Priority Review Voucher for National Security Medical Countermeasures

Congress is considering providing Priority Review Vouchers for the 13 deadly pathogens identified by the Department of Homeland Security as material threats to U.S. national security. This would encourage new MCM development at a time when it is desperately needed in the biodefense space.

New incentives are needed to ensure medical countermeasures (MCMs) are developed, licensed, and available to protect Americans from all national security threats.

- The Department of Homeland Security has identified 13 such threats, including anthrax, smallpox, Ebola/Marburg, tularemia, and botulism.
- According to the Director of National Intelligence, biological and chemical materials and technologies are more accessible than ever to groups like ISIS and al Qaeda. ISIS has already advanced a chemical weapons program, using mustard gas to kill civilians in Syria and Iraq. U.S. and French intelligence officials believe ISIS is trying to develop more advanced biological weapons to use in attacks against the West.
- The best preparedness for these threats is a robust stockpile of drugs and vaccines (a.k.a. MCMs) that can be quickly dispensed if and when a threat materializes.
- Gaps in preparedness remain where no MCMs have been developed to address certain threats.

The major incentive for biopharmaceutical companies in biodefense – the "guaranteed market" of the BioShield Special Reserve Fund – is effectively gone and procurement budgets in other agencies have been decimated.

- The government is the only market for most MCMs, because unlike other drugs and vaccines, these products are not sold to doctors, hospitals, or pharmacies.
- The BioShield Special Reserve Fund (SRF) has been the MCM market for the last decade; a 10 year advance appropriation of $5.6 billion was available to procure successful candidate MCMs.
- The SRF expired in 2013 and all funds were used to add 12 new MCMs to the national stockpile.
- Congress reauthorized the SRF but adequate funding has not followed; the SRF is now appropriated annually and has not kept pace with the need for purchasing products ready for stockpiling.
- Without a demonstrated market for these products, MCMs will languish in advanced development.

Bipartisan Priority Review Voucher (PRV) programs have successfully encouraged R&D in other challenging disease areas, such as tropical diseases and rare pediatric diseases.

- In 2007, Congress authorized FDA to award PRVs to sponsors of neglected tropical disease products. Congress identified a total of 16 neglected tropical diseases eligible for a voucher; Ebola was added in 2014.
    - The incentive has worked – numerous companies expanded research in these diseases and 4 vouchers have been awarded for products receiving FDA approval. Only 1 has sold.
- In 2011, Congress recognized the lack of effective treatments for rare pediatric diseases. A PRV program was created for pediatric diseases that affect fewer than 200,000 children in the U.S.
    - Again, this incentive worked. Many new products to treat rare pediatric diseases have begun development and FDA has awarded vouchers for 7 products receiving licensure. 3 have been sold.

How it works:

- A PRV is awarded to a company when a new product is approved. The PRV can be applied to any other product; it shortens the FDA review timeline for a new application from 10 months to 6 months. The PRV can also be sold to another company, thereby providing an important financial incentive.
- Companies are required to pay an additional user fee to use the voucher ($2.7M in 2017), thereby covering the cost of the additional resources FDA needs to expedite review under the voucher.
- PRVs are market-based incentives with no cost to the federal government.
- The structure of voucher programs limits the number of MCM eligible for a PRV, ensuring the incentive is limited and targeted.
    - MCM product itself must qualify for priority review in order to be eligible for a voucher.
    - MCMs with commercially approved indications would not qualify.
    - MCMs previously approved by the FDA would not qualify.