



Designating Additions to the Current List of Tropical Diseases in the FDA Amendments Act

FDA Public Hearing
Washington, DC
December 12, 2008

Wendy Taylor
Founder, Vice President Strategy and Operations
BIO Ventures for Global Health

BVGH PRV Working Group

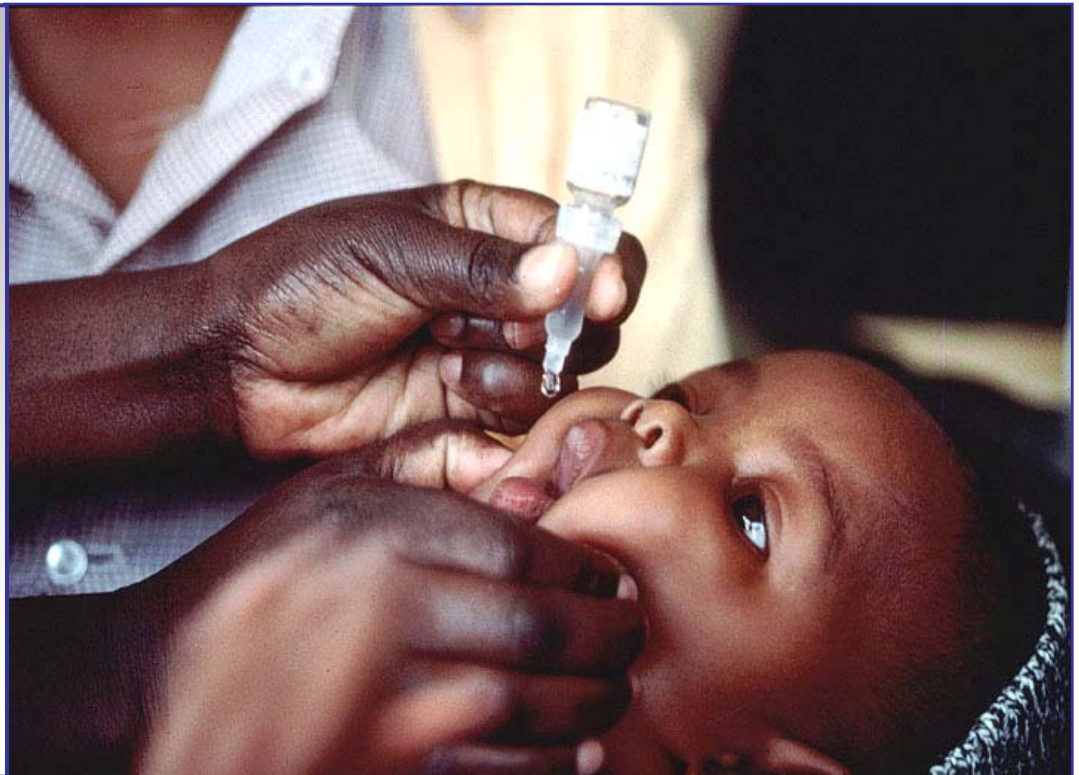
- **Tadaka (Tachi) Yamada, MD**
*President, Global Health Program
Bill & Melinda Gates Foundation*
- **Neil D. McDonnell, Pharm. D.**
*Chief of Staff, Global Health Program
Bill & Melinda Gates Foundation*
- **Hannah E. Kettler, PhD**
*Senior Program Officer; Global Health
Initiatives; Bill & Melinda Gates Foundation*
- **Nancy L. Buc**
Partner; Buc & Beardsley
- **Chris Hentschel, PhD**
*President and CEO; Medicines for Malaria
Venture*
- **Robert B. Chess**
Chairman and CEO; OPX Biotechnologies
- **Mark B. Feinberg, MD, PhD, FACP**
*Vice President for Medical Affairs and
Policy, Merck Vaccines and Infectious
Diseases; Merck & Co.*
- **Paul Antony, MD**
*Chief Medical Officer; Pharmaceutical
Researcher and Manufacturers of America*
- **Henry Grabowski**
Duke University
- **Jeffrey Moe**
Professor, Duke University
- **George Scangos**
President and CEO, Exelixis
- **Leighton Read**
Partner, Alloy Ventures
- **Mel Spiegelman**
President and CEO, TB Alliance
- **Lydia Pan**
*Director Science Policy and Public Affairs,
Pfizer*
- **Andrew Emmett**
Biotechnology Industry Organization
- **Christopher D. Earl, PhD**
*President and CEO; BIO Ventures for
Global Health*
- **Wendy Taylor**
*Founder & Vice President of Strategy and
Operations; BIO Ventures for Global Health*

BVGH Mission

BIO Ventures for Global Health, a non-profit organization, harnesses the resources of the biotechnology industry to create new medicines for neglected diseases of the developing world.

Our mission:

- To break down barriers that hinder product innovation for global health
- To catalyze R&D investment through new market-based solutions.



Focus of Presentation

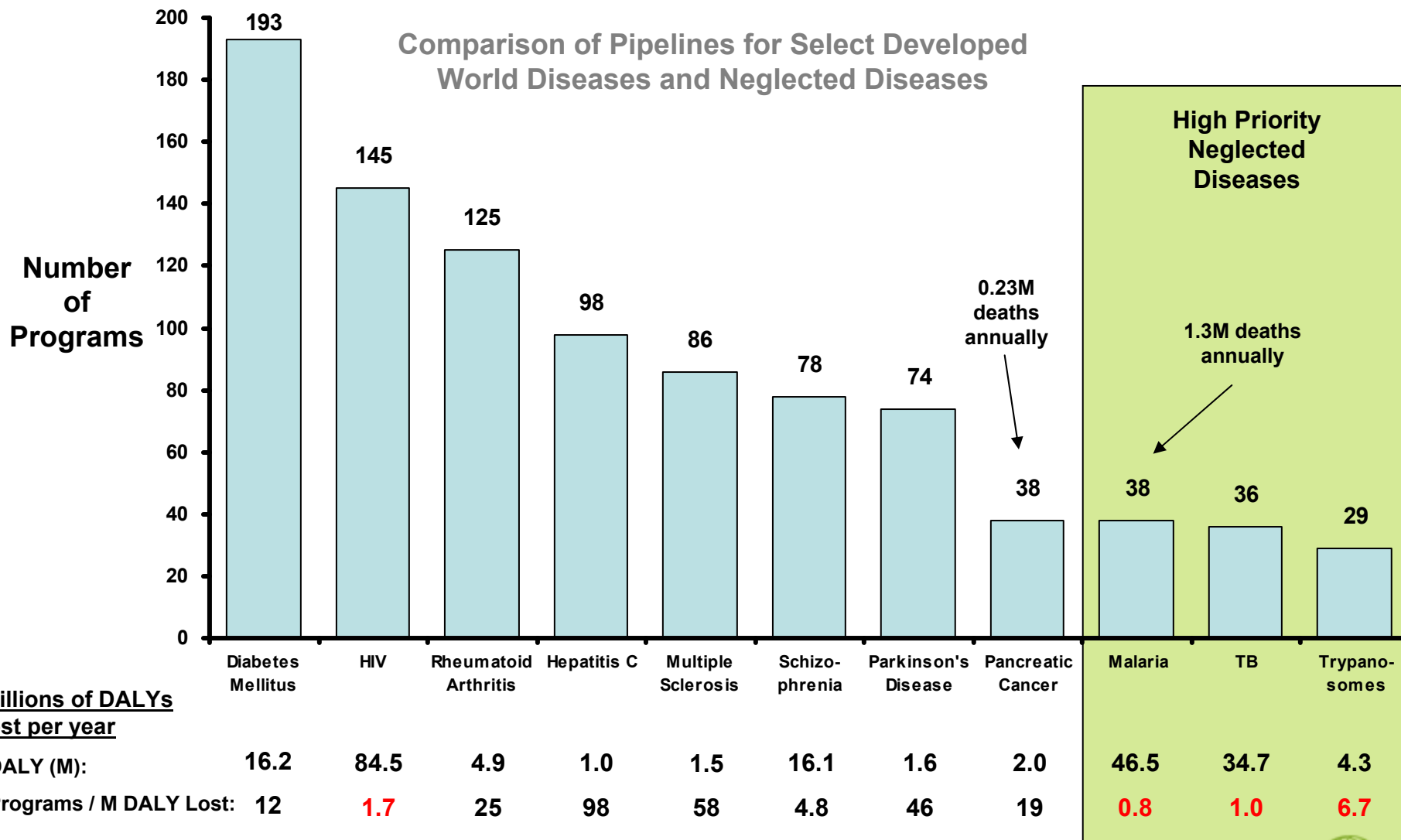
- **Expansion of diseases covered**
 - **What diseases should be added**
 - **Framework for evaluating the statutory criteria**
- **Major issue for implementation**

The Problem

- **Developing world needs new medical products to prevent and treat infectious diseases**
 - Over 10M deaths in developing countries from top 5 infectious diseases
 - Millions of others suffer from debilitating NTDs
- **New innovation needed**
 - Existing medicines inadequate
 - Current pipelines will not fully address needs

Pipeline Insufficient to Meet Need

Comparison of Pipelines for Select Developed World Diseases and Neglected Diseases



Importance of Priority Review Vouchers (PRVs)

- **Congress recognized exceptional humanitarian interest in addressing health needs of world's poorest populations**
- **Opportunity to incentivize development of drugs and vaccines that otherwise wouldn't be developed**
- **If program works well, can reduce burden of disease for millions of people**

BVGH Role

- **Helping companies + investors clarify the opportunity to encourage new investment**
- **Coordinating stakeholder input**
- **Launched PRV website**
 - **Comprehensive source of info on PRVs**
 - **Will track vouchers**
 - **Will measure impact of program**

www.prvinfo.org

16 Diseases Covered

- **Tuberculosis**
- **Malaria**
- **Blinding trachoma**
- **Buruli ulcer**
- **Cholera**
- **Dengue**
- **Guineaworm disease**
- **Fascioliasis**
- **Human African trypanosomiasis (African Sleeping Sickness)**
- **Leishmaniasis**
- **Leprosy**
- **Lymphatic filariasis**
- **Onchocerciasis**
- **Schistosomiasis**
- **Soil transmitted helminthiasis**
- **Yaws**

→ **FDA can expand list of diseases via new regulations**

Expansion of Program to Other NTDs

- **FDA can expand the list of PRV-eligible diseases to any infectious disease:**
 - For which there is no significant market in developed nations and
 - That disproportionately affects poor and marginalized populations
- **Our approach to evaluating criteria**
 - Easily implementable
 - Sufficiently flexible to allow for lack of reliable data
 - Narrow enough to keep out those not intended by Congress

Evaluation of Other Infectious Diseases

Diseases to Add

- **Chagas' disease**

Disproportionately affects poor and marginalized populations and rarely found endemic in developed countries

Diseases with sufficient markets

- **Influenza**
- **Hepatitis C**

High incidence in developing world, but also have “sufficient markets” in developed world

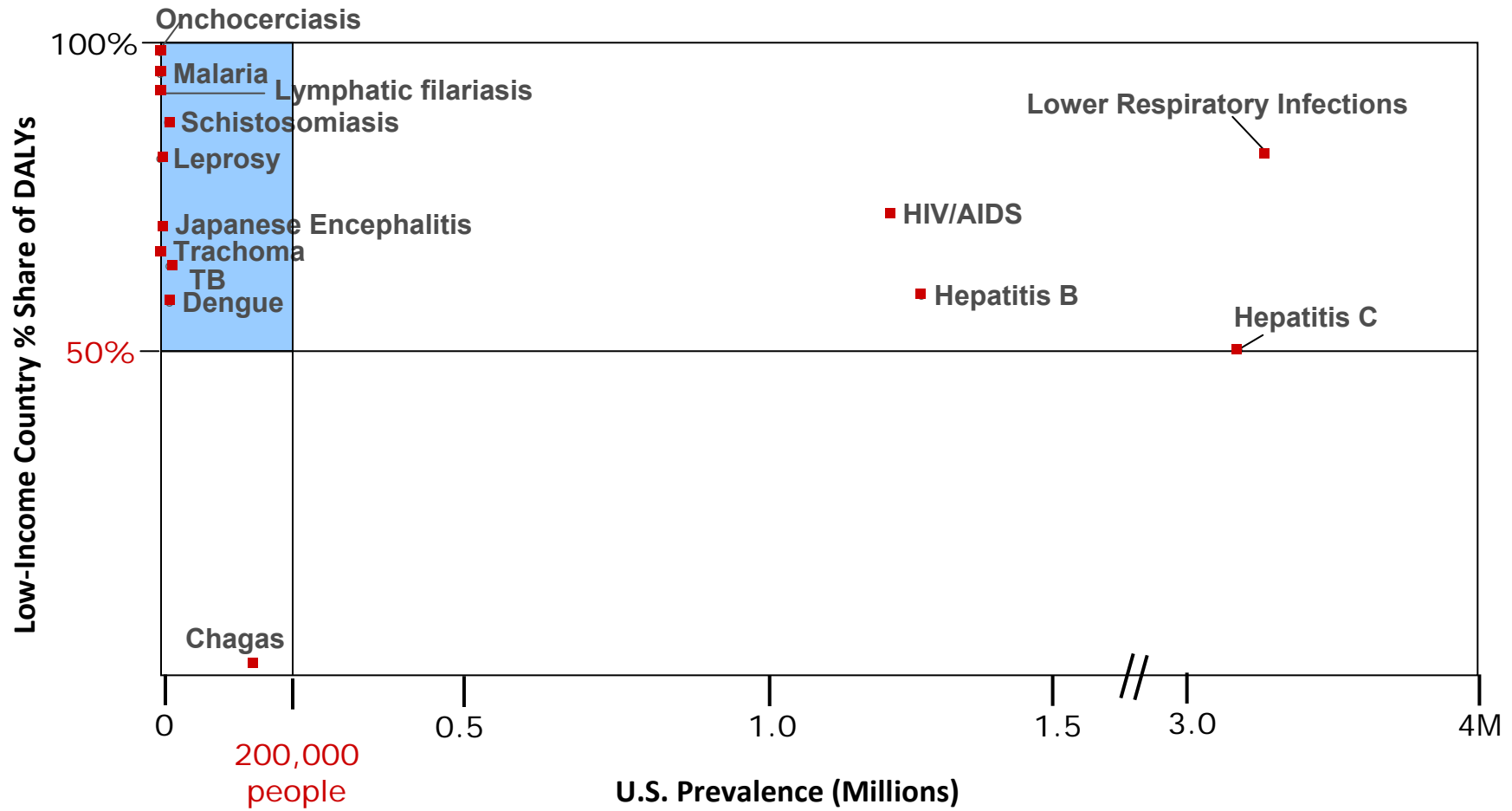
Additional Diseases/Pathogens to Consider

- **Japanese Encephalitis**
- **Enterotoxigenic e-coli (ETEC)**
- **Shigella**
- **Typhoid**

Recommendations for Adding Diseases

- **Allow for flexibility in how the statutory criteria are defined given the lack of reliable data**
 - No universally-agreed definition for either criterion
 - Best available data may be indirect measures
 - May need to consider different measures depending on the disease and where it occurs
- **Take into account qualitative, but scientifically informed, measures**
- **Form an Advisory Committee of tropical disease specialists to make recommendations**
- **Once a disease has been added, it should not be removed**

Low Income Country Share of Burden vs. U.S. Prevalence, 2005



General: Key Issue for Implementation

- **Developers need transparency and clarity when deciding whether to undertake risky R&D**
- **Suggest that FDA provide sponsors an early determination on eligibility for priority review**
 - **Must qualify for priority review to get a voucher**
 - **When sponsor notified has significant implications for program's success**
 - **If determination not made until after submission of the application, may limit program's impact**
 - **Without certainty, sponsor unlikely to take the risk**

BIO Ventures for Global Health

*Building biotech solutions
for diseases of the developing world*

www.bvgh.org