

Partnering with academia for paediatric oncology clinical development

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ONCOHEROES
BIOSCIENCES

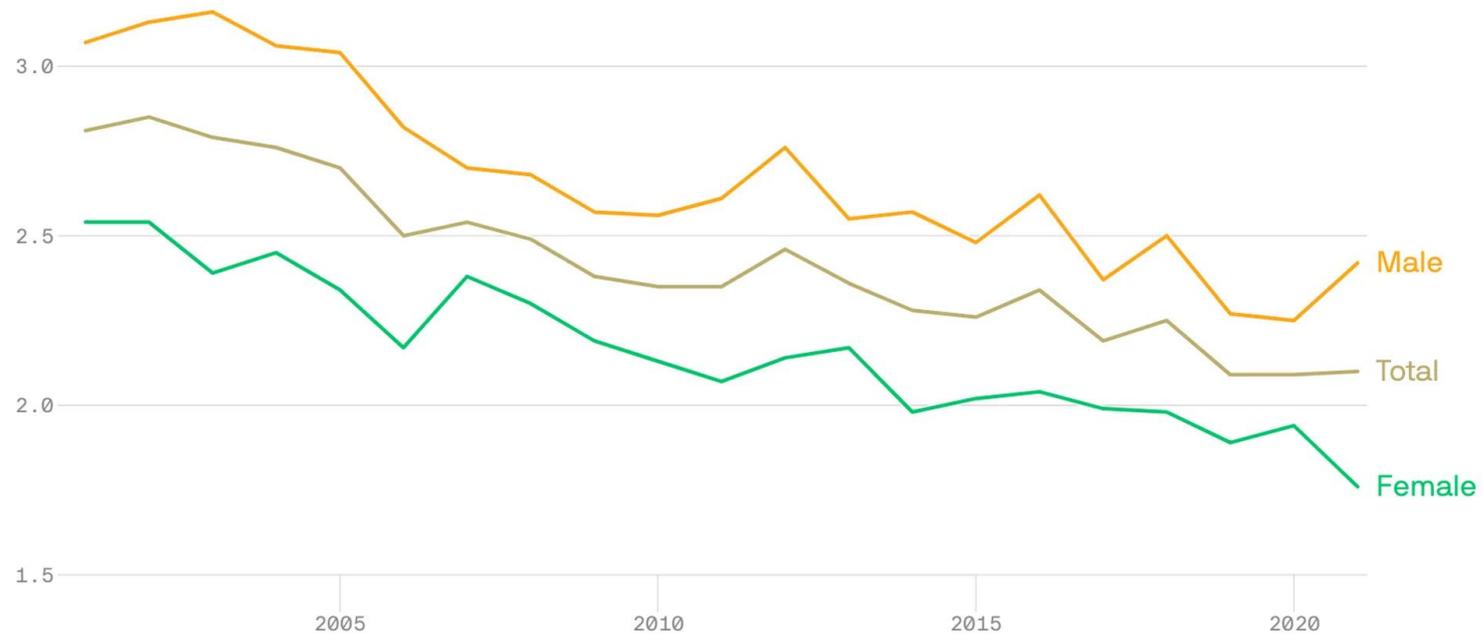
**Cancer is 1st
cause of death
by disease for
children in EU
and US**

- 400k new cases, 90-100k deaths every year
 - Cancers in children and adolescents are generally different diseases
 - Patients treated with older drugs - health consequences later in life
 - Only 7 pediatric drugs specifically developed and approved vs more than 200 for adults
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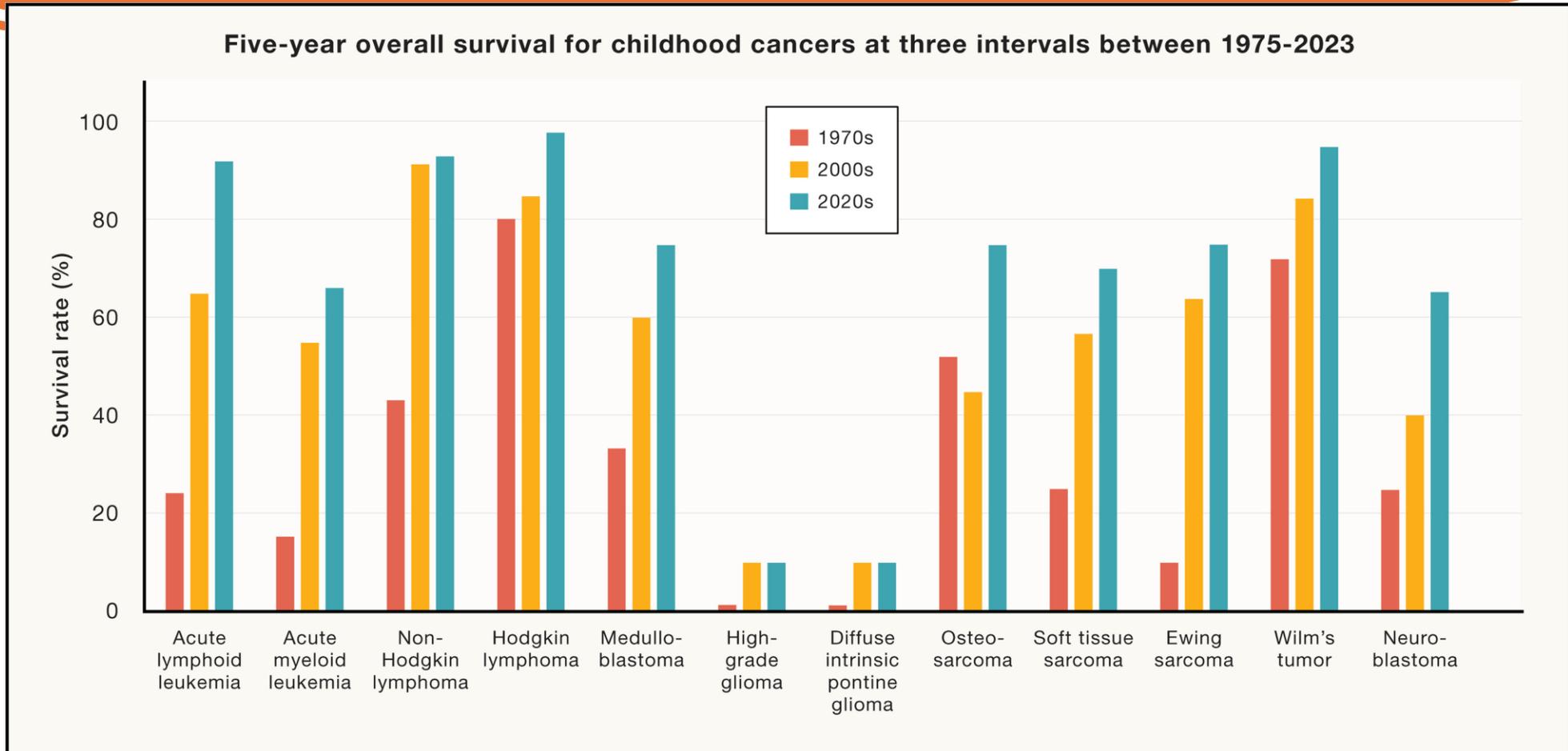
Progress is being made...

Death rate for children with childhood cancers

Rate per population of 100,000 among children 19 years and younger where cancer was the underlying cause of death; 2001 to 2021



But not everywhere



ONCOHEROES

B I O S C I E N C E S

ONCOHEROES
B I O S C I E N C E S

Boston



Barcelona

A biotech company 100% focused on pediatric oncology drug development

- 1** In-licence drug candidates that target unmet medical needs in pediatric oncology
Late preclinical and clinic-ready assets to obtain **fast-track regulatory approval** and provide substantial benefits to patients
- 2** Innovative drug discovery to identify new assets and potential biomarkers
Bring superior pre-clinical candidates that are both **more effective and less toxic** in collaboration with academic centers and technology partners.

Oncoheroes Pipeline

ONCOHEROES BIOSCIENCES

VOLASERTIB

PLK1 Inhibitor

In-licensed from



Pediatric Indications

- Ewing sarcoma
- Medulloblastoma
- Neuroblastoma
- Rhabdomyosarcoma
- DMG (*incl. DIPG)

Next Step

Coming Clinical Trial

Academic Basket Trial
(Phase 1/2)



DOVITINIB

Pan-TK Inhibitor

Pediatric license from



Pediatric Indications

- Osteosarcoma
- Ewing sarcoma
- One more TBD

Next Step

Coming Clinical Trial

Academic Trial
(Phase 1/2a)



STENOPARIB

PARP Inhibitor

Pediatric license from



Pediatric Indications

- TBD

Next Step

Preclinical Work

CMC Pediatric Formulation
IND Enabling Studies
Select Primary Indication/IND filing

Clinical Development Challenges

- Rare cancers – limited patient pool
- International trials
- Ethical constraints – informed consent
- Need for pediatric-friendly formulations
- Financial constraints – lack of incentives



Increasing Collaboration in Pediatric Cancer Drug Development

- Academic and industry partnerships are becoming more common in the pursuit of new therapies for pediatric cancers
- Academic consortia have a history of success in conducting pediatric cancer trials
- Collaborations provide increasing opportunities to evaluate novel therapeutics



Academia driving innovation in pediatric oncology

- 83% of pediatric oncology clinical trials are sponsored by academic institutions
- Much more prominent role of academia in pediatric vs adult oncology
- Established international networks
- Approved products quickly become standard of therapy



Stakeholder Engagement



A regular meeting of pediatric oncologists, diseases experts/KOLs, regulators, industry professionals and patient advocates.



“Fit-for-filing” Working Group

Academia vs Industry*

TABLE 1. Knowledge and Expertise Gaps

| Sponsor | Academic | Industry |
|-------------------|--|---|
| Trials experience | Any, often phase III interventional or noninterventional, registry type trials. Limited, if any experience with intent to file trials | Phase I, II, III, and IV all conducted with an intent to file |
| Data management | Focus on data quality and integrity with data cleaning focused on primary analysis and publication. Monitoring strategies normally on the basis of the low-risk nature of the trials with limited source data verification | Clear and concise rigorous DMPs 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 |
| AE reporting | Often pragmatic with focus on unexpected or severe AEs | Complete, to meet filing requirement |
| Communication | Public presentation and publication | Filing application, with minor focus on public distribution of results |

Abbreviations: AE, adverse event; DMP, data management plan.

*De Wilde et al J Clin Onc 2022 Oct 10; 40(29) 3456

Types of trials*

TABLE 2. Descriptors of Different Types of Trials

| Trial Type | Sponsor^a | Funding Source | Intended Use of Trial Data | Role of Industry | Intended as FFF |
|---------------------------------------|----------------------------|--|--|---|----------------------------------|
| Academic trial | Academic | Nonindustry; ie, charity, philanthropy, government competitive funding calls | Publication and to contribute to the evidence base for clinical practice | None | No |
| Investigator-initiated trial | Academic | Mixed funding from industry and nonindustry sources | Publication and to contribute to the evidence base for clinical practice | Provision of drug ± a contribution to funding | No, but notable exceptions exist |
| Academic-industry collaborative trial | Academic | Industry | Toward licensing of the asset and academic publication | Provision of drug and full funding of the trial | Yes |
| Industry trial | Industry | Industry | Toward licensing of the asset | Full responsibility and ownership of the trial | Yes |

Abbreviation: FFF, fit for filing.

^aThe sponsor is an individual, company, or an institution that assumes the responsibility for the initiation, management, and/or financing of a clinical trial.

Early Engagement with Regulators!

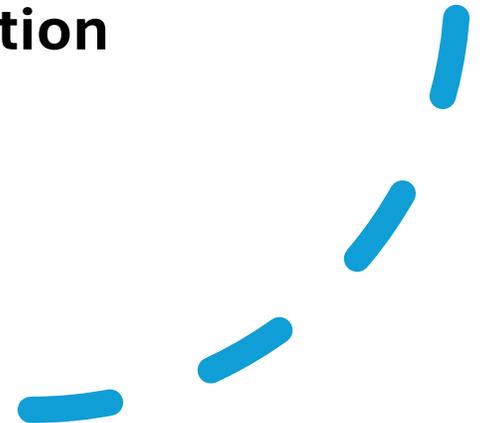
- Early involvement of regulatory agencies, like the EMA and FDA, is highly recommended
- This ensures the trial design meets regulatory requirements and addresses clinically relevant questions



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

The Need for “Fit-for-Filing” Trials

- Fit-for-filing (FFF) trials are crucial for expediting the drug approval process.
- Challenges arise when using data from academic-sponsored trials for marketing authorization applications
- FFF trials have the potential to generate data that meet regulatory requirements for approvals.
- **Communication: Key to Collaboration**



Continuous and Transparent Communication During the Trial

- **Transparency regarding the intended use of data** is essential from the beginning
 - **Regular Updates and Feedback:** Frequent communication is essential for sharing progress updates, discussing challenges, and providing feedback
 - **Open Dialogue on Safety and Efficacy:** This allows for timely decisions regarding trial modifications or potential early termination.
 - **Transparency with Stakeholders:** Engaging with patients, parents, and advocacy groups is
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Data Management

Detailed discussions are needed to align data collection, review, and quality control processes between partners.

A clear data strategy agreement should be established, covering:

- Data management plans
- Documentation practices
- Handling of data quality issues



It's all about the data: key differences

Documentation Practices - Academic trials may not collect all the essential documents required by regulatory agencies for a marketing authorization application. Industry documents "anything and everything"

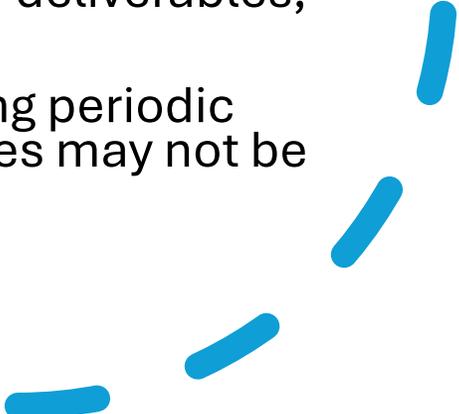
Adverse Event (AE) Reporting - Some academic sponsors may adopt a pragmatic approach, focusing only on severe or unexpected AEs.

Data Review Strategies - Industry partners employ rigorous data cleaning and review strategies from the outset of the trial. Academic trials may not have such comprehensive data review plans

Trial Databases - Academic trials may use systems that do not meet these regulatory requirements.

Quality Control (QC) Processes - Industry-sponsored trials typically have predefined QC processes to assess data quality throughout the study and before major deliverables, unlike academic-sponsored

Investigator Oversight - Databases supporting periodic electronic CRF sign-off or alternative processes may not be standard practice in academic settings



Examples



Ongoing

Future



Conclusion

- **Academia-Industry clinical collaborations can deliver innovative approaches to speed up the development of new therapeutics**
- **The “Fit-for-filing” model is a hybrid approach that takes advantage of academic and industry competencies in a cost-effective fashion**
- **Partnerships between academia and industry create potential drug development synergies for rare and pediatric cancers**
- **Operational challenges but not impossible to overcome**
- **Regulatory support**
- **Effective communication needed to align with expectations of all parties**
- **Successful examples in pediatric oncology**



Q&A

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