By Senator Burgess

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A bill to be entitled

An act relating to experimental treatments for terminal conditions and life-threatening rare diseases; providing a short title; amending s. 499.0295, F.S.; providing legislative findings and intent; defining terms; authorizing licensed physicians to prescribe and eligible facilities to administer experimental treatments, if certain requirements are met; requiring the department to adopt certain rules; requiring a specified written informed consent from eligible patients; requiring approval of a specified platform or master protocol by an institutional review board; requiring the Department of Health to establish and maintain a patient registry for specified purposes; requiring eligible facilities to submit certain information to the registry; requiring the department to enter into specified contracts with eligible facilities; providing a penalty for breach of such contact; requiring the department to annually publish a specified report; encouraging health insurers and health maintenance organizations to provide specified insurance coverage for experimental treatments; prohibiting such insurers and organizations from denying insurance coverage for experimental treatments; providing construction; authorizing licensed physicians and eligible facilities to receive reimbursement for the prescription or administration of experimental treatments if certain requirements are

met; establishing the reimbursement rate; requiring the department to adopt specified rules; providing for reimbursement rate expiration; requiring manufacturers of experimental treatments to provide for a specified temporary price concession set by the department; providing for expiration of such concession; authorizing the Department of Management Services to enter into a specified contract with an eligible facility or manufacturer; requiring the department to adopt a specified rule for such contract; requiring the Department of Health to adopt by rule certain procedures for licensing of experimental treatment centers; requiring the department to approve or deny a completed application within a specified timeframe; requiring the department to establish by rule certain procedural and operational standards; prohibiting certain actions by specified licensing boards and a specified state agency against a licensed physician in certain circumstances; providing construction and applicability; providing for limitation of liability; providing that certain participation is entirely voluntary at all times; prohibiting public officials, public employees, and public agents from denying or attempting to deny access to experimental treatment; providing construction; requiring the department to adopt rules; providing an effective date.

Be It Enacted by the Legislature of the State of Florida:

Section 1. This act may be cited as the "Promising Pathways Act."

Section 2. Section 499.0295, Florida Statutes, is amended to read:

(Substantial rewording of section. See s. 499.0295, F.S., for present text.)

499.0295 Experimental treatments for terminal conditions and life-threatening rare diseases.—

(1) The Legislature finds that residents of this state with terminal conditions and life-threatening rare diseases may benefit from access to experimental treatments that have demonstrated initial human safety but are not yet approved by the United States Food and Drug Administration.

(2) It is the intent of the Legislature to:

(a) Create a safe, regulated, and transparent pathway to experimental treatments through licensed state institutions with board oversight.

(b) Condition payment on evidence development.

(c) Encourage coverage parity by state-regulated health plans.

(d) Align temporary pricing during the conditional period with the maturity of clinical evidence.

(3) As used in this section, the term:

(a) "Board" means the Board of Medicine, created under chapter 458, or the Board of Osteopathic Medicine, created under chapter 459, as applicable.

(b) "Department" means the Department of Health, created under s. 20.43.

(c) "Eligible facility" means a hospital or health care

facility licensed under chapter 395 that operates under the federal policy for the protection of human subjects under 45 C.F.R. part 46 and maintains, or participates under, Institutional Review Board oversight for experimental treatments.

(d) "Eligible patient" means a person who:

1.a. Has a terminal condition that is attested to by the treating physician and confirmed by a board-certified physician in an appropriate specialty for that condition; or

b. Has a life-threatening rare disease that is attested to by the treating physician and confirmed by a board-certified physician in an appropriate specialty.

2. Has considered and exhausted all other treatment options for the terminal condition or life-threatening rare disease currently approved by the United States Food and Drug Administration.

3. Has given written informed consent pursuant to paragraph (4)(c).

4. Has documentation from the treating physician that the patient meets the requirements of this paragraph.

(e) "Experimental treatment" means:

1. A drug, biological product, or medical device that has successfully completed phase 1 of a clinical trial but has not been approved for general use by the United States Food and Drug Administration (FDA) and remains under investigation in a clinical trial approved by the FDA; or

2. Personalized treatment for use by a patient based on his or her genomic or molecular profile, including, but not limited to, gene-targeted therapies, antisense oligonucleotides (ASOs)-

mediated therapies, and neoantigen-targeting personalized cancer vaccines (PCVs), which have not been approved for general use by the FDA and remain under investigation in a clinical trial approved by the FDA.

(f) "Institutional Review Board" or "IRB" has the same meaning as in s. 381.86.

(g) "Patient registry" or "registry" means a clinical data collections system from which health information is collected at specific points during the course of treatment and all personal identifiers have been removed or obscured to protect patient privacy.

(h) "Rare life-threatening disease" means a progressive disease or medical or surgical condition that affects less than 200,000 persons in the United States. The term includes any progressive disease or medical or surgical condition that meets the criteria of a rare disease under 21 U.S.C. s. 360bb.

(i) "Terminal condition" means 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 the administration of life-sustaining procedures, will result in death within 1 year after diagnosis if the condition runs its normal course.

(4)(a) A physician licensed under chapter 458 or chapter 459 may prescribe to an eligible patient an experimental treatment pursuant to this section.

(b)1. An eligible facility may administer to an eligible patient an experimental treatment if such treatment is

administered pursuant to a platform or master protocol approved by an IRB pursuant to subsection (5) and complies with the registry requirements established in subsection (6).

2.a. An eligible facility that does not maintain its own IRB may establish and maintain an affiliation with another eligible facility that maintains IRB oversight.

b. The department shall adopt rules for affiliations pursuant to this subparagraph, to include, but not be limited to, the handling of drugs, adverse-event escalation, quality assurance, and health data submission.

(c) Written informed consent, signed by an eligible patient, a court-appointed guardian for an eligible patient, or a health care surrogate designated by an eligible patient, is required, and must include:

1. An explanation of the currently approved treatments for the patient's terminal condition or life-threatening rare disease and that such treatments have been considered and exhausted.

2. Identification of the specific experimental treatment that the patient is seeking to use.

3. A description of the potential risks and benefits of using the experimental treatment, including best-case outcome and worst-case outcome. The description must be based on the physician's knowledge of the experimental treatment for the patient's terminal condition or life-threatening rare disease.

4. 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 experimental treatment unless required to do so by law or contract.

5. A statement that the patient's eligibility for hospice care may be affected by the use of experimental treatment.

6. A statement that the patient understands he or she is liable for all expenses consequent to the use of the experimental treatment but that liability does not extend to the patient's estate, unless a contract between the patient and the manufacturer states otherwise.

7. An authorization from the patient to release his or her health information, collected at specific points during treatment, for submission into the registry established in subsection (6).

(5) A platform or master protocol must be approved by an IRB under which treating physicians and eligible facilities may adapt dosing, sequencing, or combinations of authorized experimental treatments, within predefined limitations, without seeking approval of an IRB for each adaptation. The platform or master protocol must specify safety limits, stopping requirements, and patient registry information pursuant to subsection (6).

(6)(a) The department shall establish and maintain a patient registry for the collection and analysis of experimental treatment outcomes under this section. Eligible facilities shall submit health information to the registry from which all personal identifiers have been removed or obscured to protect patient privacy.

(b) The department shall enter into a written contract with eligible facilities which specifies the scope of services provided, the service level, the duration of the agreement, the responsible parties, and the service costs. Failure to meet the

contract requirements may result in the filing of an action by the department and a temporary suspension of reimbursement for the prescription or administration of experimental treatment pursuant to subsection (8).

(c) The department shall annually publish a public report of experimental treatment outcomes and safety signals in the aggregate.

(7)(a) A health insurer or a health maintenance organization regulated under the Florida Insurance Code are encouraged to provide insurance coverage for experimental treatments in the same manner as any other covered treatment or therapy.

(b) A health insurer or a health maintenance organization may not deny insurance coverage based solely on a treatment being experimental or denied approval for general use by the FDA.

(c) This subsection does not require insurance coverage and does not expand the coverage an insurer or health maintenance organization must provide under the Florida Insurance Code.

(8)(a) A licensed physician or an eligible facility may receive reimbursement for the prescription or administration of an experimental treatment under this section. However, in order to receive such reimbursement, an eligible facility must actively participate in the patient registry established under subsection (6). Failure to participate in the patient registry may result in temporary suspension of reimbursement until compliance is restored.

(b)1. Reimbursement for a prescribed or administered experimental treatment under this subsection may not exceed a

discounted rate set by the department as a percentage of a recognized pricing benchmark, including wholesale acquisition cost or average sales price.

2. The department shall adopt rules that:

a. Establish an allowable discount band to reflect evidentiary uncertainty.

b. Apply a registry noncompliance adjustment which increases the discounted rate set by the department.

c. Provide for renewal and potential adjustment of the discounted rate every 2 years based on registry compliance.

(c) Upon approval by the FDA of an experimental treatment, the discounted rate set by the department for reimbursement expires and the standard reimbursement rate applies.

(9) A manufacturer of an experimental treatment shall provide for a temporary price concession, which price must be set by the department by rule as a percentage of a recognized pricing benchmark. The temporary price concession expires upon approval by the FDA of the experimental treatment.

(10) An eligible facility or manufacturer and the Department of Management Services pursuant to s. 110.123 may enter into a healthcare contract under which a portion of payment is linked to experimental treatment outcomes. The department shall adopt a rule for such contract, which rule must provide for standard contract terms and authorize reconciliation intervals not to exceed 2 years.

(11)(a) The department shall adopt by rule procedures for licensing experimental treatment centers and shall approve or deny a completed application within 90 days.

(b) The department shall establish by rule the following

minimum procedural and operational standards:

1. Written policies and procedures.

2. Oversight and governance standards.

3. Inspection and safety standards.

4. Staff training provisions.

5. Recordkeeping provisions.

6. Data quality assurance.

(12)(a) A licensing board may not revoke, deny renewal, suspend, or take any action against a physician's license issued under chapter 458 or chapter 459 based solely on the physician's recommendations to an eligible patient regarding access to or treatment with experimental treatment authorized under this section. A state agency 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 experimental treatment.

(b)1. This section does not create a private cause of action:

a. Against a manufacturer of an experimental treatment;

b. Against a person or an entity involved in the care of an eligible patient who is using an experimental treatment; or

c. For any harm to the eligible patient that is a result of the use of an experimental treatment,

if the manufacturer or other person or entity complies in good faith with the terms of this section and exercises reasonable care.

2. This paragraph does not apply to judicial action brought for gross negligence or intentional, willful, or wanton

misconduct.

(c) If an eligible patient dies while using experimental treatment pursuant to this section, the patient's heirs are not liable for any outstanding debt related to the patient's use of such treatment.

(d) A hospital or licensed health care facility is not required to provide new or additional health care services or treatments under this section without prior consent of the hospital or licensed health care facility. Participation pursuant to this section is entirely voluntary at all times.

(e) A public official, a public employee, or an agent of this state, or any political subdivision thereof, may not deny or attempt to deny access to experimental treatment under this section. Counseling, advice, or recommendations by a licensed physician consistent with the standard of care do not constitute the denial of access or the attempted denial of access to experimental treatment under this paragraph.

(13) This section does not authorize the possession, use, dispensing, or administration of a controlled substance in violation of chapter 893, except to the extent expressly permitted by state or federal law.

(14) The department shall adopt rules pursuant to ss. 120.536(1) and 120.54 to implement this section.

Section 3. This act shall take effect upon becoming a law.