All children on Board!

Why the Paediatric Regulation must be amended to benefit all children

Accelerate Conference
February 8th, 2018
Presentation’s objectives

• State of play for children with cancer: specific urgent needs;
• Mitigated success of the Paediatric Medicines Regulation: call for amendments;
• No impact of Orphan Drugs Regulation;
• What’s next?
State of play for children with cancer:

SPECIFIC URGENT NEEDS
Paediatric cancers are a public health concern

1. 1st cause of death by disease**

Some cancers are so rare that they receive little attention, despite a very poor cure rate (below 50%).

6 000 deaths**
/year

2. Every kid cancer is a rare disease

60 different paediatric malignancies, each of them requiring a specific scientific attention.

35 000 new diagnoses*
/year

* Children, adolescents and young adults up to 24 years
** Among children aged 1 year or more
QoL of survivors and cure rate must improve

3. Cure rate stalled over last 15 years

Curing cancer is making progress, except with children.

4. High-risk=poor outcome

The cure rate of certain high risk cancers remains shockingly low. For those, cancers, we need new drugs.
Innovation does not reach children who most need it

5. 2/3 survivors suffer long term side effects

Survivors will represent about half a million people in Europe by 2020. LTEs must be prevented and reduced.

Blindness, hearing loss
Amputation (foot, leg, hand...)
Organ removal (kidneys)
Coronary or artery disease
Cardiac failure
Secondary cancers
Post-traumatic stress disorders

6. Adults 70 – Kids 2

From 2011 to 2015, 70 new anti-cancer drugs were approved for adult cancers. For kids, only 2.

Source: IMS Health, MIDAS, Lifecycle, R&D Focus, IMS Institute for Healthcare Informatics, Dec 2015
Paediatric cancer is not seen as a profitable market

1. Less than 1% of all cancers are paediatric
   And yet that 1% needs to be split again into 16 different cancers and 60 different sub-types...

2. Cancer is still an acute disease (<> chronic)
   With the aim of curing cancer, treatments are to be administered for one to two years at most.

3. Children with cancer still have specific needs
   - Age-adapted formulas
   - Age-group specific toxicities
   - Patients recruitment complexity

Mitigated success of the Paediatric Regulation (PMR):

CALL FOR AMENDMENTS
The review report notes the PMR’s weaknesses

- Regulation works best where the needs of adult and paediatric patients overlap
- The **SPC reward** is most attractive in areas where the drug is a **blockbuster in adults**
- The PMR only has **limited possibilities to steer developments towards certain specific areas**
  - Unmet medical needs tend to be overlooked...
- The **article 11 waiver** poses problems where the compound can be beneficial for children, albeit in a different condition
Cancer is a problematic area when it come to waivers

• The requirement for a PIP may be waived if the disease or condition for which the product is intended occurs only in adults.

• While many paediatric cancers share biological similarities with adult cancers, they occur in different organs and are therefore usually considered as different conditions

**PIPs will only occur on a voluntary basis** even when the mechanism of action of the compound is expected to be effective in paediatric cancers
Call for a moral stance

In Europe, children should not have to wait for voluntary initiatives to access life-saving drugs developed in adults.
Most waivers are granted in oncology

- **214** All class waivers
- **154** Class waivers in oncology
- **147** Approved class waivers in oncology

PDCO minutes from June 2012 to June 2015 + Literature search then blinded panel of 16 ITCC experts
Most drugs waived could benefit children with cancer

- Relevant: 93
- More data needed: 25
- No existing data: 20
- Not relevant: 9

PDCO minutes from June 2012 to June 2015 + Literature search then blinded panel of 16 ITCC experts
We are calling for two amendments

1. **Significant therapeutic benefit** (including the relevance of its mechanism of action) over existing treatments in the paediatric population.

2. **Paediatric Strategic Forums** confirmed in the law.
... and an improved implementation

3. **No more delays**: PIPs to be submitted early.
No impact of Orphan Drugs Regulation (ODR):

NEW COMMERCIAL PARADIGM REQUIRED
ODR and PMR serve completely different purposes

Paediatric Medicines Regulation → Innovation for adults brought to children

Orphan Drugs Regulation → Policy tool to foster innovation in any rare disease
ODR aim is to foster an interest in rare conditions

- A rare condition = prevalence of less than 5 of 10,000
- Each paediatric cancer = rare condition
~40% of MAs under ODR are in oncology

Though paediatric cancer generated little interest under ODR.
Though they generated little interest under ODR.
Only 10 compounds have an authorised paediatric indication

Focus on drugs for conditions occurring both in adults and children
10-years review process: mixed feelings:

OUR PLAN GOING FORWARD
### Mixed feelings about the 10-year report

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<tr>
<th>Doubtful</th>
<th>Satisfied</th>
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<td>• Any amendments are deferred until 2019;</td>
<td>• Specific needs of children with cancer are recognised;</td>
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<td>• Implementation of waivers on basis of mechanism of action is seen as possibly impacting the predictability of the scope of a PIP and a risk to the overall product development.</td>
<td>• Policy on deferrals will be reviewed to speed up access to innovation;</td>
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<td>• EU Commission will propose implementation improvements of the PMR.</td>
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What happened since the report’s publication?

1. 10-y report presentation conference

2. Open letter to Health Commissioner  
   40 organisations from 23 countries!

   Monday, 4th of December 2017

   Dear Mr. Andriukatis,

   We are the proud parents and representatives of beautiful, funny, noisy children. Some of them live in our hearts, some of them survived cancer after a long and toxic treatment.

   When a child is diagnosed with cancer, people often comfort parents by invoking progress made in cancer treatments.

   The scandal is that in Europe, curing cancer is making progress, except for children.

3. Consultation on SPC – IP & Bollard exemption

   Public consultation on Supplementary Protection Certificates (SPC) and patent research exemptions for sectors whose products are subject to regulated market authorisations.

   Fields marked with * are mandatory.

4. Evaluation of the Commission’s roadmap

Our activity lead to media attention
Our next steps: hold and build the fort!

• Participate in the working sessions organised by the EU Commission and EMA

• Keep the media pressure

• Participate in the assessment of the SPC review

• Any suggestions welcome...
THANK YOU FOR YOUR ATTENTION AND CONTRIBUTION