ACCELERATE
INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

Interaction of Investigators, Sponsors, Regulators and Parents: The multistakeholder experience in pediatric oncolgy

Gilles Vassal,
Gustave Roussy, France

EnprEMA
European network of paediatric research at the European Medicines Agency

May 16th, 2017

Multistakeholder Paediatric Oncology Platform

To improve new oncology drug development for children

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]

Academia,
Industry,
Parents,
Regulatory Bodies

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www.accelerate-platform.eu


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Unite For Better Treatments for Children and Teenagers with Cancer
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HOW TO CHANGE THE SYSTEM

The ACCELERATE growth and journey

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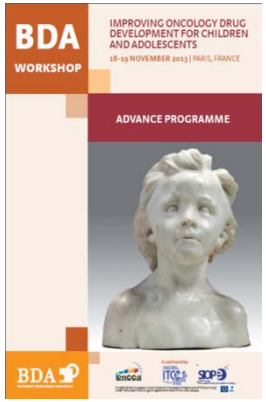
2011 London — Where to go?

2013 Paris — Improving



2015 Vienna — Prioritising

2016 Brussels — Accelerating

2017 Brussels

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PLATFORM

WP16: create a club of « industrial interests »

WG1 – new strategy
WG2 – new incentives
WG3 – long-term follow up

encca
European Network for Cancer Research in Children and Adolescents

CDDF Cancer Drug Development Forum · ITCC · SIOPE SIOP Europe

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No blame! No shame!
Generate data and propose solutions

- WG1: **New strategies** for improved development of oncology drugs for children and adolescent

  lead : Andy Pearson, ITCC

- WG2: **New incentives** for specific pediatric drug development and drug repositioning

  lead : Patricia Blanc, Imagine for Margo

- WG3: Implementation of **long-term follow up** measures of children and adolescents receiving new anticancer drugs

  lead : Raphaël Rousseau, Genentech/Roche

CDDF ITCC SIOPE

CDDF - ITCC - SIOPE 4th Paediatric Oncology Conference

ACCELERATING THE DEVELOPMENT OF NEW ONCOLOGY DRUGS FOR CHILDREN AND ADOLESCENTS

20-21 January 2016
Brussels, Belgium

The 5 objectives defined at the 2016 conference:

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
3. **Reduce delays in starting pediatric development**
4. **Break the 18 years dogma**
5. **New incentives and rewards**

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Achievements 2013 - 2016

WG1

European Journal of Cancer 62 (2016) 1–8

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.ejcancer.com

Current Perspective

Implementation of mechanism of action biology-driven early drug development for children with cancer

Andrew D.J. Pearson [a,*,1], Ralf Herold [b], Raphaël Rousseau [c], Chris Copland [d], Brigid Bradley-Garelik [e], Debbie Binner [f], Renaud Capdeville [g], Hubert Caron [h,i], Jacqueline Carleer [j], Louis Chesler [k], Birgit Geoerger [l], Pamela Kearns [m], Lynley Marshall [n], Stefan M. Pfister [o], Gudrun Schleiermacher [p], Jeffrey Skolnik [q], Cesare Spadoni [r], Jaroslav Sterba [s,t], Hendrick van den Berg [b], Martina Uttenreuther-Fischer [u], Olaf Witt [v], Koen Norga [w], Gilles Vassal [x] on behalf of Members of Working Group 1 of the Paediatric Platform of ACCELERATE[2]

Joint Adolescent - Adult Early Phase Clinical Trials - New Strategies to Improve Access to New Drugs for Adolescents with Cancer

Proposals from the Multi-stakeholder Platform - ACCELERATE

Nathalie Gaspar[1], Lynley V Marshall[2], Debbie Binner[3], Ralf Herold[4], Raphael Rousseau[5], Patricia Blanc[6], Renaud Capdeville[7], Jacqueline Carleer[8], Christopher Copland[9], Yannick Kerloeguen[10], Koen Norga[8,11], Lida Pacaud[7], Marie-Aude Sevaux[12], Cesare Spadoni[13], Jaroslav Sterba[14], Martina Uttenreuther-Fischer[15], Jean-Yves Blay[16], Jean Charles Soria[17], Stan Kaye[18], Laurence Brugières[1], Gilles Vassal[19], Andrew D.J. Pearson[2]*, on behalf of Members of **Working Group 1 of the Paediatric Platform of ACCELERATE**

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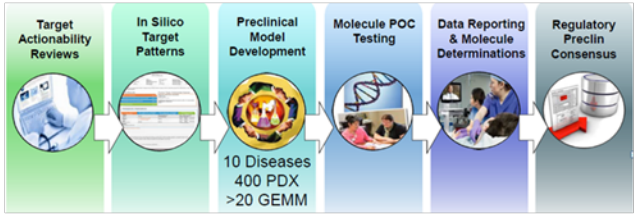
Achievements 2013 - 2016

WG1

ITCC-P4



imi innovative medicines initiative

Pediatric Preclinical POC Platform/ Public Private Partnership

www.pedpancan.com

PedPanCan - A Pan-Cancer Study of Childhood Cancers

Cure rates for childhood cancers have increased to ~80% in the last decades1, but still cancer is the leading cause of death by disease among children over 1 year of age. Furthermore, a large proportion of the surviving children suffer from severe long-term side effects of surgery, cytotoxic chemotherapy, and radiotherapy including mental disabilities and secondary cancers.

Developing more specific therapies with less long-term side effects is thus of paramount importance. One of the crucial steps in this regard is identifying the genetic repertoire of pediatric malignancies, which is being tackled by sequencing cancers from various entities to identify the driving genetic abnormalities within individual entities and across pediatric cancer types.

In our "Pediatric Pan-Cancer" (PedPanCan) study, we inventoried genetic alterations in childhood cancers on multiple levels incorporating structural variations and copy number analysis besides small mutations, both in a somatic and germline context, linking these to mutational signatures, and including an interpretation in terms of druggability. As compared to adult cancers, pediatric cancers are rare and the mutational burden is overall much lower. Thus, this comprehensive catalogue of driving events and potential drug targets is of tremendous scientific value, which is now available to the research community. Insitutions contributing data to this compendium are listed here.

Paediatric Strategy Forums

- **Objective** - provide a unique opportunity for interaction between **all stakeholders** (regulators, pharmaceutical companies, clinical and researcher academics and patient representatives) on topics requiring open discussion in drug development in children and adolescents with malignancy

- **Paediatric Strategy Forum** - scientific meeting to **share** information and advance learning which will inform a paediatric drug development strategy and subsequent decisions

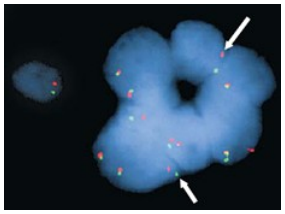
Achievements 2013 - 2016

WG1

✓. **The first Pediatric Strategy Forum**

ALK (anaplastic lyphoma kinase)

Adults



Non Small Cell Lung Cancer
EML4-ALK
(4% patients)

Adults and children

Anaplastic Large Cell Lymphoma

Inflammatory myofibroblastic tumor

ALK fusions

Children

Neuroblastoma

ALK mutation and amplification

Alk inhibition in pediatric malignancies

6 compounds

Crizotini (Xalkori™) - PFIZER

Ceritinib (Zykadia™) - NOVARTIS

Lorlatinib - PFIZER

Alectinib - ROCHE

Entrectinib - IGNYTA

Brigatinib (Alunbrig™) - ARIAD

Pediatric data

Crizotinib and Ceritinib

Activity in ALCL and IMT

Activity in a subset of neuroblastoma

As of January 2017, no PIP

Objectives 2017 - 2020

Three objectives over the next three years:

1. Development of Pediatric Strategy Forums
2. Implementation of the « break the 18y dogma » program
3. Setting up new business models and ways to invest in pediatric oncology research and drug development

These objectives were defined with all participants to the 2017 ACCELERATE conference and agreed upon in the last session of the meeting, and then approved by the ACCELERATE steering committee on March the 16th, 2017 .


conect
4children
COLLABORATIVE NETWORK FOR EUROPEAN
CLINICAL TRIALS FOR CHILDREN

WP4