

Consultation in relation to the Paediatric Report

Ref. PCPM/16 – Paediatric Report

Part I - General Information about Respondents

Your name or name of the organisation/company: **Det'om s rakovinou n.o.**

Transparency Register ID number (for organisations): **404581225774-42**

Country: **Slovakia**

E-mail address: **info@detomsrakovinou.sk**

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Please indicate whether you are replying as:

- A citizen
- A business
- A non-governmental organisation (NGO)***
- An industry association
- A patient group
- A healthcare professional organisation
- Academia or a research or educational institute
- A public authority
- Other (please specify)

If you are a business, please indicate the size of your business

- Self-employed
- Micro-enterprise (under 10 employees)
- Small enterprise (under 50 employees)
- Medium-sized enterprise (under 250 employees)
- Large company (250 employees or more)

Please indicate the level at which your organisation is active:

- Local
- National***
- Across several countries
- EU
- Global

Part II – Consultation items

(You may choose not to reply to every consultation items)

1.1. More medicines for children

Consultation item No 1: Do you agree that specific legislation supporting the development of paediatric medicines is necessary to guarantee evidence-based paediatric medicines?

There is not much attention paid on paediatric medicines in European Union, especially medicines on child cancer. The change of legislation is more than necessary. More than 50 percent of medicines have never been investigated in children. Drug companies are interested much more in adults because of unattractive business with medicines for child cancers. We are calling for change. As one of the national organizations representing the interests of children with cancer. We ourselves are parents of children, who or are still fighting a battle with cancer. And we see many others that lose their fight, because there is no appropriate treatment for them.

Paediatric Medicines Regulation currently does not address effectively children suffering from cancer. And we would like to see it changed.

1.2. Mirroring paediatric needs

Consultation item No 2: Do you have any comments on the above? To what extent and in which therapeutic areas has the Regulation contributed to the availability of important new treatment options?

For example, the number of childhood cancer diagnosis are likely to be cured, but various of drugs have been tested on adults only.

Our proposal is to introduce stricter rules for drug companies to be forced to test drugs for cancer of children's diagnosis. It is known that even if the origin of childhood cancer is different to adults, a drug that can cure lung cancer in an adult, can save a child with neuroblastoma.

1.3. Availability of paediatric medicines in the EU

Consultation item No 3: In your experience, has the number of new paediatric medicines available in Member States substantially increased? Have existing treatments been replaced by new licensed treatments?

As we have written before, we do not see a significant increase in paediatric medicines for cancer. There do not exist any regulations for medicines on child cancer as it is in adults.

1.4. Reasonable costs

Consultation item No 4: Do you have any comments on the costs for pharmaceutical companies to comply with an agreed paediatric investigation plan?

The system should be more efficient, to receive incentives to those who work on development in paediatric medicines.

1.5. Functioning reward system

Consultation item No 5: Do you agree that the reward system generally functions well and that early, strategic planning will usually ensure that a company receives a reward?

Reward investment in paediatric cancer drugs development is not proportional when we compare it with cancer drugs for adults.

1.6. The orphan reward

Consultation item No 6: How do you judge the importance of the orphan reward compared to the SPC reward?

As we have written, drug companies do not regard medicines for child cancer as financially attractive. Instead, we believe that an improved PIP process should be the main route for developing paediatric medicines.

1.7. Improved implementation

Consultation item No 7: Do you agree that the Regulation's implementation has improved over time and that some early problems have been solved?

We're not sure if it has fulfilled sufficient purpose. Medicines for children with cancer are still not adequately developed. There are no strict rules that can help control the mechanism and the system with these specific paediatric medicines at all.

1.8. Waivers and the 'mechanism of action' principle

Consultation item No 8: Do you have any comments on the above? Can you quantify and qualify missed opportunities in specific therapeutic areas in the last ten years?

We know about a number of critical opinions of experts who say that a childhood cancer is not such a problem revealing some mutations of the disease as in adults. It follows that developing of paediatric medicines should not be so demanding, both financially and timely.

For example, the view of Professor Andy Pearson and his colleagues:

“Whilst the Regulation has brought positive change and advances, the waiver mechanism means that with over 60% of 89 potentially valuable anticancer drugs granted a waiver, there are still few paediatric trials and only between 9% and 15% of all oncology agents have ongoing paediatric studies.

It is critically important to realise that the average number of non-synonymous coding mutations in childhood tumours is on average about a hundred-fold lower than in adult malignancies. This means that the likelihood of correctly identifying the Achilles' heel' of the tumour for targeted therapies is much higher, thus, comprising a much more promising and clean target population for Mechanism of Action based drugs to actually work.”

1.9. Deferrals

Consultation item No 9: Do you agree with the above assessment of deferrals?

Yes, we agree. The drug development is significantly delayed compared to that for adults.

1.10. Voluntary paediatric investigation plans

Consultation item No 10: Do you have any comments on the above?

The system without any strict rules based only on volunteering and incentives could not work and address needs of children with cancer. Community, drug companies, experts, European institutions should do everything to support development of new drugs for saving lives of more and more children with cancer.

1.11. Biosimilars

Consultation item No 11: Do you have any comments on the above?

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1.12. PUMA — Paediatric-use marketing authorisation

Consultation item No 12: Do you share the view that the PUMA concept is a disappointment? What is the advantage of maintaining it? Could the development of off-patent medicines for paediatric use be further stimulated?

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1.13. Scientifically valid and ethically sound — Clinical trials with children

Consultation item No 13: Do you have any comments on developments in clinical trials with children following the adoption of the Regulation and in view of the above discussion?

Research should also be seen in children as a step forward. Every child and parent are informed that research is about risks and benefits that have been assessed.

1.14. The question of financial sustainability

Consultation item No 14: Do you have any views on the above and the fact that the paediatric investigation plan process is currently exempt from the fee system?

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1.15. Positive impact on paediatric research in Europe

Consultation item No 15: How do you judge the effects of the Paediatric Regulation on paediatric research?

We do not have any knowledge of the significant development or impact or move forward.

1.16. “Mirror, mirror on the wall” - Emerging trends and the future of paediatric medicines

Consultation item No 16: Are there any emerging trends that may have an impact on the development of paediatric medicines and the relevance of the Paediatric Regulation?

We are full of expectation in the shift in molecular medicine. Every trend in modern medicine and its research, which is relevant for child cancer is welcome.

1.17. Other issues to be considered

Consultation item No 17: Overall, does the Regulation’s implementation reflect your initial understanding/expectations of this piece of legislation? If not, please explain. Are there any other issues to be considered?

Our NGO support the recommendations in the Position Statement by SIOPE, Unite2cure and Cancer

13 Research UK:

1. Ensure that the obligation to undertake a Paediatric Investigation Plan is based on how a drug works and its capacity to address an unmet medical need in children - rather than the type of disease in adults for which it is first introduced.
2. Set up a mechanism to choose the best potential drugs and prioritise, among drugs developed by different companies, in relation to the real needs of children affected by rare cancers.
3. Reduce delays in paediatric medicines reaching children by enabling Paediatric Investigation Plans to start not later than the start of pivotal trials in adults, if paediatric biological, preclinical and preliminary clinical data are available to better evaluate the potential therapeutic benefit in the paediatric population.
4. Add provisions for more effective and flexible rewards for companies undertaking early and

timely Paediatric Investigation Plans and those researching therapies specifically for cancers which only occur in children

To this, we would add a further item:

Introduce flexible ages of entry to adult trials based on considerations of biology and safety