Project Details

Development and registration of a new praziquantel pediatric formulation for the treatment of schistosomiasis

Awarded Year	2014
Awarded Amount	$4,856,513
Disease	NTDs Schistosomiasis
Intervention	Drug
Development Stage	Drug Clinical Phase2
Collaboration Partners	Astellas Pharma Inc., Farmanguinhos, Merck KGaA, Simcyp Limited, Swiss Tropical and Public Health Institute, Lygature

Introduction and Background of the Project

Schistosomiasis, also known as bilharzia and endemic in 78 developing countries, is a chronic inflammatory neglected tropical disease caused by parasitic worms. The disease affects more than 249 million people, including 100 million children, globally. It is one of the most prevalent tropical diseases in the world after malaria, and represents an important health burden in developing countries, especially in Africa where more than 90% of the infections occur.

The current gold standard recommended treatment for schistosomiasis, praziquantel (PZQ), is available in oral tablets for adults and children, but younger children under 4 years cannot be treated due to missing clinical data. In addition, a pediatric formulation of praziquantel that would be appropriate for preschool age children, infants and toddlers, and would permit accurate dosing and enhanced compliance in these patients is non-existent and highly needed. The development of a new pediatric formulation of praziquantel is to be seen in the context of the WHO's 2020 Roadmap and with respect to the initiative of "Uniting to combat Neglected Tropical Diseases" under which the global community agreed on control and elimination of schistosomiasis.

How can your patnership (project) address global health challenges?

In order to tackle the important public health problem of schistosomiasis, the non-profit Pediatric Praziquantel Consortium has been formed in July 2012 to develop a pediatric formulation of praziquantel. The project will contribute to the overall goal of control and elimination of schistosomiasis by developing a product urgently required for the treatment of preschool-age children, infants and toddlers, a population that is currently being considered by WHO to be included in treatment campaigns. The development and registration of a new pediatric formulation of praziquantel is regarded by the Consortium as a cornerstone to fulfill the 2020 WHO commitment to address the health burden that schistosomiasis represents and the importance of controlling disease-related morbidities. To achieve this commitment, several measures need to be integrated among which making chemotherapy treatment available to **all age groups** is an important part of.

What sort of innovation are you bringing in your project?

The Pediatric Praziquantel Consortium combines the best science and most experienced public and private partners to develop and register a new pediatric

formulation of praziquantel, the current gold standard treatment for schistosomiasis. It has developed new oral dispersible formulations of praziquantel that can be easily administered to preschool-aged children, infants and toddlers, exhibit improved taste, and are able to withstand the challenges presented by a tropical climate. The Consortium is currently conducting the clinical phase I and in the second half of 2015 the project will move into the clinical development phase II.

The Consortium is led by Merck KGaA, which brings the necessary chemistry and manufacturing expertise and support related to praziquantel. It also provides the preclinical, clinical and regulatory resources necessary to efficiently and successfully execute the project. Astellas Pharma Inc. has contributed its novel pharmaceutical technology, which helps to improve drug compliance by minimizing the bitter taste of praziquantel. It will continue to provide ad-hoc expert advice during the following steps of formulation development. Astellas will also provide expert advice in the areas of clinical development in children, pharmacokinetic modeling, and access to health. Swiss TPH brings extensive experience in schistosomiasis biological and pharmacological research, clinical research on drug effectiveness and efficiency in endemic regions and epidemiology. Farmanguinhos, the federal governmental pharmaceutical laboratory of the Fiocruz Foundation in Brazil, brings unique expertise to produce and distribute the new pediatric formulation candidates in endemic countries. Simcyp, a small UK research-based company, builds and validates a pharmacokinetic model to allow a better prediction of the appropriate dosage for use in the pediatric clinical trials. The governance is facilitated by TI Pharma, a non-profit organization with an extensive portfolio of public-private partnerships in drug research and development, including in the area of neglected diseases.

Others (including references if necessary)

For more detailed information on the Pediatric Praziquantel Consortium, the project team and the development program, please feel free to visit the Consortium website:
www.pediatricpraziquantelconsortium.org.

To receive regular updates on the progress and activities of the program, feel free to sign-up for the bi-annual Consortium Newsletter:
http://www.pediatricpraziquantelconsortium.org/nieuwsbrief.html.