PRESS RELEASE

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

- Arpraziquantel, developed by the Pediatric Praziquantel Consortium, receives positive scientific opinion by the European Medicines Agency for the treatment of schistosomiasis in preschool-aged children
- EMA assessed arpraziquantel under the EU-M4all procedure for high-priority medicines intended for use in countries outside the European Union
- The Consortium's work to develop, register, and provide access to arpraziquantel is a tangible contribution to the elimination of schistosomiasis as a public health problem

15 December 2023, Utrecht, The Netherlands. Today, the Pediatric Praziquantel Consortium announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive scientific opinion for arpraziquantel to treat the neglected tropical disease, schistosomiasis, in preschool-aged children (3 months to 6 years of age). The application was submitted by Merck KGaA, Darmstadt, Germany, on behalf of the Consortium, under the EU-M4all procedure for high-priority medicines for human use intended for countries outside the European Union.

“After more than 10 years of intense collaboration, we are thrilled to have received a positive scientific opinion from EMA,” said Dr. Jutta Reinhard-Rupp, Chair of the Pediatric Praziquantel Consortium Board and Head of the Global Health Institute at Merck KGaA, Darmstadt, Germany. “I am extremely proud of our Consortium of dedicated partners. Together, we have come a long way in our vision of providing a treatment option for the most vulnerable population – the youngest. This will contribute to reducing the global disease burden of schistosomiasis, a neglected tropical disease that affects approximately 240 million people worldwide. Now, we all need to turn our full attention to access and delivery.”
Arpraziquantel is derived from praziquantel, the standard of care treatment for schistosomiasis developed in the 1970s. Extending the range of options for the treatment of schistosomiasis, arpraziquantel is tailored for use in preschool-aged children. It is a 150 mg tablet that withstands the hot and humid challenges presented by a tropical climate. The tablet is to be administered dissolved in water and along with an improved taste that makes it palatable for very young children.

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 planned 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 the planned future large-scale local production to serve African countries.

In parallel with this regulatory work, the Consortium’s implementation research program (ADOPT) is currently ongoing, preparing for the introduction of arpraziquantel in the first endemic countries in Africa. To support equitable and sustainable access, it is essential that new procurement and funding mechanisms are collaboratively explored and established. The intent is to make the product available on an at-cost basis in sub-Saharan African countries.

Diseases of poverty, like schistosomiasis, must be overcome in order to deliver on the United Nations’ Sustainable Development Goals (SDGs) and ensure universal health coverage. By developing, registering and providing access to arpraziquantel, the Consortium is making a tangible contribution to the elimination of schistosomiasis as a public health problem and thereby also addressing the SDGs, in particular SDGs 3 (Good Health and Wellbeing) & 17 (Partnerships for the Goals).
Notes to Editors

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 20 neglected tropical diseases (NTDs).

2. **About Arpraziquantel**

The current standard of care treatment for schistosomiasis is praziquantel. Praziquantel is **safe, effective, 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 KGaA, Darmstadt, Germany, arpraziquantel contains the pharmacologically active enantiomer of praziquantel. It is a 150mg dispersible tablet. The prototype of its pediatric formulation was developed by Astellas in Japan and further optimized by Merck KGaA, Darmstadt, Germany in Germany. The manufacturing process served to produce clinical trial supplies from Merck KGaA, Darmstadt, Germany 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](http://www.pediatricpraziquantelconsortium.org).

3. **EU-M4all**

Through the [EU-M4all procedure](http://www.eu-m4all.com), 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. More information can be found [here](http://www.eu-m4all.com).
4. 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 infected 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.

The Consortium is financially supported by Merck KGaA, Darmstadt, Germany; in-kind contributions from the Consortium’s partners; and grants from the Bill and Melinda Gates Foundation (2012), the Global Health Innovative Technology Fund (GHIT) (2014, 2015, 2016, 2019 & 2020), and the European & Developing Countries Clinical Trials Partnership (EDCTP), under its second program supported by the European Union (2018 & 2021).

For more information, and to see an overview of all Consortium partners, visit the Consortium website: www.pediatricpraziquantelconsortium.org

5. Consortium Partners

- Merck KGaA, Darmstadt (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)