Legislative Council Staff
Nonpartisan Services for Colorado's Legislature

Memorandum

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TO: Interested Persons

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SUBJECT: Patient Access to Experimental Treatments

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 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 Program, or Colorado's Right to Try Act.

Clinical Trials

Clinical trials use human subjects to evaluate the health-related outcomes of an experimental treatment. Trial participants are randomly selected to receive either a placebo or 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 (20-80 people) that starts testing the safety of a treatment;
- phase two is a larger-scale study (100-300 people) that starts testing effectiveness;
- phase three is a long-term, large-scale study (1,000-3,000) that starts comparing the drug to other treatments; and
- phase four tracks the drug as it become available to the general public after FDA approval.

The NIH's National Library of Medicine clinical trial database contains information on all current and concluded public and private clinical trials around the world.

FDA Expanded Access Program

Expanded access programs are similar to clinical trials but do not typically include control groups (who receive placebos), are less restrictive in their subject inclusion criteria, and offer more geographic flexibility. Pharmaceutical or medical device manufactures may request an expanded access program for their treatment to supplement clinical trials, or to fill the gap between the end of trials and widespread market availability.

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 manufactures that offer expanded access, including links to company statements regarding their expanded access policies.

Single Patient Expanded Access (Compassionate Use)

When clinical trials and existing expanded access trials are not available to a patient with a serious disease who lacks alternative treatment options, his or her physician may request a single patient expended access program.

To receive single-patient expanded access a physician must:

- request permission from the manufacturer
- request permission from the FDA
- obtain approval from an Institutional Review Board
- obtain the patient's informed consent; and
- administer the treatment and report serious and unexpected adverse events and outcomes during the treatment

In emergencies, physicians may skip steps or streamline the process through verbal - instead of written - communications.

Program Assessment

The most recent assessment of the expanded access program was conducted in May 2018, and reported that in the previous five years the FDA received about 9,000 expanded access requests and authorized 99 percent of them. The assessment highlighted lack of awareness, administrative burden, and manufacture reluctance as common barriers to the program.

As of this writing, the clinical trial database finds 260 operational expanded access programs about 100 of which are single patient, but this may not be exhaustive.

Federal Right to Try Act

The U.S Congress passed the federal Right to Try Act in 2017, allowing eligible patients access to select investigational drugs without seeking permission from the FDA. The act does not require manufactures to make the drug available.

To be eligible patients must:

- have a life-threatening disease or condition; and
- have a physician in good standing who will not be compensated by the manufacture:
    - certify that they have exhausted approved treatment options and are unable to participate in a clinical trial; and
    - provide written informed consent.

To be eligible the investigational drug must:

- have completed phase 1 of clinical trials;
- have ongoing clinical trials; and
- be in active development.

The act requires participating manufactures to provide the FDA with an annual usage report.

According the 2025 Right to Try Annual Report, 21 drugs have been accessed through the Right to Try Act since it took effect on May 30, 2018.

In 2022, The Right to Try Clarification Act, clarifying the relationship to the Controlled Substances Act, was introduced in the U.S Senate, but no further action was taken.

Colorado Right to Try Act

Several states passed a version of Right to Try before the passage of the federal act and most states have some version of the law, some of which are more expansive than the federal act.

Colorado was the first state to pass Right to Try in 2014. Compared to the later federal act, House Bill 14-1281:

- expands beyond investigational drugs to biological products and devices;
- has no reporting requirements;
- places no requirements on the attesting physician; and
- uses different terminology (for instance it uses the term "terminal Illness" where the federal law uses "life-threatening condition" defined as one with a "high likelihood of death").

House Bill 25-1270 expanded the Colorado act to cover individualized products that are unique and produced exclusively for the patient, based on their own genetic profile, including individualized gene therapy and individualized neoantigen vaccines. Under the new law, these individualized products are available to the severely debilitated as well as the terminally ill.

Insurance Coverage of Experimental Treatments

Insurance carriers are never required to provide coverage for experimental treatments and Medicaid is federally prohibited from doing so. However, depending on how the experimental treatments are accessed, Colorado and Federal law may protect the patient's access to standard health care coverage.

Clinical Trials

In 2009, Colorado passed House Bill 09-1059 requiring insurance to cover routine patient care costs when a beneficiary participates in a clinical trial. Routine patient care costs include all services that are normally covered. This includes doctors' visits and all approved treatments that supplement the experimental treatment, but does not include the experimental treatment itself.

The Department of Health Care Policy and Financing (HCPF) conformed Colorado Medicaid coverage to the private insurance standard (see this HCPF Bulletin on Clinical Trial Coverage for the full policy)[1] and House Bill 20-1232 codified this practice into state law.

The Affordable Care Act of 2010 and the Federal Clinical Treatment Act of 2020, established similar coverage requirements in federal law.

[1] listed in the Provider Bulletin Index under B1900433

Right to Try

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 until six months after discontinuing use.

Expanded Access

Neither Colorado nor federal law outline requirements for health insurance coverage when a beneficiary accesses an experimental treatment outside of a clinical trial setting, or the Right to Try Law.