# SB 113

<TARGET><BILL>SB 113</BILL><SUBJECT>SB 113</SUBJECT><COMM>SHSS29</COMM></TARGET>

# SENATE COMMITTEE REPORT
## First Committee of Referral

DATE: 4/17/15

**Date of 5-Day Notice:** 1/28/16
(in accordance with Uniform Rule 23)

FURTHER: Judiciary

DATE TURNED IN TO OFFICE: MAR 3 1 2016

**Health and Social Services Committee** considered SENATE BILL NO. 113

SB 113-NEW DRUGS FOR THE TERMINALLY ILL

"An Act relating to prescribing, dispensing, and administering an investigational drug, biological product, or device by physicians for patients who are terminally ill; and providing immunity for persons manufacturing, distributing, or providing investigational drugs, biological products, or devices."

and recommends:

[ ] be **replaced** with **CS** SB113 (HSS) [ ] *Same Title* [X] *New Title*

[ ] adopt **previous CS** _____ (_____) [ ] *Same Title* [ ] *New Title*

[ ] attached amendment(s)

[ ] adopt _____ Letter of Intent

[ ] further referral to _____ Committee

| Dept Abbr. | |
|---|---|
| ADM | LWF |
| CED | LAW |
| COR | LEG |
| EED | MVA |
| DEC | DNR |
| DFG | DPS |
| GOV | REV |
| DHS | DOT |
| AJS | UA |

| NEW FISCAL NOTE(S) | | | | |
|---|---|---|---|---|
| Dept. | Fiscal | Indet. | Zero | FN # |
| CED | | | ✓ | 1 |
| | | | | |
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| PREVIOUS FISCAL NOTE(S) | | | | |
|---|---|---|---|---|
| Dept. | Fiscal | Indet. | Zero | FN # |
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[ ] **APPROPRIATION - no fiscal note**

| SIGNATURES AND RECOMMENDATIONS: | PRINTED LAST NAME | DO PASS | DO NOT PASS | NO REC | AMEND |
|---|---|---|---|---|---|
| Jeff Ellis | ELLIS | ✓ | | | |
| | | | | | |
| Bill Stoltze | STOLTZE | | | ✓ | |
| | | | | | |
| | | | | | |
| | | | | | |
| CHAIR: ~~STEDMAN~~ | STEDMAN | | | ✓ | |

# ALASKA STATE LEGISLATURE

**Session**
State Capitol, Rm. 419
Juneau, AK 99801
(907) 465-2435
Fax: (907) 465-6615

**Interim**
716 W. 4th Ave, Ste. 409
Anchorage, AK 99501
(907) 269-0120
Fax: (907) 269-0122

Senator.Bill.Wielechowski@akleg.gov

Resources Committee

State Affairs Committee

Joint Armed Services Committee

Judiciary Committee

## SENATOR BILL WIELECHOWSKI

**MEMORANDUM**

**April 18, 2015**

**TO:** **Senator Bert Stedman, Chair**
**Senate Health & Social Services Committee**

**FROM:** **Senator Bill Wielechowski**

**SUBJ:** **Hearing Request for SB 113 – New Drugs for the Terminally Ill**

I am writing to respectfully request a hearing for Senate Bill 113: New Drugs for the Terminally Ill. 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 and after exhausting other available options, the freedom to access safe, but experimental drugs in an effort to save their own lives.

While the United States Food and Drug Administration currently offers an "expanded access" or "compassionate use" exemption, this process can take longer than patients facing terminal illness have to wait. Over the last 15 months, at least 40 states have seen "right to try" legislation introduced, 25 of which still have bills pending and 13 of which have signed bills into law with strong, largely unanimous bi-partisan support. This is a human issue that goes beyond state and party lines.

Included in this bill packet:

- SB 113 Sponsor Statement
- SB 113 Sectional Analysis
- SB 113 ver A
- SB 113 Supporting Documents
    - NCSL State Laws & Legislation Report 3-31-15
    - Article USA Today 2-3-15
    - FDA Drug Review Process
    - Code of Federal Regulations Title 21
    - Goldwater Institute Policy Report 2-11-14

If you have any questions please feel free to contact me or my staff person, Brooke Ivy, at 465-2435.

Thank you for your consideration of this request.

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Judiciary Committee

## SENATOR BILL WIELECHOWSKI

# SPONSOR STATEMENT

## Senate Bill 113

*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

Senate Bill 113 would create a legal climate in which terminally ill patients who have exhausted other available treatments could 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.

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, but not fully approved. 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 can wait.

Over the last 15 months, at least 40 states have seen "right to try" legislation introduced, 25 of which still have bills pending and 13 of which have signed bills into law with strong, largely unanimous bi-partisan support. It is clear this is a human issue and one that goes beyond state and party lines.

In providing terminal patients the ability to access safe, but 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 Senate Bill 113.

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## SENATOR BILL WIELECHOWSKI

## SECTIONAL ANALYSIS
## Senate Bill 113 ver H
*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.

**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.

29-LS0783\H
Bruce
1/28/16

**CS FOR SENATE BILL NO. 113( )**

IN THE LEGISLATURE OF THE STATE OF ALASKA

TWENTY-NINTH LEGISLATURE - SECOND SESSION

**BY**

**Offered:**
**Referred:**

**Sponsor(s): SENATOR WIELECHOWSKI**

**A BILL**

**FOR AN ACT ENTITLED**

**"An Act relating to prescribing, dispensing, and administering an investigational drug, biological product, or device by physicians for patients who are terminally ill; providing immunity for persons manufacturing, distributing, or providing investigational drugs, biological products, or devices; and relating to licensed health care facility requirements."**

**BE IT ENACTED BY THE LEGISLATURE OF THE STATE OF ALASKA:**

*** Section 1.** AS 08.64.367 is amended by adding new subsections to read:

(c) A physician may not be subject to disciplinary action by the board for prescribing, dispensing, or administering an investigational drug, biological product, or device, or providing related treatment, to a patient if the patient

(1) has a terminal illness;

(2) is ineligible or unable to participate in a current clinical trial for the investigational drug, biological product, or device;

(3) has considered, after consultation with the physician, all other treatment options currently approved by the United States Food and Drug Administration; and

(4) has given informed consent in writing for the use of the investigational drug, biological product, or device.

(d) In this section,

(1) "investigational drug, biological product, or device" means a drug, biological product, or device that has successfully completed Phase 1 studies of clinical trials for investigation and remains in ongoing clinical trials under Phase 2 or Phase 3, but has not been approved for general use by the United States Food and Drug Administration;

(2) "terminal illness" means a disease that, without life-sustaining procedures, will result in death in the near future or a state of permanent unconsciousness from which recovery is unlikely.

*** Sec. 2.** AS 09.65 is amended by adding a new section to read:

**Sec. 09.65.325. Immunity relating to use of investigational drugs, biological products, and devices.** (a) A person is not liable in an action for damages for the injury or death of a patient with a terminal illness resulting from the patient's use of an investigational drug, biological product, or device if the person, acting in good faith and with reasonable care, is a

(1) physician or member of the medical team who prescribed, dispensed, or administered the investigational drug, biological product, or device, or provided related treatment, to the patient and, before prescribing, dispensing, or administering the drug, product, or device, or providing related treatment, the physician or member of the medical team

(A) obtained the informed consent of the patient in writing after presenting to the patient all treatment options currently approved by the United States Food and Drug Administration for treatment of the patient's terminal illness; and

(B) provided written notice of the immunity provided under this section to the patient; or

(2) manufacturer, importer, or distributor of the investigational drug, biological product, or device and, before providing the drug, product, or device to the patient's physician, presented to the physician all treatment options currently approved by the United States Food and Drug Administration for treatment of the patient's terminal illness and provided written notice of the immunity provided under this section to the patient.

(b) In this section, "investigational drug, biological product, or device" and "terminal illness" have the meanings given in AS 08.64.367.

* **Sec. 3.** AS 17.20.110 is amended by adding a new subsection to read:

(b) This section does not apply to a physician who prescribes or administers a new drug in accordance with the conditions set out in AS 08.64.367(c).

* **Sec. 4.** AS 47.32.030 is amended by adding a new subsection to read:

(d) The department may not require a licensed entity 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. In this subsection, "investigational drug, biological product, or device" has the meaning given in AS 08.64.367.

# Fiscal Note

State of Alaska
2016 Legislative Session

Bill Version: SB 113
Fiscal Note Number:
() Publish Date:

Identifier: SB113-DCCED-CBPL-02-19-16
Title: NEW DRUGS FOR THE TERMINALLY ILL
Sponsor: WIELECHOWSKI
Requester: (S) 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)

| | FY2017 Appropriation Requested | Included in Governor's FY2017 Request | Out-Year Cost Estimates | | | | |
|---|---|---|---|---|---|---|---|
| **OPERATING EXPENDITURES** | **FY 2017** | **FY 2017** | **FY 2018** | **FY 2019** | **FY 2020** | **FY 2021** | **FY 2022** |
| 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** | | | | | | | |

**Estimated SUPPLEMENTAL (FY2016) cost:** 0.0 *(separate supplemental appropriation required)*
*(discuss reasons and fund source(s) in analysis section)*

**Estimated CAPITAL (FY2017) 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?

**Why this fiscal note differs from previous version:**

Not applicable, initial version.


Prepared By: Janey Hovenden, Director — Phone: (907)465-2536
Division: Corporations, Business and Professional Licensing — Date: 02/19/2016 10:35 AM
Approved By: Catherine Reardon, Director — Date: 02/19/16
Agency: Division of Administrative Services, DCCED

**FISCAL NOTE ANALYSIS**

**STATE OF ALASKA** **BILL NO.** SB 113
**2016 LEGISLATIVE SESSION**

## Analysis

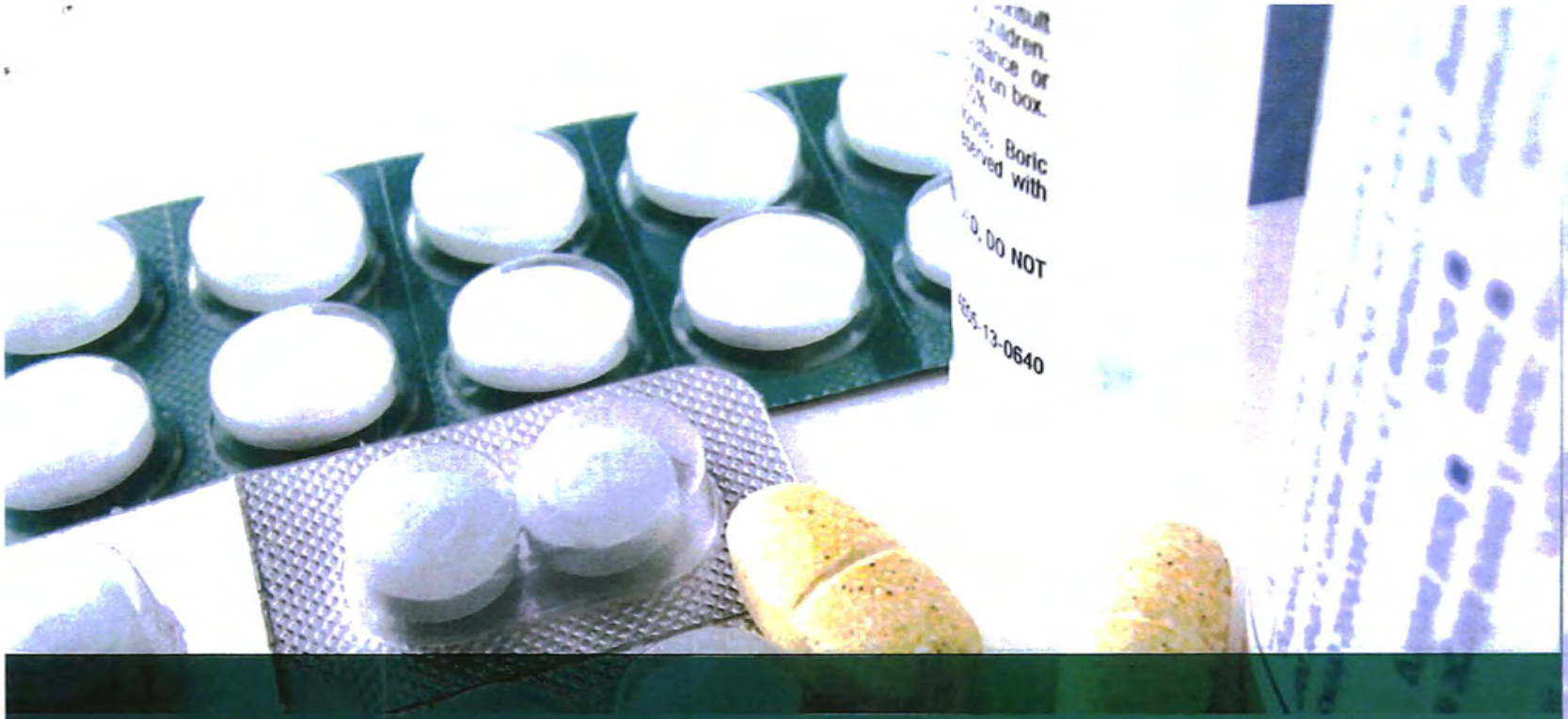
SB113 would provide certain immunities to prescribing physicians, manufacturers, and distributors acting in good faith to allow terminally ill patients who have exhausted other available treatments faster access to experimental drugs, without waiting for federal approval.

This legislation prohibits disciplinary action against physicians by the State Medical Board for prescribing, dispensing or administering an investigational drug, biological product, or device to terminally ill patients who have considered all other treatment options approved by the FDA. In addition, it prevents hospitals and health facilities from restricting the use of investigational drugs as provided.

This legislation establishes immunity for physicians, 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.

This legislation amends the statute limiting the sale and distribution of new drugs so as not to apply to physicians prescribing or administering investigational drugs under the conditions established in Section 1.

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



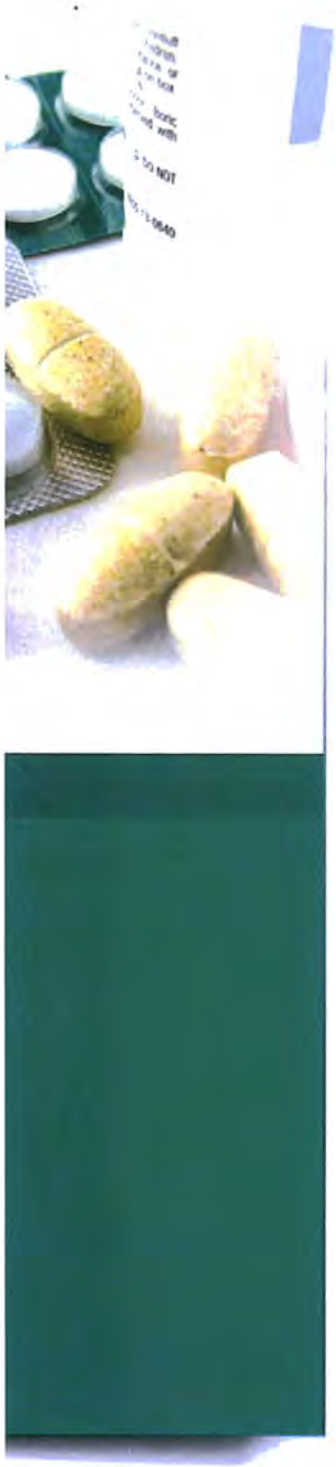

# Senate Bill 113: The Right to Try

Senator Bill Wielechowski

# 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*



# Senate Bill 113 ver H

- **Sec. 1:** Prohibits disciplinary action by the State Medical Board, under specific patient terms. Provides key definitions.
- **Sec. 2:** Physicians, medical team members, manufacturers, importers and distributors acting in good faith not held liable, with proper informed consent and notification.
- **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

Animals Tested

## Preclinical Animal Testing & Investigational New Drug (IND) Application

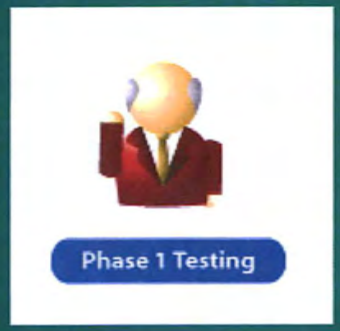
IND Application

- 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
- Typically conducted on healthy volunteers
- 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 SB 113 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

# Why SB 113 Matters

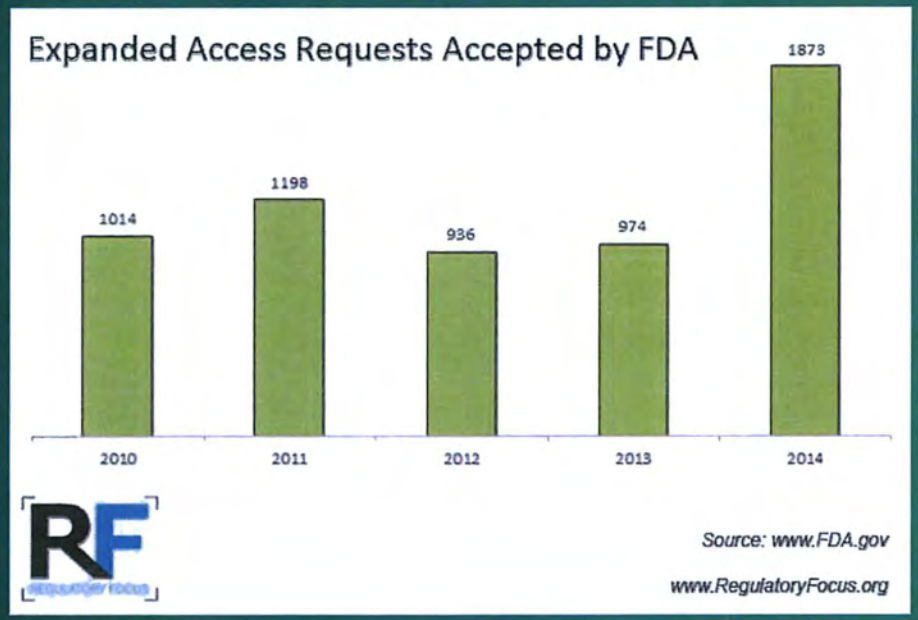
**The Stats:**

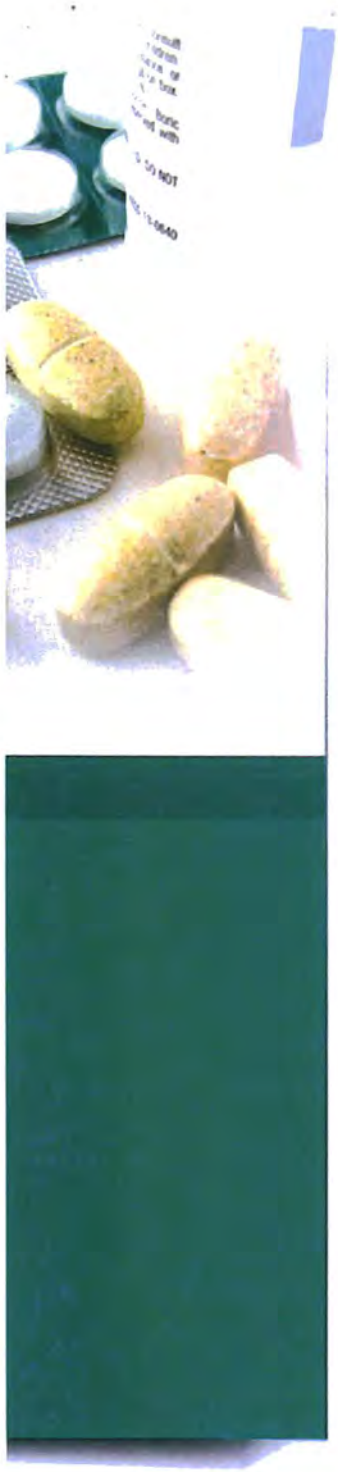
- Terminal illness takes one million Americans annually
- Fewer than 3% of those who apply for clinical trials are accepted
- The Right to Try? Hope for the other 97%

# FDA's Expanded Access Program

## "Compassionate Use"

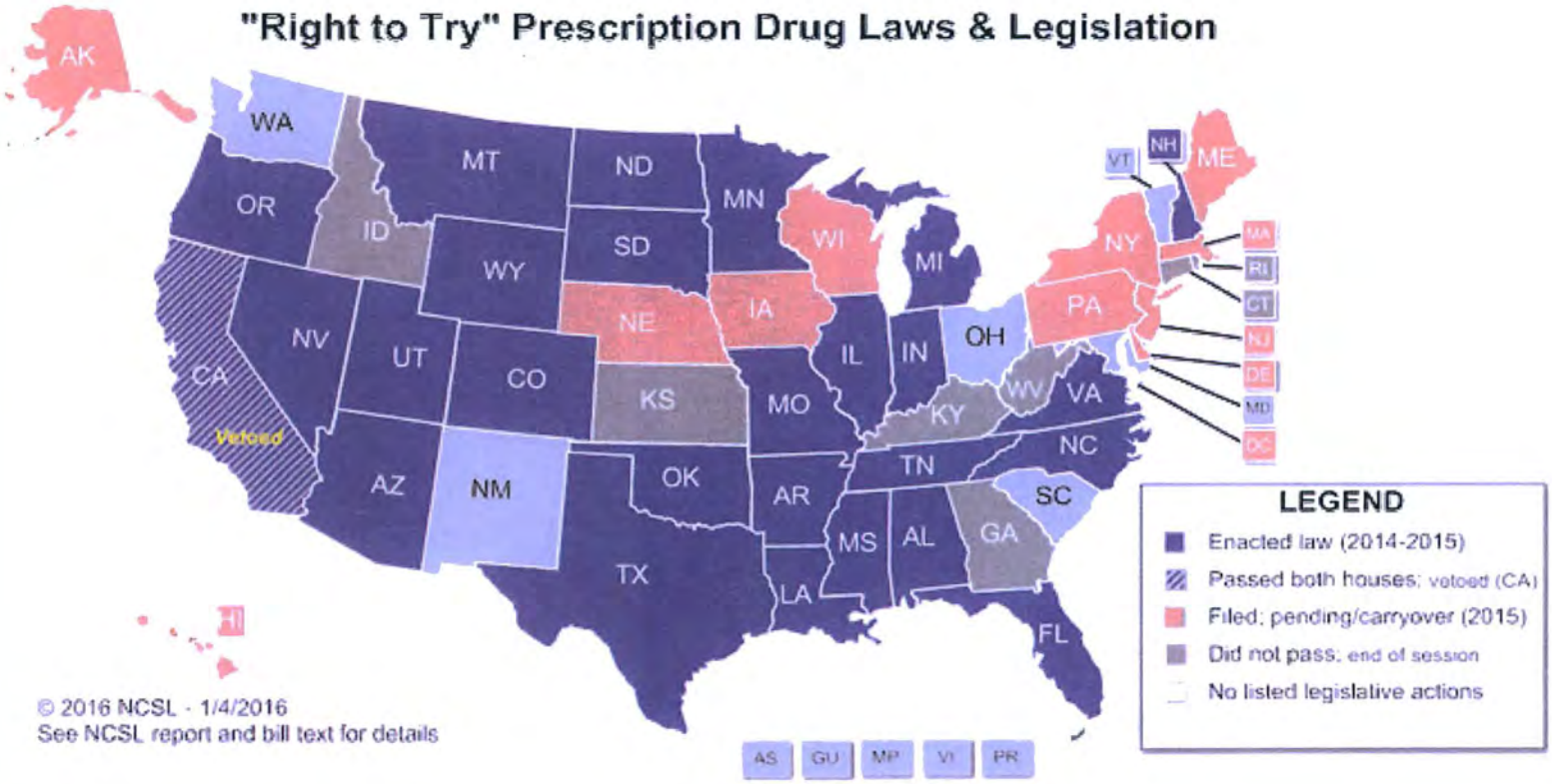
- Approx. 1,000 applicants per year make it through
- Application form can take 100 hours
- Even if streamlined, approval process remains extensive for those without time to spare





# The Right to Try - A Nationwide Effort

- 46 states have passed or introduced "Right to Try" legislation
  - 24 states have enacted law with overwhelming bi-partisan support
  - 22 states currently have pending legislation



This concludes our presentation for Senate Bill 113.

*Thank you.*

NATIONAL CONFERENCE *of* STATE LEGISLATURES

*The Forum for America's Ideas*

# "Right to Try" Experimental Prescription Drugs
# State Laws and Legislation for 2014 & 2015

**March 31, 2015 – Subject to additions**

*Compiled by Richard Cauchi, NCSL Health Program, Denver*

In the past 15 months there has been significantly increased activity related to how terminally ill patients can gain legal access to experimental drugs not yet approved by the U.S. Food and Drug Administration (FDA). As of March 2015 there are at least **36 states and D.C.** that have examined or will examine this burgeoning issue, with filed bills and **12 signed laws**.

**Update of Note:**
7 states enacted new "Right to Try" laws in March 2015

The following description is excerpted from enacted measures, including language presented by supporters of such measures, and is included for general information only. As always, NCSL takes no position on these or other state pharmaceutical or legislative measures.

**The Federal FDA Role:** FDA currently has a process by which patients can access experimental drugs through "expanded access" or "compassionate use." The Agency prioritizes these requests and frequently responds within 2-4 days, but no longer than 30 days. The process requires a patient's physician to file a form with FDA. The Agency recently announced a new proposed form which will take an estimated 45 minutes to complete, compared with the old form which carried an estimate of 100 hours. Under federal regulations, a patient cannot access an experimental drug unless FDA provides permission and the company making the drug agrees to provide it.
***UPDATE***: On Feb. 4, 2015, the FDA publicly announced a new "streamlined process" and provided a revised FDA webpage on expanded access An FDA blog post from Associate Commissioner Dr. Peter Lurie summarizes the new policy.

To allow access to experimental drugs under state law, several state legislatures have introduced "Right to Try" bills seeking to allow patients to obtain experimental drugs without first obtaining federal approval.

The "Right to Try" proposed model legislation was designed and promoted by the Goldwater Institute, a free-market advocacy group in Arizona. Their "model legislation" typically addressed several concerns about patients' use of experimental drugs.

- It would allow access only to medications that have passed manufacturers' Phase I clinical trials, or the first studies in humans. Most drugs complete three phases of testing in increasingly larger patient populations before being approved by FDA.
- Access would be limited to use by terminally ill patients who have exhausted other available treatments. Example: *"Terminal illness means an incurable and irreversible condition that without the administration of life-sustaining treatment will, in the opinion of the patient's physician, result in death within a relatively short time." [Arkansas, Act 374 of 2015]*
- A medication would be made available only if the company manufacturing it chose to do so.

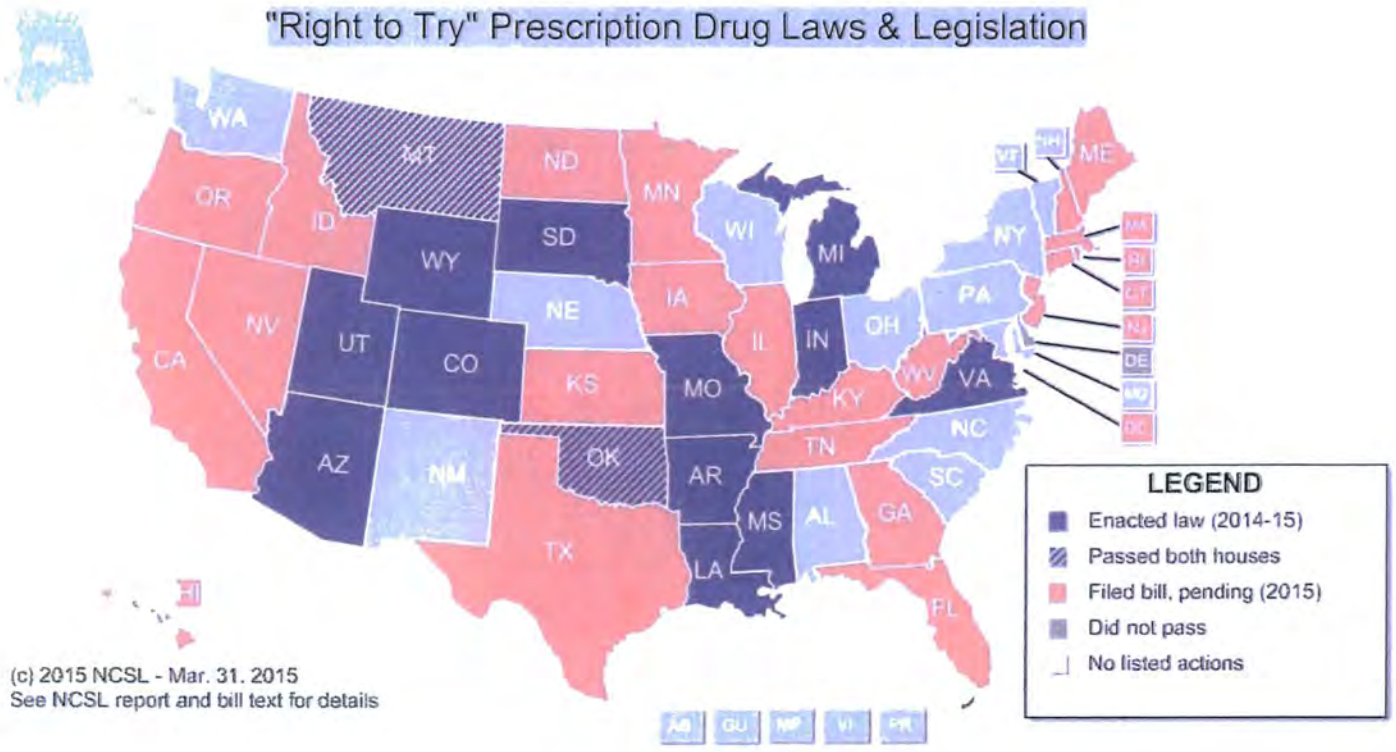
- A patient's request for access to an experimental drug would require a doctor to diagnose a terminal disease and declare that the drug represents the patient's best chance at survival.
- Patients would provide signed informed consent, thus limiting the legal exposure of the manufacturer of the drug.

**Critics** of "Right to Try" legislation note that providing experimental drugs to terminally ill patients may create a false sense of hope. There also is concern that such bills attempt to undermine FDA's authority and medical expertise in the regulation of pharmaceutical products. They also say that patients may be exposed to the dangers of drugs with limited testing and that the best way to get drugs to patients is through widespread clinical testing—a process the "Right to Try" legislation may undermine. Other critics claim that these bills won't have an effect because they don't require the companies to provide the investigational medication to patients.

**Supporters** say "any hope is better than the alternative of no hope, which is inevitable when no treatments are made available for terminal patients. 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."[2]

**2014 BILL STATUS SUMMARY:** Nine states considered "Right to Try" measures. As of Dec. 31, 2014, **five states—Arizona, Colorado, Louisiana, Michigan** and **Missouri** — had enacted laws. **Arizona** adopted a resolution to place the issue on the November 2014 ballot; it was approved and became law by a majority of voters.

**2015 BILL STATUS SUMMARY:** As of March 30, "Right to Try" bills have been filed in at least **32 states and D.C.**; additional measures may be filed in coming weeks. Table 2 below includes preliminary details. Specific provisions may vary. As of March 30, additional laws have been enacted in **Arkansas, Indiana, Mississippi, South Dakota, Utah, Virginia and Wyoming.**



| STATE LEGISLATION, 2013-2014 | | | Table 1 |
|---|---|---|---|
| **State** | **Bill/Act#** | **Status** | **Summary Description** |
| **Arizona** | HCR2005 of 2013 | **Order adopted by Legislature** 4/15/2014 **Became law as Proposition 303 11/4/2014** Yes votes: 78.3% | A concurrent resolution enacting and ordering the submission to the people as a binding November 2014 ballot question for a state statute creating a rapid approval path for terminally ill patients to the use of investigational drugs. Allows a manufacturer to make an investigational drug, biological product or device to an eligible terminally ill patient. It exempts a patient from regulatory action based solely on the physician's recommendation of the drug, product or device to the eligible terminally ill patient and classifies, as a class 1 misdemeanor, any attempt by a state official, employee, or agent to block access of the investigational drug, biological product or device to an eligible terminally ill patient. |
| **Colorado** | HB 1281 of 2014; Ch. 220 | **Enrolled 5/9/2014 Signed into law** 5/23/2014 | Provides a state process to allow terminally ill patients to have access to investigational products that have not been approved by the federal FDA that other patients have access to when they participate in clinical trials. |
| **Delaware** | S 270 of 2014 | Filed 7/1/2014 Held in Committee | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Louisiana** | HB 891 of 2014 | **Enrolled; Signed into law** as Act 346; 5/30/2014 | Enacts the *Right To Try Act*; authorizes access to investigational drugs, biological products, and devices for terminally ill patients; provides that a physician who prescribes such drug, biological product or device shall be immune from civil liability under medical malpractice provisions; provides that health insurers may choose to provide coverage for the cost of an investigational drug or device; requires a physician prescription. |
| **Michigan** | H 5649 of 2014 Act 346 | **Passed House & Senate; signed** 10/17/2014 | Defines the rights of terminally ill patients to access experimental medicines not yet approved by the Food and Drug Administration. |
| **Minnesota** | S 2985 of 2014 | Filed, 5/13/2014 Failed-Adjourned | Would permit the use of investigational drugs, biological products, or devices by certain terminally ill eligible patients. |
| **Missouri** | HB 1685 of 2014 | **Passed House & Senate; Signed into law** by governor 7/14/2014 | Authorizes a manufacturer of an investigational drug, biological product, or devices to make the manufacturer's drug, product, or device available to eligible patients; authorizes a health care insurer to provide coverage for an investigational drug, biological product, or device; prohibits taking action against a physician's license based solely on a recommendation to an eligible patient regarding treatment with an investigational drug, biological product, or device. |
| **New Jersey** | AB 3474 SB 2186 | Filed; sent to Assembly Health and Senior Services Comm. 6/26/2014 ; *carried over-- see 2015 bill list, below* | Would permit patients who are terminally ill to access investigational drugs, biological products, and devices that have not yet been approved by the United States Food and Drug Administration (FDA). To use an investigational drug, biological product, or device, the patient would be required to: have a medical condition that results in a life expectancy of less than 12 months; have consulted with a physician and considered all other treatment options currently approved by the FDA; have received a prescription or recommendation from a physician for the investigational drug, biological product, or device; and give informed, written consent to use of the investigational drug, biological product, or device. The physician would be required to document that the patient has met these requirements. |

| | | | |
|---|---|---|---|
| **Utah** | SJR 20 of 2014 | **Passed/enrolled 3/21/2014** | This non-binding joint resolution of the Legislature gives the Legislative Management Committee items of study it may assign to the appropriate interim committee. Includes as item #89: Access to Investigational Medications, Products, and Devices - to study providing terminally ill patients with access to investigational drugs, biological products, and devices that have completed at least phase I clinical trials. |

*Legislative tracking research by NCSL*

| **STATE LEGISLATION, 2015** | | | Table 2 |
|---|---|---|---|
| **State** | **Bill/Act#** | **Status** | **Summary Description** *(See bill text for specific provisions)* |
| **Arkansas** | SB 4 | Filed 1/12/2015 **passed Senate/House; signed as Act 374, 3/10/2015** | Permits certain terminally ill patients the right to try experimental treatments and drugs. |
| **California** | A 159<br>S 149<br>S715 | Filed 1/21/2015<br>Filed 1/29/2015<br>Filed 2/27/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Connecticut** | H 6292<br>H 6700<br>H 6709<br>S 60 | Filed 2/2/2015<br>"<br>"<br>Filed 1/13/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Delaware** | S 270 of 2014 | Filed 7/1/2014 Held in Comm. | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **District of Columbia** | B 125 | Filed 3/3/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Florida** | H 269<br>S1052 | Filed 1/14/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Georgia** | H34 | Filed 1/12/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Hawaii** | S 92<br>S 585<br>H 1013 | Filed 1/21/2015<br>Filed 1/21/2015<br>Filed 1/21/2015 | Would provide access for terminally ill patients to receive investigational drugs, biological products, and devices that have not received final FDA approval |
| **Idaho** | S 1156 | Filed 3/17/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Illinois** | H 207<br>H 496<br>H 1335<br>H 2508<br>S 29 | Filed 1/15/2015<br>Filed 1/30/2015<br>Filed 2/15/2015<br>Filed 2/27/2015<br>Filed 1/15/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Indiana** | H 1450<br><br><br>S 66 | Filed 1/21/2015; passed House & Senate; **signed by governor, as P.L. 2,** 3/24/2015 | Permits terminally ill patients the right to try experimental treatments and drugs. |
| **Iowa** | SSB 1264 | FilFileedd 13//61/02015/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Kansas** | H 2004 | Filed 1/12/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Kentucky** | SB 139 | Filed 2/10/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Massachusetts** | HD 2735 | Filed 1/16/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |

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|---|---|---|---|
| **Maine** | LR 476<br>LR 632 | Filed 1/5/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Minnesota** | H 236<br>S 100 | Filed 1/20/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Mississippi** | S 2485<br><br><br><br>H 722<br>H 1042 | Filed 1/21/2015; Passed Senate/House; **signed by governor, 3/30/2015**<br><br>Filed 1/22/2015; | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Missouri** | H 1072 | Filed 3/10/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. Also see **enacted law** **HB 1685** of 2014 |
| **Montana** | S 142 | Filed 1/13/2015; Passed Senate/House; sent to governor. | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **New Hampshire** | H 446 | Filed 1/8/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **New Jersey** | AB 3474<br>SB 2186 | Filed 6/16/2014; pending in 2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Nevada** | A 358 | Prefiled 7/16/2014; Filed 3/17/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **North Dakota** | S 2259 | Filed 1/19/2015; Passed Senate | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Oklahoma** | H 1074 S 616 | Filed 2/2/2015 passed House 96-0; passed Senate, 3/27/2015<br>Filed 2/2/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Oregon** | H 2300 S 543 | Filed 2/2/2015<br>Filed 2/2/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Rhode Island** | H 5093 | Filed 1/15/2015; held/study 2/11/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **South Dakota** | H 1080 | Filed 1/23/2015; Passed House/Senate; **signed by governor 3/12/2015** | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Tennessee** | H 143 S 811 | Filed 1/22/2015<br>Filed 2/11/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Texas** | H.438 | Filed 1/13/2015 | Would permit certain terminally ill patients to access investigational drugs, biological products, and devices in clinical trials |
| **Utah** | H 94 | Filed 1/23/2015; Passed House/Senate; **signed by governor 3/24/2015** | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Virginia** | S 732<br>H 1750 | Filed 1/14/2015; Passed Senate/House; **signed by governor as Ch. 655 and Ch. 656, 3/27/2015**<br>Filed; failed to pass | Permits terminally ill patients the right to try experimental treatments and drugs. |

| | | | |
|---|---|---|---|
| | H 2050 | | |
| **West Virginia** | H 2026 | Filed 1/14/2015 | Would permit terminally ill patients the right to try experimental treatments and drugs. |
| **Wyoming** | S 3 | Filed 1/13/2015; Passed Senate 29-1; House 58-1; **signed into law 3/12/2015** | Permits terminally ill patients the right to try experimental or investigational treatments and drugs. |

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## Additional Resources, Reports and Opinions

- States move to give terminally ill 'right-to-try' drugs - USA Today, Feb. 20, 2015
- Quicker Access to Experimental Drugs – Editorial Board, New York Times, Feb. 12, 2015
- Bill allows experimental meds for terminal patients (Delaware) – Feb. 12, 2015
- Legislation would allow 'right-to-try' trial drugs (Indiana) – USA Today, Feb. 3, 2015
- [2] Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment – Goldwater Institute – Feb. 11, 2015 -
- Patients Seek "Right to Try" New Drugs – New York Times, Jan. 14, 2015
- Right-to-try for the terminally ill – Pew/Stateline – June, 2014

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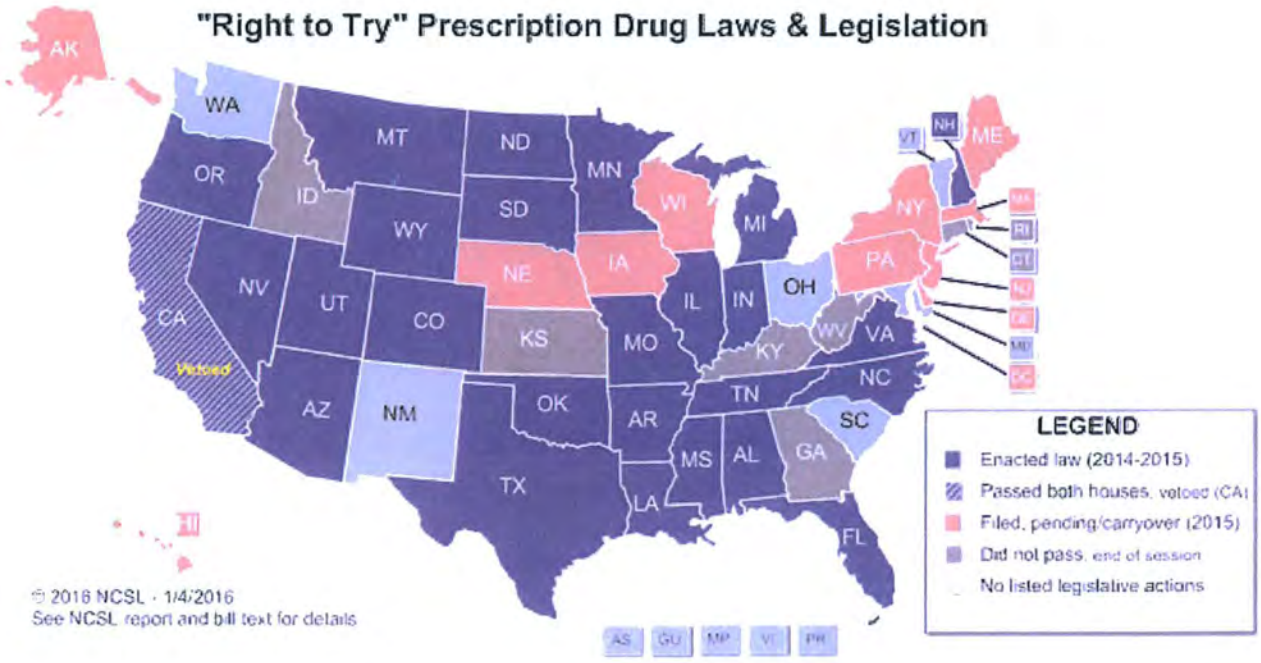


Source: http://www.ncsl.org/research/health/state-laws-and-legislation-related-to-biologic-medications-and-substitution-of-biosimilars.aspx#Right_to_Try

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## 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 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 1,000 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 an application that the FDA estimates takes 100 hours. 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. The Institutional Review Board can sometime take up to a month to reach a decision.

Sadly, there are many documented cases of patients dying while their application is being considered. Almost a year ago the FDA announced plans to shorten the application, but the other steps will still remain in place. The shorter form is still not available. 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 24 states: Alabama, Arizona, Arkansas, Colorado, Florida, Illinois, Indiana, Louisiana, Michigan, Minnesota, Mississippi, Missouri, Montana, Nevada, North Carolina, North Dakota, Oklahoma, Oregon, South Dakota, Tennessee, Texas, Utah, Virginia, and Wyoming. And it has passed with overwhelming bipartisan support in each state. It has been introduced in 12 additional states this year. 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 give their citizens the Right To Try.

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 goldwaterinstitute.org. Or contact Kurt Altman, kaltman@goldwaterinstitute.org, (602) 462-5000.

righttotry

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# 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.

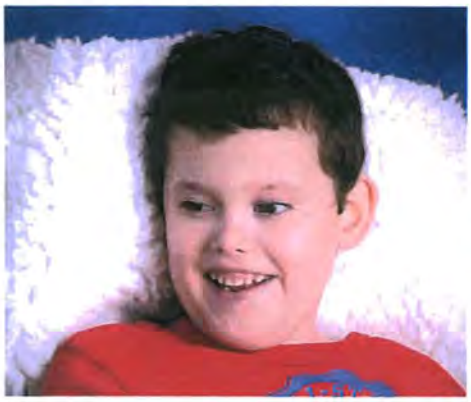
## Mikaela Knapp

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 worldwide 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.

# 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*


Animals Tested

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.


IND Application

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

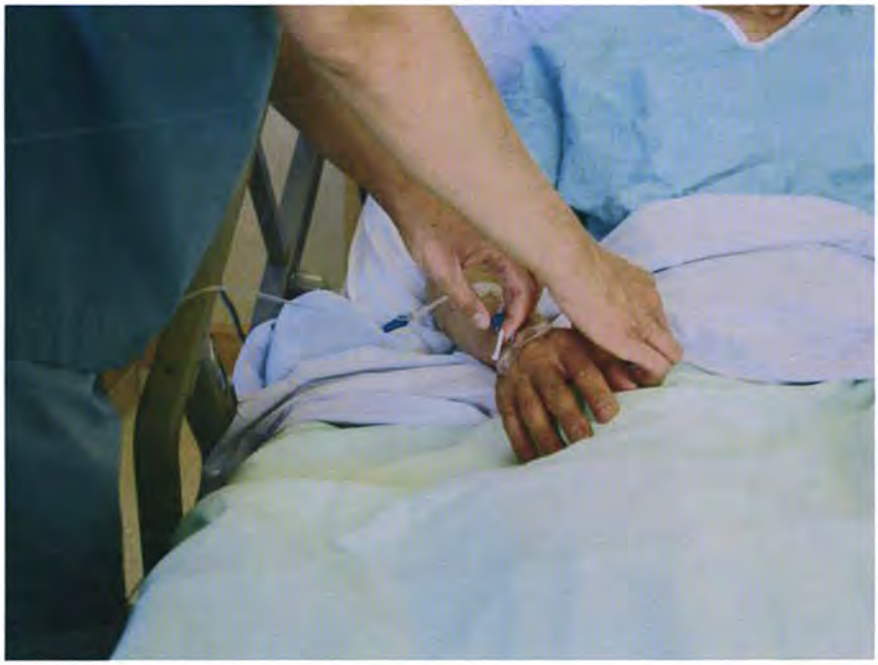
on at least 90 percent of NDAs for standard drugs no later than 10 months after the applications are received. The review goal is six months for priority drugs. (See "The Role of User Fees.")

"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.

USA TODAY

# Legislation would allow 'right-to-try' trial drugs

**Shari Rudavsky, The Indianapolis Star** *6:51 p.m. EST February 3, 2015*



*(Photo: Getty Images/iStockphoto)*

INDIANAPOLIS — In some cases, it's a matter of life and death.

A person with a terminal illness may hear of a drug that could be promising only to hear it has not yet received government approval for use. Unless that person can find his or her way into a clinical trial for that compound, he or she will most likely not benefit from it.

Now a number of states, including Indiana, are trying to change that dynamic with "right-to-try" legislation that would allow those with a terminal illness to use a drug that has gone through the first stage of approval, as long as they had the blessing of their doctor and the drug manufacturer.

Four states — Michigan, Colorado, Missouri, and Louisiana — have passed right-to-try bills. Arizona put a similar measure in place via a voter referendum.

This year, another 22 states, including Indiana, are considering similar legislation, which would allow the use of medicines that have cleared basic safety tests.

"This isn't to help drug companies. This is all patient-driven, because we have heard stories of people who are dying and there's drugs out there that could possibly save them and they're not allowed to take them and they're like, 'What have I got to lose?' " said Republican state Rep. Wes Culver, sponsor of a right-to-try bill in the Indiana General Assembly.

But some raise concerns about such efforts, saying that gaining access to early-stage drugs may not actually benefit those in the final stages of life.

Dr. Paul Helft, an oncologist and director of the Charles Warren Fairbanks Center for Medical Ethics at IU Health, said that right-to-try bills, if passed, could inadvertently wind up hurting some patients.

"I think these are mostly coming out of the illusory thinking that meaningful benefit is likely," said Helft, also a professor of medicine at Indiana University School of Medicine. "Two, these come out of the sense there's nothing to lose, you're going to die anyway. But there are things that you could lose: You might die faster or might spend time getting sick from the side effects of the drug."

Although most of those who might exercise their "right to try" have terminal cancers or diseases like Lou Gehrig's disease, Culver did not have those in mind and had never heard of the phrase when he first came up with the idea, he said.

Ebola, the lack of approved medicines for the disease, and fears of a worldwide outbreak inspired his bill, Culver said. Only after proposing it, did he realize he had tapped into a national movement.

The Goldwater Institute, a conservative think tank based in Phoenix, has taken up the cause, writing model right-to-try legislation for states, a version of which was proposed in the Indiana Senate. The institute sees the issue as protecting "the fundamental right of people to try to save their own lives."

> "The people that we hear from are talking about drugs that have shown very significant efficacy in clinical trials but they end up having to wait years for the approval, and meanwhile they die."
>
> **Frank Burroughs, co-founder of the Abigail Alliance for Better Access to Developmental Drugs**

Frank Burroughs wishes that option had been open to his daughter, Abigail, who died from head and neck cancer at age 21 in 2001. After her death, he co-founded the Abigail Alliance for Better Access to Developmental Drugs in her name. The Lawton, Va., organization advocates for earlier access to drugs for terminal patients.

During the last months of Abigail's life, her doctor told her about a drug in development that was performing well in trials at the time. It had targeted the same type of cancer Abigail had, but in the colon, not the neck, so she was not eligible to participate.

"What this is saying is that there are promising drugs that you can identify as drugs that need much earlier approval," Burroughs said. "The people that we hear from are talking about drugs that have shown very significant efficacy in clinical trials but they end up having to wait years for the approval, and meanwhile they die."

Other drugs that the Abigail Alliance has supported have been approved for use in Europe but are still awaiting a nod from the U.S. government.

While the Abigail Alliance supports state efforts for right-to-try legislation, it focuses much of its energy on the national level, trying to persuade Congress and the Food and Drug Administration to speed up approval of promising drugs.

Patients do have other avenues they can pursue to obtain investigational drugs. They can enroll in a clinical trial or ask their doctors to file an application with the FDA for a single investigational or compassionate use.

None of these is easy, however, supporters of right-to-try legislation argue.

Only about 3 percent of those who have the medical conditions that would make them eligible for clinical trials are accepted, said Kurt Altman, national policy adviser and general counsel for the Goldwater Institute.

Still, passing right-to-try legislation could also detract from participation in clinical trials, said IU's Helft. And clinical trials exist in a very different context than individual use, he added

"I have no problem with patients enrolling in early-phase trials to get drugs, because those trials are done in a systematic way so we actually get to learn something and they're done with informed consent," he said. "If patients have relatively unfettered access to drugs that could interfere with future trial participation ... it would be much easier to go out and get the drug rather than participating in a clinical trial."

But Burroughs says the idea is not to supplant clinical trials but to offer an alternative to those who, like his daughter, cannot get into a study and have no other way to access the drug.

Four and a half years after Abigail died, the drug she sought did receive approval for use in treating the type of cancer that she had.

*Source Link:* http://usat.ly/1CXqPIV

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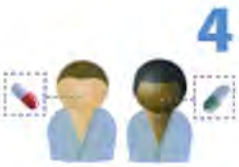


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[Code of Federal Regulations]
[Title 21, Volume 5]
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TITLE 21--FOOD AND DRUGS
CHAPTER I--FOOD AND DRUG ADMINISTRATION
DEPARTMENT OF HEALTH AND HUMAN SERVICES
SUBCHAPTER D--DRUGS FOR HUMAN USE

PART 312 -- INVESTIGATIONAL NEW DRUG APPLICATION

Subpart B--Investigational New Drug Application (IND)

Sec. 312.21 Phases of an investigation.

An IND may be submitted for one or more phases of an investigation. The clinical investigation of a previously untested drug is generally divided into three phases. Although in general the phases are conducted sequentially, they may overlap. These three phases of an investigation are a follows:

(a) *Phase 1.* (1) Phase 1 includes the initial introduction of an investigational new drug into humans. Phase 1 studies are typically closely monitored and may be conducted in patients or normal volunteer subjects. These studies are designed to determine the metabolism and pharmacologic actions of the drug in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness. During Phase 1, sufficient information about the drug's pharmacokinetics and pharmacological effects should be obtained to permit the design of well-controlled, scientifically valid, Phase 2 studies. The total number of subjects and patients included in Phase 1 studies varies with the drug, but is generally in the range of 20 to 80.

(2) Phase 1 studies also include studies of drug metabolism, structure-activity relationships, and mechanism of action in humans, as well as studies in which investigational drugs are used as research tools to explore biological phenomena or disease processes.

(b) *Phase 2.* Phase 2 includes the controlled clinical studies conducted to evaluate the effectiveness of the drug for a particular indication or indications in patients with the disease or condition under study and to determine the common short-term side effects and risks associated with the drug. Phase 2 studies are typically well controlled, closely monitored, and conducted in a relatively small number of patients, usually involving no more than several hundred subjects.

(c) *Phase 3.* Phase 3 studies are expanded controlled and uncontrolled trials. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to gather the additional information about effectiveness and safety that

is needed to evaluate the overall benefit-risk relationship of the drug and to provide an adequate basis for physician labeling. Phase 3 studies usually include from several hundred to several thousand subjects.

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POLICY report

Goldwater Institute

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# 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.

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## *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.

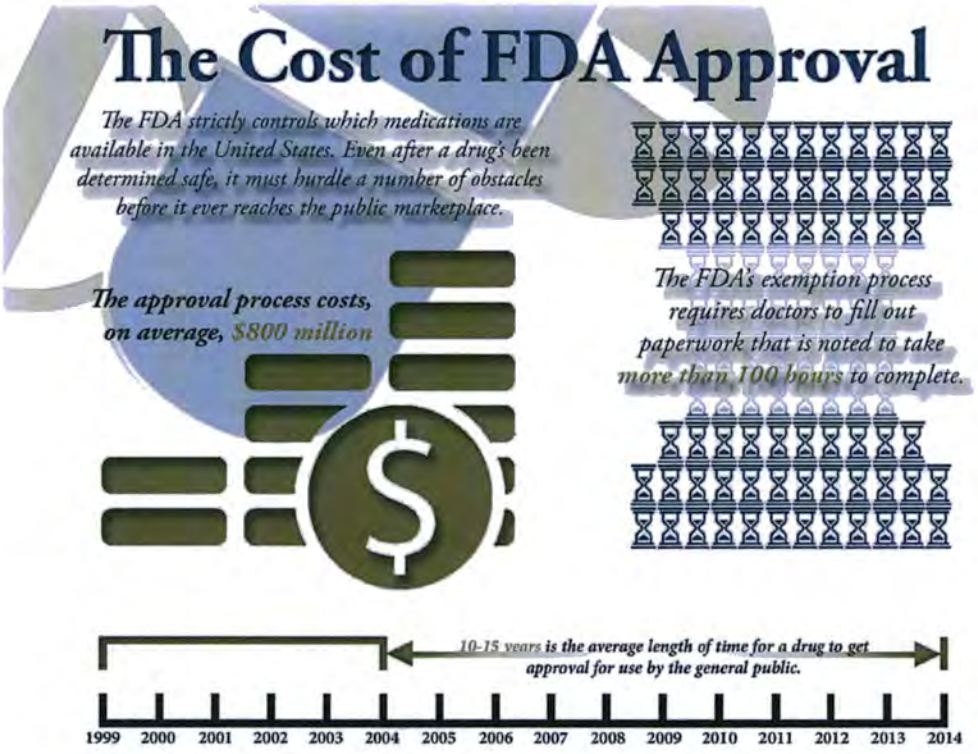
These bureaucratic impediments violate an individual's fundamental right to try to save his own life. Unfortunately, the federal government has shown little interest in reforming the FDA as bills to reform the process for terminal patients have been introduced, but have never received a vote in Congress. 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.


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]

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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.

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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]

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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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# 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.

*"I had Stage 4 prostate cancer."*

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

*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


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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

Valley Medical Care
1801 Salmon Creek Lane • Juneau, Alaska 99801
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Joseph Roth, M.D.
Dorothy Hernandez, M.D
Don Schneider, M.D.
Daniel Kim, M.D.
Taylor Dunn, M.D.
Justine Emerson, F.N.P.
Matthew Jones F.N.P.
Rebecca Young, F.N.P
Kim Gardner, F.N.P
Tina Pleasants, F.N.P.
Rachel Gladhart, C.N.M.

February 1, 2016

Senator Bert Stedman
Senate Health and Social Services Committee
State Capitol, Room 30
Juneau, Alaska 99801

Dear Senator Stedman:

This is a letter in support of Senate Bill 113, which would protect physicians and hospitals for treating terminally ill patients with experimental medications that have passed the first stage of approval for the FDA.

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

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

Thank you for your service.

Sincerely yours,

Bob Urata, M.D.
907-723-4144

BU/cmm

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

*Feb. 9, 2016*
*Re: Support for SB113*

Dear members of the Alaska legislature,

The Tenth Amendment Center fully supports passage of SB113 enacting a "Right to Try" law in the state of 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.

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

So far, 24 states have enacted "Right to Try," with 16 more including Alaska, considering the legislation this year.

We strongly urge you to vote yes on SB113.

Sincerely,

Michael Boldin, Tenth Amendment Center founder and executive director
Mike Maharrey, Tenth Amendment Center national communications director

SOUTHEAST
Radiation
Oncology
CENTER

**Eugene Huang, M.D.**
1701 Salmon Creek Lane
Juneau, AK 99801
Phone: 907.586.5762
Fax: 907.586.5777

To Whom It May Concern,

I am writing this letter in support for Senate Bill 113 (Version W) "New Drugs for the Terminally Ill." I believe that this bill will help provide terminally ill patients greater access to 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 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 Senate Bill 113 will help bring new scientific discoveries closer to patients whom need them, within the appropriate context and safeguards.

Sincerely,

Eugene Huang, MD
Medical Director
Southeast Radiation Oncology Center

Dixie A. Hood, LMFT
Juneau, Alaska

January 30, 2016

Senator Bert Stedman, Chair
Senate Health & Social Services Committee
State Capitol, Room 30
Juneau, AK 99801

Subject: Senate Bill 113 – The Right to Try

Dear Senator Stedman,

This letter is in support of Senate Bill 113, 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.

SB 113 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 Senate Bill 113.

CC: Senator Cathy Giessel
Senator Pete Kelly
Senator Bill Stoltze
Senator Johnny Ellis
Senator Bill Wielechowski