

April 12, 2012

The Honorable Tom Harkin
Chairman
Senate Health, Education, Labor, and
Pensions Committee
713 Hart Senate Office Building
Washington, DC 20510

The Honorable Mike Enzi
Ranking Member
Senate Health, Education, Labor, and
Pensions Committee
379A Russell Senate Office Building
Washington, DC 20510

The Honorable Fred Upton
Chairman
House Energy and Commerce Committee
2183 Rayburn House Office Building
Washington, DC 20515

The Honorable Henry Waxman
Ranking Member
House Energy and Commerce Committee
2204 Rayburn House Office Building
Washington, DC 20515

The Honorable Joe Pitts
Chairman
Subcommittee on Health
House Energy and Commerce Committee
420 Cannon House Office Building
Washington, DC 20515

The Honorable Frank Pallone, Jr.
Ranking Member
Subcommittee on Health
House Energy and Commerce Committee
237 Cannon House Office Building
Washington, DC 20515

Dear Senate and House Leaders:

We, the undersigned organizations representing medical societies, patients, public health, health systems, and other stakeholders urge Congress to establish a new antibacterial drug approval pathway as part of the upcoming Prescription Drug User Fee Act (PDUFA) legislation. The concept we support, put forward by the Infectious Diseases Society of America (IDSA), is a Limited Population Antibacterial Drug (LPAD) approval mechanism for drugs intended to treat the most serious bacterial infections where insufficient satisfactory therapeutic options exist. The LPAD product approval mechanism is a necessary complement to the economic incentives for antibacterial development that Congress currently is considering for inclusion in PDUFA.

We are gravely concerned about the increasing number of patients with serious and life-threatening infections who cannot be treated due to a lack of effective antibacterial drugs. These cases result in longer hospital stays, readmissions, increased healthcare costs and many thousands of deaths. As the number of patients succumbing to antibacterial-resistant infections rises, the number of new antibacterial drugs in development is plummeting. The few large companies who remain in antibacterial research and development (R&D) cite the lack of feasible and predictable regulatory approval pathways as the primary reason they are withdrawing from the U.S. market and shifting antibiotic R&D efforts overseas.

When it comes to antibacterials, and particularly antibacterials needed to treat patients with the most serious bacterial infections, FDA's risk-benefit equation has been out of balance. Of importance, it is not feasible for antibacterial drugs that treat serious infections caused by highly resistant bacterial pathogens to be developed using traditional, large scale clinical trials due to

the limited numbers of patients in which these serious infections occur. That is why establishing the LPAD mechanism is so critically important.

Under the LPAD mechanism, a drug's safety and effectiveness would be studied in substantially smaller, more rapid, and less expensive clinical trials—much like the Orphan Drug (OD) Program permits for other rare diseases. Consistent with existing drug approval standards, LPAD drug sponsors will need to demonstrate to the Food and Drug Administration's (FDA) satisfaction that LPAD products are safe and effective for their intended use and that the drugs' benefits outweigh their risks for the indicated populations. LPAD products then would be narrowly indicated for use in small, well-defined populations of patients for whom the drugs' benefits have been shown to outweigh their risks. For patients with serious infections and insufficient therapeutic options, a greater degree of uncertainty about overall risk associated with a drug can be tolerated. Through its narrow indication, the LPAD mechanism would help ensure that drug companies will narrowly market these precious drugs, which will protect patients outside of the indicated population from exposure to risk as well as slow the rate at which resistance to the drugs develops. Of importance, the LPAD mechanism would not be used to approve antibacterial drugs that treat more common infections or where sufficient alternative therapeutic options exist.

Of tremendous value, the LPAD approval pathway will reestablish an appropriate balance in FDA's antibacterial risk-benefit decision-making and will create a predictable, measured, and feasible approval pathway that will lure companies back into antibacterial R&D. If Congress fails to act, we face a future that resembles the days before these miracle drugs were developed, one in which people died of common infections, and where many medical interventions that we take for granted—including care for premature infants, surgery, chemotherapy, organ transplantation, and even dentistry for some patients—become impossible.

For further information on the LPAD mechanism, please view the attached one pager from the Infectious Diseases Society of America. Should you have any questions, please contact Amanda Jezek, IDSA's government relations director at ajezek@idsociety.org.

Sincerely,

American Academy of Orthopaedic Surgeons
American Academy of Otolaryngology—Head and Neck Surgery
American Association of Hip and Knee Surgeons
American College of Medical Quality
Alliance for the Prudent Use of Antibiotics
American College of Rheumatology
American College of Surgeons
American Thoracic Society
American Society for Microbiology
Association for Professionals in Infection Control and Epidemiology, Inc.
Food Animal Concerns Trust
Infectious Diseases Society of America
National Association of County and City Health Officials
National Foundation for Infectious Diseases

Pediatric Infectious Diseases Society

Premier (serving more than 2,500 U.S. hospitals and 80,000-plus healthcare sites)

Renal Physicians Association

Society for Healthcare Epidemiology of America

Society of Critical Care Medicine

Society of Infectious Disease Pharmacists

Treatment Action Group

Trust for America's Health