

Consultation response

On the revision of the general pharmaceutical legislation



AmCham EU speaks for American companies committed to Europe on trade, investment and competitiveness issues. It aims to ensure a growth-orientated business and investment climate in Europe. AmCham EU facilitates the resolution of transatlantic issues that impact business and plays a role in creating better understanding of EU and US positions on business matters. Aggregate US investment in Europe totalled more than €3 trillion in 2020, directly supports more than 4.8 million jobs in Europe, and generates billions of euros annually in income, trade and research and development.

Open Public Consultation on the revision of the general pharmaceutical legislation

Fields marked with * are mandatory.

Introduction

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 legislation¹ 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 consultation² 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

* 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)

If other, please specify:

Trade association

* First name

Cathrine

* Surname

Bärtel

* Email (this won't be published)

CBA@amchameu.eu

* Organisation name

255 character(s) maximum

American Chamber of Commerce to the European Union (AmCham EU)

* 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.

5265780509-97

* 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 |
| <input type="radio"/> Anguilla | <input type="radio"/> Eritrea | <input type="radio"/> Malaysia | <input type="radio"/> Senegal |

- Antarctica
- Antigua and Barbuda
- 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
- Estonia
- Eswatini
- 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
- Maldives
- Mali
- 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
- Serbia
- Seychelles
- 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

- British Virgin Islands
- Brunei
- 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
- Guyana
- Haiti
- 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
- Niger
- Nigeria
- 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
- The Gambia
- Timor-Leste
- 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

- Curaçao
- Cyprus
- Czechia
- Democratic Republic of the Congo
- Denmark
- Laos
- Latvia
- Lebanon
- Lesotho
- Liberia
- Rwanda
- Saint Barthélemy
- Saint Helena
- Ascension and Tristan da Cunha
- Saint Kitts and Nevis
- Saint Lucia
- Western Sahara
- Yemen
- Zambia
- Zimbabwe

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

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

- Modernise the pharmaceutical regulatory environment in Europe to account for the next generation of medicines and vaccines
- Maintain a robust framework of incentives that specifically tackles high unmet needs, for example against AMR
- Recognise the complexity of medicinal product development and avoid reduction of incentives coupled with increasing obligations
- Guarantee strong IP protections to entice long-term global investment in European R&D capabilities
- Champion novel approaches to clinical trial design and support the EMA and national authorities to modernise and increase uptake of cloud-based systems that enable real time-sharing between industry and regulators
- Preserve open global supply chains for medicines, in collaboration with international partners and stakeholders

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 type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Enabling timely authorisation, including scientific evaluation during crises.	<input type="radio"/>	<input checked="" type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Ensuring the availability of generic ³ and biosimilar ⁴ 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 checked="" type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input 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 type="radio"/>	<input checked="" 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

AmCham EU aims to be a key partner in ensuring the revision of the EU pharmaceutical legislation continues to provide an environment for the biopharmaceutical sector to flourish and contribute to overall EU competitiveness. As companies invested in Europe, we would like to see a situation where the EU can become a competitive actor on the global stage who recognises the complexity of medicinal product development, avoids reducing incentives and increasing obligations. Maintaining a system that rewards innovation and attracts both investment and new technologies would play a key role towards this goal. We also feel that the Commission should work to make healthcare systems more resilient by promoting the uptake of innovation and multi-winner tenders.

Looking forward

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'⁵ 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
5. Other (please specify).	<input checked="" 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

We understand the Commission's intention to define novel and flexible approaches to tackle UMN, but would caution against a narrow definition. The EU risks having fewer medicines or indications eligible for incentives, as this won't increase investment or development output. Challenges around the development of novel antibiotics are clear examples of how more, not less, incentives are needed. In this context, defining the concept of UMN may be a challenging and fundamentally subjective exercise. A patient-centred UMN approach that considers the severity and burden of disease on patients, families and caregivers should be preferred. We recommend that any definition of UMN entails sufficient level of flexibility and is based on a holistic set of criteria beyond the ones listed above.

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 checked="" type="radio"/>	<input type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
4. Introduce new types of incentives ⁶ 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Early scientific support and faster review/authorisation of a new promising medicine for an unmet medical need.	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
8. Other (please specify)	<input checked="" 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

Developing products is a complicated process with high failure rates. Any methodology put forward to capture R&D costs is unlikely to fully capture the true investment of industry. It also opens up a risk of prioritising expensive R&D programmes, not high-value products. Proposals tying incentives to R&D transparency for a single product perpetuate a mindset that aims to reduce healthcare costs. RDP, including ME and IP, are the most important incentives for the industry. A robust and predictable regulatory framework is needed to promote investment and undertake a long and uncertain development process. Complementary incentives into underserved areas can be effective drivers to prioritise high unmet needs. But they should add to the current framework, not erode it.

ANTIMICROBIAL RESISTANCE⁷

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

A risk-based approach and innovation is essential for the revitalisation of the antimicrobial pipeline. Such an approach will attract substantial private investments, which are required to address the development of antimicrobial R&D. Here, Transferable Exclusivity Extension (TEE) at EU level could be an essential approach to create traction for more investment. It could incentivise investment at the necessary scale and be aligned with the value that novel antimicrobials would bring to Europeans and the MS, all while preserving antimicrobial stewardship. Moreover, this approach would be complementary to EU and national initiatives, which includes incentives such as the Innovative Medicines Initiative (IMI) and the framework within the EU Health Emergency Preparedness and Response (HERA).

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 medicines⁸ 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 groundbreaking 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Make use of the possibility for 'regulatory sandboxes' ⁹ 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 ¹⁰) 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 type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Other (please specify)	<input checked="" 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

Regulatory pilots in a 'sandbox' environment would provide the opportunity to test the pharmaceutical framework for the development of new cutting-edge products. A Complex Clinical Trial (CCT) pilot programme would facilitate use of CCT design approaches in drug development, promote innovation and allow more timely and iterative regulatory advice. Participation of Clinical Trials Facilitation and Coordination Group experts is critical to facilitate consistent scientific advice and evaluation by the EMA Scientific Advice Working Party and national clinical trial assessors. We also ask that the Commission works with industry to provide more clarity on the application of the sandboxes for various types of products.

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

Updating definitions and the scope of the legislation needs careful consideration to avoid unintended consequences. Adjustments to the scope of the legislation reduce oversight, or an expanded role for bedside manufacture may have an impact on manufacturing quality or safety. The existing legislation on ATMPs provides a solid framework. However, harmonised guidelines would be needed to clarify the scope and requirements of the Hospital Exemption. Particularly, there is a need for a clear definition of 'non-routine preparation' to support a harmonised understanding of how specific quality standards and pharmacovigilance requirements should be met. It would be desirable to harmonise the GMO requirements for medicines that contain GMOs such as ATMPs. Other definitions for consideration; importation, drug shortages and company. The latter should be clarified as to whether it is an affiliate or a legal entity. It should also be considered if parallel traders are also defined as a 'company'.

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
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1. Maintain the current rules which provide no obligation to market medicines in all EU countries.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
6. Require companies withdrawing a medicine from the market to offer another company to take over the medicine.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
7. Introduce rules on electronic product information to replace the paper package leaflet.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
8. Introduce harmonised rules for multi-country packages of medicines.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Other (please specify).	<input checked="" 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 forecasted immediate negative economic impact expected by the EU Commission from the additional obligations it intends to introduce won't contribute to achieving the goal of harnessing Europe's industry for the improvement of healthcare systems. It overlooks the roles that countries play in the P&R processes and that marketing authorisation holders only have limited say on their product launch in a MS. The causes of unequal access are multifactorial and require a holistic approach. In the case of complex technologies (eg ATMPs), significant infrastructures need to be put in place, which makes it challenging for some companies to launch in most of the MS. IPR are key incentives for innovation and undermining them won't improve access and will erode the EU's ability to develop medicines.

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 biosimilars¹¹ 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Stimulate earlier market entry through a broader possibility to authorise generics /biosimilars despite ongoing patent protection ('Bolar exemption') ¹² . [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.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
6. Other (please specify).	<input checked="" 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

EU-wide scientific recommendations on biosimilar interchangeability that pertain to pharmacy level substitution should apply an appropriate scientific standard to interchangeability determinations. Competition plays a role in relation to affordability, but a level playing field must be ensured. Plans to harmonise the 'Bolar exemption' shouldn't be broadened to de facto (pre-) commercial activities. A key issue is the many disagreements on moment of 'patent expiry'; an early resolution mechanism is needed. This clarity could help with incentives for biosimilars. This inducement could be based upon lessons from US, which show that the 180 days exclusivity for the first approved small molecule drug works well. Joint procurement should be pursued as an emergency tool, not a standard mechanism

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

Repurposing of established medicines for new indications could contribute to the UMN. This would, however, require a revision of the significant disincentives that are inbuilt into the current legislative framework. This includes, but is not limited to, the pricing and reimbursement systems which do not take into account the high level of investment needed for repurposed medicines to reap returns on their investment in developing them. Furthermore, the possibility of cross-label use by competing medicinal products, which is part of the new indication, limits the effect of any separate protection and its overall commercial appeal. The authorisation of a new indication also includes a list of additional responsibilities and liabilities for the market authorisation (MA) holder. Minimising any additional burdens should be a voluntarily measure and, where appropriate, in partnership with other stakeholders.

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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Earlier reporting of shortages and market withdrawals to national authorities in a common format.	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
5. Monitoring of supply and demand at national level.	<input checked="" type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
8. Companies to provide more information to regulators on their supply chain.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Introduce penalties for non-compliance by companies with proposed new obligations.	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
11. Other (please specify)	<input checked="" 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

Supply chain flexibility and resilience should be achieved as part of a global and risk-based approach in collaboration with international partners and stakeholders. More harmonisation across MS on import/export requirements and format would help achieving security of supply. Transparency of stock held at e.g. manufacturer level, will not alleviate any issues. Given the fragmented real time multi-stakeholder environment, this may not be an effective approach. Adequate supply also depends on the demand. The individual MS should have an EU-wide approach to ensure supply and allow the manufacturers to keep EU-wide flexibility. Manufacturers should have the right to manage their supply chains to be able to release products for a specific MS and cover the specific needs.

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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Strengthen manufacturing and oversight rules.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Adapt manufacturing rules to reflect new manufacturing methods.	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

<p>4. Include selected environmental requirements for manufacturing of medicines in line with the one health approach on antimicrobial resistance¹³.</p> <p><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 animals may be the same or be similar to those used in humans.</i></p>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
<p>5. Increase Member State cooperation and surveillance of the supply chain in the EU and third countries.</p>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<p>6. Strengthen and clarify responsibilities of business operators over the entire supply chain on sharing information on quality, safety and efficacy.</p>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<p>7. Other (please specify).</p>	<input checked="" 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

- Strengthen the oversight by regulators, in particular towards stakeholders later in the supply chain such as agents, brokers, distributors and wholesalers (not towards manufacturers where there is currently sufficient oversight).
- Consider interplay with environmental requirements covered by legislations other than health.

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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Strengthen the environmental risk assessment during authorisation of a medicine, including risk mitigation measures, where appropriate.	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3. Harmonize environmental risk assessment by national regulators, including risk mitigation measures.	<input checked="" type="radio"/>	<input 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 type="radio"/>	<input checked="" 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
6. Other (please specify).	<input checked="" 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

AmCham EU members are committed to reduce carbon footprints and underline a need for discussion. We recommend to maintain the current rules and to:

- Strengthen the dialogue between EMA & ECHA on established processes and oversight;
- Modernise Environment-Risk-Assessment guidelines;
- Establish recognition procedures of MS decisions ;
- Enable a balanced risk communication/educational outreach program to healthcare professionals and the public on medicines in the environment;
- Allow companies to use existing data on environmental risks for authorisations of a new medicine share in the costs of any additional testing and monitoring conducted as part of eco-pharmacovigilance (EPS);
- Introduce incentives for applying the 3Rs principle (replacement, refinement and red. animal use).

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

900 character(s) maximum

We are concerned that the current approach is not fully aligned with the approach and objective of the Industrial Strategy: using the green and digital transformations to empower industry and small businesses and ensure that all policies are innovation-conducive. In 2021, 46% of companies active in pharmaceutical R&D are based in the US, with just 19% in the EU. Similarly, some 55% of medicines globally are developed in the US life sciences ecosystem. (PharmaIntelligence, 'Pharma R&D Annual Review 2021', 16). As companies invested in Europe, we would like to see in a situation where the EU ceases to be at a comparative disadvantage with the US, and where innovation is seen as an investment, not a cost. For Europe to recover from the pandemic, investment in healthcare is essential as it drives innovation and research, creates jobs and ensures competition.

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

Contact

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