SIOP Europe
the European Society for Paediatric Oncology

THE EUROPEAN SOCIETY FOR
PAEDIATRIC ONCOLOGY - SIOP EUROPE

WEBINARS ON PAEDIATRIC AND ORPHAN REGULATIONS

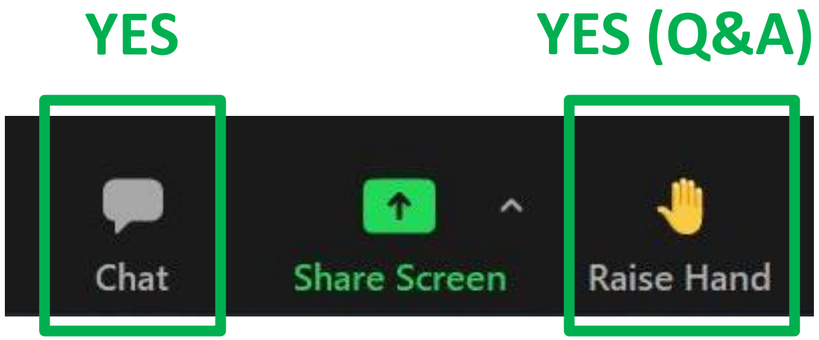
For Children with Cancer

| 23 June | 7 July

18:00 CEST | 16.30 CEST

TECHNICAL INFORMATION

- ✓ Please use chat to write your questions
- ✓ Feel free to ask any question during Q&A part



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PanCare
Pan-European Network for Care of Survivors after Childhood and Adolescent Cancer

AGENDA

1. Setting the scene (10 min)
- Pharmaceutical Strategy for Europe
- Current Timeline
- Roadmap Response

2. Orphan and Paediatric Regulations: Questionnaire
(20-30 min)
- Why to Respond
- How to Respond

3. Q&A (10 min)

PHARMACEUTICAL STRATEGY FOR EUROPE

Learning from COVID-19, towards a crisis-resistant system

Ensuring accessibility and affordability of medicines

Supporting sustainable innovation, emerging science and digitalisation

Reducing medicines shortages and securing strategic autonomy

#EUPharmaStrategy

Includes provisions on EU Paediatric Regulation governing development of new medicines for children

European Commission

CURRENT TIMELINE

OF THE EU LEGISLATION ON MEDICINES FOR CHILDREN AND RARE DISEASES

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2020 EUROPEAN COMMISSION

- ✓ Evaluation report on legislation on medicines for children and rare diseases (August)

2020 ROADMAP CONSULTATION OF LEGISLATION (Pt 1)

- ✓ Commission proposed to revise the legislation on medicines for children and rare diseases to address unmet needs (with specific mentions of paediatric cancer) (November)

2021 PUBLIC CONSULTATION OF LEGISLATION (Pt 2)

- ✓ Commission launched questionnaire on the legislation to address the unmet needs (with specific mentions of paediatric cancer) (NOW)

2022 - TBC

CHANGE OF LAW

European Commission's 2020 Evaluation Report

IMPORTANT WINS FROM PRIOR ADVOCACY

1. "Neither regulation has proven effective in boosting the development of innovative medicines **for children with rare diseases**"
2. "Neither regulation offers specific incentives to promote the successful development of **innovative medicines for use exclusively in children**"
3. "The Regulation does not necessarily address the greatest therapeutic needs of children (such as treatments of **children's cancers** and for newborns)"
4. "Publicly funded research is important"

LEGISLATION ON MEDICINES FOR CHILDREN AND RARE DISEASES

PHASE 1: ROADMAP - COMPLETED

6 Key Recommendations on Paediatric Cancers

EU's Pharmaceutical Strategy: 6 Key Recommendations to improve access to innovation and medicines for children and adolescents with cancer in Europe

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ALIGN THE REGULATIONS WITH SCIENCE AND UNMET NEEDS OF CHILDREN

ENSURE CHILD SPECIFIC AND FIRST-IN-CHILD INNOVATION

IMPLEMENT MULTI-STAKEHOLDER COOPERATION AND PRIORITISATI-ON AS A STANDARD

ALLOCATE PUBLIC INVESTMENT IN MEDICINE DEVELOPMENT FOR CHILDREN

ALIGN GLOBAL REGULATORY ENVIRONMENT

ENSURE EQUAL ACCESS TO ESSENTIAL, NOVEL ANTICANCER MEDICINES AND SUPPORTIVE CARE MEDICINES

Orphan and Paediatric Regulation: Revision

AIM

The aim of the **EU Legislation on Medicines for Children and Rare Diseases** is to incentivise the development of medicines by addressing shortcomings identified in a recent evaluation.

Tell us where Europe should focus its efforts

- Concerned Citizen (EU, non-EU)
- Patient
- Relative
- Healthcare Worker
- Researcher
- Pharma Employee

CONSULTATION PHASE 2: QUESTIONNAIRE

Why respond to the consultation?

NUMBERS COUNT

The more responses are received by EC, the more paediatric cancer needs will be noticed by policy-makers.

EXAMPLE: EUROPE'S BEATING CANCER PLAN CONSULTATION

Out of 2000 contributions, 200 came from childhood cancer community resulting in specific chapter on childhood cancers.

DISCLAIMER: Full questionnaire shall be distributed over email in the form of slide deck with proposed answers.

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How to respond?

Contribute to this consultation by filling in the online questionnaire - link attached

DEADLINE

30 July 2021 (midnight Brussels time).
If unable to fill the questionnaire contact *SANTE-ORPHAN-PAEDIATRICS-REVIEW@ec.europa.eu*

MULTILINGUAL

Questionnaire is available in some or all official EU languages.
You can submit your responses in any official EU language.

REGISTER

In order to fill the online questionnaire you will have to register at the official website of the European Union.
It will take no more than 5 minutes.

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Steps to Register on the EU Consultation Platform

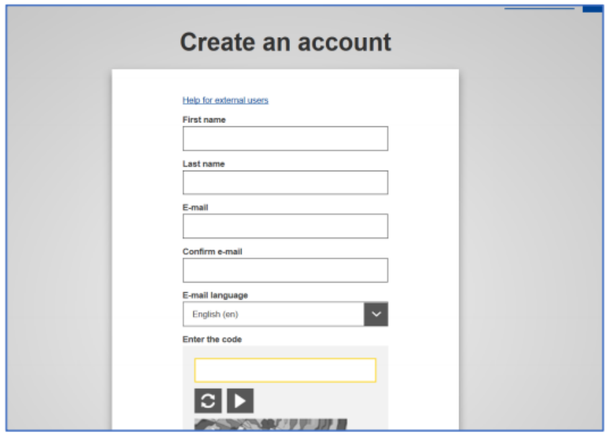
Technical instructions

Confirmation email on newly created account may take up to 24 hours!
Therefore, everyone is strongly encouraged to create account
in a timely manner in order to respond on questionnaire.

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

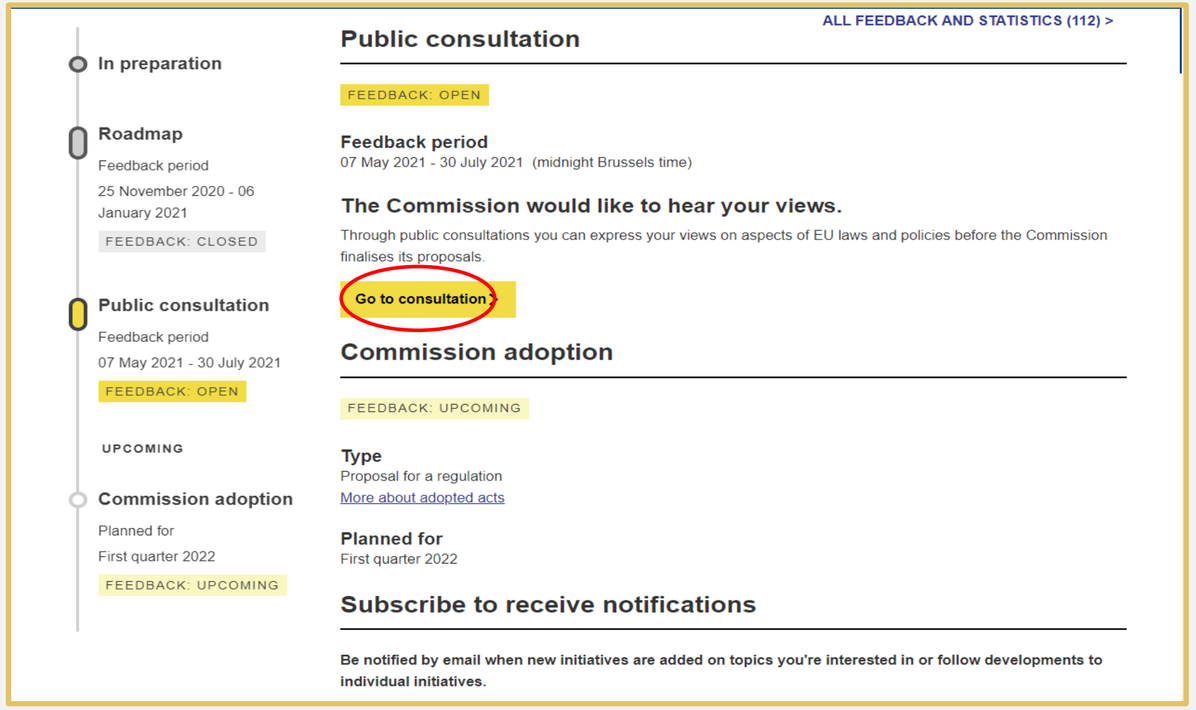


↓

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

STEP 2: Access the EU Consultation for the Legislation on Medicines for Children and Rare Diseases

2a. Go to:
https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12767-Medicines-for-children-&-rare-diseases-updated-rules_en



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The Questionnaire

ABOUT YOU

PERSONAL DETAILS

- Language of contribution*
- Giving contribution as*
- Full name*, e-mail address*
- Country of origin*
- Publication privacy settings* (anonymous/public)
- I agree with the personal data protection*

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The Questionnaire

THREE MAIN TOPICS

- Definition of rare diseases
- Definition of Unmet Medical Needs
- Rewards and incentives

Much more Orphan than Paediatric oriented questionnaire

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Question 1: In your opinion, are there any other barriers to the development of treatments for rare diseases and children? *(Open box, 2000 chars max incl. spaces)*

The main problems identified in the evaluation of the legislation for medicines for rare diseases and for children were the following:

a. Insufficient development in areas of the greatest needs for patients.

b. Unequal availability, delayed access, and often unaffordable treatments for patients in the EU MSs

c. Inadequate measures to adopt scientific & technological developments in paediatric & rare diseases

SIOPE suggestion for possible reply – key areas:

- Adult Disease Driven
- Major Delays
- Reward System
- Lack of Global Alignment
- Lack of Public Funding

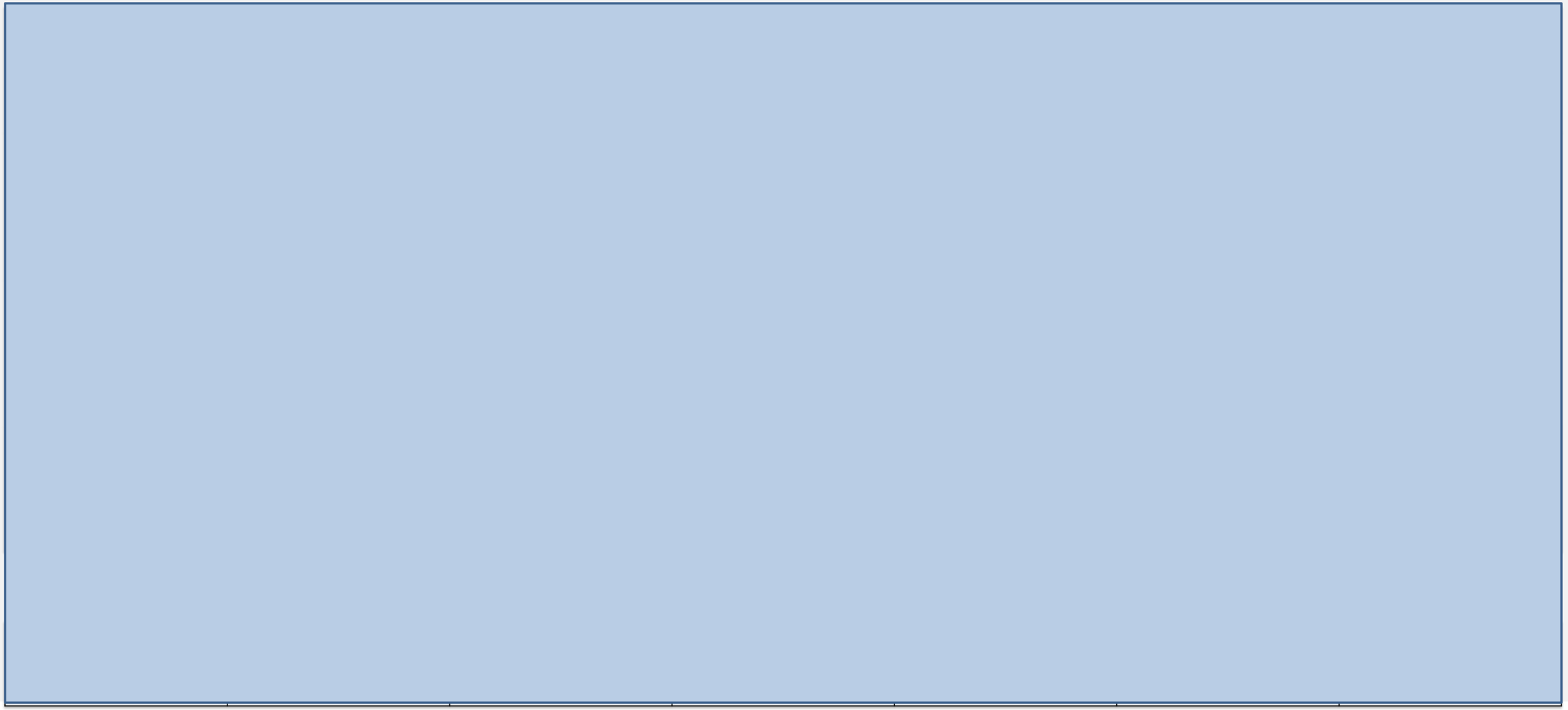
PLEASE ANSWER FROM YOUR OWN EXPERIENCE

Question 2: In your opinion, and based on your experience, what has been the additional impact of COVID-19 on the main problems identified through the evaluation? Is there a 'lesson to be learned' from the pandemic that the EU could apply in relation to medicines for rare diseases and children?

(Open box, 2000 chars max incl. spaces)

PLEASE ANSWER FROM YOUR OWN EXPERIENCE

Question 3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients? = what should be a rare disease?



Question 3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients? = what should be a rare disease?

a) When considering whether a particular medicine is eligible for support, the rarity of the disease – the total number of cases of a disease at a specific time, currently less than 5 in 10 000 people – forms the main element of the EU rules on medicines for patients suffering from rare diseases. - PREVALENCE	**MODERATELY ADEQUATE**

SIOPE suggestion for answer

Question 3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients? = what should be a rare disease?

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b) Some diseases occur frequently, but last for a relatively short period of time (for example, some rare cancers). These are covered by the EU rules on medicines for rare diseases and the principle of rarity. However, because many patients acquire such diseases during a specified, limited period of time, those diseases should not be considered as rare in the EU anymore	**NOT AT ALL ADEQUATE**

SIOPE suggestion for answer

Question 3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients? = what should be a rare disease?

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c) Amongst all medicines for rare diseases which become available to the EU patients, only those bringing a clear benefit to patients should be rewarded. Clear rules should apply to decide if one medicine brings a clear benefit to patients when compared to any other available treatment in the EU for a specific rare disease.	**MODERATELY ADEQUATE**

SIOPE suggestion for answer

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d) Additional incentives and rewards should exist for medicines that have the potential to address the unmet needs of patients with rare diseases, for example in areas where no treatments exist.	VERY ADEQUATE
	↑ SIOPE suggestion for answer

Question 3: In your opinion, how adequate are the approaches listed below for better addressing the needs of rare disease patients? = what should be a rare disease?

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d) Additional incentives and rewards should exist for medicines that have the potential to address the unmet needs of patients with rare diseases, for example in areas where no treatments exist.	**VERY ADEQUATE**

e) Other (please suggest any other criteria/approaches) *describe in 2000 characters max.* SIOPE suggestion for reply:

Situation is different for adult and pediatric cancers in the Orphan Drug field

Question 4: What factors are important to take into consideration when deciding if one medicine for a rare disease brings more benefits compared with other available treatments?

Open box, please describe in 2000 characters max, incl. spaces. SIOPE suggestion for possible reply:

- Science and biological/preclinical data
- Including comparative efficacy experiments in the preclinical setting for childhood cancers
- Early advice and cooperation between industry and academia

Question 5: What do you consider to be an unmet therapeutic need of rare disease patients & children? Multiple choice answer

- ✓ a) Authorised medicines for a particular rare disease or a disease affecting children are not available, and no other medical treatments are available (e.g. surgery).
- ✓ b) Treatments are already available, but their efficacy and/or safety is not optimal. For example, it addresses only symptoms.
- ✓ c) Treatments are available, but impose an elevated burden for patients . For example, frequent visits to the hospital to have the medicine administered.
- ✓ d) Treatments are available, but not adapted to all subpopulations. For example, no adapted doses and/or formulations, like syrups or drops exist for children.
- ✓ e) Other, please specify. *(open box, 2000 characters max, incl. spaces)*

SIOPE suggestion for possible reply:

- Unmet medical needs should be defined first by academia, parents and patients
- Multi-stakeholder process to agree on UMN and strategy for drug development at the same time

Question 6: Which of the following measures, in your view, would be most effective for boosting the development of medicines addressing unmet therapeutic need of patients suffering from a rare disease and/or for children?

1	2	3	4	5	6	7	8	9	10

(1 being the least effective, 10 being the most effective) *at most 1 answered row(s)*

a. Assistance with Research & Development (R&D), where medicines under the development can benefit from national and/or EU funding

b. Additional scientific support for the development of medicines from the European Medicines Agency

c. Assistance with authorisation procedures, such as priority review of the application from the European Medicines Agency and/or expedited approval from the European Commission

d. Additional post-authorisation incentives that complement or replace the current incentives and rewards

- **Do you have other suggestions that would allow the EU to boost the development of specific medicinal products?** SIOPE suggestion for possible reply:

  SIOPE and CCI-E 6 Key Recommendations to Improve Access to Innovation and Medicines in Paediatrics

- **Do you see any drawbacks with the approaches above?** *please describe in 2000 characters max, incl. spaces.*

Question 7: Which of the following options, in your view, could help all EU patient (irrespective of where they live within the EU) to provide them with better access to medicines and treatments for rare diseases or children? Multiple choice answer.

✓ a) Greater availability of alternative treatment options. For instance, by allowing a generic or biosimilar product to enter the market faster.

✓ b) Allowing companies that lose commercial interest in a rare disease or children medicine product to transfer its product to another company, encouraging further development and market continuity.

✓ c) For companies to benefit from full support and incentives, products need to be placed timely on the market within all Member States in need as soon as they received a marketing authorisation.

d) Other (please suggest any other solution you think might be relevant)

SIOPE suggestion for possible reply:

- Appropriate and quick pricing and reimbursement strategies
- Harmonisation of Health Technology Assessment (HTA)
- Reducing medicine shortages in Europe

Question 8: Most of the medicines for rare diseases are innovative medicines. However, in some cases, an older, well-known medicine for a common disease can be repurposed (i.e., using existing licensed medicines for new medical uses) to treat a rare disease. **In your view, what would be the appropriate way to award innovative medicines in cases where other treatments are available:**

Pre-specified answer options, single choice answer.

a) Both new, innovative medicines and well-known medicines repurposed to treat a rare disease should receive the same reward

b) New, innovative medicines to treat a rare disease should receive an enhanced reward

✓ c) Do not know / cannot answer

Despite the presence of the Paediatric Use Marketing Authorisation (PUMA) many older medicines currently used to treat children have only been studied for use in adults and lack the appropriate dosage or formulation for children. However, development of medicines that have been adapted for use in children could also result in a product being more expensive than its adult- focused counterpart. In your view:

Question 9: Should the development of appropriate dosage or formulation suitable for children of such older medicines be stimulated even if their price will be higher than that of the available alternatives?

✓ - YES

- NO

- DON'T KNOW / CANNOT ANSWER

Question 9: Should the development of appropriate dosage or formulation suitable for children of such older medicines be stimulated even if their price will be higher than that of the available alternatives?

- **Please explain your answer.** *in 2000 characters max, incl. spaces.*

  Need to answer children's needs with affordable innovative solutions.

- **How would you suggest stimulating further development of appropriate dosage or formulation suitable for children of such older medicines?** *in 2000 characters max, incl. spaces.*

- **How can it be ensured that such developed products are reasonably profitable for companies and also reach patients?** *in 2000 characters max, incl. spaces.*

  SIOPE suggestion for possible message:

  Sustainable funding stream allocated to academia would increase medicine development of cost-effective child-friendly formulations and avoid production of more expensive medicines by pharmaceutical industry.

Q&A

PLEASE SEND YOUR QUESTIONS
ON “office@siope.eu"

Questionnaire Toolkit

- Technical Guide (to fill the questionnaire)
- Overarching Messages for Questionnaire
- PPT slide deck
- Joint SIOPE and CCI-E, 6 Key Recommendations on Paediatric Cancer Medicines Access

Together for a brighter future for children and adolescents with cancer, survivors and their families!

THANK YOU

For participating and making a change

ENGAGE

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