

## News Release

Your Contact

timo.breiner@emdgroup.com

Phone: +49 6151 72-55707

November 16, 2021

### **Merck KGaA, Darmstadt, Germany, Announces Positive Phase III Results for Arpraziquantel as Part of its Schistosomiasis Elimination Program**

- **Primary efficacy endpoint of clinical cure was met with a favorable safety profile**
- **Arpraziquantel is a potential new treatment option for the estimated 50 million preschool-aged children with schistosomiasis**
- **Merck KGaA, Darmstadt, Germany, intends to submit regulatory file to the European Medicines Agency**

Darmstadt, Germany, November 16, 2021 – Merck KGaA, Darmstadt, Germany, a leading science and technology company, and the Pediatric Praziquantel Consortium today announced that the pivotal Phase III trial for arpraziquantel, a potential new treatment option for the neglected tropical disease schistosomiasis, has met the primary endpoint of clinical cure in children 3 months to 6 years of age. Arpraziquantel is tailored to the needs of the estimated 50 million preschool-aged children suffering from schistosomiasis, who currently have no treatment option, and forms part of an ongoing effort to eliminate this devastating parasitic disease.

“With this milestone, we continue our commitment to eliminating schistosomiasis and ensuring all people affected by this neglected tropical disease have access to a life-saving therapy. Together with our Consortium partners, we are steadfast in our vision to bring new hope to the world’s most vulnerable populations,” said Peter Guenter, Member of the Executive Board of Merck KGaA, Darmstadt, Germany, and CEO Healthcare.



## News Release

Through its Schistosomiasis Elimination Program, Merck KGaA, Darmstadt, Germany, addresses the key requirements of the WHO's 2021-2030 Roadmap for Neglected Tropical Diseases and contributes to the United Nations' Sustainable Development Goals, especially Good Health and Well-Being (Goal 3) and Partnerships (Goal 17), in line with the company's [sustainability strategy](#).

### **Clinical Phase III shows high efficacy across all age groups**

In the completed Phase III trial, children aged 3 months to 6 years infected with *S. mansoni* or *S. haematobium* were enrolled in different age groups and treated with a single dose of arpraziquantel. High efficacy was observed with cure rates close to or above 90% for *S. mansoni* (at a dose of 50 mg/kg) and *S. haematobium* (at a dose of 60 mg/kg). The primary endpoint of clinical cure, defined as no parasite eggs in the stool (*S. mansoni*) 17 to 21 days after treatment or urine (*S. haematobium*) 17 to 21 days and additionally 35 to 40 days after treatment, met the pre-specified success criteria. Arpraziquantel treatment at both doses demonstrated favorable safety, tolerability and improved palatability among preschool-aged children. No new potential risks or safety concerns were identified.

Derived from praziquantel, arpraziquantel is an orally dispersible tablet developed by the Pediatric Praziquantel Consortium, a public-private partnership led by Merck KGaA, Darmstadt, Germany, through its [Global Health Institute](#), with the aim to close the current treatment gap for an estimated 50 million preschool-aged children with schistosomiasis.

Conducted by the Université Félix Houphouët-Boigny in Côte d'Ivoire and the Kenya Medical Research Institute in Kenya, the Phase III trial was supported by the Global Health Innovative Technology (GHIT) Fund and the European & Developing Countries Clinical Trials Partnership (EDCTP).

Michael Makanga, Executive Director, EDCTP said: "With the completion of the Phase III trial, the Pediatric Praziquantel Consortium demonstrates that balanced North-South collaboration with complementary expertise, bidirectional knowledge sharing, and mutual trust, is a key success factor to develop and deliver safe and affordable treatments for neglected tropical diseases, such as schistosomiasis."

## News Release

Kio Yamabe, Acting CEO of the GHIT Fund said: "Having partnered with the Pediatric Praziquantel Consortium since 2013, we believe that international collaborations like this are key to addressing the burden of major infectious diseases in the developing world. The successful joint development of arpraziquantel by Consortium partners, Astellas, Merck KGaA, Darmstadt, Germany, and Farmanguinhos embodies our unwavering commitment to drive Japanese innovation and technology through global partnerships."

Merck KGaA, Darmstadt, Germany, on behalf of the Consortium, intends to apply 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 opinion by EMA would facilitate the inclusion of arpraziquantel in the World Health Organization (WHO) list of prequalified medicinal products as well as regulatory approvals in endemic countries. With continued support from the GHIT Fund and EDCTP, the delivery of arpraziquantel is being prepared through the Consortium's access program, ADOPT. In addition to Farmanguinhos in Brazil, Merck KGaA, Darmstadt, Germany, has also [partnered with Universal](#) in Kenya for extensive local production capacities for endemic African countries.

### **About Arpraziquantel**

The current standard of care treatment for schistosomiasis is praziquantel. The drug is safe, effective, and suitable for school-aged children and adults. However, an appropriate drug for preschool-aged children has to date been elusive. Arpraziquantel aims to close this treatment gap. It contains the pharmacologically active enantiomer of praziquantel. The new tablet is small, orally 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 in Japan, subsequently optimized by Merck KGaA, Darmstadt, Germany, and transferred for clinical manufacturing to Farmanguinhos in Brazil.

### **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 more than 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 Merck KGaA, Darmstadt, Germany, Schistosomiasis Elimination Program**

Merck KGaA, Darmstadt, Germany, initiated its Schistosomiasis Elimination Program in cooperation with WHO back in 2007. Since then, more than 1.3 billion tablets have been donated, enabling the treatment of 520 million school-aged children. Merck KGaA, Darmstadt, Germany, has committed itself to maintaining its efforts in the fight against the tropical disease until schistosomiasis is eliminated as a public health burden. To this end, each year the company is donating up to 250 million tablets to 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

## News Release

& Hygiene), health education, advocacy & partnerships. More information about the Schistosomiasis Elimination Program is available at [www.makingschistory.com](http://www.makingschistory.com).

All Merck KGaA, Darmstadt, Germany, press releases are distributed by e-mail at the same time they become available on the EMD Group Website. In case you are a resident of the USA or Canada please go to [www.emdgroup.com/subscribe](http://www.emdgroup.com/subscribe) to register for your online subscription of this service as our geo-targeting requires new links in the email. You may later change your selection or discontinue this service.

### **About Merck KGaA, Darmstadt, Germany**

Merck KGaA, Darmstadt, Germany, a leading science and technology company, operates across healthcare, life science and electronics. Around 58,000 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 advancing gene editing technologies and discovering unique ways to treat the most challenging diseases to enabling the intelligence of devices – the company is everywhere. In 2020, Merck KGaA, Darmstadt, Germany, generated sales of € 17.5 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 EMD Serono in healthcare, MilliporeSigma in life science, and EMD 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.

### **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 pediatric praziquantel formulation for treating schistosomiasis in this age group.

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).