

Press Release

EMA Recommends Arpraziquantel for Treatment of Schistosomiasis in Preschool-Aged Children

GHIT has financed the Pediatric Praziquantel Consortium's development of arpraziquantel since 2013, investing 1.85 billion yen

TOKYO, JAPAN (December 18, 2023) — The Global Health Innovative Technology (GHIT) Fund has supported the Pediatric Praziquantel Consortium's development of arpraziquantel since 2013 through a total investment of approximately 1.85 billion Japanese yen. On December 15, the Consortium announced that the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has adopted a positive scientific opinion for arpraziquantel to treat schistosomiasis in preschool-aged children. Arpraziquantel is the first investigational drug to receive positive scientific opinion amongst the projects funded by the GHIT Fund. Present in 78 countries and endemic in 51, schistosomiasis affects more than 240 million people, about 50 million of whom are preschool-aged children. Leveraging Japanese innovation and leadership in the global fight against neglected tropical diseases (NTDs), GHIT Fund invests in global health R&D and facilitates transformational cross-border public-private partnerships.

The application was submitted by Merck, on behalf of the Consortium, under the EU-M4all procedure for high-priority medicines for human use intended for countries outside the European Union. For more details, please refer to the Consortium's press release. (www.pediatricpraziquantelconsortium.org)

Dr. Osamu Kunii, CEO of the GHIT Fund, said “This milestone represents a major step forward in realizing the promise of global health R&D to bring game-changing products to children in need of treatment. And the transformational Consortium partnership that underpins, it is truly a testament to the three pillars of our strategic plan - galvanize innovation, catalyze partnerships, and maximize impact. The critical role of Japanese expertise and technology in collaboration with global partners to accelerate this innovation is particularly meaningful to us.”

Despite the existence of a safe, effective treatment for adults and school-aged children, 50 million preschool-aged children have been left untreated in public health programs primarily due to the lack of an appropriate child-friendly medication. 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, through improving the taste of the tablets. The formulation was optimized by Merck in Germany; the manufacturing process served to produce clinical trial supply from Merck and Farmanguinhos in Brazil. GHIT is inspired to see Japanese innovation and

knowhow join forces with incredible partners worldwide to make a transformational impact on global health.

The positive CHMP scientific opinion by EMA is the basis for the potential inclusion of arpraziquantel into the World Health Organization's list of prequalified and essential medicinal products. Together with the positive scientific opinion, the planned prequalification will support the regulatory pathway in African countries. In Brazil, regulatory submission is under preparation by Consortium partner, Farmanguinhos. As the federal governmental pharmaceutical laboratory of the Fiocruz Foundation in Brazil, Farmanguinhos brings expertise in production and distribution, and will be the manufacturing site for the future introduction of the new pediatric medication in endemic countries. The partnership with Universal Corporation Ltd., Nairobi, Kenya is also supporting a planned future large-scale local production to serve African countries.

In parallel with this regulatory work, the Consortium's implementation research program (ADOPT) is preparing for the introduction of arpraziquantel in endemic countries in Africa, which GHIT Fund is supporting. To support equitable and sustainable access, it is essential that new pooled procurement and funding mechanisms are collaboratively explored and established by working with the Consortium and additional key stakeholders. The intent is to make the product available on an at-cost basis in sub-Saharan African countries.

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 20 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 [clinical development](#), 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. The manufacturing process served to produce clinical trial supplies from Merck and Farmanguinhos in Brazil. Future manufacturing is planned to be done by Farmanguinhos and Universal Corporation Ltd. in Kenya, which is preparing for extensive local production capacities in and for Africa.

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 [Consortium website](#).

3. EU-M4all

Through the EU-M4all procedure, the European Medicines Agency (EMA), in cooperation with the World Health Organization (WHO), can provide scientific opinions on high-priority human medicines, including vaccines, that are intended for markets outside of the European Union (EU). The procedure was previously known as the Article 58 procedure, as the legal basis is Article 58 of Regulation (EC) No 726/2004.

5. 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 is 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

6. Consortium Partners

- Merck (Germany)
- Astellas Pharma Inc. (Japan)
- The Swiss Tropical and Public Health Institute (Switzerland)
- Lygature (The Netherlands)
- Farmanguinhos (Brazil)
- Unlimit Health (United Kingdom)
- Kenya Medical Research Institute (Kenya)
- Université Félix Houphouët-Boigny (Côte d'Ivoire)
- Klinikum rechts der Isar der Technischen Universität München (Germany)
- Ministry of Health Côte d'Ivoire (Côte d'Ivoire)
- African Institute for Health and Development (Kenya)

Other collaborators that contribute to the mission of the Pediatric Praziquantel Consortium:

- Makerere University School of Public Health (Uganda)

- Ministry of Health Kenya, Division of Vector Borne and NTDs (Kenya)
- Ministry of Health Uganda, Vector Borne and NTDs Control Division (Uganda)

###

About Global Health Innovative Technology (GHIT) Fund

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>