**Submission Regarding Bill H.R. 3299:**
**Suggestions to Fix the FDA PRV for Neglected Diseases**

The Honorable Joseph Pitts, Chairman
The Honorable Gene Green, Ranking Member
Committee on Energy and Commerce, Subcommittee on Health
United States House of Representatives

CC:
The Honorable Fred Upton, Chairman, Committee on Energy and Commerce
The Honorable Frank Pallone, Ranking Member, Committee on Energy and Commerce

May 19, 2016

Dear Chairman Pitts and Ranking Member Green:

We are writing to request that you support proposed changes to the Food and Drug Administration (FDA) Priority Review Voucher (PRV) program to ensure that it effectively accomplishes its goal of incentivizing new research and development (R&D) for neglected diseases, and that new neglected diseases products brought to market through the PRV program are made accessible and affordable to those who need them.

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 March 2016, the American Thoracic Society, Doctors Without Borders/Médecins Sans Frontières, the Drugs for Neglected Diseases *initiative*, the IDSA Center for Global Health Policy, the Sabin Vaccine Institute, the TB Alliance and the Treatment Action Group sent a letter to the Committee on Energy and Commerce leadership raising several concerns with the design of the FDA PRV program for neglected diseases and proposed legislative amendments.[2]

The monetary value of PRVs has been established through sales, with the most recent voucher being sold for $350 million in August 2015.[3] However, the value of these vouchers as an incentive to promote innovation for new therapeutic options for populations affected by neglected diseases depends on the PRVs being awarded only to truly new products that are accessible to those who need them.

[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.
[2] Letter sent March 29, 2016. 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.

Nevertheless, 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 neglected disease PRV program that remain unaddressed. Now, as Congress is examining the PRV program, we hope that the Committee will use this opportunity to fix the neglected disease FDA PRV program to ensure that novel 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.** Under current law, a PRV for neglected diseases can be awarded even when new R&D investments have not been made by the entity receiving the award or if 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.

Straightforward statutory changes, based on existing law for the rare pediatric disease PRV program and proposed legislation for PRV programs, could help to remedy the functioning of the PRV for neglected diseases. We have discussed these proposals with many Committee member offices, and we hope you will advance these solutions.

In addition to introducing these two critical fixes, we hope the Committee will also request a US Government Accountability Office (GAO) study and report to evaluate the effectiveness of the PRV programs as an incentive for promoting innovation, and whether and to what extent global unmet needs for biomedical innovation have been met by the PRV program. 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 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.msfaccess.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.msfaccess.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,


American Thoracic Society


Drugs for Neglected Diseases *initiative*


HIV Medicine Association


Infectious Diseases Society of America


Médecins Sans Frontières/Doctors Without Borders USA


Sabin Vaccine Institute


Treatment Action Group


TB Alliance

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 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 www.dndi.org.

TB Alliance is a non-profit organization dedicated to the discovery and development of new, faster-acting and affordable tuberculosis medicines. For more information please visit www.tballiance.org.

The HIV Medicine Association (HIVMA) is an organization of medical professionals who practice HIV medicine. We represent the interest of our patients by promoting quality in HIV care and by advocating for policies that ensure a comprehensive and humane response to the AIDS pandemic informed by science and social justice. For more information please visit www.hivma.org.

The Infectious Diseases Society of America (IDSA) represents physicians, scientists and other health care professionals who specialize in infectious diseases. IDSA's purpose is to improve the health of individuals, communities, and society by promoting excellence in patient care, education, research, public health, and prevention relating to infectious diseases. For more information please visit www.idsociety.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 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 www.treatmentactiongroup.org.

Please contact Judit Rius Sanjuan, U.S. Manager and Legal Policy Adviser of the MSF Access Campaign of Médecins Sans Frontières/Doctors Without Borders USA, if you are interested in scheduling a meeting or learning more about the content of this letter. Email: judit.rius@newyork.msf.org / Phone: +1 212 655 3762