United States Senate

COMMITTEE ON
HOMELAND SECURITY AND GOVERNMENTAL AFFAIRS
WASHINGTON, DC 20510–6250

September 19, 2016

Robert M. Califf, M.D.
Commissioner of Food and Drugs
U.S. Food and Drug Administration
10903 New Hampshire Avenue,
Hillandale Building, 4th Floor
Silver Spring, MD 20993

Dear Dr. Califf:

The Committee on Homeland Security and Governmental Affairs continues to examine the U.S. Food and Drug Administration's (FDA) drug approval process and efforts to provide new therapies to treat persons with life-threatening and severely-debilitating illnesses.

I write to express my disappointment that you have declined the Committee's invitation to attend our hearing on September 22, 2016, and to respectfully request that you reconsider your decision not to appear. I understand that there are time constraints placed on your schedule. However, it is crucial that our Committee and, more importantly, the American people—including the many patients and family members affected by the FDA's policies and decisions—hear direct answers from you to several questions that may have life or death consequences for many Americans.

As you know, I have written several letters to the FDA this year asking questions regarding the drug approval process, the more than year-long delay to move forward with the streamlined expanded access application that was announced in February 2015, and the FDA's consideration of a new drug application to treat patients suffering from Duchenne muscular dystrophy.[1] Our Committee also held a hearing on February 25th to examine strategies to connect terminal patients with potentially life-saving treatments.[2]

My investigation into the FDA's handling of drug approvals and the expanded access program led me to introduce S. 2912, *The Trickett Wendler Right to Try Act of 2016*. Trickett Wendler was a mother of three from Waukesha, Wisconsin who succumbed to Amyotrophic

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[1] Letter from Chairman Johnson to Acting Commissioner Dr. Stephen Ostroff, February 18, 2016; Letter from Chairman Johnson, Ranking Member Tom Carper, Senator Dan Coats, and Senator Joe Donnelly to Dr. Janet Woodcock, March 16, 2016; Letter from Chairman Johnson and Senator Coats to the Honorable Robert M. Califf, M.D, Commissioner of Food and Drugs, May 20, 2016; Letter from Chairman Johnson and Chairman Lamar Alexander to the Honorable Robert M. Califf, M.D, Commissioner of Food and Drugs, September 17, 2016.

[2] U.S. Senate, Committee on Homeland Security and Governmental Affairs. *Connecting Patients to New and Potentially Life Saving Treatments*, February 25, 2016.

Lateral Sclerosis (ALS) in 2015. Her widowed husband Tim told me that Trickett received the same treatment that her father did when he was diagnosed with ALS decades ago.

I believe that terminal patients like Trickett deserve the right to try to save their own lives without the need to obtain the FDA's permission. To date, thirty-one states have enacted laws to provide patients the right to try to save their own lives by using investigational treatments that have passed Phase I of the FDA's clinical trials that demonstrate safety. These laws have passed with overwhelming bipartisan support.

To date, 39 of my colleagues have joined me in co-sponsoring S. 2912. This bill is designed to resolve potential questions about the legality of state right to try laws under federal law. It would clarify that the federal government cannot prohibit doctors from treating terminal patients in accordance with state law if certain requirements are met. The bill includes liability protections for doctors and manufacturers who provide treatment under federal and state right to try laws. It also requires that the FDA not use adverse outcomes of treatments used under right to try laws when reviewing extant Investigational New Drug applications.

The FDA has an obligation to be transparent and accountable to Congress and the American people. The Committee invited you to testify at the September 22nd hearing so that we could get the FDA's views on state right to try laws, S. 2912, and other facets of the FDA's drug approval process. I am disappointed that you have declined the invitation. I am concerned that Dr. Lurie will not be able to answer the questions I and other members have about these important topics.

I understand that Dr. Lurie is an expert who can speak about the FDA's expanded access program. However, the scope of Thursday's hearing is broader than the FDA's expanded access program. My concern was heightened after my staff met with Dr. Lurie on September 16th. Dr. Lurie was unable to answer questions about state right to try laws, the Senate legislation, how the FDA treats physicians and manufacturers who provide products under state right to try laws that have not received approval of a New Drug Application (NDA), and whether data collected from treatments under right to try laws will be used during application reviews.

I continue to believe that you, as the head of the agency, are the most appropriate official to answer questions on behalf of the FDA, especially where questions may touch upon issues outside of Dr. Lurie's purview. Thus, I respectfully request that you reconsider your decision not to appear at the Committee's hearing. In the event that you remain unwilling to personally appear, I request that you send a substitute in your place that is fully prepared to answer the following questions on behalf of the FDA:

1. Does the FDA Commissioner believe terminal patients should have the ability to access Phase 1 approved treatments that 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 the FDA promulgate regulations advising pharmaceutical companies as to how, if at all, the FDA will use adverse events that occur outside of clinical trials (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 the Commissioner 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 the FDA take, and in what timeframe, to ensure this commitment?

5. Will the FDA provide the Committee with a list of each drug and the number of patients treated for all compassionate use approvals over the past year?

6. If the FDA learns that a physician or manufacturer is making products that are still in clinical trials available to patients 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 products still in clinical trials available to patients under a state-passed right to try law? How does the FDA use information it learns about a physician or manufacturer providing products pursuant to a state right to try law in its approval process for new drugs?

7. What policy changes would the FDA support to speed up access to treatments for those with life-threatening illnesses (not including the FDA's expanded access program)? Does the FDA support reciprocal drug or device approval? Does the FDA 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? Who ensures the FDA is following the requirements of the Food and Drug Administration Safety and Innovation Act?

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?

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."[3] 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."[4]

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

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