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

- **Arpraziquantel is a potential new treatment option for the estimated 50 million preschool-aged children with schistosomiasis, one of the most devastating parasitic diseases in the world**
- **A positive scientific opinion by EMA would facilitate registration of arpraziquantel in African endemic countries**
- **Merck KGaA, Darmstadt, Germany, is committed to the fight against schistosomiasis**

Darmstadt, Germany, December 2, 2022 – Merck KGaA, Darmstadt, Germany, a leading science and technology company, and the [Pediatric Praziquantel Consortium](#), today announced that the 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 milestone, we are one step closer to achieving our mission of improving the health of preschool-aged children with schistosomiasis. Our contribution forms part of our larger ambition to eliminate schistosomiasis as a public health burden by 2030,” said Peter Guenter, Member of the Executive Board and CEO of Healthcare at Merck KGaA, Darmstadt, Germany.

The application was submitted by Merck KGaA, Darmstadt, Germany, on behalf of the Consortium 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. A positive scientific opinion by EMA, if received, will facilitate regulatory



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decisions in endemic countries. Merck KGaA, Darmstadt, Germany, is designated as the future Marketing Authorization Holder for African countries.

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

The clinical development program was completed at the end of 2021. In the 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.

For next steps, the Consortium is preparing for the potential inclusion of arpraziquantel in the World Health Organization list of prequalified and essential medicinal products. Together with relevant stakeholders, it is also exploring new mechanisms for providing equitable and sustainable access to arpraziquantel, once registered, and is conducting [ADOPT](#) – an implementation research program that paves the way for the large-scale delivery in endemic countries. The aim is to start the launch phases in 2024 for product availability on a not-for-profit basis in initial sub-Saharan African countries.

As part of the Global Health strategic priorities of Merck KGaA, Darmstadt, Germany, the company's commitment to the schistosomiasis elimination and the related collaborative efforts, including the development of arpraziquantel, have been recognized in the 2022 Access to Medicine (ATM) Index where Merck KGaA, Darmstadt, Germany, [ranks fifth](#) among the 20 largest pharmaceutical companies based on initiatives to advance global access to medicines in low- and middle-income countries.

Merck KGaA, Darmstadt, Germany aims to control and eliminate schistosomiasis through scientific and technological innovation. The strategy is not only to develop

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and provide medicines but also to improve diagnosis, counter disease transmission, increase disease control, expand access to healthcare, and strengthen local health systems. With this approach Merck KGaA, Darmstadt, Germany, is also helping to improve the health of underserved populations in low- and middle-income countries. By this the company contributes to United Nation's [Sustainable Development Goal 3](#) of good health and well-being.

About Arpraziquantel

The current standard of care treatment for schistosomiasis is praziquantel. Praziquantel is safe, effective, and suitable for school-aged children and adults. However, an appropriate drug for pre-school aged children has to date been elusive. Arpraziquantel aims to close this treatment gap for schistosomiasis (*S. mansoni*, *S. haematobium*). Arpraziquantel contains the pharmacologically active enantiomer of praziquantel. The new tablet is smaller, (oro)-dispersible – it dissolves in the mouth or in water, has taste properties that are acceptable for children, and withstands the challenges presented by a tropical climate. It was developed by Astellas Pharma Inc. in Japan, subsequently optimized by Merck KGaA, Darmstadt, Germany, in Germany and transferred for clinical & manufacturing supply to Farmanguinhos in Brazil. In addition, the more recent partnership with Universal Corporation Ltd. in Kenya prepares for extensive local production capacities in and for Africa. Arpraziquantel is not approved for any use in any country.

About Schistosomiasis

Schistosomiasis is a chronic condition and one of the most common and most devastating parasitic diseases in tropical countries. It is estimated that approximately 240 million people are infected worldwide and that around 200,000 die from it each year. Flatworms transmit the disease. It is widespread in tropical and subtropical regions where large sections of the population have no access to clean water and sanitary installations. People become infected with the parasite via 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.

About the Pediatric Praziquantel Consortium

The Pediatric Praziquantel Consortium is an international not-for-profit partnership that aims to help improving child's health by addressing the medical needs of preschool-aged children with schistosomiasis for potentially reducing the global burden caused by this disease. Its mission is to develop, register and provide access to a suitable pediatric drug for treating schistosomiasis in children three-months to six years of age. For more information, and to see an overview of all Consortium partners, visit the Consortium website: www.pediatricpraziquantelconsortium.org

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) (2013, 2014, 2016, 2019 & 2021), and the European & Developing Countries Clinical Trials Partnership (EDCTP), under its second program supported by the European Union (2018 & 2021).

About the Schistosomiasis Elimination Program of Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany, initiated its Schistosomiasis Elimination Program in cooperation with the World Health Organization (WHO) in 2007. Since then, more than 1.5 billion tablets have been donated, enabling the treatment of over 600 million school-aged children. Merck KGaA, Darmstadt, Germany, has committed itself to maintaining its efforts in the fight against schistosomiasis until the disease is eliminated as a public health burden. To this end, each year Merck KGaA, Darmstadt, Germany, provides up to 250 million tablets for the treatment of schistosomiasis through its partnership with WHO. The planned annual donation has a value of USD 32.5 million. In addition, Merck KGaA, Darmstadt, Germany, has adopted an integrated schistosomiasis strategy that is implemented in close collaboration with partners worldwide and focuses on: treatment, research & development, WASH (Water, Sanitation & Hygiene) & health education, advocacy & partnerships. More information about the Schistosomiasis Elimination Program is available at www.makingschistory.com.

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About Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany, a leading science and technology company, operates across life science, healthcare and electronics. More than 60,000 employees work to make a positive difference to millions of people's lives every day by creating more joyful and sustainable ways to live. From providing products and services that accelerate drug development and manufacturing as well as discovering unique ways to treat the most challenging diseases to enabling the intelligence of devices – the company is everywhere. In 2021, Merck KGaA, Darmstadt, Germany, generated sales of € 19.7 billion in 66 countries.

The company holds the global rights to the name and trademark "Merck" internationally. The only exceptions are the United States and Canada, where the business sectors of Merck KGaA, Darmstadt, Germany, operate as MilliporeSigma in life science, EMD Serono in healthcare and EMD Electronics in electronics. Since its founding in 1668, scientific exploration and responsible entrepreneurship have been key to the company's technological and scientific advances. To this day, the founding family remains the majority owner of the publicly listed company.