

Date: April 2, 2014	BILL SUMMARY PREPARED FOR THE BOARD OF DIRECTORS	 OF NORTHERN LARIMER COUNTY
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HB14-1281 TERMINALLY ILL PATIENT ACCESS TO INVESTIGATIONAL DRUGS

Concerning the allowance for terminally ill patients to have access to investigational products that have not been approved by the federal food and drug administration that other patients have access to when they participate in clinical trials.

Bill Details

Bill Title:	Terminally Ill Patient Access To Investigational Drugs - Concerning the allowance for terminally ill patients to have access to investigational products that have not been approved by the federal food and drug administration that other patients have access to when they participate in clinical trials.
Bill Sponsors:	House – Ginal (D) and Joshi (R) (With 11 others, 7 R and 4 D) Senate – Rivera (R) and Aguilar (D)
Committee:	Health, Insurance, & Environment
Bill History:	04/01/2014 House Third Reading Passed
Next Action:	Committee Assignment in Senate

Bill Summary

HB14-1281, commonly referred to by the title “Right to Try,” offers a mechanism for a person with a terminal illness to gain access to treatments that have not completed the necessary U.S. Food and Drug Administration (FDA) trials for efficacy and safety. If such a patient’s physician recommends the treatment and the patient provides written consent, the patient can request the treatment. The drug or device manufacturer may, but is not required to, make the treatment available to the patient, who may be solely liable for expenses, at the discretion of his or her insurance provider.

Insurance providers are not required to, but may, cover these experimental treatments and may also deny coverage for all non-pre-existing conditions from the date that the experimental treatment begins until six months after the experimental treatment ends. Finally, the bill forbids action against a health providers credentialing for use of experimental treatments, forbids any state employee from blocking access to experimental treatments, and removes liability for harm done from the physician, the drug or device manufacturer, or other persons involved in the experimental treatment.

Background

The idea for HB14-1281 comes from a 2014 report by the Goldwater Institute titled, “Everyone deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment.”¹ This paper argues that patients “should be free to exercise a basic freedom — attempting to preserve one’s own life.” The proposal would allow any patient diagnosed with a terminal illness to have access to any drug or device that has passed Phase I clinical trials. The FDA has a procedure for “expanded access” to investigational drugs, but proponents of Right to Try bills cite a lengthy process and the low number of participants. The Goldwater Institute notes that the 1,014 patients receiving expanded access to investigational drugs in 2010 “is a very small number considering that, in that same year, there were 1,529,650 new cancer cases.”²

There have been a number of recent articles in national news outlets about the challenges of gaining access to experimental drugs. In a recent article on CNN.com, FDA officials said that they’ve approved more than 99% of applications for compassionate use (expanded access); pharmaceutical companies, however, often deny access due to

¹ Available at: <http://goldwaterinstitute.org/article/everyone-deserves-right-try-empowering-terminally-ill-take-control-their-treatment>

² Page 11

limited supplies and the potential risks to patients.³ The companies manufacture very limited quantities of unapproved drugs, just enough for clinical trials, and often do not have stocks to provide the drugs to every applicant. A 2013 New York Times piece discussed patients trying a variety of different experimental drugs and how, each time, they had to work to convince pharmaceutical companies to offer the drugs.⁴

The FDA's requirements are rather stringent, requiring the physician work with his organization's review board and the submission of a variety of forms, which can be time consuming.⁵ The FDA also has an emergency access protocol, which a physician can use in certain situations.⁶ However, even in emergency situations, the manufacturer of the drug or device could opt to not provide the treatment.

Clinical trials for new drugs and devices are broken into several phases. Phase one is the first, where the treatment is tried on a small number of people, usually 20-80 according to the FDA.⁷ This phase looks for frequent side effects, but drug efficacy, less immediate side effects, and interactions with other drugs are not evaluated until phases two and three, which include hundreds or thousands of participants. Phase I of clinical testing sees the highest success rate for pharmaceuticals, with a 60% success rate between 1991 and 2000. Less than 40% of drugs during that time passed Phase II testing and of those, in Phase III, 55% finally succeeded.⁸ Newer rates of success and failure by clinical trial phase are not immediately available.

Questions

The concept of the bill sounds simple, but the many different pieces of this have led to a number of questions. The Health District has not had time to complete a full analysis of this legislation, but believe the following questions should be better understood by policy makers:

1. Federal Preemption

- a. What will be the reaction of the FDA with a state process that sidesteps the established drug approval and testing process?
- b. Will this bill lead to a costly lawsuit to determine its constitutionality?

2. Liability – The bill expressly does not create a cause of action against a drug manufacturer, a physician, or any other person involved in the experimental treatment.

- a. Is anyone liable for potential misuse of the process to get experimental drugs to patients?
- b. How will this impact physicians' professional liability (medical malpractice) insurance?
- c. Are there liability issues created where a patients wants access to an experimental drug but the patient's physician refuses to recommend it over approved treatments?
- d. Could a drug or device manufacturer be liable for not providing access to an experimental treatment? The bill does not require the manufacturer to do so, but liability protections in the bill only extend to harm caused done by the treatment. If a patient were to die after being denied an experimental drug, could there be a cause of action against the manufacturer?

3. Costs – When a patient opts to use an experimental drug or treatment, the patient's insurance may decide to not cover the treatment or any other care for the patient. If the treatment causes the patient to need extensive and potentially expensive care that exceeds the patient's ability to pay, who will cover those costs? Will the costs be paid by taxpayers as uncompensated care?

4. Public insurance

- a. Will Medicaid be required to cover all costs for experimental drugs?

³ <http://www.cnn.com/2014/04/05/health/cohen-compassionate-use/>

⁴ <http://mobile.nytimes.com/2013/11/03/magazine/how-dying-patients-get-access-to-experimental-drugs.html?from=magazine>

⁵ <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM351261.pdf>

⁶ <http://www.fda.gov/downloads/Training/CDRHLearn/UCM180888.pdf>

⁷ <http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm>

⁸ Kola, Ismail, and Landis, John. Can the Pharmaceutical Industry Reduce Attrition Rates?. Nature Reviews, Volume 3, August 2004.

- b. If a HCPF employee asks questions of a doctor about the appropriateness of an experimental treatment, would that employee run afoul of the bill section regarding blocking access to the drugs?

5. Clinical Trial Procedures

- a. Will drug and device manufacturers be able to recruit patients for necessary clinical trials if patients could utilize this process to gain access more easily and without risk of receiving placebo treatments?
- b. If a participant in a clinical trial believes they might be receiving a placebo treatment, could the patient use this process to definitely gain access to the experimental drug, thereby altering the outcome of the clinical trial?
- c. With patients' ability to bypass clinical trials for access to experimental treatments, will this slow new drug development?

6. Manufacturer participation

- a. Will drug or device manufacturers participate in a state program like this that could lessen the likelihood of their experimental treatment being approved eventually by the FDA?
- b. Currently, many times when the FDA approves compassionate use, manufacturers do not allow access because there is insufficient supply. Will this bill improve the chances that a patient would have access in that situation?

7. Advertising/Information

- a. Will manufacturers be able to market experimental treatments directly to doctors and patients, in hopes that the patient's doctor will prescribe the untested treatment?
- b. If a patient requests access to an experimental drug that the patient has learned about, will doctors have access to adequate information to help the patient make an informed decision?

- 8. **Hospitalization** – The bill requires that the informed consent “makes clear that ... inpatient services may be denied if treatment begins.” Does this mean that a hospital could turn the patient away in case of an emergency requiring hospitalization?

Notable definitions in current bill text (Reengrossed bill as passed by the House)

The following definitions are copied from the text of the reengrossed bill, as amended by the House.

"eligible patient" means a person who has:

- (i) a terminal illness, attested to by the patient's treating physician;
 - (ii) considered all other treatment options currently approved by the United States Food and Drug Administration;
 - (iii) received a recommendation from his or her physician for an investigational drug, biological product, or device;
 - (iv) given written, informed consent for the use of the investigational drug, biological product, or device or, if the patient is a minor or lacks the mental capacity to provide informed consent, a parent or legal guardian has given written, informed consent on the patient's behalf; and
 - (v) documentation from his or her physician that he or she meets the requirements of this paragraph (a).
- (b) "eligible patient" does not include a person being treated as an inpatient in a hospital licensed or certified pursuant to section 25-3-101.

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

"written, informed consent" means a written document signed by the patient and attested to by the patient's physician and a witness that, at a minimum:

- (a) explains the currently approved products and treatments for the disease or condition from which the patient suffers;
- (b) attests to the fact that the patient concurs with his or her physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life;

- (c) clearly identifies the specific proposed investigational drug, biological product, or device that the patient is seeking to use;
- (d) describes the potentially best and worst outcomes of using the investigational drug, biological product, or device with a realistic description of the most likely outcome, based on the physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition;
- (e) makes clear that the patient's health insurer and provider are not obligated to pay for any care or treatments consequent to the use of the investigational drug, biological product, or device;
- (f) makes clear that the patient's eligibility for hospice care may be withdrawn if the patient begins curative treatment and care may be reinstated if the curative treatment ends and the patient meets hospice eligibility requirements;
- (g) makes clear that in-home health care and inpatient services may be denied if treatment begins; and
- (h) states that the patient understands that he or she is liable for all expenses consequent to the use of the investigational drug, biological product, or device, and that this liability extends to the patient's successors and estate.

About this Summary

This summary was prepared by Health District of Northern Larimer County staff to assist the Health District Board of Directors in determining whether to take an official stand on various health-related issues. The Health District is a special district of the northern two-thirds of Larimer County, Colorado, supported by local property tax dollars and governed by a publicly elected five-member board. The Health District provides medical, mental health, dental, preventive and health planning services to the communities it serves. For more information about this summary or the Health District, please contact Dan Sapienza, Policy Coordinator, at (970) 224-5209, or e-mail at dsapienza@healthdistrict.org.