

Tufts Center for the Study of  
Drug Development *presents*



# Neglected Diseases in the Developing World

PROGRESS, CURRENT CHALLENGES, AND PROMISING APPROACHES

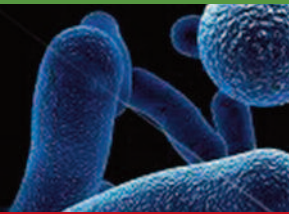


October 16, 2009

Dirksen Senate  
Office Building,  
Room SD-562

Washington, DC

# Neglected Diseases in the Developing World



**PROGRESS, CURRENT CHALLENGES, AND PROMISING APPROACHES**

**FRIDAY, OCTOBER 16, 2009**

**8:30 A.M.-2:30 P.M.**

**DIRKSEN SENATE OFFICE BUILDING**

**ROOM SD-562**

**WASHINGTON DC**

Today's conference is a rare opportunity to hear federal, industry, academic, and other leaders discuss their views on the progress being made to develop effective treatments for neglected diseases — such as Chagas disease and dengue fever — facing those in the developing world and the unique challenges in health care delivery and access in developing countries. You will learn more about the first-ever survey of global public and private investment into R&D for new products to treat neglected diseases, and an update on the number of products approved and in development for neglected diseases. You will also hear a panel discussion of public and private sector experts discuss how to prioritize and facilitate the discovery and development of new medical products for these diseases, as well as perspectives from NGO and public service experts on the public health impacts and current efforts to address this threat to global health, security and well-being.

*The opinions expressed herein reflect those of the authors/presenters, and do not necessarily represent those of Tufts CSDD or PhRMA*

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# Agenda

8:30-9:00 Continental Breakfast

9:00-9:15 Introduction

**Christopher Milne**, Associate Director  
Tufts Center for the Study of Drug  
Development

9:15-10:00 **Global Funding of R&D to Treat  
Neglected Diseases (G-FINDER) Survey**

**Mary Moran**, Director, Health Policy Division,  
The George Institute

10:00-10:30 **Progress in Neglected Disease Drug  
Development**

**Joshua Cohen**, Senior Research Fellow  
Tufts Center for the Study of Drug  
Development

10:30-10:45 Break

10:45-12:00 **Challenges and Opportunities for the  
Continued Development of New  
Treatments for Neglected Diseases**

Panel discussion on challenges, opportunities,  
best practices, successes to date, public-private-  
partnerships, and projections for future—such  
as what incentives are needed to encourage  
existing and new R&D initiatives and how might  
they continue to evolve

**Timothy Cote**, Director, Office of Orphan  
Product Development, FDA

**Steven Groft**, Director, Office of Rare Diseases,  
NIH

**Melinda Moree**, Interim President and CEO,  
BIO Ventures for Global Health

**David Ridley**, Assistant Professor of Business  
Administration, Duke University

**Christy Hanson**, Senior Public Health Advisor,  
USAID

12:00-1:00 Lunch

**Challenges to Improving Access to  
Medicines for Neglected Diseases**

**Michael Reich**, Professor of International  
Health Policy, Harvard University School of  
Public Health

1:00-1:30 **Progress and Priorities in R&D for  
Neglected Diseases**

**Valerio Reggi**, WHO, Head, Department of  
Control of Neglected Tropical Diseases

1:30-2:00 **Addressing Unmet Medical Needs from  
Neglected Diseases: Policy and Practical  
Issues in Developing Countries**

**John Kilama**, President, Global Bioscience  
Development Institute

2:00-2:30 **Impact of Neglected Diseases on Public  
Health, Prosperity and Well-Being**

**Kena Mphonda**, Deputy Ambassador,  
Embassy of Malawi

2:30-3:00 **THE WAY FORWARD:**  
Discussion and Concluding Remark



# Conference Speakers



## Joshua P. Cohen

Senior Research Fellow  
Tufts University's Center for the Study  
of Drug Development

Dr. Cohen received his undergraduate and master's degrees from the University of Amsterdam and was a management consultant for three years with Andersen Consulting (Accenture) in The Hague, Netherlands. Dr. Cohen received his doctoral degree in economics from the University of Amsterdam in 1997, after two years of dissertation work at Harvard University as a visiting research fellow. Before joining the Tufts Center in 1999 as a Senior Research Fellow, Dr. Cohen was a postdoctoral fellow at the Veterans Affairs Medical Center in Philadelphia, where he simultaneously served as research fellow at the University of Pennsylvania's Leonard Davis Institute for Health Economics, examining the use of decision-analytic models in bio-ethics.

His research focuses on public policy issues that concern prescription drug reimbursement and market access, such as the comparison of formulary and chronic disease management in US and Europe, the role of cost-effectiveness in clinical practice guideline development, and the composition of the World Health Organization's Essential Drug List.



## Timothy Cote

Director  
Office of Orphan Product Development,  
FDA

Timothy Cote received a bachelor's degree from Syracuse University, a medical doctorate from the Howard University College of Medicine, and a master's degree in Public Health from Harvard School of Public Health. He has completed residencies and is board certified in both Preventive Medicine and Anatomic Pathology. Dr. Cote began Federal service in 1989 with the CDC's Epidemiology Investigation Service (EIS) and has since continued as an officer in the U.S. Public Health Service Commissioned Corps assigned to a wide variety of positions at CDC, NIH, USDA and FDA. Most recently he served as CDC Chief of Mission in Kigali, Rwanda where he implemented the President's Emergency Plan for AIDS Relief. Dr. Timothy Cote has served as the Director of FDA's Office of Orphan Product Development since September 2007. He has authored or co-authored over 60 publications on infectious and neoplastic disease.

## Steven Groft

Director  
Office of Rare Diseases, National Institutes of Health

Stephen Groft received both his Bachelor of Science (1968) and Doctor of Pharmacy (1979) degrees from Duquesne University, and started his career as a commissioned officer in the United States Public Health Service as a pharmacist in the Indian Health Service. From 1982-1986, he served in the FDA's Office of Orphan Products Development and from 1986-1989 with the Department of Health and Human Services as the Executive Director of the National Commission on Orphan Diseases. In 1991, Dr. Groft, as the first Acting Director, established the Office of Alternative Medicine at the NIH and in 2002 completed an assignment as the Executive Director of the White House Commission on Complementary and Alternative Medicine Policy. As the Director of NIH's Office of Rare Diseases, he has devoted particular attention to working with patient advocacy groups in their efforts to stimulate research for rare diseases, and will oversee a new program focusing on Therapeutics for Rare and Neglected Diseases (TRND), (see article at <http://www.nih.gov/news/health/may2009/nhgri-20.htm>).

## Christy Hanson

Health Development Officer  
Office of Health, Infectious Diseases and Nutrition, USAID

Christy Hanson received her master's in public health from the University of Minnesota and her PhD in international health systems, with a concentration in health economics, from The Johns Hopkins University. She is a senior public health advisor with USAID's infectious disease division. Dr. Hanson has over 15 years' experience in international TB control with support to countries in Africa, Asia, and Latin America through her previous positions with the WHO, World Bank, and PATH. She has published and presented widely on various aspects of TB control. At USAID, Dr. Hanson is the research advisor for TB and a focal point for TB/HIV. She is currently chair of the Stop TB Partnership's Retooling Task Force and has been a member of the Global Fund's technical review panel. Dr. Hanson has also published on the economic burden of neglected tropical diseases (NTDs) and is USAID's technical lead for its NTD Initiative. She manages the Other Public Health Threat element for USAID, which includes containment of antimicrobial resistance, surveillance, and outbreak response for infectious diseases.



### **John Kilama**

Director  
Kilama International Consulting  
Group, LLC (KIGC)

Dr. Kilama was born in Uganda, and received his PhD in Medicinal Chemistry from the University of Arizona in Tucson,

a Pharmacy degree from the University of Kentucky, and a BA in chemistry from Berea College. He is a Founder of the Global Bioscience Development Institute (GBDI) and developed its Biodiversity, Biotechnology and Law curricula used in training professionals in Africa and other developing countries. Dr. Kilama worked for DuPont as a Senior Medicinal Research Chemist, holds several patents, and helped to establish several collaborations between DuPont and institutions in developing countries. Dr. Kilama was on the Board of Directors of the Public Private Partnership for Health (PPPH) Global Forum for Health Research, Geneva, Switzerland, and is currently an Advisor on Global Health of The Children's Hospital of Philadelphia, a Scientific Advisor to the Institute for OneWorld Health and the International Organization for Chemistry in Development (IOCD). Dr. Kilama recently founded KIGC to facilitate management of IPR at institutional and national levels in emerging markets.



### **Mary Moran**

Director, Health Policy Division  
The George Institute for International  
Health

Dr. Moran trained as a medical doctor, later earning a post-graduate degree in international relations and politics at

University of NSW and Monash University, which led her into a career as a diplomat with the Australian Department of Foreign Affairs & Trade. Mary subsequently worked for Medecins Sans Frontieres, initially as Director of the Access to Essential Medicines Campaign in Australia and later as a Europe-based advocate on a range of issues relating to access to medicines for neglected patients. In 2004, she founded a health policy unit at the London School of Economics, and subsequently transferred the unit to The George Institute, Sydney, in 2006, where she continues as Director. Mary has participated in numerous Workings Groups and Committees examining neglected diseases, including the WHO Commission on Intellectual Property Rights, Innovation and Public Health (CIPIH); the Rockefeller Health Innovation Systems in Developing Countries Working Group; the European Union "Priority Medicines for Europe and the World" project; the OECD Expert Working Group in Innovative Financing; and, currently, the WHO Expert Working Group in R&D Financing.



### **Christopher-Paul Milne**

Associate Director  
Tufts University's Center for the Study  
of Drug Development

Formerly a practicing veterinarian, Dr. Milne later attended The Johns Hopkins University where he earned a

master's degree in public health with a concentration in epidemiology and health statistics. For six years, he worked for the New Jersey Department of Health in health risk assessment and emergency response. In 1997, Dr. Milne graduated from the Franklin Pierce Law Center, and soon after joined the Tufts Center for the Study of Drug Development as a Senior Research Fellow in order to address regulatory policy issues. Some of his current research interests are: identifying and classifying factors affecting innovation efficiency and the globalization of R&D; evaluating incentive programs for neglected diseases of the developing world; assessing the impact of regulatory trends; and tracking the progress of new policy and scientific initiatives, such as the Critical Path Initiative and Translational Medicine. Dr. Milne serves as a volunteer on several committees for the Drug Information Association, and is an Honorary Fellow at the University of Edinburgh, and Associate Director of the Tufts CSDD.



### **Melinda Moree**

Interim CEO  
BIO Ventures for Global Health (BVGH)

Dr. Moree received her PhD in Medical Microbiology from the University of Maryland at Baltimore. Until early 2007, Melinda was the Director of the Malaria

Vaccine Initiative (MVI), a public-private-partnership with a mission to accelerate the development of malaria vaccines and to ensure that they are available and accessible to people in developing countries. She oversaw the growth of the program from \$50 million to \$300 million, and a tripling in staff. Most importantly a key milestone event—proof of concept (efficacy) in children in Africa—was achieved under her leadership. Before joining BVGH as Interim CEO in July 2009, she was an American Association for the Advancement of Science (AAAS) Science and Diplomacy fellow at the US Agency for International Development, Principle Investigator on the Malaria Policy Project conducted with the Center for Global Development, a member of the team evaluating the International AIDS Vaccine Initiative, and consulted with the Global Alliance for Vaccines and Immunizations.

### **Mr. Kena Mphonda**

Deputy Ambassador  
Republic of Malawi

Mr. Mphonda joined the Ministry of Foreign Affairs in 1985 and has served in the Departments of Political Affairs, Protocol as well as International Cooperation. He also served in the Ministry of Finance in the Development Aid Division and the Office of the President and Cabinet. Mr. Mphonda was a Hubert Humphrey Fellow in International Relations at American University in Washington D.C., from 1997 to 1998. Mr. Mphonda previously served as a Second Secretary in Bonn, Germany. He joined the Republic of Malawi's Embassy in Washington, D.C. in 2006, having been posted from the Ministry of Foreign Affairs where he was serving as Acting Chief of Protocol. Mr. Mphonda attended the University of Malawi from 1980 to 1984, graduating with a Bachelor of Arts; he is also a graduate of Ohio University at Athens in International Relations in 1990. Kena Mphonda is on the Board of Directors of a non-profit organization called Malawi Biomedicals. It tries to find new and used medical supplies to send to hospitals/clinics in Malawi.



### **Valerio Reggi**

Senior Adviser, Access to Medicines  
Department of Control of Neglected  
Tropical Diseases, World Health  
Organization

Valerio Reggi graduated in 1978 from the University of Milan, Italy, Faculty of Pharmacy, and received his Post-doctoral Degree in Pharmacological Research in 1982 from the Mario Negri Institute for Pharmacological Research in Milan, Italy. He worked in pharmacological research and international cooperation at the Mario Negri Institute until 1986 when he joined Unicef in New York as Programme Manager, Essential Drugs. He joined the World Health Organization in Geneva in 1989 where he has occupied different positions. He is currently Senior Adviser, Access to Medicines for Neglected Tropical Diseases in WHO's Department of Control of Neglected Tropical Diseases.



### **Michael R. Reich**

Taro Takemi Professor of International  
Health Policy  
Harvard School of Public Health.

Dr. Reich received his PhD in political science from Yale University in 1981, and has been a member of the faculty of Harvard University since 1983. Dr. Reich is a leading researcher on global health policy, particularly the political dimensions of public health policy and pharmaceutical policy. He also serves as Director of the Takemi Program in International Health at Harvard. His research activities include the politics of health system reform, access to health technologies in poor countries, and shaping global health policy (see his book with Laura Frost, *Access: How Do Good Health Technologies Get to Poor People in Poor Countries* – free download at [www.accessbook.org](http://www.accessbook.org)). During 2008-2009, he worked with the Japanese government on Japan's global health proposal for the G8 Summit. On sabbatical in 2005-2006, he was a visiting professor at the National Institute of Public Health, in Cuernavaca, Mexico, and has served on the Schistosomiasis Control Initiative Board, Trachoma Expert Committee of the International Trachoma Initiative, and TDR's Scientific and Technical Advisory Committee.



### **David Ridley**

Assistant Professor  
Duke University's Fuqua School of  
Business

David B. Ridley is an Assistant Professor at Duke University's Fuqua School of Business. David is also a graduate of Duke University having earned a doctorate in economics. David's research is concerned with entry and differentiation. In his research on geographic differentiation he examines why a firm might locate near its rival despite the resulting price competition. In his research on product differentiation he examines how 'me-too' drugs (close substitutes for the market leader) compete on price and advertising, and how regulatory policies affect firms' incentives to enter a market. To encourage more entry of drug manufacturers into neglected markets, David, with Henry Grabowski and Jeffrey Moe, proposed a priority review voucher prize for developers of treatments for tropical diseases. The proposal became law in 2007.

9:15 - 10:00 a.m.

## Global Funding of R&D to Treat Neglected Diseases (the G-FINDER Survey)

**Mary Moran**

The George Institute

### BACKGROUND

The G-FINDER is a survey of global investment into Research and Development (R&D) of new products for neglected diseases. (See Moran M, Guzman J, Ropars AL, McDonald A, Jameson N, et al. (2009) Neglected disease research and development: how much are we really spending? (To find the full report, go to [www.thegeorgeinstitute.org/research/health-policy/current-projects/g-find-global-funding-of-innovation-for-neglected-diseases.cfm](http://www.thegeorgeinstitute.org/research/health-policy/current-projects/g-find-global-funding-of-innovation-for-neglected-diseases.cfm) or google "G-FINDER: Global Funding of Innovation for Neglected Diseases." Once on the George Institute page, click on the link in the first paragraph to view the full report.)

In its inaugural year, G-FINDER surveyed 134 funders in 43 countries for their 2007 R&D investment into: 30 neglected diseases; 127 product areas for these diseases, including drugs, vaccines, diagnostics, microbicides, vector control products and platform technologies; and, all types of product-related R&D, including basic research, discovery and preclinical, clinical development, Phase IV and pharmacovigilance studies, and baseline epidemiological studies.

### FINDINGS

**Disease Funding** - Just over \$2.5 billion was spent on neglected disease R&D in 2007. Of this amount, almost 80% went to three diseases: HIV/AIDS (\$1.1 billion or 42.3%), malaria (\$468.4 million; 18.3%) and tuberculosis (\$410.4 million; 16.0%). The remaining neglected diseases and disease groupings each received less than 5% of global funding, including diarrhoeal illnesses (\$113.9 million; 4.4%), the helminth infections (\$51.6 million; 2.0%) and bacterial pneumonia and meningitis (\$32.5 million; 1.3%). Five diseases – leprosy, Buruli ulcer, trachoma, rheumatic fever, and typhoid and paratyphoid fever – received less than \$10 million or 0.4% of total global investment each.

**Funders** - Public and philanthropic funders provided around 90% of global R&D funding for neglected diseases, with the public sector providing \$1.8 billion (69.4%) and philanthropists providing \$538.3 million (21.0%). The US Government represented nearly three-quarters of global public spend (\$1.25 billion or 70.4%), while European governments and the European Commission collectively provided \$384.9 million (21.7%). Two funders made up 95% of total philanthropic spend, these being the Bill & Melinda Gates Foundation (\$452.1 million or 84.0%) and the Wellcome Trust (\$60 million or 11.1%). There was a marked concentration of funders, with

two organizations – the US National Institutes of Health (NIH) and the Bill & Melinda Gates Foundation – together providing 59.3% of the global total. Over 80% of total global funding was provided by only 12 organisations. Pharmaceutical industry funding was aggregated for confidentiality reasons. Collectively, the private sector contributed 9.1% (\$231.9 million) of global funding, making this group the third largest source of investment after the NIH and the Bill & Melinda Gates Foundation. This contribution refers only to industry's own investments, excluding funding provided by Product Development Partnerships (PDPs) or others to industry programmes.

**Funding Flows** - Around 20% of global funding was invested by public institutions and private companies into internal programmes. The remaining 80% was granted by funders to external organisations either directly or via intermediary organisations and PDPs. Overall, intermediary organisations and PDPs managed nearly one-quarter of global neglected disease product investments in 2007, with a high proportion (nearly one-third) of funder grants being routed through them.

**DALYs** - Intuitively, there is a sense that the highest 'health return on investment' would result from investing in the highest burden diseases, as measured by DALYs (Disability Adjusted Life Years). In practice, the reality is far more complex. The likely health return on a given neglected disease R&D investment depends on the potential health impact of that investment against the cost of the investment, discounted for risk. The potential health impact in turn depends on the severity of R&D need (of which DALYs and severity of product shortfall are the two main components) and the severity of underfunding in the selected area. Cost will depend on the type of products needed and the degree of advancement of the global research portfolio. This cost/benefit ratio must then be discounted for risk, which will chiefly depend on the state of science and technology in the area of investment under consideration, as well as the intrinsic risks of pharmaceutical product development. DALYs act as a multiplier of the likely health impact of a new product in a given area. However, they cannot indicate how much investment is needed to create that new product. This is because cost and risk relate to the state of science and the type of R&D needed rather than to the disease or the number of people affected. Funders will weigh up these factors based on their own agendas, preferences, risk appetite, budgetary constraints and political time horizons. However, the G-FINDER data can support funders by identifying where investment is lacking and where additional funding can potentially have a high impact.

### CONCLUSIONS

An overview of the G-FINDER data confirmed that there were marked gaps not only in terms of funders and diseases (as noted above) but also in terms of products. The lion's share of global investment went to R&D for drugs and vaccines, with very little dedicated to diagnostics. Meanwhile,



platform technologies (e.g. adjuvants, diagnostic platforms and delivery devices, which are not disease-specific) received only 0.4% of global funding. These marked variations suggest that factors beyond science, technology and opportunity were playing a role. The participation of many organisations and countries in the development of new neglected disease products is a remarkable and welcome change from past decades of inertia and neglect. However, a broadening of funding efforts so that all who are able to contribute do so, and all diseases receive the attention they deserve, would lead to a dramatically positive impact on the health of developing country patients afflicted with these diseases. This is more important than ever in tough economic times if we are to ensure that those most in need do not end up paying the highest price.

10:00 - 10:30 a.m.

## Progress in Neglected Disease Drug Development

**Joshua Cohen**

Tufts Center for the Study of Drug Development  
Tufts University

### BACKGROUND

Neglected diseases are infectious diseases that primarily, though not exclusively, affect vulnerable populations in developing countries where poor sanitation and lack of access to healthcare foster disease transmission and vector proliferation. These diseases, which include malaria, tuberculosis, diarrheal diseases, and kinetoplastids such as leishmaniasis, cause 35,000 deaths per day in the developing world along with significant morbidity (Fehr et al. 2006). There is great interest in the public health community in developing new products to treat or prevent these diseases. However, in a widely cited 2002 study, Trouiller et al. reported that of 1393 new chemical entities (NCEs) marketed between 1975 and 1999, only 16 targeted “tropical diseases” and tuberculosis (Trouiller et al. 2002). Furthermore, Trouiller et al. found that in 1999 merely \$70 million was invested in drug R&D for malaria, tuberculosis, leishmaniasis, and African trypanosomiasis combined.[4] Trouiller et al.’s call to action suggested inadequate funding was responsible for relatively few new approvals targeting neglected diseases between 1975 and 1999.

### FINDINGS

**Updated Estimate for 1975-1999** - Since Trouiller et al.’s publication, significantly more resources have been allocated to the development of products targeting neglected diseases. Nevertheless, policymakers are unsure whether these resources are being invested effectively. One way of

approaching this question is to examine whether funding has resulted in an increase in new approvals, and analyze adoption of these approvals, e.g., inclusion in the World Health Organization’s (WHO) Essential Drug List (EDL). Our paper (Cohen, Dibner & Wilson 2009):

- Revisits numbers of approved drugs targeting “tropical diseases” and tuberculosis previously published by Trouiller et al.;
- Measures progress in neglected disease product approvals since 2000;
- Explains how increased numbers of approvals are a necessary but insufficient condition to improving access.

Upon recount we found that 46 new drugs were approved between 1975 and 1999 targeting neglected diseases with a total of 56 indications. Of these, 6 were for pediatric HIV, 7 for malaria, 12 for tuberculosis, three for bacterial pneumonia and meningitis, 2 new drugs and 4 new indications for diarrheal diseases, two for kinetoplastids, 9 new drugs and 16 new indications for helminths, two for leprosy and one each for trachoma, rheumatic fever, and typhoid fever. No new drugs were approved for Buruli ulcer (though one new indication) and Dengue fever. Of the 56 drug indications approved for marketing, 46 (82%) were added to the EDL. And of the 46 new drug approvals, 39 (85%) were placed on the WHO’s EDL.

Independent of the broader G-Finder definition we adopted as well as our inclusion of new indications, the Trouiller et al. figure of 16 appears to have undercounted the total number of drugs approved for “tropical diseases” and tuberculosis between 1975 and 1999; namely, 36.

**New Estimate for 2000-2009** - Between 2000 and 2008, 26 drugs for neglected diseases were marketed with a total of 26 indications. Of these, WHO placed 12 (46%) on the 2007 EDL. The greatest number of approvals occurred in malaria with 11 new drugs being marketed. An additional 10 new HIV/AIDS drugs were granted pediatric labeling; one new drug and two vaccines for diarrheal diseases; one vaccine was approved against bacterial meningitis, and one new drug was approved for kinetoplastids. No other disease category had any new drugs approved in the last 9 years.

The percentage of approved neglected disease products sponsored by the private pharmaceutical industry dropped from 89% to 46% between the two time periods, while the percentage sponsored by private-public partnerships increased from 9% to 46%.

**Implications of Findings** - It is important to note that many of the product development efforts that began in 2000-2009 have not (yet) resulted in new product approvals, given the (variable) length of time between initial funding of R&D and registration. Indeed, collecting data on products in the clinical development pipeline, we see

promising signs, though certain therapeutic areas continue to be neglected.

There has been uneven progress in neglected disease drug development, with malaria benefitting most from increased funding. While tuberculosis has received similar funding to malaria, not a single new tuberculosis drug has been approved in the last nine years. Likewise, despite HIV/AIDS R&D towards applications specific to the developing world totaling \$1.8 billion in 2007, of which 64% went to vaccine development and 18% to microbicides, no vaccines or microbicides have been approved. Finally, not a single new drug has been approved in the last 9 years in disease categories that include Buruli ulcer, Dengue fever, trachoma, rheumatic fever, or typhoid and paratyphoid fevers.

The obvious implication of uneven progress is that funding should be better targeted, particularly with regard to neglected diseases that have hitherto received scant attention. Moreover, policymakers should focus on the larger question of access. Here, we are referring to the issue of EDL listing, but also improvements in health infrastructure and affordability.

Inclusion on the EDL does not guarantee access, but may act as a lever to increase access. The EDL recommends what it deems to be the most essential, yet cost-effective drugs available. As such, it forms the basis for public health policy in many developing nations. Comparatively low percentages of new approvals on the list could be due to lag time as the WHO deliberates following each new approval, but is also a result of the higher cost of certain newer drugs. Needless to say, non-admittance to the EDL can serve as an access barrier, with possibly deleterious health outcomes.

Besides EDL listing there is the larger question of access to medicines, which involves more than just getting new drugs or indications approved and placed on formularies. Access also comprises availability, affordability and adoption (Frost & Reich 2009). Availability refers to “the logistics of making, ordering, shipping, storing, distributing and delivering a new health technology to ensure it reaches the hands (or mouth) of the user” (Frost & Reich 2009). This implies that even if a product travels the drug development route successfully, reaches market, and is recommended for use by WHO, there is still the question of how it is to be distributed to the people who need it. Affordability encompasses both the individual patient’s ability to pay and that of governments and other payers. This is less of a concern for medications such as ivermectin, which Merck donates. But, it may be an acute issue for drugs such as ACT therapies (Economist 15 May 2008). Lastly, adoption runs the gamut from a product’s recommendation by international agencies like WHO to its acceptance by local policymakers in developing countries, to patients as well as healthcare providers, some of whom may have misgivings about taking certain products (Frost & Reich 2009).

In this context, consider, for example, the anti-helminths. Given that there are already 9 drugs that are effective at treating helminths, it appears that the need for development of new anti-helminths is less critical than the need for improved access. Hence, the helminths appear to be neglected diseases not because of a drug deficit but because of limited effective means of getting those drugs to the people who need them.

## CONCLUSIONS

In sum, funding that targets neglected disease R&D is highly concentrated, with significant funding flowing into HIV/AIDS, malaria and tuberculosis. Progress is lopsided, with marked strides in the area of malaria research, yet few advances in others (Dentzer 2009). This is to be expected given the disparity among the diseases themselves, in terms of government priority-setting, overall resource allocation, and the peculiar scientific challenges each disease presents. Moreover, a balanced, comprehensive approach to address the neglected disease problem will involve not only drug development but also attention paid to health infrastructure, affordability, and capacity-building to improve access.

12:00 - 1:00 p.m.

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## Challenges in Improving Access to Medicines for Neglected Diseases

**Michael R. Reich**

Taro Takemi Professor of International Health Policy  
Harvard University School of Public Health

## BACKGROUND

Many people in developing countries lack access to health technologies, even basic ones. These technologies include life-saving medicines, such as antiretrovirals for HIV/AIDS, as well as life-enhancing medicines, such as antiasthma medications that help stop asthma attacks and improve breathing. Access is also limited to many other health products such as vaccines that can prevent debilitating diseases, diagnostics for infectious and chronic diseases, preventive technologies like insecticide-treated bednets, and various kinds of contraceptives from condoms to pills to injectables. In 1999, the World Health Organization estimated that since the mid-1980s, around 1.7 billion people—approximately one third of the world’s population in 1999—did not have regular access to essential medicines and vaccines.

In recent years progress has occurred in placing access to medicines for neglected diseases on the global policy agenda, but enormous problems persist in closing the access gap for health technologies. The most contentious debates have focused on access to drugs and vaccines, while similar

problems exist for other health technologies. Access to diagnostics, for example, has been relatively unexplored in policy debates. And the focus on certain types of access barriers (especially pricing and patents) has tended to obscure other important obstacles to access, such as distribution, delivery, and adoption problems. Access problems are especially common for the treatments for neglected diseases.

## FINDINGS

This talk will present the key lessons of a recent book, written with Laura Frost, on *Access: How Do Good Health Technologies Get to Poor People in Poor Countries?* (Cambridge, Harvard University Press, 2008). The research and the publication were sponsored by the Bill & Melinda Gates Foundation. The full text of the book is downloadable for free at [www.accessbook.org](http://www.accessbook.org).

**Case Studies** - This book provides a comprehensive view of the challenges of creating access, based on the histories of six health technologies: praziquantel to treat schistosomiasis, hepatitis B vaccine, the Norplant contraceptive, malaria rapid diagnostic tests, vaccine vial monitors, and the female condom. Four criteria guided our selection of the case studies. We chose cases that: 1) include different types of health technologies; 2) reflect a range of health problems; 3) span different phases of access; and 4) include examples that have been successful as well as those that have encountered obstacles and faltered. Our approach in these case studies draws from anthropological research that

traces the “life-cycles” or “biographies” of medicines from production to end-user and public health case study research on barriers to technology access. For each case study, we analyzed the social, economic, political, and cultural processes that shaped access to the health technology in developing countries. We followed the technology’s flow through different phases of access, identified barriers, and looked for measures that create access. Our analysis of access was based on a framework with four components: Architecture, Affordability, Availability, and Adoption (see the figure below).

**Lessons Learned** - This talk presents seven lessons about the bottlenecks to access and the strategies to overcome them. The seven findings are the following:

Finding #1: Developing a safe and effective technology is necessary but not sufficient for ensuring technology access and health improvement.

Finding #2: Creating access depends on effective product advocacy by a product champion to construct and manage the architecture of access.

Finding #3: Product champions need to create expert consensus about their health technology in international technical agencies and global health policy communities.

Finding #4: End-user adoption of the technology is an essential but often overlooked component of the entire process of creating access.

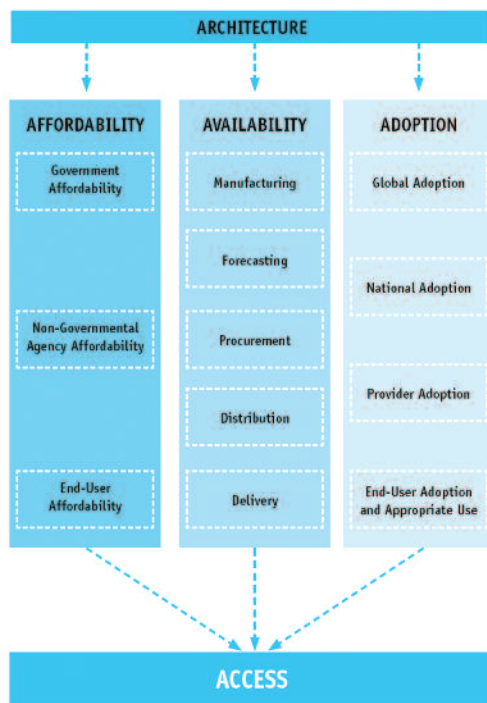
Finding #5: The cost of health technologies and related services is a key barrier to access. Strategies to expand access must address affordability.

Finding #6: Supply-side strategies that assure the availability of a technology are needed to help expand access for health technologies in developing countries.

Finding #7: Limited health infrastructure in many developing countries impedes technology access, making it important to invest in health system strengthening to ensure sustained access.

## CONCLUSIONS

Our findings have important implications for initiatives to develop medicines for neglected diseases. These efforts are seeking to introduce new health products for poor countries (through such groups as the Global Alliance for TB Drug Development, the Foundation for Innovative New Diagnostics, the International AIDS Vaccine Initiative, and OneWorld Health). Once developers demonstrate that a product can improve the health of poor people in poor countries, they confront a series of new problems related to creating access.



## About Tufts Center for the Study of Drug Development

Established in 1976, the Tufts Center for the Study of Drug Development at Tufts University provides strategic information to help drug developers, regulators, and policy makers improve the quality and efficiency of pharmaceutical development, review, and utilization. The Tufts Center conducts a wide range of in-depth analyses on pharmaceutical issues and hosts symposia, workshops, and public forums on related topics, and publishes the Tufts CSDD Impact Report, a bi-monthly newsletter providing analysis and insight into critical drug development issues.



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Following the forum, presentations and related materials  
will be available at: <http://csdd.tufts.edu/negd>.