INNOVATION

How Johnson & Johnson Is Helping Patients Through Its Compassionate Use Program

The company's mission is to care for the world—one person at a time. Just ask Chief Medical Officer Joanne Waldstreicher, M.D., who's been leading the charge to improve the process by which very sick patients may apply for access to investigational medications.

By **Barbara Brody**, February 02, 2017

Doing the right thing has always been a guiding principle for Joanne Waldstreicher, M.D.

As a physician, she aimed to treat patients as fairly and ethically as possible. And when she worked in Johnson & Johnson's Pharmaceuticals Research & Development department, she was particularly touched by compassionate use requests—that is, patient appeals to obtain potentially life-saving drugs that haven't yet been FDA-approved.

Today, as the Chief Medical Officer for Johnson & Johnson, she has made it her mission to focus on medical ethics and helping to bring much-needed care to patients in need.

And those heartfelt efforts haven't gone unnoticed.

One of the major highlights of Waldstreicher and her team's work was the creation of a unique program, known as the Compassionate Use Advisory Committee, that guides how Johnson & Johnson handles compassionate use requests. The committee takes a revolutionary approach to evaluating patient appeals by enlisting a panel of independent experts to make objective recommendations.

Recently, Waldstreicher accepted an Ethical Leadership Award from FASPE, the Fellowships at Auschwitz for the Study of Professional Ethics, on behalf of Johnson & Johnson that recognized the company's achievements in the area of compassionate use of medicines and for taking a leadership position in sharing clinical research data.

Johnson & Johnson Expands Access to Investigational Medications Through Its CompAC Program

We sat down with Waldstreicher to learn more about what differentiates Johnson & Johnson's approach to compassionate use—and why she hopes it will serve as a model for other pharmaceutical companies.

Q: So what exactly is compassionate use?

A: Most people obtain medication when their doctors write a prescription for a drug that's been thoroughly studied, evaluated and approved by the national regulatory authority, such as the U.S. Food and Drug Administration (FDA).

But sometimes patients with very serious and/or life-threatening problems exhaust all of the therapies that are currently available, and their doctor feels that their best option is to try something that's still in the experimental stages.

Many of our investigational products show great promise for people with serious diseases, but some people are so sick that their doctor feels they can't wait for a drug to go through the formal regulatory review process. So they contact the company and ask for access to a drug before it's received regulatory approval. We then have to decide who gets this special access and who doesn't.

Living With Cancer: Meet the Researcher Who Went From Designing Clinical Trials to Being in One

Q: Some people might wonder why every compassionate use request can't be granted. Why would that be problematic?

A: It's actually a really difficult decision because you have to weigh the needs of an individual versus the needs of society. Our most important responsibility is to conduct clinical trials and develop full efficacy and safety data on a product so it can get a review by the regulatory authorities and, once approved, become available to the most patients.

You don't want to delay that process in any way. If we provided pre-approval to anyone who asked, why would anyone enroll in a clinical trial?

We also have to make sure we know enough about a particular drug to feel comfortable letting someone try it before it's been FDA-approved. Another consideration is whether or not there is actually enough medicine available for us to give it to a patient, since clinical trials need to be supplied with the drug first.

> These patients and their families are looking for hope, and they deserve to have their requests treated as fairly and respectfully as possible.

Q: How do most pharmaceutical companies handle compassionate use requests? What's so unique about Johnson & Johnson's approach?

A: In the past at Johnson & Johnson—and this is how it still is at other companies—individual requests are reviewed and approved by company employees.

To help review such requests in the most equitable, fair and thoughtful way possible, Johnson & Johnson partnered with the New York University School of Medicine's Division of Medical Ethics in 2015 to create the Compassionate Use Advisory Committee, or "CompAC."

It's a first-of-its-kind, external committee comprised of medical ethicists, physicians and patient advocates that provide Johnson & Johnson with a recommendation for each request. The committee is also blinded, so it doesn't know the patient's country of origin, race, religion or economic status.

What happens now is that a patient's physician submits a request for a drug made by our company by either calling a number or visiting our website. That request then gets funneled to the medical team working on the drug to make sure there are no contraindications, or reasons why it would be dangerous for that particular patient to try it.

The request also goes to the CompAC, which does a complete review of the patient's application and medical condition. After an extremely thorough analysis, keeping our ethical principles front and center, the CompAC makes a recommendation as to whether the request should be approved or denied.

Global Trial Finder: Why It Just Got Easier to Enroll in a Janssen Clinical Study

Johnson & Johnson gets the final say, but so far, we have followed 100% of the committee's recommendations, which has led to more than 60 patients receiving access to one of our cancer treatments that has since been granted FDA approval.

These patients and their families are looking for hope, and they deserve to have their requests treated as fairly and respectfully as possible. My understanding is that NYU has already received several requests from other companies that are interested in setting up a similar protocol.

Q: How else have you and your team helped strengthen the company's commitment to patient care and public health?

A: In 2014, we partnered with Yale University to create the Yale University Open Data Access (YODA) Project, a program through which scientists around the world can access data from Johnson & Johnson's clinical trials.

Researchers apply for clinical data pertaining to our pharmaceutical products and medical devices, and an independent team at the YODA Project reviews these requests. Once a request is approved and access is granted, these scientists can perform their own analyses of our

data and hopefully reach novel and important findings.

Very recently, we expanded our partnership with the YODA Project to include access to clinical trial data on our consumer products, furthering Johnson & Johnson's commitment to data transparency.

The Yale Open Data Access Project: How Johnson & Johnson Is Leading the Way on Clinical Data Transparency

We feel that this type of data sharing honors the people who participate in our studies. We rely on them and so appreciate their commitment to helping advance science. And by letting other researchers analyze our data, we're furthering the advancement of science, which is really the foundation of medicine.

I'm so proud to lead a team of people at the helm of these unique projects that keep patients at the heart of everything we do.

Leadership | Johnson & Johnson | Compassionate Use | CompaAC | Yale Open Data Access Project | Clinical Trials | Clinical Data

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