United States Senate

COMMITTEE ON
HOMELAND SECURITY AND GOVERNMENTAL AFFAIRS

WASHINGTON, DC 20510–6250

May 31, 2018

The Honorable Scott Gottlieb, M.D.
Commissioner
Food & Drug Administration
U.S. Department of Health and Human Services
10903 New Hampshire Avenue
Silver Spring, Maryland 20993

Dear Commissioner Gottlieb:

Yesterday, President Trump signed into law S. 204, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, restoring a degree of freedom and hope to patients with a life-threatening disease and their families.[1] As the primary author of S. 204, I write to ensure that the Food and Drug Administration (FDA) implements right to try in a manner consistent with the law's intent.[2]

In a recent article about right to try, you appeared to suggest that the FDA would need to issue regulations to balance the law's requirement against "patient protections." The article quoted you as saying:

- "In terms of making sure that it balances [access to experimental drugs] against appropriate patient protections . . . with [S. 204], we'd have to do a little bit more . . . in guidance and perhaps in regulation to achieve some of those goals."[3]

- "We felt that there were certain aspects of [S. 204] that could be modified to build in additional patient protections, but if you weren't able to do that legislatively, that there [was] a pathway by which you do that administratively and still remain consistent with the letter and the spirit of this law."[4]

You later tweeted: "I stand ready to implement [right to try] in a way that achieves Congress' intent to promote access and protect patients; and build on #FDA's longstanding commitment to these important goals."[5]

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[1] S. 204, 115th Cong. (2018) (enacted).
[2] *See also* Statement of Legislative Intent, 164 Cong. Rec. H4,360 (daily ed. May 22, 2018) (statement of Sen. Ron Johnson).
[3] Ike Swetliz and Erin Mershon, *Right-to-try bill headed for vote puts bigger burden on FDA to protect patients, Gottlieb says*, STAT (May 17, 2018), https://www.statnews.com/2018/05/17/right-to-try-bill-gottlieb/.
[4] *Id.*
[5] Scott Gottlieb, M.D. (@SGottliebFDA), Twitter (May 12, 2018, 3:22 AM), https://twitter.com/SGottliebFDA/status/997223120300855299.

As I made clear to my colleagues in the Senate and the House before each body voted on S. 204, this legislation is fundamentally about empowering patients to make decisions in cooperation with their doctors and the developers of potentially life-saving therapies.[6] This law intends to diminish the FDA's power over people's lives, not increase it. It is designed to work within existing FDA regulations, definitions, and approval processes. It is not meant to grant FDA more power or enable the FDA to write new guidance, rules, or regulations that would limit the ability of an individual facing a life-threatening disease from accessing treatments. Under this law, the FDA's oversight with respect to patient safety within a Phase I trial remains unchanged; the current thresholds for successful completion of such a trial phase remains unchanged.

Given your comments, and as the law's primary author, I would like to make this legislation's intent absolutely clear.

- ***Broad access to right to try:*** S. 204, as originally introduced, applied to patients "with a terminal illness," as defined by state law. I rejected the FDA's proposed definition—"immediately life-threatening disease or condition"[7]— because it would exclude patients with Duchenne muscular dystrophy, an illness that I explicitly intended to be covered by the legislation. As enacted, S. 204 defines terminal illness as "life-threatening disease or condition," a definition that exists in current federal regulation.[8] The FDA confirmed that this definition would include patients diagnosed with Duchenne muscular dystrophy.[9]

- ***Use of outcomes:*** S. 204 requires that the Secretary of Health and Human Services may not use a clinical outcome associated with the use of an eligible investigational drug to delay or adversely affect the drug's review or approval, unless use of that clinical outcome is critical to determining safety. This language is not intended to enable the FDA to expand the scope of existing safety determinations about investigational drugs.

- ***Standard "clinical trial" definition:*** S. 204 requires that an eligible investigational drug be under investigation in a clinical trial that is intended to form the primary basis

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[6] Johnson, *supra* note 2. *See also* 163 Cong. Rec. S4,788 (daily ed. August 3, 2017) (statement of Sen. Ron Johnson); Letter from Sens. Ron Johnson and Joe Donnelly to Reps. Walden, Pallone, Burgess, and Green (October 2, 2017) ("This pursuit has always been about protecting hope. Patients (along with their doctors) are informed, capable of making rational decisions, and have the right to do whatever they can to try to save their own lives.") available at https://www.hsgac.senate.gov/imo/media/doc/10-02-17%20RHJ%20and%20JD%20letter%20to%20House%20EC%20on%20Right%20to%20Try.pdf.

[7] *HHS/FDA Technical Assistance on TAM17847 "Trickett Wendler Right to Try Act of 2017" Prepared for Senator Ron. Johnson* (May 23, 2017).

[8] 21 C.F.R. § 312.81.

[9] Email correspondence between FDA staff and Committee on Health, Education, Labor, and Pensions staff (Aug. 2, 2017) (copy on file with S. Comm. on Homeland Security & Governmental Affairs).

of a claim of effectiveness in support of approval or licensure. According to the FDA, this language simply incorporates the standard definition of a clinical trial.[10] This language is not intended to enable the FDA to exclude any clinical trial as a basis for precluding access to treatments under right to try.[11]

S. 204 gives patients with a life-threatening condition—people like Trickett, Frank, Jordan, and Matthew— a right to hope. As the FDA prepares to implement right to try, I respectfully request to meet with you as soon as possible to discuss the law's intent and FDA's plans to implement the law consistent with that intent. In addition, as the Committee reviews the FDA's implementation of right to try, I respectfully request your cooperation with this oversight.

If you have any questions, please ask your staff to contact Satya Thallam of Committee staff at (202) 224-4751. Thank you for your attention to such an important issue.

Sincerely,

Ron Johnson
Chairman

cc: The Honorable Claire McCaskill
Ranking Member

[10] Phone conversation between FDA Staff and Sen. Ron Johnson Staff (Mar. 9, 2018).
[11] *Id.*