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Treatment Action Group

Open Letter to the U.S. Senate HELP Committee Leadership:
Suggestions to Fix the FDA PRV for Neglected Diseases

The Honorable Lamar Alexander, Chairman
The Honorable Patty Murray, Ranking Member
Committee on Health, Education, Labor and Pensions
United States Senate

November 17, 2015

Dear Chairman Alexander and Ranking Member Murray:

We are writing to ask for your support, as the Chairman and Ranking Member of the Senate Committee on Health, Education, Labor and Pensions (HELP), to ensure that new research and development (R&D) for neglected diseases is effectively incentivized, and that any new products brought to market are made accessible and affordable to those who need them. We would specifically like to call your attention to the need for amendments to the Food and Drug Administration (FDA) Priority Review Voucher (PRV) program for neglected diseases.

As several of the most recent and ongoing global health emergencies have reminded the world, the need for well-functioning incentives for R&D for neglected diseases is today more urgent than ever. Yet, despite representing more than 10% of the global disease burden, only 4% of new drugs and vaccines approved across the world were indicated for neglected diseases between 2000 and 2011.¹

In July 2014, Médecins Sans Frontières/Doctors Without Borders (MSF), the Drugs for Neglected Diseases initiative (DNDi) and the Global Alliance for TB Drug Development (TB Alliance) sent a letter to all the members of the HELP Committee raising several concerns with the design of the FDA PRV program for neglected diseases and proposed legislative amendments.²

Since then, the increasing monetary value of PRVs has been established through sales, with the most recent voucher being sold for \$350 million in August 2015.³ Additionally, several of the concerns we raised in our 2014 letter have been addressed. We welcome amendments made in 2014 that lift limits on transfers of the PRV for neglected diseases, increasing the potential appeal and value to prospective PRV recipients. We also welcome the extension of the list of eligible neglected diseases, including for Ebola and Chagas disease.

¹ Pedrique B, et al. The drug and vaccine landscape for neglected diseases (2000-11): a systematic assessment. *Lancet* 2013, 1(6): [http://www.thelancet.com/journals/langlo/article/PIIS2214-109X\(13\)70078-0/fulltext](http://www.thelancet.com/journals/langlo/article/PIIS2214-109X(13)70078-0/fulltext).

² Letter sent July 30, 2014. Copies available upon request.

³ See: <http://www.wsj.com/articles/united-therapeutics-sells-priority-review-voucher-to-abbvie-for-350-million-1439981104?alg=y>.

However, the lack of requirements for a product to be novel or to be made available to and affordable for those whom the product is designed to treat or protect are two critical flaws in the design of the program that remain unaddressed. Now, as we understand, the Committee is preparing to release draft biomedical R&D legislation. We hope that the Committee will use this opportunity to fix the neglected disease FDA PRV program to ensure that neglected disease medical products, including treatments and vaccines, are appropriately incentivized and are accessible to the patients and health care providers who urgently need them.

There are two key amendments to the PRV program for neglected diseases that we strongly recommend:

1. **The PRV program should have a novelty requirement.** A PRV for neglected diseases can still be awarded even when new R&D investments have not been made by the entity receiving the award or the medical product awarded a PRV for neglected diseases is not new.

The PRV rewards successful FDA registration of drugs for select neglected diseases that have not been registered in the U.S., even if that drug has already been in use in other countries for years. Two of the three FDA PRVs for neglected diseases, awarded to Knight Therapeutics and Novartis for products for treatment of leishmaniasis (miltefosine)⁴ and malaria (artemether-lumefantrine) respectively, were for drugs already in use for a long time in other countries. This has resulted in the granting of PRVs, but not in new investment in R&D. A PRV should only be awarded to products that are truly new, or that are registered with the FDA in a timely manner after initial registration in disease-endemic countries.

2. **The PRV program should require an access strategy.** The PRV program for neglected diseases does not include any mechanism to ensure patients, governments and health care providers will have affordable and appropriate access to products for which a PRV has been awarded.

Critically, the PRV program for neglected diseases does not ensure that the qualifying products will be accessible and affordable to patients in need.⁵ PRV recipients are not even required to market a product that earns a PRV. Additionally, products that are marketed do not need to be priced affordably. For example, in the case of miltefosine, health care providers like MSF, R&D organizations like DNDi, governments and others are still struggling to access this product at an affordable price – or in some cases to access it at all. A PRV should only be awarded to companies who commit to serious efforts to make the PRV-earning neglected disease product available and accessible to patients in disease-endemic countries, whom the PRV program is intended to benefit.

There are a number of simple legislative amendments that could help to remedy the functioning of the PRV for neglected diseases, based on existing law for the rare pediatric disease PRV program or proposed legislation for PRV programs. We have discussed these proposals with many HELP Committee member offices and with HELP Committee staff, and we hope that solutions will be incorporated into the forthcoming Senate biomedical R&D legislation.

As organizations working to develop and provide access to neglected disease treatments and vaccines, we see every day the need for more effective strategies to incentivize needs-driven R&D for neglected diseases, including appropriate rewards for investments.⁶ Improvements to the PRV program will be one important step toward broader changes that are urgently needed to ensure the R&D system delivers appropriate and affordable health technologies for those who need them. We therefore hope that you will consider not only leading the Senate HELP Committee in amending the PRV program for neglected diseases, but also in considering the potential creation of additional mechanisms to ensure that R&D for neglected diseases is successfully and appropriately incentivized, and that all patients in need can benefit from the fruits of biomedical innovation.

⁴ Doshi P. US incentive scheme for neglected diseases: a good idea gone wrong? *BMJ* 2014; 349:g4665 <http://www.bmj.com/content/349/bmj.g4665>.

⁵ See, for example: <http://www.msfacecess.org/about-us/media-room/press-releases/patient-access-miltefosine-developing-countries-not-secure>, <http://blogs.plos.org/speakingofmedicine/2015/01/20/fda-voucher-leishmaniasis-treatment-can-patients-companies-win/> and <https://www.msfacecess.org/content/ready-set-slow-down>.

⁶ 2008 WHO Global Strategy and Plan of Action on Public Health, Innovation and Intellectual Property (WHA61.21) and 2012 Report of the WHO Consultative Expert Working Group on Research and Development: Financing and Coordination (CEWG report), see: <http://www.who.int/phi/en/>.

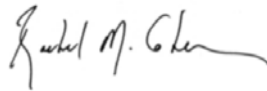
Sincerely,



Dr. Atul Malhotra
President
American Thoracic Society



Dr. Bernard Pécoul
Executive Director
Drugs for Neglected Diseases *initiative*



Rachel M. Cohen
Regional Executive Director
Drugs for Neglected Diseases *initiative*,
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Dr. Mel Spigelman
President and Chief Executive Officer
Global Alliance for TB Drug Development



Christine Lubinski
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Jason Cone
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Dr. Manica Balasegaram
Executive Director, Access Campaign
Médecins Sans Frontières



Dr. Peter J. Hotez
President
Sabin Vaccine Institute



Mark Harrington
Executive Director
Treatment Action Group

The American Thoracic Society (ATS), a 15,000 member international multi-disciplinary society, improves global health by advancing research, patient care, and public health in pulmonary disease, critical illness and sleep disorders. Founded in 1905 to combat TB, the ATS has grown to tackle asthma, COPD, lung cancer, sepsis, acute respiratory distress, and sleep apnea, among other diseases. For more information please visit <http://www.thoracic.org/>.

The Drugs for Neglected Diseases *initiative* (DND*i*) is an international not-for-profit research and development (R&D) organization that discovers and develops new, improved, and affordable medicines for neglected diseases afflicting millions of the world's poorest and most vulnerable people. DND*i* accomplishes its work through innovative, collaborative partnerships with public sector research institutions, particularly in disease-endemic countries, pharmaceutical and biotechnology companies, academia, non-governmental organizations, and governments worldwide. For more information please visit <http://www.dndi.org/>.

The Global Alliance for TB Drug Development is a non-profit organization dedicated to the discovery and development of new, faster-acting and affordable tuberculosis medicines. For more information please visit <http://www.tballiance.org/>.

The Center for Global Health Policy, established by the Infectious Diseases Society of America's Education & Research Foundation in 2008, supports and promotes U.S. efforts to combat HIV/AIDS and tuberculosis around the world. The Center provides scientific and policy information to U.S. policymakers, federal agencies, nongovernmental organizations and the news media, linking decision-makers to the latest evidence-based input and guidance from physician/scientists and other professionals from both developing and developed countries. For more information please visit www.idsaglobalhealth.org.

Médecins Sans Frontières/Doctors Without Borders (MSF) is an independent international medical humanitarian organization that delivers medical care to people affected by armed conflicts, epidemics, natural disasters and exclusion from healthcare in nearly 70 countries. In order to fulfill its mission, MSF needs access to affordable medicines to treat a range of medical conditions, including neglected diseases, for which new treatments are urgently needed. For more information please visit <http://www.doctorswithoutborders.org/>.

Sabin Vaccine Institute (Sabin) is a non-profit, 501(c)(3) organization of scientists, researchers, and advocates dedicated to reducing needless human suffering caused by vaccine preventable and neglected tropical diseases. Sabin works with governments, leading public and private organizations, and academic institutions to provide solutions for some of the world's most pervasive health challenges. Since its founding in 1993 in honor of the oral polio vaccine developer, Dr. Albert B. Sabin, the Institute has been at the forefront of efforts to control, treat and eliminate these diseases by developing new vaccines, advocating use of existing vaccines and promoting increased access to affordable medical treatments. For more information please visit www.sabin.org.

Treatment Action Group (TAG) is an independent AIDS research and policy think tank fighting for better treatment, a vaccine, and a cure for AIDS. TAG works to ensure that all people with HIV receive lifesaving treatment, care, and information. We are science-based treatment activists working to expand and accelerate vital research and effective community engagement with research and policy institutions. TAG catalyzes open collective action by all affected communities, scientists, and policy makers to end AIDS. For more information please visit <http://www.treatmentactiongroup.org/>.

CC:

The Honorable Dr. Stephen Ostroff, Acting Commissioner of Food and Drugs, U.S. Food and Drug Administration
The Honorable Tammy Baldwin, Member, Committee on Health, Education, Labor and Pensions
The Honorable Michael F. Bennet, Member, Committee on Health, Education, Labor and Pensions
The Honorable Richard Burr, Member, Committee on Health, Education, Labor and Pensions
The Honorable Bill Cassidy, Member, Committee on Health, Education, Labor and Pensions
The Honorable Robert P. Casey, Member, Committee on Health, Education, Labor and Pensions
The Honorable Susan Collins, Member, Committee on Health, Education, Labor and Pensions
The Honorable Michael B. Enzi, Member, Committee on Health, Education, Labor and Pensions
The Honorable Al Franken, Member, Committee on Health, Education, Labor and Pensions
The Honorable Orrin G. Hatch, Member, Committee on Health, Education, Labor and Pensions
The Honorable Johnny Isakson, Member, Committee on Health, Education, Labor and Pensions
The Honorable Mark Kirk, Member, Committee on Health, Education, Labor and Pensions
The Honorable Barbara A. Mikulski, Member, Committee on Health, Education, Labor and Pensions
The Honorable Lisa Murkowski, Member, Committee on Health, Education, Labor and Pensions
The Honorable Christopher S. Murphy, Member, Committee on Health, Education, Labor and Pensions
The Honorable Rand Paul, Member, Committee on Health, Education, Labor and Pensions
The Honorable Pat Roberts, Member, Committee on Health, Education, Labor and Pensions
The Honorable Bernie Sanders, Member, Committee on Health, Education, Labor and Pensions
The Honorable Tim Scott, Member, Committee on Health, Education, Labor and Pensions
The Honorable Elizabeth Warren, Member, Committee on Health, Education, Labor and Pensions
The Honorable Sheldon Whitehouse, Member, Committee on Health, Education, Labor and Pensions