FDA just simplified the “compassionate use” process. But what does this mean for the life sciences industry?

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The FDA is simplifying its [compassionate use](http://www.fda.gov/forpatients/other/expandedaccess/default.htm) process, aiming to make it easier for terminally ill patients to access potentially life-saving drugs, reports Alexander Gaffney over at [RAPS](http://www.raps.org/Regulatory-Focus/News/2015/02/04/21243/From-100-Hours-to-1-FDA-Dramatically-Simplifies-its-Compassionate-Use-Process/).

This could have some rather interesting implications for the life sciences industry – companies and regulators alike, Gaffney said, are actually [concerned](http://www.raps.org/Regulatory-Focus/Expanded-Access/) by this change in plans. It could hamper clinical trial enrollment, let proprietary technology out of the gate before company’s are IP-ready, and require more money and staff than small biotechs are able to muster.

The compassionate use process to allow patients access to unapproved, investigational drugs –[outside of a clinical trial](http://www.medscape.com/viewarticle/839292) – originally took 100 hours to complete. Thanks to the change in policy, this same process has now been shaved down to less than an hour.

“We know why patients want access to these drugs and we know how busy their treating physicians can be,” wrote FDA Acting Associate Commissioner Peter Lurie, MD, MPH, in an agency [blog post](http://blogs.fda.gov/fdavoice/index.php/2015/02/a-big-step-to-help-the-patients-most-in-need) today. “So we streamlined the new draft form to be shorter and simpler for physicians to fill out.”

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This could mean that life sciences companies will have a readier and more direct access to patients that need their therapies. But here’s the [catch](http://www.raps.org/Regulatory-Focus/Expanded-Access/#sthash.GE2WSlqx.dpuf), Gaffney points out:

For companies, expanded access means letting products out of tightly controlled and heavily monitored environments, potentially subjecting the product to incorrect use and previously unknown adverse events, which would still need to be reported to FDA. Such incidents could potentially raise questions for regulators, thereby harming the chance of a product getting to market.

Further, some companies are concerned that expanded access programs could remove the incentive for patients to enroll in clinical trials meant to provide evidence for their drug’s full approval, thereby delaying its approval and harming other patients in the process.

“People at biotech companies therefore often must make emotionally difficult decisions when trying to balance an individual’s early access to a drug still in clinical trials against the company’s obligation to develop drugs for larger groups of patients and ensure these products gain regulatory approval as quickly as possible,” the trade group BIO [wrote](http://www.bio.org/node/30869) in March 2014. “In some cases, such early access programs could create a conflict between these two principles.”

Still other companies have cited the cost and staff resources necessary to administer compassionate use programs—a problem most evident in small biotechnology startups which do not yet have any income. Sometimes there are also concerns that there won’t be enough drug product available to supply both existing trials and new clinical trials, putting both groups of patients at risk.

To paraphrase BIO’s remarks again: While you’ll hear much about the one patient who isn’t obtaining access to a drug therapy, you’re unlikely to hear much about the thousands of patients who might have to wait several more months to gain access to an FDA-approved drug. And you’re probably not going to hear much about a company’s manufacturing woes or financial problems at the same frequency on social media either.

The changes come because patients have clamored for better access to cutting-edge treatment for decades, *Medscape*writes:

Compassionate use is another name for the FDA’s expanded-access program, which began at the dawn of the HIV/AIDS epidemic when the agency allowed physicians to prescribe investigational drugs to patients considered otherwise doomed. More recently, the FDA has allowed the compassionate use of the experimental Ebola drug brincidofovir (Chimerix) and the antibiotic miltefosine for primary amebic meningoencephalitis. In each case, the agency has not yet determined that the drug is safe and effective.

The FDA has come under pressure to expedite compassionate-use approvals with the spread of right-to-try laws in various states. Such laws allow terminally ill patients to receive unapproved, experimental drugs and devices. The last state to pass such legislation was[Arizona](http://www.medscape.com/viewarticle/835121), in November 2014.