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Thank you for the opportunity to submit written testimony to the Outside Witness Hearing for the Subcommittee on State and Foreign Operations, Committee on Appropriations, United States House of Representatives. I am the Executive Director of the North America office of the Drugs for Neglected Diseases *initiative* (DNDi) – a not-for-profit organization developing new drugs for patients suffering from neglected diseases. In the following testimony I will describe the magnitude of the devastation wrought by neglected diseases, highlight some of the challenges faced by patients suffering from the most neglected diseases, and outline how DNDi is addressing the needs of neglected patients. I will also respectfully request that the Committee enhance US government support for research and development (R&D) to bring new treatments to people suffering from these diseases. **In particular, I will emphasize the urgent need for the USAID to include R&D among the activities supported by the Neglected Tropical Diseases (NTD) Program and to ensure the most fatal NTDs are incorporated into the program. I will appeal for increased support from Congress to enable these needed improvements; specifically, I will request that Congress fund the USAID NTD Program at a level of at least \$100 million in 2012, as requested by the President.**

Who suffers from NTDs? More than 1 billion people - representing one sixth of the world population - are infected with at least one of the 14 diseases listed by the World Health Organization (WHO) as neglected tropical diseases.¹ Women, children, and ethnic minorities,

¹ World Health Organization (2010) Neglected tropical diseases. Frequently asked questions. Geneva: WHO, Available: http://www.who.int/neglected_diseases/faq/en/index.html. Accessed July 14, 2010.

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 agricultural 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. 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 phenomenal 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 little attention and consequently little financial investment because the population affected is forgotten and has no voice in the political arena or 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

What is DNDi doing? DNDi specifically focuses on developing new treatments for the most neglected patients suffering from diseases such as human African trypanosomiasis (HAT or sleeping sickness), visceral leishmaniasis (VL or kala-azar), Chagas disease, and malaria and

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

recently announced an expansion of its portfolio to include specific Helminth infections and pediatric HIV. As many of you will never have heard of some of these diseases, I will take a brief moment here to give you a better idea of the situation facing these patients.

In the case of sleeping sickness, diagnostic tools are inadequate, and the few drugs that are available are toxic (i.e. melarsoprol, an arsenic derivative, which kills 1 in 20 patients), difficult to use, and increasingly ineffective in preventing death (with up to 60% resistance reported in some areas). For VL, major obstacles include invasive diagnostics, long treatment duration (30 days), and drug resistance (up to 65% in India). Meanwhile, no drugs exist at all to treat patients with chronic Chagas disease, even though more than 100 million people in Latin America are at risk of contracting the disease.

DNDi was established in 2003 by Doctors Without Borders/Médecins Sans Frontières, the Indian Council for Medical Research, Brazil's Oswaldo Cruz Foundation, the Kenya Medical Research Institute, the Ministry of Health of Malaysia, and the Pasteur Institute in France, with the UNDP/World Bank/World Health Organization's Special Programme for Research and Training in Tropical Diseases as a permanent observer. Since then, DNDi has been working to improve the lives of patients suffering from these diseases and in just seven years has delivered four new treatments: two fixed-dose anti-malarials; the combination treatment NECT (nifurtimox-eflornithine combination therapy) for late stage sleeping sickness; and a combination treatment in Africa to treat VL.

The new combination therapy NECT for sleeping sickness is currently available in 10 African countries that account for 97% of reported HAT cases – significantly reducing the number of patients who are exposed to melarsoprol. While this combination treatment is a great advance for patients because they are no longer subjected to a drug that can kill them, NECT still

involves 14 intravenous infusions over 10 days and is thus not ideal in the settings in which it must be used. That is why DNDi is testing a novel treatment that could be provided as an oral pill with a less than 10-day treatment duration. In the case of Chagas disease, which disproportionally affects children, DNDi and its partners have developed the first formulation specifically for children in the acute phase of the disease. This treatment will be available in Latin America this year. It is important to note that while primarily found in Latin America, there are 300,000 documented cases of Chagas in the US. While many challenges remain, DNDi is bringing new treatments to patients who would otherwise be subject to toxic and intolerable treatments, or have no treatment at all.

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 seven of the 14 most neglected tropical diseases identified by WHO. It does not fund diagnosis and treatment of the deadliest NTDs, such as Chagas disease, sleeping sickness, or VL, and it does not allocate any funding to R&D for much needed new treatments. 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, particularly those that are fatal if left untreated.

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 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 equitable access to the benefits of 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 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 fatal NTDs and to invest in R&D. It also highlights the US government’s comparative advantage to contribute to late-stage product development (e.g. phase IIb clinical trials and beyond).⁴

In order to ensure that the goals of the Global Health Initiative related to NTDs are met adequate funding is crucial. In addition, new tools need to be developed if there is to be any hope of achieving disease elimination for a wider range of neglected diseases. We strongly urge the Committee to enhance its support for NTDs by funding the USAID NTD Program at the level of at least \$100 million in 2012 and instructing USAID to invest in R&D for both disabling and life-threatening NTDs. Specifically, we ask for the following language: “The Committee is concerned about the burden of neglected tropical diseases (NTDs) and commends USAID’s effort to provide treatments for seven of the highly prevalent NTDs in the developing world. The Committee is concerned, however, that appropriate treatments be developed for the NTDs that do not presently have sufficient tools for treatment and encourages USAID to allocate resources to support late stage research and development for NTDs such as human African trypanosomiasis (sleeping sickness), Chagas disease, and leishmaniasis.” 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.