SCHISTOSOMIASIS

PEDIATRIC PRAZIQUANTEL CONSORTIUM

Treating preschool children

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GHIT Fund

Global Health Innovative Technology Fund

EDCTP

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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 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 (as of 6 years of age). No suitable drug is available for this high-risk group, which accounts for an estimated 50 million of the approximately 240 million people requiring treatment[1].

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 as of 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 taste that makes treating young children difficult.

THE POTENTIAL NEW PEDIATRIC TREATMENT OPTION

The Pediatric Praziquantel Consortium has developed a potential new pediatric treatment option that is suitable for younger children, including infants and toddlers. The potential new pediatric treatment option is a novel (oro)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 the pivotal clinical Phase III trial. The program is in the regulatory phase and potentially nearing the patients in need.

1 WHO. 17 April 2019. Schistosomiasis Fact Sheet. 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 medication for treating schistosomiasis in preschool-age children, with proven efficacy and safety and acceptable taste properties. The potential new treatment option meets these criteria and also withstands the challenges presented by a tropical climate. The program is currently in the regulatory phase and preparing for equitable and sustainable access to the new medication, once registered.

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, in-kind contributions by partners and by grants from the Bill & Melinda Gates Foundation awarded in 2013, from the Global Health Innovative Technology (GHIT) Fund awarded in 2014, 2015, 2016, 2019 and 2020, and the European & Developing Countries Clinical Trials Partnership (EDCTP) in 2018 and 2021.

THE IMPACT OF SCHISTOSOMIASIS[1]

- More than **700 million** people at risk
- Approximately **240 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. 17 April 2019. Schistosomiasis Fact Sheet. https://www.who.int/news-room/fact-sheets/detail/schistosomiasis

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