

"The Impact of Off-label Prescribing on the Development of New Drugs"

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One of the many potential sources of high spending in health care systems, including in the US, is off-label prescription drug use. Off-label prescribing -- the prescribing of a pharmaceutical for something other than what the FDA approved it for -- is a frequent occurrence in the United States. One of the most expensive and prevalent uses of off-label drugs is in the treatment of cancer. As many as 30% of prescriptions for anti-cancer drugs are for off-label uses. In spite of the evidence that off-label drug use is common practice, and the controversy that surrounds this circumventing of regulation in a highly regulated industry, there is very little research quantifying the costs and benefits.

The benefits of off-label prescribing are that it provides patients with more timely access to new treatments and may result in the discovery of new uses for existing drugs. Since the process of gaining approval for on-label use is lengthy and costly to the pharmaceutical company they typically focus on gaining approval for a new product for a limited set of conditions. Without off-label prescribing, some people would not be able to access the drugs they currently use. The cost of off-label drug use is that it is providing expensive treatment that may have little or no scientific basis, potentially exposing patients to unknown risks. An additional reason to be concerned about off-label prescribing is that it could impact the incentive to develop new drugs.

My policy paper examines the impact of off-label prescribing on new drug development in oncology. I compute an upper and lower bound for the effect of off-label prescribing on new drug development in 22 markets. I find a large interval over which the true effect could lie, which includes both an increase and decrease in new drug development as an outcome. Since the methodology used to make the computation uses strong assumptions, there is still much scope for future work in addressing this question.