In Search of Better Health

PRESS RELEASE

TO: News Editors & Reporters
SUBJECT: European Medicines Agency (EMA) Recommends Arpraziquantel for Treatment of Schistosomiasis in Preschool-Aged Children
DATE: Friday, 12th, January 2024

NAIROBI-KENYA: Kenya Medical Research Institute (KEMRI) is a partner member of a consortium involved in the just concluded human research for the treatment of schistosomiasis in preschool-aged children that has shown promising results.

The Pediatric Praziquantel Consortium announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive scientific opinion for arpraziquantel to treat the neglected tropical disease, schistosomiasis.

This positive opinion is not only good news for the pull of scientists involved in the study, but also for targeted preschool-aged children with schistosomiasis globally.

Schistosomiasis (commonly known as bilharzia or snail fever) is a neglected tropical disease that affects over 240 million people worldwide. It is one of the most important tropical diseases in terms of public health burden and economic impact.

The existing 'standard of care' treatment for schistosomiasis is praziquantel, which in Kenya, it is made available through mass drug administration programs to adults and school-aged children. However, the drug is not suitable for children six years of age and younger. As such, around 50 million preschool-aged children currently lack adequate treatment.

The development and introduction of this new pediatric medicine, *Arpraziquantel* is indeed an unprecedented opportunity to close the treatment gap and break the transmission of schistosomiasis in Kenya.

The application was submitted by Merck, 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. 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," said Dr. Jutta Reinhard-Rupp, Chair of the Pediatric Praziquantel Consortium Board and Head of the Global Health Institute at Merck.

KEMRI participated in the Phase III clinical trial of Pediatric Praziquantel (PZQ) conducted in Homabay, Kenya at the Homabay Teaching and Referral Hospital (HTRH). The open-label study evaluated the efficacy and safety of Levo-Praziquantel (l-PZQ) 150mg in *Schistosoma*-infected children 3 months to 6 years of age, and included a 2:1 randomized, controlled cohort of *Schistosoma mansoni*-infected children 4 to 6 years of age treated with L-PZQ Orodispersible tablets (ODT) or commercially available 600mg praziquantel.

The new drug is a small, orally dissolvable tablet that can withstand the heat and humidity of tropical climates, and has an acceptable taste that will make it tolerable to young children and enhance its pharmacological effectiveness in treating schistosomiasis.

Arpraziquantel is derived from praziquantel, the standard of care treatment for schistosomiasis developed in the 1970s. The tablet is to be administered dissolved in water and along with an improved taste that makes it palatable for very young children.

Schistosomiasis transmission in Kenya is still a major public health concern with the *Schistosoma* parasite endemic in 62 of the 290 sub-counties in Kenya.

There are three major endemic areas – the Coastal region (mainly *Schistosoma haematobium* parasite that causes the urogenital form of the disease), parts of Central and Lower Eastern areas (both *S. haematobium* & *S. mansoni*) and the Lake Victoria basin (mainly *S. mansoni* parasite that causes the intestinal form of the disease) with few pockets of *S. haematobium* further inland. Transmission is still commonplace in many areas, with people infected during a wide range of activities such as domestic, agricultural and commercial. Exposure to the worm can happen while car washing, sand harvesting, and fishing, and during recreational activities such as swimming.

"KEMRI is very committed together with other partners to confirming the potential of the new pediatric PZQ formulation. Innovation is a key driver in finding today's health solutions, as an Institute we are proud to be part of improved treatments for the close to 250,000 young children in the country that are infected by Schistosomiasis, one of the Neglected Tropical Diseases (NTDs)," said Prof. Elijah Songok, KEMRI Ag. Director General & CEO,

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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. 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).

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Notes to Editors

About KEMRI

The Kenya Medical Research Institute (KEMRI) is a State Corporation established in Kenya in 1979 through the Science and Technology (Repealed) Act, Cap 250 of the Laws of Kenya operated under the Science Technology and Innovation Act, 2013 as the national body responsible for carrying out research in human health in Kenya. Currently, KEMRI operates under Legal Notice No. 35 of March 2021KEMRI has grown from its humble beginning over 40 years ago to become a regional leader in human health research. The Institute currently ranks as one of the leading Centers of excellence in health research both in Africa as well as globally.

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. Schistosomiasis is a chronic condition and is classified by the World Health Organization (WHO) as one of 20 neglected tropical diseases (NTDs). 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 infested freshwater, during routine agricultural, domestic, occupational and recreational activities for example, while working, swimming, fishing, or washing their clothes. The infection rate is particularly high among children. Transmission occurs when people suffering from schistosomiasis contaminate freshwater sources with faeces or urine containing parasite eggs, which hatch in water. The minuscule larvae released by the intermediate snails penetrate human skin, develop into adult schistosomes and enter the blood vessels. Adult worms live in the blood vessels where the females release eggs. Some of the eggs are passed out of the body in the faeces or urine to continue the parasite's

lifecycle. Others become trapped in body tissues, causing immune reactions and progressive damage to internal organs.

Diagnosis of schistosomiasis is through the detection of parasite eggs in stool or urine specimens. Antibodies and/or antigens detected in blood or urine samples are also indications of infection. Control of schistosomiasis focuses on reducing disease through periodic, large-scale population treatment with praziquantel; a more comprehensive approach includes provision of potable water, adequate sanitation, and snail control to reduce transmission.

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, 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 in Germany. The manufacturing process served to produce clinical trial supplies from Merck 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's website (https://www.pediatricpraziquantelconsortium.org/).