

Improving Access to Drugs—Big Pharma Comes to the Table

In my academic practice group, my colleagues and I must regularly report at a variety of seminars. Last week, we were preparing for a presentation and one of the faculty mentioned that she sadly had 2 patients with cholangiocarcinoma with actionable mutations who didn't derive any benefit from the "appropriate" targeted drugs. This prompted a major e-mail volley from other doctors in the group with similar experiences. In fact, no one had a positive story to tell. Mind you, getting the drugs for these patients is time-consuming; it's straightforward if our drug development program has a trial that the patient is eligible for, but using an expensive approved agent for an off-label indication or finding a trial with an investigational drug at another facility is a major effort. Insurers are naturally reluctant to pay for off-label use, and getting "compassionate" use of an approved or experimental drug is costly in terms of both institutional and personal effort. That, coupled with largely negative outcomes for some drugs, makes us wonder whether it's even worth it sometimes.

In May, Johnson & Johnson announced its plan to form a panel to consider compassionate use requests and render decisions about access.¹ The panel will be composed of doctors, patient advocates, and ethicists. I think this is a great step forward. Right now, we have a "one-off" approach, wherein the patient wants a drug, a physician supports the request and petitions the company who has the drug, and then someone on the corporate side pushes a yes or no button. There's no real discussion, peer review, or even access to information about similar cases and the subsequent outcomes.

There is a lot to consider in providing an unapproved drug to a patient outside of a trial. First and foremost, of course, is safety. We worry about not only the side effects of the drug itself but also the possibility of drug interactions. What about the experience of the provider with the drug? For off-label use of approved drugs, this probably isn't an issue; however, unapproved drugs can involve a steep learning curve. Another issue is the temptation to leapfrog over the standard of care. When you have a terminal disease, it's easy to believe that the new treatment might be your salvation, causing you to bypass established treatment with known effectiveness.

So having an objective panel sort through these issues is a good thing. I hope other companies will follow suit. I also hope that more transparency ensues about outcomes of drugs used outside of a trial. I understand the reluctance to share this information. I fully realize that the experience of a few patients with end-stage disease doesn't compare with the findings of a well-performed clinical trial. But for some patient groups, especially those with rare subsets of diseases, we will probably never have clinical trials. In those cases, any information is better than nothing.

We all remember the early experience with imatinib mesylate. That drug transformed the lives of most patients with chronic myelogenous leukemia, but not everyone who needed it was able to get the drug. I still remember hearing painful stories about young patients facing allogeneic bone marrow transplants who were refused the drug as an alternative to that high-risk procedure.

Sadly, not too many drugs will have the impact of imatinib mesylate. But then, we don't always know when the next great breakthrough will come. And, it's nice to know that companies like Johnson & Johnson are improving the process for granting access to new drugs. It's another step forward.

Reference

1. Johnson & Johnson Announces NYU School of Medicine Partnership to Evaluate Compassionate Use of Investigational Medicines. Johnson & Johnson Web site. Available at: <http://www.jnj.com/news/all/Johnson-Announces-NYU-School-of-Medicine-Partnership-to-Evaluate-Compassionate-Use-of-Investigational-Medicines>. Accessed May 18, 2015.



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