



**STATEMENT
OF
ALISON BATEMAN-HOUSE, PHD, MPH, MA
ASSISTANT PROFESSOR, DIVISION OF MEDICAL ETHICS
DEPARTMENT OF POPULATION HEALTH, NYU LANGONE HEALTH
BEFORE THE
HEALTH SUBCOMMITTEE OF THE
COMMITTEE ON ENERGY AND COMMERCE
U.S. HOUSE OF REPRESENTATIVES
“EXAMINING PATIENT ACCESS TO INVESTIGATIONAL DRUGS”
OCTOBER 3, 2017**



Mr. Chairman, Ranking Member Green, and Members of the Health Subcommittee, I am Dr. Alison Bateman-House, an assistant professor of medical ethics at NYU Langone Health. Thank you for the opportunity to be here today to discuss the various “Right to Try” proposals that have been introduced in the House of Representatives.

I co-chair the Working Group on Compassionate Use and Pre-Approval Access. This group is composed of patient advocates, members of the pharmaceutical industry, individuals with clinical trial and compassionate use experience, bioethicists, lawyers, venture capitalists, and individuals with experience at the FDA and the Reagan-Udall Foundation for the FDA. The Working Group was formed before the Right to Try movement began, and there has been no litmus test of any sort, on Right to Try or any other topic, that members had to pass. And yet, every member of the group opposes Right to Try on ethical, legal, and pragmatic grounds.

The Working Group was founded in the aftermath of Josh Hardy’s quest to gain access to brincidofovir.¹ That case and others that made public headlines indicated that there was dissatisfaction with the existing system for accessing investigational medicines outside of clinical trials. The group was founded with a specific mission: to study access to investigational drugs outside of clinical trials from the vantage point of all stakeholders; to identify problems; and to propose solutions.

We have identified many concerns with the current system and have proposed several ways to address them. I will review some these briefly, but before I go any further, I want to make three points very clear. First, after more than three years of studying all facets of compassionate use/pre-approval access, including Right to Try, the Working Group has found that the FDA’s expanded access program has been doing an excellent job of

¹ Elizabeth Cohen, “Company denies drug to dying child,” *CNN*, March 11, 2014.



helping patients obtain access to experimental drugs. Second, no piece of Right to Try legislation, either on the state or federal level, addressed the concerns the Working Group has identified.

So, what issues have we found? First, there's a widespread lack of knowledge of the FDA's expanded access program. Consistently, in speaking with patients, doctors, reporters, and even personnel in the pharmaceutical industry, people have not known that it is possible to access investigational drugs outside of clinical trials, much less how to do so. Patient advocacy groups tend to be more knowledgeable about expanded access, but there is a vast range in knowledge when you move from large organizations like the American Cancer Society and the National Organization for Rare Diseases to smaller, more regional or community-based organizations. The Working Group has tried to address this dearth of knowledge by hosting webinars, publishing and speaking extensively, and partnering with patient organizations for events like "Ask an Expert" sessions, but our small volunteer group is obviously unable to fill a national educational gap. We have therefore called for the FDA to be more proactive in educating industry, doctors, patient advocacy organizations, and patients about this potentially very beneficial resource that it offers. We're happy to report that they've listened: Over the last 2 years the FDA has made its website more user-friendly and has introduced a new, much shorter application form for compassionate use requests for single patients. So while we applaud the agency's process, we also call on it to be more proactive in informing stakeholders about it. However, the responsibility for this education should not rest solely on the FDA. Doctors' and nurses' organizations need to step up in educating their members about expanded access. Likewise, pharmaceutical trade associations should continue their recent efforts to make sure their members understand all aspects of expanded access.

Another, especially troubling issue we have identified is that of rampant inaccurate, even mythological, beliefs about compassionate use. Some patients believe the FDA can force drug companies to make their investigational products available. This is not true. The FDA can merely approve a request to proceed; if the



company says no, there is no higher power to which a patient can appeal. Another widespread myth is that the FDA is slow in handling requests for compassionate use. This is untrue. Another myth, promulgated by Right to Try legislation's focus on shielding all involved from legal liability prosecution, is that engaging in expanded access may expose companies to legal risks. Our research has found no instances in which a drug company, doctor, or hospital was sued with regard to expanded access; a recent journal article also found no such lawsuits.² A particularly pernicious myth is that if a company provides its drugs outside of clinical trials, and a patient has a serious problem or dies, then the drug's eventual FDA approval will be threatened, if not ruined. After spending years and an enormous sum of money developing a new drug, it would be understandable if a company were to think that providing its drugs via compassionate use is simply too much of a business risk. But the FDA has studied this extensively over the past few years, and it has found *zero* instances in which a compassionate use drug that was linked to a death or serious problem was rejected based on that incident. It also has found *zero* instances in which a drug that was linked to a death or serious problem was ordered by the FDA to undergo additional clinical studies. Furthermore, the FDA has found *zero* instances in which a drug that was tied to a death or serious problem ended up with more restrictive labeling based on that incident. The FDA found only 2 instances in 10 years in which development of a drug was paused due to a death or serious problem in a compassionate use patient, a minuscule number.

Because these myths are persistent, widespread, and may well be leading companies, doctors, or hospitals to turn down patient requests for compassionate use, they must be dealt with head-on.

Another issue that the Working Group has identified is patient, family, and advocate frustration over not

² Amy E. McKee, Andre O. Markon, Kirk M. Chan-Tack, and Peter Lurie, "How Often Are Drugs Made Available Under the Food and Drug Administration's Expanded Access Process Approved?" *Journal of Clinical Pharmacology* (2017), 57(510): S136-S142.



knowing how to request an experimental drug from a company. Just stating the issue reveals another knowledge gap, as we don't know of a single company that will grant compassionate use access requests made by a patient: rather, the requests need to be made by a health care professional. So, as previously mentioned, health care professionals need training on this matter. But in the meantime, to help these professionals and all other interested parties, we have been advocating for companies to make public their access policies. We are very pleased that this provision was included in the 21st Century Cures Act, thanks to the work of Representative Michael McCaul and his colleagues, particularly Representative Upton and Representative Degette. We are, however, dismayed that the provision apparently has no enforcement mechanism and there is less than 100% compliance with the rule.

These are some of the problems that the Working Group has identified and for which we have proposed possible solutions. You will note that I have not mentioned Right to Try much, **because none of these issues are addressed in those laws**. Indeed, if I were to analyze the Right to Try laws, I would point out much that is misleading, unnecessary, confusing, vague, or downright harmful. I'm happy to do so during the Q&A, but now I'll simply quote a recent letter from 22 patient advocacy organizations that says, "Our organizations support patient access to unapproved therapies, but S.204 and H.R.878 do not effectuate policy changes that would afford our patients greater access to promising investigational therapies. Instead, these bills would likely do more harm than good."³ These are 22 groups whose sole reason for being is to help save the lives of patients.

I do want to point out that one huge way that Right to Try laws have already caused harm is through the confusion created by 37 different state laws that have been enacted. These vary from state to state on crucial matters: for example, while advocates of Right to Try often use children as examples of patients needing access to experimental drugs, 5 state right to try laws don't apply to those 18 and younger. In 19 states, patients using

³ To Rep. Greg Walden, 9/19/2017 (appended)



an investigational drug obtained via right to try can lose their hospice coverage, and 6 states say these patients may be denied coverage for home healthcare assistance. These laws apply to terminally ill patients—the very people who would naturally be dependent on hospice, home healthcare, and insurance. These are not humane, patient-centered provisions for people who are facing death. And the inconsistency in laws from state to state have real implications for patients who might travel across state lines seeking care, for healthcare institutions that operate in more than one state, and for patient advocacy groups who advise patients from a range of states.

Every Working Group member has witnessed the suffering of patients and their families when they are confronted with serious or life-threatening conditions and are out of FDA-approved treatment options. We understand why patients would want to try experimental drugs. For 3 decades, the FDA has had in place a system to help such patients gain access these drugs. Between 2010-2015, the agency has allowed more than 99 percent of the “compassionate use” requests it received from drug companies to proceed. And while such a large number of approvals may suggest that the FDA “rubber stamps” requests, this is far from the case. A recent study found that 11% of these requests had been modified after input from FDA experts, with regard to such issues as drug dosage or frequency of dosing.⁴ These modifications are made for the sole purpose of trying to improve the likelihood that these compassionate use drugs will help—and, importantly, not hurt—the patients using them. In short, the FDA’s expanded access program works, assisting patients who choose to try experimental drugs.

Of course, there will always be some requests for investigational drugs that companies will deny. In many cases this is reasonable. For instance, if the drug is available via a clinical trial in which the patient is eligible to participate, it is appropriate to tell the patient that they must obtain the drug via the trial, not

⁴ Jonathan P. Jarow, Peter Lurie, Sarah Crowley Ikenberry, Steven Lemery, “Overview of FDA’s Expanded Access Program for Investigational Drugs,” *Therapeutic Innovation & Regulatory Science* (2017), 51(2): 177-179.



compassionate use. If drug quantity is limited, it is appropriate for a company to deny a request. But it is essential that companies deny requests based on sound reasons, not on myths or unfounded fears.

The best, safest way to speed access to drugs in development to the largest number of patients is through the clinical trial process. Clinical trials are vital to the pharmaceutical industry's creation of safe and effective drugs, and once approval is secured for a new drug, all patients with the condition are able to receive it, thereby helping the largest number of patients. However, not all patients who are willing to participate in clinical trials are able to do so. In some cases, this is because no trials are available where they live, or patients are considered too young or old, or because they have something in their medical history that renders them ineligible to participate. The Working Group has called for an investigation into what can be done to make clinical trials available on an equitable basis for all patients, and we are gratified to see such a provision included in the recently enacted FDA User Fee Reauthorization Act. By expanding clinical trial access, you will reduce the number of patients forced to seek access to investigational drugs outside of clinical trials. And by educating all stakeholders, combatting myths, and continuing to review the current system for ways it can be even more streamlined, the Working Group is convinced that compassionate use will become more accessible, more transparent, and more patient-friendly.

CONCLUSION

Clinical trials remain the best option for patients wishing to gain access to investigational products, and bringing new, innovative products to market through the FDA approval process remains the best way to assure the development of and access to safe and effective new medical products for *all patients*. For those patients who cannot participate in trials and who have no other therapeutic options, the FDA's expanded access program



works. However, it faces challenges, especially a widespread lack of knowledge and confusion about the program. We need to fix this. But we do not need to undermine a working program that benefits patients by creating a deeply flawed alternative program that will only lead to further confusion and strip patients of crucial protections they currently have. Right to Try laws do not solve the problems the Working Group has identified in its years of research. And the laws not only fail to address these current problems, they will create additional, new problems. Instead of promoting Right to Try as a way to help patients, we need to focus on making the current expanded access system even better: letting people know what it is, how to use it, that they need to work with their doctor to request access, and so on. And finally, since the ultimate decision to grant access is up to individual companies, we need to work with these companies to find out why they deny requests and what, if any, policies would make them more likely to say yes.

Thank you. I look forward to your questions.

September 19, 2017

The Honorable Greg Walden, Chairman
2125 Rayburn House Office Building
U.S. House Committee on Energy and Commerce
Washington, DC 20515

The Honorable Frank Pallone, Ranking Member
2471 Rayburn House Office Building
U.S. House Committee on Energy and Commerce
Washington, DC 20515

Dear Chairman Walden and Ranking Member Pallone,

The undersigned organizations collectively represent millions of patients with serious and life-threatening diseases. We write to express our strong opposition to S.204, the *Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017*, as well as H.R.878, the *Right to Try Act of 2017*, currently under consideration in the House Energy and Commerce Committee. We urge the Committee to proceed through regular legislative order to facilitate discussion and consideration of alternative policies that would genuinely increase access to promising investigational therapies for the communities we represent.

Our organizations support patient access to unapproved therapies, but S.204 and H.R.878 do not effectuate policy changes that would afford our patients greater access to promising investigational therapies. Instead, these bills would likely do more harm than good. We encourage the Committee to hold hearings to examine these issues more closely, as well as consider other policy options to improve the ability of patients to safely access unapproved therapies.

We do not believe S.204 or H.R.878 would successfully increase access to promising investigational therapies for those in need. Both of these bills remove the Food and Drug Administration (FDA) from the initial approval process for accessing an investigational therapy outside of a clinical trial. Removing FDA from this process is not likely to facilitate increased access to investigational therapies because FDA currently approves 99.7 percent of all expanded access requests submitted by physicians and companies for patients with immediately life-threatening illnesses who cannot participate in clinical trials.¹ The Government Accountability Office (GAO) recently released a report examining the current FDA expanded access program, and found that substantial changes were not needed within the program, aside from greater clarity on the use of adverse event data.²

When access to a therapy is denied to a patient, it is generally the company that denies the request, and for reasons that appear to be reasonable, such as a determination that the benefits do not outweigh the risks, an unavailability of sufficient product to offer outside of clinical trials, costs, or concerns about adversely affecting clinical trial enrollment.

¹ Jarow, Jonathan P., et al. "Expanded access of investigational drugs: the experience of the Center of Drug Evaluation and Research over a 10-year period." *Therapeutic innovation & regulatory science* 50.6 (2016): 705-709.

² GAO, "FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used," July 2017.

It is important to remember that the current regulatory system for medical products and research in the United States was created as a result of serious patient harm and exploitation that occurred early in the 20th Century. Birth defects resulting from Thalidomide are an example of what happens when drugs are given to humans without proper safety review and approval. While obtaining unapproved therapies outside of a clinical trial is not about research, the products themselves remain experimental and have not been shown to be safe and effective. Clinical research subject protections are in place when experimental products are being tested to ensure the safe and ethical treatment of research participants. Patients seeking expanded access to unapproved therapies outside of clinical trials must be afforded the same ethical standards and protections as patients taking part in clinical trials.

Existing expanded access policies are not without room for improvement. We encourage the Committee to examine the predominant reasons why patients interested in access to experimental therapies are ultimately unable to obtain them by enrolling in clinical trials or through the current expanded access process. We also ask the Committee to provide oversight as FDA moves forward with implementation of relevant provisions enacted within the past year that improve the expanded access system. These include the requirements within the *21st Century Cures Act* for the public posting of expanded access policies on company websites, and greater clarity from FDA on the use of adverse event data. Several provisions in the *Food and Drug Administration Reauthorization Act (FDARA)* will also improve access to investigational therapies, such as the allowance for IRBs to appoint one individual to review applications rather than a fully convened IRB. FDARA also directs FDA to further investigate inclusion/exclusion criteria within clinical trials, a key factor in the number of individuals able to access investigational therapies.

We are eager to work with the Committee as it considers these proposals, and endeavors to ensure patients gain greater access to investigational therapies. We welcome the opportunity to work with members of the Committee, as well as the sponsors of this legislation, to improve and increase access to both approved and unapproved innovative, lifesaving therapies.

Sincerely,

Alliance for Aging Research
American Cancer Society Cancer Action Network
American Lung Association
American Society of Clinical Oncology
Association of Pediatric Hematology/Oncology Nurses
Cancer Support Community
Children's Brain Tumor Foundation
Children's Cause for Cancer Advocacy
Cystic Fibrosis Foundation
Fight Colorectal Cancer
Friedreich's Ataxia Research Alliance
Friends of Cancer Research
Grandparents in Action
Leukemia & Lymphoma Society
Lung Cancer Alliance
LUNGeVity Foundation

Max Cure Foundation, Inc.
National Comprehensive Care Network
National Health Council
National Organization for Rare Disorders (NORD)
TargetCancer Foundation
United Mitochondrial Disease Foundation

CC: The Honorable Paul Ryan, Speaker
The Honorable Kevin McCarthy, Majority Leader
The Honorable Nancy Pelosi, Minority Leader
The Honorable Steny Hoyer, Minority Whip

September 5, 2017

Dear Members of the United States House of Representatives:

The undersigned groups respectfully urge you to oppose S. 204 — which is deceptively titled the “Right to Try Act of 2017” but should instead be called the “False Hope Act of 2017.”

We recognize the desire of patients with terminal illness who have exhausted available treatment options to access experimental medical products that have not been approved or cleared by the Food and Drug Administration (FDA). However, the best way for patients to gain such access is through the FDA’s Expanded Access Program, which allows seriously ill patients to receive treatment with experimental medical products while also providing basic safeguards to protect patients’ rights and welfare. Importantly, the recently enacted FDA Reauthorization Act of 2017, which renewed the FDA’s user fee programs, included responsible bipartisan language intended to enhance the agency’s Expanded Access Program.

We are concerned that S. 204, as amended and passed by the U.S. Senate on August 3, 2017, would put countless patients at risk by undermining important FDA safety rules related to the use and oversight of unapproved, experimental medications. Such legislation would expose vulnerable patients to risks of serious harm, including dying earlier and more painfully than they otherwise would have, without appropriate safeguards.

FDA’s Current Expanded Access Program

Currently, the FDA oversees the use of all experimental drugs and biological products in the U.S. The FDA’s Expanded Access Program allows patients across the country to gain access to such products, provided that each patient’s doctor believes such access is appropriate and that the manufacturer of the product agrees to provide it for that use.

To protect patients, the FDA and an institutional review board (IRB) must approve each use of an experimental drug or biological product under the Expanded Access Program. As conditions of approval, there must be sufficient evidence of the safety and effectiveness of the experimental drug to support its use in a particular patient, and the probable risk to the patient from the drug must not be greater than the probable risk from the disease or condition. The program further protects patients by requiring a robust informed consent process that is similar to the consent process for a clinical trial, as well as monitoring and reporting of serious adverse events. The FDA grants 99 percent of all Expanded Access Program requests and, in urgent circumstances, can respond to such requests within one or two days. The agency also recently streamlined the program to require less paperwork. In addition, the 21st Century Cures Act of 2016 included useful provisions that require drug manufacturers to publicly post their expanded access policies and provide points of contact for requests. The potential impact of these streamlining efforts has yet to be fully realized.

It is also important to recognize that many of the experimental products made available through this program ultimately are not shown to be safe and effective in clinical testing and are not approved or cleared by the FDA.

Undermining Patient Protections While Offering False Hope

The false-hope legislation passed by the Senate and now being considered by the House would create a dangerous, uncharted pathway for access to experimental drugs and biological products that essentially bypasses the protections of the FDA's Expanded Access Program for patients diagnosed with life-threatening diseases or conditions — a patient population that is much broader than “patients diagnosed with a terminal illness,” which was the patient population covered by the original version of S. 204.

Of particular concern, this alternative pathway for accessing experimental drugs and biological products would put vulnerable patients at risk and undermine their rights by:

- Specifying completion of a single phase I clinical trial as the evidentiary threshold for allowing use of experimental drug products under the legislation. Such a threshold is insufficient for allowing use of an experimental drug outside the context of a clinical trial because initial phase I clinical trials often only involve healthy volunteers, typically involve testing of a single dose of an experimental drug, provide no meaningful data on efficacy, and yield only very limited preliminary data on safety.
- Eliminating the requirements for review and approval by the FDA and an IRB, which help to ensure that proposed uses of experimental drugs do not pose unacceptable risk to patients and that the patients are fully informed of the risks and other key information when their consent is sought.
- Eliminating the requirements that (a) the consent of the patient be sought only under circumstances that provide the patient with sufficient opportunity to consider whether or not to participate and that minimize the possibility of coercion or undue influence; (b) the information given to the patient when consent is sought be understandable to the patient; and (c) the consent process exclude exculpatory language through which the patient is made to waive or appear to waive any of his or her legal rights, or releases or appears to release the investigator, the sponsor, the institution, or its agents from liability for negligence.
- Broadly immunizing sponsors, manufacturers, prescribers, and dispensers from liability for any alleged acts or omissions related to eligible experimental drugs, unless the relevant conduct constitutes reckless or willful misconduct, gross negligence, or an intentional tort under applicable state law. This provision would bar suits in a variety of situations in which state law might reasonably impose liability. For example, it would immunize manufacturers from being held accountable for harm caused by contamination of an investigational drug product, which can be serious. It also would bar state-law negligence suits against the physician prescribers; for example, if the physician negligently prescribed an investigational drug that was known to be contraindicated for a particular patient's set of circumstances, but the situation did not arise to “gross negligence.” Decisions about liability in such situations are properly based on consideration of the specific facts, and the bill's immunity provision may cause physicians to be less careful in making prescribing decisions for seriously ill patients.
- Eliminating the requirement that the treating physician report immediately to the manufacturer or sponsor any serious adverse events regardless of whether they are considered drug-related.

In closing, we urge you to oppose S. 204 and any similar false-hope legislation that is introduced in the future. Thank you for considering our views on this important matter.

Sincerely,

Public Citizen
ACTUP New York
Breast Cancer Action
Doctors For America
END AIDS NOW
Government Accountability Project
Health GAP
Jacobs Institute of Women's Health
MedShadow Foundation
National Consumers League
National Physicians Alliance
National Women's Health Network
Richard N. Gottfried, Chair, Committee on Health, New York State Assembly
Social Security Works
The Annie Appleseed Project
The Society for Patient Centered Orthopedics
Treatment Action Group
Washington Advocates for Patient Safety



1600 20th Street, NW • Washington, D.C. 20009 • 202/588-1000 • www.citizen.org

March 6, 2017

Dear Members of the United States Senate and House of Representatives:

Public Citizen, a consumer advocacy organization with more than 400,000 members and supporters nationwide, respectfully urges you to oppose S. 204, H.R. 878 and H.R. 1020, bills with various names that would most appropriately each be titled the “False Hope Act of 2017.”

These bills provide false hope to patients and are related to a nationwide lobbying effort funded by the Goldwater Institute, which has deceptively branded such laws as “Right to Try” legislation.

We recognize the desire of patients with terminal illness who have exhausted available treatment options to access experimental medical products that have not been approved or cleared by the Food and Drug Administration (FDA). However, the best way for patients to gain such access is through the FDA’s Expanded Access Program, which allows seriously ill patients to receive treatment with experimental drugs, biological products or medical devices while also providing basic safeguards to protect patients’ rights and welfare and maintaining strong incentives for careful clinical testing and timely product development.

We are concerned that false hope legislation like S. 204, H.R. 878 and H.R. 1020 would put countless patients at risk by dramatically undermining the FDA’s role in ensuring that medical products are safe and effective before they become widely used. Such legislation would expose vulnerable patients to risks of serious harm, including dying earlier and more painfully than they otherwise would have, without appropriate safeguards. It also would undermine incentives for companies to swiftly develop life-saving products for FDA approval and impair review of these products by limiting the agency’s access to unfavorable information.

FDA’s Current Expanded Access Program

The FDA’s Expanded Access Program allows patients across the country to gain access to experimental drugs, biological products and medical devices, provided that each patient’s doctor believes such access is appropriate and the manufacturer of the product agrees to provide it for that use. The program protects patients by requiring informed consent, ethical review by an institutional review board, safety monitoring and the reporting of adverse events to the FDA. It also prevents manufacturers from profiting from the use of experimental products, which helps to maintain incentives to continue rigorous clinical testing aimed at FDA approval.

The FDA grants 99 percent of all Expanded Access Program requests and, in urgent circumstances, can respond to such requests within 1 or 2 days. The agency also recently streamlined the program to require less paperwork. In addition, the 21st Century Cures Act of 2016 included useful provisions that require drug manufacturers to publicly post their expanded access policies and provide points of contact for requests. The potential impact of these streamlining efforts has yet to be fully realized.

It is also important to recognize that many of the experimental products made available through this program ultimately are not shown to be safe and effective in clinical testing and are not approved or cleared by the FDA. Despite patients’ hopes, there is no evidence that the current Expanded Access Program helps more patients than it harms.

Broadly Attacking Patient Protections While Offering False Hope

Rather than proposing further improvements to the existing program, the false hope legislation now before Congress would undermine the FDA's fundamental authority to oversee the use of experimental medical products and to ensure they are safe and effective before they become widely used.

The legislation would put vulnerable patients at risk by:

- Offering manufacturers broad rights to sell experimental medical products after only very preliminary clinical testing, when very little is known about a product's potential risks, let alone its benefits.
- Eliminating important federal safeguards intended to protect the rights and welfare of patients exposed to such products, including appropriate, fully informed consent; ethical review by an IRB; and safety monitoring.
- Allowing manufacturers to charge high prices for experimental medical products, which forces patients to take financial risks for unproven benefits.
- Stripping away legal protections for patients by immunizing manufacturers, doctors and others against liability, even if they failed to exercise reasonable care or inform vulnerable patients about potential risks and benefits of the experimental products.
- Preventing the FDA from enforcing good manufacturing practices or intervening to stop the sale of tainted or otherwise substandard experimental medical products.

The legislation also would slow the development and impair FDA review of new medical products by:

- Reducing incentives to continue rigorous clinical testing in pursuit of FDA approval.
- Discouraging patients from enrolling in placebo-controlled clinical trials by providing them with access to experimental medical products in the general marketplace.
- Prohibiting the agency from considering (S. 204 and H.R. 878) or requesting (H.R. 1020) information about side effects, injuries or deaths in patients treated with experimental medical products under the legislation.

Congress should stop these attacks on the FDA's authority to regulate experimental medical products, an effort that will only encourage false hope for patients while ultimately doing them more harm than good.

We urge you to oppose S. 204, H.R. 878 and H.R. 1020 and any similar false hope legislation that is introduced in the future. Thank you for considering our views on this important matter.

Sincerely,



Michael A. Carome, M.D.
Director
Public Citizen's Health Research Group



Sarah Sorscher, J.D., M.P.H.
Researcher
Public Citizen's Health Research Group



Treatment Action Group

October 3, 2017

The Honorable Michael C. Burgess, MD
Chairman, Health Subcommittee
Energy and Commerce Committee
United States House of Representatives
Washington, DC 20515

The Honorable Gene Green
Ranking Member, Health Subcommittee
Energy and Commerce Committee
United States House of Representatives
Washington, DC 20515

Dear Chairman Burgess and Ranking Member Green:

Treatment Action Group (TAG) appreciates the opportunity to submit these comments to the Health Subcommittee of the U.S. House of Representatives Energy and Commerce Committee, in association with its hearing, "Examining Patient Access to Investigational Drugs," and the deliberations of H.R. 1020, the Compassionate Freedom of Choice Act of 2017, and S. 204, the Right to Try Act of 2017. TAG is an independent, activist and community-based research and policy think tank fighting for better treatment, prevention, a vaccine, and a cure for HIV, tuberculosis (TB), and hepatitis C virus (HCV). For 25 years, TAG has strongly advocated for expedited access to drugs and biologics with the greatest potential to save human lives. However, we also remain committed to stringent regulatory practices designed to minimize risk, confirm efficacy, and to protect consumers from harmful commercialization practices.

In July 2017, TAG joined with National Center for Health Research in urging the Senate to reject S. 204.¹ We reiterate here that any legislation aiming to circumvent existing expanded access processes authorized and monitored by the Food and Drug Administration (FDA) to help and protect patients with serious or life-threatening illnesses is unnecessary and dangerous.

In the early 1990s, due in large part to the influence of HIV/AIDS activism, the FDA formalized compassionate use and expanded access programs to provide patients with serious or life-threatening diseases with access to experimental drugs that have demonstrated reasonable safety and potential efficacy in phase II clinical trials, and are undergoing further investigation in phase III trials. These programs have been a lifeline for U.S. residents living with HIV, particularly those with virus resistant to approved antiretrovirals (ARVs), those unable to tolerate approved ARV options, and those unable to access phase III

¹ National Center for Health Research. Patient, consumer, and public health coalition letter to senators opposing the "Right to Try" law. July 31, 2017. Available from: <http://www.center4research.org/coalition-letter-senators-opposing-right-try-law/>

trials due to enrollment, distance, or entry criteria restrictions. They have also been vital for people affected by other life-threatening conditions, such as forms of TB and cancer that approved treatment options cannot cure.

There is empirical evidence that existing expanded access programs are sufficient for patients with serious or life-threatening illnesses. A 2016 analysis conducted by the FDA Center for Drug Evaluation and Research (CDER) found that more than 1,000 expanded access applications are received by the agency each year, the vast majority of which (99.7%) are allowed to proceed.² A follow-up analysis confirmed the high number of expanded access applications approved by CDER, while also underscoring FDA commitments to patient protections.³ Between January 2005 and December 2014, 99.3% of almost 9,000 expanded access applications were approved, with only 38 emergency treatment investigational new drugs (INDs) denied and 23 non-emergency treatment INDs not allowed to proceed. The most common reasons for denying emergency INDs was that the patient was stable on current therapy and that it was not deemed an emergency. The most common reasons for not allowing non-emergency expanded access INDs to proceed were incomplete application, unsafe dosing, demonstrated lack of efficacy for intended use, availability of adequate alternative therapies, and inadequate information provided in the application on which to base a decision.

TAG strongly supports the needs of people living with HIV, TB, and HCV to access promising drugs and biologics as quickly as possible and remains committed to the continuity of ethical and scientifically sound mechanisms in place to ensure patients with limited or no treatment options have access to the most promising investigational agents. Right-to-Try legislation in no way improves on these mechanisms and only stands to compromise patient safety and, additionally, create a lax legal and regulatory environment for the pharmaceutical industry. We urge the Health Subcommittee of the Energy and Commerce Committee to consider the following:

- **H.R. 1020 and S. 204, effectively undermine the current requirement that pharmaceutical companies develop, implement, and complete the registrational trials necessary to confirm safety and efficacy in patients with serious and life-threatening illnesses.** Not only are these data necessary to support FDA approval indications, they are essential to clinicians and patients in making informed treatment decisions. Legislation that allows manufacturers to circumvent stringent regulatory approval requirements to instead focus on commercializing its products to desperate patients—particularly with statutory language freeing manufacturers of any liability⁴—

² Jarow JP, Lemery S, Bugin K, Khozin S, Moscicki R. Expanded access of investigational drugs: the experience of the Center of Drug Evaluation and Research Over a 10-year period. *Ther Innov Regul Sc.* 2016 Nov; 50(6):705–9. Available from: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5135086/>

³ Jarow JP, Lemery S, Bugin S, Lowy N. Ten-year experience for the Center for Drug Evaluation and Research, Part 2: FDA's role in ensuring patient safety. *Ther Innov Regul Sc.* 2017 Mar; 51(2):246-9. Available from: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5443559/>

⁴ H.R. 1020, Sec. 561B

is a step in the wrong direction.

- **No risk analysis or evidence of potential efficacy is required in H.R. 1020 or S. 204.** In fact, the proposed legislation stipulates the removal of the FDA from any safety and efficacy determinations and, no less worrisome, would prevent the agency from collecting data from clinicians treating patients with an investigational agent that can be used in safety and efficacy determinations if/when the agent is submitted for approval.⁵ Existing expanded access programs not only allow for access to experimental agents, they contribute to the data sets that inform approval, labeling, and best practices—which protect patients, their providers, and companies alike.

TAG believes it is reasonable and necessary to allow the FDA to retain its regulatory oversight for expanded access and compassionate use programs in order to help mitigate safety concerns, ensure preliminary efficacy to guide risk-benefit determinations, and buttress the need for clinical trial data to inform registrational approval and prescribing practices. Especially since the agency approved more than 99% of expanded access requests, FDA's role in reviewing preapproval access requests is clearly not an impediment, and provides important oversight. H.R. 1020 and S. 204 do nothing for people who are terminally ill. It instead aims to curtail vital FDA stringency requirements that have not only largely succeeded in protecting public health, but continue to be effectively streamlined to hasten access to investigational and approvable drugs and biologics for those who need them most.

Respectfully submitted,

Tim Horn
Deputy Executive Director, HIV & HCV Programs

Erica Lessem
TB/HIV Project Director

⁵ H.R. 1020, Sec. 561A

The Honorable Michael C. Burgess, MD
Chairman, Health Subcommittee
Energy and Commerce Committee
United States House of Representatives
Washington, DC 20515

The Honorable Gene Green
Ranking Member, Health Subcommittee
Energy and Commerce Committee
United States House of Representatives
Washington, DC 20515

2 October 2017

Re: Examining Patient Access to Investigational Drugs

Dear Chairman Burgess and Ranking Member Greene,

As an American citizen and the sister of a patient who suffered from a nearly incurable form of extensively drug-resistant tuberculosis (XDR-TB), I write you in advance of the hearing "Examining Patient Access to Investigational Drugs" on October 3, 2017 to thank you for your interest in promoting the health and well-being of the American public, and to urge you **not** to pass H.R. 1020.

My brother was diagnosed with a severe case of terminal and infectious XDR-TB in 2013. Even less severe cases of TB require multi-drug therapy, and my brother was nearly out of options. His brilliant doctor, Dr. Caitlin Reed of Los Angeles, cobbled together a regimen for him, but needed access to a new drug in phase IIb development called delamanid. Unfortunately, Otsuka, the company that manufactures delamanid, would not grant access to delamanid to my brother, because it had not yet been studied with another drug in his regimen called bedaquiline. Dr. Reed and I and several clinicians and activists pressured the company for a year for access, to no avail (finally, Otsuka changed their policy about co-administration with bedaquiline, but not in time to help my brother). As a result, my brother was stuck on an inferior regimen that, while he managed to survive, caused him to develop psychosis, left him with painful permanent nerve damage, and required him to have a lobectomy to remove some of the disease in absence of enough powerful drugs, so he has permanent limited lung capacity. Meaning he cannot travel himself to be at your event tomorrow. He is actually in the hospital right now for related lung issues.

Our story painfully illustrates frustrations with pre-approval access to novel drugs. However, none of the barriers to access were caused by the U.S. Food and Drug Administration's regulations. Rather, the issue was a company's unwillingness to provide drug. They did not cite any concern about the cost of doing so. And it is well-established that the overwhelming majority of—in fact, nearly all—expanded access applications are approved by the FDA, and that data from such pre-approval use has very rarely resulted in a product not receiving approval. H.R. 1020 gives drug developers full permission to charge for access to a drug, even one that has only been in one clinical trial of unspecified size. But it does not compel them to provide access, even for desperate cases like my brother, which is what we would have needed. We need a more, not less, empowered FDA to be involved in cases of pre-approval access.

Not only would H.R. 1020 not have helped my brother's case, it could have made it worse. H.R. 1020 allows companies to charge for drugs pre-approval. So if H.R. 1020 had been enacted when my brother needed treatment, if Otsuka had decided to grant access, they could have charged the nearly \$25,000-32,000 that delamanid costs on the European market, where it is approved. We would have not had the recourse to pay for it.

The so-called "Compassionate Freedom of Choice Act of 2017" will provide neither freedom nor compassion to the thousands of Americans suffering difficult choices like my brother, our family, and Dr. Reed faced.

I urge you to please, not pass this bill, and to instead to uphold the existing expanded access policies and work to ensure an efficient, empowered, and fully funded FDA that can balance access needs with ensuring sufficient safety and efficacy of new products before they reach the market.

I appreciate your willingness to hear my concerns, and look forward to your assurance that this unhelpful and potentially dangerous legislation not be passed.

Thank you in advance,
Stephanie Aleksanyan

'Examining Patient Access to Investigational Drugs'
House Energy & Commerce Committee
Subcommittee on Health

Andrew McFadyen | The Isaac Foundation

October 3, 2017



Dear Chairman Burgess, Ranking Member Green, and members of the House Energy & Commerce Committee's Subcommittee on Health:



The Isaac Foundation is an organization based in Canada dedicated to finding a cure for a rare and devastating disease called Mucopolysaccharidosis, or MPS. Our work pushes international boundaries, with the bulk of our advocacy and patient support taking place in Canada and the United States. This is an organization that is very dear to me, because it is named after my son – my hero, and the bravest person I know – Isaac McFadyen, who suffers from MPS Type VI.

When Isaac was diagnosed at the age of 18 months, we were told that he was going to live a life of pain and suffering, and that we would endure many years of heartache and heartbreak. Essentially, every bone, muscle, organ, and tissue in his body would be ravaged by this disease until he eventually succumbed to the condition, probably in his early to late teens.

During the past decade he's battled - we've battled - to stave off the inevitable. And we've been lucky. In 2006, after a lot of work and determination, we were able to bring a new life-prolonging treatment to Canada - an enzyme replacement therapy that was approved by the FDA but not by Health Canada - to fight his disease. Isaac is now 13 years old, and the 13 that we see today is very different than the 13 we were told to prepare for.

After our success bringing Isaac's treatment to Canada, other families began contacting our organization so that we could help them obtain access to rare disease medications, and provide advocacy and support throughout their journey. Our successes brought many more families our way - families battling other forms of MPS, as well as other diseases - from Duchenne Muscular Dystrophy, to Batten Disease, to Gaucher Disease, to rare pediatric cancers. Our mission to find a cure for our son became a multi-faceted one that crossed both borders and disease families. It became a mission to help those suffering from any rare disease and in need, and we've dedicated ourselves to that mission ever since.

Today, I'm proud to say that we've never been unsuccessful gaining access to rare disease treatments for children in Canada, and our work alongside pharmaceutical companies is helping patients see similar results for countless children in the United States. We've achieved this success in part because I understand the world that our families are living in, and I understand the unbearable burden that a potentially terminal diagnosis brings. I understand because I live each and every day facing the mortality of my son. I understand because after 10 years, I still wake up every night and check to be sure that my son is still breathing, crippled by the fear that one day I'll walk in and he won't be. I understand because I've walked this lonely road, searching for hope when all hope seemed lost.

From our experience in the patient advocacy community, we understand the unbearable burden of a potentially terminal diagnosis and can see the appeal of Right to Try legislation for those with nowhere else to turn. The Goldwater Institute does a marvelous job of promoting its policy as the last chance for people to extend their lives. Goldwater claims that "Right To Try laws help patients get

immediate access to the medical treatments they need before it's too late," suggesting their legislation "restores life-saving hope back to those who've lost it."

This utopian vision of access to medications for millions of Americans who desperately need them is laudable. However, an analysis of the state Right to Try bills that have already been passed reveals that many laws leave many patients in danger of losing access to home health care, hospice care or even insurance coverage should they try an experimental product. Beyond this often-overlooked aspect of the laws, the cruel reality of Right to Try is that it does not grant patients immediate access to any treatments. Right to Try traffics in false hope, and as the advocates for desperate patients, we believe they deserve better.

Although Right to Try laws have been passed in 37 states, there is no concrete evidence of a single patient ever receiving a life-saving medication under Right to Try that they otherwise wouldn't have through the existing FDA expanded access program. Over 300 million Americans currently live in states with Right to Try laws. Why then, with nearly 80 percent of Americans having, as Goldwater claims, "immediate access to medical treatments they need," do we still have no evidence to suggest these state laws actually do what their defenders purport the laws do? The answer is simple: they aren't. If they were, people like [Jack Fowler](#), a 7-year-old with a rare metabolic disease called Hunter syndrome, would be receiving the life-saving medication he needs. Jack lives in Illinois, which has a Right to Try law, but despite the agreement of his physician, a hospital review board, and the FDA that he needs a certain drug, he is unable to begin treatment. Shire Pharmaceuticals, the company that makes the drug he needs, refuses to give him access.

Indeed, legislation does not guarantee access to investigational therapies for those in need - it never has. Right to Try legislation provides nothing to patients except the "right not to be barred from seeking access to experimental products."¹ Legislation has, however, created a misguided belief among vulnerable patients that the help they have been searching for has arrived. Right to Try is a misnomer, implying an entitlement to patients: "If a person asks, someone or some entity has a duty to provide."²

A more apt title would be "Right to Ask," because this is the only entitlement Right to Try legislation provides patients. This right to ask has been formally codified since 1987 through the FDA's Expanded Access Program. In both the FDA program and under proposed Right to Try legislation, pharmaceutical companies are under no obligation to make their investigational drugs available to patients.³ Thus, investigating what disincentives prevent companies from making their drugs available – and what incentives could be put in place to positively influence these decisions – would be a more fruitful approach than legislating a theoretical "Right to Try."

¹ Bateman-House, Alison et al. "Right-to-Try Laws: Hope, Hype, and Unintended Consequences." *Annals of internal medicine* 163.10 (2015): 796-797.

² Bateman-House, Alison et al. "Right-to-Try Laws: Hope, Hype, and Unintended Consequences." *Annals of internal medicine* 163.10 (2015): 796-797.

³ Rubin, Rita. "Experts critical of America's right-to-try drug laws." *The Lancet* 386.10001 (2015): 1325-1326.

We should be promoting enhancements to the FDA's existing Expanded Access Program, which shows promise for its high approval rates, transparent data collection, and focus on patient safety.

As patient advocates, we know that Right to Try laws can't and won't help our loved ones, some of whom are fighting for their lives. What supporters tout as a beacon of hope does nothing to change the reality for patients in need – and risks making Americans' access to healthcare even more unequal. And not only does Right to Try not work, it actually strips vulnerable patients of valuable assistance.

We urge members of the House of Representatives to vote against any form of Right to Try legislation and instead focus on measures that will provide assistance, not empty words, to everyone with serious need.

Sincerely,

Andrew McFadyen
Executive Director
The Isaac Foundation



September 29, 2017

Members of the House Subcommittee on Health of
the Energy and Commerce Committee
C/O The Honorable Greg Walden, Chairman
2125 Rayburn House Office Building
U.S. House Committee on Energy and Commerce
Washington, DC 20515

Dear Chairman Walden and Members of the Health Subcommittee of the Energy and Commerce Committee,

I am Vice Chairman and Co-Founder of Max Cure Foundation, Inc., a pediatric cancer foundation formed in 2008 in honor of my grandson Max who was diagnosed with cancer and is now a 10 year pediatric cancer survivor. I retired from the practice of law in 2008 to devote my life to assisting children diagnosed with cancer and their families. Given my background as a trial lawyer, I have been actively involved in advocating before Congress and to the public as to the needs of children with cancer. I am personally credited with leading the effort to obtain the experimental drug for 7 year old Josh Hardy that cured the Adenovirus that threatened his life back in March 2014. The Biotech firm, Chimerix, that provided the drug (Brincidofovir) had rejected over 300 prior requests for the drug. The former CEO of Chimerix (Ken Moch) was fired as a result of providing the drug to young Josh and others as part of a hastily formed clinical trial. He told me he is often asked what distinguished the Josh Hardy request for the experimental drug from the over 300 requests that were declined. He told me he answers in two words, "Richard Plotkin." The worldwide attention given to the Josh Hardy matter was a significant factor in causing Congress to address the issue of expanded access under 21st Century Cures Act.

Despite the Virginia Right to Try law not having anything to do with the Josh Hardy matter, the Goldwater Institute, the main proponent H.R. 2368, implies Josh was given the drug under that state law. This is just one of the many falsehoods promulgated by The Goldwater Institute in its effort to "pull the wool over the eyes of Congress" in order to get the Federal Right to Try law passed.

For the Committee's consideration, I wish to highlight the following:

1. In a recent study of 150 requests, selected randomly, made to the FDA to approve the use of experimental drugs in seriously ill and terminally ill patients, the FDA made suggested changes in 11% of those applications, demonstrating that it does not "rubber stamp" the

applications (one could conclude that given that the FDA approves over 99% of the applications submitted). That test, among many other examples, demonstrates that the FDA serves a very important function as the “gatekeeper” and “safety net” for those who are terminally ill and seek experimental drugs.¹ Extrapolating the 11% figure to the 1,562 applications to the FDA in 2015 for compassionate use waivers with respect to experimental drugs results in 172 of those terminally ill and seriously ill patients receiving a safer product due to the involvement of the FDA.

2. Following my involvement with the Josh Hardy matter in March 2014, I attended a meeting later that year in DC among pediatric cancer advocates. The issue of giving experimental drugs to dying children was raised. I stated, “What difference does it make, these children are going to die.” An oncologist at the meeting responded, “You would not be taking that position if you had seen as I have children being given experimental drugs who were ‘tortured’ leading to their deaths.” I have over the last 4 years educated myself to the point that I recognize the importance of the FDA in the approval process for giving experimental drugs to terminally ill patients;
3. As a trial lawyer, I have concluded that H.R. 2368 (successor to H.R. 878) exposes many unsuspecting entities and persons to lawsuits who would be involved in giving experimental drugs to terminally ill patients. I had written an article in The Hill.com² that covered that issue with respect to an earlier draft of the legislation. H.R. 2368 continues to leave unsuspecting individuals and entities, including hospitals, IRB’s, and others exposed to lawsuits based on a claim of negligence. There is clearly an issue as to who is included, and who excluded, under H.R. 2368, Section 2 (c) (1), where it states, “No liability shall lie against a producer, manufacturer, distributor, prescriber, dispenser, possessor, or user of an experimental drug” As a former trial lawyer, I would, as noted, be concerned that many folks/entities would be subject to a claim of negligence. Also, without the involvement of the FDA, I question whether any manufacturer of experimental drugs or devices, plus others, including doctors, hospitals, IRB’s, could obtain liability insurance. Absent product liability insurance or insurance for malpractice or other negligent acts, I suggest without the FDA’s involvement, there will not be any patients who would receive the experimental product – except perhaps if prescribed by less than reputable physicians working in concert with equally disreputable companies. In any event, those subject to lawsuits would not have as a defense that the

¹ Jarow, Jonathan P., et al. “Ten-Year Experience for the Center for Drug Evaluation and Research, Part 2: FDA’s Role in Ensuring Patient Safety.” *Therapeutic Innovation and Regulatory Science* (2016) 1-4

² Plotkin, Richard. “‘Right to Try’ is a Sham.” *The Hill*, February 13, 2017. Accessed September 12, 2017

250 Ashland Pl., 37E
Brooklyn, NY 11217

October 3, 2017

The Honorable Michael Burgess
Chairman of the House Energy & Commerce Committee subcommittee on Health
2336 Rayburn House Office Building
Washington, DC 20515

The Honorable Gene Green
Ranking Member of the House Energy & Commerce Committee subcommittee on Health
2470 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Burgess and Ranking Member Green:

I am the institutional review board and bioethics director at Lehman College.

My job is to evaluate proposed experiments and determine whether they are ethically compatible with the Belmont Report and applicable United States Code provisions. My position was born of the federal recognition that experimental drugs and procedures need to be held to stringent ethical safeguards - abuses such as Dr. Mengele's torture chambers in Auschwitz proved the need to supervise and build ethical boundaries around scientific research.

Right to try essentially bypasses these safety and ethics measures in two ways.

First, and contrary to the Belmont Report, it spreads benefits of biomedical research unevenly among members of society. Persons with means can gain access to research participation and potential health benefits while indigent individuals are left out of the loop. This exacerbates already extreme levels of social and economic inequality.

Second, right to try marries the understandable human desire - often a desperate desire - to get well, with the often corrupting influence of wealth. Implementing right to try procedures runs the risk of human nature taking its ugly course as common swindle the desperate and affluent. Perhaps, if Bernard Madoff went to medical school, he may have become a right to try advocate.

Finally, right to try provides no guarantee that a terminally ill patient will get well. It is cruel to tell people that they can try unapproved medical treatments without mentioning that these treatments may be out of their reach anyway due to cost barriers. In a sense, right to try is like giving me the right to drive a Maserati without mentioning that my chances of being in a position where I will get to drive a Maserati are slim indeed.

I'm not unsympathetic to the ill and their loved ones. I had a serious brain injury in infancy and almost died. I remain learning and physically disabled. Were I offered a pill or procedure to cure

these disabilities, I would perhaps do almost anything to obtain them, but predators who lurk in every office building in the land would no doubt salivate over this possibility.

Sincerely,



Zoltan Boka
IRB & Bioethics Director
Lehman College
Bronx, New York