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BILL ANALYSIS

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Senate Bill 991 (as enacted) **PUBLIC ACT 345 of 2014**
House Bill 5649 (as enacted) **PUBLIC ACT 346 of 2014**
Sponsor: Senator John Pappageorge (S.B. 991)
Representative Nancy Jenkins (H.B. 5649)
Senate Committee: Health Policy
House Committee: Health Policy

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RATIONALE

Some people believe that terminally ill patients should have a right to try new treatments before they are officially approved. Under Federal law, new drugs are subjected to an investigative and approval process by the U.S. Food and Drug Administration (FDA) before they can be offered commercially. (The process is described below, under **BACKGROUND**.) This process typically takes five to 10 years for each drug. For a person who is living with a terminal illness and has exhausted all treatment options currently on the market, that time period may be too long. Although Federal law allows people who do not qualify for clinical trials to apply to the FDA on an individual basis for a "compassionate use" exemption from the ban against using investigational drugs, medical devices, and biological products, the application process reportedly can be time-consuming and burdensome. As a result, it was suggested that State law should allow terminally ill patients to request investigational treatments directly from the manufacturers.

CONTENT

Senate Bill 991 created the "Right to Try Act" to provide for access by an eligible terminally ill patient to drugs, biological products, and medical devices not yet approved for general use. Specifically, the Act does the following:

- **Allows an eligible patient to request an investigational drug, biological product, or device from a manufacturer.**
- **Allows the manufacturer to make the drug, product, or device available to an eligible patient, either without compensation or at the patient's expense.**
- **Allows a health insurer to provide coverage for the cost of an investigational drug, biological product, or device.**
- **Provides that a governmental agency does not have to pay costs associated with the use, care, or treatment of a patient with an investigational drug, biological product, or device.**
- **Provides that the heir of a patient who dies while being treated by an investigational drug, product, or device is not liable for any outstanding debt related to the treatment.**
- **Prohibits a regulatory entity from taking any action against a health care provider's license or Medicare certification based solely on the provider's recommendations to an eligible patient regarding access to or treatment with an investigational drug, biological product, or device.**
- **Prohibits a State official, employee, or agent from blocking an eligible patient's access to an investigational drug, biological product, or device.**
- **Provides that the Act does not create a private cause of action against a person for any harm resulting from the use of an investigational drug, product, or device, if the person complies with the Act in good faith and exercises reasonable care.**

-- Provides that the Act does not affect any mandatory health care coverage for participation in clinical trials under the Insurance Code.

House Bill 5649 amended the Public Health Code to specify that a health care provider's recommendation or treatment provided under the Right to Try Act, and a health facility's cooperation in such treatment, is not grounds for disciplinary action by the Department of Licensing and Regulatory Affairs (LARA).

The bills took effect on October 17, 2014.

Senate Bill 991

Drug, Product, & Device Manufacturers

The Right to Try Act allows an eligible patient to request an investigational drug, biological product, or device. The Act allows, but does not require, the manufacturer to make it available.

A manufacturer may provide an investigational drug, biological product, or device to an eligible patient without receiving compensation. A manufacturer also may require an eligible patient to pay the costs of or associated with the manufacture of the drug, product, or device.

The Act defines "investigational drug, biological product, or device" as a drug, biological product, or device that has successfully completed phase 1 of a clinical trial but has not yet been approved for general use by the U.S. Food and Drug Administration and remains under investigation in an FDA-approved clinical trial.

"Eligible patient" means an individual who meets all of the following conditions:

-- Has an advanced illness, attested to by the patient's treating physician.
-- Has considered all other treatment options currently approved by the FDA.
-- Has received a recommendation from his or her physician for an investigational drug, biological product, or device.
-- Has given written, informed consent for the use of the drug, biological product, or device.
-- Has documentation from his or her physician that he or she meets all of the eligibility criteria.

"Advanced illness" means a progressive disease or medical or surgical condition that entails significant functional impairment, that is not considered by a treating physician to be reversible even with administration of current FDA-approved and available treatments, and that, without life-sustaining procedures, will soon result in death.

"Written, informed consent" means a written document that is signed by the patient; parent, if the patient is a minor; legal guardian; or designated patient advocate; is attested to by the patient's physician and a witness; and, at a minimum, includes all of the following:

-- An explanation of the currently approved products and treatments for the disease or condition from which the patient suffers.
-- An attestation that the patient concurs with his or her physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life.
-- Clear identification of the specific proposed investigational drug, biological product, or device that the patient seeks to use.
-- A statement that the patient's health plan or third-party administrator and provider are not obligated to pay for any care or treatments consequent to the use of the investigational drug, product, or device, unless specifically 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 curative treatment, and that care may be reinstated if the treatment ends and the patient meets hospice eligibility requirements.
-- A statement that the patient understands that he or she is liable for all expenses consequent to the use of the drug, product, or device and that the liability extends to the patient's estate,

unless a contract between the patient and the manufacturer of the drug, product, or device states otherwise.

The informed consent document also must include a description of the potentially best and worst outcomes of using the drug, product, or device and a realistic description of the most likely outcome, as well as the possibility that new, unanticipated, different, or worse symptoms might result and the proposed treatment could hasten death. The description of outcomes must be based on the physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition.

Costs & Provision of Service

The Act states that it does not expand the coverage required of an insurer under the Insurance Code. A health plan, third-party administrator, or governmental agency is allowed, but not required, to provide coverage for the cost of an investigational drug, biological product, or device or the cost of services related to its use. The Act does not require any governmental agency to pay costs associated with the use, care, or treatment of a patient with an investigational drug, biological product, or device.

If a patient dies while being treated by an investigational drug, product, or device, his or her heirs are not liable for any outstanding debt related to the treatment or lack of insurance due to the treatment.

The Act also states that it does not require a hospital or facility licensed under Part 215 (Hospitals) of the Public Health Code to provide new or additional services, unless approved by the hospital or facility.

Health Care Providers & Patient Access

A licensing board or disciplinary subcommittee may not revoke, fail to renew, suspend, or take any action against a health care provider's license issued under Article 15 (Occupations) or 17 (Facilities and Agencies) of the Public Health Code based solely on the health care provider's recommendations to an eligible patient regarding access to or treatment with an investigational drug, biological product, or device. An entity responsible for Medicare certification also may not take action against a health care provider's Medicare certification based solely on the provider's recommendation that a patient have access to such a drug, product, or device.

An official, employee, or agent of the State of Michigan may not block or attempt to block an eligible patient's access to an investigational drug, biological product, or device. Counseling, advice, or a recommendation consistent with medical standards of care from a licensed health care provider is not a violation of this provision.

Private Cause of Action

The Act states that it does not create a private cause of action against a manufacturer of an investigational drug, biological product, or device or against any other person or entity involved in the care of an eligible patient using the drug, product, or device, for any resulting harm to the patient, if the manufacturer or other person or entity is complying in good faith with the Act's terms and has exercised reasonable care.

House Bill 5649

Under the bill, except in the case of gross negligence or willful misconduct as determined by LARA, a health care provider's recommendation or treatment provided as authorized under the Right to Try Act is not grounds for LARA to investigate or for disciplinary action against a licensee.

(The Code requires LARA, with regard to a health care provider, to investigate any allegation that certain grounds requiring disciplinary action by a subcommittee exist. After finding that one or more of the prescribed grounds exist, the disciplinary subcommittee must impose certain sanctions, including probation; limitation, denial, suspension, or revocation of the person's license; restitution; or a fine.)

The bill also provides that a health facility's cooperation in a treatment recommended by a health professional under the Right to Try Act, alone, is not grounds for LARA to take any action against a licensee, except in the case of gross negligence or willful misconduct.

(Under the Code, after providing notice and an opportunity for a hearing, LARA may deny, limit, suspend, or revoke the license or certification of a health facility, or impose an administrative fine on a licensee, if certain grounds exist.)

The bill defines "gross negligence" as conduct so reckless as to demonstrate a substantial lack of concern for whether serious injury to a person will result. "Willful misconduct" means conduct committed with an intentional or reckless disregard for the safety of others, as by failing to exercise reasonable care to prevent a known danger.

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BACKGROUND

Clinical Trials

According to the FDA's website, a three-phase testing process is employed for approving a new medical treatment. Before clinical trials may begin, a treatment's sponsor, such as a pharmaceutical company or research institution, must submit an investigational new drug (IND) application that includes the results of preclinical testing in laboratory animals and a proposal for testing in humans. Then, the FDA and a local institutional review board (IRB) must review the IND.

Once the IND is approved, the sponsor can begin Phase 1 of the testing, which is meant to identify a drug's side effects and determine how it is metabolized and excreted. Typically, these studies are conducted on healthy volunteers and involve 20 to 80 subjects.

If Phase 1 does not reveal unacceptable toxicity, Phase 2 studies can begin to generate preliminary data regarding the drug's efficacy in people who have a particular disease or condition. In Phase 2, the sample size ranges from a few dozen to several hundred people.

If Phase 2 indicates that the drug is effective, Phase 3 studies can begin. This final phase usually involves several hundred to several thousand subjects, and is aimed at gathering more data about the drug's safety and efficacy among different populations, in varying dosages, and in combination with other drugs.

Following the completion of all three phases, the sponsor may submit a New Drug Application to formally request that the FDA approve the drug for marketing in the United States.

Compassionate Use Exemption

The Food and Drug Administration Modernization Act authorizes the FDA to grant single-patient INDs (i.e., compassionate use exemptions) to allow the use of an investigational treatment by an individual who is otherwise ineligible for a clinical trial. A compassionate use exemption may be granted only if all of the following conditions are met:

-- The patient's physician determines that the patient has no comparable or satisfactory alternative therapy.

-- The FDA determines that there is sufficient evidence of safety and efficacy to support use of the investigational treatment.
-- The FDA determines that provision of the treatment will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval.
-- The sponsor or clinical investigator submits information sufficient to satisfy IND requirements.

ARGUMENTS

(Please note: The arguments contained in this analysis originate from sources outside the Senate Fiscal Agency. The Senate Fiscal Agency neither supports nor opposes legislation.)

Supporting Argument

Many Americans die of terminal illness every year. When all existing options have failed, a person with a terminal illness might wish to try a treatment that has not yet completed the approval process for widespread use, despite the risk, in the hope of prolonging his or her life. Only a small percentage of patients qualify for participation in clinical trials and the sickest patients are often excluded, meaning that the vast majority of people with an advanced illness do not have access to many promising treatments. People with such serious health conditions cannot wait for up to a decade for a potentially life-saving treatment to be approved and made available to the public.

The procedures for obtaining a compassionate use exemption under Federal law can be quite onerous, potentially delaying access to investigational treatments until it is too late for the patient. The FDA has announced its intention to make a draft form available to physicians to streamline the application process; this form, however, has not yet been finalized. For the time being, doctors and patients must use the existing form for a single-patient IND, which states that the average time needed to collect the necessary information is 100 hours. The mechanism under State law created by Senate Bill 991 might reduce the time and cost burdens associated with this paperwork, encouraging more physicians to pursue expanded access for their patients. The protection afforded by House Bill 5649 to health care providers who recommend and facilitate investigational treatment will give them the confidence to help their patients who have no other options.

There is some risk in undergoing treatment that has not received full approval, and a positive outcome is not guaranteed; however, the risk associated with not trying an investigational treatment is almost certain death. Patients should be able to make their own medical decisions in consultation with their doctors, unimpeded by a bureaucratic process. State law should allow people with advanced illness to exercise their right to try to alleviate their symptoms and preserve their own lives.

Response: The rigorous treatment approval process and the FDA's role in granting compassionate use exemptions exist for a reason: to protect the public. Reportedly, the FDA approves nearly all of the hundreds of individual expanded access applications it receives every year. Establishment of a blanket mechanism for investigational treatment access is unwise. Furthermore, the new Right to Try Act does not negate the Federal law requiring FDA approval for a compassionate use exemption; whether the State law can be implemented is unclear because the Federal law likely preempts it. In addition, although the Right to Try Act allows manufacturers to make their investigational treatments available to eligible patients, whether they will choose to do so is questionable. Typically, manufacturers produce just enough for clinical trial purposes; it is unlikely that many will agree to produce incremental amounts in response to individual requests. For these reasons, it is possible that increased access to investigational treatments under Senate Bill 991 will be negligible. On the other hand, the bill may give people with terminal illness false hope by expanding access to treatments that ultimately might be ineffective or, even worse, amplify suffering and hasten death.

Legislative Analyst: Julie Cassidy

FISCAL IMPACT

Senate Bill 991

The bill permits but does not require insurers, including governmental programs like Medicaid, to cover the costs of investigational medications. If insurers choose to cover an investigational drug, it might increase costs, but also might decrease costs as an investigational treatment may be less costly than standard treatment. Therefore, the fiscal impact on Medicaid and on State and local governments as providers of employee health insurance is indeterminate.

House Bill 5649

The bill will have no fiscal impact on State or local government.

Fiscal Analyst: Steve Angelotti

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