What's a new cancer drug worth?

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The latest cancer drugs emerging from drug and biotechnology company labs come with hefty price tags. Last week, the Food and Drug Administration approved Roche/Genentech's application for a new bladder cancer drug—the fourth in the hot new class of immunotherapy drugs.

Its cost will be $12,500 a month, despite clinicians and patients not knowing how well Tecentriq (generic name atezolizumab) works. The clinical trial showed the drug shrank tumors in only 14.8% of the 310 patients in the trial. That's a 1-in-7 response rate, which lasted from two to 14 months, according to the FDA analysis.

Did it prolong survival for some or all of the patients who responded? The trial didn't follow them long enough to know. Did it do better than alternative approaches or a placebo? That's also unknown since the trial didn't have a comparison arm.

The FDA considers tumor shrinkage to be a surrogate marker—a measurable outcome that by itself doesn't have clinical benefit. Shrinking tumors suggests patients will survive longer, but that can be definitively determined only by a clinical trial with a comparison arm.

Roche/Genentech will conduct the trial, of course. That's one of the requirements if your drug gets designated as a "breakthrough therapy" and receives "accelerated approval" from the FDA. But the use of surrogate markers and those approval designations will become a lot more widespread if Congress approves the 21st Century Cures Act, which has won bipartisan support on Capitol Hill.

And that's what is so worrisome, at least to those who worry about the overall cost of healthcare. Tecentriq has been shown to help only a small portion of the patients whose cancer had resumed advancing after other treatments.

But it will be available to the estimated 16,390 patients who will die from the disease this year after failing existing therapies, according to National Cancer Institute estimates. It also will be tried in some portion of the 76,960 people who are diagnosed with bladder cancer every year. A very conservative estimate puts the price of giving them hope—because that's all it is at this point—at $600 million a year.

Can you put a price tag on giving people hope? That shouldn't have to be a metaphysical question. The Boston-based Institute for Clinical and Economic Review, which conducts comparative-effectiveness analyses, has set out to put some scientific rigor behind coming up with an answer.

This week, the not-for-profit ICER will discuss its report on five competing regimens for treating multiple myeloma, a blood cancer that strikes about 27,000 Americans a year. While more than 11,000 die annually, many live for years, often undergoing multiple rounds of chemotherapy.

Over the past two years, ICER's independent analyses have drawn fire from drug companies. Yet the six manufacturers of multiple myeloma drugs have agreed to participate along with clinicians and patient advocates. Pharmacy benefit managers and insurers, who are increasingly using ICER's work in their drug price negotiations, will be paying close attention.

The stakes are huge for payers, patients and, of course, the drug companies. ICER estimates the average cost of a round of chemotherapy for multiple myeloma at $75,000 to $250,000. Given the high price on newer agents, the out-of-pocket cost for patients is in the $20,000 to $30,000 range.

After evaluating the effectiveness of the regimens, ICER found that prices would have to be cut 30% to 70% to lower costs to $150,000 for every quality-adjusted life-year gained.

Significantly, ICER didn't bother to evaluate Johnson & Johnson's Darzalex (daratumumab), which the FDA approved last November for multiple myeloma under accelerated approval after a single-arm trial. It costs over $100,000 a year even though there isn't any outcomes data to do an analysis of its cost-effectiveness.

Here's an idea: Why doesn't Congress include in the 21st Century Cures Act a clause that says any drug given accelerated approval in a single-arm trial be sold only for the cost of manufacturing until the companies offer definitive proof of its clinical effectiveness? ●