SIOP Europe
the European Society for Paediatric Oncology

International Childhood Cancer Awareness Day

Creating a brighter future for young people with cancer

International Childhood Cancer Awareness Day 2016

**Development of paediatric cancer medicines**

***'Speeding up Innovation, Saving Lives'***

**Hosted by MEP Glenis Willmott (S&D, UK)**

**Wednesday 27 January 2016**

# Academic perspectives on current status and ways forward


## Gilles Vassal, Gustave Roussy

# New oncology drug development for children and adolescents

- Status in 2015
  - A significant change of the environment, thanks to the EU pediatric regulation (2007)
    **80 PIPs for a malignant indication**
  - But we are very far from addressing the needs:

≈ Less than 1 in 10 children with a non curable relapsed maligancy has access to an innovative therapy in Europe

**Cancer = 1st cause of death by disease >1 year**

# Multistakeholder Paediatric Oncology Platform

**December 2013**

Creating a unique, multi-stakeholder Paediatric Oncology Platform to improve drug development for children and adolescents with cancer

Eur J Cancer 2015;51:218.

Gilles Vassal [a,*], Raphaël Rousseau [b], Patricia Blanc [c], Lucas Moreno [d], Gerlind Bode [e], Stefan Schwoch [f], Martin Schrappe [g], Jeffrey Skolnik [h], Lothar Bergman [i], Mary Brigid Bradley-Garelik [j], Vaskar Saha [k], Andy Pearson [l], Heinz Zwierzina [m]

- **Goal**: to improve oncology drug development for children and adolescents
- **Principle**: **ALL stakeholders**
  - Academia, Industry, Parents,Regulatory


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gsk GlaxoSmithKline
CDDF Cancer Drug Development Forum
Innovative Therapies for Children with Cancer ITCC European Consortium
SIOPE SIOP Europe the European Society for Paediatric Oncology
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# Why?

- **There are unjustified Class waivers**
  - The adult condition does not exist in children
  - June 2012 – June 2015
    214 class waivers discussed by PDCO
    72% in oncology - 89 oncology drugs class waived

**Example – Crizotinib was class waived**

- **Lung cancer does not exist in children,**
- **but ALK (crizotinib target) is present in neuroblastoma, lymphoma, sarcoma in chidlren**

# Why?

- **There are still major delays**

## Example – PD1 inhibitors

| | | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 |
|---|---|---|---|---|---|---|---|---|---|
| **Nivolumab** | Adults | | | | | | | ● | ● |
| | Children | | | | | | | ★ | |
| **Pembrolizumab** | Adults | | | | | | | ● | ● |
| | Children | | | | | | | ★ | |

● Marketing autorisation in melanoma US

● Marketing autorisation in melanoma EU

★ Approved Pediatric Investigation Plan

**Pediatric developments started after first market approvals in adults**

# Why?

- **There are unfeasible PIPs**
  - **Example : Vemurafenib PIP EMA/193393/2011**

| | | |
|---|---|---|
| Clinical | 1 | Study 2<br><br>Open-label, multicentre, single-arm trial to evaluate the recommended dose, safety, pharmacokinetics and response of RO5185426 in paediatric patients from 12 to less than 18 years old with BRAF V600 mutation positive unresectable stage IIIC or stage IV melanoma. |

Trial Start – January 2011
26 investigating sites, 10 countries, 4 continents
As of December 2015, 6 adolescents in trial

# Proposals

1. Pediatric development should be based on drug **mechanism of action** instead of adult indication
2. **Prioritisation** should be set up to choose compounds to be evaluated or not in children
   - Based on MOA, needs, feasibility
   - Using stonger biological and preclinical data
   - Done through multistakeholder forum
3. **New incentives and rewards**

# Conclusion

- Changes in the Regulation
- Change mindset
- Incentivise work together
- Move  pediatric oncology drug development from regulatory compliance only to R&D

GOAL – **Accelerate** new oncology drug developement for children and adolescents

# Back up slides

Lancet Oncol. 2015 Sep;16(9):e425-6. doi: 10.1016/S1470-2045(15)00233-8.

# Will the revised class waiver list make it?

Vassal G[1], Blanc P[2], Copland C[3], Pearson A[4].

*Panel*: Class waivers for medicines for the treatment of lung cancer

- In the current class waiver list: treatment of lung carcinoma (small cell and non-small-cell carcinoma)
- In the revised class waiver list: the class of first-generation taxoid and thymidylate synthase inhibitor and pyrimidine-analogue-containing and pyrimidine-analogue-containing and first-generation and second-generation platinum-containing medicinal products for the treatment of lung malignancies

Applied in 2018