FAIR Trials Working Group

Fostering Age Inclusive Research

https://www.accelerate-platform.eu/work-programme/ongoing/working-group-fair/why-fair-trials/

Objective 1
To identify successful trials

Objective 2
Awareness Raising to the professionals involved in trial design and approval and the general public

Objective 3
Tools ready to use to facilitate the understanding of the problem and the initiation of trial

Objective 4
Endorsement of the adolescent strategy

Associated members
- National authorities
- Regulatory representatives
- Ethics Committees
- Academics from the main European countries

Core group

Academic drug development
Pediatric and medical oncology

Pharma
- Roche
- Genentech
- BMS
- Novartis

Patient/parent representatives
- AYA
- United2Cure
FAIR Group Action Points

The Survey:
- Early phase 1/2 trials
- Intended for academic PIs for academic trials through ITCC
- Intended for ACCELERATE pharma partners for pharma trials

<table>
<thead>
<tr>
<th>Year</th>
<th>Responses</th>
<th>Trials open</th>
<th>Trials open&lt;2017</th>
<th>No trials:</th>
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<tr>
<td>2017</td>
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<tr>
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<td>4</td>
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</tbody>
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Objective 1

<table>
<thead>
<tr>
<th>Trial phase</th>
<th>Ped. Trial with YA</th>
<th>Adult trial with Ado.</th>
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</thead>
<tbody>
<tr>
<td>Phase 1 / 1b</td>
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<td>0</td>
</tr>
<tr>
<td>Phase 2</td>
<td>3</td>
<td>0</td>
</tr>
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<tr>
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</tr>
<tr>
<td>Phase 2</td>
<td>4</td>
<td>0</td>
</tr>
</tbody>
</table>
European networks

Paediatric oncology

Guidelines
Position paper 2019

Medical oncology

Education book 2018
Website 2019
Article 2019

Paediatric oncologists
SIOP 2017, 2018
ITCC 2018

Medical oncologists
ESMO 2017 et 2018

Education book 2018
E-learning 2018

National networks

Representatives of the main EU countries ....
Meeting and cancer societies

FAIR Group Action Points

Objective 2
Objective 3

National initiatives through ITCC contacts
Contact with paediatric oncologists involved in early drug development and AYA friendly

Carole Lecinse

Objectives = to have …
An overview of national situation on the topic
An action plan to promote adolescent inclusion in early phase adult clinical trial

To identify paediatric and medical oncologists involved in early drug and parent/patient representatives who might be supportive and proactive

To provide contacts and action plan towards ethics committees and competent national authorities

To expand communication and awareness across their own country

To identify places where both adolescent and adult early phase trials could be run (same center or paediatric/adult centers collaborating)

Paper in 2019?

N.Gaspar France
L.Marshall UK
K.Nysom Denmark
AJ.Ribelles Spain
B.Wulff Germany
M.Casanova Italy
Austria, Belgium, Finland, Ireland, the NL, Sweden, Switzerland

Fostering Age Inclusive Research Group
European networks

Paediatric oncology

Medical oncology

Paediatric oncologists
SIOP 2017, 2018
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Education book 2018
Website 2019
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General public

Online
Broadcast media
Face 2 face

FAIR Group Action Points

Objective 2
Raising Awareness

Objective 2
- When safe and justified the design or amendment of clinical protocols targeting both AYA (adolescents & young adults) and adult patients in the same clinical research study can be considered

- Limited awareness on requirements and clinical-protocol specificities to enable AYA inclusion in adult trials can unnecessarily obstruct the inclusion of the AYA population in adult trials.

- The AYA toolkit is intended as a resource to support and guide design of AYA-inclusive clinical studies
- **Regulators** increasingly recognize the need of considering inclusion of adolescents in adult oncology trials to ensure timely access of these populations to innovative medicines.

- FDA released **draft guidance** in 2018 providing recommendations to industry on inclusion of adolescent patients, ages 12 to 17 in adult oncology clinical trials.
Increase awareness of tools or information supporting inclusion of AYA patients can:

- positively influence decision making in consideration of trials including AYA patients

- address scientific-knowledge gaps impacting AYA inclusion in adult trials

- Ensure minimum requirements are in place

AYA Toolkit

- Regulatory aspects
- Protocol elements
- Assent guidance
- Other protocol tools (e.g. PROs)
- List of ongoing AYA friendly trials
- AYA-friendly clinical sites
**Tool Name:**

*Assent requirements*

**Description:**

- The Assent is a form of agreement of someone not able to give legal consent to participate in an activity/clinical research study. The Assent does not substitute the informed consent which is required to be signed by the parent or legal guardian and constitutes the legal documentation of understanding of the implications of taking part in the research, and agreement to take part to it.

**Content example:**

- Protocol sections wording referring to assent requirements
- ENPR-EMA country specific guidance for Informed Consents/Assent in Pediatric Clinical Trials
- List of Lay Terms compiled by EC
- Assent template resources
**Tool Name:**
*Patient Reported Outcomes for AYA (PROs)*

**Description:**
PROs have been defined as "any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else." In other words, PRO tools measure what patients are able to do and how they feel by asking questions. These tools enable assessment of patient-reported health status for physical, mental, and social well-being.

**Content example:**
Example of Oncology PROs with versions suitable for AYA patients

<table>
<thead>
<tr>
<th>Adolescent-Ready PRO Assessment</th>
<th>Matching Adult PRO Assessment</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pediatric MDASI</td>
<td>MDASI</td>
<td>Pediatric MD Anderson Symptom Inventory</td>
</tr>
<tr>
<td>Pediatric PRO-CTCAE</td>
<td>PRO-CTCAE</td>
<td>Pediatric Patient-Reported Outcomes Common Toxicity Criteria for Adverse Events</td>
</tr>
<tr>
<td>EQ-5D-Y</td>
<td>EQ-5D-5L</td>
<td>A EuroQoL health status measure</td>
</tr>
<tr>
<td>Peds FACIT-F</td>
<td>FACIT-F</td>
<td>A fatigue measure</td>
</tr>
<tr>
<td>Peds FACT-Br</td>
<td>FACT-FBrSI</td>
<td>A brain cancer symptom measure</td>
</tr>
<tr>
<td>Peds-FAACT</td>
<td>FAACT</td>
<td>Anorexia and cachexia measure</td>
</tr>
<tr>
<td>...</td>
<td>...</td>
<td>...</td>
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</tbody>
</table>

EuroQoL=European quality of life; FACT(-Br), (-F)=Functional Assessment of Chronic Illness Therapy (−Brain Cancer (−Fatigue); FAACT=Functional Assessment of Anorexia and Cachexia Therapy
National initiatives through ITCC contacts

Pediatric and Adult sites

FRANCE

UK

DENMARK

SPAIN

FAIR Group Action Points

Fostering Age Inclusive Research Group
FAIR Group Action Points

National initiatives through ITCC contacts

Pediatric and Adult sites

GERMANY

- Phase I/II Pediatric and Adults
- ITCC Centers

In total N=58
Pediatric oncology centres accredited according to German Federal Joint Committee (G-BA)
ITCC centres: N=9
Non ITCC centres N= 49

Biology:
1,000 new diagnoses per year
ca. 350-400 patients,
ca. 40-50 of these will be cured,
> 300 children with poor prognosis

ITALY

- Milano: Istituto Polio in all age groups (ped + adults) unified SOPs, PK lab etc
- Roma Gemelli and Bologna same hospital but 2 different programs for phase 1
- Monza FFB and Torino
Different locations but in close collaboration

SWITZERLAND

- Adult & Pediatric Oncology units: Umeå, Uppsala, Stockholm, Linköping, Lund, Göteborg
- ITCC Units: Göteborg, Stockholm

Fostering Age Inclusive Research Group
On a regular basis the Paediatric Committee (PDCO) is challenging companies to include adolescents into the adult development programs whenever scientifically justified. This holds true for all therapeutic areas, not only for oncology.

The EMA’s Committee for Medicinal Products for Human Use (CHMP) has expanded the concept of age inclusive research to age inclusive marketing authorisation, again whenever there is sound scientific evidence supporting this. A recent example is Mylotarg, for which adolescents have been included into the initial marketing authorisation.

Overall, from our regulatory perspective, fostering age inclusive research whenever scientifically justified is implemented in our procedural routine from the research and development stage to the licensing stage.

Official statement?

05/11/2018
Objectives 2019

- Survey: to be modified to include the number of adolescents really included in the trials — 1 month
- Tool kit: to have it on the website/advertised on the different networks — 2 months
- Website redesign: lively and friendly user site with all the documents we produced — 3-4 months
- Country by country action
  - Document plan — 1 month
  - Paper of the current situation — 12 months
- EMA official endorsement — ????????
ACCELERATE FAIR trial group

We are ready to jump
What about you?