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:	PCS/CS/SB 1052 (592332)
INTRODUCER:	Fiscal Policy Committee (Recommended by Appropriations Subcommittee on Health and Human Services); Health Policy Committee; and Senator Brandes
SUBJECT:	Florida Right to Try Act
DATE:	April 17, 2015 REVISED:

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

Please see Section IX. for Additional Information:

COMMITTEE SUBSTITUTE - Substantial Changes

I. Summary:

PCS/CS/SB 1052 creates the "Florida Right to Try Act," which provides a framework for an eligible patient with a terminal illness to 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 or against a health care institution's state license or its Medicare certification based on its participation in the treatment.

The bill establishes a Clearinghouse for Compassionate and Palliative Care Plans for state residents. The Agency for Health Care Administration (AHCA) 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.

Lastly, the bill recognizes a Physician Order for Life Sustaining Treatment in law and directs the Department of Health (DOH) to develop the form by rule.

Implementation of the clearinghouse is subject to a specific appropriation in the General Appropriations Act. The AHCA estimates the nonrecurring costs of the Clearinghouse for

Compassionate and Palliative Care Plans to be $350,000 for FY 2015-2016, and the recurring cost to be $140,000 from general revenue.

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]

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:

[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.
[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).

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. 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.	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-

[8] Id.
[9] FierceBiotech, *FDA Approval Process*, available at http://www.fiercebiotech.com/topics/fda_approval_process.asp (last visited April 14, 2015).
[10] Supra note 2.
[11] Supra note 7.
[12] Id.

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]

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]

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[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.
[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.

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.

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

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[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).

[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).

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]

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

End of Life Decision-Making

There are a number of different advanced decision making documents an individual may use to express his or her end of life health care decisions. In Florida, state law defines advance directives as witnessed, oral statements or written instructions that express a person's desires about any aspect of his or her future health care, including the designation of a health care surrogate, a living will, or an anatomical gift.[29]

Resuscitation may also be withheld from an individual if a "do not resuscitate" order (DNRO) by the patient's physician is presented to the health care professional treating the patient. For the DNRO to be valid, it must be on the form adopted by the DOH, signed by the patient's physician and the patient, or if the patient is incapacitated, the patient's health care surrogate or proxy, court-appointed guardian, or attorney in fact under a durable power of attorney.[30] It is the responsibility of the Emergency Medical Services provider to ensure that the DNRO form or the patient identification device, which is a miniature version of the form, accompanies the patient.[31] A DNRO may be revoked by the patient at any time, if signed by the patient, or the patient's health care surrogate, proxy, court appointed guardian, or a person acting under a durable power of attorney.[32]

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[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).
[29] S*ee* s. 765.101, F.S.
[30] S*ee* ss. 395.1041, F.S., 400.142, 400.487, 400.605, 400.6095, 401.35, 401.45, 429.255, 429.73, 765.205, F.S., and Rule 64J-2.018, F.A.C.
[31] Id.
[32] Id.

A Physician Order for Life-Sustaining Treatment (POLST) documents a patient's health care wishes in the form of a physician order for a variety of end of life measures, including cardiopulmonary resuscitation (CPR). A DNRO is limited to the withholding of CPR. The POLST form can only be completed by a physician and is then provided to the patient to be kept secured in a visible location for emergency personnel.[33] It is suggested that the form be completed when an individual has a serious illness, regardless of age, as the POLST serves as a medical order for a current illness.[34]

Some questions asked on other states' POLST forms include what level of care is wanted for CPR (attempt or do not attempt); medical intervention (comfort only, limited additional intervention, or full treatment); and artificially administered nutrition (none, trial, or long-term).

III. Effect of Proposed Changes:

Florida Right to Try Act (Sections 1 and 2)

This bill creates the "Florida Right to Try Act" under s. 385.213, F.S., and provides definitions.

Compassionate Treatment and Access to Experimental Treatments

An *eligible patient* is defined as an individual who:

- Has a terminal illness[35] determined by the individual's physician and consulting physician;
- Does not have any comparable or satisfactory FDA-approved options available and the probable risk from an investigational drug, biological product, or device is not greater than the disease or condition, as determined by his or her physician;
- Has received a prescription or recommendation from his or her physician for an 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 applicable requirements.

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.

Availability of Investigational Drugs, Biological Products, or Devices

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. Relating to the investigational drug, biological product, or device, a manufacturer may also:

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[33] POLST.ORG, *FAQ*, available at http://www.polst.org/advance-care-planning/faq/ (last visited April 14, 2015).
[34] POLST.ORG, *POLST v. Advance Directives*, available at http://www.polst.org/advance-care-planning/polst-and-advance-directives/ (last visited April 14, 2015).
[35] The bill defines terminal illness as 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.

- 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.

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

Actions Against a Health Care Provider License

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.

Clinical Trials

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.

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.

No Cause of Action

The bill does not create a private cause of action 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 the bill in good faith, unless the manufacturer, entity or individual failed to exercise reasonable care.

Penalty

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 (Section 3)

The bill 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 AHCA is directed to seek input on the clearinghouse from state residents, compassionate and palliative care providers, and health care facilities for its development and implementation. The AHCA 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 clearinghouse information is available, the AHCA is required to publish and disseminate information regarding the availability of the clearinghouse to Floridians. The AHCA must also provide training to health care providers and health care facilities on how to access plans.

The bill provides that implementation of the clearinghouse is subject to a specific appropriation provided to the AHCA under the General Appropriations Act.

Physician Orders for Life-Sustaining Treatment (POLST)

Sections 4 - 13 make conforming changes to reflect the provisions in statute that require health professional staff to honor "do not resuscitate" orders (DNROs) and recognition of a POLST document. Under s. 404.45, F.S., a valid POLST is described as one completed on the form adopted by the Department of Health by rule, signed by the patient's physician, and based on a consultation with the patient's guardian or legally authorized proxy or surrogate.

The table below reflects the statutes impacted by these revisions.

Statutory Revisions - Addition of POLTS Language	
F.S. Citation	**Description**
s. 395.1041	Hospital Licensing and Regulation: Access to emergency services and care
s. 400.142	Nursing Homes; Emergency medication kits; DNROs
s. 400.487	Home Health Service Agreements; DNROs
s. 400.605	Hospices; Administration; forms; fees

s. 400.6095	Hospice; patient admission; assessment; plan of care; discharge; death
s. 401.35	Medical Transportation Services: Rules
s. 401.45	Denial of emergency treatment; civil liability
s. 429.255	Assisted Living Facilities; Use of personnel; emergency care
s. 429.73	Rules and standards relating to adult family-care homes
s. 765.205	Responsibility of the Surrogate

Effective Date

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:

A separate public records exemption may be needed to keep the Compassionate and Palliative Care plans held by the AHCA Administration exempt from public records requests under ch. 119, F.S.[36]

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, but, under the bill, insurers are not required to cover these products or the treatment resulting from an insured's participation, unless the patient is part of a clinical trial.

C. Government Sector Impact:

The AHCA estimates the costs for the Clearinghouse for Compassionate and Palliative Care Plans to be $350,000 of General Revenue for the first year of implementation and $140,000 per year for maintenance costs to participate in a national or private

[36] See SB 1626 (2015)

clearinghouse.[37] Under the bill, costs relating to the clearinghouse will not be incurred without a specific appropriation provided to the AHCA under the General Appropriations Act.

The Department of Health will incur nonrecurring costs for rulemaking but reports it has sufficient current budget authority to absorb those expected costs. Additionally, indeterminate costs may be incurred for an increase in workload related to additional complaints, but these costs are likely to be absorbed within existing resources.[38]

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.[39]

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 federal regulatory actions, even though such federal actions are unlikely under the bill's constructs.[40]

VIII. Statutes Affected:

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

This bill substantially amends the following sections of the Florida Statutes: 395.1041, 400.142, 400.487, 400.605, 400.6095, 401.35, 401.45, 429.255, 429.73, and 765.205.

[37] Agency for Health Care Administration, *Senate Bill 1052 Analysis* (February 20, 2015), pg. 4 (on file with the Senate Committee on Health Policy).
[38] Department of Health, Florida Board of Medicine, *Senate Bill 1052 Analysis* (February 25, 2015), pg. 5 (on file with the Senate Committee on Health Policy).
[39] *Supra* note 37.
[40] *Supra* note 38.

IX. Additional Information:

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

Recommended CS by Appropriations Subcommittee on Health and Human Services on April 2, 2015:
The proposed committee substitute provides that implementation of the Clearinghouse for Compassionate and Palliative Care Plans is subject to a specific appropriation under the General Appropriations Act.

CS by Health Policy - 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.

This Senate Bill Analysis does not reflect the intent or official position of the bill's introducer or the Florida Senate.