PUTTING PATIENTS FIRST

UNLEASHING INNOVATION IN AMERICAN HEALTHCARE

GOLDWATER INSTITUTE

INTRODUCTION

The COVID-19 pandemic has opened Americans' eyes to new challenges and opportunities in our healthcare system. Long-pending reforms such as easing restrictions on telemedicine became reality almost overnight, while shortcomings in our nation's drug approval process were never more evident than during the nation's wait for a coronavirus vaccine. Through it all, the Goldwater Institute provided leadership in healthcare reform, and today we offer a transformational blueprint for the Food and Drug Administration (FDA).

The Goldwater Institute has long been a champion of expanding and protecting individual liberty by working in courthouses, statehouses, and communities. Many know the Institute for our landmark Right to Try law, which upholds the principle that you—not the government—have the right to decide to pursue potentially lifesaving medical treatments. Passed in 41 states and signed into federal law in 2018, Right to Try ensures that the government does not get a veto stamp over a lifesaving treatment when it is available and recommended by your doctor.

But much work remains to protect this important principle. Despite the Right to Try's significant impact, the FDA continues to wield enormous power and influence over our medical autonomy—usually with little or no accountability. We also know that often it is not a failure of scientific progress standing in the way of lifesaving treatment, but rather bureaucratic obstacles. During the COVID-19 crisis, we have witnessed how removing red tape accelerates the availability of treatments and vaccines.

More than half a million patients will die of cancer alone this year—most without access to the 700 new treatments already under consideration. Countless more will suffer and die from terminal illnesses that have no FDA-approved cure, including ALS, Duchenne muscular dystrophy, and Alzheimer's, even though promising treatments are already in the FDA's pipeline.

That is why the Goldwater Institute will continue its work by ensuring that the FDA is transparent and accountable. This blueprint relies on important research and scientific advances, and continues to embrace important protections for vulnerable patients—all while expanding and respecting medical autonomy.

The reforms in the Goldwater Institute's FDA Blueprint move us closer to the important goal of bringing the right treatment, to the right patient, at the right time. And they do so in a way that remains faithful to the tenet that the government shouldn't stand in the way of patients who are under their doctor's care and trying to save their own lives.

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Today, many of the newest medical treatments are personalized for the individual patient, using information about that person's own genes and particular disease. This type of gene therapy can be used to directly target specific breast cancer cells while avoiding healthy cells, something that is impossible in traditional chemotherapy, to name just one example. Unfortunately, the United States' drug approval system often prevents patients from accessing these treatments, even as their lives hang in the balance. Fortunately, state and federal lawmakers have a way to change this outdated system.

The U.S. drug approval system blocks Americans from potentially lifesaving medicines and treatments until those treatments receive final approval from the Food and Drug Administration (FDA). Although the pace of medical innovation has increased rapidly in recent decades, it still takes an average of 14 years and $1.4 billion for a drug to make its way through the clinical trial process and obtain FDA approval.

Dying patients simply do not have that kind of time.

A new law called Right to Try is beginning to change things. Signed into federal law in 2018, Right to Try protects the right of terminally ill patients to try medicines that are already in the FDA's drug approval pipeline and have the agency's safety approval but have not yet been approved for sale on the market. In other words, patients can access the same cutting-edge treatments that are being given to the select few who are lucky enough to be enrolled in ongoing clinical trials. Under Right to Try, patients work in consultation with their physicians to determine a course of treatment that fits their individual needs. Right to Try remedies some of the inequities created by the FDA's approval process, an approach which all but ignores the role of the patient in making informed decisions about their own care.

Right to Try is saving lives and allowing people to have a say in their own destinies. It is a declaration that people should be able to decide for themselves, in consultation with their doctors, whether to try medicines that could prolong or even save their lives. It is a basic human right to fight for one's life.

But patients need more. Right to Try allows access to treatments that are in FDA clinical trials and therefore does not usually apply to personalized treatments. There is no initial clinical trial safety threshold for personalized treatments because safety and efficacy are simultaneously "tested" on the individual patient in the "trial." Thus, Right to Try must be taken to the next level and expanded so the growing number of patients seeking individualized treatments are not caught in needless bureaucratic red tape.

SAFEGUARD THE RIGHT TO TRY CUTTING-EDGE MEDICINE *(CONT'D)*

THE SOLUTION

WHO CAN ACT: STATE LEGISLATORS, CONGRESS

Extending Right to Try to individualized patient treatments should be on the policy agendas of every state and federal lawmaker. The current FDA approval process is a regulatory mismatch for treatments that are individualized for a single patient.

The concept of Right to Try for personalized medicine parallels that of the original Right to Try: once the FDA ensures basic safety, a terminally ill patient can work directly with her doctor to seek treatment—without having to first get government permission. In the personalized medicine context, the safety component would not be the first phase of a clinical trial, but instead would be a set of standardized safety protocols that the manufacturer and doctor must follow while providing the treatment. This approach is both imperative and sensible. In fact, a similar process has been in place for over three decades for laboratory testing performed on human specimens.

VIEW MODEL LEGISLATION

FOR MORE INFORMATION

Naomi Lopez and Christina Sandefur, "How Outdated FDA Overregulation Is Limiting Patient Access to the Next Generation of Treatments," in *Purple Solutions: A Bipartisan Roadmap to Better Healthcare in America* (2020), https://www.amazon.com/Purple-Solutions-bipartisan-roadmap-healthcare-ebook/dp/B08BF5GLJ7

REMOVE THE SECRECY FROM THE U.S. DRUG APPROVAL PROCESS

THE PROBLEM

At a time when many are calling for more transparency in healthcare, the FDA refuses to share information about its drug approval process and the circumstances under which it will or will not grant patients special access to lifesaving medications not yet approved for use. Under the Freedom of Information Act (FOIA), agencies must disclose public information when requested unless that information is clearly exempt from disclosure under the statute. And the U.S. Supreme Court has ruled that under FOIA, "the strong presumption in favor of disclosure places the burden on the agency to justify the withholding of any requested documents." Yet the FDA routinely refuses to follow the law, leaving Americans in the dark about how the agency makes life-or-death decisions.

The Goldwater Institute has repeatedly sought clarification from the FDA regarding its approval processes. For example:

★In 2014, the Goldwater Institute asked the FDA why it allowed two American aid workers infected with Ebola to use an experimental treatment that had not been tested on humans, while denying countless Americans with terminal illnesses access to treatments that had undergone years of human trials. The FDA refused to respond, and only after a lengthy court battle was it forced to release these records.

★In early 2016, the Goldwater Institute sought information from the FDA about its processes for requesting investigational treatments outside of clinical trials. It took five years and legal action before the FDA finally fulfilled the request.

★More recently, the Goldwater Institute asked why the FDA interfered with a doctor's treatment recommendations for his dying patient, especially when the doctor believed the FDA's recommendation would be ineffective and possibly dangerous. After all, the FDA is not supposed to regulate the practice of medicine. So far, the FDA has ignored that request.

It shouldn't take years and an army of lawyers to learn how and why the FDA makes decisions about who gets access to treatments, especially when those decisions can have life-or-death consequences. That information should be made readily available to all Americans. Transparency, not secrecy, should be the default.

REMOVE THE SECRECY FROM THE U.S. DRUG APPROVAL PROCESS *(CONT'D)*

THE SOLUTION

WHO CAN ACT: THE FDA, CONGRESS

Congress should hold the FDA accountable for its habitual failure to share requested information in a timely manner. Congressional hearings could shed light on how the decisions to evade FOIA requests are being made, and lawmakers could impose, through legislation, consequences for not complying with federal law.

Moreover, taxpayers are footing the bill for the FDA's transparency failures, as the only recourse citizens have (if they're lucky enough to be able to secure and afford an attorney) is to turn to the courts, and these cases can take years to resolve. Timely compliance with FOIA requests will save taxpayers the expensive, resource-heavy court battles.

There is no reason why an agency that makes life-or-death determinations over American lives should be able to evade federal law and force years-long court battles to obtain the requested information. How the FDA makes decisions over whose lives it is going to save should never be a government secret.

VIEW MODEL LEGISLATION

FOR MORE INFORMATION

Freedom of Information Act, https://www.foia.gov/foia-statute.html

Jon Riches, "The Public Has the Right to Know How Government Makes Drug Approval Decisions," In Defense of Liberty (blog), Goldwater Institute (March 24, 2020), https://indefenseofliberty.blog/2020/03/24/the-public-has-a-right-to-know-how-government-makes-drug-approval-decisions/

Naomi Lopez, "When the FDA Is Hazardous to Your Health," In Defense of Liberty (blog), Goldwater Institute (October 1, 2020), https://indefenseofliberty.blog/2020/10/01/when-the-fda-is-hazardous-to-your-health/

EMPOWER PATIENTS WITH INFORMATION ABOUT ACCESS TO TREATMENTS

THE PROBLEM

ClinicalTrials.gov is the central registry for human clinical trials. Doctors, patients, their loved ones, and researchers often rely on the website to seek out and learn about investigational treatments under FDA evaluation. This federally maintained online registry tracks hundreds of thousands of privately and publicly funded clinical trials both in and outside of the United States, providing public access to a vast repository of valuable research information.

While certain trials are required by law to be registered on ClinicalTrials.gov, many trials are listed even when there is no legal requirement to do so. Unfortunately, the federal agencies that have jurisdiction over ClinicalTrials.gov have excluded information about manufacturers' Right to Try programs now available under the federal Right to Try law, even when sponsors have requested their treatment information be included.

This means patients who cannot qualify for a clinical trial—and only about 3% of the sickest patients do—will not find information on ClinicalTrials.gov to help them access treatments available through Right to Try. This makes no sense, since a treatment does not qualify for Right to Try unless it has completed Phase 1 FDA clinical evaluation (basic safety testing) and is being given to patients in FDA clinical trials. Yet ClinicalTrials.gov includes information about nonapproved, potentially dangerous treatments that are not under FDA review and are being administered in places that lack the level of safety standards in place in the U.S., such as Azerbaijan and Togo.

Medicines available under Right to Try are now being used to save lives, but manufacturers cannot share crucial information with doctors and patients via the government's only online registry. There is no reason why the federal government should exclude information that might make it simpler and faster for doctors and patients to learn about promising and safe treatments available under Right to Try.

THE SOLUTION

WHO CAN ACT: CONGRESS, NATIONAL LIBRARY OF MEDICINE

The National Library of Medicine should be directed to accept and include information about medicines available under Right to Try on ClinicalTrials.gov. This commonsense addition to the already extensive online registry would benefit patients and physicians by providing access to valuable information about lifesaving treatments that are already in use and which may provide lifelines to other patients.

VIEW MODEL LEGISLATION

ENSURE DOCTORS HAVE INFORMATION ABOUT CUTTING-EDGE TREATMENTS

THE PROBLEM

When a drug is prescribed for a purpose, patient population, or dosage that differs from the FDA-approved indication, it is being prescribed "off-label." These kinds of prescriptions are extremely common: Roughly 20% of all drugs are prescribed off-label. If your child has ever been prescribed amoxicillin for an ear infection, for example, the prescription was off-label, since this antibiotic isn't approved for kids.

While physicians can legally prescribe treatments off-label, federal law often prevents manufacturers from sharing truthful and scientific information about off-label uses. In fact, communicating about a legal, off-label use can result in criminal prosecution. The threat of fines or even jail time for speaking the truth (and helping patients) has had a chilling effect on pharmaceutical companies' willingness to share information with doctors and payers—leaving patients in the lurch.

The ability to share truthful, scientific, and up-to-date information about off-label treatments is critical to ensuring that doctors can provide the best treatments for their patients, especially when lives are at stake. Physicians need to understand the treatment needs of patients, but too many are being kept in the dark without access to full information about treatment options.

THE SOLUTION

WHO CAN ACT: STATE LEGISLATORS, CONGRESS

State and federal lawmakers should adopt the Truth in Medicine Act, allowing manufacturers to share truthful scientific information about off-label treatments. This reform protects the right to freely exchange valuable data about legal treatments and provides doctors and insurers with the tools they need to make informed healthcare decisions with their patients.

Several states have unanimously passed laws that protect the sharing of truthful and scientific information with doctors and insurers. More states are turning their attention to these obstacles that too often suppress information about promising treatments. Congress should follow suit, making this pro-patient reform the law of the land.

VIEW MODEL LEGISLATION

FOR MORE INFORMATION

Naomi Lopez and Christina Sandefur, "Restoring Free Speech in Medicine: How State Lawmakers Can Overcome FDA Regulations that Keep Doctors and Payers in the Dark," Goldwater Institute (June 6, 2017), https://goldwaterinstitute.org/wp-content/uploads/2017/06/Restoring-Free-Speech-in-Medicine-Policy-Paper.pdf

Mark Flatten, "Gagged: Feds Use Criminal Charges, Threats to Silence Drugmakers," Goldwater Institute (February 2, 2019), https://goldwaterinstitute.org/2019-02-26-howard-root-gagged/

BRING THE WORLD'S LEADING MEDICAL TREATMENTS TO AMERICANS

THE PROBLEM

While the U.S. leads the world in treatment innovation, an estimated 30% of the newest advances in medicine are first available overseas. Treatments that have been tested and approved in other countries are still tied up in American red tape, and patients are forced to wait years for the FDA to approve that same treatment in the U.S.

When already-approved treatments from other leading countries that share the U.S.'s rigorous clinical evaluation aren't yet available to American patients facing devastating illnesses, there should be an expedited pathway for harnessing important scientific work that has already been undertaken.

THE SOLUTION

WHO CAN ACT: THE FDA, CONGRESS

Congress should adopt international reciprocity legislation, previously introduced by Congress as the 2019 RESULT Act and included as a model here, and cut the red tape and bureaucracy for treatments and vaccines commercially available in other parts of the world. International reciprocity legislation would require the FDA to quickly review drug, device, and biologic applications for products already approved in countries with drug evaluation approval processes that are similarly rigorous and scientific.

VIEW MODEL LEGISLATION

FOR MORE INFORMATION

Naomi Lopez, "Why Must U.S. Patients Wait for Drugs Available in Other Countries?," Washington Examiner (July 31, 2019), https://www.washingtonexaminer.com/opinion/op-eds/why-must-us-patients-wait-for-drugs-available-in-other-countries

Nicole C. Perez, "International Reciprocity: If a Drug Is Good Enough for Great Britain, It Should Be Good Enough for the United States," University of Miami Business Law Review (2016), https://repository.law.miami.edu/cgi/viewcontent.cgi?article=1288&context=umblr

END "EXPERT PANELS" THAT KEEP TREATMENTS FROM THE MOST VULNERABLE

THE PROBLEM

Should a panel of "expert" bureaucrats be able to put a value on how much a life is worth? Under this system of rationing, government payers and insurance companies decide which treatments to reimburse based on recommendations made by these experts, who assess the value of drug treatments and tests. The conclusion is that less money should be spent on treating a person who is sick, disabled, or old, because their life is deemed to be worth less. Assigning such a value to a patient's life—a value known as a quality-adjusted life year, or QALY—can make all the difference between whether a treatment is approved or denied for reimbursement.

Some state and federal lawmakers advocate using QALYs and similar metrics to ration care. In addition to the ethical dilemmas that such a system creates, this method of putting a value on a patient's life doesn't take into account the worth of fast-moving medical innovation, plus it consistently undervalues treatments and results in delayed and inferior care. The consequences of systematically overriding physicians' recommendations—who are at the bedside and aware of the details of a unique case—and instead relying on distant recommendations with often outdated or inadequate treatments from an "expert" panel can be truly devastating to patients.

THE SOLUTION

WHO CAN ACT: STATE LEGISLATORS, CONGRESS

Lawmakers at both the federal and state levels should oppose QALY-based value assessments to determine coverage and reimbursement decisions for government healthcare programs. At the federal level, lawmakers should also preempt the use of QALY-based approaches to inform drug approvals.

While it may seem like the twin goals of improving quality and reducing healthcare costs are incompatible, they are already being achieved through innovations in the areas of self-care, minimally invasive procedures, and pharmaceuticals. Since hospital stays and nursing home care are enormously expensive, measures that reduce these costs would certainly be appealing.

Lawmakers at both the federal and state levels have begun to limit the use of QALYs in coverage decisions. As part of the Affordable Care Act, Congress banned the use of these types of rationing metrics in cost-effectiveness reviews and evaluations in the Medicare program. In early 2020, Oklahoma lawmakers limited the use of QALYs in coverage decisions when they enacted HB 2587. Federal and state lawmakers should replicate and build upon this approach to prevent government payers from adopting QALY or similar measures to determine coverage, reimbursement, or utilization management decisions.

VIEW MODEL LEGISLATION

END "EXPERT PANELS" THAT KEEP *(CONT'D)* TREATMENTS FROM THE MOST VULNERABLE

FOR MORE INFORMATION

Rafael Fonseca, M.D., and Naomi Lopez, "Deciding What a Life is Worth: The Top Three Things Lawmakers Need to Know About QALYs," Goldwater Institute (December 2, 2020), https://goldwaterinstitute.org/qalys/

"ICER: Key Questions for Policy Makers to Consider About Health Care Treatment Value, Pioneer Institute, https://pioneerinstitute.org/wp-content/uploads/KeyQuestionsHandout.pdf

ALLOW STATES TO APPROVE TESTS AND PROTECTIVE EQUIPMENT

THE PROBLEM

The United States lagged most of the developed world throughout the COVID-19 crisis when it came to testing for the virus and having adequate personal protective equipment (PPE) for frontline workers. Many will recall that the Centers for Disease Control (CDC) approved its own COVID-19 test, which turned out to be faulty due to contaminated materials. Meanwhile, it was illegal for states to validate and use their own tests without prior federal approval. Existing rules and regulations created similar obstacles for states to manage their own production and certification of PPE.

The destructive impact of onerous and counterproductive red tape defies comprehension. For example, FDA regulators ordered a halt to the Seattle Coronavirus Network Assessment (SCAN), a program that aims to test patients for exposure to COVID-19. Promoted by Bill Gates, it had already been authorized by the state and included technical assistance from CDC. Yet the FDA shut it down because it had not received bureaucratic authorization to share the results of self-administered tests with program participants. Keep in mind there was no concern about the accuracy of the tests. This was about paperwork.

It is important to acknowledge the many examples of federal agencies moving quickly to address this crisis. But the dangers and consequences are real when states are denied the authority to act when the federal government cannot or will not.

That workers on the frontlines were caring for patients without sufficient testing supplies, and protection is nothing short of abysmal failure. The proper lesson from this pandemic isn't that the federal government should have been more coordinated and efficient; the lesson is that states should be empowered to protect and care for their residents, especially in times of crisis.

When fighting a pandemic, the fight should be against the virus, not rules, red tape, and bureaucracy.

THE SOLUTION

WHO CAN ACT: FDA, CONGRESS

Congress should adopt legislation, previously introduced and included as a model here, to grant state departments of health the authority to certify tests and equipment during times of national or state-declared emergencies.. This approach establishes the presumption that state health departments can and should be empowered to act more quickly. States would still be required to submit for federal approval but would do so without tying their hands behind their backs in order to respond in an emergency.

VIEW MODEL REGULATORY AMENDMENT

EASE THE PATHWAY FOR COMPOUNDING PHARMACIES TO SERVE PATIENTS

THE PROBLEM

Compounding pharmacies can create customized prescription doses and formulations. For example, a child may need a medicine tablet that contains a much smaller, precise dose than the size normally manufactured. The medicine can be tailored to the individual patient.

Compounding pharmacies can also create products that were already in use and "grandfathered" when the original FDA approval process was established. These drugs have been used for decades and, while not technically FDA approved since they predate the FDA approval process, the intent was to exempt them from that process (but still require a prescription).

Unfortunately, in recent years, the FDA has forced manufacturers of these decades-old, grandfathered products to seek new approval. Not only is this impractical for treatments that normally cost a few cents per dose, it imposes enormous costs on those patients who rely on the drugs. It is simply not profitable for a manufacturer to go through this FDA process for treatments in which the costs are unlikely to be recouped.

THE SOLUTION

WHO CAN ACT: FDA, CONGRESS

Congress should limit the FDA's discretion over requiring new approval for existing medicines. The FDA's overbroad interpretation of its authority could be corrected utilizing language previously introduced by Congress and included as a model here.

VIEW MODEL LEGISLATION

FOR MORE INFORMATION

Mark Flatten, "Sickening: FDA Bureaucracy Blocks Common 'Miracle Drug,'" Goldwater Institute (October 25, 2016), https://goldwaterinstitute.org/wp-content/uploads/cms_page_media/2016/10/25/D%20double%20page.pdf

Naomi Lopez, "How the FDA Continues to Fail Vulnerable Patients," In Defense of Liberty (blog), Goldwater Institute (August 19, 2020), https://indefenseofliberty.blog/2020/08/19/how-the-fda-continues-to-fail-vulnerable-patients/

OPEN THE DOOR FOR STEM CELL TREATMENTS AND REGENERATIVE MEDICINE

THE PROBLEM

Regenerative treatments seek to replace the damaged tissue and organs in one's body. This approach holds enormous promise in restoring function instead of only treating symptoms or requiring far more invasive surgical procedures. The earliest regenerative treatments date back to the ancient civilizations and included the cleansing and removal of damaged tissue from wounds. Tissue grafting, surgical implants such as knee and hip replacement, and organ transplants are common examples used today.

Modern regenerative medicine includes stem cell treatments where cells can self-renew, replicate, and differentiate to create multiple types of cells and tissue manufacturing. Tissue is created outside the body to be used for tissue repair or replacement. For example, tissue can be grown to repair and restore burn victims' skin, and stem cells can be used for spinal fusion and disc replacement.

Unfortunately, the rapid growth of these innovations is outpacing the ability of the FDA to keep up. The Alliance for Regenerative Medicine reports two stem cell clinics in the U.S. as recently as 2009. By 2017, the number had mushroomed to 700. Furthermore, the nature of these treatments is not well suited for the decades-old regulatory approval process now in place.

Recognizing the regulatory mismatch and potential many of these treatments hold, Japan has already taken the lead in the area of regenerative medicine approval. Some regenerative medicine treatments are granted a seven-year conditional approval after the treatment has passed Phase 1 safety testing. During the conditional approval period, data continues to be collected, which informs final approval.

It is time for the U.S. to recognize that the current FDA approval process no longer meets the realities of 21st century medicine and instead stifles treatment options for doctors and their patients.

THE SOLUTION

WHO CAN ACT: CONGRESS

The FDA plays an important role in ensuring that treatments adhere to scientific standards and best industry practices. But it is time to modernize and simplify the regulatory structure for these innovative treatments—those that use a patient's own cells or tissue (or those of a close relative) and involve only small changes. The field of regenerative medicine is growing dramatically, and the ability for regulators to keep up is becoming difficult. Now that these treatments are become more common both in the U.S. and around the world, it is time to allow for more access to them—and simultaneously free regulators to direct their attention to more complex treatments.

VIEW MODEL LEGISLATION

APPENDIX
MODEL LEGISLATION

Safeguard the Right to Try Cutting-Edge Medicine (Right to Try 2.0)

SEC. 1.

(1) As used in this act, and unless the context otherwise requires:

(a) "Life-threatening or severely debilitating illness," as defined in section 312.81 of Title 21, Code of Federal Regulations (or any successor regulation).

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

(i) Has a life-threatening or severely debilitating illness, attested to by the patient's treating physician.

(ii) Has considered all other treatment options currently approved by the United States food and drug administration.

(iii) Has received a recommendation from his or her physician for an individualized investigational treatment, based on analysis of the patient's genomic sequence, human chromosomes, deoxyribonucleic acid, ribonucleic acid, genes, gene products (such as enzymes and other types of proteins), or metabolites.

(iv) Has given written, informed consent for the use of the investigational drug, biological product, or device.

(v) Has documentation from his or her physician that he or she meets the requirements of this subdivision.

(c) "Individualized investigational treatment" means drugs, biological products, or devices that is unique to and produced exclusively for use for an individual patient, based on their own genetic profile. "Individualized investigational treatment" includes, but is not limited to, individualized gene therapy antisense oligonucleotides (ASO) and individualized neoantigen vaccines.

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

(i) An explanation of the currently approved products and treatments for the disease or condition from which the patient suffers.

(ii) 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.

(iii) Clear identification of the specific proposed individualized investigational drug, biological product, or device that the patient is seeking to use.

(iv) A description of the potentially best and worst outcomes of using the individualized investigational drug, biological product, or device and a realistic description of the most likely outcome. The description shall include the possibility that new, unanticipated, different, or worse symptoms might result and that death could be hastened by the proposed treatment. The description shall be based on the physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition.

(v) 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 individualized investigational drug, biological product, or device, unless they are specifically required to do so by law or contract.

(vi) A statement that the patient's eligibility for hospice care may be withdrawn if the patient begins curative treatment with the individualized investigational drug, biological product, or device and that care may be reinstated if this treatment ends and the patient meets hospice eligibility requirements.

(vii) A statement that the patient understands that he or she is liable for all expenses consequent to the use of the individualized investigational drug, biological product, or device and that this liability extends to the patient's estate, unless a contract between the patient and the manufacturer of the drug, biological product, or device states otherwise.

(e.) "Eligible facility" means an institution that is operating under a Federalwide Assurance (FWA) for the Protection of Human Subjects under 42 U.S.C. 289(a) and 45 CFR Part 46. And eligible facility is subject to the FWA laws, regulations, policies, and guidelines including renewals or updates.

SEC. 2.

(1) A manufacturer operating within an eligible facility and pursuant to all applicable FWA laws and regulations may make available an individualized investigative treatment and an eligible patient may request an individualized investigational drug, biological product, or device from an eligible facility or manufacturer operating within an eligible facility under this act. This act does not require that a manufacturer make available an individualized investigational drug, biological product, or device to an eligible patient.

(2) An eligible facility or manufacturer operating within an eligible facility may do all of the following:

(a) Provide an individualized investigational drug, biological product, or device to an eligible patient without receiving compensation.

(b) Require an eligible patient to pay the costs of, or the costs associated with, the manufacture of the investigational drug, biological product, or device.

SEC. 3.

(1) This act does not expand the coverage required of an insurer under the insurance code of

(2) A health plan, third party administrator, or governmental agency may, but is not required to, provide coverage for the cost of an individualized investigational drug, biological product, or device, or the cost of services related to the use of an individualized investigational drug, biological product, or device under this act.

(3) This act does not require any governmental agency to pay costs associated with the use, care, or treatment of a patient with an individualized investigational drug, biological product, or device.

(4) This act does not require a hospital or facility licensed under part of the public health code, to provide new or additional services, unless approved by the hospital or facility.

SEC. 4.

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

SEC. 5.

A licensing board or disciplinary subcommittee shall not revoke, fail to renew, suspend, or take any action against a health care provider's license issued under article of the public health code, and based solely on the health care provider's recommendations to an eligible patient regarding access to or treatment with an individualized investigational drug, biological product, or device. An entity responsible for Medicare certification shall not take action against a health care provider's Medicare certification based solely on the health care provider's recommendation that a patient have access to an individualized investigational drug, biological product, or device.

SEC. 6.

An official, employee, or agent of this state shall not block or attempt to block an eligible patient's access to an individualized 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 section.

SEC. 7.

(1) This act does not create a private cause of action against a manufacturer of an individualized investigational drug, biological product, or device or against any other person or entity involved in the care of an eligible patient using the individualized investigational drug, biological product, or device for any harm done to the eligible patient resulting from the individualized investigational drug, biological product, or device, if the manufacturer or other person or entity is complying in good faith with the terms of this act and has exercised reasonable care.

(2) This act does not affect any mandatory health care coverage for participation in clinical trials under the insurance code of _____.

Remove the Secrecy from the U.S. Drug Approval Process (Federal Amendment)

Amendment to the FOIA Improvement Act of 2016 / 5 U.S.C. 552:

6(a)(iii) If an agency does not make a determination and notify a requester within the timeframes set out in subsections 6(a)(I) or 6(b)(I), The agency shall be deemed to have waived any exemptions set forth in subsection (b) and shall promptly disclose responsive records in any action subsequently filed in district court.

Empower Patients with Information About Access to Treatments (Federal Amendment)

Amendment to Section 113(3)(A) of the Food and Drug Modernization Act (FDMA) of 1997 / 42 U.S.C. 282 (i)(3)(A):

"(3) The data bank shall include the following:

"(a) A registry of clinical trials (whether federally or privately funded) of experimental treatments for serious or life threatening diseases and conditions under regulations promulgated pursuant to section 505(i) of the Federal Food, Drug, and Cosmetic Act, which provides a description of the purpose of each experimental drug, either with the consent of the protocol sponsor, or when a trial to test effectiveness begins. Information provided shall consist of eligibility criteria for participation in the clinical trials, a description of the location of trial sites, and a point of contact for those wanting to enroll in the trial, and shall be in a form that can be readily understood by members of the public. A point of contact for a Right to Try program may be included, but is not required. Such information shall be forwarded to the data bank by the sponsor of the trial not later than 21 days after the approval of the protocol.

"(b) Information pertaining to experimental treatments for serious or life-threatening diseases and conditions that may be available— '

'(i) under a treatment investigational new drug application that has been submitted to the Secretary under section 561(c) of the Federal Food, Drug, and Cosmetic Act; or

"(ii) as a Group C cancer drug (as defined by the National Cancer Institute). The data bank may also include information pertaining to the results of clinical trials of such treatments, with the consent of the sponsor, including information concerning potential toxicities or adverse effects associated with the use or administration of such experimental treatments."

Ensure Doctors Have Information About Cutting-Edge Treatments (Truth in Medicine Act)

SEC. 1.

As used in this act, and unless the context otherwise requires:

(1). "Off-label" means the use of an United States Food and Drug Administration-approved drug, biological product, or device other than the use(s) approved by the FDA.

(2). "Misbranding" shall refer to either the federal definition under 21 U.S.C. § 352 or the state definition under [STATE LAW].

SEC. 2

(1). A pharmaceutical manufacturer or its representatives may engage in truthful promotion of off-label uses.

(2). This article does not require a health insurance carrier, other third-party payer, or other health plan sponsor to provide coverage for the cost of any off-label treatment. A health insurance carrier, other third-party payer or other health plan sponsor may provide coverage for an off-label treatment.

SEC. 3.

(1). Notwithstanding any other law, no official, employee or agent of this state shall enforce or apply [STATE LAW] against or otherwise prosecute a pharmaceutical manufacturer or its representatives for engaging in truthful promotion of off-label uses.

(2). Notwithstanding any other law, no state regulatory board may revoke, fail to renew or take any other action against a pharmaceutical manufacturer's or representative's, health care institution's, or physician's license solely for engaging in truthful promotion of off-label uses.

SEC. 4.

This state and all political subdivisions of this state are prohibited from using any personnel or financial resources to enforce or cooperate with federal attempts to enforce or apply 21 U.S.C. §§ 331 or 352 against or otherwise prosecute a pharmaceutical manufacturer or its representatives solely for engaging in truthful promotion of off-label uses.

Bring the World's Leading Medical Treatments to Americans (RESULT Act of 2019)

To amend the Federal Food, Drug, and Cosmetic Act to provide for reciprocal marketing approval of certain drugs, biological products, and devices that are authorized to be lawfully marketed abroad, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SEC 1. SHORT TITLE.

This Act may be cited as the "Reciprocity Ensures Streamlined Use of Lifesaving Treatments Act of 2019."

SEC. 2. RECIPROCAL MARKETING APPROVAL FOR CERTAIN DRUGS, BIOLOGICAL PRODUCTS, AND DEVICES.

The Federal Food, Drug, and Cosmetic Act is amended by inserting after section 524A of such Act (21 U.S.C. 360n–1) the following:

"SEC. 524B. RECIPROCAL MARKETING APPROVAL.

"(1) In General.—A covered product with reciprocal marketing approval in effect under this section is deemed to be subject to an application or premarket notification for which an approval or clearance is in effect under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable.

"(2) Eligibility.—The Secretary shall, with respect to a covered product, grant reciprocal marketing approval if—

"(a) the sponsor of the covered product submits a request for reciprocal marketing approval; and

"(b) the request demonstrates to the Secretary's satisfaction that—

"(i) the covered product is authorized to be lawfully marketed in one or more of the countries included in the list under section 802(b)(1);

"(ii) absent reciprocal marketing approval, the covered product is not approved or cleared for marketing, as described in subsection (a);

"(iii) the Secretary has not, because of any concern relating to the safety or effectiveness of the covered product, rescinded or withdrawn any such approval or clearance;

"(iv) the authorization to market the covered product in one or more of the countries included in the list under section 802(b)(1) has not, because of any concern relating to the safety or effectiveness of the covered product, been rescinded or withdrawn;

"(v) the covered product is not a banned device under section 516; and

"(vi) there is a public health or unmet medical need for the covered product in the United States.

"(3) Safety And Effectiveness.—

"(a) IN GENERAL.—The Secretary—

"(i) may decline to grant reciprocal marketing approval under this section with respect to a covered product if the Secretary affirmatively determines that the covered product—

"(A) is a drug that is not safe and effective; or

"(B) is a device for which there is no reasonable assurance of safety and effectiveness; and

"(ii) may condition reciprocal marketing approval under this section on the conduct of specified postmarket studies, which may include such studies pursuant to a risk evaluation and mitigation strategy under section 505–1.

"(b) REPORT TO CONGRESS.—Upon declining to grant reciprocal marketing approval under this section with respect to a covered product, the Secretary shall—

"(i) include the denial in a list of such denials for each month; and

"(ii) not later than the end of the respective month, submit the list to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate.

"(4) Request.—A request for reciprocal marketing approval shall—

"(a) be in such form, be submitted in such manner, and contain such information as the Secretary deems necessary to determine whether the criteria listed in subsection (b)(2) of this law are met; and

"(b) include, with respect to each country included in the list under section 802(b)(1) where the covered product is authorized to be lawfully marketed, as described in subsection (b)(2) of this law (A), an English translation of the dossier issued by such country to authorize such marketing.

"(5) Timing.—The Secretary shall issue an order granting, or declining to grant, reciprocal marketing approval with respect to a covered product not later than 30 days after the Secretary's receipt of a request under subsection (b)(1) for the product. An order issued under this subsection shall take effect subject to Congressional disapproval under subsection (g) of this law.

"(6) Labeling; Device Classification.—During the 30-day period described in subsection (e) of this law—

"(a) the Secretary and the sponsor of the covered product shall expeditiously negotiate and finalize the form and content of the labeling for a covered product for which reciprocal marketing approval is to be granted; and

"(b) in the case of a device for which reciprocal marketing approval is to be granted, the Secretary shall—

"(i) classify the device pursuant to section 513; and

"(ii) determine whether, absent reciprocal marketing approval, the device would need to be cleared pursuant to section 510(k) or approved pursuant to section 515 to be lawfully marketed under this Act.

"(7) Congressional Disapproval Of FDA Orders.—

"(a) IN GENERAL.—A decision of the Secretary to decline to grant reciprocal marketing approval under this section shall not take effect if a joint resolution of disapproval of the decision is enacted.

"(b) PROCEDURE.—

"(i) IN GENERAL.—Subject to subparagraph (B), the procedures described in subsections (b) through (g) of section 802 of title 5, United States Code, shall apply to the consideration of a joint resolution under this subsection.

"(ii) TERMS.—For purposes of this subsection—

"(A) the reference to 'section 801(a)(1)' in section 802(b)(2)(A) of title 5, United States Code, shall be considered to refer to subsection (c)(2) of this law; and

"(B) the reference to 'section 801(a)(1)(A)' in section 802(e)(2) of title 5, United States Code, shall be considered to refer to subsection (c)(2) of this law.

"(iii) EFFECT OF CONGRESSIONAL DISAPPROVAL.—Reciprocal marketing approval under this section with respect to the applicable covered product shall take effect upon enactment of a joint resolution of disapproval under this subsection.

"(8) Applicability Of Relevant Provisions.—The provisions of this Act shall apply with respect to a covered product for which reciprocal marketing approval is in effect to the same extent and in the same manner as such provisions apply with respect to a product for which approval or clearance of an application or premarket notification under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable, is in effect.

"(9) Fees For Request.—For purposes of imposing fees under chapter VII, a request for reciprocal marketing approval under this section shall be treated as an application or premarket notification for approval or clearance under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable.

"(10) Outreach.—The Secretary shall conduct an outreach campaign to encourage the sponsors of covered products that are potentially eligible for reciprocal marketing approval to request such approval.

"(11) Covered Product Defined.—In this section, the term 'covered product' means a drug, biological product, or device."

Bring the World's Leading Medical Treatments to Americans (RESULTs for Coronavirus Patients Act of 2020)

To amend the Federal Food, Drug, and Cosmetic Act to provide for reciprocal marketing approval of certain drugs, biological products, and devices that are authorized to be lawfully marketed abroad, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SEC 1. SHORT TITLE.

This Act may be cited as the "The Reciprocity Ensures Streamlined Use of Lifesaving Treatments for Coronavirus Patients Act of 2020."

SEC. 2. RECIPROCAL MARKETING APPROVAL FOR CERTAIN DRUGS, BIOLOGICAL PRODUCTS, AND DEVICES.

The Federal Food, Drug, and Cosmetic Act is amended by inserting after section 524A of such Act (21 U.S.C. 360n–1) the following:

"SEC. 524B. RECIPROCAL MARKETING APPROVAL.

"(1) In General.—A covered product with reciprocal marketing approval in effect under this section is deemed to be subject to an application or premarket notification for which an approval or clearance is in effect under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable.

"(2) Eligibility.—The Secretary shall, with respect to a covered product, grant reciprocal marketing approval if—

"(a) the sponsor of the covered product submits a request for reciprocal marketing approval; and

"(b) the request demonstrates to the Secretary's satisfaction that—

"(i) the covered product is authorized to be lawfully marketed in one or more of the countries included in the list under section 802(b)(1) for the treatment or prevention the coronavirus or another disease of epidemic potential;

"(ii) absent reciprocal marketing approval, the covered product is not approved or cleared for marketing, as described in subsection (a) of this law;

"(iii) the Secretary has not, because of any concern relating to the safety or effectiveness of the covered product, rescinded or withdrawn any such approval or clearance;

"(iv) the authorization to market the covered product in one or more of the countries included in the list under section 802(b)(1) has not, because of any concern relating to the safety or effectiveness of the covered product, been rescinded or withdrawn;

"(v) the covered product is not a banned device under section 516; and

"(vi) there is a public health or unmet medical need for the covered product in the United States.

"(3) Safety and Effectiveness.—

"(a) In general.—The Secretary—

"(i) may decline to grant reciprocal marketing approval under this section with respect to a covered product if the Secretary affirmatively determines that the covered product—

"(A) is a drug that is not safe and effective; or

"(B) is a device for which there is no reasonable assurance of safety and effectiveness; and

"(ii) may condition reciprocal marketing approval under this section on the conduct of specified postmarket studies, which may include such studies pursuant to a risk evaluation and mitigation strategy under section 505–1.

"(b) Report to congress.—Upon declining to grant reciprocal marketing approval under this section with respect to a covered product, the Secretary shall—

"(i) include the denial in a list of such denials for each month; and

"(ii) not later than the end of the respective month, submit the list to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate.

"(4) Request.—A request for reciprocal marketing approval shall—

"(a) be in such form, be submitted in such manner, and contain such information as the Secretary deems necessary to determine whether the criteria listed in subsection (b)(2) of this law are met; and

"(b) include, with respect to each country included in the list under section 802(b)(1) of this law where the covered product is authorized to be lawfully marketed, as described in subsection (b)(2)(A) of this law, an English translation of the dossier issued by such country to authorize such marketing.

"(5) Timing.—The Secretary shall issue an order granting, or declining to grant, reciprocal marketing approval with respect to a covered product not later than 30 days after the Secretary's receipt of a request under subsection (b)(1) of this law for the product. An order issued under this subsection shall take effect subject to Congressional disapproval under subsection (g) of this law.

"(6) Labeling; Device Classification.—During the 30-day period described in subsection (e) of this law—

"(a) the Secretary and the sponsor of the covered product shall expeditiously negotiate and finalize the form and content of the labeling for a covered product for which reciprocal marketing approval is to be granted; and

"(b) in the case of a device for which reciprocal marketing approval is to be granted, the Secretary shall—

"(i) classify the device pursuant to section 513; and

"(ii) determine whether, absent reciprocal marketing approval, the device would need to be cleared pursuant to section 510(k) or approved pursuant to section 515 to be lawfully marketed under this Act.

"(7) Congressional Disapproval of FDA Orders.—

"(a) In general.—A decision of the Secretary to decline to grant reciprocal marketing approval under this section shall not take effect if a joint resolution of disapproval of the decision is enacted.

"(b) Procedure.—

"(i) In general.—Subject to subparagraph (B), the procedures described in subsections (b) through (g) of section 802 of title 5, United States Code, shall apply to the consideration of a joint resolution under this subsection.

"(ii) Terms.—For purposes of this subsection—

"(A) the reference to 'section 801(a)(1)' in section 802(b)(2)(A) of title 5, United States Code, shall be considered to refer to subsection (c)(2) of this law; and

"(B) the reference to 'section 801(a)(1)(A)' in section 802(e)(2) of title 5, United States Code, shall be considered to refer to subsection (c)(2) of this law.

"(c) Effect of congressional disapproval.—Reciprocal marketing approval under this section with respect to the applicable covered product shall take effect upon enactment of a joint resolution of disapproval under this subsection.

"(8) Applicability of Relevant Provisions.—The provisions of this Act shall apply with respect to a covered product for which reciprocal marketing approval is in effect to the same extent and in the same manner as such provisions apply with respect to a product for which approval or clearance of an application or premarket notification under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable, is in effect.

"(9) Fees for Request.—For purposes of imposing fees under chapter VII, a request for reciprocal marketing approval under this section shall be treated as an application or premarket notification for approval or clearance under section 505(c), 510(k), or 515 of this Act or section 351(a) of the Public Health Service Act, as applicable.

"(10) Outreach.—The Secretary shall conduct an outreach campaign to encourage the sponsors of covered products that are potentially eligible for reciprocal marketing approval to request such approval.

"(11) Definitions.—In this section—

"(a) the term 'coronavirus' means SARS-CoV-2, COVID-19, or another coronavirus with epidemic potential; and

"(b) the term 'covered product' means a drug, biological product, or device that is intended to treat or prevent the coronavirus or another disease with epidemic potential."

End “Expert Panels” That Keep Treatments from the Most Vulnerable (Federal Amendment)

Amendment of Ch. 5 of the Federal Food, Drug, and Cosmetic Act, Section 505-1(a)(1) / 21 U.S.C. 355-1(a)(1):

(G) The FDA shall be prohibited from developing or employing a dollars-per-quality adjusted life year, or similar measure that discounts the value of a life because of an individual’s disability, including age or chronic illness, as a threshold to inform drug approval.

Allow States to Approve Tests and Protective Equipment (Right To Test Act)

To allow States to approve the use of diagnostic tests during a public health emergency.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Right to Test Act".

SEC. 2. STATE APPROVAL OF DIAGNOSTIC TESTS.

(1) In General.—Notwithstanding chapter V of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 351 et seq.) and section 353 of the Public Health Service Act (42 U.S.C. 263a), during any public health emergency declared by the Secretary of Health and Human Services (referred to in this section as the "Secretary") under section 319 of the Public Health Service Act (42 U.S.C. 247d) or by a State in accordance with the law of the State, the public health department of such State (or such other State entity as designated by the governor of the State) may clear or approve diagnostic tests or diagnostic devices for use in that State during the applicable public health emergency only.

(2) Application.—An approval or clearance pursuant to subsection (a) shall—

- (a) allow for the preparation, compounding, assembly, propagation, manufacture, development, sale, distribution, or use of a specified diagnostic test or diagnostic device to address the health diagnostic needs of the State during the public health emergency;
- (b) apply to a diagnostic test or diagnostic device needed to address the health diagnostic needs of the State during the public health emergency, as determined by the State, including, but not limited to, a test or device that uses reagents or swabbing (including self-swab);
- (c) apply to the testing of patients if the State certifies that the test can be validated, as determined by the State; and
- (d) apply to laboratory-developed tests performed by laboratories and hospitals certified under section 353 of the Public Health Service Act (42 U.S.C. 263a), and to such tests performed by clinical laboratory companies.

(3) Suspension of Enforcement By FDA.—

- (a) IN GENERAL.—Except as provided in paragraph (1), with respect to a diagnostic test or diagnostic device approved or cleared by a State pursuant to subsection (a), the Secretary may not, for the duration of the applicable public health emergency, engage in any enforcement action—
  - (i) with respect to the test or device, to the extent that such test or device is distributed and used within the State granting the approval or clearance in accordance with the requirements of the State;
  - (ii) against a State or State entity that clears or approves the test or device in accordance with this section; or
  - (iii) against any State, entity of a State, health care provider, health care facility, laboratory, educational institution, manufacturer, or distributor that prepares, propagates, compounds, assembles, or processes a diagnostic test

or diagnostic device by chemical, physical, biological, or other procedure for such test or device or develops, manufactures, distributes, sells, administers, or evaluates such test—

(A) within the applicable State in accordance with the requirements of the State; or

(B) for the applicable State or individuals or entities that are located within the applicable State.

(b) EXCEPTION.—The provisions of paragraph (1) shall not apply with respect to a State if the governor of the State requests that enforcement continue in the State during the public health emergency.

(4) Action By FDA After Public Health Emergency.—Not later than 180 days after the end of any public health emergency under which a State exercises its authority under subsection (a) with respect to a diagnostic test or diagnostic device, if the Food and Drug Administration has not cleared or approved such test or device under chapter V of the Federal Food, Drug, and Cosmetic Act, the Secretary shall review and make a final determination, within such 180-day period, with respect to such test or device for clearance or approval.

(5) Diagnostic Tests And Diagnostic Devices.—In this section, the terms "diagnostic test" and "diagnostic device" include in vitro diagnostic products, laboratory developed tests, viral tests, serological and antibody tests, and any other test used to identify, analyze, or investigate a disease.

Restrain FDA Overreach that Prevents Compounding Pharmacies from Serving Patients (Preserving Patient Access to Compounded Medications Act of 2019)

To amend the Federal Food, Drug, and Cosmetic Act with respect to compounding pharmacies, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SEC 1. SHORT TITLE.

This Act may be cited as the "Preserving Patient Access to Compounded Medications Act of 2019."

SEC. 2. OFFICE-USE COMPOUNDING WHEN AUTHORIZED BY STATE LAW.

Section 503A(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 353a(a)) is amended—

(1) in the matter preceding paragraph (1), by inserting "or drug order for administration to a patient in an office or clinical setting" after "is necessary for the identified patient";
(2) in paragraph (1), by striking "or" at the end;
(3) in paragraph (2), by striking the period at the end and inserting " or"; and
(4) by adding at the end the following new paragraph:

"(a) is by a licensed pharmacist or licensed physician pursuant to a valid prescription order or drug order and the compounded drug is distributed or dispensed to a licensed prescriber in accordance with State law, for administration to a patient in an office or clinical setting."

SEC. 3. UNITED STATES PHARMACOPOEIA OR NATIONAL FORMULARY MONOGRAPH REQUIREMENT.

Section 503A(b)(1)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 353A(b)(1)(A)) is amended—

(1) in the matter preceding subclause (i), by inserting ", or dietary supplements" after "Regulations"; and
(2) in clause (i)—

(a) by amending subclause (I) to read as follows:

"(I) comply with the monograph standards in any section of the United States Pharmacopoeia or National Formulary, including drug substance or dietary supplement monograph, if a monograph exists."; and

(b) by amending subclause (III) to read as follows:

"(III) if such monograph does not exist and the drug substance or dietary supplement is not a component of a drug approved by the Secretary, but appears on a list developed by the Secretary through regulations issued by the Secretary under subsection (c) of this section;".

SEC. 4. DEFINITIONS.

Subsection (e) of section 503A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 353a) is amended to read as follows:

"(1) Definitions.—In this section:

"(a) COMPOUNDING.—The term 'compounding' does not include mixing, reconstituting, or other such acts that are performed in accordance with directions contained in approved labeling provided by the product's manufacturer and other manufacturer directions consistent with that labeling.

"(b) DISTRIBUTE OR DISTRIBUTION.—The terms 'distribute' or 'distribution' do not include the act of dispensing of a compounded drug product in accordance with this section.

"(c) DISPENSE.—The term 'dispense' means for a drug product compounded in accordance with this section, the act of the drug product leaving the facility in which it was compounded for delivery to a patient, patient's agent, or health care facility (including a hospital, physician's office, or other health care setting) pursuant to a valid prescription order for an identified patient."

SEC. 5. APPLICABILITY OF RECORDS EXEMPTION FOR COMPOUNDING PHARMACIES.

(1) In General.—Section 704(a)(2)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 374(a)(2)(A)) is amended to read as follows:

"(a) pharmacies which maintain establishments in conformance with any applicable local laws regulating the practice of pharmacy and medicine and, for compounding pharmacies, the provisions of section 503A, and which are regularly engaged in dispensing or distributing prescription drugs or devices, upon prescriptions or drug orders of practitioners licensed to administer such drugs or devices to patients under the care of such practitioners in the course of their professional practice, and which do not, either through a subsidiary or otherwise, manufacture, prepare, propagate, compound, or process drugs or devices for sale other than in the regular course of their business;".

(2) Registration Exemption.—Section 510(g)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360(g)(1)) is amended to read as follows:

"(a) pharmacies which maintain establishments in conformance with any applicable local laws regulating the practice of pharmacy and medicine and, for compounding pharmacies, the provisions of section 503A, and which are regularly engaged in dispensing or distributing prescription drugs or devices, upon prescriptions or drug orders of practitioners licensed to administer such drugs or devices to patients under the care of such practitioners in the course of their professional practice, and which do not manufacture, prepare, propagate, compound, or process drugs or devices for sale other than in the regular course of their business;".

SEC. 6. REGULATIONS.

(1) Rules Implementing New Requirements.—Not later than 90 days after the date of enactment of this Act, the Secretary of Health and Human Services shall promulgate rules on the record to carry out the amendments made by this Act, in accordance with chapter 5 of title 5, United States Code.

(2) Other Rules.—The Secretary of Health and Human Services shall promulgate rules on the record to carry out any of the provisions of section 503A of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 353a) other than those amended by this Act, in accordance with chapter 5 of title 5, United States Code.

Open the Door for Stem Cell Treatments and Regenerative Medicine (Regulation)

Amend 21 C.F.R. 1271.10(a) to read as follows:

(a) An HCT/P is regulated solely under section 361 of the PHS Act and the regulations in this part if it meets all of the following criteria:

(1) The HCT/P is minimally manipulated "FOR A NONHOMOLOGOUS USE; OR MORE-THAN MINIMALLY MANIPULATED FOR A HOMOLOGOUS OR NONHOMOLOGOUS USE, BUT ARE NOT GENETICALLY MODIFIED"

(2) The HCT/P is intended for A NONHOMOLOGOUS USE; OR MORE-THAN MINIMALLY MANIPULATED FOR A HOMOLOGOUS OR NONHOMOLOGOUS USE, BUT ARE NOT GENETICALLY MODIFIED, as reflected by the labeling, advertising, or other indications of the manufacturer's objective intent;

(3) The manufacture of the HCT/P does not involve the combination of the cells or tissues with another article, except for water, crystalloids, or a sterilizing, preserving, or storage agent, provided that the addition of water, crystalloids, or the sterilizing, preserving, or storage agent does not raise new clinical safety concerns with respect to the HCT/P; and

(4) Either:

(i) The HCT/P does not have a systemic effect and is not dependent upon the metabolic activity of living cells for its primary function; or

(ii) The HCT/P has a systemic effect or is dependent upon the metabolic activity of living cells for its primary function, and:

(a) Is for autologous use;

(b) Is for allogeneic use in a first-degree or second-degree blood relative; or

(c) Is for reproductive use.

(b) If you are a domestic or foreign establishment that manufactures an HCT/P described in paragraph (a) of this section:

(1) You must register with FDA;

(2) You must submit to FDA a list of each HCT/P manufactured; and

(3) You must comply with the other requirements contained in this part.

GOLDWATER INSTITUTE