

Press Release

# **GHIT-backed Innovation Achieves Landmark:**

## New Treatment Option for Schistosomiasis Reaches Preschool-Aged Children

**TOKYO, JAPAN** (March 5, 2025) — The Global Health Innovative Technology (GHIT) Fund is today announcing a significant milestone: the first administration of arpraziquantel to preschool-aged children in Uganda, in an implementation science setting. This new pediatric treatment for schistosomiasis, developed by the GHIT-funded Pediatric Praziquantel Consortium, is being introduced through the Consortium's ADOPT program, which focuses on integrating arpraziquantel into existing healthcare platforms in countries where schistosomiasis is prevalent. This achievement marks GHIT's first supported innovation to reach people in need since its establishment in 2013.

"This isn't just a scientific achievement," said Osamu Kunii, CEO of the GHIT Fund. "It's a testament to the transformative power of global collaboration. By bringing together a diverse array of partners, each steadfastly contributing their unique expertise and sustaining momentum throughout a decadelong marathon of innovation to shepherd this breakthrough (from bench to bedside), arpraziquantel's development journey has unlocked solutions that once seemed impossible."

Schistosomiasis, or bilharzia, affects 250 million people globally, including an estimated 50 million preschool-aged children, mainly in sub-Saharan Africa. Left untreated, this poverty-related parasitic disease can lead to anemia, stunted growth, and impaired learning ability, as well as chronic inflammation of the organs, which can be fatal. Until now, a child-friendly treatment specifically tailored for preschool-aged children was not available, leaving millions of preschoolers at risk.

Early treatment of schistosomiasis in preschool-aged children is a crucial investment in public health and societal wellbeing. By intervening at this critical stage, we not only aim to shield young bodies from severe complications like organ damage and cognitive impairment but also alleviate the long-term burden on healthcare systems and hopefully better livelihoods.

The Pediatric Praziquantel Consortium developed arpraziquantel to address this gap. Japan's Astellas Pharma Inc., as a founding member of the Pediatric Praziquantel Consortium, played a pivotal role by utilizing its proprietary technology to lead arpraziquantel's initial formulation development, resulting in water dispersible, climate-stable, child-friendly tablets with acceptable taste. The formulation was optimized by Merck in Germany; the manufacturing process served to produce clinical trial supply from Merck and Farmanguinhos in Brazil. Current manufacturing is done by Farmanguinhos; future large-scale production by Universal Corporation Ltd. in Kenya is planned in and for Africa.

"Japanese innovation is a cornerstone of arpraziquantel's development," Dr. Kunii added, "embodying GHIT Fund's core mission: harnessing the power of international partnerships to revolutionize global health."





GHIT Fund has supported arpraziquantel's development journey throughout the overall development program spanning over 10 years. GHIT has and continues to support the Pediatric Praziquantel Consortium in developing their access plans, with a particular focus on exploring new and sustainable access mechanisms. GHIT is also co-funding, with EDCTP, implementation via the Consortium's ADOPT Program, which is assessing different implementation platforms in three countries: Côte d'Ivoire, Uganda and Kenya. In addition, GHIT is coordinating, together with the consortium, incountry readiness preparations for introduction of arpraziquantel in additional countries such as Senegal and Tanzania.

"Understanding what needs to be in place at country level in terms of policy framework, regulatory framework, implementation models and domestic resource mobilization is critical to the successful uptake and roll out of arpraziquantel. We hope the lessons learnt from these countries will inform other countries," said Dr Isaac Chikwanha, Senior Director of Access at the GHIT Fund.

Arpraziquantel received European Medicines Agency's positive scientific opinion in December 2023 and was included into the World Health Organization (WHO)'s List of Prequalified Medicines in May 2024. Inclusion in the WHO's Essential Medicines List is expected in 2025.

#### 1. About schistosomiasis

Schistosomiasis (also known as bilharzia) is one of the most prevalent parasitic diseases worldwide and a very important one in terms of public health burden and economic impact. It is a poverty-related disease that is widespread in tropical and subtropical regions where large sections of the population have no access to clean water. Flatworms transmit the disease and people become infected with the parasite through contact with freshwater, for example, while working, swimming, fishing, or washing their clothes. The minuscule larvae penetrate human skin, enter the blood vessels, and attack internal organs. The infection rate is particularly high among children. Schistosomiasis is a chronic condition and is classified by the World Health Organization (WHO) as one of the 21 neglected tropical diseases (NTDs).

https://www.who.int/en/news-room/fact-sheets/detail/schistosomiasis

### 2. About arpraziquantel

The current standard of care treatment for schistosomiasis is praziquantel. Praziquantel is already approved, and suitable for school-aged children and adults. Extending the range of options for the treatment of schistosomiasis, arpraziquantel is tailored for preschool-aged children against *Schistosoma mansoni and Schistosoma haematobium*. Tested in <u>clinical development</u>, under the responsibility of Merck, arpraziquantel is a 150mg dispersible tablet. The prototype of its pediatric formulation was developed by Astellas Pharma Inc. in Japan, and further optimized by Merck in Germany.

In developing arpraziquantel, the Pediatric Praziquantel Consortium established a pediatric drug development program, divided into four major steps: preclinical development, clinical development, registration, and access. All details can be found on the <u>Consortium website</u>.





#### 3. About the Pediatric Praziquantel Consortium

The Pediatric Praziquantel Consortium is an international public-private partnership that aims to reduce the global disease burden of schistosomiasis and improve child health by addressing the medical needs of preschool-aged children. Its mission has been to develop, register, and provide access to a suitable pediatric drug for treating schistosomiasis in children 3 months to 6 years of age. For more information, and to see an overview of all Consortium partners, visit the Consortium website: www.pediatricpraziquantelconsortium.org

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The GHIT Fund is a Japan-based international public-private partnership (PPP) fund that was formed between the Government of Japan, multiple pharmaceutical companies, the Bill & Melinda Gates Foundation, Wellcome, and the United Nations Development Programme (UNDP). The GHIT Fund invests in and manages an R&D portfolio of development partnerships aimed at addressing neglected diseases, such as malaria, tuberculosis, and neglected tropical diseases, which afflict the world's vulnerable and underserved populations. In collaboration with global partners, the GHIT Fund mobilizes Japanese industry, academia, and research institutes to create new drugs, vaccines, and diagnostics for malaria, tuberculosis, and neglected tropical diseases. https://www.ghitfund.org/en

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