Widening the ACCELERATE initiative

The French IMPACT platform

*Patricia Blanc, Imagine for Margo, Unite2cure*
2007

the European Paediatric Medicine Regulation

to improve the health of children in Europe by facilitating:

the development and availability of medicines
2017

Significant changes, but not in pediatric oncology

NEED to accelerate

Development and Access to innovative therapies
Each year, in France

- 2500 new cases
- 500 deaths
- 1st cause of death by disease
HOW?

Using a collaborative approach

- Cancer Committee - Leem
- Physicians Researchers
- Patients and families’ associations
AGIR AUTREMENT
POUR VAINCRE
LES CANCERS
DES ENFANTS
ET DES JEUNES ADULTES
Introduction: A collaborative approach

Phase 1...
09/2016: LEEM Cancer committee: PRIORITY 2017 = PEDIATRIC CANCERS

01-02/2017: Collect needs from academics and parents charities

07/2017: Action plan with 3 work priorities
1. Enhance new drugs development
2. Facilitate access to treatment
3. Improve the quality of life for patient and their family

...phase 2

Working groups:
- Regular meetings
- Synthesis by action

global meeting:
- Validate actions
- Define Next steps
SPECIFIC OBJECTIVES?

- To enhance new drugs development
- To facilitate the access to treatments
- To improve the life of patient and their family
Action Plan

Impact 1: Enhance new drugs development

• Elaborate a dynamic analysis of all molecules being developed in oncology: Horizon Scanning
• Ensure fast and qualitative evaluation of Clinical trial authorization requests in pediatric oncology
• *Train young researchers and doctors in pediatric oncology*
• *Mobilize for FAIR trials (include 12-18 years old in adults clinical trials)*
Plan d’actions

Impact 2 : Facilitate access to treatments

• Inventory of off-label used in children drugs and drugs with the temporary use authorizations (ATU) mechanism
• Reduce inequalities in getting access to treatments and clinical trials (U-Link project)
• *Strengthening the Pediatric Oncology Nurse Coordination System*
• *Improve conditions for the implementation of clinical trials for children*
Impact 3: Improve the quality of life of patient and their family

• Mobilize drug companies around “gifts of live” (platelets, bone marrow, umbilical cords, organs...)

• *Reduce pain and anxiety related to care*

• *Expand sports practice during care*
2018 status : where are we ?

- Elaborate a dynamic analysis of all molecules being developed in oncology : Horizon Scanning
- Ensure fast and qualitative evaluation of Clinical trial authorization requests in pediatric oncology
- Inventory of off-label used in children drugs and drugs with the temporary use authorizations (ATU) mechanism
- Reduce inequalities in getting access to treatments and clinical trials (U-Link project)
- Mobilize drug companies around blood donation
1-Elaborate a dynamic analysis of all molecules being developed in oncology: Horizon Scanning

**The Horizon scanning tool**

- **Goal:** develop a tool to select potentially efficient molecules against childhood cancer
- **How:** Match **FDA** target list (Raceforchildren Act) and molecules being developed by pharma (source: LEEM INNOVATION tool)

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**Pediatric Molecular Target List (source FDA)**

| Site Internet GeneCards | BDD INNOVATION (Leem) | X | X |
Ensure fast and qualitative evaluation of Clinical trial authorization requests in pediatric oncology

**ANSM reorganization**
Fast track process implemented
Early discussion meeting

• reduction of delay from 63 days average in December 2017 to 45 days in September 2018

**Ethical committees: reorganization**
New law: October 2018
selection according to competency and availability
3-Inventory of all off-label used in children drugs and drugs with the temporary use authorizations (ATU) mechanism

SACHA project

- **Secured Access to innovative medicines for CHildren with cAncer**

- Collect full information about prescription of off-label used in children drugs and drugs with the temporary use authorizations (ATU) mechanism, given to children in relapse who can not have access to a clinical trial.

- 450 patients, 30 centers
4. Reduce inequalities in getting access to treatments and clinical trials (U-Link project)

Part 1 Clinical trial database
Status 01/2019

- 101 CT on academics side
- 67 CT on family side (easy reading for families)

Part 2 Logistic and financial support for families

- Transportation and accommodation costs for families (routine care and clinical trials)
- 1st results: 156 files - budget 70 000 €
5-Mobilize drug companies around blood donation

Promote « gifts of live » donation: the SRE challenge

• Label creation « drug company supporting “gifts of life” donation (platelets, bone marrow, umbilical cords, organs...)

• Trophy event planned for December 2019
NEXT STEP?
LEEM Foundation main priority
Fighting kids cancer

• Call for projects launched in Jan 2019

• to support NGO acting in pediatric oncology