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Three solutions to maximize the clinical benefit and affordability of targeted cancer drugs

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Spending on cancer drugs in the United States has nearly doubled in the past five years and continues to grow, imposing substantial financial burden on patients with cancer. One of the biggest drivers of this growth is targeted cancer drugs - small molecules, monoclonal antibodies, and other therapies for cancer that target specific genomic aberrations. Now, a group led by the Abramson Cancer Center of the University of Pennsylvania has proposed three solutions to maximize the clinical benefit and affordability of targeted cancer drugs. The recommendations were published online today in the *Journal of the American Medical Association*. Lead author, Justin E. Bekelman, MD, an associate professor of Radiation Oncology and member of Penn's Abramson Cancer Center, will also discuss the recommendations today in a major symposium at the American Association for Cancer Research Annual Meeting in Chicago (Session SY04).

To consider potential solutions, the University of Pennsylvania convened the Gant Consortium, a multidisciplinary group of experts and stakeholders from cancer medicine, patient advocacy, insurance and pharmaceutical companies, and health care economics, policy, law, and regulation, co-chaired by Bekelman. On the basis of these discussions, the authors propose three principal solutions:

- The United States Food and Drug Administration (FDA) should develop guidance that defines minimum clinically meaningful

effect sizes required for approval of targeted cancer drugs.
- The Centers for Medicare and Medicaid Services (CMS) should conduct a demonstration project in which Medicare negotiates prices for targeted cancer drugs and employs formulary tools to prioritize highly effective drugs while protecting access to therapy for every cancer indication.
- Practice guidelines should prioritize targeted cancer drugs and treatment regimens by clinical benefit and price.

"The remarkable national commitment to cancer research, and the promise, expense, and projected growth of targeted cancer drugs call for solutions to promote drugs that yield meaningful clinical benefits while reducing overall price growth and out-of-pocket spending for patients." Bekelman said.

Spending on cancer drugs in the U.S. jumped from $26 billion in 2012 to more than $45 billion in 2016, and 60 percent of that growth is the result of spending on targeted cancer drugs. According to at least one estimate, cancer drugs will account for one-quarter of the late-stage pharmaceutical research and development pipeline in the U.S. by 2021, and 87 percent of these products will be targeted agents.

The authors point to two specific drugs as examples. The first is imatinib, a tyrosine kinase inhibitor used to treat chronic myeloid leukemia (CML). It's highly effective and has relatively low toxicity compared to previous CML therapies. The 10-year overall survival rate for CML patients taking the drug is 84 percent, and the introduction of the drug in 2001 more than halved the mortality rate of CML cases in the U.S. In 2015, Medicare's estimated monthly price for the drug was $9,299.

For contrast, the co-authors also consider neratinib, another tyrosine kinase inhibitor for patients with early-stage breast cancer. The drug was

approved by the FDA after improving invasive disease-free survival by 2 percent (from 92 percent to 94 percent) after two years of follow up, but without published survival data. The estimated monthly price of the drug is $10,500.

"Imatinib shows the promise of targeted therapy, whereas neratinib exemplifies the concern that marginally effective treatments are putting a strain on U.S. health care spending," said senior author Steven Joffe, MD, MPH, the Emanuel and Robert Hart Professor of Medical Ethics and Health Policy and co-Chair of the Gant Consortium. "Distorted pricing of marginally effective cancer drugs could crowd out the capacity of the US health system to pay for highly effective cancer drugs that yield meaningful clinical benefits or for other therapies of public health importance."

Regular FDA approval for drugs is based on demonstration of clinical benefit, defined as longer life, better quality of life, or an established surrogate for one of the two. Accelerated FDA approval for drugs is based on results reasonably likely to predict clinical benefit.

"The FDA has not specified what characterizes meaningful clinical benefit for regulatory approval," Bekelman said. "This ambiguity is problematic in cases where new targeted cancer drugs show statistically significant but clinically questionable improvements. Thoughtful guidance on what defines minimum clinical benefit for drug approval would help manufacturers design appropriate trials and help patients and their doctors make decisions about cancer therapies."

Researchers also pointed to the need for Medicare to be able to negotiate for targeted cancer drugs, something that's not currently allowed. As of now, Medicare pays for cancer drugs under the Part B and D benefits. In Part B, hospitals and physicians buy the drug, and then bill Medicare at 6 percent above the average sales prices. Under Part D, insurance

companies or pharmacy benefit managers typically manage pricing negotiations.

"As the largest purchaser of cancer drugs in the U.S., Medicare should pilot a program where it has the tools to negotiate directly with drug makers on price," Joffe said. "This can be achieved while still giving patients access to cancer drugs for every available indication."

Finally, the authors call for evidence-based practice guidelines to give physicians and patients a better ability to consider the prices of targeted cancer drugs alongside their benefits and harms when selecting treatments. Although organizations that produce practice guidelines have taken steps to incorporate costs, they should go further.

"Guidelines should rank order cancer regimens and targeted cancer drugs by effectiveness and price and promote greater price transparency," Bekelman said.

Provided by Perelman School of Medicine at the University of Pennsylvania

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