WHY FALSE HOPE IS DANGEROUS AND HOW TO OPPOSE IT

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One survivor's story

LA Times | By ERYN BROWN and ERYN BROWN JAN 20, 2015 | 5:00 AM

Dr. Caitlin Reed examines her patient at Olive View-UCLA Medical Center in Sylmar. He suffers from extensively drug-resistant tuberculosis. (Mel Melcon / Los Angeles Times)

Source: http://www.latimes.com/science/la-me-drug-resistant-tuberculosis-20150120-story.html

"Stuck between 'a bad choice and a worse choice'"

Dr. Caitlin Reed "has been pushing since March to get Gary access to a new TB drug called delamanid, which has not yet won approval for use in the U.S."

"Otsuka Pharmaceutical, the Japanese company that makes delamanid, has denied Reed's application to a compassionate-use program it has set up to speed the drug to patients in dire need"

"Reed argues that Gary is a perfect candidate for the drug and called the company's position 'profoundly uncompassionate'"

Source: http://www.latimes.com/science/la-me-drug-resistant-tuberculosis-20150120-story.html

"Right to try" would not have helped

"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."

Right to Try Legislation "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."

--Stephanie, Gary's sister

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.

Source: http://docs.house.gov/meetings/IF/IF14/20171003/106461/HHRG-115-IF14-Wstate-Bateman-HouseA-20171003.pdf

"right to try" is a solution for a problem we don't have

- **Patients and those who care for them do struggle to obtain medicines pre-approval**
- **BUT the FDA is not the barrier**
- **The FDA has mechanisms for allowing patients to access investigational drugs**
  - 1988-1992, in response to demands by AIDS activists, the FDA developed a range of programs to speed access to experimental therapies to people with life-threatening diseases
- **FDA now strikes a good balance**
  - FDA okays the vast majority (99.7%) of the >1,000 expanded access requests/year
  - Requires some evidence of safety and efficacy to protect patients

Source: https://www.politico.com/agenda/amp/story/2018/02/02/trump-health-care-right-to-try-000636?__twitter_impression=true

"Right to Try" confers no rights*

*(*except to drug companies)*

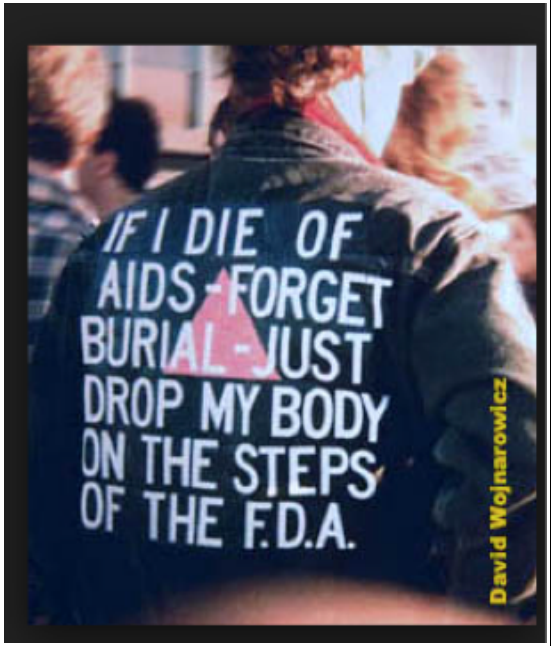
- Because FDA approves nearly all requests for pre-approval access, the **major barrier to pre-approval access is often opaque decisions from companies about who can access their experimental drugs and when**
- Passed and proposed **legislation does nothing to compel companies to make their products available** (even experimental products in late-stage research)
- It would give companies the right to make experimental products available with virtually **no evidence of safety or benefit to patient, and to charge for them**
- **Some companies have legitimate reasons for being unable to provide a product pre-approval** (inadequate resources, e.g. insufficient drug supply, not enough funding/expertise to create a program)
  - **BUT "Right To Try" does nothing to address those challenges**

AIDS ACTIVISTS CAUTION AGAINST REPEATING THEIR EARLY MISTAKES

"I remember the whole idea was that there were like hundreds of drugs at the FDA for AIDS, and if we just scream loud enough or pushed them enough, that dam would break. And there would be this torrent of new therapeutics for people who had HIV"

–Gregg Gonsalves, leader of the HIV/AIDS movement, public health professor at Yale University

"Over time, the AIDS activists came to understand that those drugs just weren't there. Yes, there were potential therapies in the pipeline, but most of them wouldn't work—and patients weren't going to benefit from decimating the federal agency that weeds out the quality medicines from the duds."



Source: https://www.politico.com/agenda/amp/story/2018/02/02/trump-health-care-right-to-try-000636?__twitter_impression=true

TAKE ACTION (1 OF 2)

Senate already passed RTT legislation (S. 204):

Stopping it in the House is our last chance!

Call or write to your Representative immediately to tell them to protect your health and pocketbooks by opposing Right to Try

Who to contact:

- **All members of the House** need to hear their constituents oppose this legislation
  - Republicans and Democrats
  - Liberals and conservatives
  - Regardless of where in the U.S. you live!
- **Find your representative and how to contact them here: https://www.house.gov/representatives/find/**
- **or call the US Capitol Switchboard: (202) 224-3121, option #2**

TAKE ACTION (2 OF 2)

Sample script:

Hello!

My name is [YOUR NAME] and I'm from [PLACE IN STATE].

I am calling to encourage Representative [NAME OF REPRESENTATIVE] to oppose Right to Try legislation.

This misleading legislation offers nothing for patients and their families other than false hopes.

It also risks allowing pharmaceutical companies to sell drugs with no evidence of efficacy.

Can I count on Representative [NAME OF REPRESENTATIVE] to oppose Right to Try?

LEARN MORE

- Resources from the NYU Langone Health Working Group on Compassionate Use and Pre-Approval Access:

  https://med.nyu.edu/pophealth/divisions/medical-ethics/compassionate-use

- Politico's take on the False Hope movement's origin in a deregulation agenda

  https://www.politico.com/agenda/amp/story/2018/02/02/trump-health-care-right-to-try-000636?__twitter_impression=true

- Contact TAG:

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