Right to Try
Pennsylvania

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Background

- *Abigail Alliance* (2007) – "…no fundamental right 'deeply rooted in the Nation's history and tradition' of access to experimental drugs for the terminally ill,…"
- Two ways to potentially gain access to unapproved therapies outside of a clinical trial:
  - FDA Expanded Access/Compassionate Use
  - Right to Try laws
    - Colorado 2014 – now 41 states
    - Federal 2018

Expanded Access v. Right to Try

- Both are intended to provide access to unapproved therapies where there are no comparable/satisfactory alternatives and where there is no access to a clinical trial
  - Expanded Access
    - serious or immediately life-threatening disease or condition
  - Right to Try
    - Federal – life-threatening disease or condition
    - PA – terminal illness

- Expanded Access
  - Physician requests therapy from manufacturer
  - FDA approval and IRB oversight required
- Right to Try
  - Physician requests therapy from manufacturer
  - No FDA approval or IRB oversight
- Both
  - Manufacturer does not have to supply therapy
  - Limited information on therapy efficacy and safety
  - Costs may accrue

Timeline

- State Right to Try laws – 1st Colorado – May 2014
- Expanded Access 'streamlined' for individual patients – 2016
- 21st Century Cures Act – December 2016
- Pennsylvania Right to Try – October 2017
  - 38th state to enact Right to Try law
- Federal Right to Try – May 2018

Pennsylvania Right to Try

- Intended to allow terminally ill patients to use potentially life-saving investigational drugs, biological products, and medical devices
- Eligible Patient:
  - Terminal illness (disease/condition that, without life-sustaining procedures, will soon result in death or state of permanent unconsciousness from which recovery is unlikely) attested to by the treating physician
  - Considered all FDA-approved treatment options

Eligible Patient (cont'd)

- Unable to participate in clinical trial within 100 miles of home or not accepted into clinical trial within one week of clinical trial application
- Receives a recommendation from the treating physician for an investigational product that has successfully completed phase 1 of a clinical trial, has not been FDA approved, and remains under investigation in a clinical trial approved by the FDA
- Provides written informed consent (or parent of minor or legally authorized representative) that is signed, attested to by the treating physician and a witness, and is placed in the medical record

Eligible Patient (cont'd)

- Have documentation from the treating physician that the patient met eligibility requirements
- *The patient may not be an inpatient in any hospital*

Informed Consent

- Consent must:
  - Explain currently approved products and treatments for the disease or condition
  - Attest that the patient concurs with the treating physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life
  - Identify the investigational product

Informed Consent (cont'd)

- Describe the potentially best and worst outcomes of using the investigational product, with a realistic description of the most likely outcome, including the possibility that new, unanticipated, different, or worse symptoms might result, and that death could be hastened by the proposed treatment – based upon the treating physician's knowledge of the proposed treatment and the patient's condition
- Make clear that the patient's health insurer and the health care provider are not obligated to pay for the treatment requested or any consequent treatment

Informed Consent (cont'd)

- Make clear that the patient's eligibility for hospice may be withdrawn if the patient begins treatment, but that it may be reinstated if the treatment ends and the patient meets hospice eligibility requirements
- Make clear that in-home health care may be denied if treatment begins
- State that the patient understands that the patient (or his estate) is liable for all expenses consequent to the use of the investigational product, unless a contract between the patient and the manufacturer states otherwise

Access

- A manufacturer is not obligated to provide an investigational product, and may provide the product with or without requiring compensation.
- Insurers are not required to provide coverage that would not otherwise be a covered benefit under the patient's health insurance policy.

Immunities

- Health care provider (licensed health care facility or person licensed, certified, or otherwise regulated to provide health care services under PA law) immunity from criminal or civil liability and from a finding of having committed an act of unprofessional conduct under PA licensure law if, *while exercising reasonable care,* the provider recommends or participates in the use of an investigational product under this law.

- A licensure board may not revoke, suspend, or otherwise take action against:
  - A PA licensed individual based solely on a recommendation to an eligible patient regarding access to or treatment with an investigational product, so long as this is *consistent with medical standards of care*, or
  - Any other PA licensee solely for participating in the use of an investigational product *in good faith* and in accordance with this law.

Private Right of Action

- There is no private cause of action against a manufacturer or any person or entity involved in the care of an eligible patient using an investigational product for any injury suffered by the eligible patient resulting from the use of the investigational product when acting in accordance with this law*, unless the injury results from a failure to exercise reasonable care.*

Federal Right to Try

- Added to FDA law to allow the provision of certain unapproved drugs and biologics:
  - To persons with a life-threatening disease or condition – not limited to terminal
  - Who have exhausted approved treatment options and are unable to participate in a clinical trial – certified by licensed physician who is not paid by the manufacturer for such certification
  - Informed consent by the patient or a legally authorized representative is required – but no content is specified

- Investigational product has completed a phase 1 study and is in active development toward FDA approval
- Exempt from IRB review if certain requirements met (labeling, promotion, cost recovery)
- Manufacturer does not have to provide the product
- Limits on use of adverse outcomes by FDA
- Manufacturer reports annually to the FDA on uses and known serious adverse events
- FDA to post annual summary of uses, including use or non-use of clinical outcomes
- Limits on liability for providing or not providing investigational products

Federal v. State Right to Try Laws

- The federal law does not expressly preempt state law.
- Field preemption v. conflict preemption:
  - Field – federal law covers the whole subject
  - Conflict – federal law rules in case of conflict
    - Where state law covers the same concepts in more detail it may be allowed, e.g., in PA content of informed consent
    - Where state law covers different areas it may be allowed, e.g., in PA insurance coverage, home health, hospice
    - Where there is a conflict state law may be challenged, e.g., PA limited to terminal illness, no hospital inpatients, clinical trial geographic limits or rejection, immunity based on compliance with state law and exercise of *reasonable care*

FDA Expanded Access

- Provides access to drugs, biologics, and medical devices for patients with serious or immediately life-threatening diseases or conditions
- 2016 streamlined process for individual patient access to drugs and biologics – Form FDA 3926
  - Physician request to manufacturer – providing product is voluntary
  - If manufacturer agrees, submit Letter of Authorization and Form 3926 to the FDA – for review of need, risk, appropriateness

- Submit expanded access protocol and consent form to IRB for review (may request waiver of full IRB review – replaced by concurrence of IRB chair or designated IRB member)
- Following IRB approval/concurrence and unless denied by the FDA or on clinical hold the product will be provided and treatment may begin not later than 30 days
- Serious and unexpected adverse events are reported to the FDA, as well as a summary of results
- Can be requested on emergency basis with FDA response in as short of a time as one day.

21st Century Cures Act

- Requires manufacturers and distributors of drugs for more serious conditions to make their policies on evaluating and responding to requests for provision of a drug under expanded access publicly available.
- See company websites or Reagan-Udall Expanded Access Navigator – navigator.reaganudall.org.
- ClinicalTrials.gov also has information about expanded access.

Project Facilitate

- A pilot FDA program to assist oncology healthcare providers in submitting expanded access requests for individual cancer patients.
  - OncProjectFacilitate@fda.hhs.gov
  - 240.402.0004, Mon-Fri, 8:00 am-4:30 pm ET
  - https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate

Summary

- The many state laws appear to have spurred the FDA into streamlining their procedures and providing more assistance with the FDA Expanded Access process.
- Federal Right to Try law provides a baseline for using the Right to Try laws.
- State Right to Try law can fill in gaps in federal law; conflicting areas should be reviewed individually.
- A manufacturer is not required to provide the investigational product in any of these processes.
- Uncertainty regarding the clinical outcome with investigational products remains.