

PRESS RELEASE

European Medicines Agency Validates Application for Arpraziquantel to Treat Schistosomiasis in Preschool-Aged Children

- 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
- A positive scientific opinion by the European Medicines Agency will facilitate registration of arpraziquantel in African endemic countries
- Arpraziquantel forms part of an ongoing effort to eliminate schistosomiasis

2 December 2022, Utrecht, The Netherlands. Today, the Pediatric Praziquantel Consortium announced that the European Medicines Agency (EMA) has validated for review the application for arpraziquantel for the treatment of schistosomiasis in preschool-aged children (3 months to 6 years of age). With this validation, the regulatory application for arpraziquantel is complete and EMA will now begin the scientific review process.

Derived from praziquantel, the standard of care treatment developed in the 1970s, arpraziquantel is tailored to meet the needs of preschool-aged children affected by schistosomiasis. This group of approximately 50 million patients currently lacks a suitable treatment option. Arpraziquantel is a novel dispersible or orodispersible tablet (150 mg). It can be taken with or without water, is palatable for preschool-aged children, and withstands the hot and humid challenges presented by a tropical climate.

The Consortium, an international public-private partnership, [successfully completed its clinical development program at the end of 2021](#). In its pivotal Phase III trial, the primary efficacy endpoint of clinical cure was met with a favorable safety profile. Adverse reactions observed in clinical studies were similar to those reported for praziquantel.

On behalf of the Consortium, Merck KGaA, Darmstadt, Germany submitted the application for a scientific opinion by EMA under the [EU-M4all](#) procedure for high-priority medicines for human use intended for markets outside the European Union. If received, a positive opinion by EMA will facilitate regulatory decisions in endemic countries. Merck KGaA, Darmstadt, Germany is designated as the future Marketing Authorization Holder for African countries.

“Having reached this milestone after 10 years of intense and collaborative work makes me very proud. We are now close to our common goal of improving children’s health with a potential innovative and suitable treatment option for the very young patients suffering from schistosomiasis,” said Jutta Reinhard-Rupp, Chair of the Pediatric Praziquantel Consortium Board and Head of the Global Health Institute at Merck KGaA, Darmstadt, Germany.

With the regulatory filing stage complete, the 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 access program, [ADOPT](#), the Consortium is also paving the way for the large-scale delivery of the potential new treatment option in endemic countries. The aim is to start the launch phase in 2024 for product availability on a not-for-profit basis in initial sub-Saharan African countries. At the moment, arpraziquantel is not approved for use in any country.

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For the media

For more information and interview requests, please contact Daniela Bonora, Project Communications Manager at Lygature, via daniela.bonora@lygature.org, info@pediatricpraziquantelconsortium.org or +31 6 48 40 13 04

Notes to Editors

1. 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 approximately 240 million people¹, mainly in communities without access to safe drinking water and with poor sanitation, with an estimated number of deaths of about 200,000² 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. It can also lead to anemia, stunted growth, and impaired learning ability with devastating consequences for the lives of young children.

2. About arpraziquantel and the clinical trials

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. 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 by addressing the medical needs of infected preschool-aged children. In doing so, the Consortium is contributing to ongoing efforts to eliminate this neglected tropical disease and improve child health. 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, visit the Consortium website: www.pediatricpraziquantelconsortium.org

4. Consortium Partners

- **Merck KGaA, Darmstadt** (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)
- **African Institute for Health and Development** (Kenya)

¹ <https://www.who.int/news-room/fact-sheets/detail/schistosomiasis>

² <https://www.who.int/en/news-room/fact-sheets/detail/schistosomiasis>

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)

Find a detailed overview of all partners and collaborators on the [Consortium website](#).

5. Acknowledgement of support

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