SIOP Europe
the European Society for Paediatric Oncology

SIOPE Toolkit

EU Pharmaceutical legislation: Public consultation

Deadline: Wednesday 8 November midnight CEST

INDIVIDUAL & FURTHER ORGANISATIONAL INPUT NEEDED FROM AS MANY AS POSSIBLE

Following the publication for the EU Commission's Proposal of the revision of the EU Pharmaceutical legislation was launched on 26 April 2023, the SIOPE and CCI-E **issued a position paper on the proposal** that you may further analyse.

Now your feedback on the EU Commissions Proposal of the Revision of the EU Pharmaceutical Legislation is needed.

Your individual responses and spreading the information to your network is key as

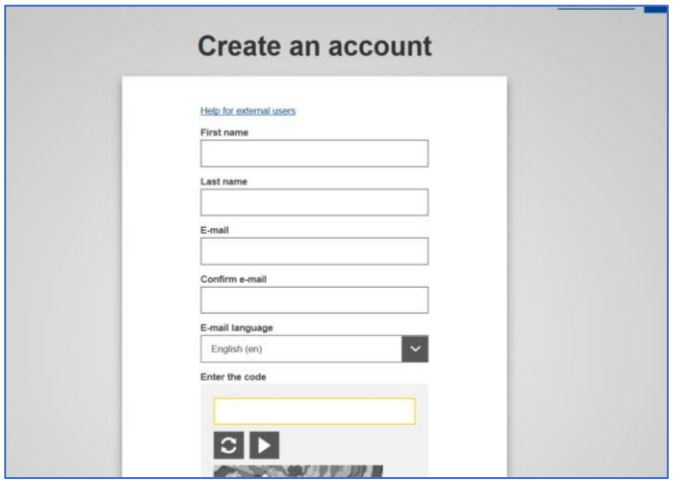
legislators will take into account the number of replies.

N.B. All feedback received will be summarised by the European Commission and presented to the European Parliament and Council with the aim of feeding into the EU Pharmaceutical legislative debate.

GUIDANCE ON HOW TO PROVIDE YOUR INPUT

STEP 1. Create an account on EU Health Policy Platform (required to be able to submit comment)

1a. Go to: https://webgate.ec.europa.eu/cas/eim/external/register.cgi **1b**.
Fill in the form



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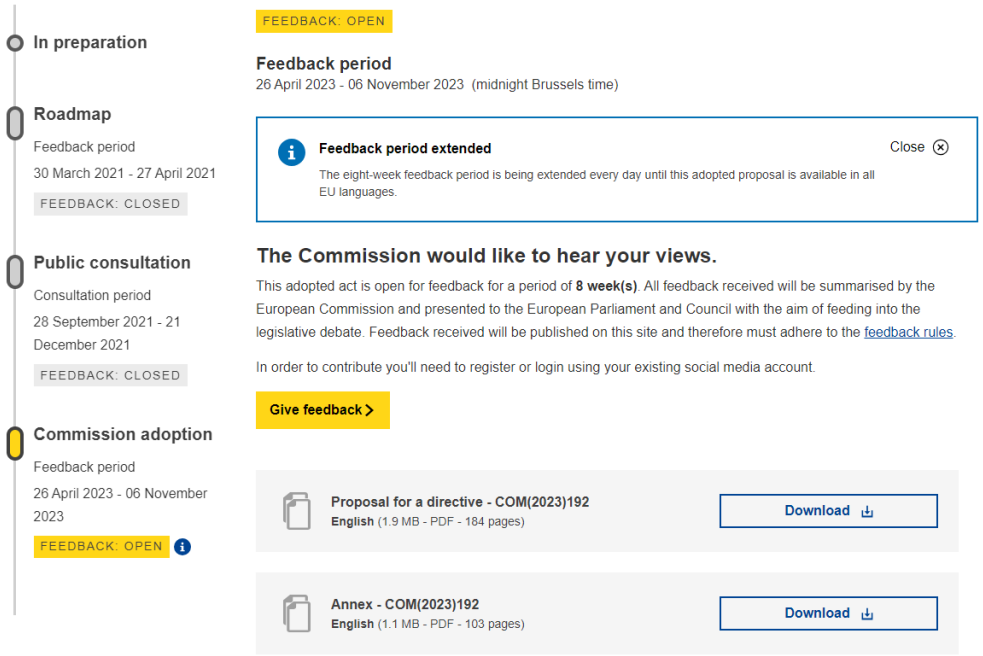
1c. Create password (activation link will be sent to your email address)

Confirmation email on newly created account may take up to 24 hours to receive!

STEP 2: Access the EU Pharmaceutical legislation – Public consultation

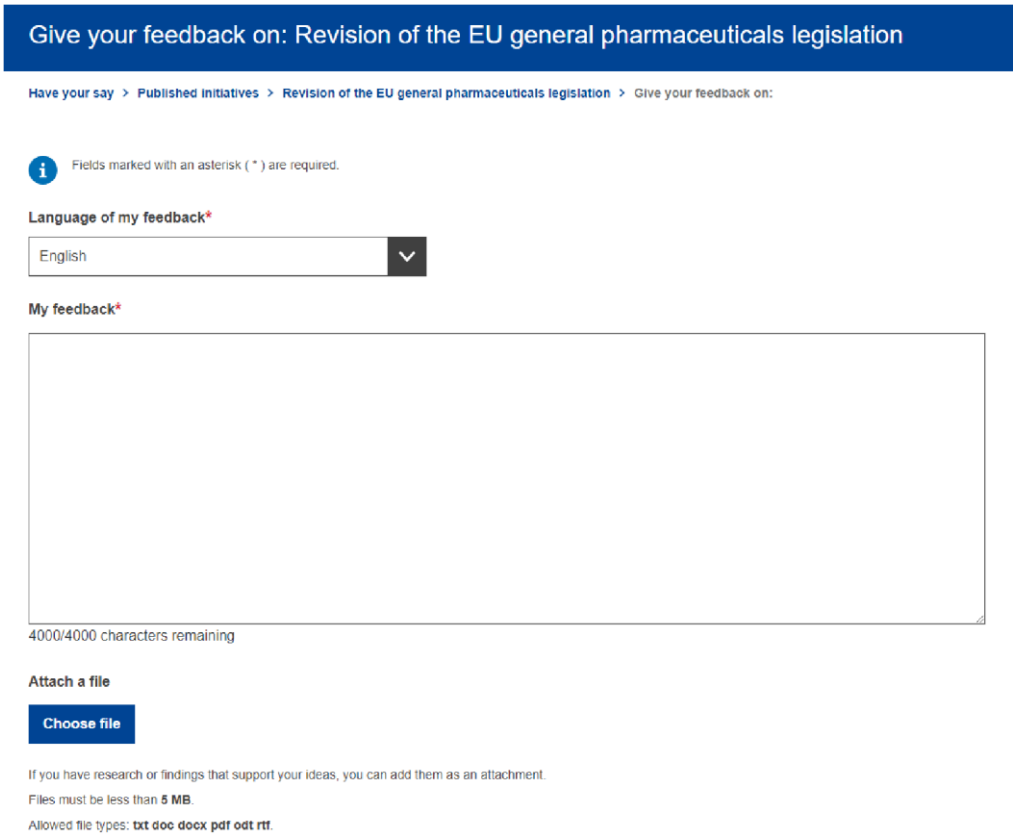
2a. Go to:

https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12963-Revision-of-the-EUgeneral-pharmaceuticals-legislation_en
Scroll to the bottom of the page where written 'Commission adoption'



2b. Click 'Give feedback' (to view the consultation and acquaint further)

2c. After clicking 'Give feedback' you will be asked to log in your account on EU Health Policy Platform and later directed to this website page: https://ec.europa.eu/info/law/better-regulation/have-yoursay/initiatives/12963-Revision-of-the-EU-general-pharmaceuticalslegislation/addFeedback_en?p_id=32050059 *(see below as well)*



STEP 3: Provide your input

The consultation is addressed to individual citizens, patients, and carers replying in their personal capacity as well as health professionals and organisations involved in the topic of access to medicines.

Type of consultation: Open comment (4000 characters, including spaces).

Language of your feedback: Preferably in your native language.

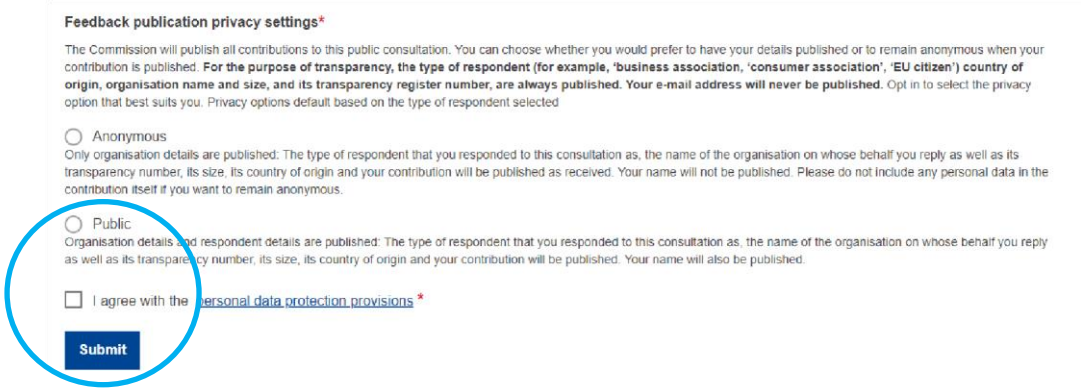
How to respond to the consultation: SIOP Europe has prepared below on the next page a concept note that you may use as the orientation. We also encourage you to give examples of the issues you are facing in your country.

DRAFT SIOPE RESPONSE IS ENCLOSED BELOW FOR YOUR GUIDANCE

In addition, next to your response you are also allowed to enclose a PDF document for which we offer you the possibility to download and include SIOPE & CCI-E joint position paper on the revision of the EU Pharmaceutical Legislation: **European Paediatric Cancer Community Proposal for Improvement of the EU Revised Pharmaceutical Legislation**

After your complete the response, please submit your personal details and select the privacy settings as suggested below. You must press the “submit button” to complete the consultation.

Are non-EU citizens welcome to respond to the consultation? Yes, they are very welcome to respond as the European Commission will be considering the opinions from the non-EU European countries and countries outside Europe.



SIOPE DRAFT RESPONSE: EU PHARMA LEGISLATION PUBLIC CONSULTATION

Below written are selected topics from the proposal for the revision of the EU Pharmaceutical legislation which require immediate attention from the EU institutions. You may choose from the list all or a couple of most pressing ones and further amplify them with your personal experiences in your native language. SIOPE & CCI-E plead for the listed topics to be either retained or amended in the proposal for the revision of the EU Pharmaceutical legislation and your voice can be instrumental to cement the needs of childhood cancer community.

The SIOPE draft response is based on the joint position paper by SIOPE and CCI-E: **'European Paediatric Cancer Community Proposal for Improvement of the EU Revised Pharmaceutical Legislation' -> click here**

SIOPE Guide to respond to the public consultation:

(3929 out of 4000 characters)

1. Unmet Medical Needs (UMN) and High Unmet Medical Needs (high UMN)

We welcome the introduction of important concepts of UMN and high UMN in the proposal for revision of the EU Pharmaceutical legislation. In the field of paediatric cancer, off-label use of anti-cancer medicine is very common due to lack of specific paediatric cancer drugs. This leads to a vast amount of side effects of currently available treatments.

We argue that investment in novel medicines overall reduces the financial burden of governments. New drugs that bear lower toxicity levels would reduce long term side effects of paediatric cancer patients and enable them to be productive members of society later in life. For the young patient, acute toxic prevents him or her to attend school and to develop social skills that his or her peers acquire at the same age. For his or her parents, this acute toxicity and the needs of care of their child can prevent his or her career pursuits. As a result, a child's acute disease also has an indirect impact on the economy and the financial prospect of the parents.

We advocate the creation of a flexible regulatory framework to define both concepts through a multistakeholder discussion process involving academia and reflecting patients' needs.

2. Science based Paediatric Medicine Development

We welcome the introduction of paediatric medicine development based on the molecular target of a new medicine in the proposal for a Regulation. Indeed, under the current framework, there is no obligation for a medicine developer to submit a Paediatric Investigation Plan (PIP) if the medicine originally developed for an adult cancer does not exist in children, even when the medicine has a

relevant mechanism of action for a given type of paediatric cancer from a biological/molecular perspective. In addition, the use of the concept of molecular target is aligned with the US RACE for Children Act and meets our expectation to harmonise the global regulatory environment in the field of drug development in paediatric oncology.

However, we recommend a clear obligation to submit PIPs upon completion of phase I of clinical studies in adults rather than prior to the initiation of the phase II clinical studies in adults. In addition, penalties to deter companies from delaying the submission of the PIP should be introduced. Furthermore, we support the cap applied to the PIP deferral to the extent that the total duration of the deferral may not exceed 5 years.

4. Academic Repurposing

We support the proposal to facilitate the repositioning of medicines, shelved or developed for other conditions, for the treatment of paediatric diseases. We also support the envisioned role of non-for-profit entities (academia) in generating data for repurposed medicines through fit-for-filing (a dataset that meets the expectations for inclusion in a regulatory package) trials. Furthermore, we appreciate the 4 years of extra data protection granted to repurposed medicinal products for the authorisation of a new indication

5. Improved Access to Novel and Essential Anticancer Medicines

Children with cancer across Europe experience inequalities in access to the best available standard diagnostics, treatment, care and research protocols. We therefore welcome the European Commission's proposal to strengthen incentives for a swifter launch and earlier access medicines for patients. Additionally, we are delighted to see a dedicated chapter on the shortages of medicine and the long-awaited Union List of Critical Medicinal Products, and we are eager to collaborate on the implementation of these important initiatives.

6. First-in-Child Innovation

The current proposal does not include specific incentives for first-in-child development and first-in-child marketing authorisation of medicines. Indeed, the various challenges in designing clinical trials in children, such as the need to centralise clinical trials in a limited number of centres and the difficulty of recruiting patient as well as the heterogeneity of the paediatric population in need of age-specific formulations are all factors that contribute to make investments in the development of drugs for a small, high-risk population economically unattractive for pharmaceutical companies. Therefore, we strongly recommend including a first-in-child marketing authorisation incentive, as this would be expected to increase commercial interest in the development of medicines specific to paediatric cancers (and paediatric rare diseases). Besides, we call for programmes allocating public funds to research projects addressing UMN in paediatric indications.

ALL SIOPE & CCI-E REFERENCE DOCUMENTS WITH RELEVANCE FOR THE EU-PHARMACEUTICAL LEGISLATION

Further enclosed documents may serve to provide you with more background information and previous SIOPE positions which tackle the issues on access to medicines in the field of paediatric oncology. You are welcome to read and use the provided intelligence at your convenience.

- **SIOPE & CCI-E - European Paediatric Cancer Community Proposal for Improvement of the EU Revised Pharmaceutical Legislation:** here
- **SIOPE & CCI-E joint statement on the proposal for the revision of the EU Pharmaceutical legislation**: here

- **SIOPE & CCI-E 6 Key Recommendations to Boost Innovation and Access to Paediatric Anticancer Medicines**: here

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For any questions, please contact: sonja.ladenstein@siope.eu or **ciara.sheehan@siope.eu**