Drugs for Neglected Diseases *initiative,* North America
Rachel M. Cohen, Regional Executive Director
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Subcommittee on State and Foreign Operations, Committee on Appropriations
United States House of Representatives

Thank you for the opportunity to submit written testimony on the Fiscal Year (FY) 2013 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 Regional Executive 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 $100 million, which is consistent with the President's FY 2012 request; 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.

Who suffers from NTDs? 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, 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]

Why do we not have better tools available to combat neglected diseases? Patients suffering from these diseases are neglected because they are poor and marginalized. The current system to develop new drugs, diagnostics and vaccines, is driven by commercial rewards. A company develops a drug or diagnostic tool, receives a patent that allows the sale of the product at high prices, and the high prices in turn are expected to "recoup" the cost of R&D. This system fails to incentivize R&D if patients cannot pay high prices—either because they are too poor or too few—and is increasingly recognized as an unsustainable business model. 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 specifically developed for tropical diseases and tuberculosis, even though these diseases account for 11.4% of the global disease burden.[3]

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

What is DNDi doing? 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. For sleeping sickness, diagnostic tools are inadequate—late-stage disease requires a painful spinal tap, and melarsoprol, the main drug used is a toxic arsenic derivative, which kills 1 in 20 patients. 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.

DNDi was established in 2003 by Doctors Without Borders/Médecins Sans Frontières, the Indian Council of Medical Research, Brazil's Oswaldo Cruz Foundation, the Kenya Medical Research Institute, the Ministry of Health of Malaysia, and the Insitut Pasteur in France, with the World Health Organization's Special Programme for Research and Training in Tropical Diseases as a permanent observer. Since then, DNDi has delivered six new treatments: two antimalarials; the first new treatment for sleeping sickness in over 25 years, NECT (nifurtimox-eflornithine combination therapy); a set of combination therapies to treat kala azar in Africa and Asia; and a pediatric dosage form of benznidazole for the treatment of children with Chagas disease.

In terms of current neglected-patient needs that the US can immediately address, today river blindness and elephantiasis are treated with the drug ivermectin, but in those co-infected with African eye worm, this treatment, alone or in combination with another drug called albendazole, can lead to brain damage, or even death. Over 14 million people are at high risk. For these patients, a new treatment is urgently needed, and DNDi is developing a new drug called flubendazole that, if successful, would be safe and effective in these patients. Without such a drug, control and elimination of river blindness and elephantiasis will not be possible.

Another urgent patient need is an easy-to-take medicine for sleeping sickness. Although NECT has helped significantly reduce the number of patients treated with the arsenic-derivative melarsoprol, it still requires a week of intravenous infusions and is thus not practical in the field where sleeping sickness is prevalent. This is why DNDi is currently developing two new drug candidates—fexinidazole and oxaborole—that could be provided as a simple oral pill.

What can the US government do? 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 17 NTDs identified by WHO. It does not fund diagnosis and treatment of the NTDs with the highest death rates (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 and diagnostics, is urgently needed to support a more robust and effective response to NTDs in both the near- and long-term. DNDi calls on the US 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 developed a planning document entitled "Neglected Tropical Diseases Draft Strategy 2010-2014," which indicates the Agency's willingness to expand the scope of diseases addressed programmatically to include some of the most lethal NTDs. It also highlights the US government's comparative advantage in contributing to late-stage product development (e.g. phase IIb clinical trials and beyond).[4] This plan and the funding scenarios required were also discussed in a NTD Portfolio Review Stakeholder meeting held by USAID in March 2011. Moreover, USAID has publicly committed to contributing to the global goals of elimination and control of certain NTDs, both through the US Global Health Initiative targets and, most recently, by signing on to the "London Declaration on NTDs" on January 30 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 $100 million in FY 2013 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.

[4] Neglected Tropical Diseases Draft Strategy 2010-2014, Drafted by USAID/GH.