PRESS RELEASE

The Pediatric Praziquantel Consortium Announces Positive Phase III Results for Arpraziquantel To Treat Schistosomiasis

- The Pediatric Praziquantel Consortium completes its pivotal clinical Phase III trial of arpraziquantel – a potential new treatment option for the estimated 50 million preschool-aged children with schistosomiasis
- Positive results of the Phase III trial confirm earlier promising Phase II trial results
- Founding Consortium partner Merck KGaA, Darmstadt, Germany intends to submit regulatory file to the European Medicines Agency

16 November 2021, Utrecht, The Netherlands. Today, the Pediatric Praziquantel Consortium, a public-private partnership dedicated to the development of arpraziquantel, a potential new treatment option for schistosomiasis in preschool-aged children, announced the completion of its pivotal Phase III trial in Côte d’Ivoire and Kenya.

The results of the trial, co-funded by the Global Health Innovative Technology (GHIT) Fund and the European & Developing Countries Clinical Trials Partnership (EDCTP), confirm a favorable efficacy and safety profile for arpraziquantel in children 3 months to 6 years of age, affected by this neglected tropical disease. This allows the program to progress towards regulatory file submission to the European Medicines Agency (EMA).

Schistosomiasis is one of the most damaging parasitic diseases, affecting the lives of around 240 million people, and is highly prevalent 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 is an orally dispersible tablet (dissolves in the mouth). It was developed by Astellas Pharma Inc. in Japan, subsequently optimized by Merck KGaA, Darmstadt, Germany and transferred for clinical manufacturing to Farmanguinhos in Brazil. The new tablet is small, has appropriate taste properties, can be taken with or without water, and withstands the hot and humid challenges presented by a tropical climate.

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.”
The successful completion of the Phase III trial has been a consolidated effort of strong and experienced in-country partners – the Kenya Medical Research Institute and Université Félix Houphouet-Boigny – with the Swiss Tropical and Public Health Institute overseeing the trial management. Merck KGaA, Darmstadt, Germany acted as trial sponsor, ensuring that the necessary quality standards and regulatory requirements from authorities such as EMA were addressed. Expert input, including from the World Health Organization, has supported the development of the program.

Dr 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.”

The rationale for the study was based on data gathered from the clinical Phase I study in adult volunteers, a taste study in children 6-11 years of age, and a Phase II dose-finding study in Schistosoma mansoni-infected children 3 months to 6 years of age, conducted in African countries.

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.

With the full clinical development phase successfully completed, the program has entered the regulatory filing stage, while preparing for the potential delivery of arpraziquantel through the Consortium’s dedicated access program, ADOPT.

On behalf of the Consortium, Merck KGaA, Darmstadt, Germany 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.

Peter Guenter, Member of the Executive Board of Merck KGaA, Darmstadt, Germany and CEO of Healthcare, said: “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.”

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For the media (not for publication)
For more information and interview requests, please send an email to info@pediatricpraziquantelconsortium.org