Biostatistician helps FDA approve first biosimilar drug

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Biosimilar drugs could replace more expensive medicines. UVM biostatistician Bernard "Chip" Cole serves on the expert advisory panel that gave the FDA the green light to approve the first biosimilar in March. Credit: Sally McCay

Bernard "Chip" Cole got the call in late December. The U.S. Food and Drug Administration summoned the University of Vermont biostatistician for another mission. Since 2013, Cole has sat on an important FDA panel that assesses applications for new cancer medications and makes recommendations to the federal agency.

This time, though, the mission was different. Cole and his fellow members of the Oncologic Drugs Advisory Committee would forge a new route through the realm of regulatory review of drugs in the United States. They would assist in the FDA's first evaluation of a "biosimilar" product. Biosimilars are close copies of biological drugs, which are derived from living cells instead of the cocktail of chemicals that make up most medicines.

When chemically based drugs lose their patents, generic versions with identical components can easily reach the marketplace, typically lowering prices. Until now, the FDA had no mechanism to approve close copies of biologics, deemed too complex to ensure that similar but inexact alternatives were equally safe and reliable. The most popular biologics, used to treat cancer and autoimmune diseases, are also some of the most expensive drugs.

Under a mandate of the Affordable Care Act, with a goal to encourage competing products that could help lower costs, the FDA now has a way to evaluate biosimilars. The federal law permits approval of a product shown as "highly similar" to a specific drug on the market and with "no clinically meaningful differences" in safety or effectiveness as that existing drug.

In March—relying on the recommendation of Cole and the advisory panel, which applied those federal guidelines for the first time—the FDA approved Zarxio. The new drug mimics the well-established Neupogen to fight infections in cancer patients undergoing chemotheraphy and other treatment.

The FDA's Center for Drug Evaluation and Research approves more than 100 new medications each year. Most never go through advisory committees.

Cole's group only sees the tough cases, those that lack a "favorable risk profile" or raise alarm bells, making it unclear whether the potential benefits outweigh the risks, he says. "They only bring stuff to us if the question is difficult."

Cole has participated in four reviews on the 13-member panel. His fellow members include oncologists and other medical professionals, a cancer patient or survivor and a consumer advocate. Cole is the only biostatistician.

"The level of the work isn't that bad, but the level of responsibility is huge," he says. "It would be terrible for a drug company to get a drug approved that doesn't work out" or that causes harm, he says. Equally terrible is the prospect of denying, because of perceived risks, a drug that is actually safe and could help millions of sick people or maybe save lives.

"I think of it from the overarching public health perspective," Cole says of his role. "Every time you make a drug available, you're altering public policy."

Where numbers meet medicine

For 23 years, Cole has tied his biostatistics background to cancer. During a post-doctoral fellowship at the Dana-Farber Cancer Institute in Boston, he found he liked collaborating with oncologists and studying patient outcomes. It put him at the cross-section of numerical science and human medicine, where he could not only advance the analysis of data but also answer questions for cancer patients.

"I look for a well-designed study," Cole says of his approach on the FDA panel. "The famous line is: 'All studies have warts.' None of them are perfect."

Cole has his own system after reviewing the briefings provided by the FDA and the drug company. After he studies the information, he writes two statements—one in support of approval, and one against it—explaining his reasoning for each. The position that sounds most convincing tells him where to lean.

Then, Cole gets on a plane and heads to FDA headquarters in Silver Springs, Md., for the meeting. It's more like a courtroom trial, and the committee is the jury.

Each side, the drug company and the FDA staff, presents its argument. Cole and the other committee members sit at a U-shaped table and can ask questions, and he revises his previous statements. The meeting, which usually lasts a day, also includes time for public comments.

"Oftentimes, they're patients who come and tell us stories about what they've been through," Cole says. "Hearing those statements really puts some perspective on what we're doing."

Unlike a courtroom jury, the panelists don't come to a consensus on a verdict at the end of the day. They simply answer "yes" or "no," to the FDA's question: "Should we approve this drug?" They also can comment at that time, and Cole says he sometimes reads his written statement.

With Zarxio, he says, the answer was relatively clear. The vote in favor was unanimous.

The committee played a significant role in the biosimilars review process, ensuring its transparency to the public and bringing expertise to vet critical information, says Tim Irvin, a spokesman for the Center for Drug Evaluation and Research. For now, the committee will look at every upcoming application for a new biosimilar, he says.

"We appreciate getting their feedback," Irvin says. "It gives everybody a chance to look at the data and make sure everything is as it should be."

Cole doesn't expect every biosimilar review to go as smoothly. A key difference is the narrower scope of the clinical trials to show that the characteristics of the new drug and the outcomes for patients are close to those of the existing drug. It's foreseeable that the measurements might not line up, he says.

"If there's one chink in the armor, it opens up a question," he says. "And then you might need a big clinical trial to answer the question."

The committee only makes recommendations, but the FDA usually follows them. Cole says he believes all the committee members feel the weight of their decision.

"You get a collection of people together, you have a collective wisdom," he says. "So it's not all on one person's shoulders."

Provided by University of Vermont

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