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M E M O R A N D U M

October 19, 2017

TO: Interested Persons

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SUBJECT: Patient Access to Experimental Treatments

Summary

This memorandum addresses patient access to experimental treatments, including access to clinical trials, participation in the U.S. Food and Drug Administration's Expanded Access (Compassionate Use) Program, and access to treatments under Colorado's Right to Try Act. Additionally, this memorandum discusses "'Right to Try" legislation in other states and in the U.S. Congress. Finally, the memorandum addresses insurance coverage of experimental treatments administered both within and outside of clinical trials.

Background

An experimental treatment is a drug, vaccine, medical device, or procedure that is not yet approved by the U.S. Food and Drug Administration (FDA) for regular use in human medicine. A person may access experimental treatments for serious or life-threatening conditions or diseases through clinical trials, the FDA's Expanded Access (Compassionate Use) Program, or Colorado's Right to Try Act.

Clinical Trials

Clinical trials use human subjects to evaluate the health-related outcome of an experimental treatment. Trial participants receive one or more interventions (e.g. drugs, medical devices, vaccines, procedures) so that a researcher can evaluate the effects of the interventions. Clinical trials may be funded by the National Institutes of Health (NIH), other federal agencies, pharmaceutical and medical device companies, or others, including individuals, universities, and community-based organizations. Regardless of funding source, a drug developer must obtain FDA approval in order to conduct clinical trials for an experimental treatment.

There are four phases to the human clinical trial process that establish the safety, efficacy, and outcomes of a given experimental treatment:

- phase one is a small-scale study that tests the safety and dosage of a treatment;
- phase two is a larger-scale study used to identify the effectiveness and side effects of the treatment;
- phase three is a long-term, large-scale study to test the effectiveness and identify adverse side effects of the treatment; and
- phase four is the final test of the treatment's safety and efficacy, and involves giving the treatment to several thousands of participants.

An online database, managed by the NIH's National Library of Medicine, contains information on all current and concluded public and private clinical trials around the world. This database is available at ClinicalTrials.gov, and the information is provided directly from the study sponsors or investigators. Clinical trials can be filtered by a number of different criteria including by state, study type, study phase, or funding source.

FDA Expanded Access (Compassionate Use) Program

A patient, through his or her physician, may request access to an experimental treatment outside of a clinical trial from the FDA. The following conditions must be met by the patient and the patient's physician for the FDA to approve the request for expanded access for the diagnosis, monitoring, or treatment of a serious disease or condition:

- the patient must be unable to obtain the treatment in any other way or participate in a clinical trial;
- the physician must determine that there are no other comparable treatments available for the patient;
- the physician must determine that the risks of the treatment are not greater than the risks posed by the patient's condition;
- the physician must submit documentation describing the treatment plan and use of the investigational treatment on behalf of the patient;
- the FDA must determine that the evidence suggests that treatment may be safe and effective in the circumstance; and
- the FDA must determine that provision of the treatment will not interfere with any current clinical trials.

A physician can complete an expanded access request for an individual patient in about 45 minutes. In emergency situations, a physician can submit a request to the FDA and receive permission for expanded access over the phone, provided that he or she subsequently submits the appropriate paperwork.

FDA expanded access is offered through three different categories: access for individual patients; access for intermediate-sized patient populations; and access for widespread use. The Reagan-Udall Foundation, an independent nonprofit created by Congress to advance regulatory science, has created an Expanded Access Navigator website for the FDA. The navigator guides patients, caregivers, and physicians through the different expanded access options and indicates how to apply for them. Specifically, the navigator provides information on pharmaceutical companies that offer expanded access, including links to company statements regarding their expanded access policies.

From 2012 to 2016, there were 7,510 individual expanded access requests submitted, and 99 percent of the requests were approved by the FDA.

Colorado's Right to Try Act

The Colorado legislature passed the Right to Try Act in 2014, which provides access to treatments for terminally ill patients. The law allows an eligible patient to receive an investigational drug, biological product, or device that has completed phase one of a clinical trial, without seeking permission from the FDA.[1] In order to be considered eligible, a patient must:

- have a terminal illness diagnosis;
- have considered all current FDA-approved treatment options;
- have been unable to participate in a clinical trial;
- have a recommendation from a physician for the investigational treatment; and
- give informed consent regarding the use of the investigational treatment.

According to the act, the manufacturer of the investigational treatment is not required to provide the treatment to a patient that requests it. In some instances, a drug manufacturer may not want to provide treatment to an individual outside of clinical trials or the Expanded Access program due to limited supply of the treatment. Further, a manufacturer may require a patient to pay any costs associated with the treatment.

Currently, it is unknown whether any Coloradans have received an experimental treatment as a result of the Right to Try Act, as there is no agency to which patients, doctors, or pharmaceutical companies must report when operating in accordance with the law.

Right to Try Legislation in the United States

According to the National Conference of State Legislatures, Colorado is one of 37 states that has passed Right to Try legislation. Further, every one of the remaining 13 states has considered such legislation, but failed to pass a bill. In Hawaii, both chambers passed a Right to Try bill, but the legislation was vetoed by the governor.

There is a Right to Try bill currently pending in the U.S. Congress. House Resolution 2368, sponsored by Representative Brian Fitzpatrick (R-PA), was introduced on May 4, 2017, and is currently assigned to the Subcommittee on Crime, Terrorism, Homeland Security, and Investigations. The bill prohibits the federal government from taking actions to restrict or prevent the production, manufacture, distribution, prescribing, and dispensing of experimental treatments for terminally ill patients, provided that those activities are authorized by and carried out in accordance with state law. It also prohibits the federal government from restricting or preventing the possession of an experimental treatment by a patient who has received certification from his or her treating physician. Additionally, the bill waives liability against producers, manufacturers, distributors, prescribers, dispensers, and users of experimental treatments. Finally, the bill prevents a federal agency from using the outcome of a patient's use

[1]Article 45 of Title 25, C.R.S.

of experimental drugs to delay or negatively affect the review or approval of the treatment for widespread use.

It appears that neither the bill pending in Congress nor any of the Right to Try laws passed in other states require tracking the number of patients who access experimental treatment, or reporting to any agencies. As such, it is not known whether any patients in other Right to Try states have successfully accessed experimental treatments.

Insurance Coverage of Experimental Treatments

Clinical trials. In 2009, Colorado passed legislation requiring individual and group insurance companies to provide coverage for routine patient care costs when a beneficiary participates in a clinical trial.[2] Further, the federal Patient Protection and Affordable Care Act (ACA) of 2010 stipulated that private health insurance companies may not deny a beneficiary participation in an approved clinical trial to treat cancer or another life threatening disease or condition. In addition, the insurer may not deny or limit coverage of routine patient costs associated with participation in the clinical trial.

State Medicaid programs are not required to provide the same coverage for beneficiaries that are participating in a clinical trial. Colorado Medicaid does not cover experimental or investigational treatments, including any treatments rendered during a clinical trial that are meant to treat the side effects of the investigational treatment.

Expanded access. Neither Colorado law nor the ACA outlines requirements for health insurance coverage when a beneficiary accesses an experimental treatment outside of a clinical trial setting. Therefore, public and private insurance providers may decide what type of coverage to provide beneficiaries who are receiving experimental treatments.

Colorado law. Colorado's Right to Try Act states that health insurance providers may deny coverage for a beneficiary from the time he or she begins using an experimental treatment without FDA approval or involvement until six months after discontinuing use. Coverage may not be denied for a preexisting condition, however.

Online Resources

The online resources referenced in the memorandum are indicated below.

[2]Section 10-16-104 (20), C.R.S.

Online Resources

National Institutes of Health Clinical Trials Database:
https://clinicaltrials.gov/

FDA Expanded Access (Compassionate Use) Information:
https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm

Expanded Access Navigator:
http://navigator.reaganudall.org/