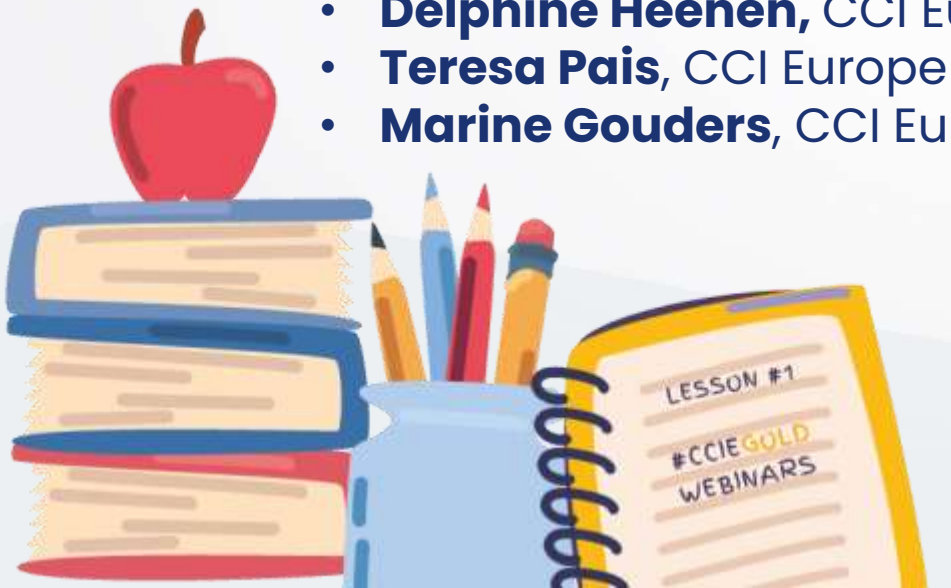
Childhood Cancer International EUROPE

Drug Regulations – What is new for the childhood cancer community?

Advance Your Knowledge with:

- **Delphine Heenen,** CCI Europe Regional Committee Member & Head of European Affairs
- **Teresa Pais**, CCI Europe Senior Policy Officer
- **Marine Gouders**, CCI Europe Policy Officer



03.06.2024
17:00 – 18:30 CET

Co-funded by the European Union

www.ccieurope.eu

SPEAKERS

Childhood Cancer International EUROPE

Delphine Heenen

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Senior Policy Officer

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#CCIEGOLD WEBINARS

HOUSEKEEPING ITEMS

1. Smile! The webinar is being recorded ☺
2. Use the chat for your burning questions
3. We'd love to hear from you! We are going to address all your written and oral questions in the Q & A session at the end of the presentation

#CCIEGOLD WEBINARS

CONTENT

1. EU Drug Regulations in a nutshell
2. Our advocacy work
3. Our 6 priorities
4. Q&A

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1. EU Drug Regulations in a nutshell

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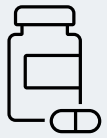
The current legislative framework

2001 & 2004

EU General pharmaceutical legislation

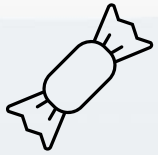
aims at regulating the procedure for authorisation and supervision of medicines



2000

Regulation on orphan medicinal products
"Orphan Regulation"

aims at providing **incentives** for research and development of medicines in **rare diseases**

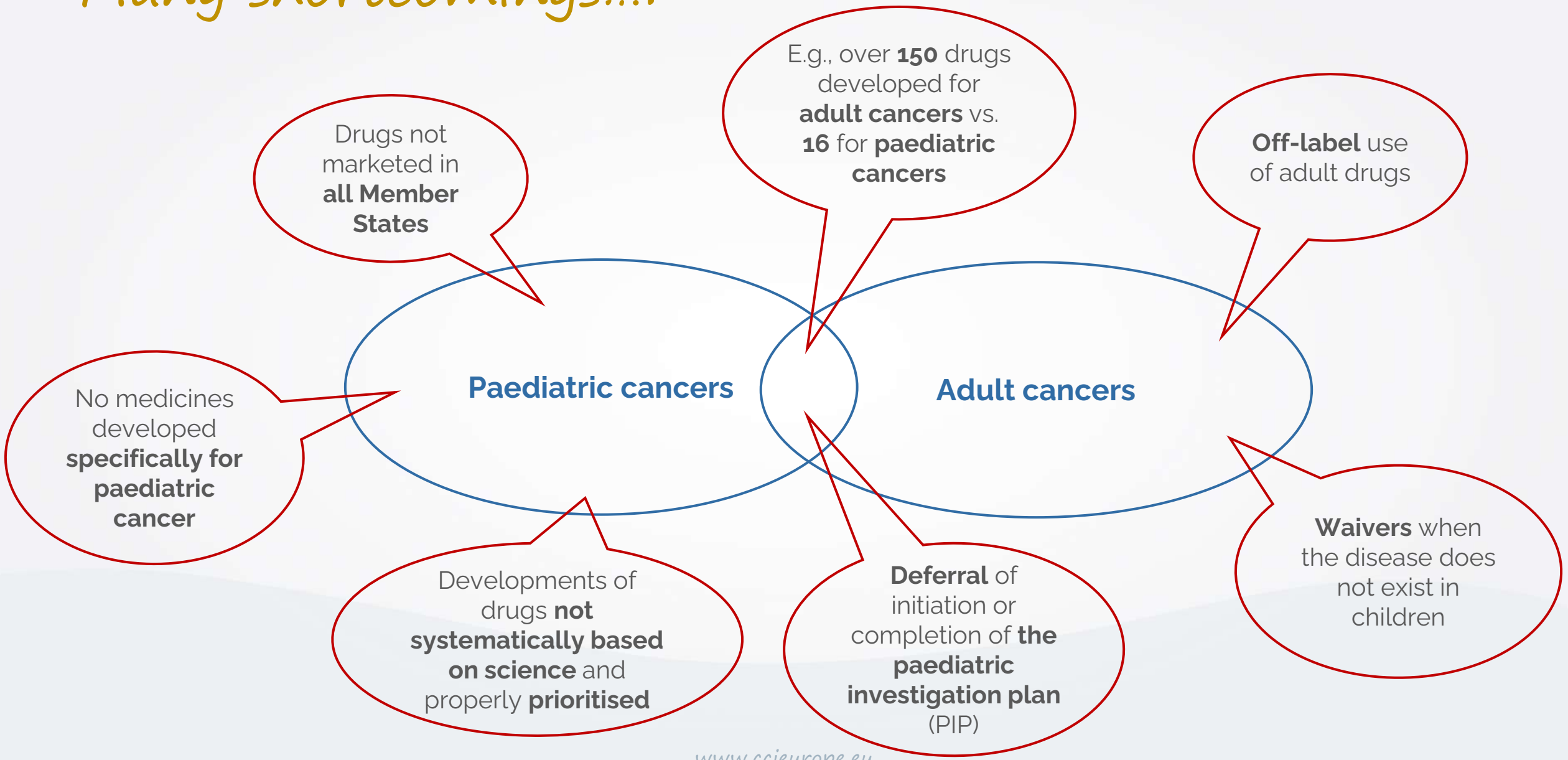


2006

Regulation on medicinal products for paediatric use
"Paediatric Regulation"

aims at regulating **the development of medicines in paediatric diseases**

Many shortcomings....



The revision of the EU pharmaceutical legislation

EU General pharmaceutical legislation — 2001 & 2004

+

The Orphan Regulation — 2000

+

The Paediatric Regulation — 2006

→

2020

"The revision of the EU Pharmaceutical legislation"

European Commission proposed a **joint revision** to **improve therapeutic landscape** and **address unmet needs** in children

The proposal by the EU Commission

26th of April 2023

THE « PHARMACEUTICAL PACKAGE »

- Proposal for a **Directive** on the Union code relating to medicinal products for human use
- Proposal for a **Regulation** laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency

Which contains **provisions on rare diseases** & **paediatric diseases**

EUROPEAN COMMISSION

Brussels, 26.4.2023
COM(2023) 192 final

2023/0132 (COD)

Proposal for a

DIRECTIVE OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

on the Union code relating to medicinal products for human use, and repealing Directive 2001/83/EC and Directive 2009/35/EC

(Text with EEA relevance)

{SEC(2023) 390 final} - {SWD(2023) 191 final} - {SWD(2023) 192 final} - {SWD(2023) 193 final}

Brussels, 26.4.2023
COM(2023) 193 final
2023/0131(COD)

Proposal for a

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulation (EC) No 1394/2007 and Regulation (EU) No 536/2014 and repealing Regulation (EC) No 726/2004, Regulation (EC) No 141/2000 and Regulation (EC) No 1901/2006

(Text with EEA relevance)

{SEC(2023) 390 final} - {SWD(2023) 192 final} - {SWD(2023) 193 final} - {SWD(2023) 194 final}

2. Our advocacy work

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Coalition building

- We **jointly discuss proposals and unite forces** at the EU level (ex: joint amendments)
- **Coalition building** is a strategy to boost advocacy and engagement with EU policy makers

Our materials

European Paediatric Cancer Community Proposal for Improvement of the EU Revised Pharmaceutical Legislation

INTRODUCTION AND BACKGROUND

The **European Society for Paediatric Oncology (SIOPE)**, the single-united European organisation representing all healthcare professionals in paediatric oncology, and **Childhood Cancer International – Europe (CCI-E)**, the largest European patient' organisation in paediatric cancer, have been involved in the review and analysis process of the EU Paediatric Regulation since 2012 and the EU Orphan Regulation since 2016.

We warmly welcome the EU Commission's revised Pharmaceutical legislation, as stated in our joint statement available here. We believe it addresses several areas of concern of the paediatric cancer community.

Today, neither the EU Paediatric nor the EU Orphan Regulations have succeeded in significantly improving young cancer patients' chance of survival. Notably, over the last ten years, only 16 anticancer medicines have been authorised for a specific paediatric cancer indication, in contrast to over 150 for adult cancers[1]. Now, we are calling for an ambitious revision that will support and accelerate innovation and put the unmet medical needs of patients and their families at the centre of a European strategy for medicine development.

Therefore, SIOPE and CCI-E have identified **six key priority areas** which require focused consideration during the negotiation of the revised EU Pharmaceutical legislation. We have **detailed below areas which should be upheld, as well as areas with specific suggestions for amendments** which can further strengthen the reform and ultimately, improve the outcomes of patients with unmet medical needs such as young cancer patients and restore them to their full health.

Attached to this document is an annex listing the articles of the revised EU Pharmaceutical legislation that we propose to maintain, as well as the amendments that we advocate.

PRIORITY AREAS FOR PAEDIATRIC CANCER COMMUNITY

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.

> *"The onset of these side effects can be acute or chronic, having the potential to result in a severe disabling, life-threatening or fatal illness, such as a cardiovascular disease, stroke,*

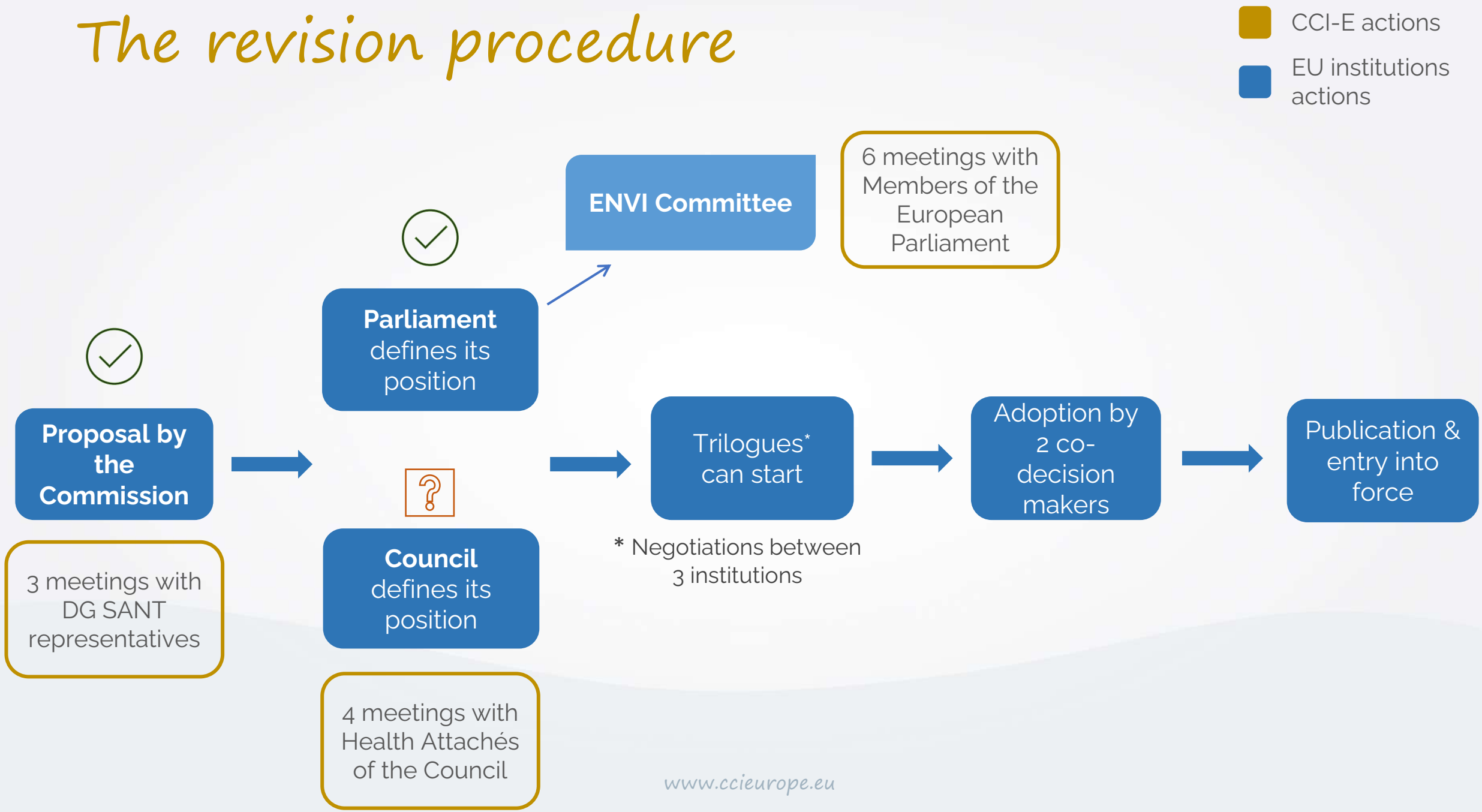
SIOPE & CCI-E VOTING LIST FOR THE REVISION OF THE EU PHARMACEUTICAL PACKAGE

Based on the Rapporteurs' and ENVI Committee Amendments to the Commission Proposals of the Pharmaceutical Directive and Regulation SIOPE and CCI-E prepared a voting list for the upcoming voting in the ENVI committee .

The Amendments are categorised by priority topics for the Childhood Cancer Community. Below each topic header, you can find highlighted the wording of the Articles that we support as well as a short reasoning. For more details, you can consult our position statement for the Revision of the Pharmaceutical Legislation included below.

AMENDMENT NUMBER	SIOPE AND CCI-E VOTING RECOMMENDATIONS YES ✓ / NO ✗
UMN and HUMN - Directive Article 83/1/b and 81/2/b; Regulation Article 70/1/b	
'acute or long-term toxicity' Reasoning: quality of life is improved through less toxic medicines; toxicity levels can be measured versus quality of life that has many different definitions Reference: 'The trouble is that, whilst cancer treatments are designed to kill cancer cells, they are often harmful to healthy cells too. This is a particular problem for children, because their bodies are still developing, and their organs have not fully matured which can impact a doctors treatment choice.' ...'Around two in three survivors will have one of these long-term effects, known as 'late effects'.	
'as well as the stakeholders referred to in Article 162' Reasoning: article 162 shall include patients and healthcare professionals	

The revision procedure



Advocacy work timeline

CCI-E actions

EU institutions actions

2020

Revision of the EU Pharmaceutical legislation

CCI-E & SIOPE 6 key recommendations

April 2023

Proposal of **the EU Commission** "the Pharmaceutical Package"

CCI-E & SIOPE joint position paper with proposed amendments

Nov. 2023

Deadline for amendments at the **EU Parliament**

CCI-E policy team meetings with Members of the EU Parliament (MEPs)

Jan. 2024

Negotiations in **Council of the EU**

CCI-E policy team meetings with Health Attachés

April 2024

Plenary vote in the **EU Parliament**

At least + 2 years to go

Conclusion: how can you support our efforts?

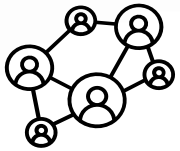
In a wrap..

3 meetings with DG SANT
6 meetings with MEPs
4 meetings with Health Attachés
CY HU IT SE
Meetings with the industry

5 joint positions papers with SIOPE

2 policy events with SIOPE
2 Gold webinars

And what about our members?



You can help us coordinate with your **Permanent Representation to the European Union**

You can meet with your **national Ministry of Health**

You can talk to your **local** politicians

Childhood Cancer International EUROPE

3. Our 6 priorities

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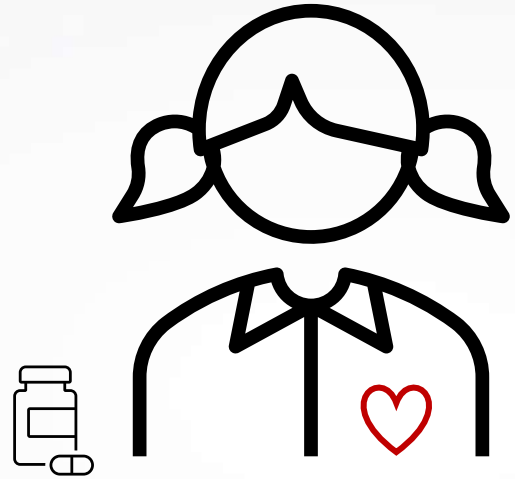
1. Unmet needs of children

Cure rates



Less toxic medicines

Quality of life

Less acute & long-term side effects

Current state of play

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6 meetings with MEPs
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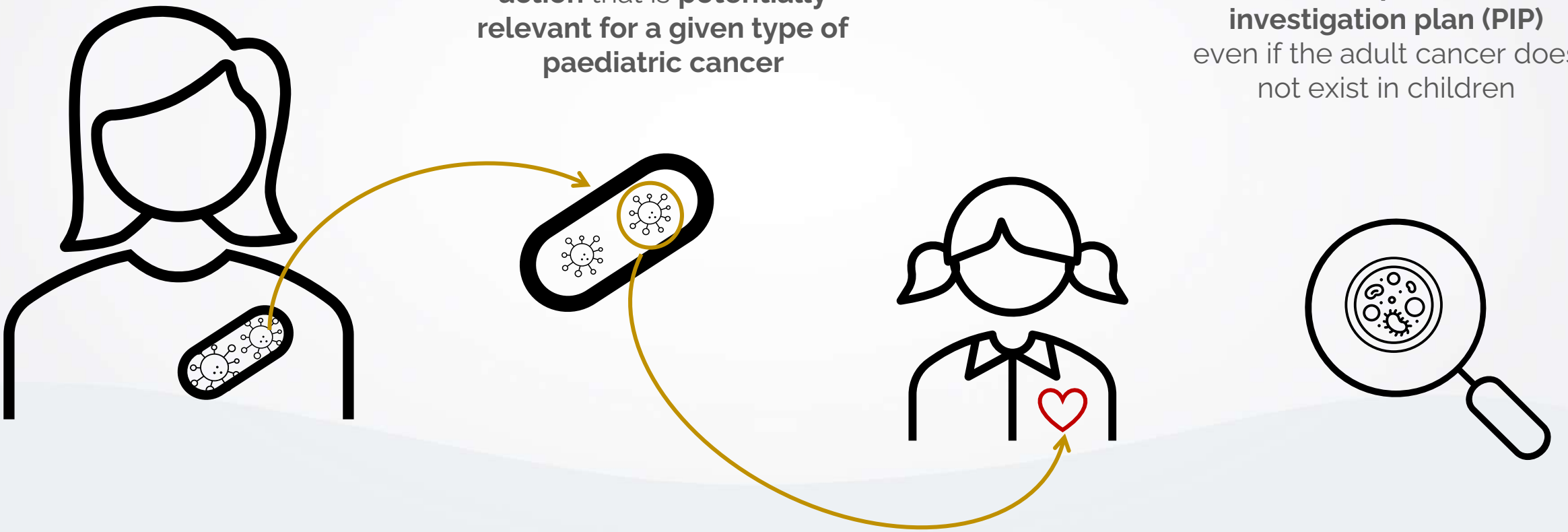
1. Unmet medical needs of children

- EU Commission
- EU Parliament
- Council

2. Mechanism of action

Drug has a **mechanism of action** that is **potentially relevant for a given type of paediatric cancer**

Drug sponsor **will have to submit a paediatric investigation plan (PIP)** even if the adult cancer does not exist in children



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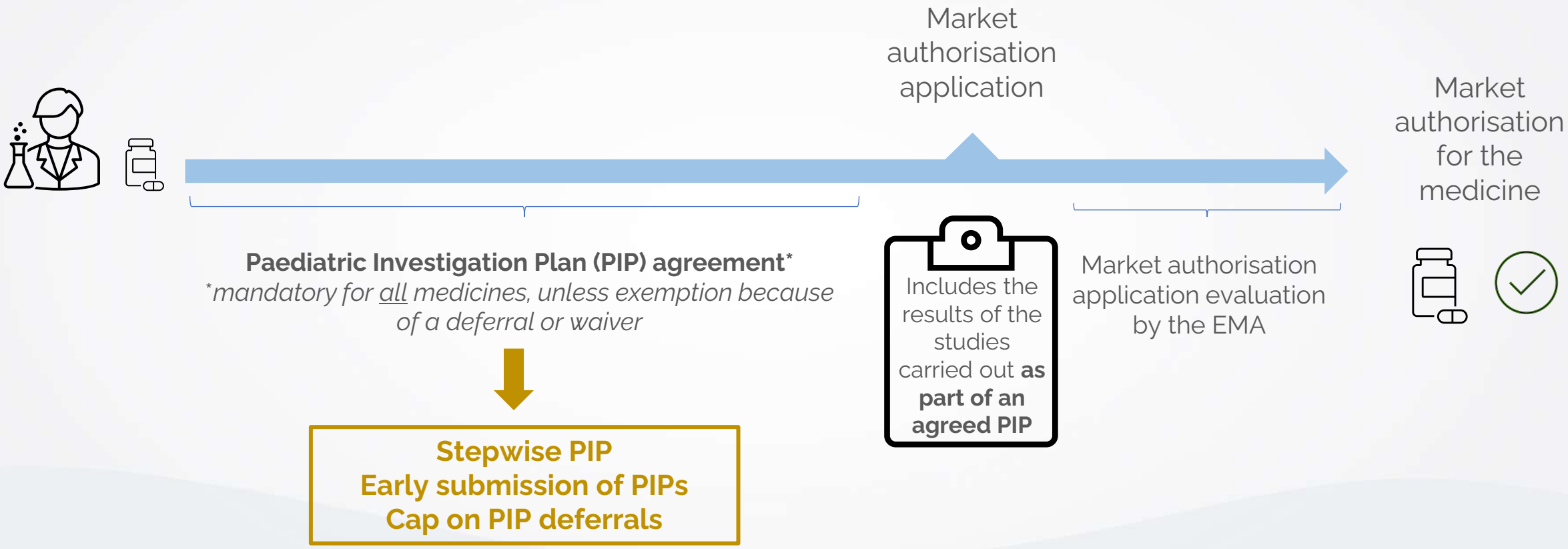
1. Unmet medical needs of children

- EU Commission
- EU Parliament
- Council

2. Mechanism of action

- EU Commission
- EU Parliament
- Council

3. Simplification of investigation plans & early start



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1. Unmet medical needs of children
   - EU Commission
   - EU Parliament
   - Council

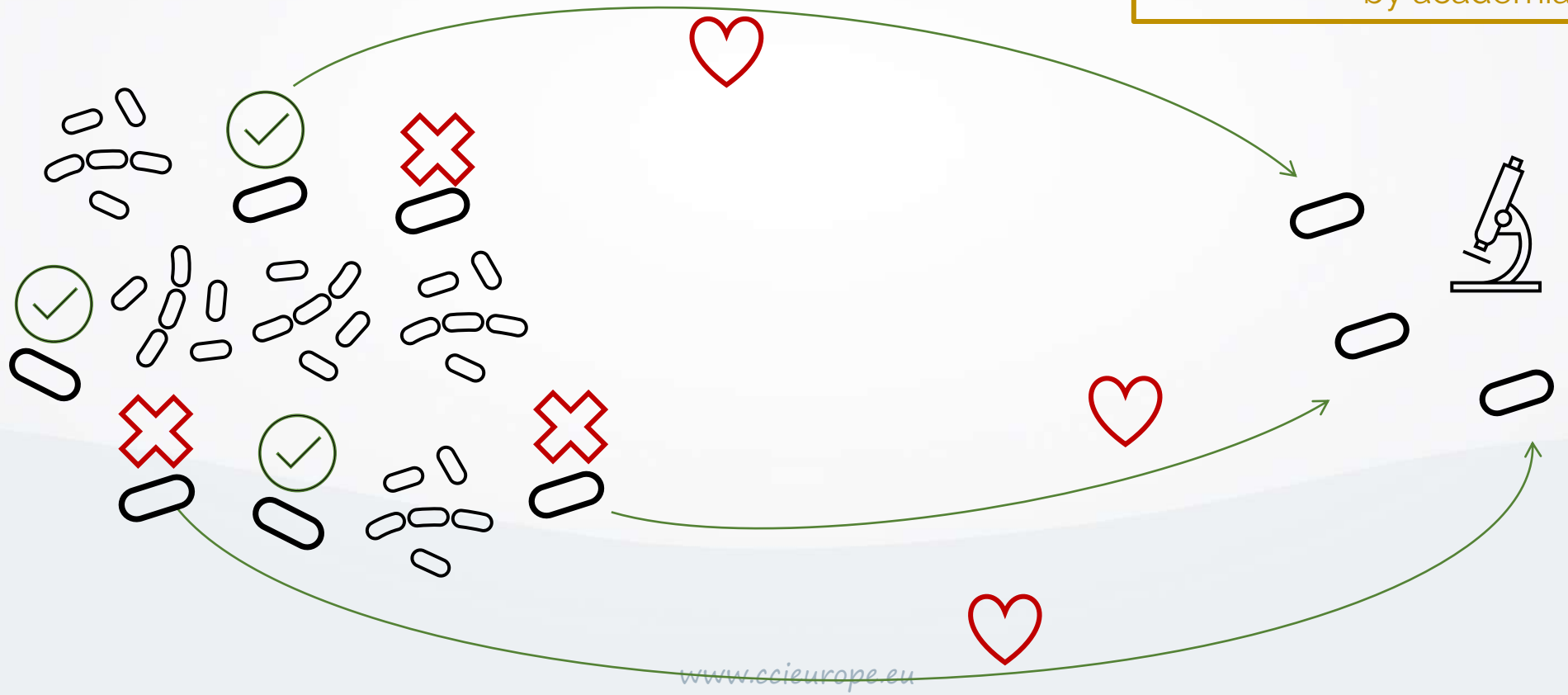
2. Mechanism of action
   - EU Commission
   - EU Parliament
   - Council

3. Early start of paediatric development
   - EU Commission
   - EU Parliament
   - Council

4. Academic repurposing of drugs

Drugs in development in adult oncology

Repurposing of drugs, shelved or developed for other indications, for the treatment of paediatric diseases by academia



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1. Unmet medical needs of children
   - ✓ EU Commission
   - ✓ EU Parliament
   - ? Council

2. Mechanism of action
   - ✓ EU Commission
   - ✓ EU Parliament
   - ? Council

3. Early start of paediatric development
   - ✓ EU Commission
   - ✓ EU Parliament
   - ? Council

4. Repurposing of drugs
   - ✓ EU Commission
   - ✓ EU Parliament
   - ? Council

5. Improved access to medicines

Essential anti-cancer medicines

Newly developed medicines

20 %

Gap in survival rates among European countries

Dedicated incentive/procedure to ensure equal access in all Member States & mechanisms to address shortages

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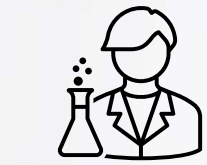
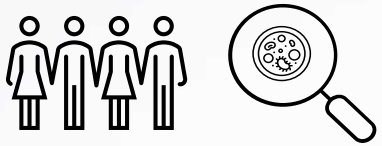
1. Unmet medical needs of children	✓ EU Commission ✓ EU Parliament ? Council
2. Mechanism of action	✓ EU Commission ✓ EU Parliament ? Council
3. Early start of paediatric development	✓ EU Commission ✓ EU Parliament ? Council
4. Repurposing of drugs	✓ EU Commission ✓ EU Parliament ? Council
5. Improved access to essential & anti-cancer drugs	✓ EU Commission ✓ EU Parliament ? Council

6. First-in-child drugs

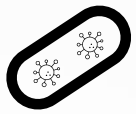


Pharmaceutical industry

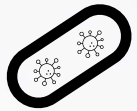
Drug development in adult oncology



Drug authorised & marketed for adult cancer



Drug authorised & marketed for paediatric use



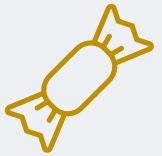
First-in-child drugs

Drug development <u>specifically</u> in paediatric oncology

TODAY

- **Economically unattractive**
- **Small** and **high-risk** patient population
- **Complexities** associated with paediatric cancers (heterogeneity, need of age-specific formulation, clinical trials design for children etc.)

Need incentives to increase commercial interest



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2. Mechanism of action	✓ EU Commission ✓ EU Parliament ? Council
3. Early start of paediatric development	✓ EU Commission ✓ EU Parliament ? Council
4. Repurposing of drugs	✓ EU Commission ✓ EU Parliament ? Council
5. Improved access to essential & anti-cancer drugs	✓ EU Commission ✓ EU Parliament ? Council
6. First-in-child drugs	✗ EU Commission ✗ EU Parliament ? Council

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4. Q&A

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4 Let's learn & play!

- Join Menti.com and insert code **2407 2264** or scan

- Play and have fun!

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Any questions?

Do not hesitate to reach out to :

t.pais@ccieurope.eu & m.gouders@ccieurope.eu

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Give us your feedback

Feedback form: Drug Regulations – What is new for the childhood cancer community?

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SAVE THE DATE

NEXT WEBINAR IS:

What could be the impact of Big Data and AI application in paediatric oncology?

On July 1st , 2024

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Childhood Cancer International EUROPE

June 29th , 2024

Childhood Cancer International EUROPE

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