SUPPORT HB 14-1281
EXPAND CHOICES FOR TERMINALLY ILL INDIVIDUALS

**INTENT:** To allow terminally ill individuals to utilize drugs and treatments which have not yet completed the FDA approval process.

**THIS PROPOSAL SEeks TO:**

- **Expand Options** - Terminally ill individuals currently have very limited options available to them.
- **Create Accessibility** - Accessibility to experimental drugs can require volumes of paperwork and many months to obtain permission, if it can be obtained at all.
- **Eliminate Bureaucracy** - This proposal seeks to eliminate unnecessary procedural burdens and provide terminally ill individuals opportunities not currently available to them.

**FACTS:**

- These individuals are *terminally ill*!
- Only those drugs and procedures that *have already successfully completed Phase One of a clinical trial* would be allowed under this proposal.
- Terminally ill individuals *should have the right to make decisions* that could impact their length and quality of life.
- Will *not require insurance companies to cover these drugs and procedures, nor would it require drug companies to make them available*, it will only allow them the option to do so.
- *Thousands of patients have died* while waiting for the FDA to approve medications, or even permit compassionate use of them.
- The *individual will assume sole responsibility* for the results of their use of these drugs and treatments.
- *Clinical trials will be able to benefit from any new information* learned from these individuals.
- *The primary side effect of not allowing this access is death.*

**PLEASE SUPPORT HB 14-1281**
HB14-1281
Investigational Drugs; Biological Products; Devices

Fact Sheet
Right to Try

For terminal patients who have exhausted their conventional treatment options, obtaining access to potentially life-saving investigational drugs is often extremely difficult. The patient can attempt to enroll in a clinical trial, but many of the sickest individuals do not qualify to join such a trial. For these patients, their only hope for obtaining these potentially life-saving drugs is to request that the FDA grant them expanded access.

Only a few hundred patients are lucky enough to be granted individual expanded access each year. This is because of the complicated, time-consuming, and expensive process currently required by the FDA. First, the patient must locate a doctor who is willing to complete the required paperwork which the FDA notes will take a minimum of 100 hours to complete. Few doctors have the skill or time necessary to complete such an undertaking, especially when their time is not reimbursed by insurance. Assuming the doctor is willing and able to complete the paperwork and the patient can cover the cost of doing so, the FDA then has a month to review the submission and either grant or deny the request. The FDA may override the medical advice of the doctor and the will of the patient for a variety of medical and non-medical reasons.

The current process takes 3 to 6 months, there may be delays, and potential denials. Medical decisions, especially those of terminal patients, should be made by the doctor and patient. Terminal patients have the right to make their own medical decisions and if there is a chance to try an investigational drug when all options have failed, it should be a choice.

Under Right to Try, a terminal patient would be able to access an investigational drug when all the following conditions are satisfied:

1.) The patient has a terminal disease and has exhausted all conventional treatment options
2.) The patient’s doctor has advised the use of an investigational drug
3.) The investigational drug has successfully completed basic safety testing
4.) The patient has provided informed consent
5.) The company sponsoring the development of the investigational drug is willing to make it available to the patient.

The law includes important protections for all parties. The basic safety testing and informed consent requirements serve to protect the patient. The pharmaceutical company is protected because it cannot be compelled to make the drug available. Insurance companies are also protected because the law notes that insurance is not required to cover the costs related to the use of investigational drugs.

Right to Try protects the medical autonomy of terminal patients by allowing a path to access potentially life-saving drugs free of needless delays. This bill streamlines the process and does not interfere in the clinical trial. The terminally ill patient would be another arm of the trial.
Supporters of Right to Try

The Abigail Alliance is committed to helping create wider access to developmental cancer drugs and other drugs for serious life-threatening illnesses. The Alliance is promoting creative ways of increasing expanded access and compassionate use programs. We are working to help promote creative ideas to get promising new drugs to the market sooner.

ANP Coalition

It is our fundamental belief that the discovery of Antineoplastons (ANP) can and will herald a new age of medical science and subsequent advancements in the treatment of previously incurable diseases. This benign yet effective drug contradicts the paradigm that cancer treatments have to be harmful to be effective, and redirects modern medicine back to its salient principa: Primum non nocere, "first, do no harm".

Carla Woods founded Fight To Live in 2011. Having spent her entire career in the biomedical industry, Carla became increasingly dismayed at the slow pace of FDA assessment and approval for promising drugs and medical devices. As she researched the issue further, her dismay turned into outrage that Americans were being denied life-enhancing and potentially life-saving treatments—solely for bureaucratic and technocratic reasons. Woods concluded that the system no longer worked and needed to be modernized to spur future progress and eliminate unnecessary premature deaths.

Stage IV Survivor

We are survivors and caregivers battling an aggressive type of breast cancer known as Her2. Her2 is a mutated gene, first identified in the 1980s. This mutated gene accounts for about two-thirds of the most aggressive breast cancers. A drug that targeted that gene, and shut it off, Herceptin, was developed in the 1990s. It was, and is, a revolutionary drug. It cures some women, and when combined with chemo, cures more. However, some women with Her2 seem to have yet unidentified mutations that Herceptin, with or without traditional chemo, cannot stop.

In 2010, the maker of Herceptin succeeded in attaching a chemo-like toxin to Herceptin called Herceptin T DM-1. Like a "heat-seeking missile" the toxin rides the Herceptin to only the mutated cells and blows them up. Herceptin T DM-1 is a breakthrough drug that has some of the best stats ever for a cancer drug. It is more effective with metastatic Her2 cancers, and has FEWER side-effects. Yet, on September 1, 2010, the FDA blocked its approval. No one knows why, and they are not required to give an explanation. TWO YEARS LATER and the FDA continues to deny access to this life-saving drug!
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<tr>
<th>Pre Clinical Testing</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
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<td>Years</td>
<td>1-2</td>
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<td>Test Population</td>
<td>20 to 100 Healthy Volunteers</td>
<td>100 – 300 Patient Volunteers</td>
<td>1,000 to 3,000 Patient Volunteers</td>
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<th>FDA</th>
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<th>Process</th>
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<th>% of all new drugs that pass</th>
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<td>FILE IND</td>
<td>Assess Safety and Dosage</td>
<td>70% of INDs</td>
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<td>Determine Safety and Dosage</td>
<td>30% of INDs</td>
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<td>Evaluate Effectiveness. Look for Side Effects.</td>
<td>27% of INDs</td>
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<td>Verify Effectiveness, Monitor Adverse Reactions from Long-Term Use</td>
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<td>FILE NDA</td>
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<td>20% of INDs</td>
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During this time, more than 500,000 Americans will die of cancer alone. That's 4,000,000 cancer victims who are prevented from even trying potentially life-saving experimental treatments.

What Medical Professionals Say

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."

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."

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."
Phases of Clinical Trials

Phase I:
Safety
(15–30 people)

Phase II:
Safety and Effectiveness
(Fewer than 100 people)

Phase III:
Effectiveness compared to standard of care; Safety
(More than 100 to a few thousand)
Informed Consent

- Eight basic elements of informed consent (21 CFR § 50.25)

1. Trial involves research; purpose of the research
2. A description of any reasonably foreseeable risks or discomforts
3. A description of any benefits to the subject which may reasonably be expected from the research
4. A disclosure of appropriate alternative procedures or treatment that may be available to the subject
5. A statement describing the extent to which confidentiality of records identifying the subject will be maintained.

6. An explanation as to whether any compensation and whether any medical treatments are available if injury occurs.

7. An explanation of whom to contact for answers to questions about the research and research subjects’ rights.

8. A statement that participation is voluntary.
Informed Consent

- Purpose
- Medicine to be studied
- Procedures and schedule

- Risks
- Potential benefits
- Alternatives to participation
- Confidentiality
The ‘Dallas Buyers Club’ Bill

In a bi-partisan effort to bring Oscar-winning attention to patients who require additional treatment, Dems and GOPs lobby to pass a possible life-saving legislation. In the much-acclaimed movie *Dallas Buyers Club*, a man dying of AIDS smuggles illegal drugs from Mexico, defies the Federal Drug Administration and its jackbooted agents, and succeeds in prolonging his life, and the lives of others. The Hollywood screenplay is based on the true story of an AIDS patient who created and carried out the audacious scheme in the 1980’s, when the virus was ravaging the gay community and people were desperate for access to life-saving drugs.

Thirty years later, medication to treat AIDS is legal and widely available, but there are many other drugs that people suffering from all kinds of terminal illnesses would like to gain access to but are being denied by an FDA bound to federal guidelines about health and safety. Enter the Goldwater Institute, a think tank devoted to the free market and libertarian principles of its namesake, the GOP’s 1964 presidential candidate, Barry Goldwater, and its “Right to Try” bill.

“I believe there is a fundamental right to save your own life. We shouldn’t be putting up government red tape,” says Christina Corieri, the healthcare policy expert who crafted the “Right to Try” bill that is popping up with bipartisan support in several state legislatures. Corieri hasn’t seen *Dallas Buyers Club*, but she did research on the AIDS crisis in the 1980’s and the ACT UP (AIDS Coalition to Unleash Power) demonstrations at the FDA and many other locations, and how that helped create the Compassionate Use policies that drug companies have today for FDA approved drugs.

Looking into the FDA process and how someone might access drugs if they can’t get into a clinical trial is what prompted Corieri to seek legislation that would give the terminally ill drugs still in development that they could not otherwise get. Only three out of 40 percent of cancer patients who pursue clinical trials gain admission, and an expanded access application for drugs
under investigation requires so much cumbersome paperwork from patient and doctor, that it’s prohibitive.

“Right to Try” is limited to drugs that have successfully passed Phase One, the first human trials for safety. Phases Two and Three test for side effects and efficacy, whether the drug actually works better than existing medicines, or is better than nothing, if nothing else is available.

“We haven’t seen a lot of pushback,” Corieri tells The Daily Beast. “It’s a visceral thing for people—male, female, young, old, Democrat, Republican—it’s your life, you have the right to fight for it.” The bill is up for a floor vote Tuesday in the legislature in Arizona, where the Goldwater Institute is based. If it passes both chambers, the Right to Try Act would go before Arizona voters on the November ballot. In Missouri, Colorado and Louisiana, the bill is moving through the legislative process, and lawmakers in states as disparate as Utah, Oklahoma, Massachusetts and California are expressing interest in what appears to be a fast-moving train that taps into a resurgent libertarian movement.

Republican legislator Jim Neely, a physician, is the lead sponsor of the legislation in Missouri. Neely’s daughter was diagnosed with stage 4 colon cancer when she was pregnant, which made her ineligible for a clinical trial, and he has seen first-hand her struggles to find treatment. Drug companies want to get the best data they can, explains a Missouri House staffer. “There’s no incentive to include dying patients, so they give it to the healthiest patients possible. Giving it to someone on their death bed or weeks from dying, there’s a greater chance of an adverse reaction,” he explains. “The FDA shuts down trials all the time because people have an adverse reaction or die.” Having drug companies provide drugs on an expanded compassionate use basis outside of clinical trials would take away the disincentive to provide treatment that currently exists.

At a public hearing last week in Missouri, Neely told his story along with two other fathers. One testified that his 23-year-old son was denied access to clinical trials because his cancer was discovered too late in its progression. The other told of his daughter, diagnosed with brain cancer during her senior year of college, being turned down for a clinical trial two weeks before she died at age 23. No one testified against the bill, but lobbyists representing the hospice community were out in force warning lawmakers that “Right to Try” legislation raises false hopes for patients when further treatment is likely futile.
In Colorado, Democrat Joann Ginal is partnering with Republican Janak Joshi to sponsor “Right to Try” in their state. Joshi is a retired physician who feels the bill is needed to circumvent an FDA process that takes up to 10 years to bring a drug to market. Ginal is an endocrinologist who spent 25-plus years working for Big Pharma, and is now working to tighten the bill’s language to insure patients are aware of the risks; that insurance companies are not going to pick up the tab; and that pharmaceutical companies will voluntarily provide the drugs pro bono or under compassionate use guidelines at cost.

Drug companies already provide a fair amount of drugs at no cost for compassionate use; how much more they will be willing to do is up to them. One positive sign that “Right to Try” advocates cite is the favorable comments made in his blog by the CEO of Neuralstem, a company developing drugs to treat brain disorders, about what the legislation could mean for patients suffering from ALS, for which there is currently no approved treatment.

That was the situation with AIDS three decades ago, and it’s why *Dallas Buyer’s Club* resonates with “Right to Try” proponents today. Colorado Rep. Ginal says she was “totally blown away” when she heard about the movie, which she has not yet seen. “This is exactly what we’re trying to do,” she enthused.