



SEPTEMBER  
CHILDHOOD CANCER  
AWARENESS MONTH

**GOLD** *for* **HOPE:**  
**CURE MORE AND  
CURE BETTER**



**‘Championing the Paediatric Regulation  
in the future revision of the EU pharmaceutical  
legislation’**

**Pamela Kearns**

**SIOPE Europe Immediate Past President**

MAC-SIOPE Webinar for Gold September  
*29 September 2022*



# A UNIQUE OPPORTUNITY FOR LIFE-SAVING CHANGE



## ❑ Important unmet needs of children and adolescents with cancer

- Access to **therapeutic innovation** in an area of relative market failure

## ❑ EU Paediatric Regulation

- European Parliament resolution from 2016 **called for targeted revision**
- European Commission recognised **the unmet needs in paediatric cancer** in the 2020 evaluation of the Regulation
- The **Pharmaceutical Strategy for Europe** also recognised in 2020 the need to revise the Paediatric Regulation

**The upcoming revision of the EU Paediatric Regulation  
is a unique opportunity to accelerate innovation for children with cancer**



# 6 RECOMMENDATIONS



## European Childhood Cancer Organisations' Recommendations

following the European Commission's Evaluation of the Legislation for Medicines for Rare Diseases and Children and the launch of the Pharmaceutical Strategy for Europe.

1. **Align the Regulations** with Science and **Unmet Needs** of Children
2. Ensure Child-Specific and **First-In-Child Innovation**
3. Implement **Multi-Stakeholder Cooperation** and Prioritisation as a Standard
4. Allocate **Public Investment** in Medicine Development for Children
5. **Align Global** Regulatory Environment
6. Ensure **Equal Access** to Essential & Novel Anticancer Medicines & to Supportive Care Medicines



# TANGIBLE ACTIONS



Align the Regulations with Science and **Unmet Needs** of Children

- ❖ **Focus on the targeting the right drug to the right disease (mechanism of action approach)**

Ensure Child-Specific and **First-In-Child Innovation**

- ❖ **Appropriate incentives balanced with obligations**

Implement **Multi-Stakeholder Cooperation** and Prioritisation as a Standard

- ❖ **ACCELERATE Paediatric Strategy Fora** as exemplars

Align **Global** Regulatory Environment

- ❖ **EMA-FDA aligned decision-making on paediatric cancer drug development plans**



Thank you very much for your valued attention and instrumental support!

