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Patient-Centered Guiding Principles for Evaluating Health Care Spending

An NPC analysis of "*Will Reducing Drug Prices Slow Innovation*," a self-published white paper by West Health Policy Center
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Background

On Aug. 12, 2021, the West Health Policy Center published a *white paper* exploring the impact of drug price controls on future biopharmaceutical innovation. The study examined the relationship between changes in revenue and research and development (R&D) for pharmaceutical companies of different sizes from 2000 to 2018. The authors concluded that drug price regulation would have minimal impact on future innovation. West Health promoted this study as evidence that "policymakers do not need to make a false choice between reducing prices to ensure the affordability of pharmaceutical products currently on the market and the innovation required to bring new products to market in the future."

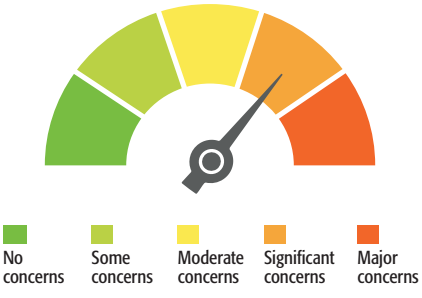
As lawmakers continue to debate drug price controls, it's critically important that they understand whether West Health's claim can be substantiated with evidence. It's also imperative that policy conversations are based on sound evidence and that the effects on innovation and patient health are fully understood. As such, the National Pharmaceutical Council (NPC) reviewed this study using the *Patient-Centered Guiding Principles for Evaluating Health Care Spending*, a framework designed to help decision-makers assess the quality, rigor, and patient-centeredness of health spending analyses. We found significant concerns, which are summarized below and detailed in the Appendix.

STRENGTHS

- ✔ Appropriately adjusts for inflation

AREAS OF CONCERN

- ✘ Promotional materials and study make conclusions not substantiated by data in the analysis
- ✘ Does not consider how changes in the level of innovation (e.g., clinical benefit) in response to price regulation will impact patient health
- ✘ Makes unsupported assumptions, is not transparent in its analysis, and lacks outside experts' review and validation to confirm its methods and findings
- ✘ Does not include a sensitivity analysis to test the uncertainty present in the study's underlying assumptions



EVALUATION

The use of this study in policymaking could pose **significant concerns** due to inadequate methods, unsupported assumptions, and conclusions not substantiated by the analysis. These shortcomings limit the study's ability to accurately inform policy discussions about pricing and innovation.

Health Care Spending Guiding Principles Scorecard

GUIDING PRINCIPLE	RATING
1.1 Consider impacts of changes in health spending on patients and society	
1.2 Incorporate estimates of the actual amounts paid for medical care	
1.3 Recognize differences in spending across patients and time	Not applicable
1.4 Account for changes in disease- or condition-specific epidemiologic measures, such as incidence and prevalence	Not applicable
1.5 Be adjusted for inflation	
1.6 Be based on data relevant to the analysis objectives	
1.7 Be accompanied by sensitivity analyses to elucidate uncertainty that may exist in the evaluation	
1.8 Place conclusions and policy recommendations in the appropriate context	

Appendix

1.1 Evaluation of health care spending should consider impacts of changes in health spending on patients and society

#	CRITERIA	EVALUATION
1	Did the analysis account for the impacts of differences in health spending on the population outcomes important to patients and society?	Promotional material for the paper claims that "These findings affirm the concerns of the American public and make clear that drug manufacturers can sustain reductions in drug spending under recent legislative proposals without impacting innovation." However, the study focuses solely on the reduction in the number of therapies coming to market and does not consider how the innovativeness (e.g., clinical benefit) of these therapies will change. For instance, will there be fewer high-risk curative therapies as a result. For that reason, the study did not fully evaluate the impact of changes on patients and society. Thus, the evidence in the study does not support the promotional claim that there will be no impact to patients and society.
2	If applicable, did the study acknowledge the limitation and implications of excluding key outcomes? Was deference given to the patient's needs?	The study and associated promotional material do not adequately acknowledge limitations related to potential impacts to patients.
RATING		 **Significant Concerns**

1.2 Evaluation of health care spending should incorporate estimates of the actual amounts paid for medical care (White and Whaley, 2019; IQVIA Institute for Human Data Science, 2021)

#	CRITERIA	EVALUATION
1	Was the spending calculated using estimates of the actual amounts paid by stakeholders relevant to the analysis for the sources of medical care under evaluation?	The use of historical data to determine the relationship between changes in R&D expenses and revenue has important limitations in predicting how R&D funding (e.g., paid amounts) will change in response to pricing policy for two reasons. First, the changes modeled were for reductions in revenue of 10% or less, which only reflects a subset of policies. For instance, the CBO estimated that H.R. 3 would have reduced global drug revenue by 19%. Second, the study did not consider changes in funding for small companies that would occur because of reduced venture capital resulting from reduced revenue.
2	Did the study document the estimation of actual paid amounts for each relevant stakeholder?	This analysis used corporate financial data reported per U.S. generally accepted accounting principles (GAAP) and reported to the U.S. Securities and Exchange Commission.
3	If the amount paid was unavailable to the author, did the study document assumptions used to estimate amounts paid and test these assumptions in comprehensive sensitivity analyses?	The study does document that the analysis was limited to the impact of reductions up to 10% due to observed empirical findings. However, this information is not mentioned in any of the study promotional materials. This matters because greater revenue reductions, such as those proposed in recent drug price control legislation, would likely have greater impact than what was modeled in the study.
RATING		 **Significant Concerns**

1.3 Evaluation of health care spending should recognize differences in spending across patients and time (Dieleman et al. 2017)

#	CRITERIA	EVALUATION
1	Did the authors report key subpopulations that may have differed from the population average?	This does not apply as the study did not analyze changes in medical spending.
2	If the study analyzed changes over time, did it report changes in the demographics, treatment patterns and key population health outcomes during the studied time frame?	This does not apply as the study did not analyze changes in medical spending.
RATING		Not applicable

1.4 Evaluation of health care spending should account for changes in disease- or condition-specific epidemiologic measures, such as incidence and prevalence (Dieleman et al., 2017)

#	CRITERIA	EVALUATION
1	Did the study report health care spending both at the per person and population level?	The study does not consider how demand side changes such as demographic and policy changes (e.g., passage of Medicare Part D) could impact the market and associated R&D for pharmaceutical products.
RATING		Not applicable

1.5 Evaluation of health care spending should be adjusted for inflation (Dunn et al., 2018)

#	CRITERIA	EVALUATION
1	Did the study report the year of the data used to estimate the spending?	Yes, the dataset included data for 1,282 companies from 2010-2018. This included 9,453 fiscal years for companies with market capitalization <$7 billion and 618 fiscal years for companies with market capitalization >$7 billion.
2	Did the study adjust spending data for general inflation? • If so, did the study use the appropriate index? • If not, was a reason provided?	Yes, financial data were adjusted for inflation to 2016. Audited financial data were obtained from the Compustat database accessed through Wharton Research Data Services (https://wrds-www.wharton.upenn.edu/).
RATING		 **No Concerns**

1.6 Evaluation of health care spending should be based on data relevant to the analysis objectives (Smith et al., 2018)

#	CRITERIA	EVALUATION
1	Did the study provide descriptions of study metrics, the population studied and the study period?	The study states that it examined changes in revenue and R&D expenses for companies of different sizes from 2000-2018. The methods have not been made public, reviewed by outside experts, replicated, nor have they undergone peer review.
2	Did the study discuss the generalizability of the study outcomes?	The study has two significant generalizability concerns that have not been adequately documented: • The study is limited to reductions in revenue of 10% or less. While documented in the study, this limitation is not mentioned in the promotional materials. • The study uses changes in R&D funding in response to small annual revenue fluctuations to model behavior in response to price controls. Biopharmaceutical response to price controls is likely to differ from small annual revenue changes for a number of reasons, including uncertainty.[1]
RATING		 **Significant Concerns**

1 Congressional Budget Office. Letter to Honorable Frank Pallone, Jr., Chairman, Committee on Energy and Commerce, U.S. House of Representatives Re: Effects of Drug Price Negotiation Stemming From Title 1 of H.R. 3, the Lower Drug Costs Now Act of 2019, on Spending and Revenues Related to Part D of Medicare, October 11, 2019. https://www.cbo.gov/system/files/2019-10/hr3ltr.pdf.

1.7 Evaluation of health care spending should be accompanied by sensitivity analyses to demonstrate the impact of uncertainty around data used for various study components (Walker and Fox-Rushby, 2001)

#	CRITERIA	EVALUATION
1	Did the study examine how changing critical assumptions affected the overall study results?	The assumptions made in the study are potentially flawed and significantly impact the results. Researchers noted that "the model embodies highly conservative assumptions that would tend to be biased towards greater reduction in the number of drug approvals." However, these assumptions are not tested and do not reflect documented marketplace dynamics. • The study assumes that data on historical changes in revenue and R&D can predict large pharmaceutical companies' reactions to significant revenue reductions. However, small market-based revenue shifts are significantly different than expected larger revenue reductions due to government price regulation. • The study assumes that the availability of capital does not impact R&D intensity for smaller companies. However, this assumption runs counter to the existing literature.[2] • The study assumes that pharmaceutical companies can easily shift significant funds from one cost center (e.g., debt payments) to another. However, it is challenging for companies to shift these expenses in the short term, which implies that areas such as R&D would require more significant cuts to sustain financial solvency. • The study assumes that large pharmaceutical companies will reduce "commercial failures" to counteract lower R&D intensity. However, it is risky to assume that companies can further reduce their commercial failure rates to offset reduced revenues because companies already have incentives to maximize their success rate.

2	Did the study report confidence intervals, standard deviations, or standard errors around each key analysis input?	This study modeled a 10% reduction in revenue and carried out three scenarios for reducing R&D expenses, assuming different levels of cost reduction by companies of various sizes. The scenarios posited differential allocation of cost reductions between phase 1, phase 2 or phase 3 of clinical development. The study displayed only point estimates as part of its analysis Based on its assumptions, the study determined a 0% reduction in future drug approvals was achievable through the allocation in scenario 3 and presented this as the main finding. The study does not include a sensitivity analysis to allow for additional projected pipeline impacts based on revenue reductions. Additionally, the expected change for small companies was set to 0%. Alternative assumptions were not tested.
3	If the study used an economic model, did the study conduct sensitivity analysis around inputs' parameter uncertainty?	The study provides no sensitivity analysis to test the impact of critical assumptions. The use of a sensitivity analysis would have helped to show how target inputs were affected by other variables and by the uncertainty inherent in the model.
RATING		 **Significant Concerns**

2 Fleming JJ. The Decline Of Venture Capital Investment In Early-Stage Life Sciences Poses A Challenge To Continued Innovation. *Health Affairs*, 34, no.2 (2015):271-276. doi:10.1377/hlthaff.2014.1051.

1.8 Evaluation of health care spending should place conclusions and policy recommendations in the appropriate context (Keehan et al., 2020; American College of Physicians [ACP], 2009)

#	CRITERIA	EVALUATION
1	Did the study analyze and clearly state the impact of changes for all relevant stakeholders?	The analysis found a 0% reduction of the pharmaceutical pipeline based on assumptions about limited impacts on large pharmaceutical manufacturers. The study did not adequately consider the downstream implications of large-scale revenue changes for other relevant stakeholders, such as venture capitalists and small biotech companies.
2	Did the impact analysis include the costs, outcomes and cost relative to the benefit measures that are important to the relevant stakeholders?	The analysis focused on costs and did not consider patient impacts Specifically, the study does not consider how price regulation will impact population or patient outcomes due to reduced innovation (applies to some scenarios analyzed) or changes in innovation.
3	Did the study's results justify the conclusions and policy recommendations?	West Health has publicly promoted conclusions that are not sufficiently supported by the data. They have stated that "Policymakers do not need to make a false choice between reducing prices to ensure the affordability of pharmaceutical products currently on the market and the innovation required to bring new products to market in the future." This conclusion is not justified given that the study: 1. Underestimates the impact of government price regulation by basing its model on reductions in revenue less than 10%. 2. Assumes small historical shifts in revenues and R&D expenses can accurately forecast larger cuts to revenue. 3. Overlooks downstream impacts on smaller biotech firms and venture capitalists by assuming no reduction in R&D intensity under any scenario. 4. Assumes large pharmaceutical companies can reduce their commercial failure rate to make up for lost revenue without any substantive evidence.

4	Did the conclusions and policy recommendations appropriately consider the study's limitations and uncertainty?	This study did not include its limitations or identify areas of uncertainty in the conclusion. It makes broad claims for policymakers that are not justified based on the study analysis.
5	Did the study clearly state both short and long-term potential tradeoffs between cost savings and impacts? The study should consider effects on the delivery of care, downstream population health and other relevant patient outcomes.	The study does consider trade-offs between small revenue reductions and number of new therapies. However, this trade-off analysis is limited per the points listed under criteria #3 in this table. In addition, the study does not consider trade-offs between price controls and patient outcomes.
RATING		 **Major Concerns**