Elizabeth Glaser Pediatric AIDS Foundation
Fighting for an AIDS-free generation

GAP·f Global Accelerator for Paediatric formulations
WHO Hosted Network

Building Trust for Global Health

Accelerating Access to Lifesaving Medicines for Children

A CALL TO ACTION FOR PARLIAMENTARIANS | UNITE GLOBAL SUMMIT 2024

The Clock is Ticking: A Child Dies Every 6 Seconds

Most causes of deaths in children could be prevented with effective treatment and interventions that are feasible for implementation, even in resource-constrained settings.[1]

However, appropriate medicines to save and improve the lives of infants and children often do not exist, are unavailable, or are not quality assured.[2] This puts children's lives at risk, hindering the achievement of the Sustainable Development Goals (SDGs) and universal health coverage targets.[3]

Ensuring access to essential medicines is an explicit component of the right to health, as enshrined in international human rights conventions. Article 24 of the Convention of the Rights of the Child states: "Parties recognize the right of the child to the enjoyment of the highest attainable standard of health and to facilities for the treatment of illness and rehabilitation of health."[4]

Through their legislative and advocacy roles, parliamentarians have a critical function in ensuring access to optimal pediatric medicines through leadership at national, regional, and global levels. This brief was prepared by the Elizabeth Glaser Pediatric AIDS Foundation with the support of the Global Accelerator for Pediatric Formulations (GAP-f) to join forces with parliamentarians promoting and protecting children's rights by ensuring access to optimal medicines.

The Reality: Children Are Still Waiting for Lifesaving Medicines

A child born today could wait up to 10 years before a new medicine for adults is tailored to their needs[5]

Medicines are usually first developed for adults; it is only when that process nears or achieves completion that the pediatric adaptation process typically begins. For example, bedaquiline and delamanid were the first new tuberculosis (TB) treatments to be developed in over 40 years, yet child-friendly formulations were not available until about seven years after the adult versions were on the market.[6] A 2021 analysis by the Access to Medicines Foundation showed that fewer than 7% of all assessed product development and deployment projects target children under the age of 12, revealing major gaps in pediatric drug development.[7]

The gaps in drug development span across disease areas and result in reduced access to lifesaving drugs.

- Less than half of WHO-recommended medicines for neglected tropical diseases are approved for children.
- The average delay from approval of cancer medicines for adults and their approval for children is 4 to 8 years.
- The approval process for some medicines for children has been delayed more than 20 years longer than approval for adults.
- Development of optimal HIV formulations still lags 8 to 10 years behind that of adults.
- In 2023, close to half of the children infected by HIV globally were not getting the treatment they need to live long, healthy lives.

The gaps in drug development span across disease areas and result in reduced access to lifesaving drugs. Recent analyses showed that less than half of WHO-recommended medicines for neglected tropical diseases (NTDs[8]) are approved for children.[9] There is a reported average delay of four to over eight years from approval of cancer medicines for adults and their approval for children. The approval process for some medicines for children has been delayed more than 20 years longer than approval for adults.[10] This is also the case for HIV, where development of optimal formulations still lags 8 to 10 years behind that of adults. Even when pediatric formulations exist, children face inequalities in access to those medicines. In 2023, close to half of the children infected by HIV globally were not getting the treatment they need to live long, healthy lives.

What's Standing in the Way: Barriers to Accessing Optimal Pediatric Medicines

Pediatric medicines represent a low-volume market. There may be limited epidemiological data for children, which disincentivizes pharmaceutical companies from investing. Difficulties in forecasting demand and pooling procurement mean that supply often cannot meet demand, and traditional incentives for innovation and manufacturing are insufficient on their own.

Clinical research in pediatric populations, especially neonatal babies, is particularly challenging. Even when new and innovative medicines are available with a pediatric indication, there is often limited evidence of long-term effects or risks. For example, a 2017 study revealed that only 11% of pharmacokinetic (PK) studies on medicines for NTDs included children, and for most medicines pediatric PK data were not available.[11]

The lack of harmonized regulatory guidance for the development and introduction of pediatric medicines slows down innovations.

There are logistical barriers to developing child-friendly formulations. Many antibiotics are not indicated for use in children and do not exist in optimal formulations for children.[12] When suitable formulations for children are designed, several factors should be considered. First, the need for reconstitution or refrigeration may be a significant barrier to widespread use in low- and middle-income countries (LMICs). Second, parenteral medications require intravenous access and health care worker expertise in administration. Third, excipients used for oral liquid or syrup formulation stability may be unsuitable for children.

Country systems are not always ready for the introduction of new medicines. These systems include health technology assessments, prioritization and policy development, investments, quantification, and planning and budgeting, among others. For instance, treatment with highly effective direct-acting antiviral drug regimens can cure hepatitis C infection and is recommended by WHO. But most countries, including LMICs with some of the highest disease burdens, do not have policies in place for the treatment of children.[13]

There are limited investments in community mobilization and advocacy to accelerate and sustain access to better medicines for children. Access to child-appropriate medicines is limited, even in countries with well-structured universal public health systems. Barriers include limited budgets, high costs, lack of clinical practice standards, inadequately trained personnel, the absence of supporting policies and structures, and limited awareness of (and therefore demand for) improved products.[14,15]

A lack of access to testing and of active case-finding strategies hampers treatment delivery and restricts essential market intelligence on demand for therapeutic products in many disease areas (e.g. HIV, TB, hepatitis C, and hepatitis B). In some cases, stigma and discrimination are additional barriers to the treatment of children affected by certain diseases.[16]

Pediatric drug research, development, and delivery are not supported by broad, dedicated funding mechanisms and targeted intervention programs. When actions and resources are not aligned across the full product development process, a single gap that prevents or stalls the transition from one phase to another can bring progress to a halt.[17]

A Parliamentarian Toolkit: Solutions Within Reach

Mobilizing leadership to implement existing policies and to improve the policy landscape for pediatric drug development and access is key to achieving better health outcomes for children. This brief proposes a set of solutions and possible activities for parliamentarians to use in their conversations when advocating for better pediatric medicines or for the cause of children's health.

Solution 1—GLOBAL ACCOUNTABILITY: Advocate for Faster Access to Essential Medicines

- Advocate for access to better medicines for children to remain on global, regional, and national agendas through advocacy and high-level political engagement. This can be done by ensuring accountability in the context of the World Health Assembly (WHA) and with WHO Member States; by advocating for access to medicines for children as part of global political forums (such as the G20, G7, and World Health Summit), and with regional institutions; and through active engagement in national-level debates and policies.

- Ensure accountability at all levels through the development and follow up of action items from specific resolutions, agreements, debates, and policies, and leverage clinical trials and diagnostics resolutions in advocating for access to care for vulnerable populations.

- Leverage pandemic prevention, preparedness, and response processes (such as those of the Intergovernmental Negotiating Body on a pandemic treaty) and integrate language on vulnerable populations in those high-level policy documents.

Solution 2—CHAMPION IN PARLIAMENT: Spearhead Access to Affordable Medicines for Children

Request a specific parliamentary inquiry on access to better medicines for children to gather evidence of the current country situation; this is a way to accomplish national and global health commitments and to promote and protect the rights of the child. This could include:

- Update the national essential medicine lists for children, monitor national pediatric medicine action plans and their implementation across national health systems, and train health workers and caregivers on the use and/or administration of optimal pediatric drug formulations.

- Ensure affordable prices for medicines for children through price negotiations or increased competition (including through the use of voluntary licensing agreements). Additionally, ensure that public insurance coverage schemes cover medicines deemed essential for children.

- Strengthen engagement of civil society, communities, and patient groups (including children), which contributes to successful introduction and sustainability of drugs through demand creation, service delivery, and accountability.

The World Health Assembly has taken important steps to improve access to better medicines for children through the adoption of resolutions such as:

- Better medicines for children (WHA60.20; 2007)

- Regulatory system strengthening for medical products (WHA67.20; 2014)

- Promoting innovation and access to quality, safe, efficacious, and affordable medicines for children (WHA69.20; 2016)

- Addressing the global shortage of medicines and vaccines, and the safety and accessibility of children's medication (WHA69.25; 2016)

- Strengthening clinical trials to provide high-quality evidence on health interventions and to improve research quality and coordination (WHA75.8; 2022)

The latest World Health Assembly resolution in 2024, entitled "Accelerate progress towards reducing maternal, newborn and child mortality in order to achieve SDG targets 3.1 and 3.2," calls on member states, stakeholders, and the WHO secretariat to enable access to essential high-quality and age-appropriate medicines for children through accelerating implementation of the actions laid out in resolutions WHA69.20 and WHA75.8 , and by promoting, supporting, and financing accelerated investigation, development, manufacturing, registration, and supply of age-appropriate, quality-controlled formulations of medicines for diseases that affect children.

Solution 3—INVEST SMARTER: Mobilize Resources for Optimal Pediatric Medicines

- Leverage government funding, development assistance, and philanthropic finances by shaping local pediatric markets, attracting impact investors, and increasing the funding available for innovative approaches to expand access to pediatric products in LMICs, thereby mobilizing additional resources for research, development, and introduction of better medicines for children.

- Advocate for organizations investing in research, development, and introduction of adult medicines to extend their work to include pediatric medicines and formulations.

Solution 4—INCENTIVIZE ACTION: Remove Regulatory Barriers and Facilitate Market Entry

- Advocate for investments in clinical research capacity and for the acceleration of the development of guidelines, norms, and standards for clinical research in neonates and children. Ensure feasibility and implementation of results and the gathering of real-world data to report on the outcomes.

- Advocate for streamlining regulatory procedures for priority pediatric medicines through the most efficient regulatory route, including through the use of collaborative registration procedures. Also, advocate for simplification and incentives to pediatric R&D processes by, for example, identifying and overcoming inefficiencies in regulatory procedures, and establishing targeted economic incentives such as market entry rewards.

- Advocate for pooled procurement (global and regional) to address small market challenges, for joint price negotiations and efforts to reduce transaction and operational costs. Accurate forecasting, demand creation, and strong advocacy to bring attention to, prevent, and solve stock outs and supply disruptions.

Solution 5—PRIORITIZE EFFORTS: Focus on Prioritized Drugs and Formulations

- Advocate for accessibility and affordability of pediatric formulations included in national essential medicines list, taking into account the WHO Model List of Essential Medicines (including the WHO Model List of Essential Medicines for Children). Regular updates of national essential medicine lists for children can facilitate the inclusion of optimal pediatric medicines in pharmacy stocks, reimbursement schemes, and hospital lists, and can support their effective distribution and availability across health care systems.

- Advocate for priority pediatric medicine formulations identified through the Paediatric Drug Optimization (PADO) process. The PADO process is a critical mechanism to identify priority drugs and formulations that need to be developed and delivered to fill important access gaps. The PADO has been applied to prioritize products and research questions across various therapeutic areas: antibiotics, pediatric cancer, COVID-19, hepatitis C virus, HIV, neglected tropical diseases, and TB.


Photo: Adelaja Temilade/EGPAF 2023

Collaboration is Critical

Collaborations among different stakeholders across the drug life cycle accelerate the pathway to improve treatment for children.

The development and approval of pDTG for effective use by children was two times faster than a common pediatric drug. This process benefited from 1) targeted efforts and joint action of researchers, industry, donors, and all relevant stakeholders on new chemical entities; 2) regulatory streamlining; 3) formulary optimization and tools for coordinated introduction and use; 4) strong advocacy driven by the HIV community for a rapid worldwide scale up.

pDTG supply started with 10 countries at the beginning of 2021 to more than 95 countries the end of the first quadrimester of 2024. The top 20 countries in terms of volumes supplied are almost entirely in sub-Saharan Africa.

The pDTG success story needs to be replicated for other HIV treatments and in other disease areas where gaps and challenges have been identified for pediatric drugs formulations, to ensure a long-term and sustainable options for pediatric treatments.

Photo: EGPAF 2023

A Call to Action: Lead the Change to Fast-Track and Secure Lifesaving Medicines for Children

EGPAF commits to advocate for better access to optimal medicines for children, particularly around HIV and TB. EGPAF commits to work with parliamentarians in country and globally by providing technical and advocacy support to ensure this topic is higher on the health political agenda and that adequate resources are available to accelerate research and introduction to better medicines for children.

A Call to Action:

Parliamentarians: Lead National Inquiries to Close Gaps in Pediatric Medicine Access

- Champion this topic in country through legislative functions and ensure this topic gets high-level attention in national political debate. This could happen through specific parliamentary inquiry where parliamentarians analyze current policies, practices, and gaps in access to optimal pediatric medicines; in these inquiries, parliamentarians could promote efficient and sustainable policies for better medicines for children, propose specific recommendations that are respectful of children rights, and advocate for their implementation nationally and globally.

UNITE Network: Elevate Pediatric Medicine Access as a Global Priority

- Consider access to medicines for children as a critical policy debate and discuss opportunities to champion this topic at national, regional, and global levels with concrete actions steps for parliamentarians.

- Consider ways to engage with the Global Accelerator for Pediatric Formulations (GAP-f) network to strengthen ongoing efforts for securing better access to medicines for children.

UNITE Network: Share Best Practices and Guide Parliamentarians in Fast-Tracking Medicine Access

- Support documentation of good practices and provide guidance to parliamentarians on opportunities to accelerate access to better medicines for children to achieve the SDGs and universal health coverage.

Endnotes

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About EGPAF

The Elizabeth Glaser Pediatric AIDS Foundation (EGPAF) is a proven leader in the global fight to end HIV and AIDS, and an advocate for every child to live a full and healthy life into adulthood. For more than 30 years, EGPAF has been a leader in meeting urgent needs in pediatric HIV and AIDS in the world's most affected regions. EGPAF leverages our core expertise in service delivery, capacity building, research, and advocacy to comprehensively address an evolving HIV and AIDS epidemic, and through our presence on the global stage, we advocate for the health and well-being of children as they transition into adulthood. For more information, visit www.pedaids.org.

About the Global Accelerator for Paediatric Formulations (GAP-f)

GAP-f is a WHO Network hosted within the Research for Health Department in the Science Division at WHO and was created to respond to the paediatric treatment gap. Following the resolution at the 69th World Health Assembly on promoting innovation and access to quality, safe, efficacious, and affordable medicines for children, GAP-f was conceived to build on and formalize the model developed within the HIV community to provide a sustainable mechanism that ensures that safer, more effective, and more durable paediatric formulations are developed and made available to children against an accelerated timeline. More information is available at https://www.who.int/initiatives/gap-f.