

THE EUROPEAN SOCIETY FOR
PAEDIATRIC ONCOLOGY - SIOP EUROPE

WEBINARS ON PHARMACEUTICAL STRATEGY FOR EUROPE



For Children with Cancer

1 September | 3 September

at 19:00 CEST



AGENDA

1. Setting the scene (10 min)

- Introductory Background
- EU Pharmaceutical Strategy
- Roadmap Response
- Projects and activities

2. Europe's Beating Cancer Plan questionnaire (20-30 min)

- Why respond
- How to respond

3. Extra Remarks – CCI Europe (5-10 min)

4. Q&A (10 min)

SLOW PACE OF INNOVATION CANNOT APPROPRIATELY SERVE PAEDIATRIC CANCER PATIENTS

KEY CHALLENGES

- The rarity of individual paediatric cancers has translated into very limited market-driven innovation
- Only 9 compared to over 150 anticancer medicines were authorised only for paediatric indication
- The Orphan & Paediatric Regulation has been ineffective for paediatric cancer medicine development
- Young cancer patients in Europe still experience lack of access to essential medicines
- Inadequate pricing and reimbursement strategies are challenging
- Lack of investment in outcome research, including on possible late effects in survivors



PHARMACEUTICAL STRATEGY FOR EUROPE



AIM

The aim of the EU Pharmaceutical Strategy is to improve and accelerate patients' access to safe and affordable medicines and to support innovation in Europe.



➤ Concerned Citizen

➤ Patient

➤ Relative

➤ Healthcare Worker

➤ Researcher

➤ Pharma Employee

➤ Policy Maker

Tell us where Europe should focus its efforts

TERMS AND DEFINITIONS

ACCORDING TO EU



1

AVAILABILITY

A medicine becomes available once it has been authorised in a Member State or centrally in the EU.

2

ACCESSIBILITY

A medicine becomes accessible to patients once it has been authorised, is being marketed, and can be reimbursed in a Member State.

3

AFFORDABILITY

Relates to payments to be made by patients (out of pocket on healthcare or through co-payments) which can be described as affordability at micro level and to the sustainability of public funding of the healthcare sector raised through social security contributions or taxes (affordability at macro level).

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

WHAT WE HAVE BEEN DOING

EXAMPLES OF PROJECTS AND ACTIVITIES



Ongoing

ADVOCACY ON ENABLING POLICY FOR THERAPEUTIC INNOVATION

AIM

- Improve the Paediatric Regulation and related initiatives



Completed

JOINT ACTION ON RARE CANCERS (JARC)

AIM

- Reduce shortages
- Improve financial accessibility
- Provide child-friendly formulations
- Ensure supportive and pain management care



Ongoing

THE ESSENTIAL MEDICINES PROJECT

AIM

- Generate a list of essential paediatric anticancer medicines to treat cancer in children and adolescents in Europe



Upcoming

PROJECT PROPOSAL THE EX-CHANGE

AIM

- Create a multi-stakeholder network to facilitate the delivery of the multi-national, multi-dimensional European Childhood Cancer Big Data Gateway



EUROPE'S PHARMACETICAL STRATEGY CONSULTATION

PHASE 1: ROADMAP - COMPLETED

Key topics covered



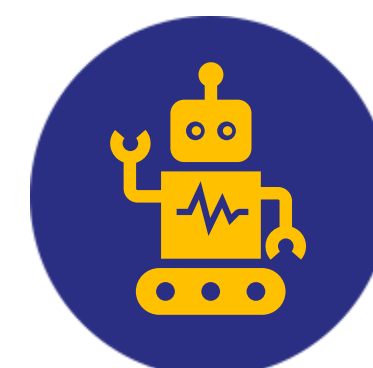
**ACCESS TO
ESSENTIAL
PAEDIATRIC
ANTICANCER
MEDICINES
AND
SUPPORTIVE
CARE**



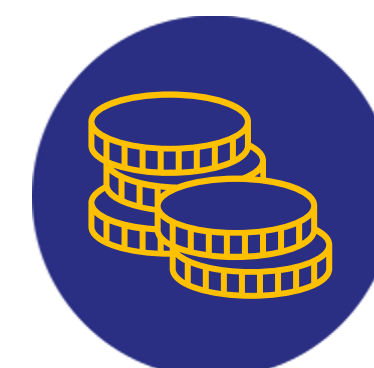
**BOOST
THERAPEUTIC
INNOVATION
IN PAEDIATRIC
CANCERS**



**IMPROVE THE
ORPHAN &
PAEDIATRIC
MEDICINE
REGULATION**



**EXPLOIT THE
ARTIFICIAL
INTELLIGENCE
POTENTIAL
FOR
PAEDIATRIC
CANCERS**



**DEFINE
APPROPRIATE
PRICING AND
REIMBURSE-
MENT
STRATEGIES**



**MINIMALISE
SIDE AND LATE
EFFECTS IN
CHILDHOOD
CANCER
PATIENTS AND
SURVIVORS**

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 originated from childhood cancer community.

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

How to respond?



Contribute to this consultation by **filling in the online questionnaire** - [link attached](#)

DEADLINE

15 September 2020

(midnight Brussels time).

If unable to fill the questionnaire contact *EU-PHARMACEUTICAL-STRATEGY@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.

Steps to Register

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 2: Access the EU Cancer Public consultation

2a. Go to:

<https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12154-Europe-s-Beating-Cancer-Plan>

Scroll to the bottom of the page to “Public consultation”

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)

Pharmaceuticals – safe and affordable medicines (new EU strategy)

[Have your say](#) > [Published initiatives](#) > [Pharmaceuticals – safe and affordable medicines \(new EU strategy\)](#) > [Public consultation](#)

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About this consultation

Target audience

Why we are consulting

Responding to the questionnaire

Contact

About this consultation

Feedback period 16 June 2020 - 15 September 2020 (midnight Brussels time)

Topic Public health

Target audience

Members of the public, stakeholders and organisations are welcome to contribute to this consultation. Organisations representing patients and civil society active in public health, healthcare professionals and providers, academia, researchers and the pharmaceutical industry might have a particular interest to contribute.

Why we are consulting

Medicines play an important role in assuring diagnosis, treatment and prevention of diseases. We as citizens across the EU expect to benefit from equal access to safe, innovative and affordable therapies because our health often depends on them. If treatments are not available due to shortages or because we simply cannot afford them, our health can be compromised. The Commission intends to design a plan that will address the current issues of access, availability and affordability of medicines, while still promoting sustainable innovation and support EU industry to

Steps to Register

Technical instructions



2b. Click 'Go to consultation' (to view the consultation and become acquainted further)

2c. After clicking 'Go to consultations', you will be directed to this website page:

<https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12154-Europe-s-Beating-Cancer-Plan/public-consultation>

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respond to public health threats such as the coronavirus pandemic, without compromising patient safety. This plan will be a new Pharmaceutical Strategy for Europe, scheduled for adoption by the end of the year. In order to ensure that all possible views are considered in the design of this strategy and to ensure transparency and accountability, the European Commission invites all interested individuals or organisations to share their views and experiences and contribute to the development of a plan putting Europeans at the centre.

Responding to the questionnaire

You can contribute to this consultation by filling in the online questionnaire. If you are unable to use the online questionnaire, please contact us using the email address below.

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

For reasons of transparency, organisations and businesses taking part in public consultations are asked to register in the [EU Transparency Register](#).

Respond to the questionnaire >

Personal data and privacy statement

The European Union is committed to protecting your personal data and to respecting your privacy. When carrying out public consultations we adhere to the policy on 'protection of individuals with regard to the processing of personal data by the Community institutions', based on [Regulation \(EU\) 2018/1725](#) on processing of personal data by the EU institutions..

Further information on the [protection of your personal data](#)

Contact

EU-PHARMACEUTICAL-STRATEGY@EC.EUROPA.EU

3d. Click 'Respond to the questionnaire' in order to access it (you will be asked to log into your account on EU Health Policy Platform)

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*

Section 1: International Dependency and Manufacturing



Recognising that each stakeholder has specific concerns, the below proposed messages come from our mutually endorsed position statement. Alignment on these in our respective replies can send a strong unified message.

1. What type of EU action or initiative do you consider helpful to incentivise the production of active pharmaceutical ingredients for essential medicines (e.g. antibiotics, oncology medicines) in the EU? *(Open box, 800 chars max incl. spaces)*

- Establish a European level reference list of essential paediatric anticancer medicines
- Overcome inequalities in access to medicines across Europe
- Support further research on access to all medicines across Europe (incl. supportive/palliative care)
- Foster child-friendly doses and formulations of medicines

Section 1: International Dependency and Manufacturing



2. What action do you consider most effective in enhancing the high quality of medicines in the EU?

- Stronger enforcement of the marketing authorisation holder responsibilities
- Increased official controls in the manufacturing and distribution chain
- Other (please specify)
- I don't know

If 'other' selected, please elaborate your reply in 500 character(s) max incl spaces

Section 2: Access to Affordable Medicines



3. Are you concerned about medicines shortages in the EU?

- I am concerned
- I am not concerned
- I have no particular opinion

PLEASE ANSWER FROM YOUR OWN EXPERIENCE

If you wish, please elaborate your reply (does not appear if 'I have no particular opinion' is selected). 500 character(s) max incl. spaces

- Healthcare professionals and patients across Europe still experience issues of access to these agents according to a survey part of the EU JARC. Shortages are the most common reason for lack of availability. Concrete recommendations on actions can be found in [JARC deliverable 9.1](#)

4. Which actions do you think would have the biggest impact on reducing shortages in the EU? *At most 3 choice(s)*

- Stronger obligations on medicines producers, and other players in the supply chain to ensure medicines are available
- Transparent information exchange among authorities on medicine stocks available in each country
- Increased cooperation among public authorities/national governments on shortage
- Multi-lingual packaging and electronic product information leaflets facilitating purchasing in different countries
- Providing incentives to companies to increase the production of medicines in the EU
- Inform on and make available to patients suitable substitutes for medicines that are at risk of shortage
- Other (please specify)

Please elaborate your reply (only appears if 'other' is selected). 500 character(s) max incl. spaces

- Transparent information exchange on medicines stocks and an EU action on essential medicines by referring to WHO EMLc and evidence created by the European paediatric cancer community as an important reference.

Section 2: Access to Affordable Medicines



5. Do you think that companies that apply for and receive an EU-wide marketing authorisation should be required to make that product available in all EU countries?

- I agree
- I neither agree or disagree
- I disagree
- I don't know

If you wish, please elaborate your reply (does not appear if 'I don't know' is selected). 500 character(s) max incl. spaces

- Major inequalities are present in access, treatment and outcomes across Europe. These drugs should be available in all countries. Better and centralised HTA assessment of all new drugs is needed.

Section 2: Access to Affordable Medicines



6. Do you have an opinion on the reasons for these market withdrawals?

➤ Yes

➤ No

If yes, please elaborate. 500 character(s) max incl. spaces

- In the field of childhood cancers the development of several drugs has been stopped in adults for inefficiency without further follow up for possible paediatric indication. Repurposing of molecules originally meant for adults may benefit in paediatric cancers. Revised EU regulatory initiatives could address this.

Section 2: Access to Affordable Medicines



7. Are you aware of patients not receiving the medicine they need because of its price?

➤ Yes

➤ No

PLEASE ANSWER FROM YOUR OWN EXPERIENCE

If you wish, please elaborate. Your reply 500 character(s) max incl. spaces

- Despite the slow innovation in paediatric cancers, the few new agents entering the market faced delays due to HTA procedures. Uncovering hurdles in the evaluation process could place pragmatic methods to improve childhood cancer journey. SIOP Europe's and CCI Europe's 'Essential Medicines Project' is evaluating the current HTA methodology.

Section 2: Access to Affordable Medicines



8. Do you think that medicine prices are justified, taking into consideration the costs associated to their development and manufacturing?

- Yes
- No
- I don't know

If you wish, please elaborate. 500 character(s) max incl. spaces

- What needs to be taken into consideration when funding and pricing expensive and unaffordable medicines is public transparency and the value a childhood cancer survivor can have for society with many expected years to live and contribute to society. Out-of-pocket costs remain burning issue.

9. What are the most effective ways the EU can help improve affordability of medicines for health systems? *At most 3 choice(s)*

- Support the EU countries in better assessing and/or evaluating the value of medicines, meaning the effectiveness of a (new) medicine compared with existing ones
 - Help EU countries share experiences and pool expertise on pricing and procurement methods
 - Better coordination among EU countries to ensure that pricing decisions taken by one EU country do not lead to negative impacts on patient access in another EU country
 - Facilitate, market entry and a healthy market functioning for generics and biosimilars
 - More transparency on how the cost of a medicine relates to the cost of its research and development
 - There should be a fair return on public investment when public funds were used to support the research and development of medicines
 - I don't know
 - Other (please specify) *Elaborate in 100 character(s) maximum. Appears if 'other' is selected.*
- Avoiding fragmentation in pricing/reimbursement for paediatric cancer patients across Europe and consider the age of survivors.

Section 3: Innovation in Early Development and Authorisation

10. What actions at EU level do you consider most effective in supporting innovative research and development of medicines?

- Make the legislative framework more adaptive to new technologies and advances in science
 - Provide more public funding for research
 - Support (including through funding) private-public partnerships
 - Support (including through funding) the creation of start-ups in medical research
 - Foster research collaboration between universities, research centres and industry
 - Provide research and development incentives in the form of intellectual property or market exclusivity rights for pharmaceutical companies investing in research
 - Simplify the requirements for the conduct of clinical trials
 - Other (please specify)
 - I don't know - *Elaborate in 100 character(s) maximum. Only appears if 'other' is selected.*
- Improved legislative framework to meet the unmet medical needs of children with cancer.

Section 3: Innovation in Early Development and Authorisation

11. What do you consider are the most effective actions related to research and development of medicines in areas where there are limited or no therapeutic options (unmet needs)? *At most 3 choice(s)*

- Provide market protection (protect a new medicine from competition)
- Provide intellectual property protection
- Provide data protection (protection of the data related to a medicine's clinical trials)
- Agree on a common understanding on what are the areas of unmet need in the EU
- Funding more targeted research at EU level
- Funding more targeted research at national level
- Provide national schemes to support companies economically
- I don't know / no opinion
- Other (please specify) *Elaborate in 100 character(s) maximum. Only if 'other' is selected.*

- Offer effective incentives for development of medicines for children with rare diseases, e.g. cancer

Section 3: Innovation in Early Development and Authorisation



12. Which opportunities do you see in digital technologies (such as artificial intelligence and use of real world data) for the development and use of medicines?

Open box, 600 character(s) maximum incl. spaces

- Generating real world data will help to evaluate new medicines in field of small populations, not only for authorisation, but HTA as well. Where randomised control trials often are not feasible. An overarching European initiative enabling data sharing across Europe is needed. Such a database would be the perfect opportunity to launch AI projects.

Section 3: Innovation in Early Development and Authorisation



13. Which risks do you see in digital technologies (such as artificial intelligence and use of real world data) for the development and use of medicines?

Open box, 600 character(s) maximum incl. spaces

- Any potential risks in use of AI and real-world data cannot compare to the benefits in paediatric oncology. Paediatric cancers are all rare, therefore centralised data integration in a comprehensive metadata catalogue will enable innovative treatments essential for the development of new and better drugs. Naturally, all safeguards in compliance with GDPR should ensure privacy protection.

Section 3: Innovation in Early Development and Authorisation



14. Are you aware of any obstacles in the EU in taking advantage of technological progress in the manufacturing of medicines?

- Yes
- No
- I don't know

If yes, could you please specify. 500 character(s) max incl. spaces

Section 3: Innovation in Early Development and Authorisation

15. How could clinical trials in the EU be driven more by patients' needs while keeping them robust, relevant and safe for participants? *At most 3 choice(s)*

- By providing more national support for the conduct of so-called “pragmatic trials” with the aim to optimise treatment to patients
- By better coordination for larger trials comparing different treatment strategies (covering medicines and other treatments such as surgery, radiotherapy, physiotherapy)
- By providing support for non-commercial organisations to conduct clinical trials in fields where financial interest is weaker
- By involving patients' experiences in early phases of medicine design (e.g. factor-in how the disease affects their lives and develop medicines to target symptoms that are particularly important to patients)
- By designing more trials that collect information on medicine tolerability or the impact of a treatment on the quality of life
- By taking into consideration during the design of a trial the burden of trial participation on patients' life
- Other (please specify) *Elaborate in 100 character(s) max incl. spaces. Only if 'other' is selected.*

- Early cooperation between academia and industry to design clinical trials that would meet the needs of paediatric cancers.

Section 3: Innovation in Early Development and Authorisation



16. Is the current legal framework suitable to support the development of cell-based advanced therapy medicines in hospitals?

- I strongly agree
- I partially agree
- I disagree
- I don't know

**If you responded partially agree or disagree, please provide examples of changes that, in your view, would be required to support the development of these products.*

- Consider to develop an adequate regulatory framework for academic manufacturing of cell-based therapies, such as CAR-T Cells.

Section 4: Environmental Sustainability of Medicines and Health Challenges



17. What actions at EU level do you consider most effective in limiting the negative environmental impact of medicines? *At most 3 choice(s)*

- Cleaner manufacturing processes
- Enhanced application of the polluter pays principle
- Review the way the Environment Risk Assessment of a medicine is conducted and its consequences on the authorisation process
- Clear labelling of environmental risks to allow informed choices among equivalent therapeutic options
- Reference to environmental risks in advertising for over-the-counter medicines
- Make medicines known to pose an environmental risk available by prescription only

NEXT SLIDE CONT.

Section 4: Environmental Sustainability of Medicines and Health Challenges



17. What actions at EU level do you consider most effective in limiting the negative environmental impact of medicines? *At most 3 choice(s)*

- Strict disposal rules for unused medicines
- Prescribe medicines only when it is absolutely necessary (more prudent use)
- Medicines dispensed to patients in the quantity actually needed (e.g. number of pills, volume of solution)
- Enhanced wastewater treatment if certain residues could be better removed
- Other (please specify)

Please elaborate your reply (only appears if 'other' is selected). 100 character(s) max incl. spaces

- Child-friendly formulations are needed as adapting adult cancer formulations leads to waste.

Section 4: Environmental Sustainability of Medicines and Health Challenges



18. Which actions do you think would have the biggest impact on fighting AMR concerning the use of medicines for patients? *At most 3 choice(s)*

- More prudent use of antimicrobials (if necessary through restrictions on prescriptions)
- Improve the treatment of wastewater and/or manure to lower the levels of antimicrobials
- Raise citizens' and healthcare practitioners' awareness by informing them on appropriate use of antimicrobials and the correct disposal of unused medicines
- Introduce an obligation to use diagnostic tests before prescribing antimicrobials, for example to verify whether it is a bacterial infection before prescribing antibiotics and to define the most adequate antibiotic
- Public finance research and innovation on new antimicrobials, their alternatives and diagnostics

NEXT SLIDE CONT.

Section 4: Environmental Sustainability of Medicines and Health Challenges



18. Which actions do you think would have the biggest impact on fighting AMR concerning the use of medicines for patients? *At most 3 choice(s)*

- Encourage public health campaigns that prevent infection through better general health including increased immunity
- Encourage public health campaigns that prevent infection through the use of vaccines
- Encourage better hygiene measures in hospitals
- Other (please specify)
- I don't know

Please elaborate your reply. Only appears if 'Other (please specify)' is selected. 100 character(s) max.

- Research consolidation on the impact of AMR on childhood cancer patients.

Section 4: Environmental Sustainability of Medicines and Health Challenges



19. Where, in your view, should the EU focus its support for the creation of new antimicrobials or their alternatives? *At most 2 choice(s)*

- Support academia for researching/discovering new antimicrobials or their alternatives
- Support industry for developing new antimicrobials or their alternatives
- Other (please specify)
- I don't know

Please elaborate your reply. Only appears if 'Other (please specify)' is selected. 100 character(s) max.

- All initiatives on AMR including the paediatric cancer perspective.

Section 4: Environmental Sustainability of Medicines and Health Challenges



PLEASE ANSWER FROM YOUR OWN EXPERIENCE

20. How has the coronavirus (COVID-19) pandemic affected you in relation to access to medicines and treatments? *Open box, 600 character(s) maximum incl. spaces*

- Big affluence of COVID-19 patients to hospitals and the fear of contamination has reduced the paediatric cancer appointments, diagnosis and treatments procedures. Reduced access to cross-border innovative research. The restrictions on mobility were a problem as some children have the need to travel abroad to get the most adequate therapies, which was challenging in past months.

Section 4: Environmental Sustainability of Medicines and Health Challenges



21. In your opinion and based on your experience, what can the EU do to prepare for and manage such a situation better in the future in relation to pharmaceuticals? *Open box, 600 character(s) maximum incl. spaces*

- The EU can help fight inequalities among EU countries to ensure everyone has access to required paediatric cancer medicines, at all the times, for all children and adolescents with cancer. Medical consultations could also be adapted to be done remotely with more patient-oriented methods. The ERN PaedCan is already advancing this vision and deserves attention and support.

Section 5: Summary Question

22. While the Commission is working on improving the EU pharmaceuticals framework, which areas of work do you find most urgent? *At most 3 choice(s)*

- Improve patients' access to medicines
- Reduce shortages
- Help national authorities ensure affordability for patients and increase health systems sustainability
- Support innovation for unmet needs
- Use of digitalisation to develop medicines
- Help reduce anti-microbial resistance
- Reduce the dependency on essential active ingredients and medicines produced outside the EU
- Environmental sustainability of medicines
- I don't know
- Other – *Please elaborate if 'other' selected. 100 character(s) max incl spaces*

Section 4: Environmental Sustainability of Medicines and Health Challenges



23. If you were asked before the coronavirus (COVID-19) pandemic, would you have responded differently to any of the previous questions?

- Yes
- **No**
- I don't know

Please elaborate your reply. Appears if 'yes' is selected. 500 character(s) max, incl. spaces

Section 5: Summary Question



24. If you were asked before the coronavirus (COVID-19) pandemic, would you have responded differently to any of the previous questions? *Open box, 900 character(s) max incl. spaces*

For boosting therapeutic innovation in childhood cancers:

- Mechanism-of-action rather than adult clinical indication-based developments
- Alignment with global regulatory advances e.g. RACE for Children Act, incl. potential revision of the Paediatric Regulation
- Regulatory incentives for the development of specific childhood anticancer medicines.
- Repurposing molecules originally meant for adults and unshelving new indications for approved medicines
- NEXT SLIDE CONT.

Section 5: Summary Question



24. If you were asked before the coronavirus (COVID-19) pandemic, would you have responded differently to any of the previous questions? *Open box, 900 character(s) max incl. spaces*

For boosting access and availability of childhood anticancer medicines:

- Child-friendly formulations as adapting adult cancer formulations leads to waste
- For improving access to essential medicines for paediatric cancers, including reducing shortages. An EU action should consider their needs and refer to WHO EMLc and evidence generated by European paediatric cancer community
- Appropriate pricing and reimbursement strategies paediatric cancer medicines are a must

Questionnaire Toolkit



- Technical Guide to fill the questionnaire (*healthcare professionals, patients, parents, survivors*)
- Overarching Messages (*same audience*)
- PPT and Webinar
- Joint Roadmap Response

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

THANK YOU

For participating
and making a
change

e-mail: office@siope.eu

e-mail: europe@cci.care

e-mail: info@pancare.eu



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