Uploaded to the VFC Website

2017

This Document has been provided to you courtesy of Veterans-For-Change!

Feel free to pass to any veteran who might be able to use this information!

For thousands more files like this and hundreds of links to useful information, and hundreds of "Frequently Asked Questions, please go to:

Veterans-For-Change

If Veterans don't help Veterans, who will?

Note: VFC is not liable for source information in this document, it is merely provided as a courtesy to our members & subscribers.

GHIT Fund invests in phase 3 clinical trial for pediatric formulation of ‘snail fever’ drug

March 31, 2017

The Global Health Innovative Technology Fund (GHIT Fund), a unique Japanese public-private partnership formed to battle infectious diseases around the globe, today announced 11 new investments totaling US$23 million that could help deliver a range of new innovative therapies for a host of debilitating conditions.

This latest round of targeted support includes funding for a Phase 3 clinical trial testing a pediatric formulation of a drug considered the gold standard for treating schistosomiasis, a water-borne parasitic disease linked to an assortment of acute and chronic health problems. Young children are most at risk, but the existing drug is so bitter and hard to swallow that kids often go untreated, leading to serious lifelong health and learning problems.

This clinical trial is one of the most advanced partnerships invested by the GHIT Fund, an organization that combines Japan’s historic leadership in global health and innovation with groundbreaking research from across the globe. The GHIT Fund also is making new investments in two malaria vaccine candidates, while accelerating work to find new drug treatments for malaria, dengue, Chagas disease, cryptosporidiosis and leishmaniasis.

“We’re reaching an exciting phase where GHIT’s approach to partnerships and drug and vaccine development is starting to produce tangible progress towards product deployment that could eventually lead to revolutionary breakthroughs,” said BT Slingsby, MD, PhD, MPH, who is CEO of the GHIT Fund. “We knew that combining Japan’s wealth of biomedical research talent and pharmaceutical capabilities with leading infectious disease experts near and far was likely to be a winning combination, and that’s been validated by the progress we are seeing across a rich diversity of projects.”

A Snail Fever Drug That’s Easy to Swallow

Schistosomiasis, sometimes called “snail fever” because it’s found in freshwater snails, leads to both acute and chronic disease. It’s caused by parasitic worms known as blood flukes and is usually transmitted through

contact with infested water. The disease is endemic in 78 developing countries and, according to the World Health Organization, more than 261 million people, including 100 million children, were infected with schistosomiasis in 2015. Some 90 percent of infections occur in Africa, where safe water is often scarce. While rarely fatal, left untreated, the disease can cause anemia, stunted growth, impaired learning ability and chronic inflammation of vital organs.

GHIT Fund's investment of US$4.7 million, with co-funding from its partners, will support a Phase 3 clinical trial in Africa to evaluate a pediatric formulation of praziquantel (PZQ) in children aged three months to six years. Since the 1970s, the gold-standard of treatment for the disease has been a single oral dose of PZQ used to treat adults and school-aged children. But children under age five who are infected with schistosomiasis are not treated with PZQ under the current policy. And data on the treatment of these children has been sparse and insufficient to define and confirm the best dosing. In addition, the current tablets have a severe bitter taste and the large size of the existing pill makes it difficult or just impossible for small children to swallow. A smaller, more palatable pill that could be administered to children as young as three months old is being developed by the Pediatric Praziquantel Consortium, a nonprofit international public-private partnership involving Astellas Pharma Inc. (Japan), Lygature (The Netherlands), Merck KGaA (Germany), the Swiss Tropical and Public Health Institute, Simcyp Limited (UK), Farmanguinhos (Brazil) and the Schistosomiasis Control Initiative (SCI, UK).

Previous GHIT Fund investments for this project took the formulation through a Phase 2 clinical trial in 2015 and 2016. If successful, the Phase 3 trial will pave the way for regulatory review and prequalification by the World Health Organization (WHO) that would deliver an affordable, effective, child-friendly formulation of PZQ to young victims around the globe.

Biting Back at Malaria

GHIT also announced today a new investment of US$600,000 to develop a unique vaccine for malaria that prevents the malaria parasite from being transmitted from an infected person to a mosquito. Although it won't protect individuals from malaria, the formulation enables the vaccinated person to become a sort of "human shield" that breaks the vicious cycle of disease, which depends on the malaria parasite being able to move from mosquito to human

and then back to mosquito. Known as a "transmission-blocking vaccine," a successful formulation would be a significant weapon in the global push to eliminate malaria, which killed 438,000 people in 2015, most of them young children in sub-Saharan Africa.

Researchers from the PATH Malaria Vaccine Initiative (US) and Ehime University (Japan) will evaluate a protein (called *Pfs230*) found in the deadly *P. falciparum* malaria parasite that appears to produce antibodies that can block disease transmission from humans to mosquitos. The investment from GHIT allows the team to examine small regions of the extremely complex protein via an innovative research tool known as wheat germ cell-free protein synthesis technology. The goal is to harness the transmission-blocking features of the protein to drive development of new vaccine candidates.

GHIT Fund also is investing US$2.8 million to continue work on a promising malaria vaccine candidate being developed by researchers from Japan's Research Institute for Microbial Diseases and the Medical Center for Translational and Clinical Research at Osaka University, the Germany-based European Vaccine Initiative (EVI), the Centre National de Recherche et de Formation sur le Paludisme (CNRFP, Burkina Faso), and Nobelpharma Co., Ltd. (Japan). Their formulation, called BK-SE36, has produced encouraging results in early testing, generating an immune response in Japanese adults and in Ugandan volunteers aged 6-32 years. Currently, it is being tested in a Phase 1b clinical trial with young children aged 1-5 years in Burkina Faso, where malaria is rampant. This new investment from the GHIT Fund will enable researchers to evaluate a different formulation of the vaccine in healthy African adults and children, one that contains an additional substance known as an adjuvant that has shown promise in boosting the immune response to the vaccine.

GHIT will also continue to support four projects that are pursuing new malaria drugs, which are urgently needed to fight the spread of parasites that have become resistant to existing therapies. Over the last few years there has been an emergence of *P. falciparum* malaria parasites—first in Southeast Asia and just recently in Africa—that can survive an assault from previously powerful antimalarial drugs. Researchers are now racing to develop new treatments that can kill the parasite in a single dose. GHIT's investments in this work include:

- US$750,000 for an HTLP partnership (Hit-To-Lead Platform) between Eisai

Co. Ltd. (Japan) and Medicines for Malaria Venture (MMV, Switzerland) to further explore a series of "hits" that emerged from screening 20,000 compounds in Eisai's library for potential activity against malaria. MMV and Eisai have been evaluating potential activity against different stages of the malaria parasite to identify compounds that could be "lead" candidates for antimalarial treatments. This new investment will allow further investigation of one of these "hits" while supporting "hit-to-lead" work with a new series of compounds.

- US$483,000 for a second HTLP partnership, this one between Takeda Pharmaceutical Company Limited (Japan) and MMV, to examine a new series of compounds in Takeda's chemical library that might have the potential to fight malaria.
- US$2 million to the Broad Institute of MIT and Harvard (US), Eisai and MMV to build on promising work to identify a series of compounds that can quickly defeat drug-resistant strains and prevent their spread by blocking transmission of the parasite. The new funding will allow researchers to narrow their focus and identify two or three compounds that warrant further study.
- US$4.5 million to the University of Kentucky (US), Eisai and MMV for a Phase 2a study of an antimalarial compound called SJ733, which is intended for use in combination with other malaria medicines to provide a fast-acting treatment that also prevents relapse. In addition to supporting a Phase 2a study in adults, this investment will allow researchers to investigate potential drug formulations and conduct "challenge" trials, in which healthy volunteers are given malaria under controlled circumstances and then treated with the drug to see if it is effective. This work will set the stage for further Phase 2 studies in children and pregnant women—populations most at risk for malaria.

Going After the World's Most Overlooked Diseases

In its latest round of investments, GHIT is continuing to confront some of the most neglected diseases in the world—diseases that burden more than 1 billion of the world's poorest people and keep them impoverished by causing a range of chronic mental and physical problems. Fighting these overlooked diseases has been a long-time priority for Japan, and for GHIT, which officially endorsed the London Declaration on Neglected Tropical Diseases in 2014, pledging ongoing funding to defeat them once and for all.

GHIT announced today the following investments in neglected diseases:

- US$5.3 million for Chugai Pharmaceutical Co., Ltd. (Japan) and A*STAR's Singapore Immunology Network (SIgN) to pursue pre-clinical development of a therapy that could treat the symptoms associated with the four types of dengue virus, as well as prevent lethal and severe cases of the disease. The global burden of this mosquito-borne disease is increasing, with about half of the world's population now at risk of contracting dengue. Dengue causes flu-like symptoms, extreme joint pain and can progress to a deadly hemorrhagic fever.
- US$780,000 to support a hit-to-lead collaboration between the Drugs for Neglected Diseases initiative (DNDi) and Daiichi Sankyo in the areas of leishmaniasis and Chagas disease. The project aims to progress promising hit series, all of which were previously identified through a GHIT-supported screening venture, into potential treatment leads for these diseases.
- US$550,000 in continued funding to DNDi, Eisai, Shionogi & Co. Ltd. (Japan) and Takeda for continued work with the Neglected Tropical Diseases Drug Booster program—a groundbreaking initiative committed to accelerating early-stage drug discovery for treatments for leishmaniasis and Chagas disease. Visceral leishmaniasis (VL) causes fever, weight loss, enlargement of the spleen and anemia. If untreated, it is almost always fatal. Chagas disease, which kills more people in Latin America than any other parasitic disease, causes severe heart damage and intestinal problems. This new investment will allow partners to continue the work of the booster program, which seeks to overcome early-stage intellectual property barriers and enable DNDi to search for new leads by evaluating thousands of unique compounds simultaneously.
- US$980,000 for an effort to identify novel drug targets for a group of deadly parasitic diseases: malaria, Chagas disease and leishmaniasis, and also for cryptosporidiosis, which is a major cause for severe diarrhea in infants and toddlers. This work will be led by the RIKEN Center for Sustainable Resource Science (Japan), the Structural Genomics Consortium at University of Toronto (Canada), the University of Melbourne (Australia), McGill University (Canada), MMV and DNDi.

Source:

https://www.ghitfund.org//about/mediacenter/pressdetail/detail/172
