

Drugs for Neglected Diseases *initiative*, North America
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Committee on Appropriations
United States House of Representatives

Thank you for the opportunity to submit testimony on the Fiscal Year (FY) 2014 State and Foreign Operations appropriations measure, specifically regarding the United States Agency for International Development's (USAID) Neglected Tropical Disease (NTD) Program. I am the Policy Director of the North America office of the Drugs for Neglected Diseases *initiative* (DNDi)—a non-profit, patients' needs-driven research and development (R&D) organization that develops new drugs for people suffering from neglected diseases.

I respectfully request that the Committee fund USAID's NTD program at \$125 million; support investment in R&D for NTDs to bring new treatments to people suffering from these diseases; and expand the current USAID list of NTDs to ensure the NTDs with the highest death rates are incorporated into the program.

When 13-year-old William fell sick, he and his father, refugees from Sudan, walked 15 miles to reach the nearest clinic in Uganda. William had contracted African sleeping sickness, a painful disease spread by the bite of the tsetse fly. He was immediately hospitalized and given the first of a three-week course of injections. Two days later—and after just one injection—William died. But not from the disease. William died because the only treatment available at the time—an arsenic-based drug called melarsoprol—was so toxic it killed 1 in 20 patients. Tragic stories like this were commonplace in villages, towns, and refugee camps all across sub-Saharan Africa just 10 years ago. Today, we are starting to see changes.

Children like William now receive a new treatment called NECT (nifurtimox-eflornithine combination therapy), a safe and effective medicine for sleeping sickness developed by DNDi.

This new treatment has dramatically reduced the use of the toxic drug melarsoprol and is saving thousands of lives.

Right now, millions of people across the globe are suffering from deadly neglected diseases we hear very little about such as sleeping sickness, Chagas disease, and leishmaniasis. And little-known but debilitating diseases caused by parasitic worms called filaria destroy the lives of many more. Neglected diseases mainly affect the poorest communities across Africa, Asia and Latin America and since these communities do not represent a lucrative market for the pharmaceutical industry, little to nothing is invested into research to discover and develop new treatments that could save lives. This is why DNDi exists—to deliver new treatments for neglected diseases that afflict millions of the world’s poorest and most vulnerable people.

More than 1 billion people—representing one-sixth of the world population—are infected with at least one of the 17 diseases listed by the World Health Organization (WHO) as neglected tropical diseases.¹ Women, children like William, and ethnic minorities, especially those living in remote or unstable areas with restricted access to services, are most at risk of infection, illness, and death. NTDs also impair worker productivity and are an important reason why the world’s poorest 1.4 billion people who live below the poverty line cannot escape destitution and despair.²

Despite the advances in medicine over the past half-century, with therapeutic innovations saving many millions of lives, adequate drugs are not available for diseases that exclusively or predominantly affect the poor. R&D for NTDs attracts less attention and consequently less financial investment as the population affected is forgotten and has no voice on the international stage. Of the 1,556 new drugs approved between 1975 and 2004, only 21 (1.3%) were

¹ World Health Organization (2012) Neglected tropical diseases. Geneva: WHO, Available: http://www.who.int/neglected_diseases/diseases/en/ Accessed March 14, 2012.

² Hotez PJ, Pecoul B (2010). “Manifesto” for Advancing the Control and Elimination of Neglected Tropical Diseases. *PLoS Negl Trop Dis* 2010; 4(5): e718. Available: http://www.dndi.org/images/stories/pdf_scientific_pub/2010/PLoS%20NTD_Pecoul_Hotez_MANIFESTO_250510.pdf. Accessed July 14, 2010.

specifically developed for tropical diseases and tuberculosis, even though these diseases account for 11.4% of the global disease burden.³

DNDi specifically focuses on developing new treatments for some of the most neglected patients in the world, including those with the three NTDs with the highest death rates—sleeping sickness (human African trypanosomiasis, or HAT), Chagas disease, and kala azar (visceral leishmaniasis, or VL); those with filarial parasitic-worm infections, namely river blindness (onchocerciasis), elephantiasis (lymphatic filariasis), and African eye worm (*Loa loa*, or loiasis); and pediatric HIV/AIDS. Sleeping sickness and kala azar are 100 percent fatal if left untreated. River blindness and elephantiasis blind and deform people, young and old. Chagas disease almost exclusively infects those in Latin America and kills more people in this region than malaria.

In the late 1990s, Doctors Without Borders field teams were becoming increasingly frustrated because they were unable to treat patients—like William—suffering from certain neglected diseases as the medicines they were using were ineffective, toxic, or no longer produced. Doctors Without Borders committed a portion of its 1999 Nobel Peace Prize funds to create a new, innovative, patient needs-driven organization dedicated to R&D for neglected diseases—and DNDi was the result.

DNDi was founded in 2003 when Doctors Without Borders joined forces with public health and research institutes from Kenya, India, Malaysia, Brazil, and France, as well as the tropical disease research program of the World Health Organization, which serves as a permanent observer. Since then, we've created six new treatments for sleeping sickness, kala azar, chagas disease and malaria—which have already reached more than 100 million patients.

³ Chirac P, Torreele E (2006) Global framework on essential health R&D. *Lancet* 2006; 367:1560-61.

And with more than ten promising new drug candidates and numerous other combination therapies in the pipeline, DNDi is connecting the dots between the patients who urgently need new medicines and those who can develop them. We're working with people all around the world committed to helping patients like William.

The U.S. government has an important role to play in NTDs as well. USAID's NTD Program was launched in 2006 and was one of the first global efforts to address NTDs comprehensively. However, the current initiative only focuses on five of the seventeen NTDs identified by WHO. It does not fund diagnosis and treatment of the NTDs with the highest death rates, like the one that plagued William, (sleeping sickness, Chagas, kala azar), and it does not allocate any funding to R&D for much-needed new treatments for NTDs. All NTDs require an increase in R&D efforts in order to bring new tools to patients, improve the effectiveness of existing tools, respond to the challenge of drug resistance, and enhance prospects for achieving disease elimination.

While basic research and early-stage product development is within the mandate of the National Institutes of Health (NIH) and should continue to be funded through traditional NIH channels, late-stage product development, including for drugs, vaccines and diagnostics—which is not done by NIH, or even by the private sector—is urgently needed to support a more robust and effective response to NTDs in both the near- and long-term. DNDi calls on the U.S. government to invest, without delay, in late-stage product development efforts for NTDs at USAID in order to bring new drugs to patients suffering from these neglected diseases and bridge the gap between innovation and access to scientific research. This would align NTDs with other USAID programs in malaria, HIV/AIDS, and TB, which currently allocate a percentage of their funding for late-stage product development.

USAID has publicly committed to contributing to the global goals of elimination and control of certain NTDs, both through the U.S. Global Health Initiative targets and, most recently, by signing on to the “London Declaration on NTDs” on January 30, 2012 at the high-level event, “Uniting to Combat NTDs.” But it will not be possible to achieve some of these goals without new tools and rather than sustaining or expanding its commitment to NTDs, USAID appears to be retreating.

In order to ensure that new tools are developed for neglected diseases, we strongly urge the Committee to enhance its support for NTDs by funding the USAID NTD Program at \$125 million in FY 2014 and encouraging USAID to invest in R&D for NTDs. Specifically, we ask for the following language in the report on the State and Foreign Operations appropriations legislation: *“The Committee is concerned about the burden of neglected tropical diseases (NTDs) and commends USAID’s effort to provide treatments for five of the highly prevalent NTDs in the developing world. The Committee is concerned, however, that for many NTDs, current diagnostic and therapeutic tools are not sufficient to properly treat patients, and encourages USAID to allocate resources to support late-stage product development for NTDs as it does for all other disease areas. Ongoing innovation is needed for all NTDs to ensure access to new treatments as well as protect against drug resistance and co-infections, which can make existing drugs less effective. Support for public-private partnerships that conduct research and development for new tools for NTDs should be a component of the research agenda at USAID.”*

Thank you for the opportunity to provide this testimony and to share the experience of DNDi in developing new treatments for patients suffering from neglected diseases throughout the developing world.