MAC–CRUK–SIOPE Event

'Review of the EU Paediatric Medicines Regulation - Let's do more for children with cancer'

15:30 – 17:00
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Parents' perspective

When YOUR child has a disease such as cancer, his parents, sisters and brothers, cousins, grand parents, his schoolmates are ALL affected. The whole community is affected for a VERY long time.

We, at **UNITE2CURE,** an European alliance of parents and non profit organizations representing 11 European countries, come as parents**,** to tell you that there is **an urgent need** to give access to innovation for children and adolescents with cancer.

We **can no longer accept the situation as it is today**

How families experience the paediatric regulation? Let me speak to you about it from 3 perspectives:

- First: The lack of childhood cancers drug development is JUST inacceptable
- Second: our assessment after 10 years of the regulation is CLEAR: it is not efficient for paediatric cancers.
- Third: We need to ACT - NOW

1 - The lack of childhood cancers drug development is JUST inacceptable

Here are 3 short stories and 2 figures:

The 1st story is mine and it is also the one of many families whose child is diagnosed with a high risk cancer.
My daughter Margo was 14 years old when she was diagnosed with an aggressive brain tumour. Her doctor told us, from the beginning, that they did not know how to cure her disease.
They had **nothing else** to give her than a **40 years old** chemotherapy, very toxic treatment, developed for adults, not authorised for children.

Can you believe this? Nothing better than a very old drug, nothing new, even for a non curable disease, no clinical trial, no innovative drug, NO HOPE and believe me, we looked everywhere!

Well, the situation has not changed today, we still **DO NOT HAVE** new paediatric oncology drugs.

Is that situation ACCEPTABLE?

I would also like to tell you about Chloe. Chloe was living in London when she was diagnosed with an Ewing sarcoma.
BUT Chloe had a problem: she was 17 and 9 months and she had to be 18 to access a quite promising clinical trial.
Chloé died at the age of 18 and 2 months. We will never know if that innovative trial might have saved her

So within UNITE2CURE, we wondered: is there any legal, ethical but mostly any MEDICAL reason for a teenager NOT to be allowed to enter an adult clinical trial?
Well, there are **NO reasons**.
We just need to change mind-sets.

Is this ACCEPTABLE?

My 3rd story is about Raphael, a little boy from Belgium who was 9 years old when he was diagnosed with an alveolar rhabdomyosarcoma. After one year of intensive treatments Raphael was in full remission BUT he had to suffer a foot amputation. Two years later, unfortunately, he relapsed and he is in treatment again for one full year of chemotherapy and radiations on his lungs.
Raphael is alive BUT; with foot amputation, severe lungs damage, fertility affected, high risk of adult leukaemia . That is today, the price Raphael has to pay to survive.

Is that ACCEPTABLE?

We need innovation to cure MORE CHILDREN and to cure them BETTER.

I want to make this really clear…. I am NOT talking with you about a rare or insignificant disease!

We are talking about **the number one cause of death by disease** for children in Europe…

specifically **35 000** young people are diagnosed with cancer every year in Europe
…..and……**6000** of them will die each year…

What does it represent?

When you see how much echo a single school bus accident is having in the news headline…well that's ONE HUNDRED.AND.SIXTY busses (1_6_0) full of school children that crash off the side of the road!
– all dead, no survivors – and yet **NO ONE IS TALKING ABOUT IT**.

SO, WE WILL.

because **childhood cancers are JUST unacceptable.**

My second point is:

2 –**For us parents, the assessment after 9 years is that the PMR is CLEAR: it is NOT efficient for paediatric cancers**

Yes…the vote of the Regulation in 2007 was a real success and the Regulation has made a big change to push industry to **consider** paediatric development. And we would like to really thank Mrs Grossetête the European Deputy who was the reporter of this PMR and is supporting our request for changes.

But unfortunately, to win the battle against childhood cancers **THIS IS NOT ENOUGH**.

ONE THOUSAND new molecules are being developed by industry BUT ONLY FOR ADULTS.
Incredible but after 10 years, there have been only **2 new drugs** developed for children

Too many unjustified waivers are obtained by industries allowing them to avoid the paediatric development of their new drug.

Why ? because :
Waivers can be applied for a new drug when the disease occurs "only in adult population".

Lung cancer, for instance, occurs only in adults.
So you can apply for a waiver because children do not have lung cancer.
BUT what if the action of that lung cancer drug could benefit some paediatric diseases such as some lymphomas, some sarcomas, or neuroblastoma?
This is how crizotinib, a new drug for lung cancers received a waiver, and was not developed for children.
We had to wait for 4 years before the 1st child was included in a clinical trial, …. with very good results.

What a waste of time ! What a waste of LIVES !

Waivers should not be given on the basis of the disease but on the basis of how the drug works: on the mechanism of Action of the drug.

The 2nd aspect of the regulation I want to tell you about are Incentives.

Today, the regulation is seen as a burden and NOT as an opportunity.

The PMR recommends to start the Paediatric Investigation Plans (PIP) after phase 1 of adult development.
In oncology, it never happens. Most PIP start just before the adult market application; on average, 4 months before.
And there are no sanctions if a PIP starts late…so why would anyone rush on paediatric?.

What a waste of time ! What a waste of LIVES !

Let's imagine a process in which if the earlier you start the paediatric development, the earlier you could get a reward? Could the 6 months incentive be split and you could get the reward at earlier stage?

LET'S imagine a process where as , like in the US, by developing a drug specifically for a rare paediatric disease you could get an even BIGGER reward?

My 3rd message to you today is that:

3- WE need to ACT - NOW

We want YOU and we want the European Commission to consider changes in the PMR based on the 4 proposals we have made, working together for the last 2 years, within the ACCELERATE platform and with SIOPE and UNITE2CURE.

Because, what do we want ?

We want :
-Drug development based on the Mechanism of Action of the drug
-best possible drugs for children
-Early start in the paediatric drug development
-More efficient incentives

We believe that our concrete proposals will dramatically and quickly change the situation.

Pr Pamela Kearns will develop those proposals in a few minutes.

And this, is what we need NOW

Why ? because :

First, **Child3en are NOT mini adult**: they deserve adapted and specific treatments.

And it should be a PRIORITY. Again, we are talking about the 1st cause of death by disease for children in Europe!

Second, when you are parents of a critically ill child, time counts in months, in days, in minutes, to continue holding your child hand.

So, I am asking you :

What is more important than taking care of 35 000 young people per year,

What is more important than SAVING LIVES of 6 000 children and adolescents every year?

Time for a change is now

And YOU can make it happen