

## **The European Medicines Agency (EMA) Validated Regulatory Application for Arpraziquantel, a potential Treatment for Schistosomiasis in Preschool-Aged Children**

- Arpraziquantel is the first investigational drug in regulatory phase amongst the different projects supported by the GHIT (Global Health Innovative Technology) Fund.
- GHIT Fund has financed the clinical development of the Pediatric Praziquantel Consortium program for arpraziquantel since 2013, by investing a total of 1.85 billion yen.
- Arpraziquantel, developed by the Pediatric Praziquantel Consortium, is a potential new treatment option tailored to improve child health by filling the treatment gap of an estimated 50 million preschool-aged children with schistosomiasis.

**TOKYO, JAPAN** (January 23, 2023) - Since 2013, the GHIT (Global Health Innovative Technology) Fund supported and invested in the Pediatric Praziquantel Consortium\*<sup>1</sup>, a public-private partnership dedicated to the development of arpraziquantel, a potential new treatment option for schistosomiasis \*<sup>2</sup> in preschool-aged children (3 months to 6 years of age). On behalf of the Pediatric Praziquantel Consortium, Merck submitted the regulatory application for arpraziquantel to the European Medicines Agency (EMA), which validated this application and started the scientific review process in December 2022.

Since its establishment in 2013, GHIT Fund has invested in a total of 114 projects and 28.4 billion yen. Arpraziquantel represents the first investigational drug that has reached the regulatory phase amongst the portfolio of projects supported by GHIT Fund.

Schistosomiasis is one of the most prevalent parasitic diseases, affecting the lives of around 240 million people, mostly in sub-Saharan Africa. The drug praziquantel – the current standard treatment developed in the 1970s – is safe, effective, and available for school-aged children and adults. At present, around 50 million preschool-aged children have been left untreated in public health programs primarily due to the lack of an appropriate child-friendly formulation of the drug.

Derived from praziquantel, arpraziquantel, developed by the Pediatric Praziquantel Consortium, is a potential new treatment option tailored to improve child health by filling the treatment gap of an estimated 50 million preschool-aged children with schistosomiasis. Arpraziquantel is a new (oro)-dispersible tablet; it is small (150 mg), has appropriate taste properties, can be taken with or without water, and withstands the hot and humid challenges presented by a tropical climate. It was developed by Astellas Pharma Inc. in Japan, subsequently optimized by Merck in Germany and transferred for manufacturing clinical supply to Farmanguinhos in Brazil. At the moment, arpraziquantel is not approved for use in any country.

Since 2013, GHIT Fund has supported the clinical development of the Pediatric Praziquantel Consortium program for arpraziquantel by investing a total of 1.85 billion yen \*<sup>3</sup>. The full clinical program included a Phase I study in adult volunteers, a taste study in children, a Phase II dose-finding study, and a pivotal Phase III trial – all conducted in African countries.

Osamu Kunii, CEO of the GHIT Fund, said, "This achievement would not have been possible without the tireless efforts of the Pediatric Praziquantel Consortium, which led the implementation of the

clinical program, as well as the cooperation with the families and communities for the participation in the clinical trials. I would like to thank the many stakeholders for the successful collaboration over the years."

With the regulatory filing stage complete, the Pediatric Praziquantel Consortium is preparing for potential inclusion of arpraziquantel in the World Health Organization list of prequalified and essential medicines. It is exploring new mechanisms for providing equitable and sustainable access to arpraziquantel, once approved. Through its dedicated implementation research program, ADOPT, the Pediatric Praziquantel Consortium is collecting data and best practices that will inform future large-scale delivery programs in endemic countries. The aim is to start the launch phase in 2024 for product availability on a not-for-profit basis, initially in sub-Saharan African countries.

\* 1 About the Pediatric Praziquantel Consortium

The Pediatric Praziquantel Consortium is an international not-for-profit partnership that aims to reduce the global disease burden of schistosomiasis by addressing the medical needs of infected preschool-age children. Its mission is to develop, register and provide access to a suitable treatment option for schistosomiasis in this age group. Consortium Partners are Merck (Germany), Astellas Pharma Inc. (Japan), The Swiss Tropical and Public Health Institute (Switzerland), Lygature (The Netherlands), Farmanguinhos (Brazil), The SCI Foundation (UK), 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) and African Institute for Health and Development (Kenya). For more information, visit the Consortium website: [www.pediatricpraziquantelconsortium.org](http://www.pediatricpraziquantelconsortium.org)

\* 2 About schistosomiasis

Schistosomiasis (also known as bilharzia) is one of the most prevalent parasitic diseases in sub-Saharan Africa, caused by parasitic flatworms called schistosomes, of which *Schistosoma mansoni* and *S. haematobium* are the two major species. The disease affects almost 240 million people, mainly in communities without access to safe drinking water and with poor sanitation, with an estimated number of deaths of about 200,000 per year. The parasites live within freshwater snails and infect humans by penetrating the skin. The disease can lead to chronic inflammation of the organs, which can be fatal but also to anemia, stunted growth, and impaired learning ability with devastating consequences for the lives of the young children.

\* 3 GHIT's investment in the Pediatric Praziquantel Consortium

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The GHIT Fund is a Japan-based international public-private partnership fund (PPP) 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 and manages an R&D portfolio of development partnerships aimed at addressing neglected diseases such as malaria, tuberculosis, and neglected tropical diseases that afflict the world's vulnerable and underserved populations. The GHIT Fund mobilizes Japanese industry, academia, and research institutes to create new drugs, vaccines, and diagnostics for malaria, tuberculosis, and neglected tropical diseases, in collaboration with global partners.  
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