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 Health Policy

BILL:	SB 1052
INTRODUCER:	Senator Brandes
SUBJECT:	Florida Right to Try Act
DATE:	March 14, 2015
REVISED:	

	ANALYST	STAFF DIRECTOR	REFERENCE	ACTION
1.	Lloyd	Stovall	HP	**Pre-meeting**
2.			AHS	
3.			FP	

I. Summary:

SB 1052 creates the "Florida Right to Try Act" (Act), which provides a framework in which an eligible patient with a terminal illness may access investigational drugs, biological products, and devices from the manufacturer after phase one clinical trials.

The bill prohibits actions against a physician's license based solely on his or her recommendation regarding access to or treatment with an investigational drug, product, or device under this Act. Action also may not be taken against a health care institution's state license or its Medicare certification based on its participation in the treatment in or with investigational drugs, biological products, or devices under this Act.

The bill establishes a clearinghouse for compassionate and palliative care plans for state residents. The Agency for Health Care Administration (AHCA or agency) is directed to establish and maintain the site, either independently or through a national or private clearinghouse. The AHCA is also directed to disseminate information about the clearinghouse once available.

The bill is effective July 1, 2015

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 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.

[1] U.S. Food and Drug Administration, *What is the approval process for a new prescription drug?* (last updated August 12, 2013) http://www.fda.gov/AboutFDA/Transparency/Basics/ucm192696.htm (last visited Mar. 14, 2015).

The first step is testing in the laboratory and on animals.[2] Next, the drug is tested for safety and efficacy when used to treat or diagnose a disease in humans.[3]

Clinical trials are part of clinical research which 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 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]

Clinical trials typically are run by a principal investigator, usually a medical doctor, and are approved and monitored by an Institutional Review Board (IRB), an independent committee of experts usually consisting of physicians and non-physicians in hospitals and research institutions.[6] The IRB's role is to ensure the safety and rights of the participants are protected and to periodically review the research.[7]

Informed consent is a critical component of the clinical research and trial process as it provides participants with important information about the trial before they decide to participate. The informed consent form includes information on the expected benefits and risks of participation and that as a volunteer, the participant may withdraw at any time. Withdrawal of too many participants; however, may make the research team ineligible to continue the study.[8]

When testing is complete, the company sends an application to the FDA called a New Drug Application (NDA). If a drug is made out of biologic materials, a company submits a different application, a Biologics License Application (BLA). Regardless of the type of application used, both require similar elements:

- The drug's test results;
- Manufacturing information to demonstrate 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 uses for which it has shown to be useful.[9]

---

[2] U.S. Food and Drug Administration, *What is the approval process for a new prescription drug?* (last updated April 11, 2014) http://www.fda.gov/AboutFDA/Transparency/Basics/ucm194949.htm (last visited Mar. 14, 2015).
[3] Id.
[4] U.S. Department of Health and Human Services, National Institutes of Health, *NIH Clinical Research Trials and You*, (last reviewed February 5, 2015) http://www.nih.gov/health/clinicaltrials/basics.htm (last visited Mar. 14, 2015).
[5] Id.
[6] U.S. Food and Drug Administration, *The FDA's Drug Review Process: Ensuring Drugs are Safe and Effective*, (last updated November 6, 2014) http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm (last viewed Mar. 14, 2015).
[7] U.S. Department of Health and Human Services, National Institutes of Health, *NIH Clinical Research Trials and You*, (last reviewed February 5, 2014) http://www.nih.gov/health/clinicaltrials/basics.htm (last visited Mar. 15, 2015).
[8] Id.
[9] U.S. Food and Drug Administration, *What is the approval process for a new prescription drug?* (last updated April 11, 2014) http://www.fda.gov/AboutFDA/Transparency/Basics/ucm194949.htm (last visited Mar. 14, 2015).

There are different types of clinical trials and the National Institutes of Health (NIH) classifies them into these categories:

- Natural history studies - provide valuable information about how disease and health progress;
- Prevention trials - look for better ways to prevent disease in people who have never had the disease or prevent the disease from returning;
- Screening trials - test the best way to detect certain diseases or health conditions;
- Diagnostic trials - determine better tests or procedures for diagnosing a particular disease or condition;
- Treatment trials - test new treatments, new combinations of drugs, or new approaches to surgery or radiation therapy;
- Quality of Life trials - explore and measure ways to improve the comfort and quality of life of people with chronic illness.

All clinical trials are conducted in phases. Prior to receiving the FDA's approval for human testing, the organization must show the FDA results of preclinical testing in laboratory animals and their proposal for human testing. The FDA must decide if it is reasonably safe for the organization to begin testing the drug in humans.[10] This approval process is based on an investigational new drug (IND) application. Most drugs that undergo preclinical (animal) testing never make it to human testing and review by the FDA.[11]

When an IND application is approved, an IRB established, the protocol approved, and the consent received from study participants, the organization can begin the process:

Clinical Trial Phases[12]			
Phase	**Activities**	**Approx. Time[13]**	**# 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

[10] U.S. Food and Drug Administration, *The FDA's Drug Review Process: Ensuring Drugs are Safe and Effective*, (last updated November 6, 2014) http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm (last viewed Mar. 14, 2015).
[11] Id.
[12] Id.
[13] FierceBiotech, *FDA Approval Process* http://www.fiercebiotech.com/topics/fda_approval_process.asp (last visited Mar. 14, 2015).

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 Once complete, can complete New Drug Application (NDA)	3 years	300-3,000
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		

When an NDA is received by the FDA, the FDA has 60 days to decide whether to file so it can be reviewed.[14] The FDA can refuse to file an application if it is incomplete. If the FDA determines that the drug's benefits outweighs 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.[15] The company receiving approval must continue, under Phase Four, to monitor short and long term results of the drug and submit those findings to the FDA. If the company wants the drug approved for another indication, for another purpose, it must also receive FDA approval.[16]

Accelerated approval is granted by the FDA to some new drugs for serious and life-threatening illnesses that lack other treatment options.[17] This option allows drugs to be approved before measures of effectiveness that are usually required are known.

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";[18]
- Intermediate size groups of patients with similar treatment needs who otherwise do not qualify to participate in a clinical trial;[19] and
- Large groups of patients who do not have other treatment options available.[20]

[14] U.S. Food and Drug Administration, *The FDA's Drug Review Process: Ensuring Drugs are Safe and Effective*, (updated November 6, 2014) http://www.fda.gov/drugs/resourcesforyou/consumers/ucm143534.htm (last viewed Mar. 14, 2015).
[15] Id.
[16] FierceBiotech, *FDA Approval Process*, http://www.fiercebiotech.com/topics/fda_approval_process.asp (last visited Mar. 14, 2015).
[17] U.S. Food and Drug Administration, *FDA's Drug Review Process: Continued, Accelerated Approval*, (updated November 6, 2014) http://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm289601.htm#accelerated (last visited Mar. 15, 2015).
[18] U.S. Food and Drug Administration, *Expanded Access Coverage for Drugs*, (last updated February 18, 2015) http://www.fda.gov/ForPatients/Other/default.htm (last visited Mar. 15, 2015)
[19] 21 U.S.C. §312.315
[20] 21 U.S.C. §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.[21] The FDA prefers that patients seek out the use of an investigational medical product through clinical trials.[22] 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 product 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.[23]

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.[24]

To apply for expanded access or compassionate use under a single patient IND, the application is made by the physician.[25] 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.

---

[21] U.S. Food and Drug Administration, *Expanded Access (Compassionate Use)* (last updated February 18, 2015) http://www.fda.gov/newsevents/publichealthfocus/expandedaccesscompassionateuse/default.htm (last visited Mar. 15, 2015).
[22] Id.
[23] Id.
[24] Id.
[25] 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) http://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/ucm432757.htm (last visited Mar. 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.[26]

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 emergent and non-emergent situations will take 45 minutes.[27]

Once the FDA has approved a patient for expanded access, the drug manufacturer must still agree to provide the product. There may also be only a limited amount of a drug available under a company's expanded access programs.[28] 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 coverage.

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. Over 60 percent of investigational drugs in Phase I testing are deemed safe enough to move on to Phase II.[29] Over 30 percent then move on from Phase II testing to Phase III.

Federal legislation to change the expanded access policy have not been successful. Since 2008, at least four bills have been introduced in Congress, but none have had a committee or floor vote.[30] The Right to Try model legislation allows a patient access to investigational medication that have passed basic safety tests without governmental interference when the following conditions are met:

- The patient has been diagnosed with a terminal disease;
- The patient has considered all available treatment options;
- The patient's doctor has recommended that the investigational drug, device, or biological product represents the patient's best chance at survival;
- The patient or the patient's guardian has provided informed consent; and
- The sponsoring company chooses to make the investigational drug available to patients outside the clinical trial.

---

[26] *Supra* note 21.
[27] 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).
[28] American Cancer Society, *Compassionate Drug Use*, (last medical review July 9, 2013) http://www.cancer.org/treatment/treatmentsandsideeffects/clinicaltrials/compassionate-drug-use (last visited Mar. 15, 2015).
[29] Michael Hay, et al, *Clinical development success rates for investigational drugs* (January 2014), Nature Biotechnology (see Figure 1- Phase success and LOA rates), http://www.nature.com/nbt/journal/v32/n1/abs/nbt.2786.html (last visited Mar. 15, 2015).
[30] Christina Corieri, *Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment*, pg. 20, Goldwater Institute (February 11, 2014).

As of March 15, 2015, seven states have Right to Try laws: Arkansas, Michigan, Colorado, Missouri, Louisiana and Wyoming.[31]

III. Effect of Proposed Changes:

Florida Right to Try Act

SB 1052 creates the "Florida Right to Try Act" (Act) under section 385.213, Florida Statutes and provides definitions applicable to the Act.

An *eligible patient* is defined as an individual who:
- Has a terminal illness determined by the individual's physician and a consulting physician;
- Does not have any comparable or satisfactory FDA-approved options available, as determined by his or her physician, and the probable risk from an investigational drug, biological product, or device is not greater than the disease or condition;
- Has received a prescription or recommendation from the his or her physician for the investigational drug, biological product, or device;
- Has provided written, informed consent for the use of the investigational drug, biological product, or device, or if a minor or lacks the capacity to provide informed consent, a parent's or legal guardian's written, informed consent on the individual's behalf; and
- Has documentation from the individual's physician indicating that the individual has met all of the requirements of this section.

An *investigational drug, biological product, or device* is defined as a drug, biological product or device that has successfully completed Phase one of a clinical trial but has not yet been approved for general use by the FDA.

Physician means a physician under ch. 458, F.S., or ch. 459, F.S., who provides the medical health treatment to the eligible patient for the terminal illness.

Terminal illness means a disease or condition that without life-sustaining procedures will result in the patient's death in the near future or a state of permanent unconsciousness from which recovery is unlikely.

A manufacturer of an investigational drug, biological product, or device has the option to make an investigational drug, biological product or device available to an eligible patient under SB 1052. Relating to the investigation drug, biological product, or device, a manufacturer may also:
- Provide without charge or require the eligible patient to pay the cost of, or the costs associated with its manufacture; and
- Require an eligible patient to participate in data collection relating to the eligible patient's use.

[31] Goldwater Institute, *Arkansas Governor Asa Hutchinson Signs "Right to Try" Bill Into Law* (2014) http://goldwaterinstitute.org/en/work/topics/healthcare/right-to-try/arkansas-governor-asa-hutchinson-signs-right-to-tr (last visited Mar. 15, 2015).

The bill does not require an insurer, health plan, or government health care program to provide coverage for the cost of an investigational drug, biological product, or device or the care or treatment that may be needed as result of an eligible patient's participation, unless it is part of a clinical trial. However, an insurer, health plan or government health care program may elect to provide such coverage, if not part of a clinical trial.

The Department of Corrections or the Department of Juvenile Justice are not required to provide coverage for an investigational drug, biological product or device for individuals in their custody.

Notwithstanding any other law, a state regulatory board or agency may not take any action against a physician's license based solely on the practitioner's recommendation regarding access to or treatment with an investigational drug, biological product, or device.

For health care institutions licensed in this state, a state regulatory board or agency may not take any action against an institution's license or its Medicare certification based solely on the institution's participation in or any other use or treatment with an investigational drug, biological product, or device.

If a clinical trial of an investigational drug, biological product, or device is not effective for a certain patient or condition and the trial is closed due to lack of efficacy, the manufacturer may continue to offer the investigational drug, biological product, or device for a different condition to the same patient or to new patients under this Act.

If the FDA or the safety committee for a clinical trial provides notice of information for an investigational drug, biological product, or device that is being taken by a patient outside of a clinical trial, the manufacturer or the patient's physician is required to notify the patient about the information. For example, the FDA may advise the public of a previously unknown side effect or hidden ingredient of a particular drug that is on the market for another condition or disease, but the drug is also part of a clinical trial for another purpose. The side effect or hidden ingredient could affect those patients taking the drug for another condition outside of a clinical trial.

Under the bill, a private cause of action is not created against a manufacturer of an investigational drug, biological product, or device or against an entity or individual involved in the care of an eligible patient for any harm to the patient resulting from use of the investigational drug, biological product, or device, if the manufacturer, entity, or individual complied with the requirements of this section in good faith, unless the manufacturer, entity or individual failed to exercise reasonable care.

An official, employee, or agent of the state may not block an eligible patient's access to an investigational drug, biological product, or device that has been recommended by his or her physician unless it has been banned or removed from a clinical trial as unsafe by the FDA. If a person does block access, he or she commits a misdemeanor of the second degree.

Clearinghouse for Compassionate and Palliative Care Plans

SB 1052 creates s. 408.064, F.S., and the Clearinghouse for Compassionate and Palliative Care Plans. The AHCA is responsible for establishing and maintaining a reliable and secure database that will allow Florida residents to electronically submit their individual plans for compassionate and palliative care. This database is a clearinghouse of plan information that may be accessed by a health care provider who is treating the resident.

The agency is directed to seek input on the clearinghouse from residents, compassionate and palliative care providers, and health care facilities for its development and implementation.

The agency may also subscribe to or participate in a national or private clearinghouse that will accomplish the same goals in lieu of establishing an independent clearinghouse.
Once available, the agency is required to publish and disseminate information regarding the availability of the clearinghouse to Floridians. The agency must also provide training to health care providers and health care facilities on how to access plans.

Effective Date

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

IV. Constitutional Issues:

A. Municipality/County Mandates Restrictions:

None.

B. Public Records/Open Meetings Issues:

A separate public records exemption may be needed to keep the Compassionate and Palliative Care plans held by the agency exempt from public records requests under ch. 119, F.S.

C. Trust Funds Restrictions:

None.

V. Fiscal Impact Statement:

A. Tax/Fee Issues:

None.

B. Private Sector Impact:

Additional Floridians may have access to investigational drugs, biological products, and devices under the Right to Try Act. Insurers are not required to cover these products or

the treatment resulting from the insured's participation, unless the patient is part of a clinical trial.

C. Government Sector Impact:

There may be a small fiscal impact to the agency to establish and maintain the Clearinghouse for Compassionate and Palliative Care Plans. No estimates have been received from the agency.

VI. Technical Deficiencies:

None.

VII. Related Issues:

None.

VIII. Statutes Affected:

This bill creates the following sections of the Florida Statutes: 385.213 and 408.064.

IX. Additional Information:

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

None.

B. Amendments:

None.