Should and could my trial be Fit For Filing?

Webinar
Sep 15, 2022
Programme

Introduction to Fit For Filing – 20-30 minutes
- From drug to medicine
- Historical perspective in paediatric oncology
- Legislation to stimulate innovation
- What is Fit For Filing?

Panel discussion – 60-70 minutes
From drug to medicine

**Clinical phase**

I. Safety/PK/Dosing in humans
II. Activity in treating disease
III. Pivotal trial: Efficacy and safety in target population
   - **Regulatory approval?**
IV. Extended information (safety, efficacy,...)
Historical perspective in paediatric oncology

- Progress through (inter)national clinical trial consortia
- **Off label** use is common
- Safety/efficacy data available from academic trials

McGregor et al. Oncology 2007
Legislation requiring paediatric investigations
Want to learn about legislation?

1st ACCELERATE Educational Webinar on... 1st ACCELERATE Educational Webinar slides
1st ACCELERATE Educational Webinar poll slides

2nd ACCELERATE Educational Webinar on... 2nd ACCELERATE Educational Webinar slides
Nefario Pharmaceuticals Brochure

www.accelerate-platform.org/education
What is Fit For Filing?

What data are required for the regulators to approve/authorize market access?

Approval is a risk/benefit assessment

Common Technical Document (international adopted standard)

- Administrative information and prescribing information (nation specific)
- Common technical document summaries
- Quality data
- Nonclinical study reports
- Clinical study reports
During this era from 1997 to 2017, only **26%** of first in child studies were **industry-sponsored** and only **six oncology drugs** had an initial approval that **included children**.

It is noteworthy that **all but one** of these six agents were approved for **acute lymphoblastic leukaemia**, the most common paediatric cancer and a malignancy that **also impacts adults**.
A 6.6 year delay is appropriate to ensure safety.

A 6.6 year delay is unacceptable for children with life-threatening diseases.
Who is supporting paediatric oncology drug development?

50% vs only 25% of trials have some industry involvement*

* Clinicaltrials.gov export 2000 – 2020 for oncology trials
ACCELERATE Fit for Filing Working Group

<table>
<thead>
<tr>
<th>Group</th>
<th>Members</th>
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<tbody>
<tr>
<td>Academia</td>
<td>Pam Kearns (University of Birmingham), Bram De Wilde (Ghent University Hospital), Beth Fox (St. Jude Children’s Research Hospital)</td>
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<tr>
<td>Regulatory</td>
<td>Greg Reaman (FDA), Dominik Karres (EMA)</td>
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<td>Patient Advocacy</td>
<td>Donna Ludwinski (Solving Kids Cancer)</td>
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<td>Industry</td>
<td>Elly Barry (Day One), John Manlay (Pfizer), Mark Kieran (Day One)</td>
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<td>Additionally</td>
<td>Rosanna Ricafort (BMS), Kathleen Neville (JNJ), Marieke Willemse (Princess Maxima)</td>
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Special thanks to: Carole Lecinse, Rosanna Ricafort, Kathleen Neville, COG, St. Jude’s Children’s Research Hospital, Children’s Hospital of Philadelphia, Dana Farber Cancer Center, ITCC, NANT, POETIC, Beat Childhood Cancer Consortium, CRCTU University of Birmingham, Princess Maxima Centre, Utrecht, Institut Gustav Roussy, Amgen, Abbvie, Pfizer, Novartis, BMS, Lilly, Gritstone, Roche, Camelia Mihaescu (EMA), Dong Ho Kim Pietsch (EMA), Andrea Demadonna (ACCELERATE), Teresa de Rojas (ACCELERATE), Beatriz Martinez (ACCELERATE)
Methodology

• Survey of the partners
  • Industry: previous experience in working with academia?
  • Academia: perception on capabilities?
  • Academia: trial resources, cost and money source?

• Consensus building discussions
  • Based on survey data and supplemented with case studies

• Formulation of recommendations
Limitations

• Industry survey results do not represent the whole industry (mainly large pharma)
• Capabilities were self assessed
• Over-representation of European sites in respondents
• Trial resources: many collaborations still in negotiation phase
# Knowledge and expertise gaps

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<th>ACADEMIA</th>
<th>INDUSTRY</th>
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<tr>
<td><strong>Trials experience</strong></td>
<td>Any, often interventional or non-interventional, registry type trials. <em>Limited, if any experience with intent to file</em> trials</td>
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<td><strong>Data management</strong></td>
<td>Focus on data quality and integrity with data cleaning focussed on primary analysis and publication. Monitoring strategies normally based on the low risk nature of the trials with <em>reduced source data verification</em></td>
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<td><strong>Documentation</strong></td>
<td>Collects <em>what is required</em> to ensure data quality and quality of trial conduct</td>
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<td><strong>Adverse Event reporting</strong></td>
<td>Often <em>pragmatic</em> with focus on unexpected or serious AEs</td>
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<td><strong>Communication</strong></td>
<td>Public presentation and <em>publication</em></td>
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What is the focus of the next webinars?

Webinar 3
With great trial needs comes great responsibilities:
Delivering high quality data and successfully file an application

Data management
Industry Data management standards

Essential Documents
Document with rationale

Webinar 2
A spoonful of data helps the medicine go down: Purposeful collection of clean and reproducible trial data

Resources
Cost of resourcing for «Fit for Filing» trials

Essential Data
Common data elements and differences
Panel composition

**Academia**

Pam Kearns (University of Birmingham)

Beth Fox (St. Jude Children’s Research Hospital)

**Industry**

Elly Barry (Former Pfizer, now Day One)

Mark Kieran (former BMS, now Day One)

**Patient advocates**

Donna Ludwinsky (Solving Kids Cancer)

**Regulators**

Martha Donoghue (FDA)

Dominik Karres (EMA)

Kim Pietsch (EMA)

Disclaimer: Views expressed during the panel discussion are the personal views of the panel members and not necessarily represent the view of the organization or company the panel members work for.
Thank you!