Statement by Sen. Ron Johnson on S. 204 (as considered by the House of Representatives)

In a recent article about pending right to try legislation, FDA Commissioner Scott Gottlieb was quoted 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, and I think those are the goals that Congress wants us to achieve."[1] The article went on to quote Commissioner Gottlieb as saying: "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."[2]

In response to this article, Commissioner Gottlieb tweeted the "FDA…stands ready to implement [right to try] in a way consistent with the intent of Congress."[3]

As S. 204's primary author and lead sponsor, I want to make this legislation's intent absolutely clear and remove any ambiguity that the FDA could use to implement right to try in a way contrary to its aim.

S. 204, as originally introduced, applied to patients "with a terminal illness," as defined by State law. In discussion with the FDA, the agency suggested it would prefer a uniform federal definition, especially one that already existed in federal statute or regulation, because an existing federal definition would facilitate implementation of the law. The FDA suggested defining terminal illness as an "immediately life-threatening disease or condition."[4] The FDA disclosed that its suggested definition would exclude, for example, patients with Duchenne muscular dystrophy—an illness explicitly intended to be covered by the legislation.

To be clear, I rejected this proposed definition because I believed it would inappropriately exclude patients with certain diseases from accessing treatments. By contrast, the legislation instead defines terminal illness as "life-threatening disease or condition" (which exists in current federal regulation).[5] which the FDA confirmed would include patients diagnosed with Duchenne muscular dystrophy.[6]

Contrary to the preference of FDA official Dr. Janet Woodcock, who expressed the FDA's desire to draft the legislation "to make sure we don't include patients we (the FDA) doesn't intend to include,"[7] I replied and rejected that notion by stating my intent was completely opposite hers:

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[1] [1] 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/.

[2] *Id*.

[3] Scott Gottlieb, M.D. (@SGottliebFDA), Twitter (May 17, 2018, 2:11 PM), https://twitter.com/SGottliebFDA/status/997223120300855299.

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

[5] 21 C.F.R. § 312.81.

[6] Email correspondence between FDA staff and Committee on Health, Education, Labor, and Pensions staff (Aug. 2, 2017).

[7] Phone conversation between Dr. Janet Woodcock and Sen. Ron Johnson (Mar. 9, 2018).

"I wanted to make sure we didn't exclude any one we didn't intend to exclude." My aim from the beginning was to be as inclusive as possible such that as many patients as possible who are facing no available alternatives could potentially qualify.

S. 204 is fundamentally about empowering terminally-ill patients and their doctors who, together with the cooperation of the developers of potentially life-saving therapies, should be in charge of making a determination about their own course of treatment. The bill is not intended to further empower any federal agency, including the FDA, to limit in any way the ability of an individual facing a life-threatening disease or condition from accessing treatment. S. 204 is about preserving a right to hope and about expanding individual freedom. It is not meant to empower the FDA to limit the right to hope by regulation or guidance.

S. 204 includes a provision ensuring the Secretary may not use a clinical outcome associated with the use of an eligible investigational drug to delay or adversely affect review or approval of the drug, unless use of such clinical outcome is critical to determining safety. This language is in no way intended to enable the FDA to expand the scope of existing safety determinations regarding investigational drugs.

S.204 requires, in certain circumstances, that an eligible investigational drug be under investigation in a clinical trial that is intended to form the primary basis 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. This language is not in any way intended to enable the FDA to exclude any clinical trial as a basis for precluding access to treatments under right to try.[8]

[8] Phone conversation between FDA Staff and Sen. Ron Johnson Staff (Mar. 9, 2018).