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Biosciences

2026 White Paper

Redefining Pediatric Cancer Drug Development
A New, Scalable, Patient-Driven Model

ONCOHEROES.COM

Intended Audience

This White Paper is intended for a broad, cross-sector audience, including:

- Foundations and patient organizations
- Academic medical centers and hospital leadership
- Biotech and life sciences companies
- Impact investors, venture philanthropy funds, and family offices
- Policymakers, regulators, and ecosystem partners

While grounded in the realities of childhood cancer, this document aims to **educate beyond the pediatric oncology community** by highlighting the structural challenges and inefficiencies in pediatric drug development and presenting a **scalable**, **evidence-based model** relevant across healthcare, mission-aligned capital, and innovation.

Executive Summary

Pediatric cancer remains the leading cause of disease-related death among children in developed countries. Yet in more than four decades, **only seven oncology drugs have been developed specifically for pediatric use**, despite extraordinary scientific advances and decades of philanthropic and public investment in cancer research. The core problem is **not a lack of discovery**. Pediatric cancer research has produced many promising targets and therapeutic hypotheses. The failure lies in what happens next: **the inability to translate those discoveries into clinical therapies for children**. The development of novel therapeutics for pediatric cancers occurs in two fundamentally different phases:

- **Drug discovery and preclinical research**, historically supported by philanthropy and public funding (including federal research grants, though pediatric cancer receives only a small fraction of overall cancer funding)

- **Clinical drug development**, which requires industry partners for manufacturing, regulatory expertise, trial operations, capital, and regulatory approval

For pediatric cancer, this second phase has been **chronically underfunded and deprioritized** by industry. When promising projects are ready to enter the clinic, they frequently fall into the well-known “valley of death” due to a lack of industry focus, incentives, or ownership.

Oncoheroes was created to address this systemic failure.

As the first biotech company built **exclusively for pediatric cancer drug development**, Oncoheroes has designed a clinically focused, capital-efficient model to bring therapies to children faster, at lower cost, and with measurable patient impact—through deep partnership with academic hospitals and mission-aligned capital, including foundations, family offices, impact-driven investors, and venture philanthropy funds.

This White Paper outlines:

- Why pediatric cancer drug development has historically failed
- Why the current regulatory, clinical, and philanthropic landscape creates a unique opportunity
- How Oncoheroes' academic-driven, mission-aligned capital-supported model works
- How this model enables multiple concurrent trials, pipeline expansion, and sustainable impact
- How Oncoheroes envisions a future where the childhood cancer community can **actively participate in building**—and potentially owning—the industry partner it has long been missing

The Unmet Need in Pediatric Cancer

Pediatric cancer remains one of the leading causes of disease-related death in children worldwide, with more than **100,000 children dying each year globally**, according to the International Agency for Research on Cancer (IARC).

While important progress has been made over time, pediatric oncology continues to face a profound structural challenge: **children have historically not been prioritized in oncology drug development**. Very few oncology drugs have been initially approved with pediatric indications, and children have often waited years after adult development to gain access to new therapies.

Analyses of FDA oncology approvals between 1997 and 2017 show that **only approximately 5% of oncology drugs were initially approved with pediatric indications**, and that the median time lag between adult approval and initiation of the first pediatric clinical trial was approximately **6.5 years**.

As a result, children with cancer are frequently treated with therapies that were:

- **Designed for adult tumors** with fundamentally different biology
- Introduced into pediatrics late in their lifecycle
- Not optimized for pediatric dosing, formulations, or long-term toxicity profiles

This persistent delay is not primarily driven by a lack of scientific opportunity, but by the **absence of a dedicated**, **execution-focused industry infrastructure** designed to efficiently translate promising therapies into pediatric clinical development.

The limiting factor is not science.
It is **how drugs are developed**, **funded**, **and prioritized**.

Where Pediatric Cancer Drug Development Breaks Down

Discovery vs. Development: The Real Gap

Drug development occurs in two fundamentally different phases:

1. Drug Discovery and Preclinical Research

- Conducted in academic and research laboratories
- Historically funded by philanthropy and public grants
- Pediatric cancer research is relatively well supported at this stage

2. Clinical Drug Development

- Requires drug manufacturing, regulatory strategy, clinical operations, data systems, and capital
- Demands an industry-capable development partner
- This is where pediatric programs most often fall into the **“valley of death”**

For decades, enormous effort has been invested in pediatric cancer discovery. Yet when the most promising projects are ready to move into patients, they are frequently abandoned—not because they lack merit, but because **no company is exclusively structured or incentivized to take ownership of pediatric development**.

Academic clinical trials continue to play a critical role in pediatric oncology, but they are often designed primarily to generate scientific knowledge and publications. They are **not typically structured**, **resourced**, **or governed to support regulatory pathways**, long-term development plans, or manufacturing ownership—capabilities that are industry-specific.

This structural gap has left children without a dedicated industry partner to advance therapies.

Why the Traditional Biotech Model Fails Children

Traditional biotech models are poorly suited for pediatric oncology because:

- Small patient populations limit perceived commercial upside
- Capital allocation favors adult indications
- Pediatric programs are often treated as regulatory obligations, not strategic priorities
- Development expertise exists, but incentives do not

Importantly, **pediatric clinical trials themselves are often faster**, **smaller**, **and more cost-efficient than adult trials**.

The issue is not **feasibility**, it is **prioritization**.

Why Pediatric Cancer Drug Development Is Capital-Efficient, and How the Oncoheroes Model Extends This Advantage

Pediatric oncology presents a fundamentally different and **more capital-efficient** development environment than adult oncology. When paired with a purpose-built execution model, these structural advantages create a compelling opportunity for scalable, mission-aligned drug development.

Structural Efficiency of Pediatric Oncology

Pediatric cancers qualify as rare diseases and are treated within highly coordinated global clinical networks. These characteristics fundamentally reshape the economics of drug development.

- **Smaller, Faster Clinical Trials**
  Pediatric trials typically require far fewer patients than adult oncology programs. Meaningful clinical signals can often be generated with limited enrollment, resulting in trials that are smaller, faster, and more focused.

- **Accelerated Regulatory Pathways**
  Rare-disease designation enables more flexible regulatory strategies. In many pediatric indications, robust Phase II data may support regulatory engagement, significantly shortening timelines compared to adult oncology.

- **Exceptionally High Trial Participation**

Trial participation has historically been far higher in pediatrics than in adults, although rates vary by age, era, and network; adult participation is commonly cited at around 2-5%. This participation rate, combined with established international pediatric oncology networks, supports rapid and predictable enrollment once trials are activated.

- **Lower Development Costs**
  Together, these factors make pediatric oncology drug development dramatically more cost-efficient than adult oncology, with programs often executed at a fraction of the cost required for adult cancer development.

Why These Advantages Have Been Underutilized

Despite these efficiencies, pediatric cancer drug development has historically failed to deliver approved therapies at scale. The limitation has not been scientific feasibility; it has been **the absence of an execution-focused industry partner**.

Traditional biotech models are optimized for adult markets and fail to capture pediatric-specific efficiencies. Without an organization capable of managing manufacturing, regulatory strategy, and integrated development planning, promising pediatric programs stall before reaching patients.

How the Oncoheroes Model Extends Capital Efficiency

Oncoheroes was purpose-built to capture and amplify the inherent efficiencies of pediatric oncology.

Rather than running fully industry-sponsored trials, where companies bear 100% of clinical site and infrastructure costs, Oncoheroes **partners with leading academic pediatric hospitals and established international networks**.

In this model:

- **Hospitals lead patient care and trial execution**, leveraging existing clinical infrastructure
- **Many sites fund or co-fund their participation**, significantly reducing development costs
- **Oncoheroes provides the industrial layer**, including manufacturing and supply, regulatory strategy, data integration, and portfolio coordination

This structure preserves the scientific and clinical strengths of academic trials while adding the capabilities required for regulatory advancement and portfolio scalability.

A Platform for Scalable Impact

By combining:

- the inherent capital efficiency of pediatric oncology,
- the execution strength of academic clinical networks, and
- a dedicated industrial development layer,

Oncoheroes can advance multiple clinical programs in parallel with **disciplined capital deployment**.

A Moment of Structural Opportunity

Several forces are now reshaping pediatric oncology:

Regulatory Alignment

- The RACE for Children Act and the EMA Paediatric Medicine Regulation may require pediatric evaluation for oncology drugs that are deemed relevant for pediatric indications

- Pediatric cancers qualify as rare diseases, enabling accelerated and flexible approval pathways
- Regulatory agencies have demonstrated increasing openness to innovative pediatric development models

Clinical & Institutional Alignment

- Pediatric oncology is organized into highly coordinated international academic networks
- Institutions increasingly collaborate across borders to test new therapies efficiently

Examples include platforms and consortia such as INFORM, OPTIMISE, PNOC, COG, and other international academic collaborations.

Philanthropic & Venture Philanthropy Evolution

Foundations are increasingly frustrated by the **disconnect between decades of funding and the limited number of approved pediatric drugs**. As a result, philanthropy is evolving:

- From funding discovery alone
- Toward supporting translational and clinical development
- Including venture philanthropy, equity investment, and program-related investments

In the U.S. alone, foundations and families raise ~$100M annually for pediatric cancer research, far more capital than traditional venture markets deploy exclusively into pediatric oncology in the same period.

Oncoheroes was the **first company to structurally align this capital with clinical drug development**. Together, these forces create the conditions for a fundamentally different development model.

The Oncoheroes Model

Only Pediatric. Only Purpose.

Oncoheroes is the first biotech company **built exclusively for pediatric cancer drug development**, designed by people who have lived the disease and supported by the pediatric oncology community itself.

Core Principles

- **100% pediatric focus**
- Prioritization of clinical execution over early-stage discovery
- Cost-effective and timely academic collaborations for early clinical studies
- Strategic partnerships with foundations and pediatric hospitals
- Capital efficiency and scalability

How the Model Works

Building the Pipeline

Oncoheroes builds its clinical pipeline by in-licensing or acquiring assets that can be rapidly advanced for pediatric cancer, including:

- Industry compounds discontinued for adult indications with **potential relevance in pediatric oncology**
- Assets originally developed for other diseases with **clear pediatric applicability**
- Validated academic discoveries **ready for clinical translation**

Importantly, asset selection is driven by a disciplined review of why a program was discontinued and whether those factors are relevant in pediatric cancer. Many assets are deprioritized in adult oncology for reasons unrelated to pediatric potential, such as commercial prioritization, strategic portfolio shifts, or adult-specific efficacy thresholds, rather than safety concerns.

Each asset is evaluated against a pediatric-specific development framework that includes:

- Strong biological and mechanistic rationale in pediatric tumors
- Clear unmet clinical need within defined pediatric populations
- Safety profile and therapeutic window appropriate for children
- Alignment with pediatric disease biology and biomarker strategies
- Feasible manufacturing and formulation for pediatric use

The current pipeline includes **volasertib**, **dovitinib**, and **stenoparib**, with additional assets under evaluation based on unmet patient need identified in close collaboration with the global pediatric oncology community.

Academic-Driven Clinical Development

Oncoheroes conducts its early clinical programs in close collaboration with leading international academic pediatric oncology networks, rather than relying exclusively on traditional, fully industry-sponsored trial models. This approach reflects both the realities of pediatric cancer development and the unique strengths of the global pediatric oncology ecosystem.

Academic pediatric hospitals play a central role in advancing new therapies for children. They generate much of the scientific evidence that motivates pediatric trials, provide direct care to the relevant patient populations, and are organized into highly coordinated international networks that enable efficient multicenter clinical research. Many of these institutions are also **willing to support or co-fund trial participation**, recognizing the clinical urgency and mission-driven value of accelerating access to new therapies for children.

Oncoheroes partners with academic institutions across a range of trial models, from early signal-generation and feasibility studies to more advanced programs with clear regulatory relevance.

Within this collaborative framework, Oncoheroes **contributes the industry capabilities that are essential for translating academic research into scalable development programs**, including:

- Ownership and coordination of drug manufacturing and supply
- Alignment of biomarker and translational strategies
- Program coordination across multiple institutions and geographies
- Early regulatory planning to inform downstream development decisions

Governance, Data Quality, and Regulatory Readiness

Oncoheroes applies a structured governance and quality approach that aligns academic trials with industry and regulatory expectations, while preserving academic independence.

The company supports academic sponsors in meeting regulatory and safety requirements, with clear accountability, appropriate oversight, and consistent standards for data integrity and patient safety. Where appropriate, trials use shared protocol frameworks and harmonized data elements to enable comparability and integration across programs.

While academic investigators retain leadership over patient care and trial conduct, Oncoheroes works collaboratively to ensure the resulting datasets are robust and suitable for portfolio decisions and future regulatory interactions. When alignment allows, programs are structured in a regulatory-informed manner to maximize downstream value without constraining academic objectives.

Unlike traditional investigator-initiated studies focused primarily on publication, Oncoheroes-supported programs are embedded within a globally coordinated development strategy, enabling **faster execution**, **lower costs**, and **scalable pipeline expansion**.

This academic-driven model enables:

- Faster patient recruitment
- Significantly reduced development costs
- Parallel execution of multiple clinical programs
- Scalable expansion of the pediatric oncology pipeline

Mission-Aligned Capital Integration

Oncoheroes is supported by a diverse base of **mission-aligned capital**, including foundations, family offices, impact investors, and mission-driven private investors. To date, **22 pediatric cancer foundations** have chosen to support or invest in the company, and the model is intentionally designed to scale globally by welcoming foundations and mission-aligned capital from any geography.

This approach reflects a fundamental shift in how philanthropy engages with pediatric cancer drug development.

From Discovery Funding to Clinical Enablement

Historically, foundations have focused primarily on funding early-stage discovery and academic research. While essential, this emphasis has contributed to a structural gap: when promising therapies are ready to enter the clinic, there is often no industry partner positioned to carry them forward.

Oncoheroes was designed to fill that gap by providing the **industrial execution layer** required for clinical drug development — including manufacturing ownership, regulatory planning, data integration, and portfolio coordination — while partnering with academic hospitals that lead patient care and trial execution.

A Fundraising Force Multiplier for Foundations and Hospitals

Beyond supporting individual programs, partnering with Oncoheroes provides a tangible fundraising and engagement advantage for foundations and pediatric hospitals.

By aligning philanthropic capital with a dedicated pediatric-only biotech company, partners gain the ability to demonstrate that donations:

- Directly enable drugs to be tested in children
- Support near-term, measurable patient impact
- Contribute to building the long-missing industry partner for pediatric cancer

This model **strengthens donor confidence**, helps **attract new supporters** seeking translational impact, and allows foundations and hospitals to move beyond long discovery timelines toward visible clinical progress.

In practice, this transforms philanthropy from "**funding hope someday**" into "**delivering impact now**."

Making the Capital Stack Explicit: Flexible, Mission-Aligned Capital Across the Lifecycle

A defining strength of the Oncoheroes model is the intentional design of a **flexible**, **mission-aligned capital architecture** that allows different forms of capital to engage **at any stage of pediatric drug development**, based on shared purpose rather than predetermined roles.

Rather than assigning specific types of funders to specific stages, Oncoheroes operates with a capital stack that is **open**, **overlapping**, **and adaptive**. Foundations, family offices, impact investors, and mission-driven private investors can participate wherever they believe their capital can create the greatest impact —whether in early translational work, manufacturing and regulatory readiness, or clinical development. This approach reflects a core belief of the platform: **what matters is not who funds which stage, but that capital is aligned with pediatric need, scientific rigor, and disciplined execution**.

Across the lifecycle, capital may be deployed in multiple forms—including **grants, Program-Related Investments (PRIs)**, **equity**, or **hybrid structures**—depending on the objectives of each partner and the needs of each program. These mechanisms are not stage-gated. They are tools that enable partners to engage in ways that reflect their risk tolerance, time horizon, and desired balance between mission impact and capital sustainability.

Importantly, this flexibility allows:

- Mission-aligned capital to support programs **from the earliest enabling activities through clinical development**
- Partners to **participate more than once**, increasing or adapting their involvement as milestones are achieved
- **Value created at any stage to be responsibly recycled**, reinforcing long-term sustainability

In practice, many partners choose to reinvest as programs advance, creating a **sustaining funding flywheel** that aligns closely with the core objective shared across the pediatric cancer community: raising capital year after year to advance new therapies for children.

By avoiding rigid funding lanes and enabling broad participation across the development continuum, Oncoheroes creates a capital environment that is **inclusive**, **durable**, **and scalable**—one that allows mission-aligned capital to move as dynamically as the science itself.

Enabling Scale Through Academic Partnerships

Because Oncoheroes' clinical model leverages academic pediatric hospitals that already treat relevant patient populations and operate within established international networks, the company is able to expand clinical activity without being constrained by traditional site-level funding or infrastructure limitations. Hospitals **increasingly fund or co-fund their own participation**, while Oncoheroes provides centralized manufacturing, supply, regulatory coordination, and data integration.

This model enables scale by design rather than scale through capital intensity. By decoupling clinical expansion from proportional increases in internal infrastructure, Oncoheroes can advance multiple programs efficiently while remaining fully focused on pediatric cancer.

This structure allows Oncoheroes to:

- Expand its pipeline without exponential capital requirements
- Advance multiple clinical programs in parallel
- Maintain exclusive focus on pediatric oncology, without dilution into adult markets

Together, **mission-aligned capital and academic partnerships form the backbone of a scalable, repeatable, and sustainable pediatric drug development platform**—one that aligns incentives across foundations, hospitals, investors, and, most importantly, patients.

Co-Development Partnerships: A Platform Beyond the Owned Pipeline

In addition to advancing its own assets, **Oncoheroes operates as a co-development partner of choice** for:

- Academic groups
- Foundations
- Biotech companies

that lack pediatric-specific development infrastructure.
Oncoheroes provides:

- **Pediatric-focused** development expertise
- Manufacturing and regulatory ownership
- Access to global academic networks
- Alignment with mission-aligned capital

Importantly, Oncoheroes is willing to advance programs **even when assets are not retained in its own portfolio**, reflecting a mission-first philosophy.

Economic Design as an Execution Advantage

The Oncoheroes operating model is intentionally designed not only to enable clinical execution but to withstand the economic realities of pediatric drug development. In this field, execution without financial structuring is not sustainable.

Pediatric oncology has historically failed not because of scientific limitations, but because traditional biotech financial structures are poorly suited to rare diseases, small patient populations, and long development timelines. For pediatric cancer, **how programs are financed and structured is as critical as how they are executed**.

The Oncoheroes model explicitly integrates financial design into its operating strategy, drawing on established insights from pediatric oncology and health economics. Key principles include:

Portfolio construction
Single-asset pediatric biotech companies are inherently fragile. A portfolio approach reduces variance, allows learning to compound across programs, and enables continued execution even when individual assets fail.

Phase diversification
Portfolios spanning late preclinical, Phase I, and Phase II programs materially improve organizational durability and increase the probability that at least one program reaches a meaningful value inflection point.

Mission-aligned and non-dilutive capital
Grants, Program-Related Investments (PRIs), and foundation-backed equity reduce the effective cost of capital, buffer downside risk, and enable multiple “shots on goal” that would be impossible under purely commercial venture economics.

Together, these elements allow Oncoheroes to advance multiple pediatric programs in parallel, sustain momentum despite individual setbacks, and allocate capital toward high-leverage activities rather than fixed overhead. This financial architecture underpins Oncoheroes' ability to function as a durable, long-term industry partner for pediatric cancer—not a single-asset experiment.

Proof of Execution: Clinical Programs

volasertib – Phase I/II (Start-Up)

- Multinational academic basket trial
- ~110 relapsed/refractory patients
- Indications include sarcomas, medulloblastoma, neuroblastoma, and DMG/DIPG
- Biomarker-driven enrollment
- Conducted within the **INFORM** academic platform, led by Hopp Children's Cancer Center Heidelberg (KITZ)

dovitinib – Phase I/II (Preparing)

- Led by Children's Cancer Institute (Australia) and SickKids (Canada)
- Conducted within the **OPTIMISE** international academic platform
- Seamless Phase I/II design
- Bone sarcomas with expansion to brain tumors
- Planned launch: Q2–Q3 2026

stenoparib – Preclinical

Why This Model Scales

Because trials are:

- Academically anchored
- Institutionally funded
- Capital-efficient

Oncoheroes can:

- Run multiple trials simultaneously
- Expand its pipeline without exponential capital needs
- Act as both a pipeline company and a **partner of choice** for pediatric drug development

This is not a one-asset company.
It is a **platform for sustainable pediatric impact**.

A Long-Term Vision: Democratizing Ownership

Beyond advancing new therapies, Oncoheroes was created with a broader long-term vision: **to enable the childhood cancer community to actively participate in building—and sustaining—the industry partner it has long lacked**.

For decades, the childhood cancer community—led by foundations, families, clinicians, and advocates—has demonstrated extraordinary commitment, resilience, and generosity. In the United States alone, more than **500,000 childhood cancer survivors** are living today, with approximately **15,000 new pediatric cancer diagnoses each year**. Globally, more than **400,000 children are diagnosed with cancer annually**, creating a worldwide community of survivors, families, and supporters deeply invested—emotionally, financially, and morally—in improving outcomes.

Philanthropy remains essential to this ecosystem. It fuels discovery, supports families, advances advocacy, and underwrites early scientific innovation. Nothing in this vision is intended to replace or diminish the central role foundations play in the childhood cancer community.

Rather, Oncoheroes seeks to **expand the ways supporters can engage** by offering an additional, complementary pathway for those who wish to participate more directly in building long-term solutions.

Oncoheroes envisions a future where, alongside traditional philanthropy:

- Families, survivors, foundations, and supporters may choose to **invest as well as donate**
- Ownership becomes an **optional extension of advocacy**, not a substitute for giving
- Pediatric cancer drug development becomes a **shared**, **collective endeavor**, **globally inclusive** and mission-driven

Importantly, this approach is designed to **strengthen—not bypass—foundations**. By partnering with a biotech company dedicated exclusively to pediatric cancer, foundations and hospitals gain:

- A credible, long-term industry partner to share with donors
- A compelling narrative of **near-term**, **measurable patient impact**
- An additional engagement tool for supporters seeking deeper, sustained involvement

As Oncoheroes evolves, the company is exploring future structures—potentially including public-market pathways—that could allow broader participation over time. Such pathways would be intended to **complement existing philanthropic models**, enabling participation from supporters not only in the U.S. but across the global childhood cancer community. For funders, this represents a shift not away from philanthropy, but toward a **more durable continuum of impact**—where donations, grants, and mission-aligned investment **coexist and reinforce one another** in service of children with cancer, everywhere.

Conclusion

Oncoheroes represents a new category of biotech:

- **Purpose-built for pediatric cancer**
- Aligned with regulators, hospitals, and mission-aligned capital
- Focused on execution, collaboration, and sustainability

By bridging the long-standing gap between discovery and clinical development, and by empowering the childhood cancer community to help build the missing industry partner, **Oncoheroes is redefining what is possible—for children with cancer and for those committed to changing their outcomes**.