

SCHISTOSOMIASIS

PEDIATRIC PRAZIQUANTEL CONSORTIUM

Treating preschool children



EDCTP

This project is part of the EDCTP2 programme supported by the European Union

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Schistosomiasis is one of the most prevalent parasitic diseases in Africa, and a very important one in terms of public health burden and economic impact. Left untreated, this poverty-related disease can lead to anemia, stunted growth and impaired learning ability, as well as chronic inflammation of the organs, which can be fatal in the most serious cases.



Scanning Electron Microscope image of a pair of *Schistosoma* blood worms. The adult female worm resides within the adult male worm's gynaecophoric canal.

The potential new pediatric treatment option (right) is a quarter of the size of the currently available 600 mg tablet (middle). The 10 yen coin (left) has a diameter of 2.35 cm or 0.93 inch.



As efforts focus on morbidity control and elimination, there is a pressing need to treat preschool-age children (3 months to 6 years of age). No suitable drug is available for this high-risk group, which accounts for an estimated 50 million of the approximately 250 million people requiring treatment¹.

THE STANDARD OF CARE TREATMENT

The existing 'standard of care' treatment for schistosomiasis is praziquantel (PZQ), which was developed in the 1970s. This oral anthelmintic is available as a generic drug and, in partnership with the World Health Organization (WHO), is donated by Merck for the treatment of schistosomiasis, mainly in school-age children in Africa. It is safe and effective, and a tablet formulation is available for adults and school-age children but not for those six years of age and younger.

The existing PZQ formulation is a racemic mixture of levopraziquantel (L-PZQ) and dextropraziquantel (D-PZQ). Only one of these components is pharmacologically active: the L-PZQ enantiomer. The other component, D-PZQ has been shown to be

inactive and significantly contributes to the bitter taste that makes treating young children difficult.

THE NEW PEDIATRIC TREATMENT OPTION

The Pediatric Praziquantel Consortium has developed a new pediatric treatment option that is suitable for younger children, including infants and toddlers. The new pediatric treatment option is a novel dispersible tablet containing levopraziquantel (L-PZQ), the biologically active PZQ enantiomer. The new 150 mg tablets are small, allow precise dosing, and have improved taste properties for preschool-age children.

The Consortium completed its clinical development program with positive results from its pivotal clinical Phase III trial. In December 2023, the European Medicines Agency (EMA) issued a positive scientific opinion of the new treatment option for schistosomiasis in preschool-aged children. In May 2024, the treatment was included in the World Health Organization (WHO) list of prequalified medicines.

1 WHO, Schistosomiasis Fact Sheet, 1 February 2023.

The Consortium

The Pediatric Praziquantel Consortium was founded in July 2012 as the first international, non-profit, public-private partnership in schistosomiasis, supported by world leading experts in tropical parasitic infectious diseases.

The Consortium has been working to develop, register and provide access to a pediatric medication for treating schistosomiasis in preschool-age children, with proven efficacy and safety and acceptable taste properties.

The new treatment option meets these criteria and also withstands the challenges presented by a tropical climate. The program has completed the clinical and regulatory phases and is currently preparing for equitable and sustainable access to the new medication.

The Pediatric Praziquantel Consortium is the only partnership targeting the preschool age range.

THE PEDIATRIC PRAZIQUANTEL CONSORTIUM SET-UP



The Consortium is financially supported by Merck, with in-kind contributions from partners and grants from the Bill & Melinda Gates Foundation (2012), the Global Health Innovative Technology Fund (GHIT) (2014, 2015, 2016, 2019 & 2020), and the European & Developing Countries Clinical Trials Partnership (EDCTP) (2018 & 2021), under its second program supported by the European Union.

THE IMPACT OF SCHISTOSOMIASIS¹

- More than **700 million** people at risk
- Approximately **250 million** people require treatment
- **Transmission** has been reported from **close to 80 countries**
- Up to **200 thousand** deaths per year
- Many **disabling** complications
- **High infection rates** in young children
- **No suitable treatment** for about 50 million preschool-age children



¹ WHO, Schistosomiasis Fact Sheet, 1 February 2023.
www.who.int/news-room/fact-sheets/detail/schistosomiasis

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