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Rare and Neglected Tropical Diseases

Introduction

Chairwoman Kohl, Ranking Member Brownback, distinguished members, thank you for inviting me to testify on interventions to combat rare and neglected diseases.

The U.S. Agency for International Development (USAID) is the principal U.S. agency providing development and humanitarian assistance in more than 100 countries. On behalf of my staff at USAID and the people we serve, I want to thank members of Congress for your longstanding support of health programs.

I especially want to thank Senators Brownback and Brown for their efforts over the years to promote access to medicines for neglected diseases such as tuberculosis, malaria, and other infectious diseases that primarily afflict developing countries. These diseases disproportionately impact poor and rural populations who lack access to basic health services and essential medicines.

We are delighted that the Brownback-Brown provisions have led to such initiatives as the "priority review voucher" program and the establishment of a technical review group on neglected diseases within the Food and Drug Administration to consider and make recommendations to the FDA Commissioner on the appropriate pre-clinical, trial design and regulatory paradigms and on optimal solutions for the prevention, diagnosis and treatment of neglected diseases in the developing world. USAID is pleased

that the FDA review group for neglected diseases held its first meeting in March. We look forward to the recommendations it will issue by March 2011.

USAID is hopeful that the FDA priority review voucher program will encourage private sector companies to invest more of their research dollars and talent in the fight against these terrible diseases.

USAID welcomes Senator Brownback's recommendation to Administrator Shah to support the review process to shorten pathways for neglected diseases. The suggestion is timely, given that the recently launched Global Health Initiative applies a "whole-of-government" approach to global health, mandating that USAID build upon and complement the innovative work of other U.S. Government agencies. The ambitious targets of the Initiative also give us a mandate to increase our focus on supporting innovation and applying new technologies.

USAID is eager to support the review process and ensure that the U.S. Government makes maximum use of the fruits of FDA's regulatory efforts to encourage development of new health products for neglected diseases.

USAID has developed considerable experience both *upstream* and *downstream* of the FDA regulatory process: (1) *upstream* by supporting late-stage pharmaceutical product development as well as providing technical assistance for pharmaceutical companies to help meet product quality standards, and (2) *downstream* by actually purchasing these drugs and working with developing country Ministries of Health to make sure they are aware of innovative new products, and that these products are quickly introduced and scaled up within national programs. USAID stands ready to share its experience and knowledge of product development and introduction in developing countries. This knowledge and experience may be useful to the FDA review group on neglected diseases as it works to identify ways that could help shorten the pathways for bringing medicines for neglected diseases to the market and to the patient. USAID also stands ready to contribute to implementation of any recommendation by the FDA review committee within the confines of its mandate as a development agency.

I would like to describe in more detail the support USAID currently is providing around the approval process by the FDA or other regulatory authorities. This is the area of most immediate interest to the FDA review

committee. I will focus in particular on product development efforts in malaria, tuberculosis, and neglected tropical diseases.

USAID and the Global Health Initiative place a high priority in the rapid introduction of new technologies in the field. USAID employs a “research to use” strategy to guide its investment in innovations of low-cost and effective health products. Through its product development plans, USAID works to ensure that as new products become available and are proven to be effective, they can be quickly introduced in developing countries.

USAID Promotion of Pharmaceutical Innovation

In the area of malaria, USAID provides support to product development through the Medicines for Malaria Venture (MMV), a non-profit, public-private partnership created in 1999 by the World Health Organization, the World Bank, and bilateral donor governments. MMV’s goal is to register at least one new antimalarial drug every five years; its focus is on discovery and development of drugs that will be affordable to populations living in malaria endemic areas; that are effective against drug-resistant strains of *P. falciparum*; and that can be used safely in young children and pregnant women. MMV's current portfolio contains more than thirty projects actively pursuing novel compounds and promising analogues. USAID will contribute \$2.5 million to MMV in FY2010.

USAID also funds the Malaria Vaccine Development Program (MVDP) to accelerate the development of a vaccine that can be used as part of malaria control efforts. The Program emphasizes support for promising vaccine candidates through the production and testing of investigational vaccines. The MVDP works closely with academia, the commercial sector, and other government agencies. Experimental vaccines manufactured by several companies have been tested under the program.

In the area of TB, USAID supports late-stage product development of needed TB medicines through the Global Alliance for TB Drug Development and Tropical Disease Research partnership. USAID is supporting the continuation of late-stage clinical trials of four medicines or compounds with the aim of shortening the duration of TB treatment. Our TB Research Strategy also funds new diagnostic technologies that increase the sensitivity and specificity of TB testing and evaluates new diagnostic technologies that more easily detect TB, enable rapid detection of drug resistance, and detect latent TB infection.

Finally, in the area of neglected tropical diseases, USAID focuses currently on approaches that are “tool ready,” in other words, for which medicines are already available and have proven to be safe and effective and amenable to mass drug administration. Going forward, USAID hopes to support late-stage trials of new drugs that may accelerate progress toward the elimination of some NTDs and may enable the inclusion of other NTDs as “tool ready.” As safe and effective new drugs and tools become available for other neglected diseases, USAID looks forward to adding these diseases to its program.

Neglected Diseases and the Global Health Initiative (GHI)

All of USAID's work is coordinated with other donors, the private sector and host country groups through operational partnerships in each of the priority countries, as well as with other Federal agencies such as the Centers for Disease Control and Prevention.

This approach guides not only all USAID-led programs, but also whole-of-government Presidential Initiatives in global health, global climate change, and food security.

So let me take a few minutes to describe the core components of the Global Health Initiative, and how they relate to neglected diseases. The goal of the Global Health Initiative is to achieve dramatic improvements in sustainable health outcomes, with a particular focus on improving the health of women, newborns and children. USAID's efforts to reach the goals of the GHI will depend on significant progress against neglected diseases.

The United States will invest \$63 billion to help the approximately 80 partner countries where the U.S. Government provides health assistance to improve health outcomes, including the continuation of commitments for HIV/AIDS, tuberculosis and malaria, with a particular focus on improving the health of women, newborns and children. The GHI is a global commitment to invest in healthy and productive lives, building off of, and expanding, the U.S. Government's successes in addressing specific diseases and issues. Addressing wide-ranging health needs in partnership with governments, communities and other partners represents an ambitious agenda that can be met only if we work together, aligned toward common

goals, with a commitment to fundamentally improve the way we do business.

Achieving major improvements in health outcomes is the paramount objective of the Initiative. To that end, the GHI supports the following goals and targets with regard to rare and neglected diseases:

- **Malaria:** USAID, in partnership with the Centers for Disease Control and prevention, will seek to reduce the burden of malaria by 50 percent for 450 million people, representing 70 percent of the at-risk population in Africa. USAID was very pleased that malaria was included in the definition of neglected diseases within the context of the Brownback-Brown amendment.
- **Tuberculosis (TB):** The U.S. Government's tuberculosis program will save approximately 1.3 million lives by reducing TB prevalence by 50 percent. This will involve treating 2.6 million new TB cases and 57,200 multi-drug resistant (MDR) cases of TB. The Brownback-Brown amendment's inclusion of TB drugs within the review committee's purview will be essential in helping us address the long-term challenge of tuberculosis control, particularly with the emergence of multi-drug resistant strains of the disease.
- **Maternal and Child Health:** Save approximately 360,000 women's lives by reducing maternal mortality by 30 percent across assisted countries. The GHI strategy seeks to improve health systems in ways that particularly target maternal health, including through the expansion of support for antenatal care, and for significant increases in the number of trained health care providers. These health systems can also provide a foundation for the diagnosis, treatment and prevention of neglected diseases, ensuring that newly developed drugs are procured and prescribed as needed.
- **Child Health:** Save approximately 3 million children's lives, including 1.5 million newborns, by reducing under-5 mortality rates by 35 percent across assisted countries. Some of the highest mortality and morbidity associated with rare and neglected diseases occurs in children. The GHI cannot meet its goal of reducing mortality of children under five years of age without addressing rare and neglected diseases.
- **The Neglected Tropical Diseases (NTDs) Program:** USAID's NTD Program seeks to reduce the prevalence of 7 NTDs by 50 percent among 70

percent of the affected population, contributing to: (1) the elimination of onchocerciasis in Latin America by 2016; (2) the elimination of lymphatic filariasis globally by 2017; and (3) the elimination of leprosy. This program began in five countries in 2006 and has since expanded to integrated NTD control in 14 countries — Bangladesh, Burkina Faso, Cameroon, Democratic Republic of Congo, Ghana, Haiti, Mali, Nepal, Niger, Sierra Leone, Southern Sudan, Tanzania, Togo, and Uganda. As anticipated, following 4-6 years of repeated mass drug administration, transmission of some NTDs is being interrupted. Ghana, supported by USG efforts since 2006, has eliminated trachoma as a blinding disease and may be able to stop annual drug administration for lymphatic filariasis after the coming year.

Innovation and the GHI

And finally, one of the hallmarks of the GHI is its emphasis on innovation. The GHI strategy puts at its an understanding of the importance of United States leadership in research and robust use of rigorous peer-reviewed research that is already available. The U.S. Government recognizes that advancements in health often occur through the discovery and development of new biomedical technologies, including not only new drugs but also diagnostics and vaccines; medical devices, such as the female condom; and information and communication technologies, such as mobile telephones and other data transmitting devices that have the potential to improve people's health.

Although the GHI is not expected to make direct investments in clinical trials for new products, we will take advantage of opportunities to link the results of these trials with the means for improving service delivery. The GHI also will look to accelerate the appropriate use of existing technologies as well as create favorable conditions for more rapid introduction and successful scale-up of technological advances that have been demonstrated to improve health outcomes.

The GHI will work with in-country partners to overcome bottlenecks and accelerate delivery pathways to ensure that innovative technologies can be widely adopted, including improving demand forecasting for new products, supporting evidence-based decision making within partner countries, and conducting operational and implementation research. These systems will pay particular attention to the FDA review processes, to ensure that they translate discoveries in the lab into success in clinical care in developing

countries. Operational and implementation research will help identify critical problems and improvements, including: sustainable and cost-effective service delivery approaches; obstacles to rapid scale-up and approaches to reduce such obstacles; and strategies to help improve health service delivery models.

Given the whole-of-government approach of the GHI, the Initiative will work through existing governmental mechanisms and partner with private sector donors and foundations to support research. Synergies will be sought between GHI programmatic activities and existing research partnerships of the National Institutes of Health, the Centers for Disease Control and Prevention, and other agencies that have active biomedical and public health research programs, and the FDA priority review voucher program and the neglected diseases review group will be important parts of those efforts.

Conclusion

In conclusion, USAID stands ready to support initiatives to shorten pathways for medicines against neglected diseases. Neglected diseases can be beaten if there is full engagement of all concerned government agencies throughout the product development continuum: from basic research to efficacy trials, to regulatory review and promotion and distribution of new products into the hands of health care workers. USAID is one link in this chain. We will work with our colleagues across the U.S. Government to ensure that innovative new products are developed and put to use in developing countries to combat malaria, TB, and other neglected diseases.

Thank you.