PATRICK SNYDER
STATE REPRESENTATIVE • 85th ASSEMBLY DISTRICT

Thank you Chairwoman Vukmir and members of the committee for holding this public hearing today on Senate Bill 84/Assembly Bill 69, more commonly referred to as Right to Try.

For the past four years, I have been a volunteer with Wausau Aspirus Comfort Care & Hospice. Throughout this experience, I have spent time in patients' homes and at the hospice house, having the opportunity to form unforgettable relationships with terminally ill patients and their loved ones. Far too many times, I have witnessed patients with a terminal illness exhaust all conventional treatment options and be told by their doctor that there are no other alternatives.

When a patient with a terminal illness reaches this devastating point, they have two options. They can attempt to enroll in a clinical trial, but typically the sickest individuals are ineligible. If enrollment in a clinical trial is not possible, a patient may ask the FDA for special permission through their expanded access (compassionate use) application. The expanded access process is complicated, expensive, and time-consuming. In 2015, the FDA reported granting a total of 1,256 expanded access applications. This number is miniscule compared to the millions of Americans that are dying from a terminal illness every year.

Right to Try is designed for those patients who run out of options.

In just a matter of 38 months, 37 states have passed similar legislation into law with overwhelming bipartisan support, including our neighboring states of Illinois, Indiana, Michigan, Minnesota and Ohio. A Texas doctor is on record testifying that he alone has used Right to Try to prolong the lives of 78 patients with a terminal illness. We have crafted our legislation in the same vein as this movement that has taken off across the country. This bill gives certain people with a terminal illness access to investigational drug and treatment options that are not yet available on pharmacy shelves, but have passed Phase 1 basic safety testing by the FDA. Often times, these people do not have the luxury of waiting for these drugs to make it to the market. The average length of time for a drug to get approval for use by the general public is more than a decade long and costs over $2 billion dollars. Right to Try is an optional process for a patient and his/her doctor to close the gap between when this treatment is needed and when it's received. Far too often in our current system, by the time the drug is available, it's too late.

By putting these medical decisions back in the hands of the patient and their physician, we are ensuring that the patient has the right to explore every treatment option that is safe, legal, and available. Fortunately, I do not have a personal story to share with you on how this legislation would have benefited a loved one of mine in the past, but I do know if the unspeakable were to happen, I would want them to have the option to try a potentially life-saving drug or treatment if they chose to do so. They shouldn't have to wait for the government to grant them special permission. Even if these additional treatments don't save a person's life, at least their family will have peace of mind that every available option was pursued.

I ask for your support in my effort to bring Right to Try here to Wisconsin. It is common sense legislation that has been enacted by over half of the United States. These patients are already fighting for their lives, why make them fight the government for access to these potential lifesaving treatment options too?

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TERRY MOULTON
WISCONSIN STATE SENATOR
23RD SENATE DISTRICT

From: **Senator Terry Moulton**

To: **Senate Committee on Health and Human Services**

Re: **Testimony on Assembly Bill 69/Senate Bill 84**
Relating to: access to investigational drugs, devices, and biological products and limitations on liability related to their use.

Date: **August 16, 2017**

Mr. Chairman and committee members, thank you for the opportunity to speak to you today about Assembly Bill 69, the Right to Try bill.

Every year in Wisconsin more than 11,000 Wisconsinites will be told that their cancer is terminal. 2,000 Wisconsin families will find out their loved one has Alzheimer's, and 400 Wisconsinites will continue to battle with Lou Gehrig's Disease. Every year more and more people who have been diagnosed with a life-threatening condition undergo life-saving treatment. However, for those whose diagnosis is terminal, few options exist.

Only a handful of terminally ill patients qualify for clinical trials, and for those who do qualify, their access to potentially life-saving treatment ends when the clinical trial is over even if the treatment was successful and will be approved by the FDA. For those who aren't eligible, their only option is to apply for the FDA's compassionate use program, a process that is complicated, time-consuming, and can take months for an application to be approved.

Over the past few years 33 states, including our neighbors Minnesota, Michigan and Illinois, have passed Right to Try laws, giving terminally ill patients legal access to investigational drugs and treatment options. In Texas, a board certified nuclear medicine physician was kept from continuing life-saving treatment to his patients when the clinical trial he was conducting was completed. Although this drug was in the final stages of approval and was keeping his patients alive, approval was denied. Dr. Delpassand recently testified before the US Senate that under Texas' recent Right to Try laws, he was able to resume treatment, and today nearly 80 of patients diagnosed with terminal pancreatic cancer are still alive.

Assembly Bill 69 is modeled after other successful state models and allows a terminally ill patient who has exhausted all other treatment options to seek investigational treatment under the recommendation of a physician. Under this bill a manufacturer may choose to make an investigational drug that has completed FDA's phase 1 safety trials available to an eligible patient, and provides limited liability under state law for a manufacturer, distributor, pharmacist, or healthcare practitioner who provides this type of treatment while exercising reasonable care.

Put simply, this bill gives those who have no other alternative and are facing death, the ability to try and save their own lives. I ask you to please consider the passage of Assembly Bill 69. Thank you again for allowing me to submit testimony today.

Serving the 23rd Senate District

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Wisconsin Medical Society

Your Doctor. Your Health.

TO: Senate Committee on Health and Human Services
Senator Leah Vukmir, Chair

FROM: Mark Grapentine, JD – Senior Vice President, Government Relations

DATE: August 16, 2017

RE: Opposition to Senate Bill 84/Assembly Bill 69

On behalf of more than 12,000 members statewide, the Wisconsin Medical Society thanks you for this opportunity to share our testimony opposing Senate Bill 84/Assembly Bill 69, deemed "Right to Try" legislation. While the Society opposes the version of the bills before the committee today, we note that on August 3 the U.S. Senate amended a federal version of "Right to Try" that answers many of the concerns the Society raised when AB 69 was proceeding through the State Assembly. The Society believes the substitute amendment proposed by U.S. Senator Ron Johnson should also be incorporated into SB 84/AB 69. That amendment is attached to this memo.

Emotions surrounding the issue of terminal illness are powerful. Physicians understand all too well the tremendous toll a terminal illness can take on an entire family and how the desire to find a cure can become all-consuming. It is therefore understandable that someone suffering from a terminal illness or the family of that patient might want to turn to drugs that haven't yet made it through the typical U.S. Food and Drug Administration (FDA) review and approval process.

The Society's members sympathize with this desire – besides family members, a patient's physician is often the most trusted person when discussions take place about therapy options. Physicians also play an important role as patient advocates, including informing the patient and family members about what therapies and/or treatments are available in any particular case.

Physicians are also scientists, keeping abreast of the latest research and developments constantly abounding in the medical world. When serving as advocates, physicians rely upon the latest facts and data to make recommendations about treatments that could be effective for a patient's conditions. These data come from sophisticated clinical trials designed to weed out drugs that are dangerous, drugs that might be safe but don't work, drugs that might work but may have significant side effects for some, drugs that are "breakthroughs," seemingly beneficial to most patients with a minimum of negative impacts, and drugs that fall somewhere else in that spectrum.

The Society believes the FDA's current scientific methods and procedures are important for determining which drugs can serve the greatest good and which drugs should not be approved – even if a patient has a terminal illness. And while SB 84/AB 69 are genuinely promoted as legislation providing hope where little exists for an individual facing the worst circumstances, the Society believes the potential unintended negative consequences for society as a whole might outweigh supporting an individual's desires. The Society therefore respectfully opposes the bills in their current form and urges amendment.

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The FDA's Drug Development and Review Process is Scientifically Sound
Senate Bill 84/Assembly Bill 69 allow access to drugs that have passed a Phase 1 clinical trial. While this may sound rigorous to the layperson, it is far from it. According to the FDA a Phase 1 trial is generally conducted with healthy volunteers to determine toxicity – that is, whether the drug is minimally safe, not whether it is effective.[1] The FDA describes the typical Phase 1 sample size as being between 20 and 80 people.

Drugs surpassing this relatively low hurdle – a drug fails a Phase 1 trial if it is shown to have unacceptable toxicity – can then proceed to a Phase 2 trial. The FDA describes a Phase 2 trial thusly:

> Phase 2 studies begin if Phase 1 studies don't reveal unacceptable toxicity. While the emphasis in Phase 1 is on safety, the emphasis in Phase 2 is on effectiveness. This phase aims to obtain preliminary data on whether the drug works in people who have a certain disease or condition. For controlled trials, patients receiving the drug are compared with similar patients receiving a different treatment--usually an inactive substance (placebo), or a different drug. Safety continues to be evaluated, and short-term side effects are studied. Typically, the number of subjects in Phase 2 studies ranges from a few dozen to about 300.[2]

If a drug shows signs of effectiveness, Phase 3 trials can be scheduled. This type of trial involves many more people than the earlier phases – from several hundred to around 3,000 people – and studies different populations and different dosages while also determining potential drug interaction effects.

The FDA Has Worked to Accelerate the Drug Approval Process Where Appropriate
Critics of the FDA process cite the sometimes-lengthy process for new drug approval. In 2009 the FDA revised its regulations to allow for accelerated approval for drugs that can treat serious and life-threatening illnesses that don't currently have established medicines.[3] Often called the "compassionate use" or "expanded access" procedure, the FDA approves more than 99 percent of the applications it receives for such use.[4] Even then, the FDA continues to monitor effects of the drug, with the FDA holding the right to withdraw approval if the drug eventually proves ineffective or even harmful. Notably, the push for Right to Try predates the FDA's more recent "compassionate use" procedure, which pursues many of the same objectives in a more systematic and arguably safer manner.

The U.S Government Accountability Office (GAO) released a report[5] in July on the FDA data. Of 5,753 expanded access requests received between FY 2012-15, 5,697 were allowed to proceed. Response time was also encouraging: for those requesting emergency use of an investigational new drug (IND) the FDA responded in less than a day. All other IND requests were processed within 30 days, as required under updated regulations.

The U.S. Senate substitute amendment utilizes this expedited and responsive FDA process rather than create a mechanism to circumvent the FDA. The Society prefers the U.S. Senate's version.

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[1] https://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm
[2] Id.
[3] https://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm289601.htm
[4] https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm
[5] https://www.gao.gov/products/GAO-17-564

Allowing an Experimental Drug Process Outside the FDA Could Harm Scientific Studies
Because science is so important in the development of potentially-lifesaving drugs, it is important to promote as many thorough, rigorous studies as possible when exploring potential new medicines. By creating and promoting an alternative route to a drug, the Right to Try effort could ironically slow down the process for finding potentially life-saving drugs by siphoning potential participants away from scientific trials. The New York University School of Medicine Working Group on Compassionate Use and Pre-Approval Access takes a dim view of both Right to Try and even Compassionate Use procedures due to this risk of impeding quality drug studies:

> **How else could granting a dying person access to an unapproved medical product harm an ongoing clinical trial?**
> Several ways. If patients learn that pre-approval access is a possibility, they may not be willing to enroll in a clinical trial, fearing they'll receive a placebo or standard-of-care treatment instead of the desired product. If people had a choice to receive the investigational medical product they wanted through a compassionate use program or by entering a clinical trial, it is not surprising that they would choose the compassionate use program—after all, they would know for sure what they were receiving. While that may be the most rational choice for an individual, it could imperil the availability of patients for clinical trials, which would have dire consequences for the drug development process and for future patients.
>
> Many manufacturers have limited supplies of their investigational products, and granting compassionate use access can threaten those supplies. If they run out of supplies, manufacturers may have to scale back or suspend clinical trials. Especially with biologics, it is no easy task to "just make more drug." Some biologics can take more than a year to produce, and there are limited numbers of manufacturing facilities that can perform these complex tasks. Other drugs are hugely costly to make.[6]

The U.S. Senate's substitute amendment folds the Right to Try process into the FDA process and requires that a patient have exhausted approved treatment options and efforts to join a clinical trial. The amendment also promotes important data collection and requires the FDA to public an annual report summarizing any Right to Try experiences. The Society prefers these requirements.

There are Unanswered Questions Regarding the Overall Cost of Expanded Experimental Drugs
One aspect of Right to Try expanded access to experimental drugs concerns insurance. Simply put, if a patient suffers harm due to a drug taken via Right to Try, what are the implications for health care coverage of the additional costs borne due to taking the drug? While the bill provides extensive protections for a "manufacturer, distributor, pharmacist, practitioner, health care facility, or other person who lawfully makes available, delivers, distributes, prescribes, dispenses, or administers an investigational drug, device, or biological product",[7] left unanswered is insurance's responsibility for any additional costs.

The Wisconsin Medical Society deeply appreciates the spirit behind Senate Bill 84/Assembly Bill 69. Physicians join with policymakers in the desire to alleviate suffering and passionate pursuit of breakthrough drugs. Unfortunately, the bill's provisions could theoretically delay rather than promote those discoveries due to interference with current scientific standards, and questions remain about additional costs due to the potentially negative consequences caused by an experimental drug.

Thank you again for this opportunity to provide the Society's testimony on these bills. Please feel free to contact the Society on this and other health-related issues.

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[6] http://www.med.nyu.edu/pophealth/divisions/medical-ethics/compassionate-use/nyu-working-group-compassionate-use-pre-approval-access
[7] 2017 Assembly Bill 69, Assembly Substitute Amendment 1, page 4, lines 4-7

AMENDMENT NO._____ Calendar No._____

Purpose: In the nature of a substitute.

IN THE SENATE OF THE UNITED STATES—115th Cong., 1st Sess.

S. 204

To authorize the use of unapproved medical products by patients diagnosed with a terminal illness in accordance with State law, and for other purposes.

Referred to the Committee on _____ and ordered to be printed

Ordered to lie on the table and to be printed

AMENDMENT IN THE NATURE OF A SUBSTITUTE intended to be proposed by _____

Viz:

Strike all after the enacting clause and insert the following:

SECTION 1. SHORT TITLE.

This Act may be cited as the "Trickett Wendler, Frank Mongiello, and Jordan McLinn Right to Try Act of 2017".

SEC. 2. USE OF UNAPPROVED INVESTIGATIONAL DRUGS BY PATIENTS DIAGNOSED WITH A TERMINAL ILLNESS.

(a) IN GENERAL.—Chapter V of the Federal Food, Drug, and Cosmetic Act is amended by inserting after section 561A (21 U.S.C. 360bbb–0) the following:

"SEC. 561B. INVESTIGATIONAL DRUGS FOR USE BY ELIGIBLE PATIENTS.

"(a) DEFINITIONS.—For purposes of this section—

"(1) the term 'eligible patient' means a patient—

"(A) who has been diagnosed with a life-threatening disease or condition (as defined in section 312.81 of title 21, Code of Federal Regulations (or any successor regulations));

"(B) who has exhausted approved treatment options and is unable to participate in a clinical trial involving the eligible investigational drug, as certified by a physician, who—

"(i) is in good standing with the physician's licensing organization or board; and

"(ii) will not be compensated directly by the manufacturer for so certifying; and

"(C) who has provided to the treating physician written informed consent regarding the

eligible investigational drug, or, as applicable, on whose behalf a legally authorized representative of the patient has provided such consent;

"(2) the term 'eligible investigational drug' means an investigational drug (as such term is used in section 561)—

"(A) for which a Phase 1 clinical trial has been completed;

"(B) that has not been approved or licensed for any use under section 505 of this Act or section 351 of the Public Health Service Act;

"(C)(i) for which an application has been filed under section 505(b) of this Act or section 351(a) of the Public Health Service Act; or

"(ii) that is under investigation in a clinical trial that—

"(I) is intended to form the primary basis of a claim of effectiveness in support of approval or licensure under section 505 of this Act or section 351 of the Public Health Service Act; and

"(II) is the subject of an active investigational new drug application under section 505(i) of this Act or section 351(a)(3)

of the Public Health Service Act, as applicable; and

"(D) the active development or production of which is ongoing and has not been discontinued by the manufacturer or placed on clinical hold under section 505(i); and

"(3) the term 'phase 1 trial' means a phase 1 clinical investigation of a drug as described in section 312.21 of title 21, Code of Federal Regulations (or any successor regulations).

"(b) EXEMPTIONS.—Eligible investigational drugs provided to eligible patients in compliance with this section are exempt from sections 502(f), 503(b)(4), 505(a), and 505(i) of this Act, section 351(a) of the Public Health Service Act, and parts 50, 56, and 312 of title 21, Code of Federal Regulations (or any successor regulations), provided that the sponsor of such eligible investigational drug or any person who manufactures, distributes, prescribes, dispenses, introduces or delivers for introduction into interstate commerce, or provides to an eligible patient an eligible investigational drug pursuant to this section is in compliance with the applicable requirements set forth in sections 312.6, 312.7, and 312.8(d)(1) of title 21, Code of Federal Regulations (or any successor regulations) that apply to investigational drugs.

"(c) USE OF CLINICAL OUTCOMES.—

"(1) IN GENERAL.—Notwithstanding any other provision of this Act, the Public Health Service Act, or any other provision of Federal law, the Secretary may not use a clinical outcome associated with the use of an eligible investigational drug pursuant to this section to delay or adversely affect the review or approval of such drug under section 505 of this Act or section 351 of the Public Health Service Act unless—

"(A) the Secretary makes a determination, in accordance with paragraph (2), that use of such clinical outcome is critical to determining the safety of the eligible investigational drug; or

"(B) the sponsor requests use of such outcomes.

"(2) LIMITATION.—If the Secretary makes a determination under paragraph (1)(A), the Secretary shall provide written notice of such determination to the sponsor, including a public health justification for such determination, and such notice shall be made part of the administrative record. Such determination shall not be delegated below the director of the agency center that is charged with

the premarket review of the eligible investigational drug.

"(d) REPORTING.—

"(1) IN GENERAL.—The manufacturer or sponsor of an eligible investigational drug shall submit to the Secretary an annual summary of any use of such drug under this section. The summary shall include the number of doses supplied, the number of patients treated, the uses for which the drug was made available, and any known serious adverse events. The Secretary shall specify by regulation the deadline of submission of such annual summary and may amend section 312.33 of title 21, Code of Federal Regulations (or any successor regulations) to require the submission of such annual summary in conjunction with the annual report for an applicable investigational new drug application for such drug.

"(2) POSTING OF INFORMATION.—The Secretary shall post an annual summary report of the use of this section on the internet website of the Food and Drug Administration, including the number of drugs for which clinical outcomes associated with the use of an eligible investigational drug pursuant to this section was—

"(A) used in accordance with subsection (c)(1)(A);

"(B) used accordance with subsection (c)(1)(B); and

"(C) not used in the review of an application under section 505 of this Act or section 351 of the Public Health Service Act.".

(b) NO LIABILITY.—

(1) ALLEGED ACTS OR OMISSIONS.—With respect to any alleged act or omission with respect to an eligible investigational drug provided to an eligible patient pursuant to section 561B of the Federal Food, Drug, and Cosmetic Act and in compliance with such section, no liability in a cause of action shall lie against—

(A) a sponsor or manufacturer; or

(B) a prescriber, dispenser, or other individual entity (other than a sponsor or manufacturer), unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under any applicable State law.

(2) DETERMINATION NOT TO PROVIDE DRUG.—No liability shall lie against a sponsor manufacturer, prescriber, dispenser or other individual entity for its

determination not to provide access to an eligible investigational drug under section 561B of the Federal Food, Drug, and Cosmetic Act.

(3) LIMITATION.—Except as set forth in paragraphs (1) and (2), nothing in this section shall be construed to modify or otherwise affect the right of any person to bring a private action under any State or Federal product liability, tort, consumer protection, or warranty law.

SEC. 3. SENATE OF THE SENATE.

It is the sense of the Senate that section 561B of the Federal Food, Drug, and Cosmetic Act, as added by section 2—

(1) does not establish a new entitlement or modify an existing entitlement, or otherwise establish a positive right to any party or individual;

(2) does not establish any new mandates, directives, or additional regulations;

(3) only expands the scope of individual liberty and agency among patients, in limited circumstances;

(4) is consistent with, and will act as an alternative pathway alongside, existing expanded access policies of the Food and Drug Administration;

(5) will not, and cannot, create a cure or effective therapy where none exists;

(6) recognizes that the eligible terminally ill patient population often consists of those patients with the highest risk of mortality, and use of experimental treatments under the criteria and procedure described in such section 561A involves an informed assumption of risk; and

(7) establishes national standards and rules by which investigational drugs may be provided to terminally ill patients.

AMERICANS FOR PROSPERITY
WISCONSIN

Memorandum

TO: Members of the Senate Committee on Health and Human Services

FROM: Eric Bott, Americans for Prosperity State Director

DATE: August 16, 2017

RE: Support Senate Bill 84/Assembly Bill 69, Right to Try

Chairman Vukmir, thank you for holding this hearing and for the opportunity to provide written testimony. We also want to thank Senators Moulton and Risser and Representatives Snyder and Kleefisch for authoring this bill.

On behalf of the more than 130,000 Americans for Prosperity activists in Wisconsin, I urge you to support proposed legislation that will make Wisconsin a Right to Try state.

Americans for Prosperity-Wisconsin is the state's leading advocate for patient-centered, free market healthcare reforms aimed at increasing access to affordable, quality care for all Wisconsin families. Senate Bill 84 will help advance these goals by increasing access to treatment options for Wisconsinites who are battling terminal illnesses.

Every year, thousands of terminally ill patients are not allowed to access treatment protocols that have passed phase one clinical trials because these treatments have not yet received final approval by the Food and Drug Administration. In many cases, these treatments have already been approved and are being used effectively in other countries. In other cases, patients here in the United States have achieved favorable results while using these treatments as part of a clinical trial, but find themselves cut off from a therapy that works when their clinical trial ends.

Currently, terminally ill patients in Wisconsin only have two options if they want to gain access to an experimental treatment: they can join a clinical trial or apply for a compassionate use approval from the FDA. Neither of these options work well for many terminally ill patients. When operating clinical trials, researchers look for patients who meet very specific criteria. If a patient does not fall within these criteria – and most don't – they won't be entered into the trial. And even if they do get accepted into a trial, as previously mentioned, their access to the treatment is cut off when the trial ends.

The odds are even slimmer on compassionate use approvals. A highly bureaucratic, expensive, and drawn out process, only around 1,200 people each year are able to obtain a compassionate

use approval. Some of the nation's leading medical research departments are only able to obtain one or two such approvals each year.

This bill does not place any unfunded mandates on government healthcare programs, nor does it create burdensome new regulations or requirements for healthcare providers or insurers. This bill does not require public or private insurers to cover the cost of these experimental treatments, nor does it require healthcare providers to offer experimental treatments. Providers and drug manufacturers are also not held liable for the results of experimental treatments.

In Right to Try states, providers and patients both enter willingly into a treatment agreement with an understanding of the potential risks. And with the regulatory hurdles cleared away, many terminally ill patients are able to access even very costly experimental drugs with the help of philanthropic donations and other sources of private funding.

Terminally ill patients in Wisconsin deserve to be able to fight for their own lives, and with reforms like Senate Bill 84, they will have more available options to help them in their battle to extend their lives and improve their quality of life. This bill represents an important step forward in expanding access to potentially life-saving medical interventions for the Wisconsin patients who need it most. Americans for Prosperity strongly supports its passage, and we look forward to working with you in the future.

Americans for Prosperity (AFP) exists to recruit, educate, and mobilize citizens in support of the policies and goals of a free society at the local, state, and federal level, helping every American live their dream – especially the least fortunate. AFP has more than 3.2 million activists across the nation, a local infrastructure that includes 36 state chapters, and has received financial support from more than 100,000 Americans in all 50 states. For more information, visit www.AmericansForProsperity.org.

August 16, 2017
Conference Room 411
To: Senate Committee on Health and Human Services
Sen. Vukmir, Chair

From: Rare Disease United Foundation
Courtney Waller, Director

Senate Bill 84

In Support

Thank you for having me this morning. My name is Courtney Waller. I am a Williams Bay, WI resident and mother to Theodora, age 3 who was born with Timothy Syndrome, an ultra rare genetic disorder which is hallmarked by neurological and cardiac issues, affecting only 20 children in the United States. I am also the Executive Director for Rare Disease United Foundation. Today, I speak to you, not just as the mother of a child who has already surpassed her life expectancy, but, on behalf of the rare disease community here in Wisconsin.

Right to Try legislation is important to patients for many reasons, and today I would like to touch on a few of them.

The rare disease community is unique in the fact that with over 7,000 rare diseases recognized by the National Institutes of Health, many in our community are part of patient populations that number only in double digits. We have a child in WIsconsin who is the only person in the world with his diagnosis and genetic mutation. Collectively, there are 30 million people living with a rare disease in the United States. Approximately half of those patients are children, 5 million of whom will not live long enough to attend kindergarten.

For patients in the rare disease community Right to Try becomes important because we can often be left out of clinical trials due to the very specific mutations that cause our diseases, or the very specific clinical trial guidelines. When my daughter was born, with an arrhythmia known as Long QT, prior to her full diagnosis of Timothy Syndrome, there was only one medication that seemed to correct the issue; Mexitil. In less than 24 hours after starting Mexitil, Theodora went from having a QT number of over 600 down to 480. The QT number in a average person in around 450.
Although already FDA approved by the time my daughter needed the medication, the story of Mexitil is a prime example of how Right to Try and the ability to bring drugs to mass market can work hand in hand. In clinical trials, Mexitil was tested only in 483 subjects, in either a one month or 3 month controlled study. However, over 10,000 patients were able to access the drug outside of these trials. The drug was subsequently approved by the FDA, and was, quite honestly a game changer in our ability to manage my daughter's disease.

Accessing medications through Right to Try is not, as some have said the return of the "snake oil salesman". The decision to access treatment in this manner, in our community, means that very well informed patients have exhausted all other options. They, along with their doctors, have determined that trying an experimental treatment is the best hope.

Legislation like Right to Try, means, at its very core; self determination for patients. In the ethical debate on Right to Try, a picture is often painted of patients as desperate and gullible; ready to accept any sliver of hope, even against better judgement. This is not only untrue, but insulting to our community. While I will not go into great detail, as a mother to a very sick little girl, I know more about her disease, at both the genetic and symptomatic level than most doctors. As most rare disease patients will tell you, this is knowledge we must have, if we wish to survive or to help our children survive and live their best lives.

When attempting an experimental treatment, we are not blindly walking with hope, but fully informed that there may be unintended consequences. We accept the possibility of these consequences, armed with the scientific knowledge of our specific disease. We accept them with the belief that we have a right to fight to survive, that we have a fundamental right to make these kind of decisions and determine our own fate.

Right to Try is the right policy to help a community of patients who are often left behind in the clinical trial process and have exhausted all other options. Many in the rare disease community have benefitted from the ability to access medications outside of the clinical trial process, either directly, or like my daughter, indirectly.

As a mother, and on behalf of Rare Disease United Foundation, I believe that the patients have the right to self-determination in their care. Right to Try is the right step to uphold this ideal when all other options have been exhausted.

08/16/2017

Good morning Senators. I am honored to be here today to share my experiences and thoughts on the assembly bill 69, the Right to Try law before this committee. My name is Juran Cook and I am a lifelong Wisconsin resident currently residing in New Berlin, Wisconsin, and live in the chairwomans district.

I have been a registered nurse for 32 years and have cared for many people throughout my career as a critical care nurse. I have cried tears of joy as a witness to some miraculous recoveries against all hope and with tears of sadness I have held the hand of many patients and families as their journey on earth ended. My greatest challenge however is a personal one. I share this journey in honor of my husband Mitch Cook.

My husband was an avid outdoorsman. Fishing, golfing ,weather and music were his passions. He was hardworking and spent his entire life in retail management. 3 years ago he came home from helping a friend paint and said "I dropped the paintbrush 3 times today " Now anyone married to a nurse knows you can't tell us these things and excpect us to ignore them. Mitch went through a series of doctor visits, testing and specialists and on June 12th , 2014 we were in the neurologist's office to get all the results. - He sat us down and somberly told us –

You have ALS. It is also known as Lou Gehrig's disease. There is no treatment for it. There is no cure. There is 1 medication that may prolong your life by 3 or 4 months. Your life expectency with ALS is 3 to 5 years. The words hung in the air like in one of those cartoon balloons. He repeated it - you have ALS , there is no treatment , there is no cure and your life expectency is 3 to 5 years. Do everything you want to do while you still can. We left devastated, Mitch was only 55 years old.

After the shock began to wear off we were determined that we would beat the odds. We were so full of hope. Surely there are new treatments to try. Then the ice bucket challenge came along . Surely now this under funded disease would make great strides. I spent the next few months researching any treatment options I could find. We traveled to Northwestern memorial in Chicago for his care . Much to my suprise there was so little available. Either the studies were underway or with the flood of money into ALS research now they were just starting to develop studies. We went to hear Dr. Clive Svendson speak. He was at UW Madison for over a decade researching stem cell and gene therapy and had recently relocated to California to pursue his research . His treatment sounds quite promising, the first in the nation to combine stem cell and gene therapy for halting the progression of ALS. That was in June of 2014. We were hopeful again that Mitch would be able to particiapte in this very promising treatment.

8 months after diagnisis Mitch had a feeding tube placed as he could barely eat and had already lost 50 lbs. By now his speech was almost gone, he no longer had the use of his right arm , his left arm was getting weaker and walking was becoming a challenge without assistance. He needed to use a breathing machine at night. And we waited for Dr. Svendon's trial to begin.

11 months after diagnosis he could no longer walk, use his arms, very little speech, and breathing was worsening daily. He was now in a wheelchair and depended on me for all of his needs. Yet , despite all of this, he remained hopeful , his motto became "Never give up". I would come home from work and he would say please look into this new drug they're talking about for ALS or please find out about this new breathing device for ALS. But by now his breathing had worsened and sadly he no longer qualified for most of the studies that were happening. Yet he never gave up hope, he said I won't go down without a fight. It's

not in my DNA to give up. And we waited for Dr. Svendon's trial to begin.

2 years after disgnosis he was no longer eating, drinking, moving , his speech was gone and he had to wear catheters. Pain and anxiety were his constant companions, fearful that at any moment he would stop breathing or I wouldn't hear his computer cries for help. All he had left was communication through his eye gaze computer , his sense of humor and hope. And we waited.

Every October there is a walk for ALS. Last October Mitch insisted I get our team "Snappers Strutters" back together and fundraise for ALS . By now he was weaker and his breathing was deeply affected and we weren't sure if he could attend the walk or not. When the day came we were thrilled that he made the whole 2 mile walk in his wheelchair surrounded by friends and family and love,laughter and a few tears. The next day he thanked me for getting our team together and how proud he was of the money we raised. He told me that he now knew it was too late for him but if he could raise money to help anyone else not have to suffer like he did then he was going to do it. This was the first time that I realized he had given up hope. My heart broke as he told me he was at peace with dying.

One week later on October 17th 2016, my beloved MItch died peacefully at home in our bed in my arms. He fought a courageous battle for 2 1/2 years and was only 58 years old. Ironically 3 days later Dr. Clive Svendson got the FDA approval to begin phase 1 /2A clinical trails for the stem cell / gene therapy combination that 2 years prior we had pinned our hopes and dreams on, 13 years after it's inception. This study is enrolling patients right now.

The Right to Try act is a call for compassion and reason. For rapidly fatal diseases like ALS and others that lack treatment options, there are promising therapies that are simply inaccessible. They are stuck in a drug approval process that dates back to 1962. In 2012 Congress directed the FDA to utilize their accelerated approval program for fatal diseases

lacking treatments but they are still resisting this directive.

The Right to Try bill provides the necessary legal protections that only applies to products in the FDA approval pipeline and in the very specific case of a terminal illness lacking life saving treatments. Without these protections companies are largely unwilling to make their treatments available. There are already life saving treatments approved in Japan, Europe and elswhere that we cannot legally obtain here. Radicava which was just approved in the US this year has been in use in Japan for almost a decade. A privileged few are taking up residence in foreign countries in an effort to save their own lives. We would have done this if we had the money.

Mitch wanted to try promising new treatments , knowing fully that there would be risk. He could accept failure but he couldn't accept not trying. He wanted the right to try to save his own life.

We live in a country with extraordinary medical capabilities, I see this every day as a nurse. But we are being denied access to the only lifelines we have- therapies showing promising benefits in clinical trials. Unquestionably we need the FDA to protect public health, but we also deserve an FDA that is reasonable and responsive, especially in the case of fast moving fatal diseases that lack approved treatments.

Desmond Tutu said " Hope is being able to see that there is light despite all of the darkness" .

Please support this bill and be the light that shines through our darkness.

Thank you for your attention and consideration.