

# 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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Treating preschool children



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 new formulation (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 (under 6 years old). No suitable drug formulation is available for this high-risk group, which accounts for an estimated 50 million of the 220 million people already infected<sup>1</sup>.

## 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 generic drug and currently donated through the Praziquantel Donation Program of Merck KGaA, Darmstadt, Germany, via the World Health Organization (WHO), mainly to school-age children, to fight schistosomiasis in Africa. It is safe and effective, and a tablet formulation is available for adults and school-age children but not for those below 6 years old.

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 taste that makes treating young children difficult.

## THE NEW PZQ FORMULATION

The Pediatric Praziquantel Consortium has developed a pediatric formulation that is more suitable for younger children, including infants and toddlers. The formulation is smaller, orodispersible and has improved taste properties compared to the currently available 600mg tablet.

Two novel PZQ formulation candidates have been developed and tested by the Consortium: a racemic mixture (rac-PZQ) and an enantiopure version (L-PZQ). The clinical phase II trial testing both formulations in children (aged 2 to 6 years) confirmed the formulation with L-PZQ as the one to be pursued by the Consortium until registration. The Consortium program is in clinical Phase III to acquire confirmatory data needed by the regulators.

1 WHO. 17 April 2019. Schistosomiasis Fact Sheet. <https://www.who.int/en/news-room/fact-sheets/detail/schistosomiasis>

# 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 aims to develop, register and provide access to a pediatric praziquantel formulation for treating schistosomiasis in preschool-age children, with proven efficacy and safety and acceptable

taste properties. The formulation should also withstand the challenges presented by a tropical climate. The program is currently in the clinical development phase.

**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 KGaA, Darmstadt, Germany**, in-kind contributions by partners and by grants from the **Bill & Melinda Gates Foundation** (2013), from the **Global Health Innovative Technology Fund** (2014, 2015, 2016 and 2019), and the **European & Developing Countries Clinical Trials Partnership** (2018).

# THE IMPACT OF SCHISTOSOMIASIS

- More than **700 million** people at risk
- At least **220 million** people require treatment<sup>2</sup>
- Nearly **half** are school-age children
- **120 million** people with morbidity
- Many **disabling** complications
- **High infection rates** in young children
- **No suitable treatment** for preschool-age children

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2. <https://www.sciencedirect.com/science/article/pii/S1471492220301094>



Merck KGaA,  
Darmstadt, Germany

**Merck KGaA (Darmstadt, Germany)** leads the program. Expert in praziquantel, it provides resources and support from different areas: pre-clinical, clinical, drug substance/drug product development and manufacturing, regulatory and access. It is responsible for the clinical development program and is the sponsor of the clinical trials.



**Astellas Pharma Inc. (Japan)** has developed the new pediatric PZQ formulations, and provides expert advice on clinical development in children, and pharmacokinetic modeling.



The **Swiss Tropical and Public Health Institute (Switzerland)** is a world-leading institute in global health with a particular focus on low- and middle-income countries. Associated with the University of Basel, Swiss TPH combines research, services, and education and training at local, national and international levels. About 850 people from 80 nations work at Swiss TPH focusing on infectious and non-communicable diseases, environment, society and health as well as health systems and interventions.



**Lygature (The Netherlands)**, a not-for-profit foundation, acts as the independent coordinator of the Consortium, providing governance in terms of progress, finance and collaboration. Since 2006, Lygature has supported close to a hundred public-private partnerships in the field of life sciences & health, including poverty-related diseases.



**Farmanguinhos (Brazil)**, the federal governmental pharmaceutical laboratory of the Fiocruz Foundation in Brazil, brings unique expertise to addressing the production and distribution of new pediatric formulations in endemic countries.



The **SCI Foundation (United Kingdom)**, is a non-profit initiative that supports treatment programs against schistosomiasis and soil-transmitted helminthiasis in sub-Saharan African countries and Yemen. It will facilitate preparation and implementation of the Access and Delivery plan.



*In Search of Better Health*

**Kenya Medical Research Institute (Kenya)** provides expertise on local disease epidemiology, clinical trials and clinical care and will be responsible for the conduct of the trials in Kenya according to Good Clinical Practice and national and local regulatory and ethics standards.



**Université Félix Houphouët-Boigny (Côte d'Ivoire)** was previously involved in the phase II clinical trials of the pediatric praziquantel formulation. It provides expertise on local disease epidemiology, clinical trials and clinical care and will be responsible for the phase III trials in Côte d'Ivoire according to Good Clinical Practice and national and local regulatory and ethics standards.



[www.pediatricpraziquantelconsortium.org](http://www.pediatricpraziquantelconsortium.org)