



March 4, 2016

The Honorable Ron Wyden
Ranking Member
Senate Finance Committee
221 Dirksen Senate Building
Washington, DC 20510

The Honorable Charles Grassley
Senate Finance Committee
219 Dirksen Senate Building
Washington, DC 20510

Dear Senators Wyden and Grassley,

Thank you for the opportunity to provide comment on policy issues surrounding the issue of high priced prescription drugs. The American College of Physicians (ACP) is the largest medical specialty organization and the second-largest physician group in the United States. ACP members include 143,000 internal medicine physicians (internists), related subspecialists, and medical student. Internal medicine physicians are specialists who apply scientific knowledge and clinical expertise to the diagnosis, treatment, and compassionate care of adults across the spectrum from health to complex illness.

Prescription drugs are a critical component of the physician's toolkit to prevent and treat disease and avoid costlier medical interventions. ACP is concerned about the increasing price of prescription medications that can result in higher out of pocket costs for patients, potentially threaten patient access to medications, and strain the budgets of public health plans. These concerns about price are not just limited to new, brand name drugs or biologics; some generic drugs have spiked in price without justification while other drugs are purchased by pharmaceutical companies who subsequently raise the price.

As the investigation showed, the pricing of some prescription drugs are priced without significant consideration given to the financial impact on the health care system or the potential problems these prices may cause for patients. ACP's policies on prescription drug pricing recognize that a market-driven approach to addressing the issue of high prescription drug costs is ideal and that it is important that the market support and incentivize the development of new drugs; however recent trends suggest that the market is broken. Comprehensive efforts among stakeholders, including those in the pharmaceutical industry, must be taken to address the rising cost of prescription drugs.

What are the effects of a breakthrough, single source innovator drug on the marketplace?

Breakthrough drugs represent many important medical advances. The intent of the breakthrough therapy designation is to help speed the regulatory approval process and communication between regulators and manufacturers to speed access to therapies that show significant clinical benefit over existing therapies for serious and life-threatening diseases early in clinical testing. As of December 31, 2015, 38 drugs that were granted breakthrough therapy designation have been approved by the FDA¹ and many others have sought the designation.

These important contributions often come with very high price tags. Breakthrough drugs are commonly priced in the five-figures for a treatment cycle, with some reaching into six-figures. For example, cystic fibrosis drug Orkambi was granted breakthrough therapy designation and is priced at \$259,000 per year². In addition to Orkambi, another cystic fibrosis drug, Kalydeco, carries a price of over \$310,000. Some immunotherapy drugs, several of which have received breakthrough designation and harness a patient's immune system to fight diseases like cancer, can cost \$150,000 per patient per year or higher.

An analysis by health care consulting firm Avalere evaluated the potential financial impact of ten breakthrough therapy drugs in the development pipeline on Medicare, Medicaid, and health insurance exchanges. The report found that the ten drugs with the highest potential revenue and representing cancer treatments and treatments for hepatitis C, cystic fibrosis, and diabetic retinopathy, are likely to cost \$49.3 billion over a ten year period including \$31.3 in Medicare, \$15.8 in state and federal Medicaid, and \$2.1 billion in exchange plans³. This analysis did not measure the potential cost savings impact associated with the drugs, though, and long-term evaluations of FDA-approved breakthrough, single source drugs are needed to understand the full scope of potential impact.

What role does the concept of "value" play in this debate, and how should an innovative therapy's value be represented in its price?

ACP believes that the concept of value should be vital in the ongoing debate about prescription drug pricing. There has already been a shift in the health care system toward promoting high-quality, high-value care. However, transitioning to a system that pays for and rewards value requires a baseline level of data on the most clinically and cost effective treatments.

Understanding the comparative- and cost-effectiveness of drugs can help in assessing various approaches that may encourage value-based decision making. Several value-based frameworks or concepts, such as the American Society of Clinical Oncology's conceptual value framework, Memorial Sloan Kettering's Drug Abacus, and initiatives by the Institute for Clinical and Economic Review and the National Comprehensive Cancer Network, have been introduced to help patients understand the value of certain prescription drugs. Although these efforts can help patients and physicians understand the potential value of a drug taking into consideration patient preference and goals, there is a lack of consistency in assessing value for the purposes of informing the price of a drug.

ACP has longstanding policy supporting comparative effectiveness research to measure the effectiveness of health care strategies and believes that all health care payers, including public programs, should employ comparative effectiveness and cost effectiveness in the evaluation of clinical interventions.

Comparative and cost effectiveness information can be beneficial for patients, physicians, and payers but the ability to analyze this information in a meaningful way is currently lacking. Pharmaceutical companies are often required to submit effectiveness data for consideration of coverage in other countries, but they are not required to disclose that information to regulators or the public in the United States. Groups like the Patient-Centered Outcomes Research Institute (PCORI) the Institute for Clinical and Economic Review (ICER) perform accessible comparative or cost effectiveness research, although PCORI, by statute, is not allowed to use the quality adjusted life years (QALYs) metric as a “threshold to establish what type of health care is cost effective or recommended.” QALYs assign an index number to a treatment and are used in cost-utility studies to determine the cost of a treatment per QALY and compare medical interventions. Allowing PCORI to consider QALYs in its research can help to establish an evidence base to which the value of new drugs to existing drugs can be evaluated at the most basic of levels before taking into consideration other factors such as patient preference or goals.

What measures might improve price transparency for new higher-cost therapies while maintaining incentives for manufacturers to invest in new drug development?

ACP acknowledges it can be very expensive and time-consuming to bring a new drug through the regulatory process and to market and that private investments are necessary to bring new drugs to market. Pharmaceutical companies maintain they make large investments into research and development, and estimates of the cost to get a new drug through the regulatory process take research and development costs including those investments in drugs that fail to make it to market into consideration. The widely-cited cost estimate of \$2.6 billion to bring a drug to market has been heavily criticized for overestimating the cost of capital and lacking transparency in its methodology. The cost of drug development may also differ from drug to drug; a drug that fails in late-stage clinical trials will have had significantly higher investments than a drug that fails early in trials.

Improving prescription drug pricing transparency will require pharmaceutical manufacturing companies to disclose certain information that may influence their pricing. ACP believes pharmaceutical manufacturers should disclose production and research and development costs to regulators, and those companies marketing drugs that were developed using publicly-funded research, whether developed by that company or acquired after the drug had entered the market, should be held to a high level of transparency standards. Biomedical investments have some of the highest returns on investments, and companies should disclose any grants, licensing agreements, or other investments by the federal government in the development of a new drug.

We appreciate your leadership on this important issue and your consideration of ACP's comments. If you have any questions regarding this letter, please do not hesitate to contact Hilary Daniel, Senior Analyst, Health Policy at hdaniel@acponline.org.

Sincerely,

A handwritten signature in black ink, appearing to read "Wayne J. Riley". The signature is fluid and cursive, with the first name "Wayne" written in a larger, more prominent script than the last name "Riley".

Wayne J. Riley, MD, MPH, MBA, MACP

President

¹ U.S. Food and Drug Administration. CDER Breakthrough Therapy Designation Approvals As of December 31, 2015. Accessed at <http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/DrugandBiologicApprovalReports/NDAandBLAApprovalReports/UCM481542.pdf>

² Pollack, A. *Orkambi, a New Cystic Fibrosis Drug, Wins F.D.A. Approval*. New York Times. July 2, 2015. Accessed at http://www.nytimes.com/2015/07/03/business/orkambi-a-new-cystic-fibrosis-drug-wins-fda-approval.html?_r=0

³ Avalere Health. *The Future Cost of Innovation: An Analysis of the Impact of Breakthrough Therapies on Government Spending*. June 2015.