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HEARING

TREATING RARE AND NEGLECTED PEDIATRIC DISEASES:
PROMOTING THE DEVELOPMENT OF NEW TREATMENTS AND CURES

*Innovation and Access for Neglected Diseases:
The Experience of Médecins Sans Frontières*

Testimony of
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EXECUTIVE SUMMARY

Doctors Without Borders/ Médecins Sans Frontières (MSF) is an international independent medical humanitarian organization. For decades, MSF has been one of the only actors providing care and treatment to impoverished people suffering from neglected diseases, such as Chagas disease, kala azar, sleeping sickness and Buruli ulcer. Globally, neglected diseases target the bottom billion – those living in the most rural locations, with poor or no access to healthcare, and extraordinarily limited resources. As a founding member of the Drugs for Neglected Diseases *initiative* (DNDi), a product development partnership (PDP), MSF is also the third largest philanthropic funder of neglected disease research. The problems we face are twofold: there is limited access to the tools that exist to diagnose and treat these diseases, but the existing tools are also terribly insufficient – new products are urgently needed.

However, the current commercially-driven system for drug, diagnostic and vaccine development leaves many urgent health needs unanswered. New medicines for sleeping sickness were not developed for fifty years despite pressing needs. There is no test to determine whether patients have been cured of Chagas disease after a course of treatment. A diagnostic tool for tuberculosis (TB) does not exist in a form appropriate for resource-poor settings. The populations afflicted by these diseases are simply too poor to provide adequate commercial incentives for R&D in a system that relies almost entirely on the ability to sell products at high prices to incentivize drug and diagnostic development. New incentive mechanisms are needed.

MSF believes that de-linking the cost of R&D from the price of health products needs to be the key principle used to evaluate and develop mechanisms to stimulate R&D and ensure access. De-linkage would separate the market for R&D from the market for product manufacturing. The concept of de-linkage fully accepts that R&D costs money, but seeks alternative ways to fund it. By paying for R&D through financing rather than through product prices, de-linkage removes the need to incentivize R&D through high prices. In this way, de-linkage can also stimulate R&D where there is no profitable market – that is, for neglected, rare, orphan diseases, or diseases like pediatric HIV/AIDS. From our experience with DNDi, we know that a range of different funding mechanisms that allow de-linkage are needed, either to “push” R&D via upfront funding (e.g. through PDPs) or to “pull” R&D to ensure that the right products reach the end of the pipeline.

Prizes are one attractive “pull” mechanism for de-linking the markets for R&D and product manufacturing. The key potential benefits of a well-designed prize include: the ability to drive R&D based on health needs; allowing competition (rather than governments or donors) to determine the path or team most likely to succeed; attracting a broader, more diverse base of potential “solvers” to a problem; and the flexibility to build in provisions for collaboration, knowledge-sharing, and affordability of end products. Prize designs can vary, and they can also be given for different stages of the R&D process. Prize funds would be promising, and could quickly be established, in at least two areas of urgent need: a point-of-care TB diagnostic test and new products for Chagas disease.

In 2008, the U.S. government established the Presidential Initiative on Neglected Tropical Diseases. However, the initiative only focused on five of the 14 most neglected tropical diseases identified by the WHO, did not fund diagnosis and treatment of the deadliest neglected diseases, and did not provide support for the development of innovative products for these diseases. MSF urges the US government to include the most deadly tropical diseases (Chagas disease, sleeping sickness, kala azar, and Buruli ulcer) within the scope of its new Global Health Initiative, and to provide support for improved access to *existing* health tools, as well as for the development of new and improved ones.

We also urge the US government to craft its policies and mobilize its financial resources to support new incentive mechanisms that embrace the principle of de-linkage, such as prize funds, in order to generate the innovation that we need to improve the lives of the world’s poorest children and families.

Innovation and Access for Neglected Diseases: The Experience of Médecins Sans Frontières

**Testimony of
Suerie Moon**

Board of Directors

Doctors Without Borders/Médecins Sans Frontières - USA

Thank you, Chairperson Harkin, Ranking Member Enzi, and the Senate Health, Education, Labor, and Pensions Committee for calling for this important hearing. This is a critical moment of both need and opportunity for innovation and access for neglected tropical diseases.

My name is Suerie Moon and I am on the U.S. Board of Directors of Doctors Without Borders, known as MSF, an acronym for our French name, Médecins Sans Frontières. MSF is an international independent medical humanitarian organization. My experience with MSF dates back to 1999 and includes fieldwork in the Democratic Republic of Congo and China, as well as over a decade of research and analysis on access to medicines and innovation policy issues.

We are most known for our emergency responses during armed conflict or following devastating natural disasters, or for our work against medical disasters like HIV/AIDS.

Less visible is our engagement in providing care and treatment to impoverished people suffering from diseases so neglected that many in the world have never heard of them before – Chagas disease, kala azar, sleeping sickness and Buruli ulcer, to name a few. From our decades of experience running programs and conducting operational research, we know that there is limited access to the tools that exist to diagnose and treat these diseases. But we also know very well that these tools are terribly insufficient, and new products are needed.

Globally, neglected diseases can best be thought of as the diseases of the bottom billion – those living in the most rural locations, with poor or no access to healthcare, and extraordinarily limited resources. People suffering from these diseases do not represent a profitable potential market and therefore current market incentives have proven insufficient to generate the development of better tools for prevention, diagnosis, treatment, and cure for these diseases. Between 1975 and 2004, only 1.3% of all new drugs were specifically developed for tropical diseases and tuberculosis, even though these diseases account for 11.4% of the global disease burden.¹ In addition, even when effective tools do exist, these populations can be difficult to reach due to geographic or social marginalization. Political will is often lacking, and healthcare infrastructure can be weak.

I would like to take the opportunity to share with you today the experiences of MSF in both treating and supporting innovation in treatments and diagnostics for neglected diseases.

MSF Experiences with Neglected Diseases

Many diseases, such as tuberculosis and tropical diseases, are neglected because they primarily affect people in poor countries. Across many of the diseases that disproportionately affect developing

¹ Chirac, P., & Torreele, E. (2006). Global Framework on Essential Health R&D. *The Lancet*, 367(9522), 1560-1561.

countries, children are particularly neglected: adapted pediatric medicine formulations are missing for diseases such as tuberculosis, Chagas disease and HIV/AIDS.

The World Health Organization (WHO) has identified as neglected tropical diseases (NTDs) 14 major parasitic, bacterial and viral diseases that are the most common infections in the 2.7 billion people living on less than \$2 a day. Those affected are often marginalized and forgotten by governments, left to suffer in silence. Other diseases like tuberculosis and pediatric HIV/AIDS are also neglected but are not within the WHO list of NTDs.

MSF has for many years provided diagnosis and treatment for individuals afflicted with NTDs, primarily focusing on visceral leishmaniasis (VL, or kala azar), human African trypanosomiasis (HAT, or sleeping sickness), Chagas disease (American trypanosomiasis), and Buruli ulcer. Three of these NTDs—VL, HAT, and Chagas disease—are often fatal if left untreated and have the highest rates of death of all of the NTDs. MSF is one of the only actors in the world involved in the treatment of these diseases.

Governments and donors have continued to neglect those who suffer from these diseases. These four diseases are largely left out of control and treatment programs by health actors and donors because they are considered too difficult and costly to treat; the available tools are limited; little investment has been made into research and development (R&D); and their disease burdens are poorly understood due to inadequate screening and surveillance systems. Nevertheless, the diseases are no less devastating for the individuals and countries affected. These barriers beg greater, not less, attention for effective responses to these diseases.

In 2008, the U.S. government established the Presidential Initiative on Neglected Tropical Diseases. However, the initiative only focused on five of the 14 identified by the WHO.² It did not fund diagnosis and treatment of the deadliest neglected diseases, and did not provide support for the development of innovative products for these diseases. As part of the Global Health Initiative (GHI), the U.S. government has now proposed a significant increase in funds for NTDs. MSF welcomes this increased attention to the NTDs. However, there remains an ongoing neglect of the most deadly and most forgotten diseases.

It may be impossible in an illustrious committee room in the U.S. capital to paint a picture of the diseases that affect the poorest of the poor, who often live in the most remote areas of the world, but I will try.

Chagas disease (American trypanosomiasis)

Chagas disease is an appropriate place to start if only because there are currently an estimated 300,000 people living with this disease in the United States today. There are 15 million people living with Chagas disease around the world. It is the largest parasitic killer in the Americas, responsible for about 14,000 deaths per year, mostly in South and Central America.

This disease is caused by a parasite transmitted by a bug (the triatome). They call it the “kissing bug” because it bites gently, and victims often do not even know they have been bitten. It also can be transmitted from mother to child during pregnancy; and through blood transfusions and organ transplantation, and sometimes through oral transmission. If untreated, it infects the heart and

² The Presidential Initiative on Neglected Tropical Diseases disaggregates one WHO identified disease into three, therefore identifying the Presidential Initiative as responding to seven neglected diseases.

digestive system of one-third of those carrying the parasite – with fatal effects in 30 percent of patients over a period of time.

Diagnosis currently requires confirmation through laboratory tests. In many cases, the endemic countries do not have the necessary facilities or staff available to carry out these tests.

MSF has provided free diagnosis and treatment for Chagas disease since 1999 in countries including Honduras, Nicaragua, Guatemala, and Bolivia, which has the highest prevalence in the world. In Cochabamba, Bolivia, MSF runs free, urban and rural Chagas programs that are carried out in collaboration with the Bolivian Ministry of Health in an integrated way in five primary care centres, where children and adults up to the age of 50 are treated and diagnosed. Through 2009, MSF has screened over 60,000 people for Chagas disease and treated more than 4,000. We are also currently exploring the possibility of opening a project here in the US to improve detection and access to treatment for people living with Chagas disease.

The tools we have at hand can be used for treatment, but are insufficient. Currently, there are only two medicines to combat Chagas disease: benznidazole and nifurtimox. Both were developed over 45 years ago through research that was not even specifically targeting Chagas disease. Presently, neither of these drugs is adapted for use in children, although a paediatric formulation of benznidazole is anticipated in the coming months. As the side effects of the treatment are more common in older patients, doctors have been reluctant to administer the medicine out of fear of the consequences. Further, there is no test for cure for Chagas disease.

Millions suffering from Chagas disease, especially in rural areas, have neither the opportunity to find out that they are infected nor the possibility of being treated. New diagnostic tests, better medicines, a vaccine, and a test for cure are urgently needed to help prevent, diagnose and treat this disease.

Sleeping sickness

Sleeping sickness, otherwise known as human African trypanosomiasis (or HAT), is a fatal parasitic disease found in 36 countries in sub-Saharan Africa, with an estimated 70,000 annual cases and 60 million at risk. During 2009 less than 10,000 cases were diagnosed and treated, but many more are affected – the true size of the problem remains unknown. Sleeping sickness occurs in the poorest rural areas of Africa, where difficulty of diagnosis, political instability, and lack of health surveillance make diagnosis and care difficult. Sleeping sickness rapidly deteriorates into coma and death – and is fatal in 100% of patients within approximately two years if untreated.

Up to 10 years ago, patients with advanced sleeping sickness would have received an arsenic-based treatment called melarsoprol. Melarsoprol is more than fifty years old and highly toxic, with rising rates of treatment failure. No new treatments had been developed for a half-century for sleeping sickness even though melarsoprol was killing the patient in about five to ten percent of cases, and in some affected areas had only fifty percent effectiveness.

Thanks to the efforts of many partners, including MSF, the WHO, Epicentre, the Drugs for Neglected Diseases *initiative* and the Swiss Tropical Institute (STI), there is now a new treatment for patients with advanced sleeping sickness. These partners have also supported the development of research capacity in countries where sleeping sickness is endemic. Using nifurtimox-eflornithine combination therapy (NECT) has proven to be safer and more effective compared to the existing

standard of care. Eflornithine is given intravenously twice a day for seven days alongside orally-administered nifurtimox. The treatment is life-saving and prevents relapse back into the sickness. In May 2009, the WHO added NECT to the Essential Medicines List (EML) for the treatment of advanced sleeping sickness.

Despite these improvements, the current treatment for sleeping sickness remains long and difficult – for both patients and health workers. Both diagnosis and staging – which requires painful lumbar punctures – demand significant technical capacities and are therefore difficult to implement in remote areas where the disease occurs. There is an immediate need to improve current diagnostic and treatment options, particularly for patients in the advanced stages of this disease.

Tuberculosis

Tuberculosis (TB) is a major public health problem, with over 9.4 million new cases and almost 1.8 million deaths in 2008 alone— or nearly 5000 people every day. TB is a leading cause of mortality in children worldwide, with approximately one million cases and 400,000 deaths each year in children under 15 years old as of 2006. The most commonly used TB diagnostic test is Sputum Smear Microscopy (SSM)³. It is relatively fast and easy to implement in resource-limited settings, but it has significant limitations: it detects less than half of all TB cases⁴ and performs even worse in children and people living with HIV who either have difficulties producing enough sputum, or don't have sufficient or any mycobacteria in their sputum to be detected under the microscope. It also completely misses the extrapulmonary form of TB.^{5,6}

A study analyzing the contribution that improving TB diagnostics could make to reducing the global burden of TB, shows that improving the performance, speed and accessibility of TB diagnostic tests are key factors.⁷ The study calculates that 392,000 deaths or 22% of annual deaths due to TB in the four highest-burden WHO regions, could theoretically be avoided by the introduction of a new TB point-of-care diagnostic.

We desperately need a new point-of-care diagnostic test able to diagnose active TB in adults and children who may also be co-infected with HIV; has high sensitivity and specificity; is simple to use and can be operated without the need for extensive infrastructure . Despite the valuable work supported by grant programs administered by entities such as the Foundation for Innovative New Diagnostics (FIND), there is widespread agreement that there is insufficient progress on the development of a new test that meets these needs.

MSF experience in innovation

A decade ago, MSF created the Campaign for Access to Essential Medicines because of our

³ Sputum smear microscopy is done by staining a sputum sample with an acid-fast stain and then examining the sample with a microscope for acid-fast bacilli.

⁴ In countries characterized by high HIV prevalence, the challenge of providing timely TB diagnosis and treatment initiation is even greater. In a study recently conducted in Rwanda, where 62 per cent of the recruited patients were TB/HIV co-infected, only 18 per cent of TB confirmed cases were started on treatment within one month and only 56 per cent within two months.

⁵ Perkins MD, Roscigno G, Zumla A. (2005) Progress towards improved tuberculosis diagnostics for developing countries. *The Lancet* 367(9514): 942–943.

⁶ Shingadia D, Novelli V. (2003) Diagnosis and treatment of tuberculosis in children. *Lancet Infect. Dis.* 3(10): 624–632.

⁷ Keeler, E., et al., (2006) Reducing the global burden of tuberculosis: the contribution of improved diagnostics. . *Nature* 444: 49- 57.

concern about barriers for access to medicines in low- and middle-income countries. People in developing countries are dying because medicines do not exist due to inadequate incentives for their development; or because they are unavailable due, in part, to high costs.

Our work on NTDs convinced us that we wanted not only to advocate for new tools, but also to engage actively in the development of these tools. Therefore, MSF became a founding member of the Drugs for Neglected Diseases *initiative*, or DNDi, a product development partnership (PDP). We continue to contribute funding, making MSF the third largest philanthropic funder of neglected disease research.⁸ From our experience as a founding member of DNDi, we know that a critical role is played by “push” funding – that is, grants invested into promising candidates for future drugs. While push funding and PDPs play an important role, our experience also tells us that incentives are needed throughout the innovation process to ensure that the right products reach the end of the pipeline. For this reason, we also need “pull funding” – that is, incentives at the end of the product development process, such as the promise of a profitable market or other reward. While donors and governments have invested increased amounts in *push* funding, we are just beginning to see serious efforts to explore how best to put in place *pull* funding.

Prioritization of Access Considerations: The Importance of “De-linkage”

The current system for drug, diagnostic and vaccine development creates both innovation and access barriers. Driven by commercial rewards, it is a system that leaves many pressing health needs unanswered— needs that we identify in our medical programs every day. New medicines for sleeping sickness were not developed for fifty years despite pressing needs. The diagnosis of sleeping sickness is complicated, and often requires a blood sample, lymph node aspiration and a painful lumbar puncture. There is no test to determine whether patients have been cured of Chagas disease after a course of treatment. A diagnostic tool for tuberculosis does not exist in a form appropriate for resource-poor settings. These populations are simply too poor to provide adequate commercial incentives for R&D in a system that relies almost entirely on the ability to sell products at high prices to incentivize drug and diagnostic development.

But what if we could separate the market for medicines production from the market for R&D? What if we could encourage robust competition in both?

MSF believes that de-linking the cost of R&D from the price of health products needs to be the key principle used to evaluate and develop mechanisms to stimulate R&D and ensure access. This principle has gained increasing acceptance worldwide. The concept of de-linkage fully accepts that R&D costs money, but seeks alternative ways to fund it. Rather than relying on high prices charged after the innovation has been developed, de-linkage would seek to stimulate innovation from many sources and consider access issues in advance. This approach would broaden incentives for innovation beyond just the profitable diseases, and remove the access barriers created by high prices.

The concept of de-linkage has been included in the Global Strategy and Plan of Action on Public Health, Innovation and Intellectual Property (GSPoA), which was agreed upon in 2008 by all WHO

⁸ Moran, M., Guzman, J., Henderson, K., Ropars, A., McDonald, A., McSherry, L., Wu, L., Omune, B., Illmer, A., Sturm, T., & Zmudzki, F. (2009). *Neglected Disease Research and Development: New Times, New Trends*. Sydney, Australia: The George Institute for International Health, p62.

Member States, including the United States.⁹ In conjunction with this plan, several governments have proposed the creation of new incentive mechanisms, including prizes, based on the principle of de-linkage. Just two months ago, the Council of the European Union decided to explore “models that dissociate the cost of Research and Development and the prices of medicines,” as a part of its global health efforts.¹⁰

Why the broad interest in “de-linkage”? De-linkage is important because the price of the final product is critical for affordability and access, and because R&D should be driven by health priorities, not the size of the market. Innovation by itself is of little value if the tools developed are unavailable or unaffordable to the people who need them. By paying for R&D through financing rather than through product prices, and by addressing the price and availability of the product at the outset, de-linkage removes the need to incentivize R&D through high prices. De-linkage also stimulates R&D where there is no profitable market – that is, for neglected, rare, orphan diseases, or diseases like pediatric HIV/AIDS which has been all but eliminated in rich countries even as a rich country market continues to exist for adult HIV/AIDS medicines.

De-linkage is not just about breaking the link to high prices, but is also about pro-actively designing into any new incentive mechanisms ways to ensure that the affordability and availability of any new health tool are incorporated from the outset of the R&D process. A range of different funding mechanisms that allow de-linkage are needed, either to “push” R&D via upfront funding (e.g. through PDPs) or to “pull” R&D via incentives that focus investment efforts on products needed in developing countries (such as prize funds).

Once the market for R&D is “de-linked” from high medicines prices, we can encourage robust competition among producers of the end product. Our experience shows that competition is the most effective way to achieve reliable price reductions and sustainable, affordable prices. Intellectual property can and should be managed in a way that ensures that a new health tool can be manufactured by other producers, fostering competition and access. A recent example is the patent-free development of the anti-malarial fixed-dose combination of artesunate and amodiaquine by DNDi, in collaboration with the pharmaceutical company Sanofi-Aventis. (In cases such as vaccine development where competition may not be technically feasible in the immediate term, even when favorable licensing terms exist, a pathway to facilitate access is needed, including technology transfer.)

Breaking the Innovation Barriers

Prizes are one attractive option for de-linking the markets for R&D and product manufacturing. Prizes can act as powerful incentives for innovation, but need to be designed carefully in order to maximize the sharing of knowledge, access to end products, and overall return on the public’s investment. Prize designs can vary, and they can also be given for different stages of the R&D process, such as identifying biomarkers, proof of concept, product synthesis, or developing a

⁹ The Global Strategy and Plan of Action Section 5.3.a states: “explore and, where appropriate, promote a range of incentive schemes for research and development including addressing, where appropriate, the de-linkage of the costs of research and development and the price of health products, for example through the award of prizes, with the objective of addressing diseases which disproportionately affect developing countries.” World Health Assembly. (2008). Global Strategy and Plan of Action on public health, innovation and intellectual property. Resolution 61.21. Geneva. Available: http://www.who.int/gb/ebwha/pdf_files/A61/A61_R21-en.pdf

¹⁰ Council of the European Union. (2010) “Council conclusions on the EU role in Global Health.” 2011th Foreign Affairs Council meeting. Brussels, 10 May 2010. Section 18.c. Available: http://www.consilium.europa.eu/uedocs/cms_Data/docs/pressdata/EN/foraff/114352.pdf

finished product all the way through the registration process. The key potential benefits of a well-designed prize include some of the following¹¹:

1. It would allow R&D efforts to be driven by health needs.
2. It would establish a bold and important goal without having donors or governments pick winners by choosing in advance the path or team that is most likely to succeed in reaching it.
3. Payment would only be made when results are achieved. The prize is only paid if the challenge has been met, i.e. if donors can see a direct connection between their funding and the outcomes.
4. With the right backing, a prize can create a “lighthouse” effect by highlighting a problem to a whole new range of potential innovators, who may have previously been unaware of the problem. This increases the number and diversity of potential “solvers” for a problem, which could include, for example, both commercial enterprises and academics. An even wider range of participants could be sought through the award of intermediate prizes for solutions to specific technical challenges.
5. A prize could include incentives for collaboration and knowledge-sharing;
6. By including affordability criteria, the prize could promote both innovation and access.

Two specific examples of urgent needs that we’ve identified in our programs – and for which there will be little engagement from the major R&D players without novel innovation mechanisms – are related to TB and Chagas disease.

Millions would benefit from the creation of a point-of-care (POC) test that would allow the diagnosis of TB at local health centers in resource-poor contexts. The dearth of R&D in TB diagnostics is demonstrated by the chronic lack of investment in this area, particularly from the private sector. Only US\$ 41.9 million was directed towards TB diagnostics R&D – a mere nine percent of total resources spent on TB product development, which is already an under-funded field. Of this amount, only US\$ 2.5 million came from the private sector. A TB diagnostic test designed for use in resource poor areas, which necessarily has to be low cost, requires a different form of incentive that would allow for the cost of the final product to be de-linked from the cost of R&D. A prize competition would create the incentives for R&D in this neglected area.

As noted above, a prize fund would allow for many different approaches to be pursued without deciding at an early stage which is the most promising. This is particularly important in the field of TB POC diagnostic development since there are several approaches that could potentially lead to the delivery of the right test, but it is not clear which angle will be the most successful. Current R&D in different areas of the POC diagnostic market, such as bioterrorism, pandemic influenza, and HIV viral load testing, holds the potential for breakthroughs in the area of TB diagnosis. The governments of Bangladesh, Barbados, Bolivia and Suriname have proposed a prize fund of \$100 million or more for a TB POC diagnostic.¹² By providing a sizeable incentive, the prize would attract many developers to the neglected area of TB.

Prizes are not a new mechanism, but have successfully been used in the past to induce innovation. For example, recently the Global Alliance for TB Drug Development (a PDP) and the Rockefeller

¹¹ See, e.g., Love, James and Hubbard, Tim (2009). "Prizes for Innovation of New Medicines and Vaccines," *Annals of Health Law*, 18 (2): 155-186.

¹² Governments of Bangladesh, Barbados, Bolivia and Suriname. (2009) Prize Fund for Development of Low-Cost Rapid Diagnostic Test for Tuberculosis. 15 April. Contributions from Member States. WHO Secretariat on Public Health, Innovation and Intellectual Property. Available: http://www.who.int/phi/Bangladesh_Barbados_Bolivia_Suriname_TBPrize.pdf

Foundation awarded two prizes for more efficient ways to synthesize a new tuberculosis drug candidate, PA-824. Prizes are also receiving renewed attention in policy circles because of their potential to help address our most pressing public problems. Just this past spring, the White House issued guidance on the Open Government Directive, supporting the use of prizes to encourage innovation in a range of areas, including climate change technology and promoting open government.¹³

While individual initiatives that can be established quickly, such as a TB POC diagnostic prize fund, are important, others are exploring how prizes could be used as part of longer-term systemic changes that are needed to provide sustainable financing for health needs-driven R&D that ensures equitable access.

Similarly, we need innovative tools for the diagnosis, treatment, and test of cure for Chagas disease. The governments of Bangladesh, Barbados, Bolivia and Suriname have proposed creating a \$250 million prize fund to reward the development of new products that would decrease the burden of disease from Chagas.¹⁴

Prizes are also flexible tools. There is not just one model, and they can be designed to fit the medical, scientific, and technical problems that need to be addressed and the specific access issues for a disease area. In some areas it may be more appropriate to have a prize that rewards the development of the final product. In others, it might be more effective to support a prize that can be focused on a critical milestone that could overcome a key barrier to further development. In all cases, however, it is critical that methods to ensure affordable access must be part of the prize design at the start.

DNDi has been considering milestone prizes for Chagas drug development. Substantial rewards for attaining specified milestones along the path to a new drug or other health technology could be a useful supplement to grants for diseases for which market incentives are deficient and where patents are not an effective incentive. Milestone prizes promise earlier pay-outs and are likely to attract new actors such as biotechnology firms, which cannot make major investments in pursuit of rewards that may be many years away.

Several discussions to explore de-linkage mechanisms for the technological needs of Chagas are also ongoing at the regional level as part of the Pan American Health Organization's (PAHO) regional implementation of the GSPoA. These discussions provide a framework for agreement on new incentive mechanisms, including appropriate prize designs to stimulate innovation for Chagas disease.

Conclusion

MSF welcomes the growing attention to patients who suffer from neglected diseases around the world. We ask the US government to include the most deadly tropical diseases (Chagas disease, sleeping sickness, kala azar, and Buruli ulcer) within the scope of its new Global Health Initiative,

¹³ Executive Office of the President: Office of Management and Budget. (2010) "Memorandum for the Heads of Executive Departments and Agencies (M-10-11)." Washington, D.C. 8 March. Available: http://www.whitehouse.gov/omb/assets/memoranda_2010/m10-11.pdf

¹⁴ Governments of Bangladesh, Barbados, Bolivia and Suriname. (2009) Chagas Disease Prize Fund for the Development of New Treatments, Diagnostics and Vaccines. 15 April. Contributions from Member States. WHO Secretariat on Public Health, Innovation and Intellectual Property. Available: http://www.who.int/phi/Bangladesh_Barbados_Bolivia_Suriname_ChagasPrize.pdf

and to provide support for improved access to *existing* health tools, as well as for the development of new and improved ones. We also urge the US government to craft its policies and mobilize its financial resources to support ambitious, visionary approaches to generating medical innovation that can improve the lives of the world's poorest children and families. In particular, the US should support relevant discussions at the WHO and PAHO, and the efforts of the Consultative Expert Working Group that will be formed in the coming months to analyze new innovation mechanisms in depth.¹⁵

I have outlined today just two promising possibilities – the potential of a prize fund for TB diagnostics and for Chagas disease – but there are many others. We need strong political commitment and financial support from governments and other donors if we are to make new incentive mechanisms work. There is increasingly widespread recognition that the existing R&D system is failing – failing patients with neglected tropical diseases, with orphan diseases, and children, among others. Now is the time to begin testing new approaches to generate the innovation that we need to meet global public health needs.

Thank you very much for this opportunity to share our experience with you.

¹⁵ See the mandate given to WHO by the 2010 World Health Assembly. (World Health Assembly (2010). "Establishment of a consultative expert working group on research and development: financing and coordination." Resolution 63.28. Geneva. Available: http://apps.who.int/gb/ebwha/pdf_files/WHA63/A63_R28-en.pdf