



CAC2 Patient Advocacy Industry Relationships (PAIR) Workshop

October 10, 2023 | Ottawa, Canada

MEETING REPORT



CAC2
COALITION AGAINST
CHILDHOOD CANCER

The PAIR
Project 


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Executive Summary

Participants gathered at the **CAC2 Patient Advocacy Industry Relationships (PAIR) Workshop** in Ottawa, Canada, on October 10, 2023, to address the critical need for effective partnerships between patient advocacy organizations (PAOs) and pharmaceutical companies in pediatric cancer drug development. Co-Chairs Caitlyn Barrett and Vickie Buenger facilitated the workshop that sought to explore the challenges, frustrations, and untapped opportunities in this space.

The workshop was moderated by Colab Health, where Gary Nolan took on the role of the primary moderator, with support from Tharshini Ragupathy and James Badman, who played roles in facilitating the sessions and documenting the proceedings.

Workshop Objectives

The workshop sought to establish standards and principles for pediatric oncology drug development to drive meaningful, productive, and ethical interactions between PAOs and pharmaceutical companies. By fostering such interactions, the goal is to expedite and enhance drug development for pediatric cancer patients, ultimately improving their access to effective treatments and care.

Session Highlights

Session 1: Setting the Scene

- We highlighted the importance of trust, which was introduced through prework questions and consistently reinforced during the workshop discussion.
- Participants unanimously agreed that:
 - trust, fostered through extended interactions and dialogue, is a crucial ingredient for collaborative efforts.
 - cultivating trust requires ongoing efforts, even when certain stakeholders are absent.
 - recognizing and respecting differing priorities was of paramount importance.

Session 2: Projects and Partnerships

- The group identified essential projects and partnerships that might arise between the PAOs and industry participants, highlighting the significance of diverse stakeholder collaboration.
- The projects emphasized empowering patients and families in clinical trial design and the need for a community-centered approach.
- Transparency, data sharing, and timely patient feedback emerged as key themes throughout the entire drug development pipeline, from the pre-clinical phase to post-registration and launch activities.

Session 3: Partnerships Pathway

- Diverse representation—encompassing various stakeholder groups such as patients, families, researchers, industry representatives, and PAOs—was emphasized as essential for a holistic and comprehensive approach to pediatric cancer drug development.
- Empowering patient voices, community-centered trials, and the importance of transparency in data sharing featured prominently.
- Projects were designed to include patients throughout the drug development process from clinical trials to data sharing.

Session 4: Establishing Mutual Standards and Principles

- Participants discussed the Patient Engagement Quality Criteria from Patient Focused Medicine (PFMD) to guide ethical engagements.
- Conversations centered on building a higher level of engagement and strengthening relationships, reflecting the changing dynamics of the last decade where the pharmaceutical industry has increasingly focused on pediatric cancer.
- The need to cultivate pediatric drug development champions within the pharmaceutical industry was emphasized.
- Participants came to the workshop with different expectations, which sometimes resulted in a mismatch in their approaches to collaborative work.
- The importance of valuing disease-specific drug development and considering various factors including ethical engagements, a comprehensive understanding of the diverse stakeholder landscape, continuity, and the role of champions, was underscored.

These discussions emphasized the evolving approach to patient engagement, moving away from a transaction-centered approach towards continued collaboration for the benefit of pediatric oncology patients and their families. While industry is increasingly engaging with PAOs in the pediatric oncology space, the transition is ongoing, requiring alignment of expectations and priorities. This shift in patient engagement aligns with broader industry trends, and PAOs are also adapting to these changing dynamics.

For detailed information and the full set of criteria discussed in Session 4, please refer to the Appendix.

Workshop Agenda*

TIME	SESSION
Workshop Introduction	
8:30 – 8:50 AM	Welcome and introductions
8:50 – 9:10 AM (20 min)	Background and objectives
Session 1: Setting the Scene	
9:10 – 9:55 AM (45 min)	Understanding and defining trust
Session 2: Projects and Partnerships	
9:55 – 10:40 AM (45 min)	Part A: Identifying essential projects and partnerships
10:40 – 10:55 AM (15 min)	<i>Coffee break</i>
10:55 – 11:55 AM (60 min)	Part B: Sharing and ranking essential projects and partnerships
11:55 – 1:10 PM (75 min)	<i>Lunch</i>
Session 3: Partnership Pathway	
1:10 – 2:25 PM (75 min)	Constructing a partnership pathway
Session 4: Establishing Mutual Standards and Principles	
2:25 – 3:10 PM (45 min)	Part A: Seven Patient Engagement Quality Criteria
3:10 – 3:25 PM (15 min)	<i>Coffee break</i>
3:25 – 4:40 PM (75 min)	Part B: Establishing mutual standards and principles
Meeting Close	
4:40 – 4:55 PM (15 min)	Workshop summary and conclusions
4:55 – 5:00 PM (5 min)	Final remarks

* Please note that the workshop agenda was agile and adapted in real-time to ensure the most productive and insightful discussions for all participants. Any modifications to the original sessions have been noted in the report.



Background

As part of the workshop introduction, workshop Co-Chairs Caitlyn Barrett and Vickie Buenger opened the session by extending a warm welcome to all participants and setting the tone for the day's discussions and activities. Notably, all PAO and industry participants were CAC2 members. PAO participants represented a diverse array of backgrounds, disease focus, and experiences, while industry representatives ranged from small biotech firms to substantial pharmaceutical companies. At the outset, Caitlyn and Vickie introduced the CAC2 values, which served as a unifying force, providing participants with a shared foundation for their discussions. They followed with an overview of the workshop objectives and introduced the theme of fostering effective partnerships between PAOs and pharmaceutical companies in pediatric cancer drug development.

Workshop Participants

- **Caitlyn Barrett**, Board Member, Coalition Against Childhood Cancer (CAC2)
- **Vickie Buenger**, President Emeritus, CAC2
- **Ann Graham**, Founder and Executive Director, MIB Agents Osteosarcoma
- **Christa Kerkorian**, Vice President of Patient Advocacy, Day One Biopharmaceuticals
- **Gavin Lindberg**, President & Co-Founder, The EVAN Foundation
- **Donna Ludwinski**, Director of Research Programs, Solving Kids' Cancer US
- **Joe McDonough**, President, The Andrew McDonough B+ (Be Positive) Foundation
- **Gwen Nichols**, Chief Medical Officer, The Leukemia & Lymphoma Society*
- **Vasiliki Rahimzadeh**, Assistant Professor, Medical Ethics and Health Policy, Baylor College of Medicine
- **Samantha Reyes**, Patient Engagement Lead: Pediatric Cancer/Neurology, Sanofi
- **Cesare Spadoni**, Founder & Chief Operating Officer, Oncoheroes Biosciences*
- **Amy Weinstein**, National Director of Research Investments, Pediatric Brain Tumor Foundation

*Bring unique perspectives as an academic researcher, clinician, industry representative, and PAO.



Apologies for Absence

- AstraZeneca
- Jazz Pharmaceuticals
- United Therapeutics

Note Regarding Absent Participants

Invited representatives from three of the six pharmaceutical companies were unable attend the workshop: AstraZeneca, United Therapeutics, and Jazz Pharmaceuticals. The relatively last-minute absences required the Moderator and Co-Chairs to make real-time revisions to the workshop format and working groups to maintain the event's effectiveness. Their absence, including both large and mid-sized companies, likely also limited the workshop's findings and led participants to note the importance of their involvement in driving meaningful advancements in pediatric oncology.

Attendees agreed to move forward and engage with absent parties retroactively after the workshop.

What Drives the PAIR Project?

CAC2 members were motivated to launch the PAIR Project based on experiencing several key challenges related to establishing and sustaining PAO/industry partnerships:

- **Limited Collaboration:** Historically, pediatric cancer PAOs and pharmaceutical companies have operated in siloes, with little collaboration in the vital realm of pediatric cancer research and drug development space. The limited interaction between these stakeholders can be attributed to the absence of dedicated pediatric-focused drug development efforts. However, as attention has increasingly shifted toward drug development for pediatric cancer, a new era of potential collaboration and advancements in pediatric oncology drug development has evolved.
- **Frustrating Engagements:** The few interactions that do occur often leave parties frustrated. PAO members have previously characterized their experiences with industry representatives as transactional, devoid of meaningful progress, and lacking effectiveness.
- **Perceived Problems:** Historically, PAOs have struggled to find common ground and meaningful project collaborations with pharmaceutical companies, while pharmaceutical companies often weren't connecting with the target pediatric audiences they need to further pediatric drug development and provide answers to key questions.
- **Actual Problems:** The lack of substantial cooperation between these stakeholders poses real challenges to advancing pediatric cancer drug development.
- **Consequences of Inaction:** Without improved collaboration, we risk stalling progress in pediatric cancer drug development and, ultimately, denying young patients the breakthrough treatments they urgently need.
- **Value of PAOs:** It's worth acknowledging that PAOs often serve as a primary resource to connect with patient populations. Despite the limited attention in the pharmaceutical world toward pediatric cancer, PAOs remain plentiful and resilient, equipped with valuable connections to families who could significantly contribute to the drug development process. This unutilized potential emphasizes the need for stronger collaboration.

Embracing the PAIR Project

The values presented below represent the guiding principles for all interactions within the CAC2 community. For this workshop, these core values have been thoughtfully extrapolated to serve as the foundational framework, ensuring that the PAIR Project aligns with CAC2's overarching principles and values in fostering effective collaborations between members and stakeholders.

The primary value underlying CAC2 is to put the children and their families first in everything we do					
	Respect	Efficiency	Inclusiveness	Amplify	Drive
CAC2 Values	Enable the community while respecting our members' unique missions	Strive for cost effectiveness and efficiency with resources	Emphasize inclusiveness and collaboration towards our common goals	Help give voice to the community and amplify it in a coordinated fashion	Stay action-oriented and flexible
PAIR Project Motivation	Empower our community to foster effective partnerships with pharmaceutical companies in pursuit of shared missions	Co-create a PAO and Industry Relationship (PAIR) framework with our members, for our members, ensuring efficient and effective implementation	Bring together PAOs and industry to assess successful PAO/pharma engagement models	Define PAO's expectations in collaborative partnerships with industry while meeting the needs of all stakeholders; working together as unified partners	Respond to an immediate and growing demand for PAO and industry collaboration to accelerate drug development

Workshop Objectives

The workshop aimed to establish standards and principles for pediatric oncology drug development to drive meaningful and ethical interactions between PAOs and pharmaceutical companies. By fostering such interactions, our goal is to expedite and enhance drug development for pediatric cancer patients, ultimately improving their access to effective treatments and care:

- **Assess** and evaluate successful PAO/pharma engagement models in drug development.
- **Identify** when patient and family engagement is required and when collaboration is essential in the drug development lifecycle.
- **Co-create** a best practice framework to support PAO/pharma collaboration throughout the drug development process.

Session 1: Setting the Scene

Icebreaker Exercise: Passion Projects

The session began with an icebreaker exercise to foster trust and connections among the participants. The “Passion Projects” exercise encouraged individuals to share their interests and hobbies outside their professional roles. Participants had the opportunity to discuss their side projects or hobbies, whether completed or upcoming.

Follow-Up Discussion: Defining Trust in Professional Relationships

Following the icebreaker activity, the group engaged in a discussion focused on key questions related to trust in professional relationships. The questions, which were distributed as pre-reads prior to the workshop, included:

- How do you create trust in your **professional** relationships?
- How would you describe a healthy ‘**business-to-business**’ relationship?
- What **actions** or **behaviors** do you believe build trust with colleagues and partners?
- What **strategies** or **experiences** have helped you maintain trust over time?

Key Discussion Topics

- Emphasized the significance of spending time together with members from both PAO and industry, virtually and/or in person, particularly extended interactions, and actively engaging in question-and-answer dialogues.
- Explored the importance of mutual recognition of differing priorities and the need to understand why certain important aspects to one party might not hold the same significance to others.

Key Themes

- **Financial Support vs. Relationship Building:** Participants recognized that focusing on transactions, like providing funding, doesn't create meaningful partnerships. They emphasized the importance of fostering enduring relationships between organizations, highlighting that meaningful collaboration depends on these strong, long-lasting connections, rather than relying solely on individual relationships.
- **Keeping the Door Open:** The group agreed that leaving room for open communication and extending the benefit of the doubt can strengthen relationships. It was suggested that making assumptions about disinterest should be replaced with understanding the limitations of the relationship.
- **Advocate Maturation:** Participants discussed the evolution of advocates and how experience leads to greater patience and realism. New advocates might be more impulsive, while experienced ones tend to be more patient and action-oriented.
- **Cultivating Industry Champions:** The need for strategies to cultivate industry champions, both internally and externally, was recognized. Capacity building was identified as vital for both patient advocates and industry representatives.
- **Defining Commitment:** It was suggested that more pharmaceutical companies should clearly define their commitment to pediatric cancer.
- **Mutual Value Creation:** Building "stickiness" in partnerships was discussed, emphasizing that both parties should create and receive value from the collaboration.
- **Demonstrating Selflessness:** Participants considered how to tangibly demonstrate that their actions are not self-serving and how to avoid assuming that others are behaving selfishly. An important yet less emphasized theme highlighted the challenge of navigating occasional competitive attitudes among PAOs for exclusive relationships or preferred status with industry partners. This underscores the importance of fostering open communication and enhanced collaboration among PAOs in the pediatric oncology space.

The discussions in Session 1 provided valuable insights into the foundations of trust, emphasizing the importance of mutual understanding, effective communication, and building genuine partnerships.

Session 2: Projects and Partnerships

In this session, participants were divided into groups to identify essential projects and partnerships between PAOs and pharmaceutical companies that seek patient/parent involvement and aim to integrate the patient voice in pediatric drug development.

Group 1 (Patient Organization Group 1)	Group 2 (Patient Organization Group 2)	Group 3 (Pharmaceutical Industry Group 3)
<ul style="list-style-type: none">• Gwen Nichols• Gavin Lindberg• Amy Weinstein• Caitlyn Barrett (Moderator)	<ul style="list-style-type: none">• Joe McDonough• Ann Graham• Donna Ludwinski• Gary Nolan (Moderator)	<ul style="list-style-type: none">• Christa Kerkorian• Samantha Reyes• Cesare Spadoni• Vickie Buenger (Moderator)

Projects Identified

- 1. Clinical Trial Engagement Council (Diversity and Inclusion Focus):** Collaborate with patients and families to design clinical trials and develop feasible trial protocols that prioritize patient participation without imposing significant challenges.
- 2. Building Equity in the Clinical Trial Space:** Facilitate knowledge-building around available clinical trials and encourage patients to explore opportunities beyond local clinics. This PAO-driven initiative, endorsed by the industry, aims to expand patient and family participation for improved clinical trial outcomes.
- 3. Exploratory Data Collection and Sharing:** Improve the reporting of clinical data by creating educational resources to assist and PAOs in sharing patient information. This initiative seeks to build a diverse patient pool for clinical trials and enhance recruitment efficiency.
- 4. Patient and Parent Panels:** Establish trust, build relationships, and ensure transparent communication between industry and PAOs. This creates a sense of safety and assurance among patients and families volunteering for initiatives. It also emphasizes putting a human face to data and closing the feedback loop.
- 5. Target Product Profiling:** Involve PAOs in early target discovery processes to address unmet needs in pediatric oncology.
- 6. Repurposing Drugs:** Identify diseases and/or pathways suitable for drug repurposing and determine the optimal points in the treatment pathway for testing these repurposed drugs.

7. **PAO/Industry Newsletter:** Develop a newsletter that provides information on mutual needs, collaborative ventures, and opportunities for cooperation between PAOs and industry.
8. **Multi-Stakeholder Strategy Forum:** Foster collaboration among industry, academia, and PAOs to facilitate their partnership in pediatric oncology drug development. This forum aims to identify and prioritize research projects of mutual interest.
9. **Collaboration Around Pooled Funding:** Implement the venture philanthropy model in pediatric oncology. This involves a patient-driven approach working with biotech and pharmaceutical companies to establish funds for critical initiatives.
10. **Diverse Patient Engagement in Clinical Trial Design:** Enhance patients' experience with pediatric diseases and improve their treatment journey. This objective focuses on understanding and addressing patients' perspectives, including their input on therapy innovation and design.

Key Discussion Topics

Mutual Areas of Interest

- **Patient-Centric Approach:** Participants across all groups expressed commitment to a patient-centric approach. They recognized the significance of involving patients and their families in various aspects of drug development and clinical trials. This shared emphasis on prioritizing the well-being and experiences of patients underscored the overall sentiment in the session.
- **Collaborative Partnerships:** There was a consensus on the importance of collaboration between PAOs and the pharmaceutical industry. Participants recognized that meaningful collaboration could lead to more effective and equitable drug development.
- **Transparency and Data Sharing:** Many participants highlighted the critical role of transparency and data sharing. They saw this as necessary to build trust between PAOs and industry, facilitating informed decision-making.
- **Multi-Stakeholder Engagement:** Multi-stakeholder and strategy forums resonated with participants to promote collaborative decision-making and knowledge-sharing among diverse stakeholders. This approach was seen to address the complexity of pediatric cancer drug development.
- **Empowering Patients:** Empowering patients and families to actively participate in data reporting and decision-making processes, such as clinical trial design, was a shared priority. Participants believed that this empowerment not only benefits patients but also contributes to better outcomes and drug development.
- **Standardization and Consensus:** Standardizing patient engagement practices, particularly within clinical trials, was recognized to ensure consistent and meaningful involvement of patients and their families. This theme of standardization indicated a desire for structured and effective collaboration.

Differing Perspectives and Priorities

- **Balancing Profit and Patient Advocacy:** Industry attendees might have been focused on the profitability and market success of their products. This could potentially conflict with the PAO's emphasis on patient welfare and empowerment. However, participants acknowledged the profit-seeking necessity of pharmaceutical companies to sustain their operations. They shared that this profit-seeking aspect isn't necessarily counterproductive to patient priorities and goals.
- **Regulatory Compliance:** Industry representatives may have concerns about regulatory compliance and navigating the approval process for new drugs. This might lead to different priorities than PAOs, which focus more on patient access to new treatments.
- **Resource Allocation:** Priorities regarding resource allocation can vary. Industry participants may have financial constraints and prioritize projects that offer a quicker return on investment. On the other hand, PAOs might prioritize initiatives that improve long-term patient outcomes.
- **Data Sharing and Privacy:** Handling patient data and privacy concerns can sometimes lead to differing viewpoints. Industry participants might aim to collect extensive data for research purposes, while PAOs may be more cautious about data sharing to protect patient privacy. However, PAOs believe that while patient privacy is important, patient families could be interested in providing their data if it meant that more effective treatments might be developed. This highlights the need to strike a balance between safeguarding privacy and encouraging family empowerment to share data.
- **Regulatory Interactions:** The timing and extent of industry and regulatory agency interaction can be a complicated issue for industry sponsors, which often makes them cautious and private about these interactions. However, they may benefit from early meetings with regulatory authorities that include academics and PAOs who have knowledge of the landscape and a stake in successful drug development.

The identified projects in each group emphasize the importance of patient involvement in various aspects of drug development. These projects are instrumental in promoting collaboration, trust, and inclusion between PAOs and the pharmaceutical industry. Simultaneously, they address unmet needs and enhance the overall patient experience, providing valuable insights to pharmaceutical companies for their clinical trials and drug development endeavors. This shared vision focused on putting patients and their needs at the forefront of decision-making and promoting multi-stakeholder engagement to achieve meaningful progress in the field.

For more detailed information and completed proformas from Session 2, please refer to the Appendix.

Session 3: Partnerships Pathway

In Session 3, participants embarked on a journey to delve deeper into the projects identified in Session 2. They worked to identify when the projects and partnerships take place along the drug development journey and who needs to be involved.

Although this session initially incorporated a ranking exercise, it organically transformed into a cooperative exercise focused on evaluating these initiatives. The projects identified in Session 2 were distributed across three diverse participant groups, including PAOs and industry representatives. Each group was charged with completing proformas for the assigned projects.

For more detailed information and completed proformas from Session 3, please refer to the Appendix.

Working Group 1	Working Group 2	Working Group 3
<ul style="list-style-type: none">• Gwen Nichols• Joe McDonough• Christa Kerkorian• Gary Nolan (Moderator)	<ul style="list-style-type: none">• Gavin Lindberg• Ann Graham• Cesare Spadoni• Caitlyn Barrett (Moderator)	<ul style="list-style-type: none">• Amy Weinstein• Donna Ludwinski• Samantha Reyes• Vickie Buenger (Moderator)

Key Discussion Topics

- **Target-focused Research Funding:** Discussing the importance of well-designed research and the necessity of contingency plans for funding challenges.
- **Collaboration Between Academics and Industry:** Addressing the need for academic research to be “fit for filing” and exploring strategies for aligning the interests of various stakeholders.
- **Patient Advocates’ Role:** Examining the challenges and opportunities for patient advocates in bridging the gap between informing research and successful implementation. Advocating for pediatric patients involves addressing age-specific nuances, such as representing young children or adolescents and balancing their unique needs and autonomy. In some cases, the advocate may be the parent, especially when the child is too young to advocate for themselves adding complexity to the role.
- **Clinical Trial Equity:** Evaluating strategies for ensuring equity and diversity in clinical trials, so that patients treated outside of cancer centers of excellence have equal opportunity to participate in clinical trials. This includes simplifying the process and providing education for patients unfamiliar with clinical trials.

- **Patient Data and Tissue Sharing:** Discuss how patient data is handled, including the responsibility for data shifting from clinical teams to patients and the need to bridge data sharing between academic institutions and pharmaceutical companies.
- **Standardization of Patient Engagement:** Addressing the importance of establishing a standard for patient engagement in clinical trials to ensure consistent and meaningful involvement of patients and families.
- **Community-Centered Approach:** Focusing on the need for community-centered clinical trials, engaging patients at local clinics, and making trials accessible, especially in underserved communities.
- **Empowering Patients as Drivers:** Discussing initiatives that empower patients and their families to take an active role in pediatric oncology research and drug development.

Key Themes

- **Diverse Collaboration:** Participants stressed the significance of diverse collaboration across various stakeholder groups, promoting a holistic approach to pediatric cancer drug development.
- **Empowering Patient Voices:** Several projects were centered on empowering patients and families, enabling their active participation in drug development and clinical trial design, ensuring their interests are well-represented.
- **Transparency and Data Sharing:** The importance of transparency, data sharing, and educational campaigns was emphasized, highlighting their role in bridging the gap between pharmaceutical companies and PAOs.
- **Patient and Parent Involvement:** Integrating the patient and parent perspective into disease understanding, marketing, and access decisions emerged as a key theme.
- **Timely Patient Feedback:** A focus on collecting patient feedback throughout the clinical trial process to understand patient experiences and needs, ensuring continuous improvements in the drug development journey.
- **Consensus and Standardization:** The need for consensus and standardization in patient engagement, particularly in clinical trials, to ensure consistent and meaningful involvement of patients and their families.
- **Collaborative Forums:** The idea of multi-stakeholder and strategy forums was discussed, highlighting their role in collaborative decision-making and information sharing among diverse stakeholders.

This session highlighted the pivotal role of diverse collaboration, transparency, and patient empowerment in shaping the landscape of pediatric cancer drug development. By actively involving patients and their families in educational campaigns and standardizing patient engagement, the aim is to create a more inclusive, equitable, and patient-centric approach to drug development.

Session 4: Establishing Mutual Standards and Principles

In Session 4, participants discussed the Patient Engagement Quality Criteria from Patient Focused Medicine (PFMD). These criteria were designed to guide the establishment of standards and principles for engagements between PAOs and pharmaceutical companies in the adult drug development space. The primary focus was ensuring these engagements were characterized by ethical conduct, transparency, and productivity. Participants shared their experiences and insights, considering how these criteria relate to their specific disease areas and patient communities.

For more detailed information and the full set of criteria, please refer to the Appendix.

Key Discussion Topics

- **Shared Purpose and Diverse Perspectives:** Central to the discussions was the concept of shared purpose, addressing preconceived biases, recognizing diversity in objectives, and understanding the unique characteristics of stakeholders.
- **Ethical Engagements:** The importance of ethical, transparent, and productive collaborations between PAOs and pharmaceutical companies was emphasized.
- **Continuity and Sustainability:** Participants discussed the significance of creating continuity and sustainability in these engagements, ensuring enduring relationships for building trust and achieving long-term objectives.
- **Tools for Shared Purpose:** Tools and frameworks to articulate shared purpose were explored such as the patient engagement criteria, especially when a shared purpose may not be immediately evident. The ongoing process involves identifying and developing these tools to facilitate mutual understanding among participants.
- **Self-Awareness and Flexibility:** The importance of self-awareness and flexibility in engagements, adapting to unique requirements for different situations, was highlighted.
- **CAC2 as a Hub:** The pivotal role of CAC2 as a central hub for pediatric oncology and PAOs, providing resources, tools, and frameworks for effective collaboration between these two stakeholder groups was emphasized.
- **Creating Pharma Champions:** The workshop underscored the importance of cultivating champions for patient engagement, both within the pharmaceutical industry at large and within specific pharmaceutical companies. These champions are seen as essential drivers of change and consistent efforts in advancing pediatric cancer drug development.

- **Paradigm Shift:** A key discussion point was to recognize that in the last decade, there has begun a significant shift in the approach to collaborations, with the pharmaceutical industry increasingly focusing on pediatric oncology and inviting PAOs to actively participate in shaping and influencing key initiatives.
- **Valuing Disease-Specific Drug Development:** The theme stressed the importance of not assuming that all drug development should be disease-specific, advocating for a more comprehensive approach.

These discussions underscored the importance of the ongoing transformation in the industry's approach to patient engagement and the need to continue building meaningful partnerships that serve the best interests of pediatric oncology patients and their families.



Appendix

The group’s detailed project proformas and insights can be found in the Appendix for reference and further exploration. These insights provide a valuable foundation for shaping future collaborations between patient advocacy organizations and the pharmaceutical industry in pediatric cancer drug development.

Session 2 Proformas

Project Title	Goals/Objectives	Project Scope	Expected Outcomes	Target Patient/Family Population	Measuring Success
Clinical Trial Engagement Council (Diversity and Inclusion focus)	Engage patients and families in clinical trial design to produce a feasible clinical trial protocol that doesn’t challenge patients’ ability to participate in trials. To ensure the inclusion of academics and PAOs in FDA conversations regarding drug development strategy.	Build advisory Council. Industry to interact regularly throughout the trial design. This council is to include the patient’s voice in study and output but not as an opportunity to endorse.	Engage patients and families in clinical trial design to produce a feasible clinical trial protocol that doesn’t challenge patients’ ability to participate in trials. To ensure the inclusion of academics and PAOs in FDA conversations regarding drug development strategy.	Clinical trial protocol that is feasible and doesn’t hinder the ability of patients to participate in trials.	The great differentiator for industry. More feasible and efficient trials.
Build Equity in Clinical Trials Space	Knowledge building around available clinical trials and encourage patients to move beyond local clinics. A PAO-driven initiative that is Industry endorsed to expand participation of patients/families for better outcomes.				
Patient and Parent Panels	To share patient stories (frontline, relapsed, or bereaved) with the industry. This will help to build relationships, trust, and full disclosure between the Industry and PAOs to create a feeling of safety and assurance when volunteering patients/families. The aim is to help the industry put a face to data and drive the positive feedback loop’s closure.	Virtual meetings are held on Zoom and recorded, and outputs are purposed for R&D, sales, and marketing. Questions should stimulate understanding of disease and have intention while allowing patients to tell their stories. Patients should sign NDAs and be compensated for their time. PAO to hold the industry accountable for closing the feedback loop.	The industry can put a face to data and understand that what they are doing is reducing patient suffering and driving meaningful change. Patients feel heard, and the industry creates a more tailored patient experience.	Disease-specific populations of relevance (frontline, relapsed, or bereaved). Parents accompany patients under 18 or patients over 18.	Industry to better understand disease experience and see the feedback contribute successfully to sales and marketing to benefit patients. Patients feel heard.

Session 2 Proformas (continued)

Project Title	Goals/Objectives	Project Scope	Expected Outcomes	Target Patient/ Family Population	Measuring Success
Collaboration Around Pooled Funding	To apply the venture philanthropy model to pediatric oncology.	A patient-driven process that works alongside biotech and pharmaceutical companies to build funds.	Encouraging companies to run pediatric studies and fund more drug development - venture philanthropy fund can propel this.		
Multi-stakeholder Strategy Forum	Giving industry a more prominent role: Industry, academia, and PAO to be partners in drug development. Helping to highlight what makes the research project of interest.				
Exploratory Data Collection and Sharing	To encourage the collection/reporting of clinical data (not clinical trial data) and create a patient organization-driven registry to generate a diverse patient pool for clinical trials and increase the efficiency of clinical trial recruitment.	To build a registry platform and educational materials that coincide with this to educate and encourage PAOs to share patient information.	A diverse patient pool for clinical trials. An informed patient population.	Disease-specific populations of relevance.	More efficient clinical trial recruitment.
Target Product Profiling	PAOs are involved in early target discovery (unmet needs).				
PAO/Industry Newsletter	To detail what PAOs and Industry can do for each other, a platform to share potential joint needs and ventures.	Resource of PAOs that can be introduced to Industry. To establish a trade association.			
Repurposing Drugs in Pediatric Oncology for Drug Development	To identify which diseases should be considered and where in the treatment pathway testing should occur.	PAO will inform the process/strategy for repurposing drugs.	Plan for clinical testing.	Disease-specific populations of relevance.	Repurposing for indication vs. taking off the shelf.
Diverse Patient Engagement in Clinical Trial Design	Improving patient experience, including disease and treatment journey. To understand what matters to patients, including thoughts on innovation and design.	A 1-year commitment to patient interviews/panels. Touchpoints throughout the study with patients/carers, not just a one-off interaction. Co-create patient materials, educational materials, disease awareness, and HCP soft skills with Industry.		Adolescent and young adult (AYA) patients and care partners are adults/adolescents. Mix of CT naïve and CT proficient. Only care partner panels, as they don't want to share worries with children. Bereaved parents.	Post-engagement incorporation into protocols. Measure burden, impact on R of R.



Session 3 Proformas

Project Title	Description	Key Individuals Involved: Identify the key individuals, roles, and organizations involved in the project.	Key Individuals Involved: <ul style="list-style-type: none"> Explain how critical individuals from all parties will work together to build and maintain trust. Think about behaviors and processes. 	Timing: <ul style="list-style-type: none"> Specify when along the drug development journey this project takes place. Specify the timeline for different phases or milestones of the project if applicable.
Clinical Trial Engagement Council (Diversity and Inclusion Focus)	Engage patients and families in CT design.			
Build Equity in Clinical Trials Space	Knowledge building around clinical trials to encourage patients to move beyond community clinics.	<p>PAO</p> <ul style="list-style-type: none"> Lay clinical trial search support. Provide a framework around the specific needs of the community. Build a knowledge base so patients who trust you can come to you for education. Conferences (in-person and virtual) for education Help build the knowledge base. <p>Industry</p> <ul style="list-style-type: none"> Accelerate the trial and improve recruitment. The number of sites increases the cost. Help understand CT needs and industry drug development process. <p>Other</p> <p>Academic CTs as well.</p>	<ul style="list-style-type: none"> Think about this from a disease-specific focus. PAO identifies multiple industry partners worth CTs + builds knowledge and educational resources. Packet for newly diagnosed disease. A free packet with meaningful info. English/Spanish/Mandarin. Talks/Q&A on disease in native languages. International tumor board - investigators learn from one another. Generate awareness around CTs. Equitable care doesn't always look the way you think it will look. 	
Patient and Parent Panels	Inform the industry on the impact of a disease within the organization. To inform medical marketing and access decisions.	<p>PAO</p> <ul style="list-style-type: none"> PAO identifies patients and parents (develops profiles of patients). <p>Industry</p> <ul style="list-style-type: none"> Company with a license to an asset. <p>Other</p> <ul style="list-style-type: none"> Payers/HTAs regulatory agencies, academic consortia, CROs, care team. Nurses, study coordinator. 	PAO and industry describe patient profiles. PAOs need to know what asset will ideally be used for/when in the treatment pathway.	<p>Pt feedback before, during, and at the end of the trial.</p> <p>All the way through.</p>
Collaboration Around Pooled Funding	To apply the venture philanthropy model to pediatric oncology.	<p>PAO</p> <ul style="list-style-type: none"> Due diligence Scientific ad board <p>Industry</p> <ul style="list-style-type: none"> Clear path to clinic Dedication to PAO communication 	A consortium of charities brings a clinical development project to the members. Feasible?	

Session 3 Proformas (continued)

Project Title	Description	Key Individuals Involved: Identify the key individuals, roles, and organizations involved in the project.	Key Individuals Involved: <ul style="list-style-type: none"> Explain how critical individuals from all parties will work together to build and maintain trust. Think about behaviors and processes. 	Timing: <ul style="list-style-type: none"> Specify when along the drug development journey this project takes place. Specify the timeline for different phases or milestones of the project if applicable.
Repurposing Drugs in Pediatric Oncology For Drug Development	Knowledge building around clinical trials to encourage patients to move beyond community clinics.	PAO <ul style="list-style-type: none"> PAOs that fund research (pre-clinical and clinical w/SABs) Connecting research with a pharma company willing to develop a molecule Industry <ul style="list-style-type: none"> R&D, clinical leadership (who is in charge of the asset) Other <ul style="list-style-type: none"> Academic Pediatric researchers Policymakers to incentivize repurposing 	PAO set objectives to identify promising targets and molecules to invest in.	<ul style="list-style-type: none"> Pre-clinical Phase Clinical Study Phase 1 Clinical Study Phase 2
Patient Data and Tissue Collection and Sharing	The educational campaign and invitation to patient families to submit their data to an existing patient/data registry.	PAO <ul style="list-style-type: none"> R&D through Phase 1 or 2 Industry <ul style="list-style-type: none"> Phase 2 through the end of the pipeline 	<ul style="list-style-type: none"> Education campaign to inform patient families about the need for their submission of data, records, etc. Create a bridge between pharma and PAO to help fund the creation of an education campaign. 	PAOs: <ul style="list-style-type: none"> Research and Discovery Phase Pre-clinical phase Clinical study phase 1 Pharma: <ul style="list-style-type: none"> Clinical Study Phase 2 Clinical study phase 3 Regulatory review and/or registration phase HTA Post-registration/ launch activities

Session 4 PFMD Patient Engagement Quality Criteria

Patient-Focused Medicines Development (PFMD) is a global consortium that aims to transform how medicines are developed by integrating the voice of the patient at every step of the process.

PFMD's goal is to improve global health by co-designing the future of healthcare for patients with patients. Its mission is to bring together initiatives and best practices integrating the patient's voice, accelerating the creation and implementation of a practical, globally standardized framework. The tool was co-developed with over 100 people from 51 organizations, representing patient associations, industry, academics, researchers, and external experts. The Patient Engagement Quality Guidance introduces seven quality criteria to assess patient engagement practices.



1. Shared Purpose

This refers to the project's aims and outcomes and the expectation that all stakeholders agree to them before starting the project.

Having processes in place to help facilitate discussions between all stakeholders will help identify each other's values, expectations, and objectives and allow parties to review and discuss priorities.

Consider:

- ways to encourage stakeholders to exchange views openly to allow a better understanding of the scope and objectives of the project.
- how to allow stakeholders to acknowledge that some of their objectives may differ.
- how all parties concerned can share a description of the common goals of the project.

2. Respect and Accessibility

This refers to:

- respecting each other and respectful interactions to be established among partners.
- openness to and inclusion of individuals and communities without discrimination.

Consider:

- optimal conditions to implement the project and how these will be established.
- ways to involve stakeholders who could benefit from and contribute to the project.

3. Representativeness of Stakeholders

This refers to the mix of people involved, which should reflect the project's needs and the interests of the target audience of the project outputs.

Consider:

- diversity in expertise, experience, demographics, and other relevant criteria.

4. Roles and Responsibilities

This refers to:

- all stakeholders agree on co-created roles and responsibilities.
- ensuring that all roles and responsibilities of the project are established upfront and revisited regularly by the team.

Consider:

- how roles and responsibilities can be agreed upon and allocated across stakeholders.
- processes involved in revisiting roles and responsibilities, adapting them if necessary, and ensuring all stakeholders are updated on changes.

5. Capacity and Capability for Engagement

This refers to:

- the need to have relevant and dedicated resources from all stakeholders.
- the need to understand the required capabilities of all stakeholders to enable meaningful engagement.

Both capacity and capability building are intended to facilitate participation and lower barriers to collaboration.

Consider:

- how stakeholders can support each other to build the required capacity and capabilities for this project and how this may entail different forms of training with or from sponsor organizations or stakeholders.
- how capability needs may vary depending on project needs or personal/professional circumstances.

6. Transparency in Communication and Documentation

This refers to establishing a communications plan and project documentation that can be shared with stakeholders regularly.

Communication among stakeholders must be open, honest, and complete.

Adequate, up-to-date documentation must facilitate communication with all stakeholders throughout the project.

Consider:

- ways to share progress updates proactively and openly throughout the project externally.
- methods to communicate project outcomes to all stakeholders, including how their contribution was of value to the project's success.

7. Continuity and Sustainability

This refers to the smooth progression of the project, as well as efforts to maintain an ongoing relationship with stakeholders.

Consider:

- the role of stakeholders beyond a single project.
- including the actions needed for maintaining the expected flow of the project from beginning to end in the project plan.
- plans to nurture relationships with your partners and stakeholders during the project, including when they might be needed beyond the project.



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