

Integrating paediatric praziquantel into schistosomiasis prevention and control

Building blocks for equitable access

Introduction

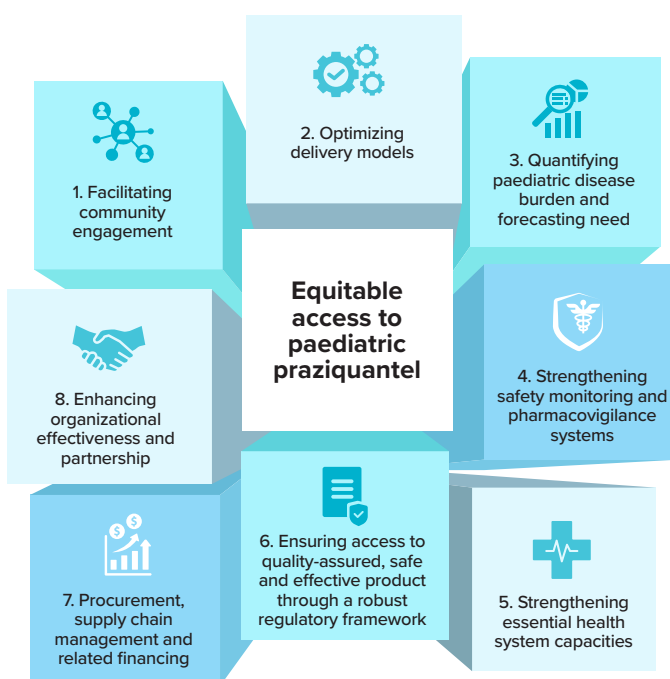
This document outlines the essential building blocks for successful introduction of the new paediatric treatment option for preschool-aged children with schistosomiasis (hereafter paediatric praziquantel). These are building blocks for both its initial introduction in endemic countries and communities, and for longer-term integration into existing schistosomiasis prevention and control programmes.

The eight building blocks outline the necessary capacities, processes and milestones for the successful introduction of paediatric praziquantel, allowing for customization based on specific needs and situations.

The building blocks framework considers the introduction of paediatric praziquantel – and control of schistosomiasis in preschool-aged children – as a critical component of achieving the broader goal of control and ultimately, elimination of schistosomiasis. They guide countries in tailoring each component to local needs.

This is designed as a dynamic and evolving document, which will be adapted and refined based on real-world experiences and feedback from the initial introduction of paediatric praziquantel in endemic communities.

Schistosomiasis, a chronic parasitic disease caused by blood flukes, is among the most socioeconomically devastating tropical diseases, second only to malaria. Globally, schistosomiasis is thought to afflict approximately 135 million school-aged children, and an estimated 50 million within the preschool age bracket (aged 3 months to 6 years). The disease is predominantly endemic in Africa, where it poses a significant public health challenge, particularly affecting children, who are highly



Eight essential building blocks for promoting equitable access to paediatric praziquantel

susceptible to infection due to their frequent contact with contaminated water. Beyond the immediate health consequences of persistently untreated infections among children – including anaemia, stunted growth and impaired cognitive development – the disease burden extends beyond childhood, with serious long-term effects into adulthood. The disease exerts profound social and economic impacts on affected individuals and communities, hampering educational achievement and economic productivity, and perpetuating cycles of poverty.

In 2020, the World Health Organization (WHO) published a [new road map](#) to guide action against neglected tropical diseases (NTDs) during the decade 2021–2030. It targets the elimination of schistosomiasis as a public health problem globally and the interruption of schistosome transmission to humans in selected countries by 2030. The road map identified access to treatment for all in need as a critical action. In addition, WHO published [guidelines](#) in 2022 that recommend the treatment and inclusion of preschool-aged children in preventive chemotherapy with age-appropriate formulations of praziquantel.

Despite their vulnerability to the disease, there has not been a formulation of praziquantel suitable for preschool-aged children largely due to the challenges related to dosing and palatability for this age group. The resulting treatment gap in this age group represents a glaring health inequity considering the availability of treatment for schistosomiasis in other age groups and also an obstacle to the achievement of the public health targets set for schistosomiasis in the NTD road map 2021–2030.

The journey towards the development of the first paediatric formulation of praziquantel has been a testament to the power of collaboration and innovation in addressing global health challenges.

Successful introduction of paediatric praziquantel hinges on a collaborative approach that leverages the strengths of various stakeholders. Through effective coordination, collaboration and partnership, endemic countries can overcome challenges to make significant strides toward the control and eventual elimination of schistosomiasis among preschool-aged children and other at-risk populations.

The [Paediatric Praziquantel Consortium](#) is an international partnership led by Merck. It comprises 11 public and private partners and 3 affiliated collaborators, and is supported by the Global Health Innovative Technology Fund (GHIT Fund) and the European & Developing Countries Clinical Trials Partnership (EDCTP). Over a decade-long process, the Consortium has been dedicated to the development, registration and equitable access of a treatment that is effective and palatable for preschool-aged children.

The United Nations Development Programme (UNDP)-led [Access and Delivery Partnership](#) (ADP) supports strengthening health systems to ensure equitable access to and sustainable delivery of new health technologies, tailored to national priorities. This is in line with the UNDP HIV and Health Strategy 2022–2025, which prioritizes strengthening health systems to scale up responses to tuberculosis (TB), malaria and NTDs, as a means of achieving universal health coverage.

[Uniting Efforts for Innovation, Access and Delivery](#) (Uniting Efforts) is a joint initiative of the Government of Japan, the GHIT Fund and ADP. In collaboration with WHO's Global Neglected Tropical Diseases Programme and the Paediatric Praziquantel Consortium, Uniting Efforts has facilitated multistakeholder consultations on accelerating access to paediatric praziquantel, in alignment with WHO guidelines. The consultations highlighted the key building blocks towards equitable access to paediatric praziquantel and underscored the collective effort required to achieve this goal.



Enabling equitable access: Eight key building blocks

Considerable preparations have already been made in anticipation of the introduction of paediatric praziquantel. Remarkable efforts by a wide array of partners have set the stage for this crucial phase. Looking ahead, the building blocks outline the essential steps needed to support introduction of the new formulation and ensure equitable access.

The building blocks are a means to:

- identify the crucial policy and programmatic building blocks for equitable access to paediatric praziquantel
- enable unified planning and synchronized actions; and,

- provide a standardized framework for national and international stakeholders to tailor according to their specific circumstances, goals and resource availability.

They outline strategic considerations and programmatic steps that require multi-stakeholder involvement across health systems, international and in-country collaboration, health investment and financing, and strong advocacy to elevate schistosomiasis as a priority on the health agenda at all levels.

Together, these elements form an adaptable plan for countries to assess their needs and readiness, build requisite support structures, and implement paediatric praziquantel introduction to maximize public health benefits.

Building block 1. Facilitating community engagement



Facilitating community engagement in the context of paediatric praziquantel introduction and broader schistosomiasis control efforts is pivotal for their success and sustainability. Active involvement and mobilization of local communities will not only generate demand and promote the uptake of paediatric praziquantel but will also foster a sense of ownership and responsibility towards eliminating schistosomiasis.

Early engagement and comprehensive mapping ensures that the rollout and uptake of paediatric praziquantel are met with enthusiasm and cooperation. A critical first step is the comprehensive mapping and analysis of relevant stakeholders at the local level.

Sensitization and capacity building of local stakeholders about paediatric praziquantel will enhance awareness and understanding and lead to greater acceptability and demand. During the pilot phase, sensitization training and exchange of experience can guide national programmes on developing social mobilization strategies tailored to the specific needs of target populations.

Community participation in the design and implementation of paediatric praziquantel delivery programmes ensures that interventions are culturally sensitive, well-accepted, and effectively address the needs of affected populations. Fostering community participation will help generate demand, mobilize caregivers and support tracking efforts for those who may require treatment.

The Paediatric Praziquantel Consortium is implementing the [ADOPT programme](#), which collaborates with country partners in Côte d'Ivoire, Kenya and Uganda to prepare for the large-scale delivery of paediatric praziquantel. The ADOPT programme addresses aspects ranging from social mobilization and acceptance by communities, to the assessment of existing drug delivery strategies and the establishment of monitoring and evaluation frameworks. In Tanzania, ADP partners have also collaborated with country stakeholders to plan and prepare for the introduction of paediatric praziquantel. Through the 'Delivery and uptake of paediatric praziquantel formulation for schistosomiasis' (STEPPS) initiative, engagement with community stakeholders raised awareness of the new treatment to improve acceptability and demand.

Building block 2. Optimizing delivery models



In endemic communities with a prevalence of *Schistosoma spp.* infection ≥ 10 percent, WHO recommends annual preventive chemotherapy in all age groups from two years old. In areas of higher prevalence (defined as areas with baseline prevalence ≥ 50 percent in school-aged children and persistent hot-spot settings already achieving high levels of coverage of annual preventive chemotherapy without appropriate response), WHO recommends that biannual preventive chemotherapy should be prioritized.

Optimizing the delivery models for paediatric praziquantel in different countries and localized contexts requires a multi-faceted strategy that aligns with both overarching schistosomiasis control programmes and the unique socio-cultural, economic and health system landscapes of schistosomiasis-endemic regions.

Delivery models may be adapted to fit the local context, health services and health system capacities, to ensure feasibility and sustainability. This involves comprehensive analyses to determine health system readiness, including infrastructure, workforce capabilities and existing health services that can support the integration of paediatric praziquantel delivery.

Integration of paediatric praziquantel within existing health systems and services is crucial for maximizing resource utilization and ensuring coherence with ongoing schistosomiasis control programmes. By embedding paediatric praziquantel delivery within established structures, countries can leverage existing networks and platforms, enhancing efficiency and reach.

Conducting formative studies will improve understanding of the barriers, gaps and challenges specific to local communities that may hinder effective access, delivery and uptake of paediatric praziquantel. These studies should aim to inform the selection of pilot areas, identify potential platforms to reach preschool-aged children, and reveal community perceptions and attitudes towards paediatric praziquantel treatment. Understanding these elements can guide the development of tailored strategies that address local hesitations and logistical challenges.

During the pilot stage, implementation research (IR) becomes indispensable for uncovering implementation barriers and gaps. IR provides real-world evidence on the feasibility, acceptability, coverage and cost-effectiveness of different delivery models, such as community/school mass drug administration (MDA), 'test-and-treat' strategies and integration into routine public health services.

Analyses on the management and cost-effectiveness of the last-mile logistics chain across various delivery models are also important, to inform resource allocation decisions, ensuring that investments in paediatric praziquantel delivery yield the highest possible returns in terms of health outcomes.

Information and lessons from the community engagement processes under the ADOPT and STEPPS initiatives will inform strategies that can be tailored to the specific needs of target populations, including the design of appropriate delivery models for paediatric praziquantel.

Building block 3. Quantifying paediatric disease burden and forecasting need



Although the true global prevalence and impact of schistosomiasis on the health of preschool-aged children remains largely unquantified, it is known that, in some settings, this age group can have prevalence rates exceeding 50 percent. Accurate quantification of the local schistosomiasis infection burden in preschool-aged children is important because it provides information for forecasting of paediatric praziquantel requirements for the target population.

To accurately quantify the treatment needs of preschool-aged children, the range of strategies for accessing treatment for preschool-aged children must be identified, including utilizing local primary health centres, implementing test and treat approaches, empowering health workers to identify and treat suspected cases, and incorporating preschool-aged children by extrapolating national and regional metrics on schistosomiasis. Child Health Days also offer a potential opportunity for reaching preschool-aged children. WHO guidance suggests that the test and treat approach is used in endemic communities with prevalence of *Schistosoma spp.* infection <10 percent and where there has not been a programme of regular MDAs.

Advancements in epidemiological methodologies permit the estimation of baseline prevalence utilizing existing demographic data alongside the WHO community data tool, obviating the need for extensive field surveys.

Building block 4. Strengthening safety monitoring and pharmacovigilance systems



In line with WHO guidance, the integration of routine safety monitoring into relevant public health programmes is a critical component of preventive chemotherapy programmes.

Post-introduction of paediatric praziquantel, national programmes must implement a risk-based pharmacovigilance approach. This encompasses processes to efficiently monitor, detect and manage suspected adverse reactions. Key pharmacovigilance activities include assessing system gaps and readiness, developing a pharmacovigilance plan for paediatric praziquantel, conducting necessary safety monitoring and ensuring data-sharing links between national, regional and global pharmacovigilance databases.

Building block 5. Strengthening essential health system capacities



The introduction of paediatric praziquantel presents a significant opportunity to enhance the global fight against schistosomiasis through a concerted effort to strengthen essential health system capacities. Addressing these localized and cross-cutting barriers will help to ensure the successful delivery and sustained impact of paediatric praziquantel, ultimately contributing to improved public health, child development and socioeconomic outcomes in at-risk communities. Key interventions to consider include:

Multisectoral coordination involving health, education, finance and community organizations to ensure a cohesive approach to paediatric praziquantel introduction and schistosomiasis control.

Human and institutional capacity building across the health system to improve relevant policy and regulatory measures, procurement and supply chain management and health workforce capacities.

Health technology assessment to provide scientific evidence to inform policy and decision-making on the prioritization of and resource allocation for new health technologies such as paediatric praziquantel.

National and global collaborations and innovative partnerships across the innovation, access and delivery value chain are essential. This includes engaging larger communities in the implementation process and leveraging experiences from other health interventions.

Digital health integration and accelerated digital transformation of health systems will enhance service delivery and quality, and support a more effective rollout of paediatric praziquantel.

Building block 6. Ensuring access to quality-assured, safe and effective product through a robust regulatory framework



The European Medicines Agency (EMA) [positive scientific opinion on the use of paediatric praziquantel for preschool-aged children](#) and the WHO prequalification listing, are pivotal moments, and set the stage for its anticipated addition to the WHO Model List of Essential Medicines.

Given the positive scientific opinion from the EMA, there are a number of expedited regulatory pathways available for the accelerated assessment and registration of the product in countries, followed by pharmacovigilance activities for safety monitoring. The collaborative registration procedure using stringent regulatory authorities' medicines evaluations is one expedited pathway available to countries. This provides the opportunity for participating countries to leverage the EMA's positive opinion, enabling them to accelerate the product review and product regulatory approval through the exchange of relevant product information between the EMA, the developing company and the relevant national regulatory agency (NRA), while preserving the NRA's sovereignty on the outcome.

At the national level, regulatory pathways and approaches for introducing paediatric praziquantel will be based on local jurisdictions and preferred national approaches. Alternative regulatory pathways, such as import waivers or special permits, may also be explored to allow for expedited access in the initial phase of introduction.

Moreover, member states of regional economic communities – such as the East African Community, Southern African Development Community and West African Health Organization – can employ existing full product review mechanisms for regulatory reliance in the region, joint assessments and work-sharing to expedite paediatric praziquantel's market authorization.

Building block 7. Procurement, supply chain management and related financing



Addressing the challenge of paediatric schistosomiasis necessitates a holistic and long-term strategy that emphasizes not only the immediate procurement and distribution of paediatric praziquantel, but also the broader issues of supply chain management and financing these efforts. The journey of paediatric praziquantel from production to administration involves multiple stakeholders, including manufacturers, health care providers, governments and international organizations, each playing a critical role in ensuring that treatments reach at-risk preschool-aged children.

First, the manufacturing and supply chain logistics for paediatric praziquantel must be robust and responsive to endemic regions' needs. Partnerships with local pharmaceutical manufacturers are critical for ensuring large-scale production and distribution. These collaborations not only support the localization of production but also aim to make paediatric praziquantel available at an at-cost basis in affected countries, facilitating broader access. In this regard, the Paediatric Praziquantel Consortium is [collaborating with partners](#) to enable large-scale production, including with Universal Corporation Ltd. in Kenya for the future provision of the treatment in endemic countries across Africa.

Secondly, sustainable financing mechanisms are imperative for effective procurement and distribution of paediatric praziquantel. Beyond traditional funding sources, [innovative options](#) – such as debt swaps, development impact bonds and pooled procurement, among other approaches – may warrant further evaluation for their potential to provide the financial sustainability needed for long-term procurement. These mechanisms may help ensure a predictable market and financing, and may help improve affordability of health technologies for governments and other purchasers, aligning the interests of governments, donors and manufacturers towards the common goal of eliminating schistosomiasis as a public health problem.

Lastly, integrating efforts to combat paediatric schistosomiasis, given its significant implications for child health, with broader global health agendas, such as the Sustainable Development Goals, as well as national health strategies and strategic development plans, can enhance the visibility of this NTD and secure more substantial and diversified funding and support.

Building block 8. Enhancing organizational effectiveness and partnership



The strategic sharing of knowledge and best practices among schistosomiasis-endemic countries and communities is a cornerstone of effective public health interventions. This approach, especially through ‘South–South’ collaboration, is pivotal in the design and implementation of programmes for the introduction of paediatric praziquantel and for the broader control of schistosomiasis. The integration of experiences, lessons learned and innovative solutions across regions can significantly enhance the efficiency and effectiveness of these health initiatives.

This should also be linked with leveraging of global advocacy platforms for sharing best practices, mobilizing resources and advocating for increased attention and investment in paediatric schistosomiasis treatment.

By fostering an environment of open communication, collaboration and mutual learning, endemic countries and communities can learn about and implement the most appropriate and effective programmes.

Key resources

1. Ending the neglect to attain the Sustainable Development Goals: A road map for neglected tropical diseases 2021–2030. Geneva: World Health Organization; 2020 (<https://iris.who.int/handle/10665/338565>).
2. WHO guideline on control and elimination of human schistosomiasis. Geneva: World Health Organization; 2022 (<https://iris.who.int/handle/10665/351856>).
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12. Discussion paper: Innovative finance for neglected tropical diseases. Abu Dhabi: Global Institute for Disease Elimination; 2023 (<https://glideae.org/resources/white-paper-innovative-finance-for-neglected-tropical-diseases/>).
13. Discussion paper: Landscape of funding and financing opportunities for access and delivery of health technologies for neglected diseases. New York: Uniting Efforts for Innovation, Access & Delivery; 2020 (<https://adphealth.org/resource/68/discussion-paper-landscape-of-funding-and-financing-opportunities-for-access-and-delivery-of-health-technologies-for-neglected-diseases/>).



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