AROUND NYU LANGONE HEALTH PATIENT CARE

A Pioneering Partnership to Bring Fairness to 'Compassionate Use' Requests

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Imagine you are a medical director at a pharmaceutical company and you face the following situation: a patient with a life-threatening illness wants to try a drug your company is currently testing in a clinical trial. This patient has tried everything else, she does not qualify for the trial, and she, her doctor, and her family view your drug as a last-ditch, but potentially life-saving chance.

On its face, the answer may seem obvious, but consider this: the drug is still in clinical trials, and federal regulators have not approved it. Supplies are very limited. Distributing the drug to this patient and others like her could hold up the trial process, and deprive other patients of a solution.

Moreover, once you expand access, patients eligible for the trial may no longer wish to enroll. And the patient and her family may not truly understand that your drug is still in the testing phase—it may fail entirely, and have serious side effects. The right thing to do no longer seems as clear.

“It can raise the dilemma of balancing the needs of the many and the needs of the few,” said Amrit Ray, MD, MBA *(pictured top left)*. As part of his responsibilities as chief medical officer at Janssen, Johnson & Johnson’s pharmaceuticals group, it’s up to him to consider the kinds of dilemmas described above—what is known as a “compassionate use” request for early access to investigational drugs. “It’s an issue that has kept me up at night,” he said.

A Pioneering Partnership, and a New Strategy

Dr. Ray joined Arthur Caplan, PhD, director of the Division of Medical Ethics in the Department of Population Health at NYU Langone, for a December 10 grand rounds to discuss a unique partnership between Johnson & Johnson and NYU Langone to ensure that compassionate use requests are treated in the most fair and ethical manner.

“The question has come up again and again, ‘How should we deal with these requests?’” said Dr. Caplan. “[The people asking] are people who wouldn’t qualify for a clinical trial, and probably wouldn’t even qualify for an expanded access program that is allowed to be created, but still they want to take a chance on a new drug.”

From left, Dr. Amrit Ray and Dr. Arthur Caplan discuss their organizations' partnership to improve fairness in weighing compassionate use requests, at a grand rounds presentation at NYU Langone on Thursday, December 10.

As Dr. Ray repeatedly considered such requests in his role as chief medical officer, it was clear to him that “these decisions should not rest on the shoulders of a single person,” and should be “grounded on ethical and moral

principles." Janssen began to consider a new approach: an independent decision-making body that could advise them. They set out exploring academic institutions, nonprofits, and individuals who could be potential partners, and approached Dr. Caplan about a year ago.

Tackling Thorny Issues

Dr. Caplan has a history of dealing with knotty biomedical questions. As the New York Times wrote back in May, he has been "eager to opine on the toughest decisions in medical ethics." Dr. Caplan helped create the system for distributing organs for transplants in the U.S., and was vocal during the recent Ebola crisis in western Africa about the need to modify clinical trials to get drugs to patients faster.

But the idea of an independent advisory body to weigh compassionate use requests was new even to him. There is no standard policy, and it has often been unclear who at pharmaceutical companies is making such decisions. "I wouldn't say it's the first-of-its kind committee to make hard decisions . . . but it's pretty unique in terms of being in this space," Dr. Caplan said.

Compassionate use questions have existed for a number of years but, "No one had solved this yet, and we felt that a new solution for patients was needed," said Dr. Ray.

Developing a Group Approach to Ethical Decision Making

In May of 2015, Johnson & Johnson and NYU Langone announced the creation of a pilot initiative called the Compassionate Use Advisory Committee, or CompAC, comprised of a group of internationally recognized medical experts, bioethicists, and patient representatives. CompAC's members were selected by Dr. Caplan. He also chairs the committee, and it is administered by the Division of Medical Ethics.

CompAC considers patient requests Janssen receives which do not qualify for a clinical trial or an "expanded access program" through the Food and Drug Administration (FDA) or a foreign regulator. Additionally, these patients must have exhausted other therapies and must not have an unacceptable medical risk. CompAC recommends to Janssen whether the patient should receive access to the investigational drug. Janssen physicians make the final decision, taking CompAC's recommendation into account, as well as their familiarity with their medicines and the potential medical benefit.

From left, Dr. Alison Bateman-House, deputy chair of CompAC, with Dr. Amrit Ray and Dr. Arthur Caplan at NYU Langone.

The first drug for which CompAC considered requests is DARZALEX™ (daratumumab), a treatment for refractory multiple myeloma, which was subsequently approved by the FDA in November 2015. With American patients now able to get DARZALEX through normal channels, CompAC continues to consider requests for daratumumab from outside the U.S. Following this pilot, CompAC may start considering requests for other drugs.

Neither Dr. Caplan, nor Alison Bateman-House, PhD, the Division of Medical Ethics' Rudin Postdoctoral Fellow and deputy chair of CompAC, is compensated individually for their work, nor does either of them have a final say on whether to grant access, although they present the patient cases and manage the votes. They have also committed to being available 24/7 in case of emergencies.

Leveling the Playing Field

For Dr. Caplan, a primary motive for creating CompAC was bringing fairness to the process of compassionate use requests. It has been an "uneven playing field," he said, with access to investigational drugs too often determined by who can mount the largest social media campaign, draw mainstream media attention, or work connections with drug companies and others.

In order to make the process more fair, Dr. Caplan and his team have advised Johnson & Johnson on the development of a clear, easy to understand website, explaining how to submit compassionate use requests. "Making a website user-friendly [is] very, very, very important for patient access to investigational medicines . . . to give people a fair shot at the

drug," he said. The pilot team also led an effort to develop a standardized intake form for requests, to ensure the committee and Janssen had comparable information about each patient.

Less than one year in, both Drs. Caplan and Ray believe the committee has brought insights and advances to this challenging area, and are currently working on articles about the committee for publication in medical journals to share what they've learned so far.

When they announced the creation of CompAC, they knew it would generate significant attention, but even they did not expect the level of coverage it has received. The story has appeared across major American and global media outlets, and landed Dr. Caplan on the front page of the New York Times.

"For patients, a step forward in compassionate use is clearly of great importance," said Dr. Ray.

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