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COMMITTEES:
BUDGET
COMMERCE, SCIENCE AND TRANSPORTATION
FOREIGN RELATIONS
HOMELAND SECURITY AND GOVERNMENTAL AFFAIRS

United States Senate

WASHINGTON, DC 20510

October 21, 2016

The Honorable Sylvia Mathews Burwell
Secretary
U.S. Department of Health & Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

Dear Secretary Burwell:

The Committee on Homeland Security and Governmental Affairs continues to examine the federal government's drug approval process and explore efforts to more quickly provide access to therapies for the treatment of terminal and severely-debilitating conditions. I appreciate your assistance with this important effort.

As part of this effort, the Committee recently held a hearing in which we sought definitive answers from the U.S. Food & Drug Administration (FDA) regarding potential enforcement or disciplinary actions directed at parties acting pursuant to state "right to try" laws now adopted in 32 states.[1] The Committee invited FDA Commissioner Robert Califf, as the head of the federal agency charged with regulating medical products, to testify at the hearing. Unfortunately, Dr. Califf declined the invitation and the FDA instead made available Dr. Peter Lurie, the Associate Commissioner for Public Health Strategy and Analysis.

Prior to the hearing, at a meeting in which Dr. Lurie briefed committee staff on this and other issues pertinent to the hearing, Dr. Lurie was unable to answer questions about the agency's posture toward state "right to try" laws.[2] In particular, Dr. Lurie was unable to answer how or if the FDA would respond to physicians who provide treatments which have not received FDA marketing approval to patients pursuant to state right to try to laws.[3] Following the meeting, I wrote to Commissioner Califf to express my disappointment that he would not testify and to ask that he reconsider appearing before the Committee.[4] In my letter, I also requested that the FDA witness be fully prepared to answer nine specific questions relevant to the Committee's work, including how the FDA would respond to physicians providing treatment to patients under state right to try laws.[5]

During the hearing, the Committee heard a video statement from a physician in Texas who is currently treating patients under that state's right to try law, despite a lack of clarity about whether the FDA would pursue legal action against him for doing so.[6] I asked Dr. Lurie: "Is

[1] U.S. Senate Committee on Homeland Security and Governmental Affairs. *Exploring a Right to Try for Terminally Ill Patients*, Hearing (September 22, 2016).
[2] Briefing from the U.S. Food and Drug Administration to Committee staff (September 16, 2016).
[3] *Id.*
[4] Letter from Chairman Johnson to Commissioner Dr. Robert M. Califf (September 19, 2016).
[5] *Ibid.*
[6] *Supra*, note 1 (statement of Dr. Ebrahim Delpassand).

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there going to be any enforcement action against doctors that have the courage" to administer experimental treatments pursuant to their state's respective law. Despite my request to Dr. Califf that the FDA witness be prepared for this question, Dr. Lurie responded: "I can't really speak to what enforcement action we might take."[7]

To date, the Committee has been unable to obtain clear answers about the FDA's position on state right to try laws and how the FDA will respond to physicians who provide treatments to terminally ill patients pursuant to those laws. For this reason, because the FDA is not forthcoming, I am requesting that you provide answers to the following questions:

1. Does the Department of Health & Human Services believe terminal patients should be permitted to access treatments that have completed Phase I testing and are continuing toward final drug approval if no other treatment options are available and enrollment in a clinical trial is not possible; the patient, his or her doctor, and the manufacturer consent; and if authorized by state law?

2. Why was the FDA's streamlined application for expanded access, announced in February 2015, not finalized until June 2, 2016?

3. Will HHS or the FDA promulgate regulations or guidance advising pharmaceutical companies as to how, if at all, the FDA will use adverse events that occur outside of clinical trials conducted in accordance with FDA approved protocols (through FDA's expanded access program, or otherwise) in the FDA's decision-making process about whether a trial can continue and/or the drug can be approved?

4. How often does the FDA update the information made available to patients on clinicaltrials.gov? Will you commit to ensuring this information is up-to-date and accurate so that patients can learn about and pursue their options under clinical trials, expanded access, and right to try? What specific steps will HHS take, and in what timeframe, to ensure this commitment?

5. Will HHS provide the Committee with a list of each treatment and the number of patients treated for all expanded access approvals over the past year?

6. If the FDA becomes aware that a physician or manufacturer is administering or making available to patients a treatment that has not received approval of a New Drug Application and remains in clinical study phase, pursuant to a state-passed right to try law, will the FDA attempt to enforce Federal laws against the physician or manufacturer? Has the FDA ever referred a physician or manufacturer to the Department of Justice, another law enforcement agency, or a state medical board for making treatments still in clinical trials available to patients under a state-passed right to try law? How does the FDA use information about a physician or manufacturer providing treatments pursuant to a state right to try law in its approval process for new drugs?

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[7] *Supra*, note 1.

7. What policy changes would HHS support to speed access to treatments for those with life-threatening illnesses (not including the FDA's expanded access program)? Does HHS support reciprocal drug or device approval with international peer agencies? Does HHS support personal importation of drugs or devices fully approved in other countries?

8. How are questions for advisory committee consideration developed? How does the FDA or a committee ensure they are presented with appropriate questions that do not unnecessarily hinder evaluation of a drug's effects? What is the public's role in developing the questions?

9. The FDA has finally made public a decision on the Priority Review of a NDA for a treatment of Duchenne muscular dystrophy. On February 8, 2016, the FDA delayed the Prescription Drug User Fee Act (PDUFA) date by three months to May 26, 2016? Why did the FDA miss this goal date by nearly four months? Has the FDA approved any expanded access applications for this treatment?

Please provide those answers by no later than November 7, 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."[8] 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."[9]

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

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

cc: Hon. Robert M. Califf, M.D.
Commissioner, U.S. Food & Drug Administration

The Honorable Thomas R. Carper
Ranking Member