

March 20, 2014

### **Pediatric Praziquantel Consortium awarded US\$1.86 million GHIT grant**

#### **Research award recognizes importance of development and registration of a new pediatric formulation of praziquantel, the current gold standard treatment for schistosomiasis**

Leiden, The Netherlands, March 20, 2014 - The Pediatric Praziquantel Consortium, launched in 2012 as an international non-profit public-private partnership, has announced that it has been awarded a prestigious research grant from the Japanese Global Health Innovative Technology (GHIT) Fund. The grant supports the Consortium's clinical development program of a newly developed praziquantel pediatric formulation targeted for use in very young children, including infants and toddlers, infected with schistosomiasis.

"GHIT grants are awarded on the basis of a highly competitive, double peer review process, which underlines the importance of a newly developed praziquantel pediatric medication for millions of infected children in the developing world" said Annalisa Jenkins, Chair of the Consortium Board and Head of Global R&D at Merck Serono. "We very much look forward to working with the GHIT Fund and are grateful for its support in addressing the global health burden that schistosomiasis represents.", she added. BT Slingsby, CEO of the GHIT Fund, said: "We welcome collaboration with the Pediatric Praziquantel Consortium. This project will enable Japanese formulation technology and innovation to play a more direct role in the fight against the second-most severe tropical disease in Africa."

The standard recommended praziquantel treatment is available in tablets, suitable for adults and children from the age of six. Proper treatment of younger children with schistosomiasis is hampered due to missing clinical data and the fact that they cannot swallow these tablets because of their size and bitter taste. Astellas Pharma has played a pivotal role in the development of new oral dispersible praziquantel candidates specifically for very young children. As Kazuhiro Sako, fellow Consortium Board member and Vice President Pharmaceutical Research and Technology Labs at Astellas Pharma, explained: "The newly developed tablet has been reduced to a quarter of the size of the current commercial praziquantel tablet to swallow easily. We designed it to be oral dispersible so that it can be taken with or without water, allowing treatment, in principle, of very young children, including infants from 3 months onwards." He added: "A major challenge, which required a great deal of effort, was to reduce the bitter taste but at the same time keep the formulation straightforward and robust, to allow future local manufacturing and storage in endemic countries."

Whether taste masking has been successful remains to be determined. A taste study in African children is planned during the first half of 2015, in which the newly developed praziquantel candidates are compared to the commercial praziquantel. This study is preceded by two phase I clinical trials in healthy adult volunteers in the second half of 2014. In addition to funding three clinical studies, the research grant is intended to cover the costs of a high-level expert meeting in which the future regulatory and access strategy will be discussed and the costs of communication activities. The funding by GHIT will help the Consortium to take one of the praziquantel candidates to the phase II clinical trial stage.

### **Pediatric Praziquantel Consortium**

The Pediatric Praziquantel Consortium was founded in July 2012. Its ultimate objective is to develop and register a new praziquantel pediatric formulation for the treatment of schistosomiasis in preschool-age children, including infants and toddlers. In this consortium of highly driven partners, Merck, responsible for leading the program, also brings the necessary chemistry and manufacturing expertise, resources, and support related to praziquantel. In addition, it provides the preclinical, clinical and regulatory resources necessary to efficiently and successfully execute the project. Astellas Pharma Inc. contributes by providing its innovative pharmaceutical technologies in the area of drug formulation development and clinical development in children. Swiss TPH brings extensive experience in helminths biological and pharmacological research, epidemiology and clinical research on drug effectiveness and efficiency in endemic regions. The governance is facilitated by TI Pharma, a non-profit organization and independent party with an extensive portfolio of international public-private partnerships in drug research and development, including in the area of neglected diseases.

#####

### **About Astellas Pharma Inc.**

Astellas Pharma Inc., based in Tokyo, Japan, is a pharmaceutical company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceutical products. Astellas Pharma Inc. has approximately 17,000 employees worldwide. The organization is committed to becoming a global category leader in Urology, Immunology (including Transplantation) and Infectious Diseases, Oncology, Neuroscience, and DM Complications and Kidney Diseases. Astellas Pharma Inc. is committed to increasing access to health in developing countries through its partnership initiatives. Astellas Pharma Inc.'s pharmaceutical research and technology labs have contributed to improving convenience for use and the functionality of treatments by developing novel formulations and bringing additional value based on its excellent pharmaceutical technologies (e.g. solubilization, controlled release, oral disintegration tablet and drug delivery technologies).

For more information, please visit [www.astellas.com/en](http://www.astellas.com/en)

### **About Merck**

Merck is a leading pharmaceutical, chemical and life science company with total revenues of €11.2 billion in 2012, a history that began in 1668, and a future shaped by approx. 38,000 employees in 66 countries. Its success is characterized by innovations from entrepreneurial employees. Merck's operating activities come under the umbrella of Merck KGaA, in which the Merck family holds an approximately 70% interest and free shareholders own the remaining approximately 30%. In 1917 the U.S. subsidiary Merck & Co. was expropriated and has been an independent company ever since.

The Merck Praziquantel Donation Program was launched in 2007 in partnership with WHO. Since then more than 100 million tablets have been supplied and over 38 million children have been treated. By 2011, Merck's annual donation had grown to about 25 million tablets. At the beginning of 2012 Merck announced that it will fight schistosomiasis until the disease will be eliminated in Africa. To reach this goal the company will increase the annual donation of tablets tenfold in the medium term.

For more information, please visit [www.merckgroup.com](http://www.merckgroup.com)

### **About Swiss TPH**

Swiss TPH is an associated institute of the University of Basel. Its mandate is to contribute to the improvement of public health at international, national and local levels through excellence in research, services, teaching and training. The strategy to pursue this mandate is based on a broad interdisciplinary approach for a sustainable iterative process between the laboratory, field, classroom and bedside. Swiss TPH addresses the continuum between innovation (developing concepts, methods and products), validation (providing evidence for what works) and application (strengthening public health systems & policies). Over 700 staff members from 62 nations are organized in two research and three service departments.

For more information, please visit [www.swisstph.ch](http://www.swisstph.ch)

### **About Top Institute Pharma**

Top Institute Pharma (TI Pharma), a non-profit organization, enables groundbreaking pharmaceutical research for the development of new medicines by establishing and managing international public-private partnerships. Within an open innovation model, scientists, the business world, patient organizations and others collaborate in frontrunner, multidisciplinary research aimed at improving the development of socially valuable medicines. For more information, please visit [www.tipharma.com](http://www.tipharma.com)

Contacts for inquiries or additional information
Astellas Pharma Inc. Corporate Communications Tel: +81-3-3244-3201 Fax: +81-3-5201-7473 <a href="http://www.astellas.com">http://www.astellas.com</a>