United States Senate

COMMITTEE ON
HOMELAND SECURITY AND GOVERNMENTAL AFFAIRS

WASHINGTON, DC 20510–6250

February 18, 2016

Dr. Stephen Ostroff, M.D.
Acting Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Dr. Ostroff,

The Committee on Homeland Security and Governmental Affairs is examining the U.S. Food and Drug Administration (FDA) Drug Approval Process. Recent estimates of the cost per new prescription drug approved indicate that the pre-tax industry cost could be as high as $2.6 billion.[1]

Since the Prescription Drug User Fee Act (PDUFA) was passed in 1992, more than 1,000 drugs and biologics have come to market, including new medicines to treat cancer, AIDS, cardiovascular disease, and life-threatening infections.[2] The FDA states that PDUFA has enabled access to new drugs as fast or faster than anywhere in the world, all while maintaining the same thorough review process.[3] Additionally, under PDUFA, drug companies agree to pay fees that boost FDA resources, and FDA agrees to timeframes for its review of new drug applicants.[4]

But despite the number of drugs approved, patients and patient advocacy groups remain concerned about the time it takes to approve new treatments through the clinical trial pipeline.[5] These concerns are most painfully expressed by the patients, and their family members, who are suffering from terminal illness and are desperate to have a chance to use potentially lifesaving treatments.

In February 2015, the FDA announced draft guidance for industry for public comment, which would create a new draft form, which, when finalized, FDA intends to make available for licensed physicians to use for expanded access requests for individual patients to have access to

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[1] Tufts Center for the Study of Drug Development, *Cost of Developing a New Drug* http://csdd.tufts.edu/files/uploads/Tufts_CSDD_briefing_on_RD_cost_study_-_Nov_18,_2014..pdf (January 20, 2016)
[2] *Id.*
[3] *Id.*
[4] *Id.*
[5] Milken Institute FasterCures, *Why Translational Research Matters,* http://www.fastercures.org/assets/TranslationalScience.pdf (February 8, 2016)

drugs that are not yet approved.[6] Individual patient expanded access allows for the use of an investigational drug outside of a clinical trial for an individual patient who has a serious or immediately life-threatening disease or condition and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition.[7] When finalized, this new process could provide a streamlined alternative for terminally ill patients to access potentially life-saving treatments.[8] As of February 18, 2016, the streamlined process has not yet been implemented.

To enable the Committee to better understand FDA's current drug approval process and what the FDA is doing to improve the American public's access to treatments, please provide the following information:

1. Provide the FDA's estimate for the amount of time for a drug to complete the FDA's drug approval process as well as the FDA estimated cost per drug approved.

2. How many of the drugs in the Accelerated Approval, Fast Track and Compassionate Use programs were later approved?

3. What percentage of the FDA budget is dedicated to the drug approval process? What percentage is dedicated to expanded access? Please provide actual dollar amounts for FY2015.

4. Why has the streamlined application process for expanded access announced in February 2015 not been implemented? What is the FDA's timeline for implementing the streamlined application?

5. What is the FDA doing to streamline the requirements for manufacturers in the expanded access/compassionate use application process?

6. Please describe how data from the use of therapies administered through the expanded access process is used. For example, does the FDA use adverse event data from expanded access cases in its evaluation of clinical trials?

7. Please describe, in detail, the process used to review each of the requests under the Emergency Use Authorization or Expanded Access Program with respect to the diagnosis or treatment of Ebola.

8. How often are FOIA requests relating to expanded access denied? What justification is used to deny FOIA requests? Why was a FOIA request relating to the Expanded Access Program for Ebola denied and what was the reason for denial?

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[6] U.S. Food and Drug Administration, *Individual Patient Expanded Access Applications: Form FDA 3926; Draft Guidance for Industry; Availability* https://www.federalregister.gov/articles/2015/02/10/2015-02561/individual-patient-expanded-access-applications-form-fda-3926-draft-guidance-for-industry (January 20, 2016)

[7] *Id.*

[8] *Id.*

9. Please list the most common cases and conditions for accelerated approval, fast track and compassionate uses.

Please produce this material as soon as possible, but by no later than 5:00 p.m. on March 3, 2016.

The Committee on Homeland Security and Governmental Affairs is authorized by Rule XXV of the Standing Rules of the Senate to investigate "the efficiency and economy of operations of all branches of the Government."[9] Additionally, S. Res. 73 (114th Congress) authorizes the Committee to examine "the efficiency and economy of all branches and functions of Government with particular references to the operations and management of Federal regulatory policies and programs."[10]

For purposes of this request, please refer to the definitions and instructions in the enclosure to this letter. If you have any questions about this request, please contact Satya Thallam on the Committee staff at (202) 224-4751. Thank you for your attention to this matter.

Sincerely,

Ron Johnson
Chairman

cc: The Honorable Thomas R. Carper
Ranking Member

Enclosure

[9] S. Rule XXV(k); *see also* S. Res. 445, 108th Cong. (2004).
[10] S. Res. 73 § 12, 114th Cong. (2015).