

## **Assistance for Neglected Diseases: A Time for Partnership**

There were 8.8 million new tuberculosis (TB) cases in 2005, 80% of which were in just 22 countries. Of those infected, up to three million individuals, most in less developed countries, will die from TB in one year alone. Two billion people, one third of the world, test positive for TB bacteria. That means there are more people in the world today with TB than there were in 1950. If not treated, each person with active TB can infect on average 10 to 15 people a year.<sup>1</sup>

Every year, 515 million people contract malaria, and up to three million of them, the majority of whom are young children in Sub-Saharan Africa, die from it. Malaria, one of the most common infectious diseases in the world, is commonly associated with poverty, but is also a cause of poverty and a major hindrance to economic development. These statistics are staggering, yet they do not even touch the millions more who suffer daily from preventable and treatable diseases like river blindness, cholera, dengue fever, and human African trypanosomiasis (sleeping sickness).

### **Neglected Diseases: Devastating Effects, Dearth of Therapies**

Nearly one out of every six people worldwide is affected by a neglected disease, diseases, including those listed above, for which the developed world has dedicated few, if any, resources to the development of new preventions, therapies or vaccines. In fact, only 1% of the 1,400 drugs registered with the Food and Drug Administration (FDA) between 1975 and 1999 treat these neglected diseases. This disparity is tragic—and it underscores the fundamental need for greater involvement from companies that can and should develop better treatments for diseases that plague people in the developing world.

In some cases, the treatments and medications for these diseases exist, but the affected populations face barriers to access. In other cases, the clinical knowledge and research capacity may exist for the creation of new therapies, preventions and diagnostic tools, but varied forces present significant hurdles to furthering research and development of new products in this space.

Despite these difficulties, effective solutions are within our reach. Biopharmaceutical companies, nongovernmental organizations (NGOs), and humanitarian aid groups need to join forces to advocate innovative, practical solutions that can transcend current research and development hurdles. Together, we can be a force for change.

### **A Partnership for the Pursuit of Innovation and Access**

The Genzyme Corporation, a Cambridge, MA biotechnology company focused on cures for rare, debilitating diseases, believes in change. In 2006, Genzyme created the Humanitarian Assistance for Neglected Diseases (HAND) initiative to assist in the

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<sup>1</sup> WHO, Top Ten Facts on Tuberculosis  
<http://www.who.int/features/factfiles/tuberculosis/en/index.html>

development of treatments for neglected diseases. Yet with the initial successes of HAND, Genzyme quickly realized that if the resources of one company could reduce the devastating impact of infectious disease in the developing world, the resources of many could bring tremendous relief.

With broad participation from a diverse range of groups—drug, vaccine, and device manufacturers, non governmental organizations, humanitarian assistance groups—the talents and skills of many could foster a shared goal of incentivizing cures and treatments for people with neglected diseases. Genzyme believes that society has a responsibility to do more, and reaching out for other partners is the first step in the process.

The working group we envision will come together to develop practical and innovative policies to ensure that the promise of breakthrough treatments can become a reality for individuals suffering from neglected diseases. Companies in the developed world can and should utilize their vast resources to develop new therapies and speed their delivery to affected patients in impoverished countries. Keeping in mind the dual concerns of access and development, the group should work to identify problems and propose solutions and incentives to bring to leaders in Congress, both this year and next year.

What we need to accomplish these goals is your organization's participation and help.

### **Building on Previous Success**

Taking a cue from the sweeping success of the Orphan Drug Act adopted in 1988, Congress enacted a “priority review voucher” program as part of last years’ Food and Drug Administration Amendments Act (FDAAA). The Orphan Drug Act was aimed at encouraging companies to invest in the research and development of drugs for diseases which affect fewer than 200,000 patients in the U.S. Similarly, Congress hopes that that “priority review voucher” will encourage research and development for treatments of neglected diseases. A transferable priority review may be but one step to spur on research and development for neglected diseases.

We believe there are other incentives that can leverage the knowledge and resources of both innovator companies and humanitarian groups to effectively address neglected diseases, and we hope you will consider working with us to that end.