in the legislation as described in the relevant [inception impact assessment](#).

This public consultation aims to collect views of stakeholders and the general public in order to support the evaluation of the existing general pharmaceutical legislation and the impact assessment of its revision. It builds further on the public consultation<sup>2</sup> conducted for the preparation of the pharmaceutical strategy for Europe. The replies to that consultation will be taken into account for the revision of the general pharmaceutical legislation. The present questionnaire should be seen as a continuation of that process.

In parallel, the legislation for medicines for rare diseases and children is being [revised](#) as well. Separate consultation activities have been carried out for that [revision](#).

This questionnaire is available in all EU languages and you can reply in any EU language. You can pause any time and continue later. You can download your contribution once you have submitted your answers.

A summary on the outcome of the public consultation will be published by the Commission services on the [‘Have your say’ portal](#).

We thank you for your participation.

*[1] Directive 2001/83/EC of the European Parliament and of the Council of 6 November 2001 on the Community code relating to medicinal products for human use (OJ L 311, 28.11.2001, p. 67)*

*Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency (OJ L 136, 30.4.2004, p. 1)*

*[2] A [report](#) analysing the results of the pharmaceutical strategy consultation was published in November 2020.*

## About you

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\* Language of my contribution

- Bulgarian
- Croatian
- Czech
- Danish
- Dutch
- English
- Estonian
- Finnish
- French
- German
- Greek
- Hungarian
- Irish
- Italian
- Latvian
- Lithuanian
- Maltese
- Polish
- Portuguese
- Romanian
- Slovak
- Slovenian
- Spanish
- Swedish

\* I am giving my contribution as

- Academic/research institution
- Business association
- Company/business organisation
- Consumer organisation
- EU citizen
- Environmental organisation
- Non-EU citizen

- Non-governmental organisation (NGO)
- Public authority
- Trade union
- Other

\* Which stakeholder group do you represent?

- Individual member of the public
- Patient or consumer organisation
- Healthcare professional
- Healthcare provider organisation (incl. hospitals, pharmacies)
- Healthcare payer
- Centralised health goods procurement body
- Health technology assessment body
- Academic researcher
- Research funder
- Learned society
- European research infrastructure
- Other scientific organisation
- Environmental organisation
- Pharmaceuticals industry
- Chemicals industry
- Pharmaceuticals traders/wholesalers
- Medical devices industry
- Public authority (e.g. national ministries of health, medicines agencies, pricing and reimbursement authorities)
- EU regulatory partner / EU institution
- Non-EU regulator / non-EU body
- Other (Please specify)

\* First name

Yannis

\* Surname

Natsis

\* Email (this won't be published)

yannis.natsis@epha.org

\* Organisation name

*255 character(s) maximum*

European Public Health Alliance (EPHA)

\* Organisation size

- Micro (1 to 9 employees)
- Small (10 to 49 employees)
- Medium (50 to 249 employees)
- Large (250 or more)

Transparency register number

*255 character(s) maximum*

Check if your organisation is on the [transparency register](#). It's a voluntary database for organisations seeking to influence EU decision-making.

18941013532-08

\* Country of origin

Please add your country of origin, or that of your organisation.

- |   |  |                                     |  |
|---|--|-------------------------------------|--|
| <input type="radio"/> Afghanistan         | <input type="radio"/> Djibouti           | <input type="radio"/> Libya         | <input type="radio"/> Saint Martin                     |
| <input type="radio"/> Åland Islands       | <input type="radio"/> Dominica           | <input type="radio"/> Liechtenstein | <input type="radio"/> Saint Pierre and Miquelon        |
| <input type="radio"/> Albania             | <input type="radio"/> Dominican Republic | <input type="radio"/> Lithuania     | <input type="radio"/> Saint Vincent and the Grenadines |
| <input type="radio"/> Algeria             | <input type="radio"/> Ecuador            | <input type="radio"/> Luxembourg    | <input type="radio"/> Samoa                            |
| <input type="radio"/> American Samoa      | <input type="radio"/> Egypt              | <input type="radio"/> Macau         | <input type="radio"/> San Marino                       |
| <input type="radio"/> Andorra             | <input type="radio"/> El Salvador        | <input type="radio"/> Madagascar    | <input type="radio"/> São Tomé and Príncipe            |
| <input type="radio"/> Angola              | <input type="radio"/> Equatorial Guinea  | <input type="radio"/> Malawi        | <input type="radio"/> Saudi Arabia                     |
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| <input type="radio"/> Antarctica          | <input type="radio"/> Estonia            | <input type="radio"/> Maldives      | <input type="radio"/> Serbia                           |
| <input type="radio"/> Antigua and Barbuda | <input type="radio"/> Eswatini           | <input type="radio"/> Mali          | <input type="radio"/> Seychelles                       |

- Argentina
- Armenia
- Aruba
- Australia
- Austria
- Azerbaijan
- Bahamas
- Bahrain
- Bangladesh
- Barbados
- Belarus
- Belgium
- Belize
- Benin
- Bermuda
- Bhutan
- Bolivia
- Bonaire Saint Eustatius and Saba
- Bosnia and Herzegovina
- Botswana
- Bouvet Island
- Brazil
- British Indian Ocean Territory
- British Virgin Islands
- Brunei
- Ethiopia
- Falkland Islands
- Faroe Islands
- Fiji
- Finland
- France
- French Guiana
- French Polynesia
- French Southern and Antarctic Lands
- Gabon
- Georgia
- Germany
- Ghana
- Gibraltar
- Greece
- Greenland
- Grenada
- Guadeloupe
- Guam
- Guatemala
- Guernsey
- Guinea
- Guinea-Bissau
- Guyana
- Haiti
- Malta
- Marshall Islands
- Martinique
- Mauritania
- Mauritius
- Mayotte
- Mexico
- Micronesia
- Moldova
- Monaco
- Mongolia
- Montenegro
- Montserrat
- Morocco
- Mozambique
- Myanmar/Burma
- Namibia
- Nauru
- Nepal
- Netherlands
- New Caledonia
- New Zealand
- Nicaragua
- Niger
- Nigeria
- Sierra Leone
- Singapore
- Sint Maarten
- Slovakia
- Slovenia
- Solomon Islands
- Somalia
- South Africa
- South Georgia and the South Sandwich Islands
- South Korea
- South Sudan
- Spain
- Sri Lanka
- Sudan
- Suriname
- Svalbard and Jan Mayen
- Sweden
- Switzerland
- Syria
- Taiwan
- Tajikistan
- Tanzania
- Thailand
- The Gambia
- Timor-Leste

- Bulgaria
- Burkina Faso
- Burundi
- Cambodia
- Cameroon
- Canada
- Cape Verde
- Cayman Islands
- Central African Republic
- Chad
- Chile
- China
- Christmas Island
- Clipperton
- Cocos (Keeling) Islands
- Colombia
- Comoros
- Congo
- Cook Islands
- Costa Rica
- Côte d'Ivoire
- Croatia
- Cuba
- Curaçao
- Cyprus
- Heard Island and McDonald Islands
- Honduras
- Hong Kong
- Hungary
- Iceland
- India
- Indonesia
- Iran
- Iraq
- Ireland
- Isle of Man
- Israel
- Italy
- Jamaica
- Japan
- Jersey
- Jordan
- Kazakhstan
- Kenya
- Kiribati
- Kosovo
- Kuwait
- Kyrgyzstan
- Laos
- Latvia
- Niue
- Norfolk Island
- Northern Mariana Islands
- North Korea
- North Macedonia
- Norway
- Oman
- Pakistan
- Palau
- Palestine
- Panama
- Papua New Guinea
- Paraguay
- Peru
- Philippines
- Pitcairn Islands
- Poland
- Portugal
- Puerto Rico
- Qatar
- Réunion
- Romania
- Russia
- Rwanda
- Saint Barthélemy
- Togo
- Tokelau
- Tonga
- Trinidad and Tobago
- Tunisia
- Turkey
- Turkmenistan
- Turks and Caicos Islands
- Tuvalu
- Uganda
- Ukraine
- United Arab Emirates
- United Kingdom
- United States
- United States Minor Outlying Islands
- Uruguay
- US Virgin Islands
- Uzbekistan
- Vanuatu
- Vatican City
- Venezuela
- Vietnam
- Wallis and Futuna
- Western Sahara
- Yemen

- Czechia
- Lebanon
- Saint Helena  
Ascension and  
Tristan da Cunha
- Zambia
- Democratic  
Republic of the  
Congo
- Lesotho
- Saint Kitts and  
Nevis
- Zimbabwe
- Denmark
- Liberia
- Saint Lucia

The Commission will publish all contributions to this public consultation. You can choose whether you would prefer to have your details published or to remain anonymous when your contribution is published. **For the purpose of transparency, the type of respondent (for example, 'business association, 'consumer association', 'EU citizen') country of origin, organisation name and size, and its transparency register number, are always published. Your e-mail address will never be published.** Opt in to select the privacy option that best suits you. Privacy options default based on the type of respondent selected

### \* Contribution publication privacy settings

The Commission will publish the responses to this public consultation. You can choose whether you would like your details to be made public or to remain anonymous.

**Anonymous**

Only organisation details are published: The type of respondent that you responded to this consultation as, the name of the organisation on whose behalf you reply as well as its transparency number, its size, its country of origin and your contribution will be published as received. Your name will not be published. Please do not include any personal data in the contribution itself if you want to remain anonymous.

**Public**

Organisation details and respondent details are published: The type of respondent that you responded to this consultation as, the name of the organisation on whose behalf you reply as well as its transparency number, its size, its country of origin and your contribution will be published. Your name will also be published.

I agree with the [personal data protection provisions](#)

## Looking back

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As mentioned in the [Inception Impact assessment](#), the revision aims to tackle the following problems:

- Unmet medical needs and market failures for medicines other than medicines for rare diseases and children;
- Unequal access to available and affordable medicines for patients across the EU;
- The current legislative framework may not be fully equipped to respond quickly to innovation;
- Inefficiency and administrative burden of regulatory procedures;
- Vulnerability of supply of medicines, shortages of medicines;
- Environmental challenges and sustainability;
- Any other issues, which might emerge from the evaluation.

## Q1 In your opinion, are there any other issues that should be addressed in this revision?

*800 character(s) maximum*

It should incentivise the timely generation of quality comparative data on the benefits- harms of new drugs and high-risk devices both before and after their market entry, profiting patients, clinicians, and healthcare systems. Valuable information on treatment optimization is currently missing at the time of marketing authorization. The review should aim to cover these evidence gaps by encouraging confirmatory trials to generate comparative data. Compounding is one of the tools that allow hospitals to alleviate certain shortages of drugs of major therapeutic interest, incl. when a product is not marketed anymore. Directive 2001/83 / EC should not limit the possibility for hospital pharmacies to compound medicines in shortage for all patients who need them.

## Q2 How has the legislation performed in terms of the following elements?

	Very well	Well	Moderately	Poorly	Very poorly	Don't know
1. Fulfilling its public health protection mission for patients and society.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Promoting the development of new medicines, especially for unmet medical needs.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Enabling timely development of medicines at all times, including during crises.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Enabling timely authorisation, including scientific evaluation, of medicines in normal times.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Enabling timely authorisation, including scientific evaluation during crises.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Adapting efficiently and effectively to technological and scientific advancements and innovation.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Ensuring medicines are of high quality, safe and effective.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

8. Addressing the competitive functioning of the market to support affordability.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Ensuring the availability of generic <sup>3</sup> and biosimilar <sup>4</sup> medicines.  <i>[3] "Generic" is a copy of a medicine based on simple or chemical molecules.</i> <i>[4] "Biosimilar" is a copy of a medicine based on biological molecules.</i>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
10. Ensuring that new medicines are timely available to patients in all EU countries.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
11. Ensuring that medicines stay on the market at all times and that there are no shortages.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
12. Ensuring that authorised medicines are manufactured, used and disposed of in an environmentally friendly manner.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
13. Ensuring that the EU system for development, authorisation and monitoring of medicines, including its rules and procedures, is understandable and easy to navigate.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
14. Attracting global investment for medicine innovation in the EU.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, including positive or unintended effects of the legislation, or would you like to justify your replies?

*800 character(s) maximum*

Many of the centrally authorized medicines are not marketed in numerous EU countries because companies do not deem it to be sufficiently profitable although they do benefit from the central EU marketing authorisation. In other words, it is left up to the companies' marketing, business strategies and launches' sequencing to define if/when a product will be marketed in a country. At the same time, widely used, long-established, safe, efficient and cheap medicines are artificially withdrawn from national markets for commercial reasons. The absence of systematic evaluation of the added value of new medicines is another drawback along with the increasing impact of pharmaceuticals on the overall national healthcare budgets

## Looking forward

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This section reflects on possible solutions to address the problems identified in the inception impact assessment mentioned in the previous section.

Your contribution will help us in defining the way forward.

## UNMET MEDICAL NEEDS

One of the aims of the strategy is to stimulate innovation and breakthrough therapies, especially in areas of ‘unmet medical need’.

Regulators, health technology assessment experts and representatives of bodies responsible for reimbursing or paying for medicines (‘payers’) are discussing a definition or a set of principles for ‘unmet medical needs’<sup>5</sup> in order to achieve the objectives of the general pharmaceutical legislation. The discussions reveal different perceptions of what is an ‘unmet medical need’. Convergence on this key concept should facilitate the design of clinical trials, generation of evidence and its assessment, and the quick availability on the market of these products and ensuring that innovation matches the needs of patients and of the national health systems.

The purpose of this question is to identify elements that are important in defining what is unmet medical need and in which areas of unmet medical need innovation should be stimulated.

*[5] Please note that a similar discussion is taking place in the context of medicines for rare diseases and for children. The concept of ‘unmet needs’ in the context of rare diseases and children might be slightly differentiated compared to ‘unmet needs’ in the context of the general pharmaceutical legislation.*

### Q3 How important are the following elements for defining ‘unmet medical needs’?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Seriousness of a disease.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Absence of satisfactory treatment authorised in the EU.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. A new medicine has major therapeutic advantage over existing treatment(s).	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Lack of access for patients across the EU to an authorised treatment.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Other (please specify).	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined elements, or would you like to justify your replies?

*800 character(s) maximum*

Impact on quality of life of the patient should be amongst the criteria. The quantification of the uncertainty is important too. A definition would help as we currently get new products with limited evidence, unsure benefits and exuberant prices. The definition should take into account broader societal elements too. We should ensure the highest level of evidence possible pre- and post launch, identify current comparators and targeted patient subpopulations. The early on dialogue and collaboration between all relevant stakeholders will enable the clarification of the timely evidence generation needs. Electronic health records are an important tool. Quality-of-Life and patient-reported outcomes must be part of the criteria for 'significant benefit'.

## **INCENTIVES FOR INNOVATION**

The general pharmaceutical legislation guarantees the pharmaceutical innovator, typically a company, regulatory data and market protection for its new medicinal product. This data protection makes sure that another pharmaceutical company cannot re-use the proprietary data of the innovator for 8 years. Market protection makes sure that a generic or biosimilar medicine cannot be marketed until 10 years after authorisation. This dual protection shields a pharmaceutical innovator from generics or biosimilars on the market for 10 years. This protection is part of the EU system of incentives for innovation. The EU regime of [intellectual property protection](#) provides an additional protection coverage but is beyond the scope of this questionnaire and the revision of the general pharmaceutical legislation.

#### Q4 What do you think of the following measures to support innovation, including for ‘unmet medical needs’?

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. The current data and market protection periods for innovative medicines: 10 years of market protection, and 8 years of data protection.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Provide different data and market protection periods depending on the purpose of the medicine (i.e. longer period of protection in areas of unmet medical need).	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Reduce the data and market protection periods to allow earlier access for generic and biosimilar medicines to the market.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Introduce new types of incentives <sup>6</sup> on top of the existing data and market protection for medicines addressing an ‘unmet medical need’.						
<i>[6] Examples of new incentives are a transferable exclusivity voucher or a priority review voucher. A transferable exclusivity voucher would give the legal right to extend the protection time period of any other patented medicinal product, in exchange for the successful regulatory approval of a specified medicine for unmet medical need (e.g. an antibiotic). The voucher would be transferable or saleable, and may impact the turnover and profitability levels of other products in a developer's portfolio. A priority review voucher gives priority to the assessment of the application of the medicine in question or another medicine in the applicant's portfolio.</i>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
5. Early scientific support and faster review/authorisation of a new promising medicine for an unmet medical need.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Public listing of priority therapeutic areas of high unmet medical need to support product development by providing incentives.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Require transparent reporting from companies about their research and development costs and public funding as a condition to obtain certain incentives.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
8. Other (please specify)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

*800 character(s) maximum*

Different levels of incentives (and market exclusivity duration) should be foreseen depending on the UMN that the new medicine would cover, as long as the maximum of 8y and 10y is not extended. A list of priorities based on the EC evaluation published in August 2020 would be helpful to steer proven innovation where it is needed. Access to incentives should be dependent on transparent reporting of R&D costs considering that EU patients already pay the R&D costs through taxes. To increase access to medicines: (i) allow generics and biosimilars into the market once the period of protection is over and any attempt of hampering this should be financially punished; (ii) facilitate authorization of treatments developed in academic settings for non-commercial purposes.

## **ANTIMICROBIAL RESISTANCE<sup>7</sup>**

Antimicrobial resistance (AMR) is the ability of microorganisms (such as bacteria, viruses, fungi or parasites) to survive and grow over time and no longer respond to medicines making infections harder to treat and increasing the risk of infections, severe illness and death. Antimicrobials include antibiotics, which are substances that fight bacterial infections. Overprescribing, overuse and inappropriate use of antibiotics are key drivers of AMR, leading to harmful health outcomes. The question below is intended to collect opinions on both the incentives for the development of new antimicrobials as well as possible option on their prudent use.

*[7] [amr\\_2017\\_action-plan.pdf \(europa.eu\)](#).*

**Q5 Should there be specific regulatory incentives for the development of new antimicrobials while taking into account the need for more prudent use and if so what should they be?**

*1000 character(s) maximum*

Patent term extensions, premium pricing strategies (increasing the cost of the medicine, for example, by claiming that a higher price reflects its “value”), transferable market exclusivity (in exchange for developing a new antibiotic, a company is allowed to extend a monopoly on a different medicine that can or is earning blockbuster revenues) should all be avoided. Data exclusivity extensions (providing companies with extra years of exclusive right over clinical trial data, which prevents a drug regulatory authority from referring to an originator’s clinical trial data to approve a generic competitor) should also be avoided.

## **FUTURE PROOFING: ADAPTED, AGILE AND PREDICTABLE REGULATORY FRAMEWORK FOR NOVEL PRODUCTS**

Novel products and innovative solutions continue to challenge the understanding of a “medicinal product” with low volume, and cutting-edge products (e.g. medicines combined with self-learning artificial intelligence) becoming a new reality. ‘Bedside’ manufacture of more individualised medicines changes the way medicines are produced. There are classification and interplay challenges with other medical products,

such as medical devices and substances of human origin, or related to the combination of clinical trials with in vitro diagnostics/medical devices and medicines. In addition, certain cell-based advanced therapy medicines<sup>8</sup> are offered in hospital settings and are exempted from aspects of the pharmaceutical legislation. These developments offer possibilities for novel promising treatments and new ways of authorising and monitoring medicines but they are also testing the limits of the current regulatory system. They need to be addressed to unfold their potential while safeguarding the principles of high quality, safety and efficacy of medicines.

Digital transformation is affecting the discovery, development, manufacture, evidence generation, assessment, supply and use of medicines. Medicines, medical technologies and digital health are becoming increasingly integral to overarching therapeutic options. These include systems based on artificial intelligence for prevention, diagnosis, better treatment, therapeutic monitoring and data for personalised medicines and other healthcare applications.

*[8] Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells. They offer ground-breaking new opportunities for the treatment of disease and injury.*

**Q6 How would you assess the following measures to create an adapted, agile and predictable regulatory framework for novel products?**

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Create a central mechanism in close coordination with other concerned authorities (e.g. those responsible for medical devices, substances of human origins) to provide non-binding scientific advice on whether a treatment/product should be classified as a medicine or not.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Make use of the possibility for 'regulatory sandboxes' <sup>9</sup> in legislation to pilot certain categories of novel products/technologies.  <i>[9] Some very innovative solutions fail to see the light of day because of regulations which might be outdated or poorly adapted for fast evolving technologies. One way to address this is through regulatory sandboxes. This enables innovative solutions not already foreseen in regulations or guidelines to be live-tested with supervisors and regulators, provided that the appropriate conditions are in place, for example to ensure equal treatment. Regulatory sandboxes provide up-to-date information to regulators and supervisors on, and experience with, new technology, while enabling policy experimentation. See COM(2020) 103 final.</i>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Create adaptive regulatory frameworks (e.g. adapted requirements for authorisation and monitoring with possibility to adjust easily to scientific progress) for certain novel types of medicines or low volume products (hospital preparations) in coherence with other legal frameworks (e.g. medical devices and substances of human origin <sup>10</sup> ) and respecting the principles of quality, safety and efficacy.  <i>[10] Substances that are donated by humans such as blood, plasma, cells, gametes, tissues and organs and are applied as therapy. Some substances of human origin can also become starting materials to manufacture medicines.</i>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

5. Introduce an EU-wide centrally coordinated process for early dialogue and more coordination among clinical trial, marketing authorisation, health technology assessment bodies, pricing and reimbursement authorities and payers for integrated medicines development and post-authorisation monitoring.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Other (please specify)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

*800 character(s) maximum*

The current regulatory system is flexible enough. Creating new loopholes i.e. expedited approval pathways should be dealt with caution. Regulators should inform patients of uncertainties in drug benefits at the time of approval. An EU-wide centrally coordinated process for early dialogue on clinical trials with marketing authorisation applicants, HTA bodies, payers, and patients for an integrated medicine development and post-authorisation monitoring is welcome. Patient representatives should contribute to the design of the clinical trials for marketing authorisation application. Real-world data should be clearly regulated and monitored. A robust collection of high-quality data coming from patient-reported outcomes is important to better evaluate the added value of a medicine.

**Q7. Do you think that certain definitions and the scope of the legislation need to be updated to reflect scientific and technological developments in the sector (e.g. personalised medicines, bedside manufacturing, artificial intelligence) and if so what would you propose to change?**

*1000 character(s) maximum*

It is critical that the EC rewards and acknowledges the investments and the resources that charities, academia, and non-profit research centers dedicate for cancer research. Given the growing importance and increasing use of the following, it will be important to include key terms and definitions relevant to the use of RWD-RWE, such as: real-world data, real-world evidence, disease registry, patient registry, registry-based study, registry-based clinical trial. In addition, the revised legislation shall identify legal basis for the adoption of guidance (or implementing acts) to establish data standards, data quality framework, metadata and data protection.

## **REWARDS AND OBLIGATIONS RELATED TO IMPROVED ACCESS TO MEDICINES**

Some medicines and therapies do not always reach patients in all EU countries, so patients in the EU still have different levels of access to medicines, depending on where they live. Even if a medicine received an EU-wide authorisation, companies are currently not obliged to market it in all EU countries. A company may decide not to market its medicines in, or decide to withdraw them from, one or more countries. This can be due to various factors, such as national pricing and reimbursement policies, size of the population and level of wealth, the organisation of health systems and national administrative procedures. Smaller markets in particular face challenges for availability and supplies of medicines.

**Q8 How would you assess the following measures to improve patient access to medicines across the EU?**

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules which provide no obligation to market medicines in all EU countries.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
2. Require companies to notify their market launch intentions to regulators at the time of the authorisation of the medicine.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Introduce incentives for swift market launch across the EU.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Allow early introduction of generics in case of delayed market launch of medicines across the EU, while respecting intellectual property rights.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Require companies to place – within a certain period after authorisation – a medicine on the market of the majority of Member States, that includes small markets.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Require companies withdrawing a medicine from the market to offer another company to take over the medicine.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Introduce rules on electronic product information to replace the paper package leaflet.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
8. Introduce harmonised rules for multi-country packages of medicines.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Other (please specify).	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

If new incentives were to be introduced they should not undermine the sustainability of the healthcare systems, nor affordability of products. Transparency around the costs to develop & place a product on the market should be fostered to promote fair prices and boost competition. A stricter interpretation of Article 14, namely that availability in just a single Member State would not suffice, could provide an additional incentive to speed up the market launch of authorised medicinal products. Publication of data on "marketing status" could increase pressure regarding the effective fulfillment of supply needs. An obligation to market in all countries should include a deadline by which a product has to be launched in all MS either automatically or on request.

### ENHANCE THE COMPETITIVE FUNCTIONING OF THE MARKET TO ENSURE AFFORDABLE MEDICINES

The affordability of medicines has implications for both public and household finances. It poses a growing challenge to pay for medicines in the majority of Member States. Often, innovative medicines have higher prices, while there are growing concerns among stakeholders about the real-life effectiveness of some medicines and related overall costs. This puts the budgetary sustainability of health systems at risk, and reduces the possibilities for patients to have access to these medicines. Generics and biosimilars<sup>11</sup> of medicines which no longer benefit from intellectual property protection (off-patent medicines) may provide accessible and affordable treatments. They also increase the availability of alternative treatment options for patients. They may also increase competition between available medicines. However, experience shows that there are still barriers for medicines entering the EU market, including for generics or biosimilars.

[11] "Generics" are copies of medicines based on simple or chemical molecules; "biosimilars" are copies of medicines based on biological molecules.

### Q9 In your view, to what extent would the following measures support access to affordable medicines?

	To a great extent	To a certain extent	No change	Very little	Not at all	Don't know
1. Maintain the current rules.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
2. Stimulate earlier market entry through a broader possibility to authorise generics /biosimilars despite ongoing patent protection ('Bolar exemption') <sup>12</sup> .	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<p>[12] The Bolar exemption allows companies to conduct research on patent protected medicines under the condition that it is with a view to apply for a marketing authorisation for a generic.</p>						

3. Create a specific (regulatory) incentive for a limited number of biosimilars that come to the market first.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Introduce an EU-wide scientific recommendation on interchangeability for specific biosimilars.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Introduce other, non-legislative measures, such as joint procurement to reinforce competition while addressing security of supply and environmental challenges.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Other (please specify).	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

*800 character(s) maximum*

Introducing a broad transparency obligation, in application of the transparency resolution of WHA 2019, would promote access to affordable medicines in the EU. Health systems do not have access to information on the actual costs of health products, incl. investments in R&D and the share of public funding. EU member states negotiate prices in isolation, in competition with each other, and without knowing the actual prices (net of discounts) of drugs negotiated by other countries. Remove existing patent linkage mechanisms that link marketing authorisation or P&R decisions for generic medicines to the status of the patent of the reference product.

## REPURPOSING OF MEDICINES

Repurposing is the process of identifying a new use for an established medicine in a disease or condition other than that it is currently authorised for. Repurposing of older (off-patent) medicines constitutes an emerging and dynamic field of medicines development, often led by academic units and medical research charities, with the potential for faster development times and reduced costs as well as lower risks for companies. This is because repurposing commonly starts with substances that have already been tested and many have demonstrated an acceptable level of safety and tolerability. The objective is to identify the opportunities and address any regulatory burdens to facilitate repurposing of off-patent, affordable medicines.

**Q10 What measures could stimulate the repurposing of off-patent medicines and provide additional uses of the medicine against new diseases and medical conditions? Please justify your answers.**

*1000 character(s) maximum*

Current regulatory pathways do not foresee submission of data by parties that are not intending to be a MAH, however third parties 'champions' (not for profit) should be allowed to directly connect with the regulator/HTA for joint clinical assessment if no MAH has interest in adding a new indication on the label of an existing off-patent drug.  
The current STAMP Pilot project for drug repurposing is limited to Scientific Advice (SA) from the regulator.

Firstly, it should be mandatory that the SA is joined with HTA (EUnetHTA21). Secondly, when there is UMN and strong clinical evidence based on published data or trials aligned with SA, a new 'public interest' label extension pathway should be created to enable Type II variation to handle cases where there is no MAH interested and a champion exists. Alternatively, EMA could come up with a "scientific opinion" under article 5 (3) as happened for dexamethasone resulting in "good off-label use" practice.

## **SECURITY OF SUPPLY OF MEDICINES**

Shortages of medicines and the vulnerabilities in the pharmaceutical supply chain continue to be concerns in the EU. Shortages of medicines can have serious impacts on patient care. Under the current pharmaceutical legislation, pharmaceutical companies and wholesalers must, within the limits of their responsibilities, ensure a continued supply of medicines once they are placed on the market in the EU. Companies must also notify national authorities at least two months before an expected shortage or planned market withdrawal.

**Q11 What is your view on the following measures to ensure security of supply of medicines in the EU?**

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
2. Earlier reporting of shortages and market withdrawals to national authorities in a common format.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Companies to have shortage prevention plans.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Companies to have safety stocks.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Monitoring of supply and demand at national level.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Introduce a shortage monitoring system at EU level.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Require companies to diversify their supply chains, in particular the number of key suppliers of medicines and components.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
8. Companies to provide more information to regulators on their supply chain.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Introduce penalties for non-compliance by companies with proposed new obligations.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
10. EU coordination to help identify areas where consolidation in the supply chain has reduced the number of suppliers.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
11. Other (please specify)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

800 character(s) maximum

It is important to have transparency in the status of shortages by product and country, to identify at-risk products and products with very low volumes or prices and set a common list of essential medicines. It is significant to have transparency over API sources. Stockpiling needs to be guaranteed (either general or targeted stockpiling of 'at-risk' or essential medicines) along with coordinated "best use" of safety stocks. The notification obligation must also apply to potential shortages. Market withdrawals must be notified at least one year in advance. Duration of security stocks must be proportionate to the average duration of shortages and must be accompanied by an obligation of transparency of available stocks. Shortage prevention plans must be public.

### QUALITY AND MANUFACTURING

Medicines manufactured for the EU market must comply with the principles and guidelines of good manufacturing practice (GMP). GMP describes the minimum standard that a medicines manufacturer must meet in their production processes. GMP requires that medicines are of consistent high quality, are appropriate for their intended use and meet the requirements of the marketing authorisation or clinical trial authorisation.

### Q12 What is your opinion of the following measures to ensure manufacturing and distribution of high quality products?

	Very adequate	Adequate	Neutral	Less adequate	Not adequate	Don't know
1. Maintain the current rules.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Strengthen manufacturing and oversight rules.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Adapt manufacturing rules to reflect new manufacturing methods.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Include selected environmental requirements for manufacturing of medicines in line with the one health approach on antimicrobial resistance <sup>13</sup> .	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<i>[13] The one-health approach is a holistic and multi-sectorial approach to addressing antimicrobial resistance since antimicrobials used to treat infectious diseases in</i>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

<i>animals may be the same or be similar to those used in humans.</i>						
5. Increase Member State cooperation and surveillance of the supply chain in the EU and third countries.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Strengthen and clarify responsibilities of business operators over the entire supply chain on sharing information on quality, safety and efficacy.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Other (please specify).	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

*800 character(s) maximum*

- As many APIs come from Asia, it is essential to have a clear overview of the steps of the pharmaceutical pathway, from raw ingredients to final packaging. Indeed, if only step is missing, the product does not reach the patient.
- It is important to increase transparency of the supply in EU to avoid unfair competition among MSs and encourage collaboration;
- It is essential to maintain an open strategic autonomy to avoid protectionist measures, even beyond healthcare.
- An increased local production of pharmaceuticals should be carefully assessed considering the impact on the economy, environment, health, labor market.
- It is critical to clarify accountability and responsibility of the actors involved and to enforce adequate measures should supply duties not be fulfilled.

## ENVIRONMENTAL CHALLENGES

While access to pharmaceuticals is a priority, it is also important that the environmental impacts of those pharmaceuticals are as low as possible. The environmental risk assessments (ERAs) is currently not taken into account in the overall benefit/risk analysis which influences the delivery of a marketing authorisation (MA) of a medicine. ERA can influence risk management measures. Yet, ERA results are not decisive in the MA process.

**Q13 How would you assess the following measures to ensure that the environmental challenges emerging from human medicines are addressed?**

	Very important	Important	Fairly important	Slightly important	Not important	Don't know
1. Maintain the current rules.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
2. Strengthen the environmental risk assessment during authorisation of a medicine, including risk mitigation measures, where appropriate.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Harmonize environmental risk assessment by national regulators, including risk mitigation measures.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Increase information to the health care professionals and the general public about the assessment of environmental risks of medicines.	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Allow companies to use existing data about environmental risks for authorisations of a new medicine to avoid duplicating tests.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Other (please specify).	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Is there any other aspect you would like to mention, for example on the potential economic, social, environmental or other impacts of the outlined measures, or would you like to justify/elaborate your replies?

*800 character(s) maximum*

The impact of pharmaceuticals on the environment is critical because human beings, animals, and environment are interconnected and a 'one-health approach' is needed in all policies. Nevertheless, it is also important to support pharmaceutical companies with limited resources to renovate their buildings and machineries to reduce pollution. It is fair to set requirements but it is also important to place stakeholders in the position to change. It is of utmost relevance to analyse the economic, social, economic consequences should the number of pharmaceutical production sites increase in the next years. Ethical considerations play also an important role in this conversation.

**Q14 Is there anything else you would like to add that has not been covered in this consultation?**

*900 character(s) maximum*

Importance of independent evidence based information to medical doctors, instead of information provided by medical sales reps. Data and market protection should be linked to conditions, such as placing on the market of a pharmaceutical product on most/all MS, at a fair price. Compulsory license is an important tool to protect the balance between the interests of the owner of the IP on the one hand and society on the other. During the 8y period of data exclusivity, a compulsory license cannot function, because a firm that wants to make use of the compulsory license needs access to the data. Therefore, an exception must be incorporated in European data-exclusivity law to enable the granting of compulsory licences. Develop alternative ways to (i) incentivise and award medicine development (eg delinkage) and (ii) ensure R&D models result in affordable products.

**Q15 In case you would like to share a document that substantiates your replies, please upload it below (optional).**

Only files of the type pdf,txt,doc,docx,odt,rtf are allowed

**1fcd0529-b553-4706-8458-544ab6b94621/epha-recommendations-unleashing-meaningful-innovation-through-regulatory-reform.pdf**

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