The Florida Senate

BILL ANALYSIS AND FISCAL IMPACT STATEMENT

(This document is based on the provisions contained in the legislation as of the latest date listed below.)

Prepared By: The Professional Staff of the Committee on Fiscal Policy

BILL:	CS/CS/SB 1052
INTRODUCER:	Fiscal Policy Committee (Recommended by Appropriations Subcommittee of Health and Human Services); Health Policy Committee; and Senator Brandes
SUBJECT:	Florida Right to Try Act
DATE:	April 22, 2015 REVISED:

	ANALYST	STAFF DIRECTOR	REFERENCE	ACTION
1.	Lloyd	Stovall	HP	**Fav/CS**
2.	Brown	Pigott	AHS	**Recommend: Fav/CS**
3.	Pace	Hrdlicka	FP	**Fav/CS**

Please see Section IX. for Additional Information:

COMMITTEE SUBSTITUTE - Substantial Changes

I. Summary:

CS/CS/SB 1052 creates the "Florida Right to Try Act," which provides a framework for an eligible patient with a terminal condition to access investigational drugs, biological products, and devices from the manufacturer.

The bill has no known fiscal impact.

II. Present Situation:

The U.S. Food and Drug Administration (FDA) has wide regulatory authority over what drugs are marketed and sold within the United States. Prescription drugs and over-the-counter drugs are regulated by the FDA's Center for Drug Evaluation and Research.[1] If a drug company wants approval to sell a new prescription drug in the United States, it must be tested in several steps. The first step is testing in the laboratory and on animals.[2] Next, the drug is tested in humans for safety and efficacy when used to treat or diagnose a disease.[3]

[1] U.S. Food and Drug Administration, *FDA Basics: Drugs* (last updated August 12, 2013), available at http://www.fda.gov/AboutFDA/Transparency/Basics/ucm192696.htm (last visited April 14, 2015).
[2] U.S. Food and Drug Administration, *What is the approval process for a new prescription drug?* (last updated April 11, 2014), available at http://www.fda.gov/AboutFDA/Transparency/Basics/ucm194949.htm (last visited April 14, 2015).
[3] Id.

Clinical research is medical research that involves humans. Clinical trials look at new ways to prevent, detect, or treat disease through new combinations of drugs, new surgical procedures or devices, or new ways to use existing treatments.[4] Clinical trials are part of clinical research, which is conducted as part of a protocol. A protocol describes:

- Who is eligible to participate in the trial;
- Details about tests, procedures, medications, and dosages; and
- The length of the study and what information will be gathered.[5]

All clinical trials are conducted in phases. Before a clinical trial (human testing) can begin, the company must submit an investigational new drug (IND) application. The IND shows the FDA results of animal testing, the company's proposal for human testing, and the clinical trials protocol.[6] At this time the FDA must decide if it is reasonably safe to begin testing the drug in humans.[7] The table below describes the clinical trial phases:

Clinical Trial Phases[8]			
Phase	**Activities**	**Approx. Time[9]**	**# of Participants**
One	Test drug with healthy human volunteers. Determine drug's most frequent side effects. Determine how the drug is metabolized and excreted. Determine the correct dosing. Move to Phase Two if drug does not show unacceptable toxicity.	1 year	20-80
Two	Test drug with small number of targeted patients. Test drug is compared with those receiving a placebo, or a different drug (if a controlled trial). Evaluate safety and short-term side effects. Decide scope of Phase Three with FDA; drug must have shown effectiveness to move to Phase Three.	2 years	100-300
Three	Implement large scale study for effectiveness and safety. Study different populations and different dosages and using the drug in combination with other drugs. Review logistics of creating a large supply.	3 years	300-3,000

[4] U.S. Department of Health and Human Services, National Institutes of Health, *NIH Clinical Research Trials and You* (last reviewed February 5, 2015), available at http://www.nih.gov/health/clinicaltrials/basics.htm (last visited April 14, 2015).
[5] Id.
[6] The clinical trials protocol is approved and monitored by an Institutional Review Board (IRB). The IRB is an independent committee of experts that help ensure the safety and rights of participants and periodically review the research. *See* supra note 4.
[7] U.S. Food and Drug Administration, *The FDA's Drug Review Process: Ensuring Drugs are Safe and Effective* (last updated November 6, 2014), available at http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm (last visited April 14, 2015).
[8] Id.
[9] FierceBiotech, *FDA Approval Process*, available at http://www.fiercebiotech.com/topics/fda_approval_process.asp (last visited April 14, 2015).

	Once complete, can complete New Drug Application (NDA).		
Four	Post-market requirement and commitment studies must be agreed to by the sponsoring organization and are conducted after a product has been approved for sale. Information used to gather additional data about product's safety, efficacy, or optimal use.	n/a	n/a

When testing is complete, the company sends an application to the FDA describing:
- The drug's test results;
- Manufacturing information to demonstrate that the company can properly manufacture the drug; and
- The company's proposed label for the drug, which must provide necessary information about the drug, including the conditions for which it has shown to be useful.[10]

The FDA has 60 days to decide whether to file the application to be reviewed.[11] If the FDA determines that the drug's benefits outweigh its risks and the drug can be manufactured in a manner that ensures a quality product, the drug can be approved for marketing in the United States.[12] The company receiving approval must continue, under Phase Four, to monitor short-term and long-term results of the drug and submit those findings to the FDA. If the company wants the drug approved for another purpose, it must also receive FDA approval.[13]

Accelerated approval is granted by the FDA to some new drugs for serious and life-threatening illnesses that lack other treatment options.[14] This option allows drugs to be approved without measures of effectiveness.

The FDA established regulations allowing expanded access to, or "compassionate use" of, experimental drugs, biological products, or devices in 1987, and individual patient "emergency use" expanded access in 1997. These regulations provide access to:
- Individuals on a case-by-case basis, known as "individual patient access";[15]
- Intermediate-sized groups of patients with similar treatment needs who otherwise do not qualify to participate in a clinical trial;[16] and
- Large groups of patients who do not have other treatment options available.[17]

[10] Supra note 2.
[11] Supra note 7.
[12] Id.
[13] Supra note 9.
[14] U.S. Food and Drug Administration, *FDA's Drug Review Process: Continued, Accelerated Approval* (updated November 6, 2014), available at http://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm289601.htm#accelerated (last visited April 15, 2015).
[15] 21 C.F.R. 312.310.
[16] 21 C.F.R. 312.315.
[17] 21 C.F.R. 312.320.

Compassionate Use

"Expanded access" or "compassionate use" refers to the use of an investigational medical product outside of a clinical trial, meaning that the medical product has not yet been approved by the FDA.[18] The FDA prefers that patients seek out the use of an investigational medical product through clinical trials.[19] Clinical trials help generate the necessary data to support approval or disapproval of medical products, investigational drugs, and devices. However, under the federal Food, Drug, and Cosmetic Act, an individual may seek individual patient access to investigational products if the following conditions are met:

- The individual's physician determines that there is no comparable or satisfactory alternative therapy available to diagnose, monitor, or treat the person's disease or condition, and the probable risk to the person from the investigational product is not greater than the risk from the disease or condition;
- The FDA determines that there is sufficient evidence of the safety and effectiveness of the investigational product to support its use in the particular circumstance;
- The FDA determines that providing the investigational product will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval;
- The sponsor or the clinical investigator submits a clinical protocol that is consistent with FDA's statute and applicable regulations for INDs or investigational device exemption applications, describing the use of the investigational product.[20]

Additionally, in order for the expanded access or compassionate use request to move forward:

- Both the patient and his or her licensed physician must be willing to participate;
- The patient must have a serious or immediately life-threatening disease or condition;
- The patient must have no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; and
- The patient must be unable to obtain the investigational drug under another IND or to participate in a clinical trial.[21]

To apply for expanded access or compassionate use under a single patient IND, the application must be made by the physician.[22] The physician must also have his patient's informed consent. If applicable, the physician should also ask the medical product company for a Letter of Authorization (LOA). The LOA allows the physician to satisfy some of the requirements for submission by relying on information that the medical product company has already submitted to the FDA.

---

[18] U.S. Food and Drug Administration, *Expanded Access (Compassionate Use)* (last updated February 18, 2015), available at http://www.fda.gov/newsevents/publichealthfocus/expandedaccesscompassionateuse/default.htm (last visited April 15, 2015).
[19] Id.
[20] Id.
[21] Id.
[22] The form's questions include whether the request is an emergency, the patient's clinical history, a proposed treatment plan, the informed consent form. See U.S. Food and Drug Administration, *How to Complete Form FDA 1571 and Form FDA 1572* (last updated February 3, 2015), available at http://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm432757.htm (last visited April 15, 2015).

For non-emergency requests, treatment may begin 30 days after the FDA receives the request if the treating physician fails to hear from the FDA. For emergencies, once authorization is received from the FDA, the physician may begin treatment within 5 working days.[23]

On February 10, 2015, the FDA released draft guidance for comment that would revise the expanded access process. The federal Office of Management and Budget (OMB) estimates that the current process takes physician approximately 8 hours to request for non-emergency situations and 16 hours for emergency cases. For the new process, OMB estimates the process for both emergency and non-emergency situations will take 45 minutes.[24]

Once the FDA has approved a patient for expanded access, the drug manufacturer must still agree to provide the product. There may be only a limited amount of a drug available under a company's expanded access programs.[25] Generally, under expanded access, the drug is provided free of charge, but not always. However, the other costs associated with care related to the patient's disease and condition would be the responsibility of the patient and any available insurance.

Right to Try

Several states have implemented "Right to Try" laws that allow terminally ill patients access to investigational drugs that have completed basic safety testing. More than 60 percent of investigational drugs in Phase I testing are deemed safe enough to move on to Phase II.[26] More than 30 percent then move on from Phase II testing to Phase III. As of March 31, 2015, 12 states have Right to Try laws.[27] Federal legislation to change the expanded access policy has been proposed, but no bill has passed yet. For example, the "Andrea Sloan Compassionate Use Reform and Enhancement Act" would:

> require the sponsor of a 'breakthrough drug' (which is a drug that qualifies for expedited approval, is an infectious disease product, or qualifies the sponsor for a priority review voucher) to submit to the Secretary of Health and Human Services (HHS) and make available to the public a policy on requests for access to the drug for compassionate use, including the minimum criteria for consideration or approval of requests and the time needed to make a decision. [28]

---

[23] *Supra* note 18.
[24] Individual Patient Expanded Access Applications: Form FDA 3926; Draft Guidance for Industry; Availability, 80 Fed. Reg. 7318 (proposed Feb. 10, 2015) (to be codified at 21 CFR pt. 312).
[25] American Cancer Society, *Compassionate Drug Use* (last medical review July 9, 2013), available at http://www.cancer.org/treatment/treatmentsandsideeffects/clinicaltrials/compassionate-drug-use (last visited April 15, 2015).
[26] Michael Hay, et al, *Clinical development success rates for investigational drugs* (January 2014), Nature Biotechnology (see Figure 1- Phase success and LOA rates), available at http://www.nature.com/nbt/journal/v32/n1/abs/nbt.2786.html (last visited April 15, 2015).
[27] National Conference of State Legislatures, *"Right to Try" Experimental Prescription Drugs, State Laws and Legislation for 2014 & 2015* (March 31, 2015), available at http://www.ncsl.org/documents/health/RighttoTry2015.pdf (last visited April 16, 2015).
[28] H.R. 5805 (113th Congress).

Other bills filed have permitted terminally ill patients to access investigational medication before the medication has passed basic safety tests under certain conditions.

III. Effect of Proposed Changes:

This bill creates the "Florida Right to Try Act" under s. 499.0295, F.S.

The bill provides the following definitions:

- An *eligible patient* is defined as an individual who:
  - Has a terminal condition that is attested to by the patient's physician and confirmed by a second independent evaluation by a board-certified physician in an appropriate specialty for that condition;
  - Has considered all other treatment options for the terminal condition currently approved by the FDA;
  - Has given written informed consent for the use of an investigational drug, biological product, or device; and
  - Has documentation from his or her treating physician that the individual has met all of the applicable requirements.

- An *investigational drug, biological product, or device* is defined as a drug, biological product, or device that has successfully completed phase 1 of a clinical trial but has not been approved for general use by the FDA and remains under investigation in a clinical trial approved by the FDA.

- A *terminal condition* is a progressive disease or medical or surgical condition that causes significant functional impairment, is not considered by a treating physician to be reversible even with the administration of available treatment options currently approved by the FDA, and, without life-sustaining procedures, will result in death within 1-year after diagnosis if the condition runs its normal course.

- *Written informed consent* is a document that is signed by a patient, a parent of a minor patient, a court-appointed guardian for a patient, or a health care surrogate designated by a patient and includes:
  - An explanation of the currently approved products and treatments for the patient's terminal condition;
  - An attestation that the patient concurs with his or her physician in believing that all currently approved products and treatments are unlikely to prolong the patient's life;
  - Identification of the specific investigational drug, biological product, or device that the patient is seeking to use;
  - A realistic description of the most likely outcomes of using the investigational drug, biological product, or device. The description must include the possibility that new, unanticipated, different, or worse symptoms might result and that death could be hastened by the proposed treatment and must be based on the physician's knowledge of the efficacy of proposed treatment for the patient's terminal condition;
  - A statement that the patient's health plan or third-party administrator and physician are not obligated to pay for care or treatment consequent to the use of the investigational drug, biological product, or device unless required to do so by law or contract;

- A statement that the patient's eligibility for hospice care may be withdrawn if the patient begins treatment with the investigational drug, biological product, or device and that hospice care may be reinstated if the treatment ends and the patient meets hospice eligibility requirements; and
- A statement 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 liability extends to the patient's estate, unless a contract between the patient and the manufacturer of the investigational drug, biological product, or device states otherwise.

The bill provides that upon the request of an eligible patient a manufacturer may:

- Make an investigational drug, biological product, or device available to an eligible patient;
- Provide an investigational drug, biological product, or device without charge; or
- Require the eligible patient to pay the cost of, or the costs associated with, the manufacture of the investigational drug, biological product, or device.

The bill provides that an insurer, third party administrator, or governmental agency may provide coverage for the cost of, or the costs of services related to the use of, an investigational drug, biological product, or device. However, the bill does not expand coverage an insurer must provide under the Florida Insurance Code and does not affect mandatory health coverage for participation in clinical trials. Additionally, a hospital or health care facility licensed under ch. 395, F.S. is not required to provide new or additional services unless those services are approved by the hospital or health care facility.

If an eligible patient dies while using an investigational drug, biological product, or device pursuant to this section, the patient's heirs are not liable for any outstanding debt related to the patient's use of the investigational drug, biological product, or device.

A licensing board may not revoke, fail to renew, suspend, or take any action against a physician's license issued under chs. 458 or 459, F.S., based solely on the physician's recommendations to an eligible patient regarding access to or treatment with an investigational drug, biological product, or device. A state entity responsible for Medicare certification may not take action against a physician's Medicare certification based solely on the physician's recommendation that an eligible patient have access to an investigational drug, biological product, or device.

The bill does not create a private cause of action against the manufacturer of an investigational drug, biological product, or device; against a person or entity involved in the care of an eligible patient who is using the investigational drug, biological product, or device; or for any harm to the eligible patient that is a result of the use of the investigational drug, biological product, or device if the manufacturer or other person or entity complies in good faith with the terms of this section and exercises reasonable care.

The effective date of the bill is July 1, 2015.

IV. Constitutional Issues:

A. Municipality/County Mandates Restrictions:

None.

B. Public Records/Open Meetings Issues:

None.

C. Trust Funds Restrictions:

None.

V. Fiscal Impact Statement:

A. Tax/Fee Issues:

None.

B. Private Sector Impact:

The bill may increase the number of Floridians who have access to investigational drugs, biological products, and devices.

C. Government Sector Impact:

None.

VI. Technical Deficiencies:

None.

VII. Related Issues:

The AHCA notes a potential conflict with federal law and regulations. The bill prohibits the AHCA from taking action against a health care institution's Medicare certification based solely on a health care provider's recommendation to an eligible patient regarding access to an investigational drug, biological product, or device; however, the federal Centers for Medicare & Medicaid Services (CMS) could direct the AHCA to conduct a complaint investigation regarding such as an issue. The AHCA would be required to report its finding to federal CMS.[29]

The Department of Health (DOH) observed that the bill extends regulatory protection to a physician's *recommendation* of an investigational drug, biological product, or device, but not to the health care provider's *administration* of an investigational drug, biological product, or device. The DOH also advises that while the bill may provide protections to physicians and health care institutions from state regulatory actions, the bill cannot shield such providers from

[29] *Supra* note 37.

federal regulatory actions, even though such federal actions are unlikely under the bill's constructs.[30]

VIII. Statutes Affected:

This bill creates the section 499.0295 of the Florida Statutes.

IX. Additional Information:

A. Committee Substitute – Statement of Substantial Changes:
(Summarizing differences between the Committee Substitute and the prior version of the bill.)

CS/CS by Fiscal Policy on April 20, 2015:
The committee substitute revises the provisions of the "Right to Try Act," including revising definitions and removing provisions related to requiring a patient to participate in data collection, clinical trials, that the bill does not require the Department of Corrections or the Department of Juvenile Justice to provide coverage, and the creation of the misdemeanor penalty for blocking access. The CS also removes provisions related to Physical Orders for Life Sustaining Treatment and the creation of a clearinghouse for compassionate and palliative care plans at the ACHA.

CS by Health Policy on March 17, 2015:
The committee substitute:

- Recognizes Physician Orders for Life Sustaining Treatment (POLST) as evidence of a patient's health care wishes in the same circumstances as "do not resuscitate" orders when presented to a health care professional; and
- Requires a POLST document, in order to be considered valid, to be on a form adopted by the Department of Health and signed by the patient's physician based on a consultation with the patient, the patient's guardian, or a legally authorized proxy or surrogate.

B. Amendments:

None.

[30] *Supra* note 38.