EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Role of academic clinical trials in paediatric (cancer) drug approvals

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Disclaimer

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Historical perspective on paediatric (oncology) drug development

- Common off-label use
- Progress through (inter)national clinical trials and consortia
- Safety/efficacy data available from academic trials

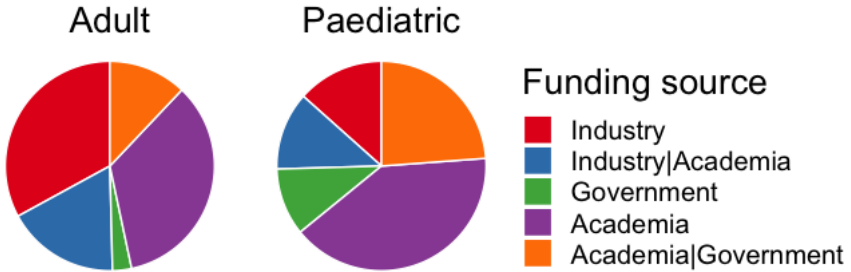
Who is supporting paediatric (oncology) drug development?

Discovery:
10.000 drugs

Pre clinical phase:
250 drugs

Clinical phase:
10 drugs

Regulatory approval:
1 medicine



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

* Clinicaltrials.gov export 2000 – 2020 for oncology trials

Objective

→ How can we further **enhance academic/industry collaboration** for the acceleration of drug approvals.

Fit for filing working group

Academia:

- Pam Kearns (University of Birmingham)
- Bram De Wilde (Ghent University Hospital)
- Beth Fox (St. Jude Children's Research Hospital)

Regulator:

- Greg Reaman (FDA)
- Dominik Karres (EMA)

Patient Advocacy:

- Donna Ludwinski (solvingkidscancer)

Industry:

- Elly Barry (Day One)
- John Manlay (Pfizer)
- Mark Kieran (Day One)

Additionally:

- Rosanna Ricafort (BMS)
- Kathleen Neville (JNJ)
- Marieke Willemse (Princess Maxima)

ACCELERATE
INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

Thank you also 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

Institute Gustav Rousey

Amgen

Abbvie

Pfizer

Novartis

BMS

Lilly

Gritstone

Roche

Camelia Mihaescu (EMA)

Dong Ho Kim Pietsch (EMA)

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

Knowledge and expertise gaps

		
Trials experience	Any, often **interventional** or non-interventional, registry type trials. **Limited, if any experience with intent to file** trials	conducted with an **intent to file**
Data management	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 **reduced source data verification**	Clear and concise **rigorous data management plans** with **full monitoring** fixed data cleaning and data locking strategies
Documentation	Collects **what is required** to ensure data quality and quality of trial conduct	Documents **anything and everything** that ensures data quality, researcher qualification and (financial) independence assuring **objectively verifiable trial conduct**
Adverse Event reporting	Often **pragmatic** with focus on unexpected or serious AEs	**Complete, to meet filing requirement**
Communication	Public presentation and **publication**	**Filing application**, with minor focus on public distribution of results

Set of recommendations – general principles

- **Early planning and prospective collaboration.**
- **Defining the type of trial and the nature of the collaboration.**
- **Continuous and transparent communication.**

Specific recommendations

- **Essential documents**
  - Identifying essential documents
  - Storing and filing essential documents
- **Essential data**
  - Relevant essential data identification and capture

Specific recommendations

- **Data management**
  - Development of a data strategy agreement
  - Data management plan
  - Trial databases
  - Case report form development
  - Quality control

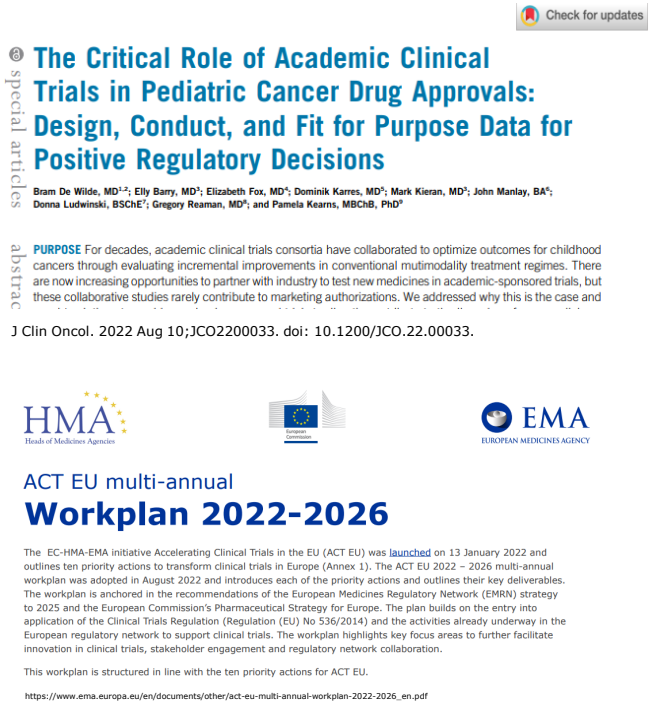
- **Trial resources**
  - Transparent expectations and capabilities
  - Experience sharing

- **Call for early dialogue with regulators**

Wider context

While this paper focuses on paediatric oncology, the recommendations hold true across all populations and therapeutic areas.

It can therefore also inform discussions under the ACT EU work plan, where academia-led clinical trials are a particular focus area.



Check for updates

special articles

The Critical Role of Academic Clinical Trials in Pediatric Cancer Drug Approvals: Design, Conduct, and Fit for Purpose Data for Positive Regulatory Decisions

Bram De Wilde, MD[1,2]; Elly Barry, MD[3]; Elizabeth Fox, MD[4]; Dominik Karres, MD[5]; Mark Kieran, MD[3]; John Manlay, BA[6]; Donna Ludwinski, BSChE[7]; Gregory Reaman, MD[8]; and Pamela Kearns, MBChB, PhD[9]

abstract

PURPOSE For decades, academic clinical trials consortia have collaborated to optimize outcomes for childhood cancers through evaluating incremental improvements in conventional mutimodality treatment regimes. There are now increasing opportunities to partner with industry to test new medicines in academic-sponsored trials, but these collaborative studies rarely contribute to marketing authorizations. We addressed why this is the case and

J Clin Oncol. 2022 Aug 10;JCO2200033. doi: 10.1200/JCO.22.00033.

HMA Heads of Medicines Agencies

EMA EUROPEAN MEDICINES AGENCY

ACT EU multi-annual

Workplan 2022-2026

The EC-HMA-EMA initiative Accelerating Clinical Trials in the EU (ACT EU) was launched on 13 January 2022 and outlines ten priority actions to transform clinical trials in Europe (Annex 1). The ACT EU 2022 – 2026 multi-annual workplan was adopted in August 2022 and introduces each of the priority actions and outlines their key deliverables. The workplan is anchored in the recommendations of the European Medicines Regulatory Network (EMRN) strategy to 2025 and the European Commission's Pharmaceutical Strategy for Europe. The plan builds on the entry into application of the Clinical Trials Regulation (Regulation (EU) No 536/2014) and the activities already underway in the European regulatory network to support clinical trials. The workplan highlights key focus areas to further facilitate innovation in clinical trials, stakeholder engagement and regulatory network collaboration.

This workplan is structured in line with the ten priority actions for ACT EU.

https://www.ema.europa.eu/en/documents/other/act-eu-multi-annual-workplan-2022-2026_en.pdf

Webinar series

Improving academia-industry collaborative trials

10 Nov 2022

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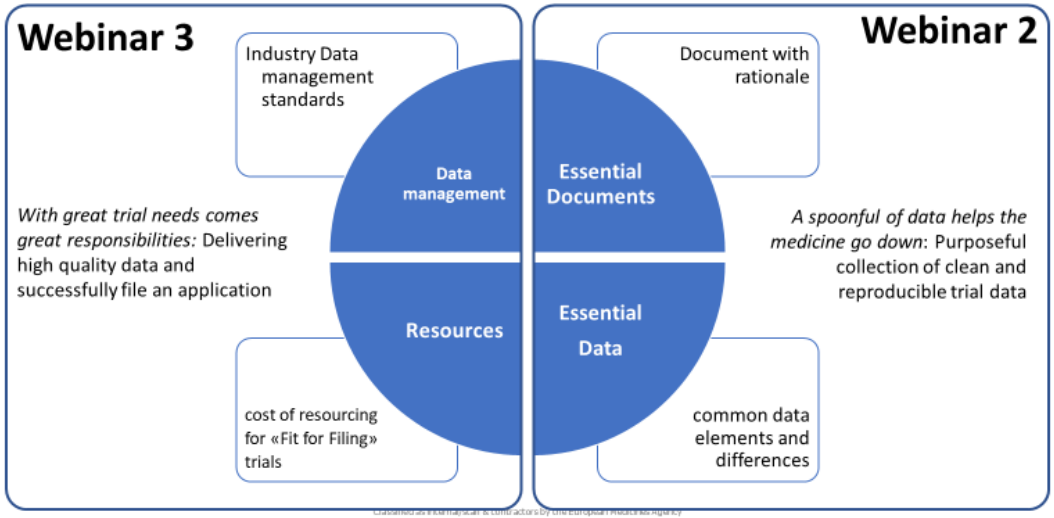

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https://www.accelerate-platform.org/fff-webinars

Any questions?

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