St. Jude Children's Research Hospital

World Health Organization

Closing the gaps: the Global Platform's approach to childhood cancer medicine access

A short introduction to the work and progress of the Global Platform for Access to Childhood Cancer Medicines

In collaboration with

unicef

PAHO Pan American Health Organization World Health Organization Americas Region

Strategic Fund

The Global Platform for Access to Childhood Cancer Medicines (Global Platform) aims to provide an uninterrupted supply of quality-assured cancer medicines to low- and middle-income countries (LMICs). St. Jude Children's Research Hospital (St. Jude) has committed to a US$ 200 million investment to support the development and launch of the Global Platform in partnership with the World Health Organization (WHO), the United Nations Children's Fund (UNICEF), and the Pan American Health Organization (PAHO) Strategic Fund.

This document provides a general summary of the work of the Global Platform for interested readers in the scientific and civil society communities. It is a summary document intended to provide a contextualized snapshot of the achievements, reflecting work undertaken by several agencies.

Updated June 2025

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नेपाल सरकार
स्वास्थ्य तथा जनसंख्या मन्त्रालय
भक्तपुर क्यान्सर अस्पताल
दुधपाटी, भक्तपुर

1 Where we started: a broken market and broken lives

1.1 The childhood cancer medicine market, fragmented and forgotten

The effectiveness of the childhood cancer medicine market is constrained by complex, interrelated factors, resulting in market fragmentation, lack of availability of essential medicines, low-quality products, and high purchasing costs (Fig. 1). *(1)*

A primary contributing factor is the inadequate purchasing of childhood cancer medicines in the public sector, which is compounded by a limited availability of diagnostics and treatment services. A World Health Organization (WHO) review involving 42 countries found that public financing for childhood cancer services was low: only one in eight low-income countries financed outpatient chemotherapy and covered a basic set of antineoplastic medicines for children with cancer; of 20 middle-income countries, only 12 financed outpatient chemotherapy and only 7 reimbursed basic childhood cancer medicines. *(2)* Often, the procurement of childhood cancer medicines is not centralized at the national level and forecasting processes are either not performed, not completed, or not coordinated. An analysis by WHO found that in at least 50% of countries, each individual hospital/treatment centre is managing its own procurement of childhood cancer medicines. This involves individual facilities procuring small volumes of medicines on the local market, through local suppliers who are unable to ensure uninterrupted supply as they lack adequate information or notice of the volumes needed. *(3)* As a result, the procurement of childhood cancer medicines is severely impacted by fragmentation.

Fig.1 Overview of primary challenges in accessing childhood cancer medicines

AVAILABILITY
of essential cancer medicines around the globe is inconsistent due to supply and demand issues and complicated regulations.

QUALITY
of medicines is threatened when governments and administrations favour lowest cost bids.

ACCESSIBILITY
at the point of care is challenged by the limited network of facilities providing medicines and care, their service capacity, and the absence of consistent delivery standards.

COST
of childhood cancer medicines is often excluded from budgets, creating financial hardship for families in low- and middle- income countries.

In the absence of a supply of reliable or sustainable health products through standard procurement channels, treatment centres receive medicines through nongovernmental organizations or local foundations. Procurement approaches outside standardized pathways may not be able to secure safe, quality-assured products and their use can result in stockouts and/or higher out-of-pocket expenditure. The smaller sizes and lower revenue margins of markets in low- and middle-income countries (LMICs), as well as their unreliability, mean that pharmaceutical companies or other relevant stakeholders do not have the incentive to develop, sell or register products in LMICs.

Given these characteristics – and the additional challenges posed by the absence of the necessary infrastructure and lack of access to end-to-end oncology treatment – there is little prospect of the situation resolving itself organically.

The smaller sizes and lower revenue margins of markets in LMICs, as well as their unreliability, mean that pharmaceutical companies or other relevant stakeholders do not have the incentive to develop, sell or register products in LMICs.

1.2 A small market with a big impact

Although the market for children's cancer medicines is viewed as relatively small commercially, the number of children with cancer worldwide is significant – and for every single child affected, the impact of cancer on them and their families is beyond measure. Accurately assessing the global paediatric cancer burden has been hampered by inadequate data availability in LMICs, where – due to different data collection systems – cancer mortality data are often not well linked to incidence data. This means there is the potential for significant underestimation of the actual mortality.

This stark difference in a child's chances of survival is perhaps the most profound inequality in cancer outcomes among all cancer types.

While survival in high-income countries is more than 80%, in many LMICs less than 30% of children diagnosed with cancer are cured.

1.3 A profound survival difference

The prognosis confronting a child at the beginning of their cancer journey varies dramatically depending not only on the type of cancer, but also on where that child lives. As is the case with so many other diseases, cancer reflects the inequalities and inequities that exist in society and health systems.

Whether a child survives after a cancer diagnosis is significantly linked to where that child is treated – that is, to the socioeconomic status of the child's family and the economic development of the country. Each year, approximately 400 000 children develop cancer, 80% of whom live in LMICs. While survival in high-income countries is more than 80%, in many LMICs less than 30% of children diagnosed with cancer are cured. *(4)*

The stark difference in a child's chance of survival observed between high-income countries and LMIC is one of the largest disparities among cancers and child health. Narrowing that gap will require a reduction in inequities in access to diagnostics, to medicines, and to essential health services.

The interplay of factors contributing to global inequality in childhood cancer survival is complex and rooted in frail health systems unable to meet the needs of children and their families, and in weak political prioritization of the disease. One of the fundamental root causes is that childhood cancer services are simply not available or affordable, resulting in children and their families not seeking, receiving, or completing care. Inequities in childhood cancer are a threat to accelerating improvements in cancer control and achieving health for all children, regardless of where they live.


THE REPUBLIC OF ZAMBIA
MINISTRY OF HEALTH
ISEASES HOSPITAL

2 What we set out to do

2.1 A goal to provide medicines to approximately 120 000 children in LMICs

In September 2018, at the third high-level meeting on the prevention and control of noncommunicable diseases during the United Nations General Assembly, St. Jude Children's Research Hospital and WHO, working with other global partners, launched the Global Initiative for Childhood Cancer. The target of the Initiative is to achieve a global survival rate of at least 60% for children with cancer, while ensuring that the suffering of all these children is reduced.

Access to childhood cancer medicines continues to be a major obstacle to achieving that goal of 60% survival for children with cancer. Many childhood cancer hospitals, particularly in LMICs, struggle to achieve consistent access to reasonably priced, safe, effective, and standard quality medicines for their patients. Children lack access to essential curative treatments, or experience prolonged interruptions in therapy, thus increasing their risk of treatment failure and death.

On 13 December 2021, St. Jude and WHO announced plans to establish the Global Platform for Access to Childhood Cancer Medicines to dramatically increase access to childhood cancer medicines around the world. The first of its kind, the Global Platform aims to provide an uninterrupted supply of quality-assured childhood cancer medicines, at no cost to LMICs participating in the pilot phase, where childhood cancer survival rates are often less than 30%, far below the rates in high-income countries. The goal is to reach 50 nations in the next 5 to 7 years, eventually providing medicines to treat approximately 120 000 children with cancer in LMICs and significantly reducing mortality rates.

2.2 Defining the four pillars of "access"

Delivering "access" to childhood cancer medicines rests on four pillars – availability, affordability, accessibility at the point of care, and quality assurance.

Availability focuses on maintaining consistent stocks at facilities by leveraging accurate forecasting, effective global procurement, and reliable national supply chains, with progress measured by the presence of medicines on shelves and the duration of stock-outs.

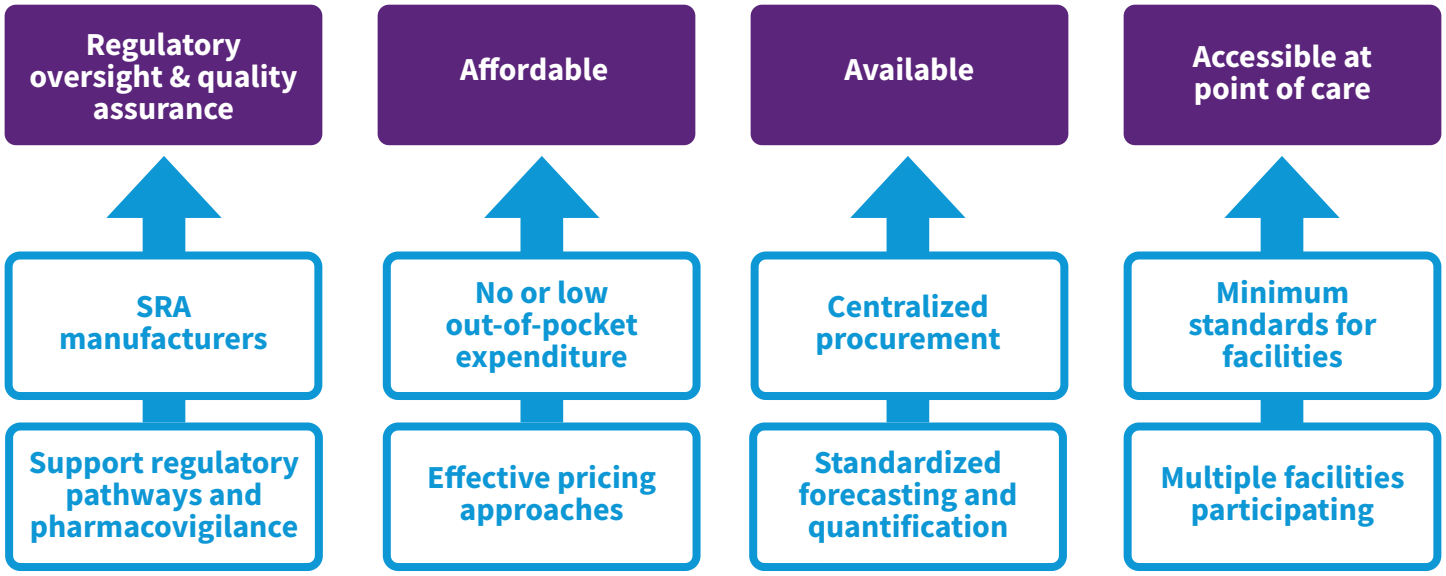
Affordability aims to reduce families' financial burden by securing the best possible prices and ensuring market stability, measured through reduced out-of-pocket expenses and increased use of services.

Accessibility at the point of care ensures medicines and care are delivered through a reliable network, close to children's homes, minimizing treatment delays or abandonment.

Quality assurance guarantees that all medicines are safe, efficacious, and of high quality through robust procurement systems, partnerships with reliable suppliers, and monitoring to detect Substandard and falsified medical products.

All four of these elements – availability, affordability, accessibility at the point of care and quality assurance – are necessary in order to bring about equitable and sustainable access to essential treatments for the most common childhood cancers (Fig. 2).

Fig.2 Quality-assured, affordable, available, and accessible at the point of care: the pillars of improved access to childhood cancer medicines



SRA: stringent regulatory authorities

2.3 How we set out to tackle it: theory of change

Recognizing the need to tackle all four of these aspects – availability, affordability, accessibility and quality assurance, the Global Platform understood that effecting change would require partners to bring their efforts to bear on multiple constraints at once.

The Global Platform is not a donation programme, but a co-created investment in health systems for cancer control. Therefore, although participating countries will be provided with children's cancer medicines free of charge, the driving purpose of the Global Platform's work is to bring about sustainable improvements in cancer care, with the goal of transforming the market for children's cancer medicines, with the intention that countries are able to access an affordable, quality-assured, and sustained supply long into the future.

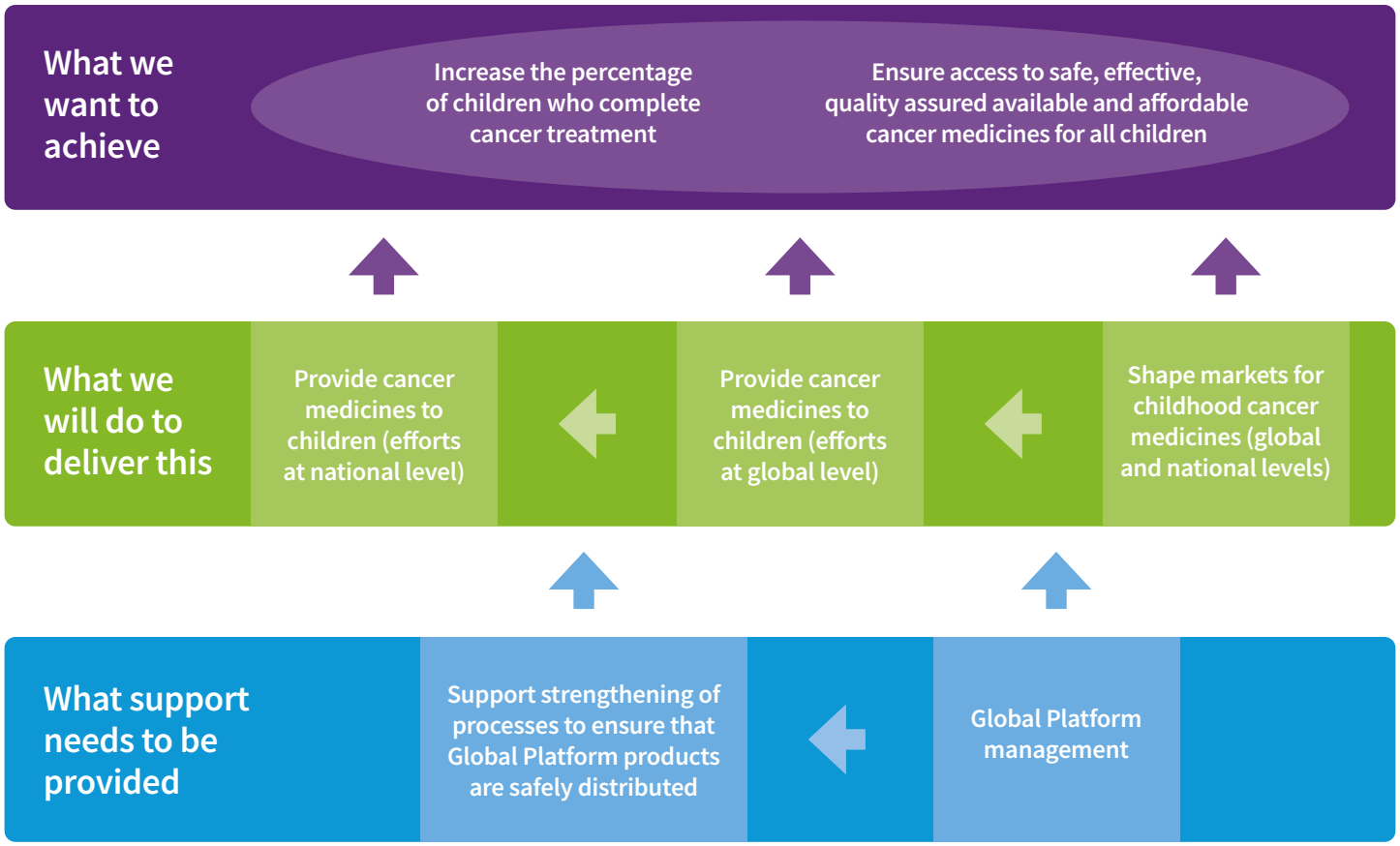
To deliver on this goal, efforts are focused on two key levels:

At country level, the Global Platform works with countries to strengthen processes to ensure that cancer products are received, distributed, stored and administered safely and effectively, as well as procuring cancer medicines for children, paid for by the Platform.

At global level, the Global Platform works closely with its procurement partners UNICEF and PAHO Strategic Fund, facilitating dialogue that allows it to identify and overcome barriers that stand in the way of an effective global market for childhood cancer medicines – with goals that include sustainability, affordability, and ensuring uninterrupted supply.

By integrating these efforts as shown in Fig. 3, the Global Platform seeks to create a comprehensive and sustainable solution that improves outcomes for children with cancer worldwide.

Fig.3 High-level theory of change



Box. 1

Global Platform List of Medicines (as of May 2025)

In childhood cancer treatment, clear and effective communication about available medicinal products is essential for high-quality patient care. To support healthcare providers, policymakers, and other stakeholders, the Global Platform has developed a curated list of childhood cancer medicines and product formulations.

The Global Platform List of Medicines is aligned with the World Health Organization's Model List of Essential Medicines for Children (EMLc), ensuring that medicines needed to provide complete, evidence-based treatment are included. By streamlining access to these targeted products, the Global Platform serves as a centralized, reliable source for essential childhood cancer medicines.

Our goal is to improve access to treatment across healthcare systems in low- and middle-income countries, by simplifying procurement and enhancing supply chain efficiency.

Allopurinol	Dasatinib	Mercaptopurine
Arsenic trioxide	Daunorubicin	Mesna
Asparaginase	Dexamethasone	Methotrexate
Bleomycin	Doxorubicin HCl	Pegfilgrastim
Calcium Folinate	Etoposide	Prednisolone
Carboplatin	Filgrastim	Procarbazine
Cisplatin	Fluorouracil	Rituximab
Cyclophosphamide	Hydroxycarbamide	Tioguanine
Cytarabine	Ifosfamide	Tretinoin
Dacarbazine	Imatinib	Vinblastine
Dactinomycin	Irinotecan HCI	Vincristine

3 Where we are now: supporting countries to strengthen systems

3.1 Working systematically to deliver medicines into countries

In January 2025, the distribution of medicines commenced across the six pilot countries of Ecuador, Jordan, Mongolia, Nepal, Uzbekistan, and Zambia.

This pivotal moment in the Global Platform's history is the culmination of over two years' work for, with and by countries.

In the initial phases of its work, the Global Platform's focus has been on developing strategic processes, laying solid foundations for the work ahead as it prepares to roll out participation to a wider group of countries.

The scope of the Global Platform's work has been far-reaching, which includes: working closely with national stakeholders on forecasting and quantification, supply chain management and regulatory barriers; collaborating with the procurement partners UNICEF and PAHO Strategic Fund to provide procurement planning information to suppliers; and articulating the Global Platform's own policies, consulting extensively to co-design and streamline its processes.

In its work with countries, the Global Platform is helping to overcome the various practical barriers that make it difficult for LMICs to introduce childhood cancer medicines.

For example, one component of this is facilitating pooled procurement in the form of a framework purchase order, leveraging the potential of consolidating multiple countries' medicine needs into fewer, larger orders, making them more cost-effective to manufacture and making it easier to demonstrate to pharmaceutical companies – with data – the size and value of the market for childhood cancer medicines.

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3.2 Laying solid foundations: technical cornerstones for strategic success

Many of the key achievements of the work so far have technical significance in laying the foundations for change, or have impact upstream of the more visible outcomes that will be seen in countries.

Nationally and globally, the Global Platform's work spans six distinct but interdependent work themes: governance, forecasting and quantification, clinical aspects, supply management, regulatory pathway, and monitoring and reporting.

The expertise required for each of these themes is technical in nature, requiring input from a variety of specialists with expertise across diverse fields such as paediatric oncologists and nurses, warehouse managers, hospital pharmacists, logistics experts and the regulatory authority. The early involvement of the national regulatory authority, for example, ensures that potential regulatory barriers can be identified in adequate time for the participating country to resolve these.


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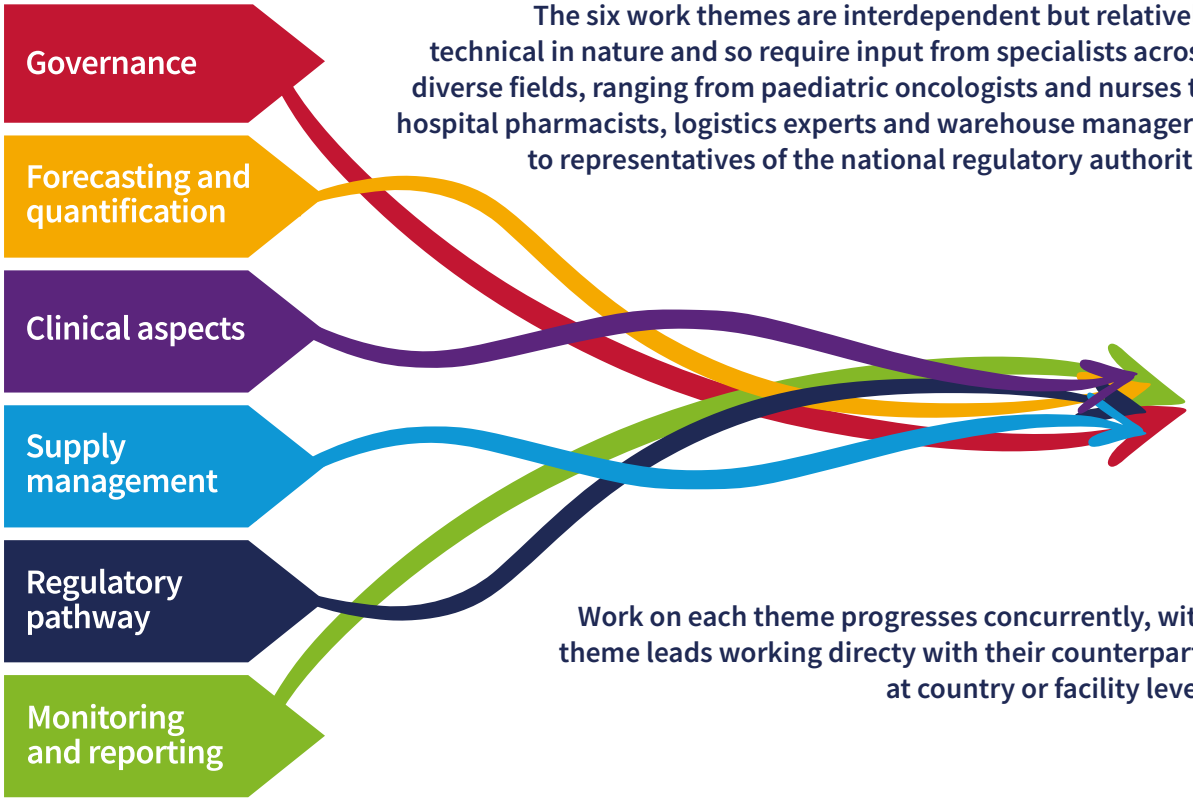
Fig.4 Six interdependent work themes



Across each of these work themes, partners have worked collaboratively and in detail to develop the technical cornerstones that have formed the foundation of its work with the six pilot countries, and will form a springboard for its work going forward. Some examples of this are given in the sections below.

3.3 Developing governance

The Global Platform has worked extensively to establish mechanisms for country-level governance of the processes. Key activities included:

- **Governance structures:** key national stakeholders actively participated in information-sharing networks, fostering transparency and accountability in medicine delivery. National technical working groups were established to facilitate in-country leadership and coordination.
- **Monitoring and evaluation:** a structured framework has been established to ensure continuous support in assessing the impact of interventions, leading to sustainable improvements in health care delivery. A data-driven approach was adopted to guide forecasting and data-informed decision-making.

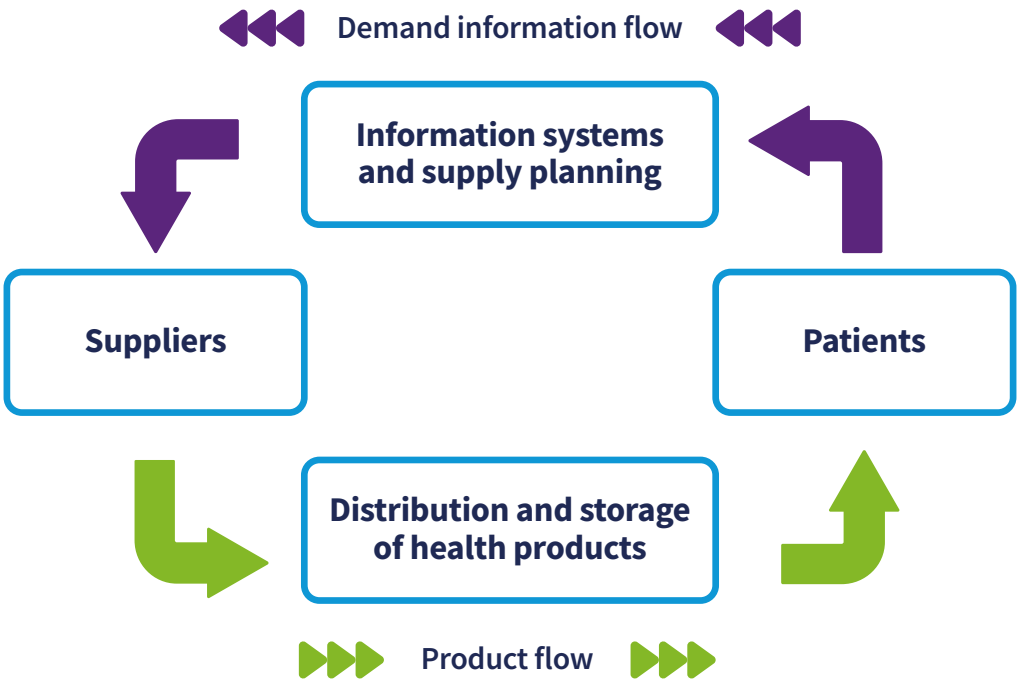
3.4 Strengthening information flows and streamlining procurement

Provisioning health supply systems requires the capacity to forecast how many patients will need treatment in order to increase the predictability of demand.

At present, inadequate forecasting and supply planning renders national procurement of childhood cancer medicines unpredictable and inefficient, contributing to unstable market size, compromising production planning and leading to unsuccessful tenders, resulting in frequent stock outs.

By supporting countries to strengthen their data gathering and forecasting systems, the Global Platform instead intends to contribute to a virtuous circle (see Fig. 5), whereby more accurate predictability of demand, coupled with the amplification of quantities through pooled procurement, can bring economies of scale and efficiencies of planning, minimizing supply interruptions and improving profitability for companies while enabling negotiation of lower prices. Working with countries to develop their demand information systems and supply planning has therefore been a key aspect of the work.

Fig.5 A virtuous circle: strengthening the market by supporting countries



The Global Platform team has worked extensively with the six pilot countries to strengthen forecasting and quantification and strengthen information flow. Forecasting and quantification is closely intertwined with procurement, as well as with clinical aspects of paediatric oncology.

Key activities included:

- **Forecasting and tender processes:** the Global Platform developed an efficient procurement strategy, incorporating various forecasting tools and country-specific disease burden assessments to generate national forecasting and compare with national consumption data, ensuring accurate forecasting and timely medicine supply. This included using an applied morbidity-based quantification tool that strategically quantifies cancer medicine demand by integrating epidemiological data, treatment protocols, and real-world utilization patterns. This supports cost-effective, data-driven decision-making as it enhances the precision in forecasting, optimizing procurement planning and thus helping to reduce stockouts and wastage.
- **Harmonizing and streamlining treatment protocols:** in the process of drawing up the Global Platform list of medicines, clinicians worked with pilot countries to harmonize and streamline treatment regimens, such that a smaller selection of cancer medicines and formulations could be used to effectively treat the same range of cancers, reducing stock inefficiencies. This helped to ensure consistency in care, enhance regulatory efficiency, and accelerate the approval of essential medicines like dasatinib, arsenic trioxide, and pegylated asparaginase into Ecuador's national essential medicines list. This approach also enables demand consolidation within the country and across regions, optimizing economies of scale and improving procurement efficiency to increase the affordability and availability of cancer medicines.
- **Centralized procurement:** assessments in the six pilot countries revealed the existence of multiple decentralized procurement systems, posing challenges to efficient medicine distribution and cost optimization. For many of the pilot countries, this was the first time they had used a centralized procurement mechanism for supply planning for their childhood cancer medicines.
- **Consolidated procurement approach:** the Global Platform facilitated the establishment of framework agreements to enable bulk purchasing, lowering costs and improving supply reliability. This joint tender by UNICEF and PAHO Strategic Fund for global childhood cancer medicines was the first of its kind.

3.5 Enhancing supply management

A robust procurement and supply chain system is critical for ensuring the uninterrupted availability of childhood cancer medicines. In addition to supporting the countries to enhance their use of demand information and procurement processes (see section 3.4 above), the Global Platform also accelerated strengthening of clinical and supply chain infrastructure.

The Global Platform team conducted extensive work to identify, assess and support implementation of the procedures, practices and systems countries need to have in place to ensure that products procured through the Global Platform can be received, transported, stored and administered safely and effectively once they arrive in country. This is enhancing countries' warehousing and distribution networks and helping to develop their human resource capacity through training and knowledge exchange.

Key activities included:

- **On-site assessments:** clinical and supply chain-management assessments were conducted in all six pilot countries. Global Platform experts spent hundreds of hours in countries to visit and assess each participating facility, identify gaps and provide technical and financial assistance to help countries undertake the activities necessary for them to be ready to safely store, transport, administer and dispose of medicines.
- **Minimum Standards** were developed to describe in detail what "ready" looks like, specifying the procedures, practices and systems that the Global Platform considers necessary. Adherence to minimum standards is assessed at ward, hospital pharmacy and central warehouse level, in relation to ambient and cold chain storage and for hazardous and non-hazardous medicines.
- **Development of standard operating procedures:** the Global Platform developed model standard operating procedures for safe delivery of chemotherapy and for facility inventory management, for adaptation by participating countries. These were developed for and utilized by the pilot cohort but will now be made available to countries joining the Global Platform in future.
- **End-to-end monitoring:** medicines were tracked from procurement to delivery at the port-of-entry in each pilot country, ensuring that essential medicines reached their intended destinations without disruptions.
- **Fostering cross-sector collaboration**, such as by facilitating the integration of national expertise between cancer and immunization programmes in Ecuador, in order to leverage best practices, especially in cold chain management of medicines.

3.6 Influencing the market for childhood cancer medicines

The Global Platform engaged in work to support its long-term vision of shaping the market for childhood cancer medicines.

Key activities included:

- **Facilitating new product introduction and regulatory waivers:** the Global Platform is supporting regulatory agencies to address regulatory barriers for essential cancer medicines.
- **Ensuring quality assurance:** Procurement was limited to products registered with stringent regulatory authorities.
- **Stabilizing the market:** through global tenders, the Global Platform is working to facilitate competitive pricing and fair market access, helping to prevent shortages and ensure affordability.

3.7 Impacts we're already seeing

The Global Platform represents a long-term investment towards a transformation that may take a long time to be fully realized.

However, we are already seeing some specific examples of how the Global Platform's work with countries and suppliers is already creating impact. Some of these are highlighted in the following boxes.

Box. 2

Nepal declares free treatment for cancer patients up to the age of 14 years through government designated institutions

Nepal became a Global Initiative for Childhood Cancer focus country in 2020 and joined the Global Platform in March 2024. The country has four hospitals with a dedicated unit for children with cancer, with 15 paediatric haematologists and oncologists, treating approximately 1500 new patients (ages 0–19) each year. The National Cancer Control Strategy endorsed by the Ministry of Health and Population (MoHP) incorporates components addressing childhood cancer. The country's commitment to prioritizing childhood cancer is evident through a series of actions including setting up the Standard Guideline for Wilms Tumour Management.

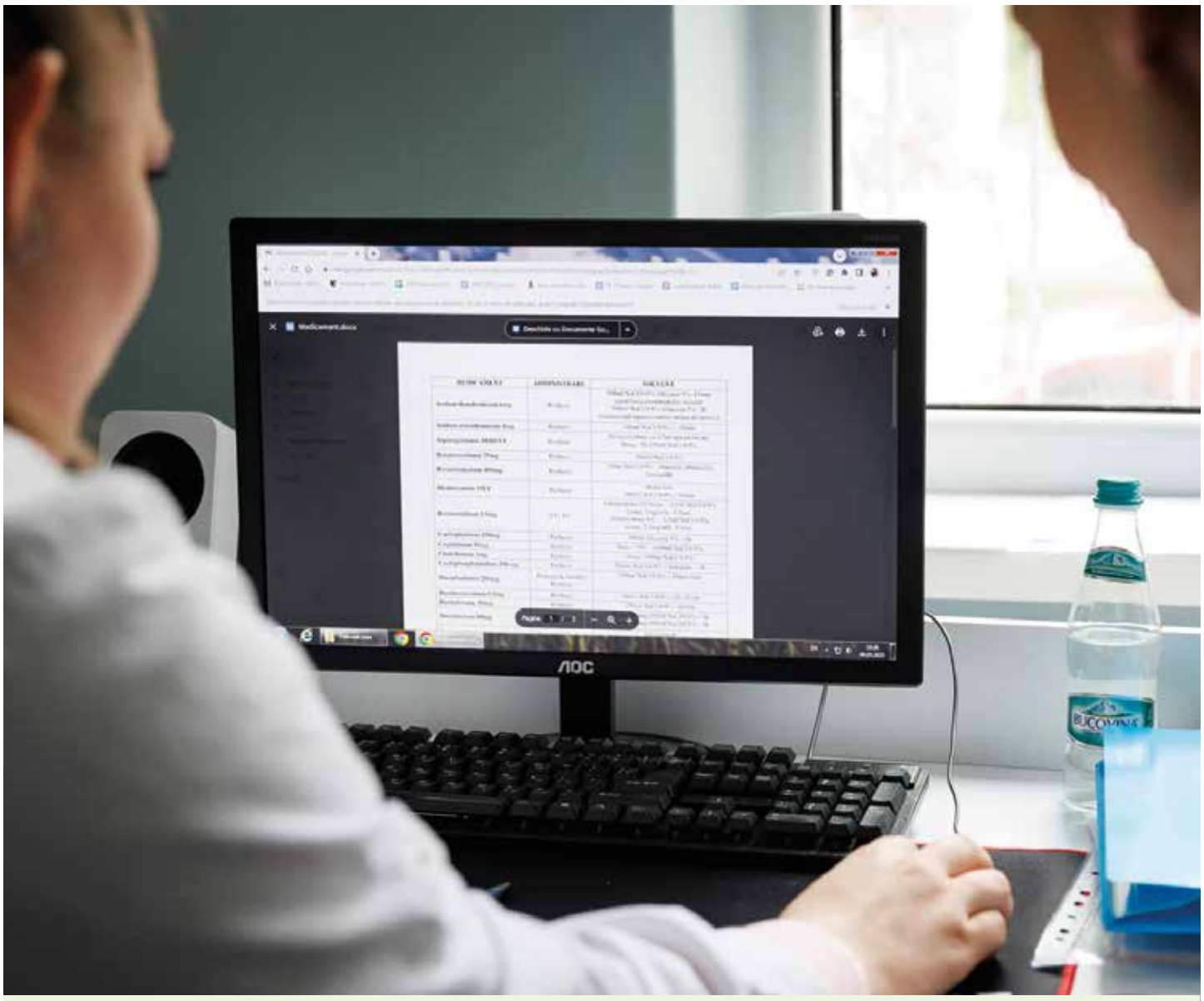
The involvement and support of the Government of Nepal have played a crucial role in the country's progress on childhood cancer. With effect from 16 November 2024, the Government of Nepal declared free treatment for cancer patients up to the age of 14 years through government-designated institutions – a decision influenced by the previous years of Global Initiative for Childhood Cancer activities and backed by the anticipation of medicines delivered through the Global Platform.

Box. 3

Ecuador adds three formulations to its national essential medicines list

Ecuador became a Global Initiative for Childhood Cancer focus country in 2021 and joined the Global Platform in July 2023. The country has 13 hospitals with a dedicated unit for children with cancer, with 26 paediatric haematologists and oncologists, treating approximately 1074 new patients (ages 0–19) each year. Ecuador is making significant progress in paediatric oncology care: efforts include introducing paediatric formulations, harmonizing treatment protocols, improving quantification, and strengthening storage and distribution.

Ecuador is becoming a regional pioneer in improving access to paediatric oncology treatment and has approved the inclusion of three key formulations – dasatinib, arsenic trioxide, and pegylated asparaginase – to its national essential medicines list. The formal publication of this update is underway and expected in the next edition of the national list. This important step, led by the national paediatric oncology working group, supported by the Ministry of Health and driven by the Global Platform for Access to Childhood Cancer Medicines, marks tangible progress towards ensuring equitable access to essential cancer medicines for children and adolescents.



Box. 4

Paediatric drug optimization for childhood cancer medicines *(5)*

Childhood cancers are often rare and can be biologically distinct from adult cancers, which means treatment strategies and drug development pathways are less well-established. Working with the Global Accelerator for Paediatric Formulations network (GAP-f), the Global Platform has been contributing its expertise to efforts to prioritize childhood cancer medicines listed in WHO Essential Medicines for Children for paediatric drug optimization, leading to the development of target product profiles.

A target product profile is a detailed description of the characteristics and specifications that it is desired a product should have at the end of its development, including the clinical, regulatory, and commercial attributes. It serves as a roadmap to guide development teams, from preclinical stages to market launch. Developing target product profiles is a strategic approach in the drug development process, that ties in directly to the Global Platform's goal of shaping the market for childhood cancer medicines.

Target product profiles ensure that the product will meet the unmet needs of the patient population while also addressing broader market considerations, such as reimbursement, accessibility, and adoption. The process of developing a target product profile allows pharmaceutical companies to engage with key stakeholders early in the development process – including oncologists, paediatric experts, regulatory bodies, patient advocacy groups, and payers. In childhood cancer, where there are often fewer options for treatment and limited market competition, stakeholder engagement becomes critical to market shaping. Childhood cancer treatment is especially challenging in low-resource settings: by contributing to the development of target product profiles that prioritize affordability, local production, or accessibility in diverse global markets, the Global Platform team has a chance to influence the shaping of the market for childhood cancer drugs on a global scale.

4 Where we go next: transforming the market to transform lives

4.1 Poised to power up

As the first deliveries of childhood cancer medicines procured by the Global Platform are arriving in our six pilot countries of Ecuador, Jordan, Mongolia, Nepal, Uzbekistan, and Zambia, the Global Platform is poised to power up its offer so as to be able to deliver medicines to more children in more countries.

Six additional countries – El Salvador, Ghana, Moldova, Pakistan, Senegal and Sri Lanka – joined the Global Platform in 2025 and have embarked on a supported process to get their countries' policies, practices and systems ready to safely receive, transport, store and administer cancer medicines procured through the Global Platform.

This new cohort of countries are benefitting from a new, more streamlined process of engagement with the Global Platform, thanks to our collaboration with and learning from the first six pilot countries. The Global Platform process has been designed with scalability in mind: the initiative is set to become the largest of its kind, with the goal of reaching 50 countries in the next 5 to 7 years, eventually providing medicines for the treatment of approximately 120 000 children with cancer in LMICs, significantly reducing mortality rates.

4.2 The long view: the future we want to see

Although the Global Platform's immediate focus is on its work with participating countries, its ultimate goal is to permanently change the landscape for access to children's cancer medicines for children everywhere.

By supporting participating countries to have – and to share with suppliers – better data about their childhood cancer burden and medicines needs, and by reducing upstream barriers that thwart an effective global market for childhood cancer medicines, the Global Platform has taken the first steps towards reducing the impact of significant constraints on the market that have prevented children's access to medicines for too long. In the long term, it is hoped that this will allow a more intentional approach that will also facilitate the development of child-friendly formulations, and pave the way for sustainable access to quality childhood cancer medicines for all countries.

The Global Platform has taken strong first steps towards transforming the market for childhood cancer medicines and transforming the lives of children with cancer everywhere. In 2025, we are shipping over 2.4 million medicines* to six countries, with additional medicines expected to be delivered to six more starting at the end of the year.

4.3 Join us

Governments interested in joining the Global Platform will find the country eligibility criteria and information about how to express their interest in participating on the Global Platform pages of the WHO and St. Jude Children's Research Hospital websites.

The Global Platform is starting to build its community of practice on the Knowledge Action Portal, a flagship online portal launched by the WHO Global Coordination Mechanism on the Prevention and Control of Noncommunicable Diseases. We actively collect expert opinions, country stories and impactful data from governments and would welcome your submission.

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* Representing 19 months of consumption (including some buffer to potentially mitigate supply chain issues)

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