Evidence to Inform Forecasts of Biopharmaceutical Policy Impact On Drug Development in the U.S. (Focus on Price Setting)

Identified limitations in the data and methods currently used to demonstrate the implications of policy such as the Inflation Reduction or its expansion in the U.S. on investment in clinical study and drug development.

The aim of this analysis is to identify the gaps in evidence, data, and methods available to understand the degree to which centralized price setting or other policies that meaningfully change biopharmaceutical market size such as changes to regulatory incentives or intellectual property affect the level of investment in or nature of drug development.

To what extent do the analyses generated by the CBO and others adequately allow policymakers to weigh the tradeoffs between drug prices, drug revenues and investment in drug development in the U.S. and what evidence can be generated to inform better decision making

Focus group discussion and survey with economists, policy thought leaders and data analysts ***

considering CBO model methods and evidence that informs these models

Number of new drugs not developed is an inadequate outcome metric:

It ignores implications for new indications, impact on disease areas, health outcomes, or health equity.

Price setting also limits investment in post market development and on other drugs in same class as the selected drug.

Does not reflect flow of capital and risk in R&D:

Simulation of a representative firm's decisions does not reflect heterogenous risk across phases of development and investor type (e.g., VC), alternative ways to deploy capital and access to capital in biopharma R&D

Timing of the revenue reduction may also be meaningful.

Communication implies false precision does not allow for validation:

The CBO models are not transparent, externally validated or replicable. Communication of a single point estimate implies more precision than is warranted or would be allowed in published research.

Assumes that all projects with profit greater than zero advance.

The Congressional Budget Office (CBO) issued a call for research to inform the assessment of the impacts of major changes in policy on clinical development for medicines. This includes but is not limited to price setting and could also model the implication of changes to intellectual property. This focus group was convened to identify specified needs for better information.

CBO Director Blog, December 20, 2023, A Call for New Research in the Area of New Drug Development ** CBO New Model of Drug Development January 2022 * Q4 2023-Q1 2024**

See also ITIF The Link Between Drug Prices and Research on the Next Generation of Cures JOE KENNEDY | SEPTEMBER 2019, | Poster ID 137370

The existing studies examining the relationship of the expected financial return from an approved drug, or biopharmaceutical market size, to R&D do not adequately inform policymakers about the impact on R&D for new and existing medicines of a change in law such as price setting. There is a need to evaluate the impact of the Inflation Reduction Act on investment in clinical study and drug development. Expansion or additional policy change affecting the U.S. biopharmaceutical market is a risk without further understanding of the impact.

Needs for information and methods to estimate the relationship between R&D, expectations of financial return, or market size:

- **Post market development:** Focusing on new drug development disregards the effects of late in lifecycle price setting on investment in R&D for existing medicines. This type of investment supports new indications, combination therapy development for conditions like oncology or HIV, or on special populations that are typically not included in the trials for drug approval like children or people with co-morbid conditions.
- **Dated analogs:** Even the leading estimates of the relationship between investment in R&D and biopharmaceutical market size are outdated; both the risk and cost of R&D has changed over time as drugs are increasingly specialized with more complex development protocols. Existing evidence of the cost and risk are not necessarily reflective of today's investment decisions.
- **Disparate impact on certain disease states:** Because the relationship of R&D to market size is variable based on therapeutic class, a more accurate representation of therapeutic dynamics would serve to better inform forecasts.
- **Variable ability to tolerate risk to capital:** The approach of simulating the decisions of a representative firm is informed by a limited dataset and does not include information about the decisions not to invest in certain drugs, nor does it reflect the differing risk profiles. Furthermore, simulation models should reflect the mobility of capital throughout the stages of drug development and how decisions are made across a portfolio of investments.
- **Effect on prices of other drugs in class:** Estimates of the effect of administrative price setting or other policy changes on market size. Administrative price setting would also impact revenue of competitor drugs, which would likely also reduce their net prices in response to this policy. It is still unknown to what degree that will happen and its effect.
- **Focus on cost over health or equity:** Existing models do not adequately consider the health or health equity effects that would come with a reduction in new drugs or R&D on existing drugs.