Statement of Ranking Member Tom Carper
"Exploring a Right to Try for Terminally Ill Patients"
September 22, 2016

Thank you, Mr. Chairman, for calling this hearing today. I appreciate your willingness to continue a conversation about an important issue that is critical for Americans seeking access to potentially lifesaving treatments. I also want to thank you and your staff for the ongoing work that we are engaged in to try and move the ball forward and find ways to help patients gain access to experimental therapies, including through a forthcoming GAO report on these issues. I also want to thank our witnesses, especially Representative Neely, Mr. Matthew Bellina, Mr. Richard Garr, and Mr. Andrew McFadyen for their willingness to share their personal stories with us.

Before I begin my formal statement I would just like to mention my appreciation for the Chairman sharing this video. I look forward to learning more about this physician's experience. My understanding is that in what would seem to be similar situations, the U.S. Food and Drug Administration (FDA) has approved over 99 percent of patient applications for expanded access to these new experimental treatments. In fact, I understand the FDA has even granted drug approvals based solely on expanded access data. The FDA is, by law, precluded from discussing the details of any drug under review, but if the doctor was with us today, I would ask him why he did not appear to use the expanded access program which has worked quickly and efficiently for so many patients and their doctors. With that in mind I would like to ask unanimous consent that we place an FDA fact sheet on expanded access along with the recently updated application form in the record.

Today, we will have an opportunity to hear from the FDA, state representatives, patients, their loved ones, and other advocates on ways we could improve access to experimental medical treatments. These individuals and their families have faced some of the most difficult and painful challenges anyone could face. They deserve to be heard, and they deserve better access to experimental treatments. We will also have an opportunity today to review the Chairman's Legislation, S. 2912 the Trickett Wendler Right to Try Act. I appreciate the intent of Chairman Johnson's bill, and certainly support expanding access to experimental therapies to terminally ill patients.

We must keep in mind, however, that there is already what I understand to be an effective framework in place at the FDA that gives patients access to experimental drugs while those drugs are still being tested. The agency has given an extraordinary level of attention to the requests of patients with life-threatening conditions. In fact, I'm told it has approved more than 99 percent of requests for emergency treatments between 2010 and 2015. The agency has also taken constructive steps to greatly simplify its application process and further improve and streamline patients' access to experimental treatments.

Despite the high approval rates and ongoing reforms, I understand that the FDA believes more can be done and is continuing to work to improve patient access to experimental treatments. I hope to learn more about those steps today, as well as some additional ideas for how to ensure

that all patients in need have the information and resources necessary to access experimental medicines.

For terminally ill patients and their loved ones, safe and effective treatments cannot come quickly enough. That is why we need to do everything we can to give patients, doctors, and the companies that make these drugs the tools they need to participate in clinical trials, utilize the FDA's expanded access programs, and develop new treatments as safely, effectively, and quickly as possible. I hope this committee can help with those efforts and work with patients, health care providers, the pharmaceutical industry, and the FDA to ensure that all patients and their families can access safe and effective treatments as quickly as possible.

I want to close by thanking the witnesses and their families again for their willingness to share their stories and put forward possible solutions to these challenging issues.