

# Partnering with academia for paediatric oncology clinical development

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**Chief Operating Officer**

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**ONCOHEROES**  
BIOSCIENCES

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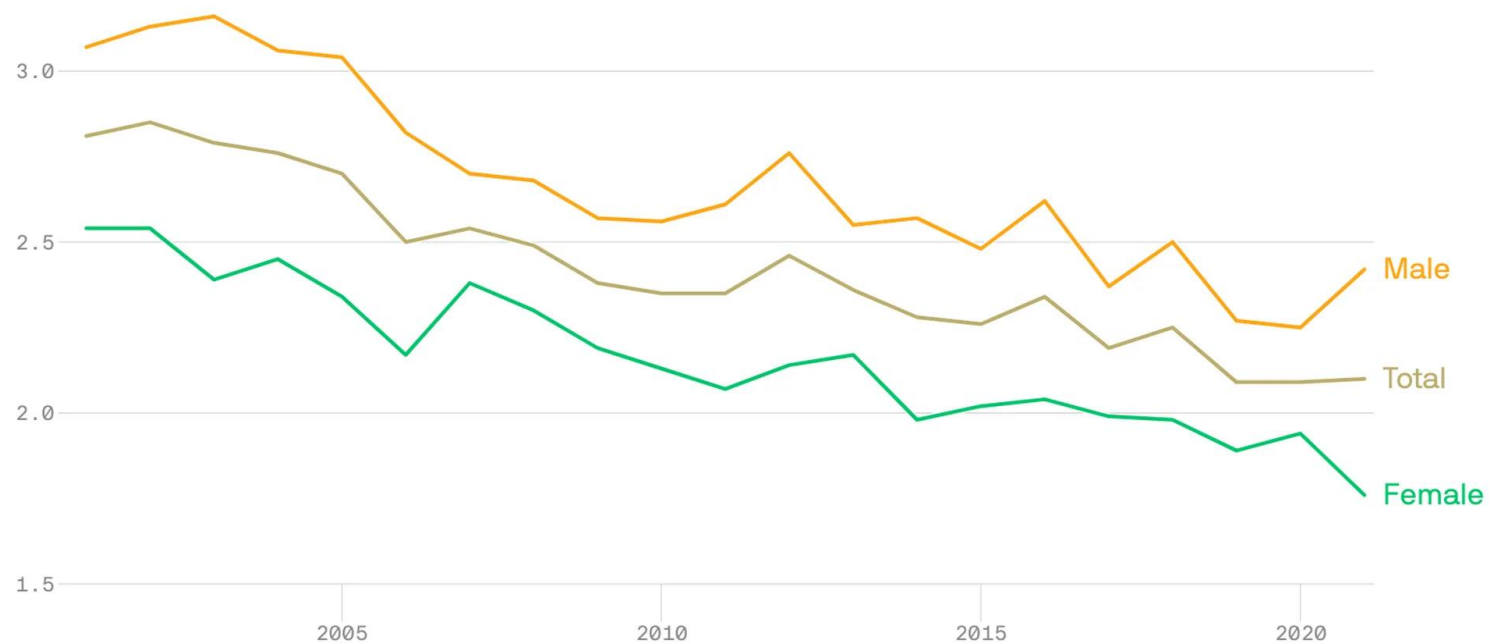
# Cancer is 1<sup>st</sup> cause of death by disease for children in EU and US

- 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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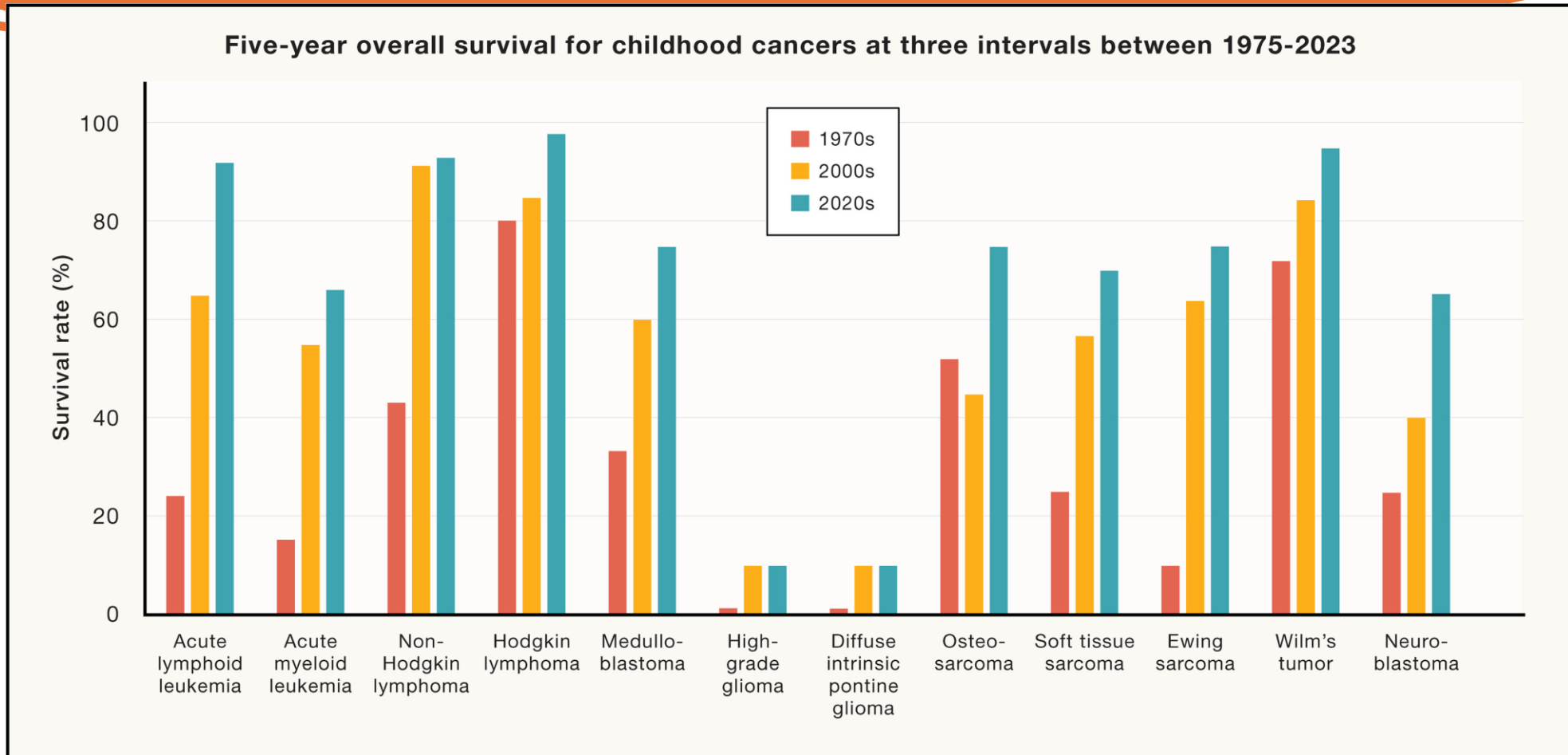
# Progress is being made...

## 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



**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



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## 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
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# 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

<b>Trial Type</b>	<b>Sponsor<sup>a</sup></b>	<b>Funding Source</b>	<b>Intended Use of Trial Data</b>	<b>Role of Industry</b>	<b>Intended as FFF</b>
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.

<sup>a</sup>The 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





# 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



# 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
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# 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

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# Examples



Ongoing

Future



# Conclusion

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- **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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