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Cost-benefit analysis of the FDA: The case of the prescription drug user fee acts ☆

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Abstract

The U.S. Food and Drug Administration (FDA) is estimated to regulate markets accounting for about 20% of consumer spending in the U.S. Despite the FDA's strict adherence to evidence-based evaluation of the safety and efficacy of the products it regulates, there exists no generally agreed upon evidence-based methodology to evaluate the agency's own safety and efficacy record. This paper proposes a methodology to evaluate FDA policies in general, and the central speed-safety tradeoff it faces, in particular. We apply this methodology to estimate the welfare effects of a major piece of legislation affecting this tradeoff, the Prescription Drug User Fee Acts (PDUFA). These acts mandated FDA performance goals in reviewing and acting on drug applications within specified time periods, in return for levying fees on drug manufacturers' submissions. Our methodology uses data on the U.S. sales of drugs as well as the FDA review and withdrawal times for those drugs to estimate measures of the private and social surplus associated with the agency in general, and changes in the speed-safety tradeoff induced by PDUFA, in particular. We find that PDUFA raised the private surplus of producers, and thus innovative returns, by about $7 to $11 billion. Depending on assumptions about the market power of producers during patent protection, we find that PDUFA raised consumer welfare between $7 and $20 billion; thus the combined social surplus was raised by $14 to $31 billion. Converting these economic gains into equivalent health benefits, we find that the more rapid access of drugs on the market enabled by PDUFA saved the equivalent of 140,000 to 310,000 life years. Additionally, we estimate an upper bound on the adverse effects of PDUFA based on drugs submitted during PDUFA I/II and subsequently withdrawn for safety reasons, and find that an extreme upper bound of about

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56,000 life years were lost. This estimate is an extreme upper bound as it assumes all withdrawals since the inception of PDUFA were due to PDUFA and that there were no patients who benefitted from the withdrawn drugs. We discuss how our general methodology could be used to perform a quantitative and evidence-based evaluation of the desirability of other FDA policies in the future, particularly those affecting the speed-safety tradeoff of the agency.

Keywords: FDA; Regulation; Prescription drugs; Safety

1. Introduction

In virtually all developed countries, regulatory authorities provide public oversight of the safety and efficacy of prescription drugs prior to their being approved for marketing. In the U.S., such oversight is conducted by the Food and Drug Administration (FDA). A central tradeoff facing the FDA, and argued by many to be the most central one, involves balancing two goals — fulfilling its mission set by Congress to assure the safety and efficacy of drugs, while at the same time advancing public health by not slowing down or disabling the innovative process by which new medical products reach the market.

Critics of the FDA, domestic and foreign, appear on both sides of this tradeoff. Some observers have argued that the FDA is not taking enough time to evaluate new drugs, thereby allowing unsafe drugs to be marketed, while others have argued that the agency is taking too long to do so and therefore is inflicting harmful effects on innovative returns and patient welfare.[3] However, surprisingly very little quantitative empirical evidence has been put forward to evaluate the degree to which the speed and safety tradeoff facing the FDA is being resolved efficiently. More generally, there seems to be no suggested quantitative methodology or framework for assessing the economic efficiency of the central speed-safety tradeoff of the agency.[4] This is somewhat paradoxical, since despite the agency's strict adherence to evidence-based evaluation of the products it oversees, there is less evidence on its own safety and efficacy track record. Put differently, no product application would pass the FDA approval process with the quality and type of evidence that currently exists for evaluating the FDA policies themselves. The welfare consequences of this lack of methodology and systematic evidence may be quite substantial, as the FDA is estimated to regulate markets accounting for about 20% of consumer spending in the U.S.

Motivated by this lack of quantitative assessment of FDA policies, this paper proposes a general methodology to evaluate the common speed-safety tradeoff of FDA regulations. The methodology relies on the most common form of data available surrounding the drug approval process, namely, the distribution of approval and withdrawal times of drugs as well as the distribution of sales of the approved drugs. We use these commonly available data to estimate the social value of approved drugs. As indicated in Fig. 1, in this paper we interpret the overall social value of a drug as the discounted sum of its yearly social welfare from the time of review and approval to the time of withdrawal, if the drug is withdrawn.

As shown in Fig. 1, the annual social surplus occurs after the drug is reviewed and approved, then is split up into consumer and producer surplus components while on the market, and vanishes completely once the drug is withdrawn (if ever). Therefore, if the drug is beneficial, as it is in the figure, its overall social value falls with the review time and rises with the time until withdrawal. However, if the drug is harmful, as when the social surplus is negative and below the x-axis in the figure, then its overall social value rises with the review time and falls with the time until withdrawal. The agency in general, and separate regulations in particular, influence aggregate social welfare by affecting the distribution of review and withdrawal times, as well as the magnitude and signs of the post-approval annual flows of social surplus.

We apply this general framework to quantify the change in aggregate social welfare induced by major legislative acts comprised of the Prescription Drug User Fee Act (PDUFA) of 1992, later continued as PDUFA II in 1997 and PUDFA-III in 2002. The just recent renewal of PDUFA in 2007 occurs after our data time period ends. These legislative acts specified performance goals for the FDA in terms of faster review times, while levying taxes in the form of user fees on the sponsoring applicant for consideration of new and supplemental drug applications, as well as for existing

[3] See, for example, Peltzman (1974); Olson (1997, 1998, 2004b); Carpenter (2002); Dixon and Gagnon (2004).

[4] The only related study of which we are aware that assesses costs and benefits of regulations is that by Sam Peltzman, who compared growth in market shares of drugs launched prior to 1962 to those launched after the 1962 Kefauver–Harris Amendments were passed by Congress. See Peltzman (1973, 1974).