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FROM
INNOVATION

TO
IMPACT

THE GLOBAL HEALTH
INNOVATIVE
TECHNOLOGY FUND



Leveraging Japan's wealth of expertise in technological innovation, drug development and overseas aid, the GHIT Fund strives to tackle the developing world's growing burden of infectious diseases. Here, Drs B T Slingsby and Kiyoshi Kurokawa explain the reasons behind the organisation's inception, the benefits of implementing an 'open innovation' model and the ways in which its funded projects are having a marked impact

Can you outline the Global Health Innovative Technology (GHIT) Fund's core vision and the ways in which the organisation is striving to achieve this mission?

BTS: Our vision is to help remove the crushing burden of infectious disease that prevents billions of people in the developing world from seeking the level of prosperity and longevity now common in the industrialised world. We aim to achieve this by facilitating and investing in international R&D partnerships that bring Japanese innovation, investment and leadership to the global fight against infectious diseases and poverty in the developing world.

Importantly, the mechanisms of action in 75 per cent of our pipeline products are new molecular entities (NMEs) or new chemical entities (NCEs), which are innovative in the purest sense. This is particularly exciting for diseases that have not had new drugs or vaccines in 80 years, as well as for diseases with no effective existing treatment or prevention tool. NMEs and NCEs breathe new life into global health R&D by illustrating new pathways and mechanisms of action where deadly pathogens are vulnerable. Many of these NCEs have come out of our Screening Platform, which gains access to Japan's previously untapped chemical compound libraries. These libraries offer enormous R&D innovation potential, as well as hope for the millions of people worldwide who suffer from and live in fear of devastating infectious diseases.

The GHIT Fund is a young organisation, having only been formed in 2013. Why was it established?

KK: The magnitude of the infectious disease problem in the developing world and the market failure to address society's needs were the main driving forces. Almost half of the world's population is at risk of infectious diseases including malaria, tuberculosis and neglected tropical diseases, and over 1 billion people globally are already infected with one or more of these diseases. There is such a strong demand for new drugs, vaccines and diagnostics, yet the market has failed to address these needs.

With this troublesome reality in mind, the GHIT Fund was conceived in 2012 by leaders within Japan's pharmaceutical industry. Our partners sought to tap into Japan's legacy of global leadership in drug development, technological innovation and overseas development assistance (ODA), and create a public-private partnership fund for global health R&D. At the same time, the Japanese Government had been actively seeking ways to better support R&D and use the country's significant ODA funds to create more value through its deep well of available technology and innovation. These leaders saw that harnessing Japan's considerable resources, know-how and existing innovation to create unprecedented medicines, vaccines and diagnostics for neglected diseases would require global partnerships.

How has your previous career prepared you for your roles at the GHIT Fund?

BTS: As a medical doctor I have seen first-hand the pain and suffering associated with infectious diseases, and this experience will always serve as an important foundation for the work I do with the GHIT Fund.

Additionally, my work as a bioethicist also morally compels me to participate, in whatever way I can, in addressing inequity. But perhaps the most important preparation for my role at the GHIT Fund has been the work I was able to do with Eisai & Co., which gave me a comprehensive understanding of the perspectives and challenges, and the very sincere interest, of the private sector in engaging in global health. As Director for Global Access Strategies, I was able to dive into the development of new business models for R&D and market access as well as help develop a major drug donation programme for the treatment of parasitic diseases. I also served as Executive Director for Eisai-Quintiles World Health Initiative, where I led product development partnerships in the US, Europe, Japan and Brazil. This experience gave me very useful insight into the value of cross-sector partnerships, which form the cornerstone of the GHIT Fund's business model.

KK: My academic experience in the US and Japan, and my time working with policy makers from both the Government of Japan and the World Health Organization (WHO), led me to support the GHIT Fund's establishment, work and unique partnership-based governance structure. I really enjoy, and have seen great benefits emerge from, facilitating cross-sector and cross-border partnerships.

Why is there a current R&D gap in the treatment and prevention of infectious diseases? Who is this predominantly affecting?

BTS: It costs upwards of hundreds of millions of dollars – not including the cost of failures – for a company to develop a new drug or vaccine. If the expected financial return from a paying market is not large enough to cover the investment and return a profit, companies are unlikely to invest in development. Too often, when the number of people affected is too small or when massive numbers of people are affected but are all too poor to pay, drug and vaccine development stops at the level of the scientists.

Over the past decade, though, the situation has begun to change. Several drugs and vaccines are now being developed by companies for malaria, tuberculosis, Chagas disease and other neglected tropical diseases. What shifted? The simple acknowledgement that no one sector or institution can solve the big global health challenges alone. Companies need to share the technical and financial risk with governments and charities.

What is the GHIT Fund's 'no gain, no loss' principle? How does this encourage research advances in health issues that significantly impact developing countries?

KK: As an international global health R&D fund, we understand that innovation without access is meaningless. In recognition, many health R&D philanthropies hold policies that ensure access at reasonable prices to patients in the developing world while still allowing profit in developed markets. For example, the GHIT Fund ensures affordable pricing in low- and middle-income countries through a 'no gain, no loss' policy, which requires products resulting from our investments to be affordably priced in the developing world while allowing for prices that enable a profit in developed markets.



POWERFUL PARTNERSHIPS

The product development partnerships (PDPs) in which GHIT invests are built on the foundation of the open innovation model – an approach that is making real impact. To illustrate how these PDPs are contributing to global improvements in health innovations for infectious diseases, examples of outstanding progress are highlighted below.

SCHISTOSOMIASIS

The Pediatric Praziquantel Consortium – comprising Merck KGaA, Astellas Pharma Inc., Swiss Tropical and Public Health Institute, Top Institute Pharma, Farmanguinhos and Simcyp – has developed a much-needed paediatric formulation (oral dispersible tablets) of the gold standard drug for schistosomiasis. This disease affects 200 million people worldwide, with up to 60 per cent being children under four years old. Human clinical trials are already underway.

MALARIA

Malaria kills more than 600,000 people every year, most of them young children in sub-Saharan Africa, and drug resistance poses an increasing challenge. In response, a malaria drug partnership has been formed between the Medicines for Malaria Venture (MMV) and Takeda Pharmaceutical Company Ltd. Currently approved antimalarial drugs have safety liabilities that restrict their use, and MMV and Takeda's DSM265 drug candidate has the potential to become the replacement drug for combination treatments requiring a long-lasting partner. If, in addition, it shows to have prophylactic potential against liver stages of the parasite, it would dramatically change the landscape of malaria treatment in disease-endemic countries.

DENGUE

Dengue is endemic in over 100 tropical and sub-tropical countries, and WHO estimates that 100 million people are newly infected every year. KAKETSUKEN and Mahidol University, Thailand, are collaborating on the development of a dengue vaccine. Their live attenuated tetravalent dengue vaccine candidate is expected to mimic the natural dengue virus infection and induce more solid and comprehensive immune responses against pathogenic dengue viruses compared with other vaccine candidates.



Japan is a global leader in technological innovation, drug development and overseas aid. How is the organisation harnessing the country's expertise in these areas to drive itself forward?

KK: Japan has long been the number three global leader in drug development, a globally recognised technology innovator and a critical ODA provider. It also put health on the G8 agenda and played a leading role in the creation of the Global Fund. However, prior to the GHIT Fund's establishment, Japan had yet to realise its potential in the development of technology specifically for global health. As mentioned earlier, this recognition converged a few years ago, resulting in the creation of the organisation, and we are proud to say that Japan has backed the GHIT vision in very meaningful, concrete ways.

Not only have six world-class Japanese pharmaceutical companies contributed funding and significant R&D resources, they have also set a new standard for industry engagement in global health. The GHIT Fund's establishment represents the first time that a group of pharmaceutical companies has joined together to initiate a major fund – and invested directly – to facilitate and advance global health R&D. Additionally, and critically, the Japanese Government has stepped up in game-changing ways: two government ministries collaborated on one budget to provide over US \$50 million to the effort. Moreover, in 2013 the Government made global health a central component of its foreign policy and domestic economic revitalisation strategy. This is a policy milestone – the culmination of a longer-term intensification of efforts to more effectively support R&D and leverage the country's abundance of technology and innovation in order to maximise the value of its foreign aid.

In what ways does the GHIT Fund assess the impact of the research projects it financially supports?

BTS: In order to assess the impact of our R&D investments, we track progress through three key metrics that are common to the pharmaceutical and biotech industries. Our product development milestones track the progress of our investments through 33 key steps in the R&D process – from discovery to clinical development to approval. Of these 33 milestones, 13 are also Stage-Gates, which serve as critical decision points for continued investment. Additionally, we quantify the number of products that are targeted to result from our investments as a way to track innovation. Alongside these three metrics, we illustrate the

long-term socioeconomic impact of our investments through targeted case studies available on our website.

'Open innovation' is a new model for product development that takes a strong collaborative approach to creating new drugs, vaccines and diagnostics. What are the advantages of this novel method?

BTS: Open innovation is advantageous in that it creates a foundation where organisations can contribute their best assets and resources at the most relevant stages of research, and where no one entity shoulders the entire massive cost burden. In other words, it connects the best of the best at the right moments in the R&D process, reducing risk for all partners while increasing R&D productivity and ultimately creating new, affordable medicines for patients faster.

Traditionally, product development for a certain drug, vaccine or diagnostic has been housed and advanced by a single entity – a life science company, university or research institution. But no one entity excels at every step in the discovery-development-delivery process, and so progress in bringing new, affordable tools to global health in the past has been slow. In our experience, and what we are seeing through our development partnerships, is that open innovation delivers sources of innovation and ideas that have previously been untapped or unconnected across diverse sectors.



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