HB

43

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43</SUBJECT><COMM>HHSS30</COMM></TARGET>

ALASKA STATE LEGISLATURE

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REPRESENTATIVE JASON GRENN

SPONSOR STATEMENT

House Bill 43

The Right to Try: New Drugs for the Terminally Ill

"Patients should be free to exercise a basic freedom – attempting to preserve one's own life."
- Christina Corieri, Health Care Policy Analyst

House Bill 43 would allow terminally ill patients who have exhausted other available treatments and do not qualify for clinical trials to gain faster access to safe, but experimental drugs in an effort to save their own lives. By providing certain immunities to prescribing physicians, manufacturers and distributors acting in good faith, this bill would allow terminal patients, in consultation with their doctor, the freedom to try new treatments as they fight to survive, without the burden of waiting for federal approval.

More than 1 million Americans die from a terminal illness every year. Many spend years searching for a potential cure, or struggle in vain to get accepted into a clinical trial. Unfortunately, FDA red tape and government regulations can often restrict access to promising new treatments, and sometimes for those who do get access, it is too late.

The United States Food and Drug Administration currently offers an "expanded access" or "compassionate use" exemption that allows terminally ill patients that meet certain criteria to access drugs in the clinical trial phase. However, even with recent efforts by the FDA to streamline the application process, this exemption program is known to be arduous and can take longer than patients facing terminal illness have to wait.

Since 2014, 33 states have signed "right to try" legislation into law with strong, bi-partisan support. It is clear this is a human issue that goes beyond state and party lines, and could provide Alaskans increased access to potentially life-saving treatments in the times they need it most.

In allowing Alaskans facing terminal illness the ability to access safe, though experimental drugs in consultation with a doctor they trust, this bill offers new hope when all FDA-approved options have been exhausted. I urge your support of House Bill 43.

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SECTIONAL ANALYSIS

House Bill 43 ver D

The Right to Try: New Drugs for the Terminally Ill

Section 1: Prohibits disciplinary action of physicians by the State Medical Board for prescribing, dispensing or administering an investigational drug, biological product or device to terminally ill patients that are ineligible or unable to participate in a current clinical trial, have considered all other treatment options approved by the FDA and have provided written consent.

Defines "investigational drugs, biological products and devices" as those that have successfully completed Phase 1 of the FDA drug review process and remain in ongoing Phase 2 or 3 clinical trials, but have not been approved for general use.

Defines "terminal illness" as a disease that will result in death in the near future or permanent state of unconsciousness from which recovery is unlikely.

Section 2: Establishes immunity for physicians, medical team members, manufacturers and distributors in the case of injury or death of a terminally ill patient from the use of an investigational drug, biological product or device, provided informed consent was obtained from the patient and notice of immunity was given in advance.

Establishes immunity for physicians and manufacturers who choose not to participate in the distribution of an investigational drug, biological product or device.

Section 3: Amends statute limiting the sale and distribution of new drugs (AS 17.20.110) so as not to apply to physicians prescribing or administering investigational drugs under the conditions established in Section 1.

Section 4: Prohibits the Department of Health and Social Services from requiring a licensed health care facility to increase its services solely to accommodate physicians prescribing, dispensing or administering investigational drugs to a patient.

Clinical Trials

A student's interviews of nurses, patients and survivors experience and opinions of cancer and cancer clinical trials in Alaska.

Written by: Jennifer Kiser MSW | Edited by: Amanda McDade & individual interviews final edited by Interviewee.

Introduction

Clinical Trials in Alaska

This booklet combines a student's interviews of nurses, patients and survivors experience and opinions of cancer and cancer clinical trials in Alaska.

A clinical trial is a research study to answer specific questions about vaccines or new therapies or new ways of using known treatments.

Clinical trials (also called medical research and research studies) are used to determine whether new drugs or treatments are both safe and effective.

Contents

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Krista Rangitsch

Research Nurse, Providence Cancer Center

Krista Rangitsch, a research nurse at Providence Cancer Center, works closely with doctors and their patients by providing them with information on clinical trials. She explains that "cancer clinical trials are research studies involving human subjects that look at ways of preventing, detecting, and/or treating cancer in the hopes of improving over all survival and patient quality of life. These trials help doctors find better ways of improving cancer care by answering certain scientific questions."

"Clinical Trials ... look at ways of preventing, detecting, and/or treating cancer."

She also explained "There are a variety of different ways cancer clinical trials are designed. Some trials look at new experimental treatments for cancer, while others look at treatments that are already approved by the United States Food and Drug Administration (FDA) for one type of cancer but are being studied in another type of cancer. Alternatively, clinical trials can compare the difference between two or more treatment regimens (i.e. medication "A" compared to medication "B"). Some trials investigate administering an FDA approved medication on a different schedule (i.e. once a week versus every three weeks) or in a different manner (i.e. oral versus intravenous)."

When discussing with a patient potentially interested in participating in a cancer clinical trial, Rangitsch informs them of the risks and benefits of participating and that their insurance may not cover some or all of the care associated with the clinical trial. Acting as an intermediary for the patient, Rangitsch will often contact their insurance provider to request coverage for the routine care costs associated with the clinical trial. Generally the items considered to be investigational are provided at no charge to the participants or their insurance company.

"These trials help doctors find better ways of improving cancer care by answering certain scientific questions"

Some insurance companies have been more difficult to work with than others. One company in particular refused to pay for routine care costs of a patient considering participating in a clinical trial for

her soft tissue sarcoma. Rangitsch stated that the sponsor of the study was willing to "pay for everything outside the standard of care"; meaning that all extra costs would be paid for. The only thing the insurance company was being asked to cover were the routine care costs associated with standard sarcoma care follow-up, which they would have incurred even if the patient was not enrolled in the clinical trial. The patient was very frustrated, saying that she has worked her entire life paying for health insurance coverage and expected that it would be there when she need it. Now, because they will not pay for routine care costs during this clinical trial she is actually considering paying for her treatment out of pocket.

"Clinical trials are the only way to get new drugs approved"

Other insurance companies have made things very easy. Rangitsch mentioned that working with one company in particular has been a great experience; they cover the routine care cost during a clinical trial and the process for approval is clear and simple. Rangitsch has testified as a private citizen in support of Senate Bill 10 which would mandate that insurance companies in Alaska cover the routine care costs associated with a clinical trial. Creating equal access to clinical trials is "so important because it is currently a huge barrier. Clinical trials are how we improve and advance medicine, and clinical trials are the only way to get new drugs approved."

Kathy

Clinical Trials Story

Kathy found a lump in her breast but was not alarmed by it, it just seemed kind of weird that there were little red dots on the outside of the breast and there was something on the inside that was a little bit hard. She thought that cancer was large, defined, marble size lumps, not something like this, so she thought it just might go away and went back to her busy life taking care of her children and supporting her husband. Kathy stated "I wasn't thinking about myself," too much seemed to be going on for her to worry about something that didn't seem like cancer.

Several months later she noticed the lump was still there, and her body started to get progressively weaker. She tried to make an appointment at the local Breast Cancer Detection Center for a mammogram, but was told she needed a prescription. Her insurance company insisted that she go to the urgent care clinic for doctor visits, so she decided to go there hoping they could give her a prescription. Still not too alarmed, because throughout her life she had taken precautions to reduce the risk of cancer, Kathy was checked by an urgent care doctor. Because the urgent care center doctor wouldn't give her a prescription for a mammogram, the Dr., with urgency in her voice, made an appointment for Kathy to be seen by a gynecologist - that hour! It was then that she experienced fear for the first time. She drove straight to the gynecologist's office, and was examined. He stated that he would call and order a mammogram and ultrasound, and that she should make the appointment as soon as possible. She went home a little shook up, and thought about how scared she was over the possibility that she might have breast cancer. Finally, after several days, she got enough nerve to call and make an appointment. However, the next available appointment was three weeks out, which gave her even more time to be frightened.

"I wasn't thinking about myself," too much seemed to be going on for her to worry about something that didn't seem like cancer.

During the mammogram, the radiologist just kept taking pictures. As he looked at the ultrasound he said he thought the mammogram looked "suspicious." Kathy said "I felt sick to my stomach." Not wanting to delay anything further, the radiologist set up an appointment for a biopsy later that week. Since they

were already booked solid, he set it for a time that was outside the normal "biopsy schedule." It was for 7 AM — a time that would be most inconvenient for her, because she had four kids at home to get ready for school. Unable to bare the thought of explaining to her kids why she had to leave so early in the morning, she got up in the middle of the night and left while everyone was sleeping. She left notes for her husband and kids telling them she had gone to her friend's house. Unbeknown to anyone, her friend was going to accompany her to the appointment. The next morning, the gynecologist called her while she was at school with her youngest daughter; he told Kathy it was a malignant tumor.

"I felt sick to my stomach."

According to Kathy, she got up, kissed her six year old daughter, and told her she'd still be at the school, but would be outside. She went to her car, and cried. After a while, she called her mother, her husband, and two of her friends. One of them came up to the school and sat with her in the parking lot — they both cried. She knew she had to collect herself because in just a little while school would be out, and not just for the day; it was the last day of school before summer, and there were year end traditions to uphold.

"Not knowing what her future held, she wondered if this would be the last time she'd ever see her kids poking their heads out the bus window, waving goodbye for the summer."

Things weren't going to change just because she had breast cancer. At her kids' elementary school, on the last day of school, once the kids board the buses for home, they do a bus parade and circle the parking lot several times. The entire school staff comes outside and waves goodbye to the kids. With four kids, this was a tradition she hadn't missed in ten years. She tried to pretend things were perfectly normal but did not do very well. Instead, she felt very emotional.

Not knowing what her future held, she wondered if this would be the last time she'd ever see her kids poking their heads out the bus window, waving goodbye for the summer. Her other daughter's teacher noticed she was teary eyed. Unable to speak at first, she finally got the words out – she was "just diagnosed with breast cancer three hours ago." As difficult as it was for her, she was trying to hide her emotions. She surely didn't want her two youngest daughters seeing her cry. She remembers him putting his arm around her, trying, in his most reassuring tone,

"Kathy wanted to do something... she found a press release on the Zometa clinical trial and showed it to her doctor."

to tell her that she would be okay. The most difficult part for Kathy was the fear of what would happen to her kids if she died.

After visiting the surgeon she felt hopeful because the tumor was less than two centimeters. Things were looking better, and the surgeon was very encouraging. After more than a week of not being able to eat, or even drink water, she was finally able to eat, and enjoyed a plate of fettuccine, which is her favorite food. She had already lost more than five pounds by then. Kathy remembered thinking how she wanted to go on a diet that summer, but the cancer diet was never in the plan.

Kathy wanted to do something. Shortly after her diagnosis, she heard about the drug Zometa. Manufacturer's had been working with Zometa, and found that the drug that has shown positive correlations with fewer reoccurrences of breast cancer. At the time, her Dr. told her Zometa was normally used to treat bone cancer, and they had not heard of it being used to treat breast cancer. A couple of weeks later, at the ASCO Conference in Chicago, it was announced that Zometa had shown promise is a limited clinical trial. Kathy found a press release on the Zometa clinical trial and showed it to her doctor. Her internet research quoted one doctor to say that Zometa will "probably become the standard of care."

It took a lot of thought to make sure it was something she would want to do and wondered if it would be beneficial.

After several months into chemotherapy, Doctor Cox mentioned the Zometa study was expanding and she would be a likely candidate. She advised her to read about the clinical trial to see if it was something she really wanted to pursue. This trial was a much more aggressive treatment than what she had heard about in the previous study. It took a lot of thought to make sure it was something she would want to do and wondered if it would be beneficial. Her initial reasons for participating in the clinical trial were so that she would receive additional treatment. It seemed as though the additional drug would be beneficial. After careful consideration, Kathy decided to enroll in the clinical trial. She was hoping to be randomized to the Zometa arm of the study. Instead, she was selected to take Clodronate, which is not approved in the United States. The three drugs within the study are all in the same drug family and, to her knowledge Clodronate has only been used in the UK, Canada, and Italy. This led her to weigh her options to see if she wanted to drop out or remain in the study. Kathy knew remaining in the study was optional. She began to research Clodronate but she could not find much information about it. What she did find she was conflicting, and there were not any significant end result findings from using this drug. The study is looking at its effectiveness in the reoccurrence of cancer as compared to the other two drugs, Zometa being one of them. Kathy said "I will just go ahead and do it. There are thousands and thousands of women before me that

this was not even offered to, and it has promise. I am fortunate to have this option. A year ago, I would not have had the option to be on this drug." She now has follow-up care scans which would have not been done otherwise. According to Kathy "people are going to monitor me for a long time." In Kathy's case, the standard of care was going to be less than what she will get from the clinical trial.

It was not easy to get on this study. Kathy encountered several challenges when dealing with her insurance company even though she pays $900 dollars a month for health insurance coverage. They denied her requests to participate in the study three times. That was no surprise to her - they denied more than half the cost of her surgery too, possibly due to doing a double mastectomy, rather than the suggested lumpectomy or single mastectomy. Kathy researched her cancer type and felt there was a high chance of the cancer occurring in both breasts, so she opted for the double mastectomy to reduce her risk of reoccurrence. In Kathy's clinical trial, only the drug is paid for by the manufacturer, the follow up care is not. Without approval, remaining on the study would not be an option for her because she could not afford it on her own. Finally, with only three days to spare in the 8 week window, they agreed to cover the costs associated with it.

"There are thousands and thousands of women before me that this was not even offered to, and it has promise. I am fortunate to have this option. A year ago, I would not have had the option to be on this drug."

Kathy continues to fight her breast cancer, and is learning to live with the diagnosis. As the interview ended, she stated "Where ever you go, there you are" because she can't get away from herself. Her diagnosis will follow her wherever she goes. She left me with a quote that she often tells her daughters "The sky is always blue above the clouds. Its always sunny somewhere." This left me with an understanding that Kathy is a fighter, looking for a silver lining in a sky full of gray.

"The sky is always blue above the clouds. Its always sunny somewhere."

Claire

Clinical Trials Story

Claire's sister had breast cancer eight years ago. When Claire found out she too had breast cancer, it was shocking but not surprising. It was found during a mammogram and after three scans, the doctors found the cancerous cells. Claire ended up having a mastectomy which found a small node of cancer. Doctors recommended that she also undergo radiation and chemotherapy but Claire chose not to do either. According to Claire it took a lot of research and studying statistics to figure out her chances of survival.

It took a lot of research and studying statistics to figure out her chances of survival.

In 2006, Claire had the opportunity to be enrolled in a clinical trial for bone strengthening, and she thought it would be beneficial. The trial had several different groups which would be receiving different forms of treatment. Claire would be randomly assigned to a group, but because of her fear of needles, she projected which group she would be in. Just as she had guessed, she was assigned to be in the only group that required an IV. At first, Claire was going to the hospital quite often to have treatments done. As time progressed, she had the IV treatments less often and did not require as many hospital visits. She has had no side effects from the IV treatments unlike the other groups who received different drugs. Because Claire chose to take part in the clinical trial she will receive lab tests and doctors visits to monitor the effectiveness of the treatment for years to come. Claire is convinced that this clinical trial will help her bone strength and decrease the probability of bone cancer.

Not only did Claire receive numerous personal benefits from the clinical trial, she also helped advance research.

Even though there were several positive aspects to the clinical trial, Claire did encounter one problem. During the clinical trial the insurance company would only cover a part of routine care cost during the clinical trial even though some of the treatment options on the clinical trial were well established and widely prescribed in other countries. When Claire's sister went through treatment, she was living in Britain and did not encounter any problems when participating in research. She received fabulous care with universal health coverage.

Claire — Clinical Trials Story

Not only did Claire receive numerous personal benefits from the clinical trial, she also helped advance research. Claire thinks of herself as a statistic that could be measured and studied through her clinical trial journey. It is important to have these statistics and patients associated with new treatment options in order to give the treatment more credibility and help it become well-established. As Claire stated, "How do we find out about new drugs or treatments if we do not conduct clinical trials?"

"How do we find out about new drugs or treatments if we do not conduct clinical trials?"

Connie

Clinical Trials Story

In early 1999, there was a lump on the side of Connie's neck. The lump did not come with a cold or any sickness, so she let it go. Three months later the lump had not gone, yet there was still no pain, illness or anything substantial that would cause her to worry. As a precautionary measure she went to her doctor and received blood work.

In April 1999, Connie was diagnosed with Non-Hodgkin's Lymphoma, a cancer that affects the immune system and the bodies' ability to fight infection. She learned that this type of cancer is less aggressive than Hodgkin's Lymphoma; however, there is also no cure.

Non-Hodgkin's Lymphoma, a cancer that affects the immune system and the bodies' ability to fight infection.

Through the use of her computer and her husband by her side, Connie learned as much as she could about the Non-Hodgkin's' Lymphoma which helped ease some fears. She found that this type of cancer is slow growing which gave her time to look at various treatment options and how the disease progressed in the body.

By looking at treatment options for this type of diagnosis, Connie learned that it would be treated with chemotherapy. As Connie continued to learn about her disease she discovered a clinical trial her doctor had mentioned from the National Cancer Institute. She would be able to advance medical treatment through this clinical trial. The vaccine study would take a sample of one of Connie's nodes and mix it with another enzyme to try to create a vaccine which would attack her specific cancer. According to Connie the study "gave me hope for a possible cure," and although the study was a double blind study, she would be able to take part in possible advances in cancer research. In this study Connie will be followed for the next ten years which is much longer than a person who just receives standard treatment.

Clinical trials "gave me hope for a possible cure."

Statistically 30 to 40 percent of those with Non-Hodgkin's Lymphoma convert to a more aggressive type. Connie's did while on vacation in New Zealand in 2004. She began to have severe stomach pains and flew to Australia for a scan. The scan showed that her belly was full of cancer. Connie chose to come back to Alaska for treatment. At this point the cancer had blocked

the blood flow to the legs which caused a clot. The clot then traveled to her pulmonary artery which caused a pulmonary embolism, leaving her weak, short of breath and her body atrophied. The doctors suggested that she begin chemotherapy. After treatment she felt much better however the doctor suggested a stem cell transplant which sifts old and new cells, and stores the new cells. She received a high level of chemotherapy treatment with the hope of killing off all cancer affected cells in her body & essentially destroying her immune system. Then, they reintroduce the saved stem cells which were also treated with chemotherapy and put back into her body with the hope of introducing a healthy immune system. About this time she was offered a second clinical trial. The new trial involved a medication that would help to stimulate the mucus membrane cells in the mouth. This trial would help those who receive chemo have less pain and sores in the mouth. In Connie's case, the drug helped reduce the number of days with sores by a day or two.

Connie chose to come back to Alaska for treatment.

Connie also participated in a third clinical trial which was very different from the first two. The researchers conducted a psychological study to gage feelings about the stem cell treatment before and after the treatment. It gave her the opportunity to find how her feelings have changed over time. The study also gave researchers insight on mood and attitude during and after treatment.

A third Clinical trial...

Throughout the three clinical trials Connie was worried about insurance covering the costs. But the National Institute of Health paid for all of the clinical trials and gave her a partial stipend for travel, room, and board. All of her clinical trials had to be preapproved by the insurance company and she worries that the insurance she has will soon run out because the cost of treatment is so expensive.

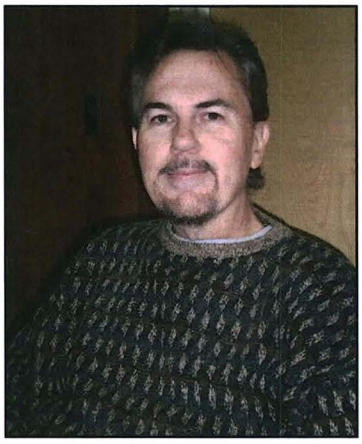
Clinical trials "made me feel like I was advancing medical treatment and patient care."

Connie feels supported by her family, friends and doctors. She stated the clinical trials "made me feel like I was advancing medical treatment and patient care." She is encouraged by clinical trials and feels like there are not nearly enough people in them. After all, no cancer advancement can occur without participation in clinical trials. Connie also feels that each person has to choose for themselves if they want to participate, weighing the benefits and risks of the trial.

Each person has to choose for themselves if they want to participate, weighing the benefits and risks of the trial.

Dennis

Clinical Trials Story



Late summer early fall of 2007, Dennis started to notice a change. It was prostate cancer, not that he knew it at the time. He had problems urinating and a sore left shoulder. The year also involved a broken ankle, which took several months to heal, and continual shoulder pain. By early April of 2008 Dennis became very ill. His roommate was very concerned and convinced him to see a doctor. After the appointment was set up, Dennis began to feel better so he thought about just skipping it because going to the doctor seemed like overreacting. Though he was feeling better, he kept his appointment. The doctor gave him several blood draws and he waited to hear the results from the doctor's office.

Dennis stated "On April 9th 2008 I was told I had kidney failure." He was directed by his primary care physician, Dr. Reeves, to report immediately to the hospital emergency room. Dennis went through a series of tests and was then admitted to Providence Hospital where he remained for one week. Further testing revealed that prostate cancer was blocking the urinal tubes. Short term treatment for the kidneys was dialysis for several days a week for up to a year. Stents were placed to drain the kidneys and they were so effective that his dialysis treatment was rescinded.

Dennis stated "I had Stage 4 prostate cancer; Dr. Ferucci, my urologist, explained that normal PSA (Prostate Specific Antigen) levels range from .1 to 4, and my PSA level was well over 400." Dennis was ready to fight prostate cancer. Dr. Ferucci suggested hormone treatment which is the standard treatment for those with stage 4 prostate cancer. This treatment halts testosterone and attempts to prevent the cancer from growing. A group of medical professionals, including a urologist, oncologist and hospital staff teamed up to help Dennis fight his cancer. They began hormone treatment which consisted of monthly injections of Lupron along with an oral medication called Casodex which is consumed once a day. In addition, they began radiation treatment in both shoulders and the left and right femur areas to reduce the heavier concentration of the cancer which had spread to other parts of his body. The hormone treatment was very effective and within 30 days Dennis' PSA level

"I had Stage 4 prostate cancer."

Dennis was ready to fight prostate cancer.

dropped to around 20. Within another 60 days his PSA level was below 4.

Dr. Ferucci introduced Dennis to Krista a research nurse who knew of a clinical trial for prostate cancer taking place in Alaska. Dennis jumped on board "not in an effort to get better, but to participate in research. Prostate cancer research is ten years behind that of breast cancer, and men are less likely to participate in clinical trials." The clinical trial utilized a new type of drug substituting Lupron to Zoladex. The use of Zoladex was monitored through blood draws. After PSA levels began increasing it was decided by Dennis and his doctor that removal from the study was the best option.

Prostate cancer research is ten years behind that of breast cancer, and men are less likely to participate in clinical trials."

During this clinical trial, Dennis incurred very little out of pocket expenses because his insurance company was very supportive by covering the cost of his routine care during the clinical trial.

After the clinical trial, Dennis' medications were returned to current treatment options. He plans to bring his team back together to look at treatment options for cancer in his femurs, along with trying a new diet plan for a more natural treatment option.

Though Dennis has gone through a very difficult time his presence is felt when he walks into a room. He has personal strength and support from his friends. Through this difficult time Dennis is encouraged that research will help future generations find better options to cancer treatment.

Hannah

Clinical Trials Story

Brice Smith, a cancer research nurse, screens people to determine whether or not they are eligible for clinical trials. She educates staff and patients on what clinical trials are. When patients are enrolled in a clinical trial she is the liaison and case manager during the trial.

During one experience in attempting to enroll a patient in a clinical trial the patients' physicians' office staff and the data center staff, both called the insurer to attain coverage and both were declined three separate times. The data center staff contacted Hannah regarding the insurance companies' decline of coverage for the patient during the clinical trial. When Hannah contacted the insurer they referred her to the case management group where she spoke with a lead case manger who would review the patient's file. The lead case manger stated the insurance company was "emphatic" that no payment for treatment would be provided while the patient was participating in clinical trials and none of the clinical trial would be paid for. Hannah then asked the lead case manager for something with the insurance companies' logo on it stating the reasons the client was declined, because the Clinical Trials Workgroup is gathering documentation for legislation. The lead case manager from the insurance company called back three days later stating the company needed more information in the form of a letter on some of the benefits of the clinical trails for example: while in the clinical trial all medications will be paid for. After sending in the letter identifying the benefits of the clinical trial an approval was granted within 48 hours.

The best care a patient can receive is when enrolled in clinical trials.

Attaining coverage for this one patient was five weeks of work; the amount of money spent in time to get the coverage would have cut the cost of insurance company's time and the cost of time for hospital staff.

Hannah pointed out that NCI released a statement that the best care a patient can receive is when enrolled in clinical trials. Under the scrutiny of clinical trials patients are monitored more closely. This particular patient will receive ten years of follow up care. By participating in a clinical trial, this person is on the radar when they would not normally be on the radar.

Judy

Clinical Trials Story

For over fifteen years doctors have told Judy that she had a fibroid in her pelvis. In the summer of 2006 it started to interfere with the flow of her urine, the kidney was not draining properly. It was probably never a fibroid, she was diagnosed with Leiomyosarcoma. It was probably a low grade cancer but somehow it had kicked up into high gear and spread. The cancer Judy has is very rare and there is not much research on it, she states "the research needs to get done."

"The research needs to get done."

During a hysterectomy the doctors did not do anything to the tumor but they urged her to go to a cancer center and get an appointment with an oncologist. She stated "it was difficult to get an appointment" and after contacting several hospitals she decided to go to Memorial Sloan Kettering Cancer Center. So Judy went to New York and was seen by a surgical oncologist who told her she needed to shrink the tumor before they removed it. She flew back to Alaska and had four rounds of chemotherapy, she went back and forth from Alaska to New York before the surgical oncologist and urologist at Sloan Memorial felt that they should go forward with surgery because the cancer was not diminishing in size.

According to Judy, Memorial Sloan did an incredible job. She returned home receiving cat scans every four months in Alaska and once a year in New York. In January of 2008 Judy learned that the tumor had returned, two more nodes were present in her right lung, she was able to have the two nodes removed. She was sent home for radiation with surgery on the primary pelvic tumor scheduled for the end of May. Returning in May, the surgeon decided removal was unsafe, she was sent back to Alaska for more chemotherapy. During this trip she was able to stay at a place called Hope Lodge sponsored by the American Cancer Society which provided free housing during her treatment in New York. When back in Alaska Judy received chemotherapy, the doctors stated if it stabilized there was an oral drug she could take; she just had to finish the fourth round of her chemotherapy. During the fourth round, the cancer grew and spread.

With all treatment options exhausted Judy is only left with clinical trials.

Now with all treatment options exhausted Judy is only left with clinical

trials, however the insurance company is not willing to pay for routine care costs during the clinical trial. They refuse to help with the cost even though the doctors are recommending them as a best option because all other treatments have failed. The health board for her insurance company will look at supporting clinical trials in April of 2009. If the health care plan still refuses coverage, she will have to pay out of pocket. Her next step is to have a metal plate put into her right femur from hip to knee, because of a tumor, doctors are worried it could break without this support. Judy is waiting to continue the clinical trial until after this treatment and radiation.

Judy mentions the importance of having a support network, and feels even if the clinical trial doesn't do anything for her at least we learn something. She stated "I would hate to think that this was all (the cancer) just a waste" in the mean time "I have worked, played, and seen my grandson." A team approach to health care is needed because it's a lot of work managing your own health care when you have cancer with oncologist, urologist, doctors, radiologist, and nurses at various places across the country. Judy continues to fight her battle with cancer; her strength is seen in her courage to continue to fight, even though getting around is sometimes a battle.

"I would hate to think that this was all (the cancer) just a waste."

Myths and Facts

About Cancer Clinical Trials

Myth: Cancer patients avoid clinical trials because they are too risky.

Fact: Many patients simply don't know that clinical trials are a treatment option. In one survey*, most of these patients said they would have enrolled if they had known. In clinical trials, patients are watched closely by their doctor. They are also watched by other members of their medical team.

Myth: Patients in clinical trials are treated like "guinea pigs."

Fact: 97 percent of people in one survey* said they were treated with dignity and respect. They also said that the care was very good.

Myth: Cancer clinical trial patients are given "sugar pills."

Fact: Patients who join clinical trials are given the best treatment available or the chance to receive a new treatment being considered.

Myth: Medicare does not cover the patient care costs of clinical trials.

Fact: Medicare has been covering these costs since June of 2000.

Myth

You need to be near a big hospital to take part in a clinical trial.

Fact: Many cancer clinical trials take place at local hospitals. Some also take place at local cancer clinics and doctors' offices.

—Coalition of cancer cooperative groups

*Harris Interactive Survey, 2000

Additional Resources

For More Information

Alaska

Anchorage

Alaska Regional Hospital
Cancer Care Center
2741 DeBarr Road. Building c-414
Anchorage, AK 99508
Phone: (907)264-1579 or 264-1431
http://www.alaskaregional.com

Alaska Clinical Research
Center, LLC
1200 Airport Heights Drive, Suite 330
Anchorage, AK 99508
Phone: (907)276-1455
http://www.centerwatch.com

Providence Cancer Center
Research Department
3851 Piper Street
Anchorage, Alaska 99508
Phone: (907) 212-6871
Fax: (907) 212-3674
http://www.providence.org/Alaska

Fairbanks

Oncology Data Center
Fairbanks Memorial Hospital
1640 Cowles St. Suite 2
Fairbanks, Alaska 99701
Phone: (907)458-5458 or 4458
Toll Free: 1-888-678-5458
http://www.bannerhealth.com/
Locations/Alaska/Fairbanks

Cancer Treatment Center
Cancer Treatment Center Entrance
1640 Cowles St. Suite 2
Fairbanks, AK 99701
Phone: (907) 458-5380
http://www.bannerhealth.com/Services/
Cancer

Nationwide

American Cancer Society Clinical
Trials Matching Service
http://www.cancer.org
or 1-800-303-5691

CenterWatch Clinical
Trials Listing Service
http://www.centerwatch.com/

Clinical Connection
http://www.clinicalconnection.com/

Coalition of Cancer
Cooperative Group
http://www.CancerTrialsHelp.org

NCI's Clinical Trials Locator
http://www.cancer.gov/clinical trials

U.S. National Institutes of Health
http://www.clinicaltrials.gov

Made available by:
Alaska Department of Health and Social Services
Division of Public Health
Section of Chronic Disease Prevention and Health Promotion
Comprehensive Cancer Control Program
& the Clinical Trials Work Group
Phone: 269-8092
December, 2009

ALASKA
Comprehensive Cancer Partnership

The FDA's Drug Review Process: Ensuring Drugs Are Safe and Effective

The path a drug travels from a lab to your medicine cabinet is usually long, and every drug takes a unique route. Often, a drug is developed to treat a specific disease. An important use of a drug may also be discovered by accident.

For example, Retrovir (zidovudine, also known as AZT) was first studied as an anti-cancer drug in the 1960s with disappointing results. Twenty years later, researchers discovered the drug could treat AIDS, and Food and Drug Administration approved the drug, manufactured by GlaxoSmithKline, for that purpose in 1987.

Most drugs that undergo preclinical (animal) testing never even make it to human testing and review by the FDA. The drugs that do must undergo the agency's rigorous evaluation process, which scrutinizes everything about the drug--from the design of clinical trials to the severity of side effects to the conditions under which the drug is manufactured.

Stages of Drug Development and Review

Investigational New Drug Application (IND)--The pharmaceutical industry sometimes seeks advice from the FDA prior to submission of an IND.

Sponsors--companies, research institutions, and other organizations that take responsibility for developing a drug. They must show the FDA results of preclinical testing in laboratory animals and what they propose to do for human testing. At this stage, the FDA decides whether it is reasonably safe for the company to move forward with testing the drug in humans.

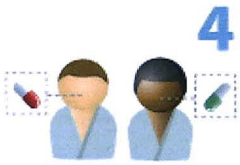
Clinical Trials--Drug studies in humans can begin only after an IND is reviewed by the FDA and a local institutional review board (IRB). The board is a panel of scientists and non-scientists in hospitals and research institutions that oversees clinical research.

IRBs approve the clinical trial protocols, which describe the type of people who may participate in the clinical trial, the schedule of tests and procedures, the medications and dosages to be studied, the length of the study, the study's objectives, and other details. IRBs make sure the study is acceptable, that participants have given

consent and are fully informed of their risks, and that researchers take appropriate steps to protect patients from harm.

Phase 1 Testing

Phase 1 studies are usually conducted in healthy volunteers. The goal here is to determine what the drug's most frequent side effects are and, often, how the drug is metabolized and excreted. The number of subjects typically ranges from 20 to 80.



Phase 2 Testing

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.

Phase 3 Testing

At the end of Phase 2, the FDA and sponsors try to come to an agreement on how large-scale studies in Phase 3 should be done. How often the FDA meets with a sponsor varies, but this is one of two most common meeting points prior to submission of a new drug application. The other most common time is pre-NDA--right before a new drug application is submitted.

Phase 3 studies begin if evidence of effectiveness is shown in Phase 2. These studies gather more information about safety and effectiveness, studying different populations and different dosages and using the drug in combination with other drugs. The number of subjects usually ranges from several hundred to about 3,000 people.

Review Meeting

Postmarket requirement and commitment studies are required of or agreed to by a sponsor, and are conducted after the FDA has approved a product for marketing. The FDA uses postmarket requirement and commitment studies to gather additional information about a product's safety, efficacy, or optimal use.

NDA Application

New Drug Application (NDA)--This is the formal step a drug sponsor takes to ask that the FDA consider approving a new drug for marketing in the United States. An NDA includes all animal and human data and analyses of the data, as well as information about how the drug behaves in the body and how it is manufactured.

Application Reviewed

When an NDA comes in, the FDA has 60 days to decide whether to file it so that it can be reviewed. The FDA can refuse to file an application that is incomplete. For example, some required studies may be missing. In accordance with the Prescription Drug User Fee Act (PDUFA), the FDA's Center for Drug Evaluation and Research (CDER) expects to review and act

on at least 90 percent of NDAs for standard drugs no later than 10 months after the applications are received. The review goal is six months for priority drugs. (See "The Role of User Fees.")

"It's the clinical trials that take so long--usually several years," says Sandra Kweder, M.D., deputy director of the Office of New Drugs in the CDER. "The emphasis on speed for FDA mostly relates to review time and timelines of being able to meet with sponsors during a drug's development," she says.

Facts About "Right To Try"

For terminal patients who have exhausted their conventional treatment options, obtaining access to potentially life-saving investigational medications is often extremely difficult. The patient can attempt to enroll in a clinical trial, but many of the sickest individuals do not qualify. In fact, only 3 percent of cancer patients today are enrolled in clinical trials. For everyone else, their only hope for obtaining potentially life-saving medications is to ask the FDA for special permission.

Only about 1,200 people make it through the FDA's "compassionate use" application process each year. The process is complicated, time-consuming, and expensive. The first step in the process requires a doctor to complete a complex and time consuming application. After the doctor submits the application to the FDA, the manufacturer must also submit lengthy documentation requirements. The FDA then has a month to review the submission and either grant or deny the request, but if there are any questions the one-month clock starts over. After the FDA approves a request, a separate committee not affiliated with the FDA, called an Institutional Review Board, also must approve the patient's use of the drug. There is no time limit on when an Institutional Review Board must review and approve or deny an application. Sadly, there are many documented cases of patients dying while their application is being considered.

After a year and half of deliberation, the FDA recently shortened its application form. A shorter application for the first step is helpful, but it only addresses one part of the approval process. And ultimately, it's still an application to the government to ask permission to try to save your own life. If you have a terminal illness, you don't have time for a multi-step government process. If your child is dying from a terminal illness and you know there's an investigational medication that is already helping other children survive, a shorter form isn't good enough.

We need to remove barriers that limit doctors from providing the care they are trained to give—and this is exactly what Right To Try does.

Right To Try allows terminally ill Americans to try medicines that have passed Phase 1 of the FDA approval process but are not yet on pharmacy shelves. Right To Try expands access to potentially life-saving treatments years before patients would normally be able to access them.

Goldwater Institute
(602) 462-5000
www.goldwaterinstitute.org

Under Right To Try, a terminal patient would be able to access an investigational medicine if:

- ✓ The patient has a terminal disease and has exhausted all conventional treatment options;
- ✓ The patient's doctor has advised the use of an investigational medication;
- ✓ The medication has successfully completed basic safety testing and is part of the FDA's on-going approval process;
- ✓ The patient has provided "informed consent" acknowledging the potential risk of the drug; and
- ✓ The company developing the medication is willing to make it available to the patient.

Right To Try includes important protections. The basic safety testing and informed consent requirements protect the patient. And doctors and the manufacturer are protected from liability if the investigational medication doesn't work. But this is not protection from medical malpractice.

Right To Try is already law in 33 states: Alabama, Arizona, Arkansas, California, Colorado, Connecticut, Florida, Georgia, Idaho, Illinois, Indiana, Louisiana, Maine, Michigan, Minnesota, Mississippi, Missouri, Montana, Nevada, New Hampshire, North Carolina, North Dakota, Ohio, Oklahoma, Oregon, South Carolina, South Dakota, Tennessee, Texas, Utah, Virginia, West Virginia, and Wyoming. It has passed with overwhelming bipartisan support in each state and has been introduced in 16 additional states. Right To Try isn't a red or blue issue; it's a human dignity issue. That's why lawmakers from both sides of the aisle are coming together to protect their citizens' right to try to save their own lives.

The FDA says providing dying people with investigational medications should be an exception. We think it should be the rule. People fighting for their lives should have access to medicines that could save them without needing a permission slip from the government.

For more information about Right To Try visit RightToTry.org. Or contact Starlee Coleman at scoleman@goldwaterinstitute.org or (602) 758-9162.

Right To Try: Patient Stories

Jordan McLinn

Six-year-old Jordan says he wants to grow up to be a firefighter so he can save lives. Jordan has Duchenne muscular dystrophy, which could leave him paralyzed within 5 years and shortens his life expectancy to only 20 years. There is a drug being used in clinical trials now that is helping young children like Jordan. But it could take another seven years for the drug to be available. His parents say they cannot afford to wait for the FDA to give the drug its final approval. He could be in a wheelchair by then. An investigational drug could add years to Jordan's life, which would give him the chance to save others.

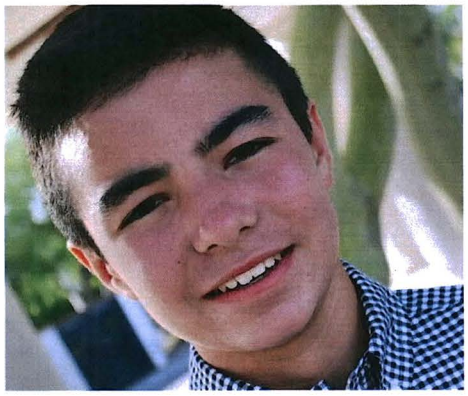
Josh Hardy

By the time Josh Hardy was seven years old he had already beat cancer four times. After a bone marrow transplant, he was infected with a rare virus that no drug on the market could effectively treat. But there was a new medicine being made in North Carolina that was having a positive effect in a small clinical trial. But Josh's doctors couldn't get access to it. Aimee, Josh's mom, started telling Josh's story to anyone who would listen. She created a social media campaign that got worldwide attention. Finally, the FDA and the drug company agreed to let Josh have the drug they were already safely giving to others enrolled in the clinical trial. Now, a year later, Josh is home and healthy. It's no exaggeration to say this investigational drug saved his life.

Mikaela Knapp

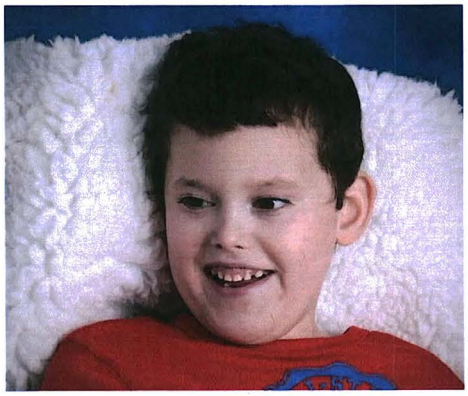
At 24, Mikaela was diagnosed with a deadly form of kidney cancer that had already migrated into her bones before she even knew she was sick. She went through every known treatment in a matter of months and nothing worked. Mikaela's high school sweetheart, Keith, heard about a drug under development that was successfully treating people with this same cancer. But Mikaela wasn't allowed to enroll in the clinical trial. Mikaela and Keith launched a social media campaign to try to get access to the drug. But it wasn't enough. The FDA didn't help. Mikaela died on April 24, 2014. Five months later, on September 4, the FDA gave final approval to the drug that could have saved her.

Right To Try: Patient Stories



Diego Morris

When 10-year-old Diego woke up with a sore leg, his mom thought "just another sports injury." When the pain didn't go away, they knew something was wrong. But they never expected Osteosarcoma, a rare form of bone cancer. After exhausting all treatment options available, Diego's doctors recommended he try, Mifamurtide, which wasn't available in the United States, but was being safely used and had been given the Prix Galien Award, the gold medal for pharmaceutical research and development, in England. The Morris family wasted no time, and made the move abroad to try to save Diego's life. The treatments worked and now Diego is home in Phoenix, Ariz. and back to playing his favorite sports. Without access to this drug, currently under approval in the U.S., Diego's story could have ended very differently.



Bertrand Might

Bertrand is a very special little boy. He was the first person ever to be diagnosed with a rare, fatal genetic disorder called NGLY1 that has left this seven-year-old paralyzed. Because the disease was only identified by scientists in 2012, and only a few people world-wide have been diagnosed with it, there is no cure and no treatment available. Because the disorder is so rare, a drug may not ever be developed to treat it. But, scientists have found that Bertrand responds to certain investigational therapies. So, Bertrand's family will have to rely on trying new, investigational medications as long as they have access to them.

Ted Harada

Ted was diagnosed with ALS at 38. With no cure, ALS is a certain death sentence—and usually within three years. Ted didn't want to leave his wife and three young children behind in his early 40s. That was just too soon. Lucky for Ted, he was able to enroll in a clinical trial testing a new ALS treatment. Within weeks of beginning the investigational treatment, something miraculous began to happen. Ted set aside his cane and started to regain his strength. While the ALS didn't go away, the symptoms began to subside, allowing him to walk 2.5 miles for ALS awareness in a local campaign. Ted is still going strong because of the investigational treatment he is receiving, and now he is fighting for the right of all terminally ill people to take investigational medications.

POLICY report

Goldwater Institute

No. 266 | February 11, 2014

Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment

by Christina Corieri, Health Care Policy Analyst

EXECUTIVE SUMMARY

In 2002, Kianna Karnes, a 41-year-old mother of four children, was diagnosed with kidney cancer.[1] She was treated with interleukin-2, the only medication approved by the Food and Drug Administration (FDA) at the time to treat her disease. When that treatment failed, her father began researching investigational medications, learning in 2004 that both Pfizer and Bayer were conducting clinical trials for new investigational medications to treat kidney cancer. Karnes was ineligible for the clinical trial because her cancer had previously spread to her brain. Although her brain tumors had been removed, she was still disqualified from joining the clinical trial, so her father sought expanded access for his daughter. Months passed before he was able to secure access for his daughter. He contacted Congressman Dan Burton's (R-IN) office for assistance, and drew media coverage of Karnes' struggle in the *Wall Street Journal*. On March 24, 2005, the FDA notified the family that it had approved a single-patient IND for Karnes. Tragically, it was too late—Kianna Karnes died the same day access was approved.[2] Less than a year later, both drugs were given final FDA approval to treat advanced kidney cancer. Speaking after his daughter's death, her father said, "I don't know that either of these drugs would have saved Kianna's life, but wouldn't it be nice to give her a chance?"[3]

In the case of Kianna Karnes, she had a better chance than most patients at receiving expanded access. As her father explained, "Here is a case where her old man understood clinical trials. I knew about compassionate use; I had a friendship with a powerful member of Congress; I've got the *Wall Street Journal* behind me. But I still couldn't save her life. Now, what about the thousands of people out there who don't have these kinds of resources available to them?"[4] To most patients, and many physicians outside of major institutions, the process of obtaining expanded access is excessively time-consuming and extremely difficult to navigate.

For patients suffering from terminal illnesses, the FDA is the arbiter of life and death. These patients, suffering from diseases ranging from ALS to Zellweger Syndrome, face little chance of recovery. For patients like Kianna, investigational medicines provide a glimmer of hope. The FDA, however, often stands between the patients and the treatments that may alleviate their symptoms or provide a cure. To access these treatments, patients must either go through a lengthy FDA exemption process or wait for the treatments to receive FDA approval, which can take a decade or more and cost hundreds of millions of dollars. Sadly, over half a million cancer patients and thousands of patients with other terminal illnesses die each year as the bureaucratic wheels at the FDA slowly turn.[5]

Patients should be free to exercise a basic freedom – attempting to preserve one's own life. The burdens imposed on a terminal patient who fights to save his or her own life are a violation of personal liberty. Such people should have the option of accessing investigational drugs which have passed basic safety tests, provided there is a doctor's recommendation, informed consent, and the willingness of the manufacturer of the medication to make such drugs available.

States should enact "Right to Try" measures to protect the fundamental right of people to try to save their own lives. Designed by the Goldwater Institute, this initiative would allow terminal patients access to investigational drugs that have completed basic safety testing, thereby dramatically reducing paperwork, wait times and bureaucracy, and, most importantly, potentially saving lives.

GOLDWATER INSTITUTE

patient who met the stated criteria from accessing investigational medications. Likewise, other procedural burdens such as the IND application and IRB review requirement could be deemed undue burdens and either eliminated or drastically curtailed.

The concept of ordered liberty cannot include allowing a government agency to promulgate and enforce regulations that impair an individual's health or cause death by denying or delaying access to potentially life-saving medications. The way in which the FDA currently regulates access to investigational medications may be rational for non-terminal patients, but its application to terminal patients, who lack other treatment options, is not. Preventing such a patient from accessing a potentially life-saving medication will, without question, result in the fulfillment of the diagnosis — death.

Without the action of state lawmakers, terminal patients are at the mercy of a federal bureaucracy that can literally cause death by delays, denials, and unnecessary procedural requirements.

It has long been established that the U.S. Constitution creates a floor of protection for individual rights – not a ceiling. States can and do provide additional and enhanced protections for individuals.

Conclusion

From her sickbed, Edie Bacon wrote of the travails a terminal patient faces and made a final plea for the only medication that might save her. "The government wants proof of efficacy before it will allow me to take this drug outside of an approved trial. But the 'proof' is years away, and I need the drug now. It's safe. It might work. Johnson & Johnson would let me have it if they could do so without the threat of a government hassle. But they're so caught up in the FDA web that the life of an individual patient has no importance whatsoever. Without ET 743, I'm a dead woman walking. Five kids are going to wonder why they're left without a mother. Won't somebody help me get this drug?"[130] Edie died two years later, but there are thousands of patients who face this same battle every day – patients who have to make the same pleas that Edie did for a chance to try to protect their own lives.

Such pleas should anger anyone who believes in the concept of personal liberty. No free person should have to come to the government as a supplicant to beg for a right to try to save his or her own life. In a country dedicated to the idea that all people have certain "unalienable Rights, that among these are Life, Liberty, and the Pursuit of Happiness," no government official should have the power to deny a person's last chance at all three – life, liberty, and happiness.[131] Yet that is the power the FDA wields today. States should challenge this regulatory authority by passing Right to Try and returning medical decision making back to the rightful hands of patients and doctors.

The Goldwater Institute

The Goldwater Institute was established in 1988 as an independent, non-partisan public policy research organization. Through policy studies and community outreach, the Goldwater Institute broadens public policy discussions to allow consideration of policies consistent with the founding principles Senator Barry Goldwater championed—limited government, economic freedom, and individual responsibility. Consistent with a belief in limited government, the Goldwater Institute is supported entirely by the generosity of its members.

Guaranteed Research

The Goldwater Institute is committed to accurate research. The Institute guarantees that all original factual data are true and correct to the best of our knowledge and that information attributed to other sources is accurately represented. If the accuracy of any material fact or reference to an independent source is questioned and brought to the Institute's attention with supporting evidence, the Institute will respond in writing. If an error exists, it will be noted on the Goldwater Institute website and in all subsequent distribution of the publication, which constitutes the complete and final remedy under this guarantee.

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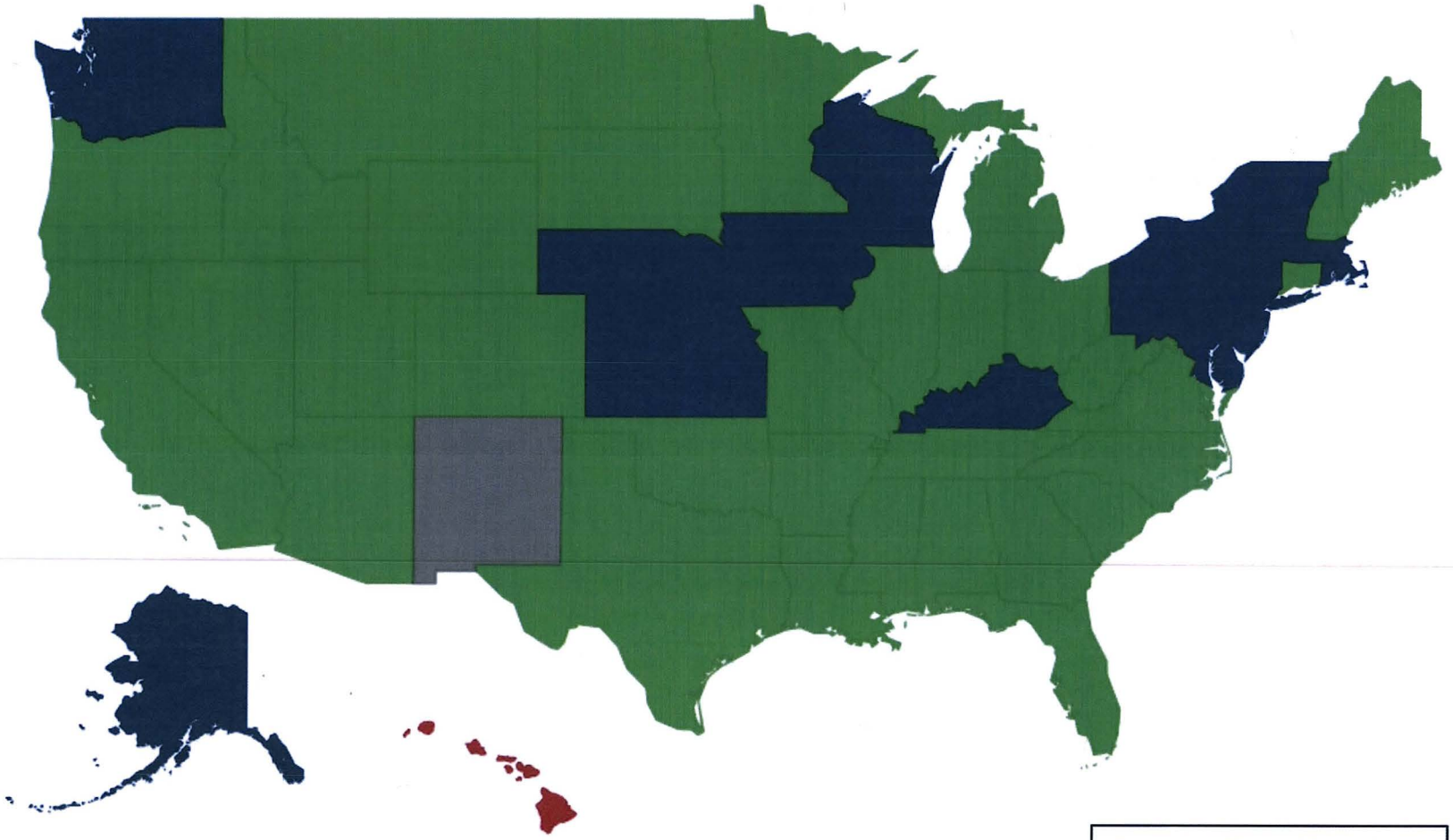
Where freedom wins.

500 East Coronado Rd., Phoenix, AZ 85004 | Phone (602) 462-5000 | Fax (602) 256-7045 | www.goldwaterinstitute.org

"Right to Try" Legislative Map

Updated: February 22, 2017



Green = Signed into Law
Blue = Introduced
Gray = Not Introduced
Red = Vetoed

Fiscal Note

State of Alaska
2017 Legislative Session

Bill Version: HB 43
Fiscal Note Number:
() Publish Date:

Identifier: HB043-DCCED-CBPL-02-24-17
Title: NEW DRUGS FOR THE TERMINALLY ILL
Sponsor: GRENN
Requester: (H) Health and Social Services

Department: Department of Commerce, Community and Economic Development
Appropriation: Corporations, Business and Professional Licensing
Allocation: Corporations, Business and Professional Licensing
OMB Component Number: 2360

Expenditures/Revenues

Note: Amounts do not include inflation unless otherwise noted below. (Thousands of Dollars)

	FY2018 Appropriation Requested	Included in Governor's FY2018 Request	Out-Year Cost Estimates				
OPERATING EXPENDITURES	**FY 2018**	**FY 2018**	**FY 2019**	**FY 2020**	**FY 2021**	**FY 2022**	**FY 2023**
Personal Services							
Travel							
Services							
Commodities							
Capital Outlay							
Grants & Benefits							
Miscellaneous							
Total Operating	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**

Fund Source (Operating Only)

None							
Total	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**

Positions

Full-time							
Part-time							
Temporary							

Change in Revenues

None							
Total	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**

Estimated SUPPLEMENTAL (FY2017) cost: 0.0 *(separate supplemental appropriation required)*
(discuss reasons and fund source(s) in analysis section)

Estimated CAPITAL (FY2018) cost: 0.0 *(separate capital appropriation required)*
(discuss reasons and fund source(s) in analysis section)

ASSOCIATED REGULATIONS

Does the bill direct, or will the bill result in, regulation changes adopted by your agency? No
If yes, by what date are the regulations to be adopted, amended or repealed? N/A

Why this fiscal note differs from previous version:

Not applicable, initial version.

Prepared By: Janey Hovenden, Director — Phone: (907)465-2538
Division: Corporations, Business and Professional Licensing — Date: 02/24/2017 11:00 AM
Approved By: Catherine Reardon, Director — Date: 02/24/17
Agency: Division of Administrative Services, DCCED

FISCAL NOTE ANALYSIS

STATE OF ALASKA **BILL NO.** HB 43
2017 LEGISLATIVE SESSION

Analysis

HB 43 prohibits disciplinary action being taken against physicians by the State Medical Board for prescribing, dispensing, or administering an investigational drug, biological product, or device to terminally ill patients that have considered all other treatment options approved by the U.S. Food and Drug Administration and are ineligible or unable to participate in a current clinical trial.

This legislation establishes immunity for physicians, manufacturers, and distributors in the case of injury or death of a terminally ill patient from the use or nonuse of an investigational drug, biological product, or device, provided that informed consent was obtained from the patient and notice of immunity was given in advance.

HB 43 amends the statute limiting the sale and distribution of new drugs so it does not apply to physicians prescribing or administering investigational drugs under the conditions established in Section 1 of the bill. Additionally, this legislation prohibits requiring licensed entities subject to AS 47.32.030 to increase services for the sole purpose of accommodating a physician's practice of prescribing, dispensing, or administering an investigational drug, biological product, or device, or providing related treatment to a patient.

The Division of Corporations, Business and Professional Licensing does not anticipate fiscal impact from this legislation.

February 17, 2017

Bob Urata MD
Valley Medical Care
1801 Salmon Creek Lane
Juneau, Alaska 99801

Alaska State Legislature
State Capitol - Room 418
Juneau, Alaska 99801
Fax: 907-465-6597

To Whom It May Concern:

This is a letter of support of for House Bill 43 which would protect physicians and hospitals for treating terminally ill patients with experimental medications that have passed the first stage of approval from the FDA.

I personally have not had to use this, but feel if this was available it would allow me to likely pursue this more as I will know that there is protection from potential lawsuits should something go wrong causing unforeseen patient harm.

I hope you will find this bill favorable for your support. I urge passage by your committee.

Thank you for your service.

Sincerely,

Bob Urata MD
907-723-4144

Dixie A. Hood, LMFT
Juneau, Alaska

February 20, 2017

Alaska State Legislature
State Capitol
Juneau, AK 99801

Subject: House Bill 43 – The Right to Try

Dear Alaska State Legislature,

This letter is in support of House Bill 43, the Right to Try: New Drugs for the Terminally Ill.

For 30 years as a licensed marriage and family therapist, substance abuse counselor and present member of the Juneau Suicide Prevention Coalition, I have provided mental health services and support to many individuals who were suffering from life-threatening diseases, as well as their families. Some were referred to Hospice and Home Care. When help and hope seemed unobtainable, several patients turned to suicide.

I have had HIV clients who were eligible to receive medical marijuana, but moved away from long-time friends and relatives because Alaska state law required administering physicians to be publicly identified. Their doctor believed that was professionally unethical and violated privacy laws. Therefore, they refused to provide marijuana as treatment for their illness. Protection of both patient and physician is critical when the patient's well-being, and even life, are at stake.

HB 43 would enable a patient who has provided "informed consent" acknowledging the potential risk of investigational medication to receive treatment of a drug which has successfully completed Phase 1 of the U.S. Food and Drug Administration's drug review process. It would provide immunity from disciplinary action and liability of doctors and manufacturers who have been willing to make the investigational medication available to the patient.

This would enable a terminal patient the ability to access safe, but experimental drugs when their doctor has exhausted all the FDA-approved options.

This is a humanitarian issue, not a political one. I urge support of House Bill 43.

CC: Representative Jason Grenn

-----Original Message-----

Sent: Tuesday, February 21, 2017 8:14 PM
To: Rep. Jason Grenn <Rep.Jason.Grenn@akleg.gov>
Subject: HB 43

From: E. C. Krome [redacted]
Subject: HB 43

I have been a registered nurse since 1979. I fully support this bill and am incredibly thankful to you for introducing it. It is terrible to take hope away from those with a terminal illness.

E. C. Krome
3642 N. Sams Dr.
Wasilla, AK. 99654

--

P.O. Box 13458
Los Angeles, CA 90013
213.935.0553

Feb. 24, 2017
Re: Support for HB43

Dear members of the Alaska legislature,

The Tenth Amendment Center fully supports passage of HB43 enacting a "Right to Try" law in Alaska.

Sometimes the wheels of bureaucracy move slowly. For most of us, this merely presents an inconvenience, but for a patient suffering from a life-threatening illness, a few months could mean the difference between life and death.

The FDA approval process is meant to protect the public and ensure only safe and effective treatments find their way into the marketplace. But sometimes the nature of the process means delays in getting medicines into the hands of those who desperately need them. "Right to Try" laws create a bridge spanning a gap between federal regulations and the needs of terminally ill patients.

We've seen firsthand what "Right to Try" means for more than 70 cancer patients in Texas. Treatment ended after the FDA declined to extend a clinical trial.

"Essentially, my job was to go back to these patients and family members, and tell them that, 'Sorry, I mean the FDA has told me not to continue in this treatment,'" Dr. Delpassand said.

But when the Texas Right to Try bill went into effect, he was able to resume treating his patients under the new state law.

"I can tell you that when I was talking to our patients, when I told them about the news, they were just so happy to hear this. This was such good news for them and gave them such a great hope to understand and know they can get their treatment."

This type of legislation illustrates the beauty of federalism. People have recognized a glaring need and met it through legitimate state action.

So far, 33 states have enacted "Right to Try," and several states will likely be added to the list this year.

We strongly urge you to vote yes on HB43.

Sincerely,

Michael Boldin, Tenth Amendment Center Founder and Executive Director
Mike Maharrey, Tenth Amendment Center National Communications Director

THE STATE
of ALASKA
GOVERNOR BILL WALKER

Department of Commerce, Community, and Economic Development

STATE MEDICAL BOARD

550 West 7th Avenue, Suite 1500
Anchorage, AK 99501-3567
Main: 907.269.8163
Fax: 907.269.8196

February 24, 2017

The Honorable Bill Wielechowski
State Senate
Alaska State Capitol
Juneau, Alaska 99801

The Honorable Jason Grenn
State House of Representatives
Alaska State Capitol
Juneau, Alaska 99801

Senator Wielechowski and Representative Grenn:

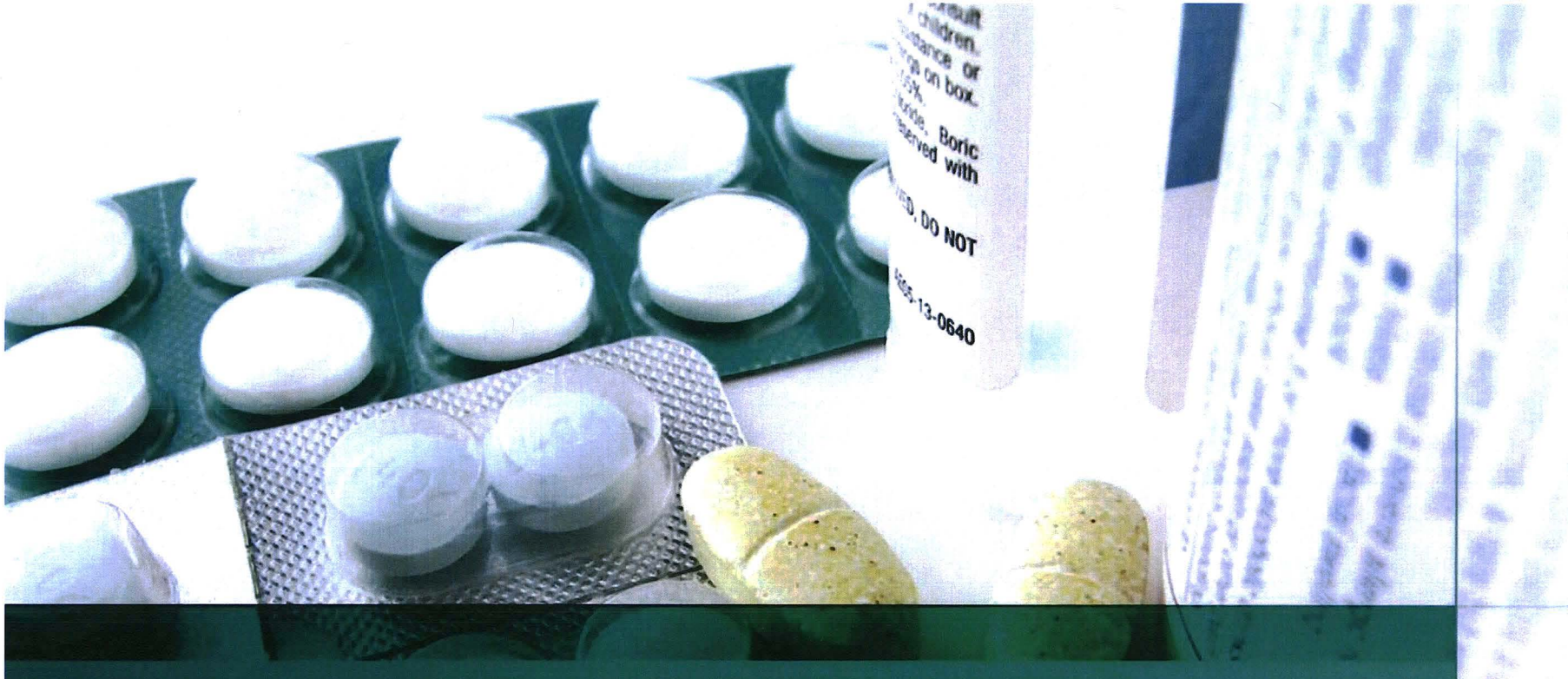
The Alaska State Medical Board has reviewed Senate Bill (SB) 19 and House Bill (HB) 43, which propose to provide immunity for, and prohibit disciplinary action of, physicians for prescribing, dispensing, or administering an experimental drug to terminally ill patients that have considered all other treatment options approved by the FDA and is ineligible or unable to participate in a current clinical trial.

During the previous legislative session, the Board had opposed similar bills; however, the previous position is not relevant to the current bills, as they are different. The Board noted that the previous bills allowed for use of drugs that may be in Phase 1 of testing, and the current bills are for drugs in Phases 2 or 3. The Board also noted that the two current bills are nearly identical, except that HB 43 includes provisions for the both use or non-use of these drugs.

The Board determined to take a neutral position on SB 19 because it does not include the "non-use" clause; the Board supports HB 43 as written.

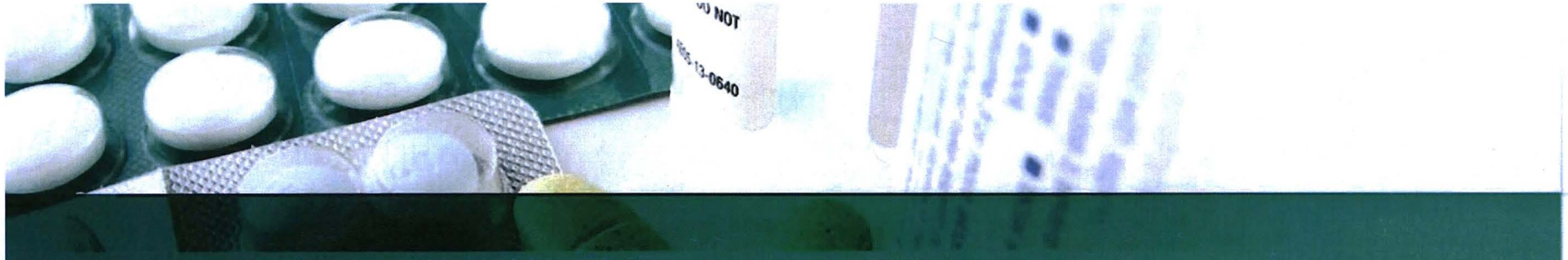
Sincerely,

Grant Roderer, M.D.
Board President
Alaska State Medical Board



House Bill 43: The Right to Try

Representative Jason Grenn



The Right to Try

"Patients should be free to exercise a basic freedom – attempting to preserve one's own life."

- Christina Corieri, Health Care Policy Analyst

House Bill 43

- **Sec. 1:** Prohibits disciplinary action by the State Medical Board, under specific patient terms. Provides key definitions.

- **Sec. 2:** Physicians, medical team members, manufacturers and distributors acting in good faith are not held liable, with proper informed consent and notification. Also not held liable for choosing not to participate.

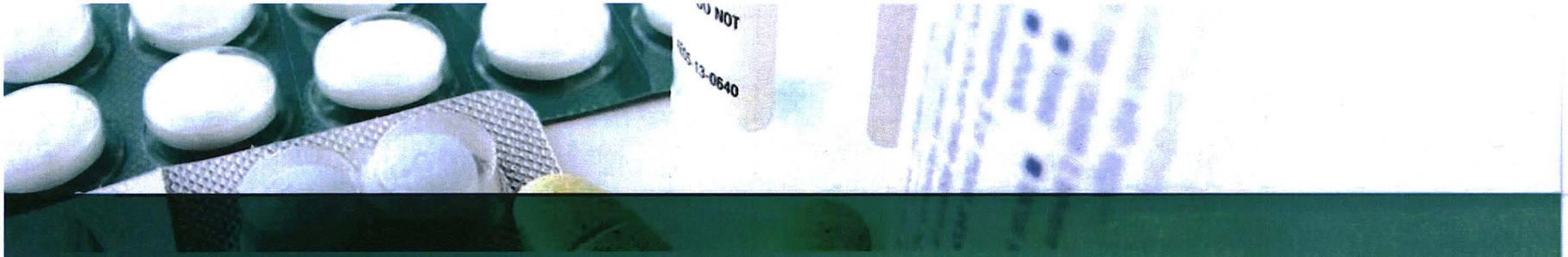
- **Sec. 3:** Amends statute limiting the sale and distribution of new drugs to allow for physicians to prescribe and administer under conditions of Sec. 1.

- **Sec. 4:** Precludes hospitals and healthcare facilities from being required to provide increased services.

FDA Drug Review Process

Preclinical Animal Testing & Investigational New Drug (IND) Application

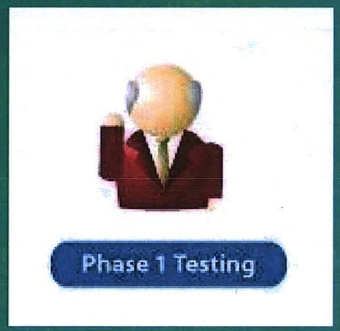
- Drug sponsors conduct preclinical testing in animals
- Upon IND application, results are reviewed
- FDA determines if drug is reasonably safe for human testing

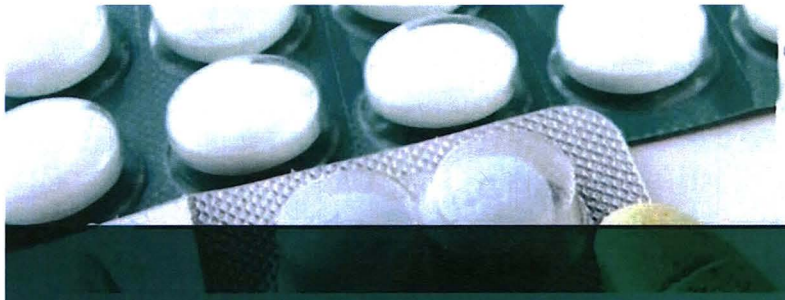


FDA Drug Review Process (cont'd)

PHASE 1 - Safety

- Studies occur after approval of IND application
- Some conducted on healthy volunteers, but not all – depends on purpose of medication
- Determine side effects and toxicity levels

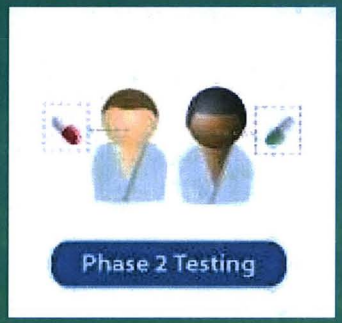


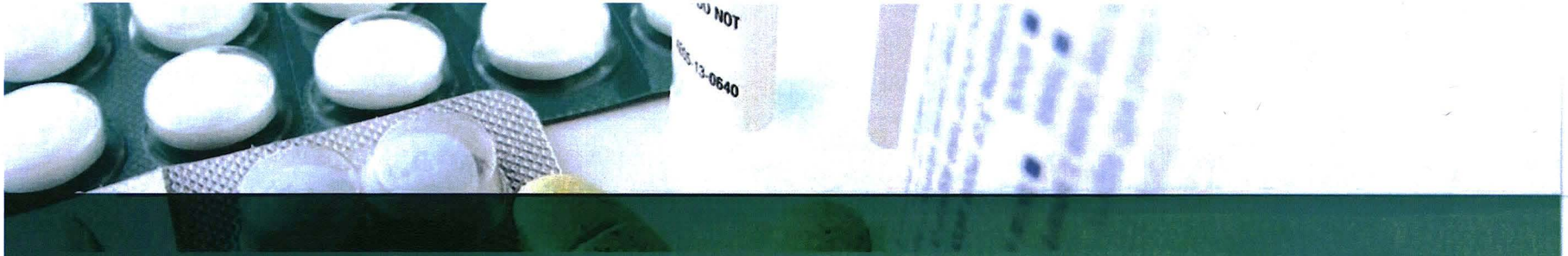


FDA Drug Review Process (cont'd)

PHASE 2 – Efficacy

- Studies begin when drug is determined relatively safe
- Preliminary data on people with specific disease or condition
- Sets stage for scale of Phase 3 studies

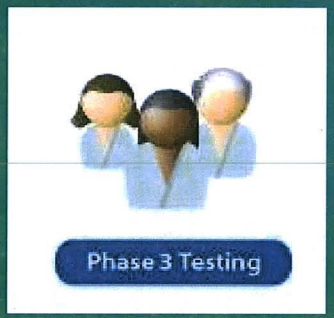




FDA Drug Review Process (cont'd)

PHASE 3 – Comprehensive

- Studies begin if Phase 2 shows evidence of effectiveness
- Gather more info on safety and effectiveness
- Different dosages, populations and combination with other medications

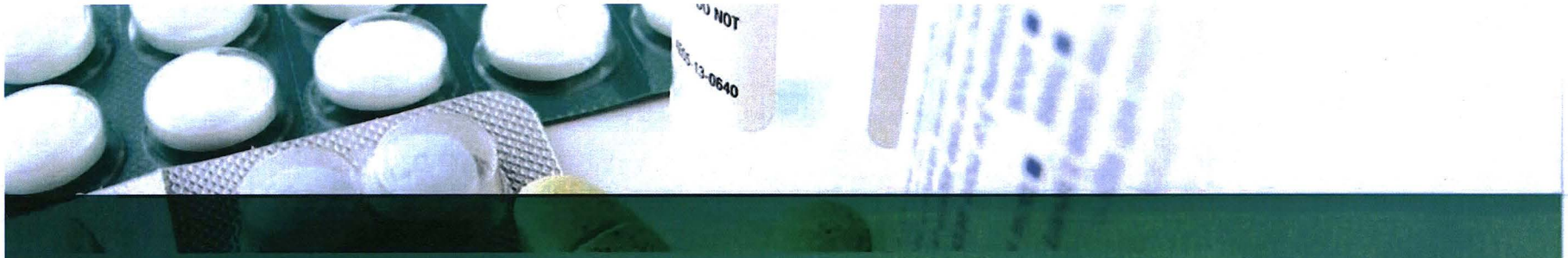


FDA Drug Review Process (cont'd)

Review Meeting & New Drug Application (NDA)

- Sponsors meet with FDA
- Submit NDA to officially request marketing approval
- FDA has 60 days to decide to file application
- 90% of applications are processed within 10 months of filing

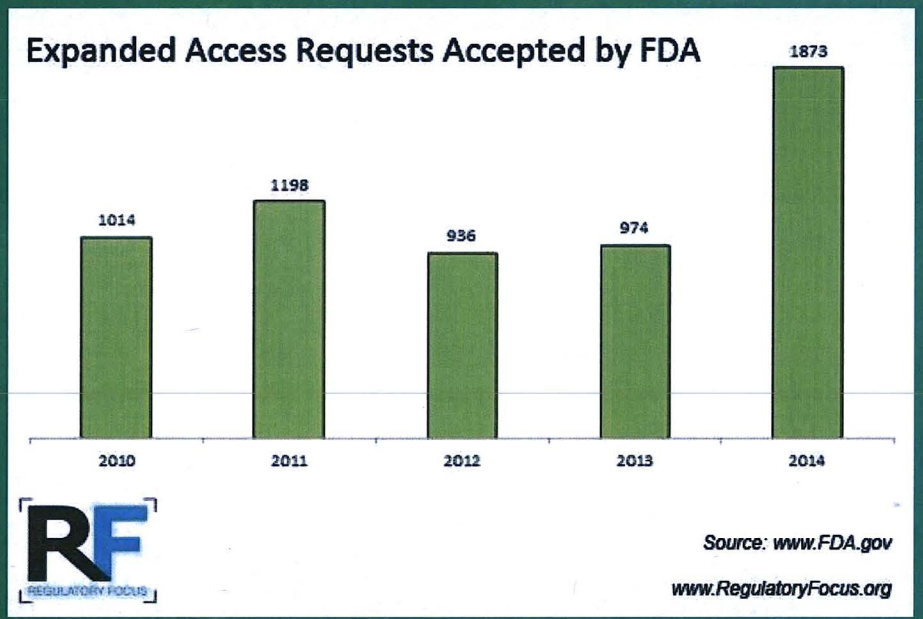
An "*investigational drug, biological product, or device*" in HB 43 has completed Phase 1 and remains in ongoing clinical trials under Phase 2 or 3, but is not yet approved for general use by the FDA

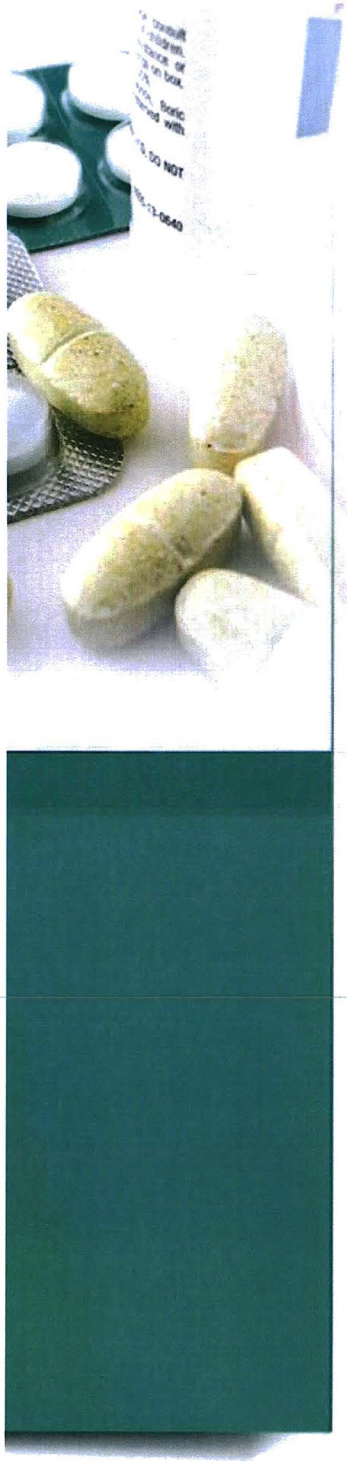


FDA's Expanded Access Program

"Compassionate Use"

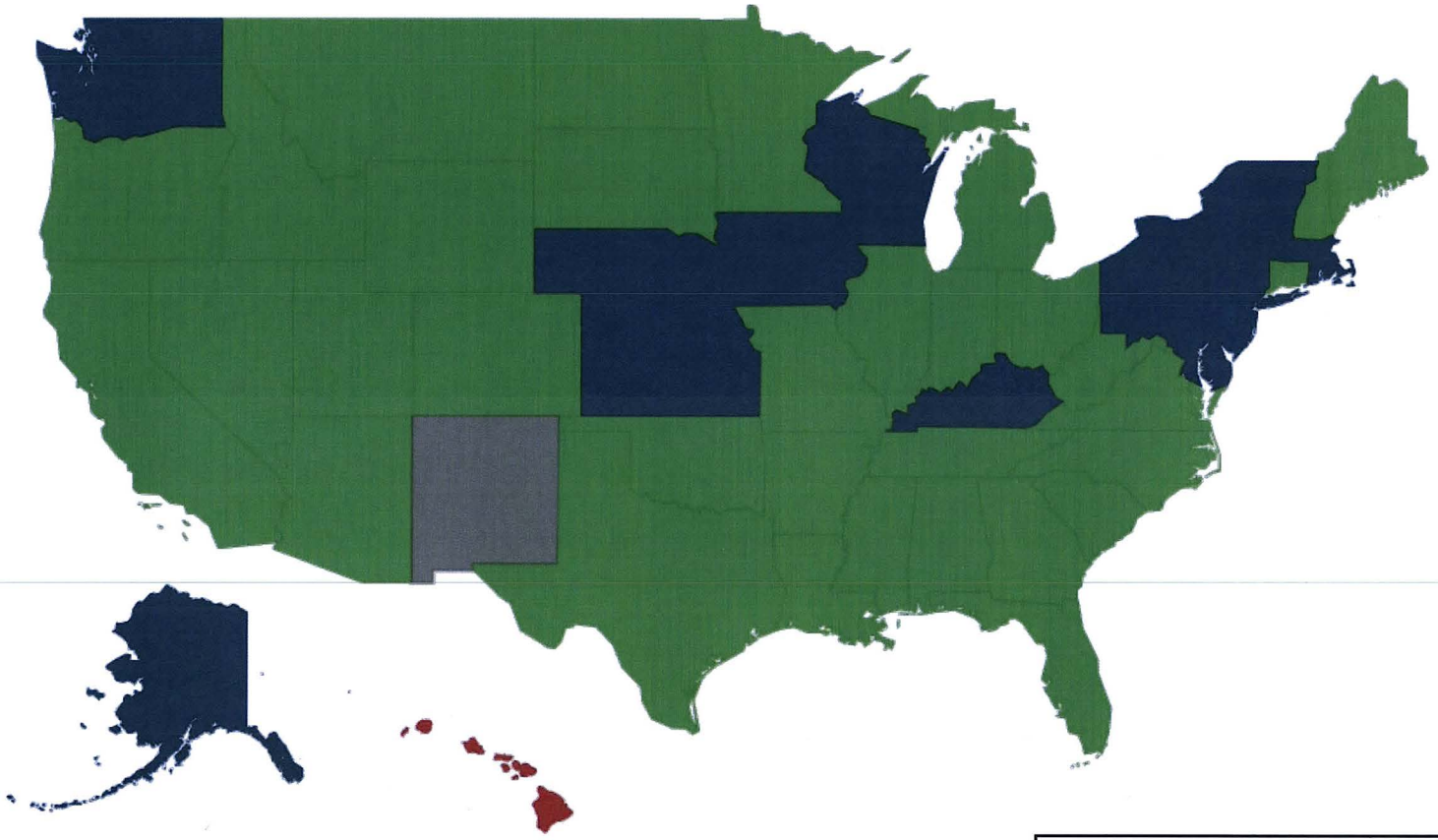
- Approx. 1,200 applicants per year make it through
- Application form has been streamlined, but approval process remains extensive



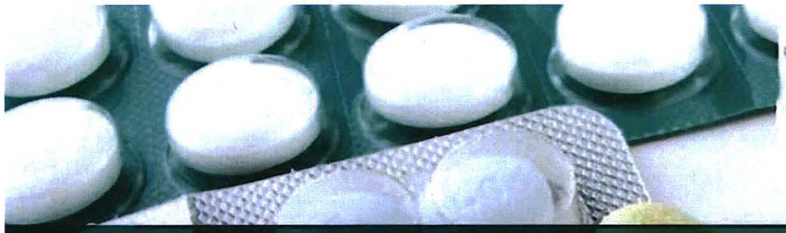


The Right to Try - A Nationwide Effort

49 states have passed or introduced "Right to Try" legislation



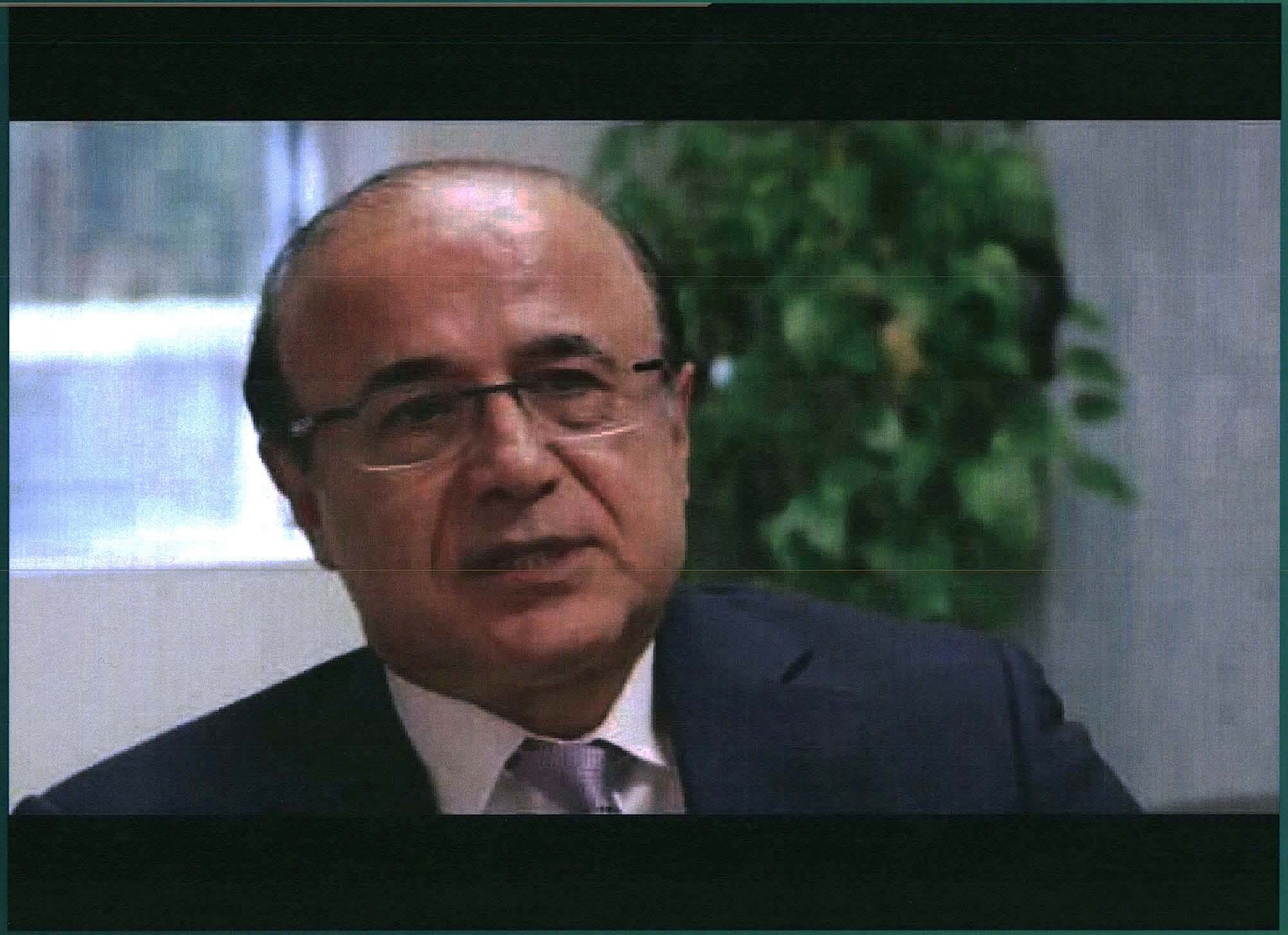
Green = Signed into Law
Blue = Introduced
Gray = Not Introduced
Red = Vetoed



This concludes our presentation for House Bill 43.

Thank you.

Right to Try – How it's Working



-----Original Message-----
Sent: Monday, February 27, 2017 2:12 PM
Subject: Written Testimony in support of HB43 New Drugs for the Terminally Ill

Representatives Drummond, Gara, Grenn, and Kawasaki (Sponsors of HB43):

I write to you today in support of HB43 with the short title "New Drugs for the Terminally Ill". I understand that your time is limited and therefore I will effort to be as brief as possible. In June of 2011, my father was diagnosed with Amyotrophic Lateral Sclerosis (ALS), sometimes known as "Lou Gehrig's Disease". For those unfamiliar, this disease slowly saps a person's ability to move, rendering them bedridden. Eventually, it takes away their ability to breathe, at which point, they die. It should be noted that the disease does not affect a person's mind. Therefore, they are acutely aware of their daily deterioration and because of this, the mind becomes a prisoner within the body. For my father, the deterioration began in June with a reduced range of movement in his left leg. At this point he had been working 12-hour days 5-6 days per week as a machinist. I feel this is important to point out because of the extended hours and physical nature of the job. He was by all accounts a very strong and healthy man at the time of diagnosis. By late fall, he had completely lost the ability to walk. At Thanksgiving, he held our then 5-month old son for the last time as his arms had become too weak for him to trust with such precious cargo. When he finally lost all movement in his limbs, he would "hug" our children by nuzzling his face against them.

As we entered the depths of winter, he and I carried on what conversations we could, trying to accelerate what should have been many more years of passing knowledge and wisdom from Father to son. These conversations became increasing difficult as the disease made his breathing quite labored, even with the assistance of a bipap machine. While the painful and emotional moments are too many to list, the most painful came when I asked him the simple question, "Dad, are you angry?" He responded with uncommon grace and humility by uttering one word with every labored breath. "I'm. Not. Angry. I'm. Just. Sad. That. I. Won't. See. Your. Kids. Grow. Up." In the early morning hours of February 1, 2012, he died at the all-too-young age of 58.

My father was a good man and my hero. He was a tireless worker, a great role model, and an unparalleled family man. He took a chance on Alaska in 1996 when he moved his family from the only home it had ever known to a place where we had no connections, no network, no family, and no friends. The gamble paid off for all of us. He saw the potential in this place. He fought like we all do to make a life here. In the end, I wish the State had afforded him the right to fight his disease with the same ferocity. Recently, in the Washington Post, there was an article detailing the experience of a man who has for all intents and purposes beaten ALS through advanced therapies pioneered by doctors at the Emory ALS Center in Atlanta, Georgia. In reading about this man, I became aware of the "Right To Try" movement. Soon thereafter I became aware of the bill which I write to you in support of today. I implore you to pass this bill and to encourage your colleagues in the Senate to do the same. I cannot say for sure that these advanced therapies would have saved my father's life, but they may yet save someone's father or mother, daughter or son.

Thank you all for your consideration of this important legislation. If you have any questions, feel free to contact me via this email address or at the information below.

Jason Norris
12026 Tidepool Place
Anchorage, Alaska
99515

CC: House Speaker Rep. Edgmon, House Majority Leader Rep. Tuck, House Minority Leader Rep. Millett, Rep. Kopp (House District 24), and Sen. Von Imhof (Senate District L)

SOUTHEAST
Radiation
Oncology
CENTER

Eugene Huang, M.D.
1701 Salmon Creek Lane
Juneau, AK 99801
Phone: 907.586.5762

3/1/2017

To Whom It May Concern,

I am writing this letter in support for House Bill 43, "New Drugs for the Terminally Ill." I believe that this bill will help provide terminally ill patients greater access to the new drugs that may potentially make a significant difference in their lives.

Sometimes in our practice, a patient may be in a situation where they have exhausted all available options, yet have not given up hope and want to try additional measures that may help them. Every other week, even just within my limited scope of cancer practice, there are new drugs being developed and tested in early-phase clinical trials. The pace of biotechnology research and drug development has significantly accelerated in just the last few years, offering patients more options and hope for treatment.

I believe that House Bill 43 will help bring new scientific discoveries closer to patients whom need them, within the appropriate context and safeguards.

Sincerely,

Eugene H. Huang, MD
Medical Director
Southeast Radiation Oncology Center

THE STATE of ALASKA
GOVERNOR BILL WALKER

Department of Health and Social Services
ALASKA COMMISSION ON AGING
P.O. Box 110693
Juneau, Alaska 99811-0693
Main: 907.465.3250
Fax: 907.465.1398

February 28, 2017

Representative Jason Grenn
Alaska State Capitol, Room 418
Juneau, AK 99801-1182

Subject: Support for HB 43, New Drugs for the Terminally Ill

Dear Representative Grenn:

The Alaska Commission on Aging (ACoA) is pleased to offer our support for HB 43, a bill authored by you and co-sponsored by Representatives Scott Kawasaki, Les Gara, and Harriet Drummond, that would allow terminally ill patients who have exhausted other available treatments the "right to try" investigational treatments after consultation with their doctors and to provide immunity to their prescribing physicians, manufacturers and distributors of new treatments that have not yet received federal approval.

End of life care is particularly important to older Alaskans and their families. In addition to the potential lifesaving measures that experimental drugs may offer, new treatments can also reduce the pain, discomfort, and inflammation that often accompany terminal illnesses and provide another option to use instead of narcotic drugs. Although the Food and Drug Administration has a process called "compassionate use" that provides patients with terminal illness access to unapproved treatments still in clinical trials, we understand that this process is onerous and can take considerable time to pursue, which is a luxury that a person at the end of life simply cannot afford.

Decisions regarding medical care are personal and belong to patients in consultation with their doctors. If a patient is willing to try a new treatment and understands the risk, and the doctor believes that the treatment may help the patient more than anything else that is available, then the patient with limited and precious time should have the final say in their treatment. Further, patients also have the right to know that these treatments are costly and typically not covered by private insurance and public funding. Ultimately, payment for an investigational treatment will depend on the agreement reached by the patient, his/her doctor, and the drug manufacturer. To this end, we recommend language to be included in the bill that would require medical practitioners to counsel their patients about costs and payment responsibility for unapproved treatments in order to promote transparency and reduce disappointment.

The Commission supports HB 43 and appreciates your leadership of this legislation. We believe that HB 43 will provide hope to Alaskans who suffer from a terminal illness by offering access to investigative treatment options and creating new research pathways to save lives. Please feel free to include the Commission's letter in the bill packet for HB 43.

Sincerely,

David A. Blacketer

David A. Blacketer
Chair, Alaska Commission on Aging

Sincerely,

Denise Daniello
ACoA Executive Director

Cc: Representative Scott Kawasaki
Representative Les Gara
Representative Harriet Drummond

POLICY report

Goldwater Institute

No. 266 | February 11, 2014

Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment

by Christina Corieri, Health Care Policy Analyst

EXECUTIVE SUMMARY

In 2002, Kianna Karnes, a 41-year-old mother of four children, was diagnosed with kidney cancer.[1] She was treated with interleukin-2, the only medication approved by the Food and Drug Administration (FDA) at the time to treat her disease. When that treatment failed, her father began researching investigational medications, learning in 2004 that both Pfizer and Bayer were conducting clinical trials for new investigational medications to treat kidney cancer. Karnes was ineligible for the clinical trial because her cancer had previously spread to her brain. Although her brain tumors had been removed, she was still disqualified from joining the clinical trial, so her father sought expanded access for his daughter. Months passed before he was able to secure access for his daughter. He contacted Congressman Dan Burton's (R-IN) office for assistance, and drew media coverage of Karnes' struggle in the *Wall Street Journal*. On March 24, 2005, the FDA notified the family that it had approved a single-patient IND for Karnes. Tragically, it was too late—Kianna Karnes died the same day access was approved.[2] Less than a year later, both drugs were given final FDA approval to treat advanced kidney cancer. Speaking after his daughter's death, her father said, "I don't know that either of these drugs would have saved Kianna's life, but wouldn't it be nice to give her a chance?"[3]

In the case of Kianna Karnes, she had a better chance than most patients at receiving expanded access. As her father explained, "Here is a case where her old man understood clinical trials. I knew about compassionate use; I had a friendship with a powerful member of Congress; I've got the *Wall Street Journal* behind me. But I still couldn't save her life. Now, what about the thousands of people out there who don't have these kinds of resources available to them?"[4] To most patients, and many physicians outside of major institutions, the process of obtaining expanded access is excessively time-consuming and extremely difficult to navigate.

For patients suffering from terminal illnesses, the FDA is the arbiter of life and death. These patients, suffering from diseases ranging from ALS to Zellweger Syndrome, face little chance of recovery. For patients like Kianna, investigational medicines provide a glimmer of hope. The FDA, however, often stands between the patients and the treatments that may alleviate their symptoms or provide a cure. To access these treatments, patients must either go through a lengthy FDA exemption process or wait for the treatments to receive FDA approval, which can take a decade or more and cost hundreds of millions of dollars. Sadly, over half a million cancer patients and thousands of patients with other terminal illnesses die each year as the bureaucratic wheels at the FDA slowly turn.[5]

Patients should be free to exercise a basic freedom – attempting to preserve one's own life. The burdens imposed on a terminal patient who fights to save his or her own life are a violation of personal liberty. Such people should have the option of accessing investigational drugs which have passed basic safety tests, provided there is a doctor's recommendation, informed consent, and the willingness of the manufacturer of the medication to make such drugs available.

States should enact "Right to Try" measures to protect the fundamental right of people to try to save their own lives. Designed by the Goldwater Institute, this initiative would allow terminal patients access to investigational drugs that have completed basic safety testing, thereby dramatically reducing paperwork, wait times and bureaucracy, and, most importantly, potentially saving lives.

GOLDWATER INSTITUTE

Proposed Statutory Language

Section 1 Title
This act may be cited as the "Right to Try Act"

Section 2 Findings
A. The process of approval for investigational drugs, biological products, and devices in the United States often takes many years.
B. Patients who have a terminal disease do not have the luxury of waiting until an investigational drug, biological product, or device receives final approval.
C. The standards of the Food and Drug Administration for the use of investigational drugs, biological produces, and devices may deny the benefits of potentially life-saving treatments to terminal patients.
D. The State of _____ recognizes that patients who have a terminal disease have a fundamental right to attempt to pursue the preservation of their own life by accessing available investigational drugs, biological products, and devices.
E. The use of available investigational drugs, biological products, and devices is a decision that should be made by the patient with a terminal disease in consultation with his or her physician not a decision to be made by the government.

A. Right to Try Act Definitions
1. Eligible Patient – an eligible patient is a person who meets the four requirements of eligibility in section B.
2. Investigational Drug, Biological Product, or Device – a drug, biological product or device which has successfully completed Phase One of clinical trials, but has not been approved for general use by the Food and Drug Administration. Additionally, the drug must currently be under investigation in an FDA clinical trial.
3. Terminal Disease – an advanced stage of a disease with an unfavorable prognosis and no known cure

B. Eligibility - In order for a patient to access an investigational drug, biological product, or device under this act, a physician must document in writing that the patient:
1. Has a terminal disease;
2. Has, in consultation with a physician, considered all other treatment options currently approved by the FDA;
3. Has been given a prescription or recommendation by a physician for an investigational drug, biological product, or device; and
4. Has given informed consent in writing for the use of the investigational drug,

biological product, or device. In the case that the patient is a minor or lacks the mental capacity to provide informed consent, a parent or legal guardian may provide informed consent on the patient's behalf.

C. Availability – a manufacturer of an investigational drug, biological product, or device has the option of making its investigational drug, biological product, or device available to eligible patients under this act. Nothing in this act shall be interpreted to require that a manufacturer make an investigational drug, biological product, or device available.

D. Costs

1. Manufacturers are permitted to provide an investigational drug, biological product, or device to eligible patients without receiving compensation.
2. Manufacturers may require eligible patients to pay the costs associated with the manufacture of the investigational drug, biological product, or device.

E. Insurance Coverage

1. Nothing in this act shall be interpreted as requiring any insurance company or government health care program to provide coverage for the cost of any investigational drug, biological product, or device.
2. Insurance companies may include coverage for investigational drugs, biological products, or devices.

F. Professional Licensing

1. No medical licensing board shall revoke a license, fail to renew a license, or take any other action against a license solely based on a medical professional's recommendation, prescription, or treatment with an investigational drug, biological product, or device.

G. Remedy

1. Any official, employee, or agent of the State of _____ who attempts to block or who does block access of an eligible patient to an investigational drug, biological product, or device shall be guilty of a Class One Misdemeanor punishable by up to six months imprisonment and up to a $2,500 fine.

H. Severability

1. If any provision of this act or its application to any person or circumstance is held to be invalid, the invalidity of such provision shall not affect any other provision of this act. The provisions of this act are severable.

"Patients should be free to exercise a basic freedom – attempting to preserve one's own life.

Introduction

Anna was only 13 years old when she died of an embryonal sarcoma, a rare form of liver cancer.[6] Six months before she died, she had exhausted all conventional therapies, and her doctors informed the family there was nothing more they could do. Her parents were not willing to accept the news without a fight. They began researching experimental medications and soon discovered a number of investigational drugs in clinical testing to treat sarcomas like Anna's. Anna's age and advanced diagnosis, however, disqualified her from participating in the clinical trials, leaving the Tomalis family with one only option – asking the FDA for permission for Anna to try investigational drugs through an expanded access program – the single patient IND.

For months, the family sought approval for expanded access for their daughter. However, the process was difficult, uncertain, and time consuming. Anna's mother said, "I came into this process so naïve, thinking that those of us who seek compassionate use of drugs actually get them. It was a shock to find out I had been seriously misled."[7] By the time the FDA finally granted access, it was too late. Anna died three weeks later, leaving her grieving family wondering whether Anna could have won her battle if she had been granted access sooner.

Before a drug can be made available to the general public, it must undergo a lengthy and expensive clinical trial process to determine its safety and efficacy, which takes on average 10 to 15 years and over $800 million dollars to complete.

The FDA strictly controls which medications are available in the United States. Before a drug can be made available to the general public, it must undergo a lengthy and expensive clinical trial process to determine its safety and efficacy, which takes on average 10 to 15 years and over $800 million dollars to complete.[8] Terminally ill patients can request exemptions, but the exemption process can take several months and requires doctors to complete paperwork that the FDA itself notes will require more than 100 hours to complete.[9] Ultimately, the decision still rests with the FDA.

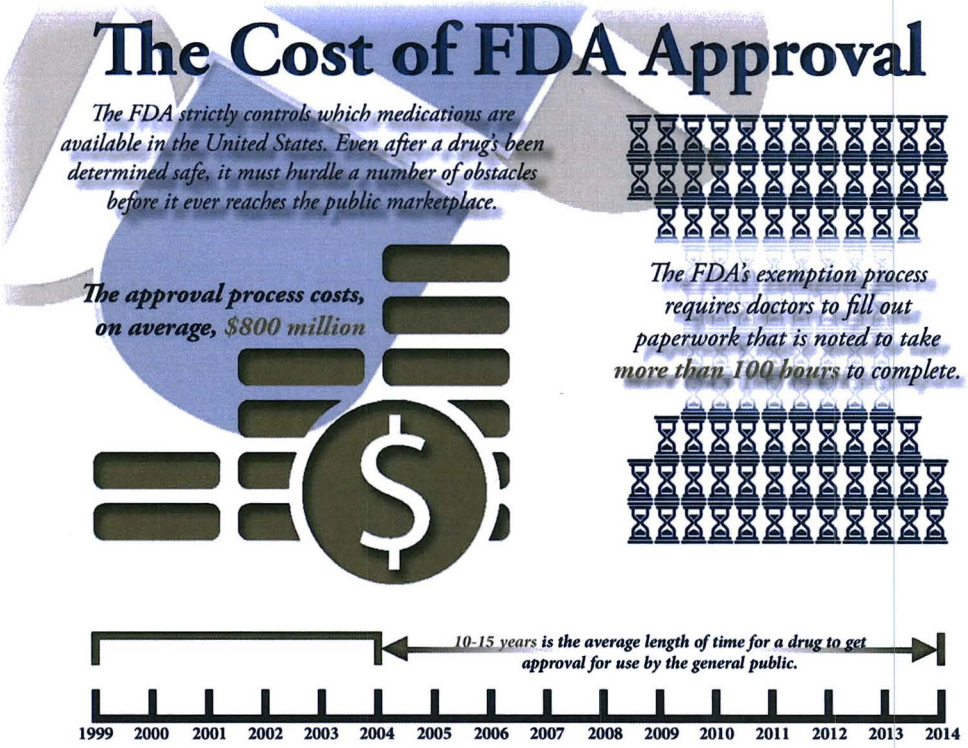
These bureaucratic impediments violate an individual's fundamental right to try to save his own life. Unfortunately, the federal government has shown little interest in reforming the FDA as bills to reform the process for terminal patients have been introduced, but have never received a vote in Congress. State legislators, however, have the opportunity to protect their citizens' right to try investigational medications by enacting Right to Try measures. These measures would ensure the right to protect one's life by returning medical decisions where they belong – to patients and doctors.

History of FDA Regulations of Medications

Today, the FDA possesses wide regulatory authority to control which drugs may be sold within the United States. This regulatory authority was not granted in one fell swoop, but was the result of over a half century of legislation. During the twentieth century, the FDA evolved from a minor bureau with only 28 food and drug inspectors into a

mammoth agency with a budget of nearly $4 billion.[10]

The Pure Food and Drug Act, passed in 1906, marked the beginning of federal regulation of drugs.[11] The regulation prohibited the manufacture or sale of adulterated or misbranded foods and drugs which were produced in federal territory or transported across state lines.[12] Enforcement of the act was given to the Bureau of Chemistry which was later renamed the FDA in 1927.[13]



> *During the twentieth century, the FDA evolved from a minor bureau with only 28 food and drug inspectors into a mammoth agency with a budget of nearly $4 billion.*[10]

Although there had been some earlier calls to require pre-market safety testing, it was due in large measure to the public outcry over the Elixir Sulfanilamide incident that Congress passed the Food, Drug, and Cosmetic Act of 1938 (FDCA). The previous year, Elixir Sulfanilamide, a drug which had been used for years in tablet and powder form to treat streptococcal infections, was converted to a liquid form.[14] The new liquid version of Elixir Sulfanilamide used diethylene glycol as a solvent, a poisonous compound.[15] Tragically, the company was unaware of the solvent's deadly effects.[16] Within days of the first shipments, the drug began to claim lives across the country. Before the drug could be recalled by the manufacturer, more than 100 people had died.[17] Congress responded

by passing the FDCA, which for the first time granted the FDA the authority to require pre-market safety testing of all new drugs.[18]

After the enactment of the FDCA, the FDA began to require pre-market testing for drug safety, however pre-market testing for efficacy was not required until the 1960's with the passage of the Kefauver-Harris Amendments.[19] The Kefauver-Harris Amendments were enacted as a direct result of worldwide Thalidomide-caused birth defects. Although Thalidomide was sold in 46 countries, it was never approved for sale in the United States due to the FDA's lingering safety concerns.[20] While over 10,000 children worldwide were born with birth defects attributed to Thalidomide, only 17 of those children were born in the United States, where access to the drug was limited to those patients undergoing the FDA safety trial.[21] The Kefauver-Harris Amendments drastically expanded the FDA's regulatory authority by requiring drug manufacturers to prove efficacy prior to being approved for sale.[22]

During the ensuing 50 years, everything in the medical world — from the way diseases are diagnosed and treated to the way medical records are kept — has been modernized, but the FDA continues to adhere to an approval process that is half a century old.

This vast new granting of power was unwarranted. Thalidomide presented a safety problem (over which the FDA already had authority) – not an efficacy problem. As a result of the Kefauver-Harris Amendments, no drug could be sold within the United States until it had been deemed both safe and effective by the FDA.[23]

In response, the FDA designed a clinical trial process which is substantially the same practice still in place today. During the ensuing 50 years, everything in the medical world — from the way diseases are diagnosed and treated to the way medical records are kept — has been modernized, but the FDA continues to adhere to an approval process that is half a century old.

Unfortunately, by clinging to this dated process, the FDA creates substantial barriers which inhibit a company's ability to bring new drugs to market in a timely fashion, even when those drugs have the potential to save lives.

Approving New Medications

New drugs are vitally important to improving the lives and health of Americans. Between 1986 and 2000, new drugs were responsible for 40 percent of the total increase in life expectancy.[24] Yet, the FDA's clinical trial process remains lengthy and expensive. It takes, on average, more than a decade and $800 million dollars (though the cost often can exceed a billion dollars) to bring a new drug from the laboratory to the market.[25] Polls show a clear majority of specialists believe the FDA clinical trial process is too slow and most report having been personally hindered in treating a patient due to the FDA approval process.[26]

The clinical trial process begins when a drug developer submits an Investigational New

Drug Application (IND) to the FDA.[27] The IND application includes all available data on the proposed investigational drug, including the results of any animal testing. In reviewing IND applications, the FDA seeks to ensure that the proposed trial does not expose patients to "unreasonable risk of harm."[28] Clinical trials then move ahead in three mandatory human testing phases.[29] Phase I involves administering the investigational drug to a small group of 20 to 80 volunteers to test for toxicity and immediately observable side effects.[30] The major emphasis of Phase I testing is safety. Over 60 percent of investigational drugs in Phase I testing are deemed safe enough to move on to Phase II.[31]

While safety continues to be evaluated, the main focus of Phase II is the drug's effectiveness in treating the targeted disease or condition.[32] Approximately one-third of the drugs in Phase II trials show enough evidence of efficacy to move on to Phase III.[33]

During Phase III, a much larger group of individuals receive the drug as the sponsor gathers additional evidence of efficacy by studying the drug's effects in diverse populations, different dosages, and in combination with other medications. One rationale for Phase III is that as more patients are treated with the investigational drug, less common side effects are more likely to be discovered.[34] During Phase III, the drug is administered to hundreds or even thousands of individuals.

Outside of participating in a clinical trial, patients have few options to access potentially life-saving drugs.

Upon completion of Phase III, a drug sponsor may submit a New Drug Application (NDA) to the FDA's Center for Drug Evaluation and Research (CDER) for review.[35] The FDA then has 60 days to consider the NDA to determine if the application should move forward and filed for FDA review. The final review process can then take up to a year.[36] To obtain final approval, the FDA requires that data amassed from the clinical trials indicate "substantial evidence" of both safety and effectiveness.[37] According to the former head of the CDER, Janet Woodcock, the FDA approves approximately 75 percent of all filed NDAs.[38]

Clinical trials offer a way for patients to access investigational medications, but many of the sickest individuals are barred from participation. An estimated 97 percent of the sickest patients are ineligible for or otherwise lack access to clinical trials.[39] Outside of participating in a clinical trial, patients have few options to access promising drugs.

The Era of Patient Activism and Demands for Change

Prior to the emergence of AIDS in the 1980's, access to investigational drugs was limited almost exclusively to patients admitted into clinical trials.[40] With the outbreak of AIDS, the FDA faced a group of patients who lacked any available treatment options.

AIDS was first identified by the Center for Disease Control in 1981 and spread rapidly

among certain population groups.[41] With an average of 10 years to bring a drug to market and no known treatments available, an AIDS diagnosis in the early 1980's was akin to a death sentence.[42] By April 1986, only 200 to 300 AIDS patients out of tens of thousands had been allowed to participate in clinical trials.[43] An FDA official asserted that embarking on a wider scale clinical trial to provide expanded access would be "wasteful of resources."[44] Despite calls from AIDS patients desperate for any chance, the FDA clung to its assertion that it was simply protecting patients from potentially ineffective drugs. For these patients, confronted with a terminal diagnosis, questions of efficacy and side effects were irrelevant. One patient explained, "I know what the side effects of untreated AIDS are. Based on past experience, there's a 75 percent chance I'll be dead in two years."[45] These patients, who faced imminent death, began to demand access to drugs for which efficacy was unknown. This was the beginning of the movement for the recognition of terminal-patient rights.

FDA Expanded Access Programs

The promulgation of the 1987 expanded access regulations marked the first time the FDA had formalized an expanded access program to allow patients, under very limited circumstances, to access investigational drugs prior to final FDA approval.

In response to AIDS patients' demands for access to investigational drugs, the FDA began its first formal expanded access programs to allow limited access to patients outside the clinical-trial setting. While these new expanded access programs were a step forward for terminal patients, they proved largely ineffective at solving the problem of access. The promulgation of the 1987 expanded access regulations marked the first time the FDA had formalized an expanded access program to allow patients, under very limited circumstances, to access investigational drugs prior to final FDA approval. Expanded Access Programs (EAPs), including treatment INDs and later individual INDs, are often referred to colloquially as "compassionate use" programs.

The first formal expanded access program was the treatment investigational new drug (treatment IND) application process, which began in 1987.[46] Under this program, a company sponsoring a clinical trial may submit a treatment IND application requesting FDA permission to allow specific groups of terminal patients to use the drug prior to FDA approval outside of the clinical trial.[47] Treatment INDs are generally limited to investigational drugs that are in Phase III of clinical trials or have completed Phase III and are awaiting NDA approval. Although regulations permit granting a treatment IND during Phase II, such instances are rare.[48] As the FDA describes it, for the agency to consider a treatment IND, the clinical trials must be "well underway, if not almost finished."[49] The FDA may approve the application if the clinical trials show promising evidence of the drug's efficacy. If the treatment IND is approved, the sponsor of the investigational drug may begin providing access to a predefined patient group outside the ongoing trial setting.

While AIDS activist Martin Delaney called the new policy a "giant step for the sick and dying," treatment INDs did not prove to be the boon that many patients hoped.[50] Following the expanded access program, access to investigational drugs did not expand

in a significant measure. By March 1990, the FDA had approved 18 treatment INDs for various conditions, which gave almost 20,000 patients who were otherwise ineligible for clinical trials access to investigational drugs.[51] With tens of thousands of AIDS patients and over one and a half million cancer diagnoses each year, 20,000 was a minor improvement.[52] In fact, from 1987 until 2002, the FDA approved only 44 treatment IND applications for conditions ranging from AIDS to chronic pain – an average of less than three per year.[53]

In 1997, 10 years after the first expanded access program, the FDA approved the individual, also called single-patient IND. Unlike treatment INDs, which grant access to a wider group of patients, the single-patient IND is designed to allow an individual patient who is otherwise ineligible for a clinical trial to obtain access to an investigational drug. An application for a single-patient IND may be submitted by either the patient's doctor or the sponsor of the investigational drug.

Although the FDA had occasionally permitted individual patients to use investigational drugs outside of clinical trials, there were no formal rules governing how such grants were authorized prior to 1997. Because of concerns that the informal process was arbitrary and inconsistent, the issue was addressed as part of the Food and Drug Administration Modernization Act (FDAMA) of 1997. FDAMA specifies that single-patient INDs are permissible only when all of the following conditions are met:

From 1987 until 2002, the FDA approved only 44 treatment IND applications for conditions ranging from AIDS to chronic pain – an average of less than three per year.[53]

1. The patient's physician determines the patient has no comparable or satisfactory alternative therapy;
2. The FDA determines there is sufficient evidence of safety and effectiveness to support the use of the investigational drug;
3. The FDA determines that provision of the investigational drug will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval; and
4. The sponsor or clinical investigator submits information sufficient to satisfy the IND requirements.

Submission of an application for a single-patient IND is only permissible when the sponsor of the investigational drug has expressed willingness to supply the drug to the patient. If the sponsor is willing to provide access, the treating physician or the drug's sponsor submits an IND application, an outline of the patient's medical history, a proposed treatment plan, and a commitment to obtain informed consent from the patient and Institutional Review Board (IRB) approval.[54]

Although the FDA claims the paperwork burden placed on doctors who wish to apply for a single-patient IND on behalf of a patient is "non-labor intensive, straightforward, and appropriate," the burden is actually quite extensive.[55] The application itself reads,

"the burden of time for this collection of information is estimated to average 100 hours per response, including the time to review instructions, search existing data sources, gather and maintain the data needed and complete and review the collection of information."[56] In rare situations, the request may be made over the phone, but the complex paperwork must still be completed soon after the initial verbal request.[57] Although the FDA may believe the filing of an IND to be a small burden on physicians, members of the medical profession feel different. As Dr. Judy Stone, a physician with an independent practice explained, "Except perhaps for academic settings with an extensive infrastructure, INDs are incredibly burdensome, time-consuming, and expensive for an independent practitioner to obtain. They involve hours of paperwork. My office practice consisted of me and 1-1.5 secretaries. Who has time?"[58]

Once a single-patient IND application has been submitted, the FDA has 30 days to review the application.[59] During this time, the FDA assesses risks and benefits posed to the patient (an analysis already performed by the treating physician), including whether there is enough evidence of the drug's efficacy, and whether allowing access by a patient outside the clinical trial setting would harm the on-going clinical-trial. Although the FDA grants most single-patient INDs, the FDA retains the power to refuse an application in spite of the treating physician's belief that the investigational drug represents the patient's last hope.[60]

While the FDA is tasked with protecting the public from unsafe and ineffective medications, the agency's approach is inappropriate in the context of terminally ill patients.

Burdens of the FDA's Expanded Access Programs

"The decision for terminally ill patients to take an investigational drug should be between the physician and the patient, not government bureaucrats." ~ Senator Sam Brownback (R-KS)

While the FDA is tasked with protecting the public from unsafe and ineffective medications, the agency's approach is inappropriate in the context of terminally ill patients. The terminally ill face a much different risk-benefit analysis than the public at large. Patients who are not battling an immediately life-threatening illness are likely less risk-tolerant and more willing to wait for a proven cure, but terminal patients do not have the luxury of time. Many terminal patients who lack other treatment options may be willing, even eager, to try medications whose efficacy has not yet been established. Even the FDA has recognized that "for a person with a serious or life-threatening disease, who lacks a satisfactory therapy, a promising, but not yet fully evaluated product may represent the best available choice."[61]

Despite this promising observation by the FDA, as of August 18, 2013, there were over 60,000 ongoing clinical trials, but only 210 ongoing expanded access trials.[62] This number includes both treatment INDs and single-patient INDs. Reports from previous years show a similarly small number of patients gaining expanded access. In 2011, just shy of 1,200 patients received expanded access through either a single-patient or

treatment IND.[63] While the total had slightly increased from 1,014 patients in 2010, this is a very small number considering that, in that same year, there were 1,529,560 new cancer cases.[64] In 2012, the number of patients granted expanded access dropped down to a mere 940.[65] The onerous process the FDA requires a patient to go through to request expanded access contributes to the number being so low.

Despite the real possibility of death that is ever-present for terminal patients, the FDA persists in burdening a person's right to try to save his own life by preventing access to investigational medications in three distinct ways. First, by requiring physicians to complete an IND for each request for single-patient expanded access, the FDA discourages doctors from even attempting to obtain access for their patients. Second, the FDA has unfettered authority to deny a terminal patient access to potentially life-saving medications for a variety of reasons, including nonmedical reasons. Third, the FDA's requirement that all applications approved by the agency must then receive approval from an institutional review board further delays and inhibits access for patients in smaller and rural treatment centers. Together, these burdens create significant delays that can further endanger a person's life.

Forty percent of cancer patients attempt to enroll in clinical trials.[66] Many of these patients are turned away because they do not meet the stringent eligibility requirements or because they do not live near or have the ability to travel to a medical facility where the trial is being conducted.

The Burden of the IND Application

The requirement that physicians complete an IND for each request for single-patient expanded access is a significant hurdle standing between terminally ill patients and potentially life-saving medications. While some amount of paperwork may be reasonable, this form is so needlessly lengthy and complex that few doctors are willing or able to complete it.

Forty percent of cancer patients attempt to enroll in clinical trials.[66] Many of these patients are turned away because they do not meet the stringent eligibility requirements or because they do not live near or have the ability to travel to a medical facility where the trial is being conducted.[67] With more than a half-million deaths due to cancer every year in the United States and such a high level of interest from cancer patients in obtaining investigational medications, one would assume there would be a significant number of applications for expanded access to these medications every year. Yet, the average number of single-patient IND applications granted access to investigational medications for the last three years has been only 544. The burdensome IND application required by the FDA explains why the number is so low.

The FDA is aware of the fact that the IND application requirement creates a serious impediment that discourages doctors from applying for single-patient expanded access. This is illustrated in the recent FDA attempt to require an IND application for fecal transplants.[68]

Polling reveals that specialty doctors, who are the most likely to treat terminal patients, recognize the problems inherent in the current FDA policy and support earlier access to investigational medications.

Would you say the FDA's approval process has hurt your ability to treat your patients with the best possible care?

Yes

- 80% of neurologists and neurosurgeons
- 78% of orthopedic surgeons
- 77% of oncologists
- 71% of cardiologists
- 58% emergency room doctors

Do you agree or disagree with the following statement: "The FDA is too slow in approving new drugs and medical devices"

Agree

- 76% of orthopedic surgeons
- 67% of neurologists/neurosurgeons
- 65% of cardiologists
- 64% of emergency room doctors
- 61% of oncologists

Would you support a "proposal to change FDA law so that unapproved drugs or medical devices could be made available to physicians as long as they carried a warning label about their unapproved status?"

Yes

- 73% neurologists/neurosurgeons
- 70% of orthopedic surgeons
- 69% of emergency room doctors
- 68% of oncologists

Source: http://cei.org/sites/default/files/The%20Polling%20Company%20-%20A%20National%20Survey%20of%20Orthopedic%20Surgeons%20Regarding%20the%20Food%20and%20Drug%20Administration%20and%20the%20Availability%20of%20New%20Therapies.pdf

Polling reveals that specialty doctors, who are the most likely to treat terminal patients, recognize the problems inherent in the current FDA policy and support earlier access to investigational medications.

Such transplants are used to treat patients suffering from recurrent clostridium difficile infections. According to the Center for Disease Control, approximately 14,000 Americans die each year from clostridium difficile, but fecal transplants promise to greatly reduce that number.[69] A recent study by the New England Journal of Medicine revealed that 81 percent of patients with a clostridium difficile infection were cured after the first transplant, and that number increases to 94 percent after a second transplant

from a new donor.[70] Despite the fact that clinicians have been providing this treatment with a very high success rate, the FDA announced in the spring of 2013 that henceforth physicians would need to seek an IND for each treatment.[71] The outcry from physicians against this new requirement was swift.

Requiring an IND places a huge burden on doctors in terms of both time and cost – a burden that will result in fewer doctors who are willing to perform the procedure. As one gastroenterologist noted, "I'm already seeing that because of this requirement, a lot of doctors that were doing fecal transplants have either shut down or put their patients on hold."[72] Dr. Trevor Van Schooneveld of the University of Nebraska Medical Center had performed 20 fecal transplants since 2011, but after the FDA instituted the IND requirement, he had to delay treatment for three patients while he prepared and submitted an IND for each patient.[73] Of course, not all doctors are able to put in the extensive time necessary to complete an IND, leading many to opt out of offering the procedure altogether.

Completion of an IND is complicated and time-consuming. When she was informed that the FDA would be requiring an IND for each transplant, Dr. Colleen Kelly, who had previous experience in completing INDs, began the process of filing an IND for the procedure. "I literally cleared my schedule in the office for two weeks of 12-hour days. The IND process is not ideal. There's no 'IND for Dummies.' When you're a doctor who wants to do this, it's not a real straightforward process."[74] Furthermore, physicians are prohibited from submitting an individual patient access protocol to an existing IND for which the physician is not a sponsor, which means that a physician unfamiliar with the IND procedure cannot avail himself of a successful IND submitted by another physician.[75] Dr. Kelly was not the only physician to take note of the IND burden. Another doctor complained of the increased cost, stating, "Just putting [an IND] together and carrying it out and managing data to the level of sophistication required by the FDA, just running it all costs a lot of money."[76] Patients have expressed their concerns as well. Barat McClain, whose clostridium difficile had been treated and cured with a transplant, said, "I fear many doctors will say, 'It's just a procedure I can't afford to do. Time is money, and I can't afford to spend my precious time filling out the damn forms.'"[77]

Despite the fact that clinicians have been providing this treatment with a very high success rate, the FDA announced in the spring of 2013 that henceforth physicians would need to seek an IND for each treatment.

After receiving warnings from patients, physicians, and organizations such as the American Gastroenterological Association cautioning that requiring physicians to complete an IND for each transplant would result in the virtual elimination of this life-saving procedure, the FDA abruptly reversed course.[78] On July 18, 2013, the agency released guidance for the transplants. The guidance was issued without prior public participation because such public participation was "not feasible or appropriate" as the subject dealt with "an urgent issue affecting patients with life-threatening infections."[79] In response to provider warnings that requiring an IND would essentially make fecal

transplants largely unavailable, the FDA decided not to require an IND for each procedure provided there was adequate informed consent by the patient. The objective of the guidance was to "ensure widespread availability of FMT [fecal microbiota transplants]."[80] In doing so, the FDA openly conceded that requiring individual INDs seriously inhibits, if not eviscerates, access to life-saving medical procedures.

FDA officials have stated that the agency wants patients with life-threatening diseases to have "early access to promising medical interventions."[81] Despite that oft-repeated statement, the FDA requires the completion of an IND that the agency has admitted makes certain procedures largely unavailable, especially since many doctors lack the time or expertise to deal with the burdensome application. In a recent survey, 60 percent of orthopedic surgeons said that the FDA hindered their ability to use "promising new drugs and medical devices."[82] In fact, studies show that among the reasons many doctors do not participate in clinical trials is the overly rigid protocols, concern about uncompensated staff time, lack of resources, and the burden of data management.[83] Many of these concerns would be mitigated by eliminating the IND requirement.

The FDA burdens the rights of terminal patients by claiming the authority to override both the will of the patient and the recommendation of a doctor by bureaucratic veto.

The FDA's Expansive Veto Power

Next, the FDA burdens the rights of terminal patients by claiming the authority to override both the will of the patient and the recommendation of a doctor by bureaucratic veto. The law allows the FDA to deny an individual request for expanded access if the agency believes there is insufficient evidence of either safety or efficacy, or if the agency determines that allowing access will interfere with clinical investigations.[84] While on the surface this appears to be the worst of the three burdens, in reality, by making the IND so complicated and time consuming, most requests never even make it to this stage. Even so, it is troubling that when a doctor has taken the time to complete an IND and the company sponsoring the clinical trial has agreed to provide the patient access to the investigational drug, the FDA still has the power to deny the will of the patient, the advice of the doctor, and the charity of the sponsor. The FDA has acknowledged that people might question why, if a doctor already determined an investigational drug represents the last and best hope for a terminal patient and the patient is willing to assume the risk, the FDA should have veto power.

Michael Friedman, the Lead Deputy Commissioner of the FDA, addressed this very question during congressional testimony, stating, "In a typical single-patient IND situation, especially those involving emergency IND requests, the patient's physician may have only limited information about the investigational therapy being requested."[85] It is certainly true that information available during a clinical trial is limited, but the information is equally limited to patients enrolled in the ongoing clinical trial of the same investigational drug. For patients in the clinical trial process, the FDA deals with the lack of information not by banning access but by requiring informed consent to

ensure that participants are aware of the possibility the drug could cause unknown side effects. Terminal patients should be afforded that same opportunity. As one father who fought to gain expanded access for his daughter explained, "If the only alternative is death, then for God's sake, let 'em have the drug."[86]

According to FDA officials, "the Agency's primary role in deciding whether to allow a single-patient IND to proceed is to determine whether use of the therapy in the particular patient involved is reasonable."[87] Although the FDA believes each request should be evaluated individually, the agency maintains there could be times when two people with the same life-threatening illness may receive different responses to IND applications, just as there may be circumstances which "make the risks acceptable for one patient, but not for another."[88] The reasonableness of a course of treatment, however, is not an objective fact that can be ascertained by a bureaucrat reviewing records – it is a deeply personal decision that should be made by the patient in consultation with his or her doctor and should not be second-guessed by government officials.

The FDA disagrees. As Patty Delaney, the former director of the FDA's cancer liaison program explained in 2007, "the patient has a right to be heard, but in the end, it's the data that matters. FDA opinions about safety and efficacy are always based on data."[89] If a trained medical doctor believes that, given the patient's diagnosis and medical history, the patient's best and perhaps only chance at life is to try an investigational medication and the sponsoring drug company is willing to supply the medication, the FDA should not have authority to overrule both the advice of the doctor and the wishes of the patient.

If a trained medical doctor believes that, given the patient's diagnosis and medical history...the FDA should not have authority to overrule both the advice of the doctor and the wishes of the patient.

By the beginning of Phase II of a clinical trial, the FDA has already seen enough evidence of a drug's safety to allow it to be tested on an expanded group of subjects. While the FDA talks at length about the potential risks expanded access patients would be exposed to, the risk to an individual patient outside the clinical trial is no greater than the risk the FDA is permitting patients inside the trial to take. No one expects investigational medications to be a panacea that will cure all those who use them, and indeed it is impossible to say how many will be helped by these medications. What can be said is the number who will be helped is unquestionably greater than zero. Patients and their families understand this, and most are realistic in their expectations. They are simply looking for a chance. As Jonathan Agin, a father of a young girl who was unable to obtain expanded access, explained in the *Huffington Post*, "We will never know whether the drugs we were not afforded access to could have helped Alexis. This is a heavy burden to shoulder in two simple words, 'what if.'"[90] That is a burden no parent should have to bear, yet it is a burden the FDA imposes.

If there is a chance for improvement and the patient is willing to accept the risk, which is no greater than the risk posed to any other patient enrolled in the ongoing clinical trial, government should not stand in the way. No government agency should have

the authority to deny a terminal patient access to potentially life-saving medications, especially those already deemed safe enough for expanded human trials.

Perhaps the most troubling argument in favor of the FDA's veto power is that the agency is always mindful of the effect expanded access may have on the clinical-trial process.[91] As one FDA official put it, "An individual with a life-threatening and chronic illness for which there is no adequate remedy has a compelling case. As compelling as an individual case is, however, the cost of providing individual access cannot be to sacrifice the system that ultimately establishes whether therapies are safe and effective."[92] Mr. Friedman was referring to nonmedical reasons why the FDA may deny an IND application. In discussing why the agency might deny IND requests, the FDA recently noted that the "FDA could also have become aware, since authorizing previous requests for access, that access is impeding the clinical development of the drug and, on that ground, deny further requests for access."[93] The practical result is that a person who does not qualify for the clinical trial of an investigational drug could be denied access simply because there are not enough participants enrolled in the trial.

The practical result is that a person who does not qualify for the clinical trial of an investigational drug could be denied access simply because there are not enough participants enrolled in the trial.

The FDA is concerned that allowing wider access to investigational medications outside the clinical trial setting will create a lack of test subjects who are willing to join a clinical trial, because in clinical trials some patients receive placebos or already-approved medications instead of the investigational drug.[94] The agency argues that freer access to such medications would discourage enrollment in the double blind clinical trials and ultimately harm scientific understanding of the medications. Therefore, the FDA puts protection of the clinical-trial process above the lives of terminally ill patients.

Beyond the lack of humanity inherent in this policy, there are additional flaws to the FDA's position. Experimental medications designed to treat terminal illnesses are only a subset of the drugs undergoing clinical trials. The FDA's position makes the assumption that the current clinical trial process, complete with the double blind studies, is the only sound way to test new medications. However, many scholars and even the former Director of the FDA, Andrew von Eschenbach, have urged alternatives to the current clinical trial process. [97] Nevertheless, the agency continues to place its outdated processes above all other concerns.

The Inequity of IRB Review Requirement

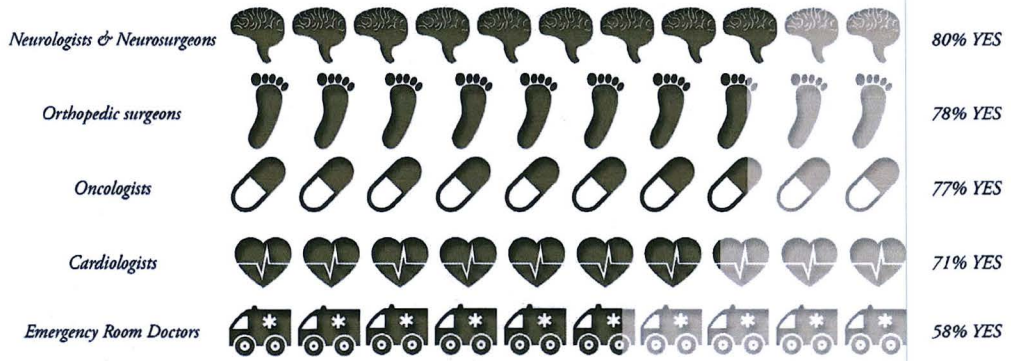
An additional way in which the FDA burdens the rights of terminal patients is to require that even when the agency grants access, the patient's treatment must await review by an IRB.

An IRB is an independent board, often affiliated with a major medical or research institutions that must be registered with the FDA. An IRB is composed of at least five

individuals with varied backgrounds, who review all IND applications for the purpose of protecting the welfare of human subjects undergoing clinical trials.[98] IRB review is required for all IND applications before treatment may begin.[99]

IND applications for single-patient use are subject to "full IRB review." Full IRB review means that the IND must be considered at a convened meeting at which a majority of the IRB members are present, including at least one member whose primary concerns are in nonscientific areas.[100] To be allowed to proceed to treatment, the IND must be approved by a majority vote of the members present at the meeting.[101] Although some IRBs at major academic institutions meet on a weekly basis, many IRBs meet only once a month. This can cause additional delays for a patient seeking the use of investigational

Would you say the FDA's approval process has hurt your ability to treat your patients with the best possible care?



Most IRBs are located at major academic research institutions and large hospitals, many of which prioritize the review of applications originating from within their own institution over outside applications.

medications, as treatment cannot begin before full IRB approval has been granted.

Additionally, the requirement for full IRB review creates a barrier for patients located in rural regions or who are being treated at smaller hospitals. Most IRBs are located at major academic research institutions and large hospitals, many of which prioritize the review of applications originating from within their own institution over outside applications. The FDA is aware of this barrier and in October of 2011 asked HHS's Secretary's Advisory Committee on Human Research Protection (SACHRP) to study the issue.[102] The report generated by SACHRP stated that "substantial barriers" exist that inhibit access to investigational drugs and that these barriers are "exacerbated for physicians and patients outside of an institutional setting" in large part because of the requirement of full IRB review.[103] Thus, the practical result of the IRB requirement is that patients in rural areas or who otherwise lack access to large medical institutions will, in many cases, lack the opportunity to obtain expanded access to investigational medications. The American

Pharmacists Association describes the requirement of full IRB review as "prohibitively costly" and "burdensome," and asserts its firmly held belief that the requirement "creates an impossible and undue burden on medical doctors treating individual patients in a community clinical setting."[104]

The FDA's requirement for full IRB review of all applications for single-patient INDs delays and limits access to investigational medications. The FDA itself recently noted that the agency "is aware of concerns that this requirement for full IRB review may deter individual patient access to investigational drugs for treatment use," especially for patients "in settings in which IRB review is not readily accessible (e.g., health care settings that do not have IRBs)."[105]

Bureaucratic Delays Endanger Lives

The FDA's long, costly, and burdensome process makes it difficult for patients to get the medications that may save their lives.

The FDA's long, costly, and burdensome process makes it difficult for patients to get the medications that may save their lives. Take the case of Everett Davis. At the age of 17, he was diagnosed with paroxysmal nocturnal hemoglobinuria (PNH).[106] The disease caused the formation of major blood clots and caused his kidneys to fail. For two years, his condition escalated until his hematologist was convinced his only chance for survival was an investigational drug called Soliris. After Davis and his family made countless calls to lawmakers to seek assistance in obtaining access to Soliris, Davis was eventually granted expanded access. The drug was so successful in improving his condition that within weeks Davis was approved for the transplant list that had previously been denied him. Luckily for Davis, his expanded access approval "came at the exact right moment" and the medication followed by the transplant saved his life.[107] Sadly, many are not as fortunate.

Dr. Mark Puder of Boston Children's Hospital has spent years treating infants who have fatal liver disease using a promising investigational medication called Omegaven.[108] The FDA has permitted the medication to be given to patients through an expanded access program. A former FDA official, Dr. Timothy Cote, argues that the FDA's expanded access application process is appropriate even in cases such as this where an infant is facing death.[109] But a bureaucratic delay of weeks or months can mean the difference between life and death. As Dr. Puder explained, "The problem with this disease is it's so rapidly progressive that you may lose the time to be able to rescue them. So, if their liver disease is bad at two months, and then it's at four months now, you've hit a point where there's a point of no return."[110]

Patients and their families should not have to wait for bureaucratic whims to turn in their favor. When patients are facing terminal diseases, every day counts. Each extra day that it takes a doctor to fill out copious amounts of administrative paperwork, a bureaucrat to review an application, or to get on the schedule for an IRB, brings the

patient a day closer to death and gives the possibly life-saving medications less time to work. Such procedural delays and hurdles threaten the lives of patients and should not be tolerated. We must move to protect the right of patients to access potentially life-saving medications.

Resisting Change

The FDA is extremely sensitive to the fact that every time it approves a new medication, the agency puts its reputation and power at risk. If the medication should later prove dangerous, the FDA will come under intense scrutiny from the media and Congress. In contrast, if the FDA is slow to approve a new medication, insisting upon more and more testing, the risk of scrutiny is much lower. This makes the distinction between the FDA committing a type one versus a type two error very important.

A type one error occurs when the agency approves a medication that is later discovered to produce serious side effects. In the case of a type one error, victims are clearly identifiable and visible to both the media and lawmakers. While the FDA can take corrective action, the damage to the agency's reputation will have already been done as it faces media and legal scrutiny. Dr. Henry Miller, the founding director of the FDA's Office of Biotechnology, illustrated the deep-seated fear the agency has of these type one errors. Dr. Miller described an instance in which he possessed reams of data detailing both the efficacy and safety of a new drug only to have his supervisor hedge on the approval after two and a half years of clinical trials, stating, "If anything goes wrong, think how bad it will look that we approved the drug so quickly."[111]

If the medication should later prove dangerous, the FDA will come under intense scrutiny from the media and Congress. In contrast, if the FDA is slow to approve a new medication, insisting upon more and more testing, the risk of scrutiny is much lower.

A type two error occurs when the agency moves slowly and delays approving a beneficial medication. Although a type two error will result in needless deaths as patients await approval of the medication, the victims are largely unidentifiable. Without identifiable victims to be paraded in front of the cameras or a congressional committee, the danger to the FDA's reputation is significantly less.

As underscored by Dr. Miller, the FDA is aware of the comparative danger to its reputation posed by type one and type two errors. The agency's sensitivity to this issue is clearly reflected in the statements of former FDA Commissioner Alexander Schmidt who noted "in all our FDA history, we are unable to find a single instance where a Congressional committee investigated the failure of the FDA to approve a new drug. But the times when hearings have been held to criticize our approval of a new drug have been so frequent that we have not been able to count them."[112]

Legislators Must Act to Protect Patients

"The decision on what course of action to take is the patient's. After given the facts, if someone with a life-threatening or terminal illness wants to seek treatments that may offer a cure or slowdown in the progression of disease, then Federal agencies and red tape should not stand in their way." ~ Congressman Dan Burton (R-CA)[113]

The delays and denials, which are inherent in the FDA's current expanded access policy, have prompted recent attempts at the federal level to broaden access for terminal patients. Since 2008, four such bills have been introduced in Congress.[114] Although these bills all had bi-partisan sponsors, none received a vote in committee, let alone a floor vote. Despite such federal inaction, there is no right more basic than the right of the individual to protect his or her own life. The law recognizes this natural right by acknowledging a person's right to self-defense. Individuals have the right to defend their lives. Through the lengthy approval process, the government has effectively denied the individual's right to try to preserve his or her own life.

The delays and denials, which are inherent in the FDA's current expanded access policy, have prompted recent attempts at the federal level to broaden access for terminal patients

To protect the rights of patients with immediately life-threatening conditions, states should pass "Right to Try" legislation. Right to Try declares that the right of a terminal patient to access available investigational medications, devices, or biological products is a fundamental right and prohibits any government or government agent from interfering with that right.[115]

The Right to Try model legislation (Appendix A) designed by the Goldwater Institute is narrowly tailored and addresses many of the concerns that the FDA and others have expressed. To address the legitimate government interest of protecting the lives of citizens, Right to Try only allows access to medications that have passed basic safety testing (Phase I).[116] Further, this legislation does not allow unfettered access to such medications after Phase I. It is limited to investigational medications for terminal patients who have exhausted other available treatments.[117] Finally, the investigational medications are only available to patients under Right to Try if the sponsoring company chooses to make them available.[118]

Simply stated, Right to Try allows a patient to access investigational medications that have passed basic safety tests without interference by the government when the following conditions are met:[119]

1. The patient has been diagnosed with a terminal disease;[120]
2. The patient has considered all available treatment options;[121]
3. The patient's doctor has recommended that the investigational drug, device, or biological product represents the patient's best chance at survival;[122]
4. The patient or the patient's guardian has provided informed consent;[123] and

5. The sponsoring company chooses to make the investigational drug available to patients outside the clinical trial.[124]

For patients suffering from conditions for which there is no approved known cure, the FDA's traditional role of protecting patients from drugs and devices that have not yet proven effective has little meaning. These medications have already been deemed safe enough to enlarge the group of patients involved in the clinical trial to several hundred or even several thousand individuals. The requirement for informed consent ensures that terminal patients considering this option are fully aware of the risks involved. Moreover, allowing earlier access to investigational medications with informed consent is supported by the medical community. Recent studies show that a clear majority of specialists, including neurologists, oncologists, orthopedic surgeons, and emergency-room doctors support making investigational drugs available prior to full FDA approval.[125] Further, the Right to Try initiative allows the company producing the investigational medication or device to determine whether it will be made available.[126] If a company does not wish to make a medication available due to lack of adequate inventory, fear of liability, or any other reason, the company is not compelled to do so. Furthermore, insurance companies are not compelled to provide coverage for investigational medications.[127] Thus, Right to Try protects a patient's right to medical autonomy without infringing on a company's rights.

For patients suffering from conditions for which there is no approved known cure, the FDA's traditional role of protecting patients from drugs and devices that have not yet proven effective has little meaning.

Constitutional Right to Medical Autonomy

It has long been established that the U.S. Constitution creates a floor of protection for individual rights – not a ceiling. States can and do provide additional and enhanced protections for individuals. For example, several states provide greater protections for speech or privacy than the U.S. Constitution does.

Additionally, the U.S. Supreme Court has recognized a series of fundamental rights protected by the Due Process Clause. These constitutionally protected rights include the right to marry, to use contraceptive medications, to live with one's family, and to teach children a foreign language.[128] Among the recognized fundamental rights, the Supreme Court has recognized several fundamental liberty interests in the area of medical autonomy. The right of a patient to control his own medical treatment has been a component of many due process cases, with the Supreme Court noting the existence of the "right to care for one's health and person."[129] Although the right of terminal patients to access investigational medications has not yet been recognized by the Supreme Court, it is consistent with and can be supported by existing precedent.

If Right to Try is upheld, the government would be restricted from placing excessive regulatory requirements on terminal patients seeking access to investigational medications. The result is that the FDA would not be able to prevent a terminally ill

patient who met the stated criteria from accessing investigational medications. Likewise, other procedural burdens such as the IND application and IRB review requirement could be deemed undue burdens and either eliminated or drastically curtailed.

The concept of ordered liberty cannot include allowing a government agency to promulgate and enforce regulations that impair an individual's health or cause death by denying or delaying access to potentially life-saving medications. The way in which the FDA currently regulates access to investigational medications may be rational for non-terminal patients, but its application to terminal patients, who lack other treatment options, is not. Preventing such a patient from accessing a potentially life-saving medication will, without question, result in the fulfillment of the diagnosis — death.

Without the action of state lawmakers, terminal patients are at the mercy of a federal bureaucracy that can literally cause death by delays, denials, and unnecessary procedural requirements.

"It has long been established that the U.S. Constitution creates a floor of protection for individual rights – not a ceiling. States can and do provide additional and enhanced protections for individuals.

Conclusion

From her sickbed, Edie Bacon wrote of the travails a terminal patient faces and made a final plea for the only medication that might save her. "The government wants proof of efficacy before it will allow me to take this drug outside of an approved trial. But the 'proof' is years away, and I need the drug now. It's safe. It might work. Johnson & Johnson would let me have it if they could do so without the threat of a government hassle. But they're so caught up in the FDA web that the life of an individual patient has no importance whatsoever. Without ET 743, I'm a dead woman walking. Five kids are going to wonder why they're left without a mother. Won't somebody help me get this drug?"[130] Edie died two years later, but there are thousands of patients who face this same battle every day – patients who have to make the same pleas that Edie did for a chance to try to protect their own lives.

Such pleas should anger anyone who believes in the concept of personal liberty. No free person should have to come to the government as a supplicant to beg for a right to try to save his or her own life. In a country dedicated to the idea that all people have certain "unalienable Rights, that among these are Life, Liberty, and the Pursuit of Happiness," no government official should have the power to deny a person's last chance at all three – life, liberty, and happiness.[131] Yet that is the power the FDA wields today. States should challenge this regulatory authority by passing Right to Try and returning medical decision making back to the rightful hands of patients and doctors.

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111. Dr. David Gratzer, *The Cure: How Capitalism Can Save American Health Care* (New York: Encounter Books, 2008).
112. Henry I. Miler, "The FDA at 100," *Medical Progress Today*, July 6, 2006, http://www.medicalprogresstoday.com/enewsletters/mpt_ind.php?pid=1293&nid=0
113. Opening Statement of Congressman Dan Burton, Committee Chairman, "Hearing on Compassionate Use of Investigational New Drugs: Is the Current Process Effective?" House

Committee on Government Reform, June 20, 2001, http://www.gpo.gov/fdsys/pkg/CHRG-107hhrg77088/html/CHRG-107hhrg77088.htm

114. Examples of recent attempts at reform at the federal level include the 2008 Access, Compassion, Care, and Ethics for Seriously Ill Patients Act (S. 3046), the 2010 Compassionate Access Act (H.R. 4732), the 2011 Access to Medical Treatment Act (H.R. 2736), and the 2012 Compassionate Freedom of Choice Act (H.R. 6342).
115. The full text of Right to Try is located in Appendix A
116. Right To Try Act, Section A(2)
117. Right To Try Act, Section B
118. Right To Try Act, Section C
119. See Appendix A for a full text of Right to Try.
120. Right to Try, Section B(1)
121. Right to Try, Section B(2)
122. Right to Try, Section B(3)
123. Right to Try, Section B(4)
124. Right to Try, Section C
125. "A National Survey of Orthopedic Surgeons Regarding the Food and Drug Administration and the Availability of New Therapies," Competitive Enterprise Institute, 2007, http://cei.org/sites/default/files/The%20Polling%20Company%20-%20A%20National%20Survey%20of%20Orthopedic%20Surgeons%20Regarding%20the%20Food%20and%20Drug%20Administration%20and%20the%20Availability%20of%20New%20Therapies.pdf
126. Right to Try, Section C
127. Right to Try, Section E
128. Loving v. Virginia, 388 U.S. 1 (1967); Griswold v. Connecticut, 381 U.S. 479 (1965); Roe v. Wade, 410 U.S. 113 (1973); Moore v. East Cleveland, 431 U.S. 494 (1977); Meyer v. Nebraska, 262 U.S. 390 (1923).
129. Doe v. Bolton, 410 U.S. 179, 218 (1973) (Douglas, J. concurring).
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