Off-Label Innovation

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Modern medicine faces two significant challenges. The first is a dwindling pipeline of new treatments. The second is the routine practice of physicians prescribing approved drugs for unapproved uses—so-called "off-label" uses. These problems seem very different. The lack of new treatments is an innovation problem: novel drug discovery has become more difficult, and firms lack incentives to research and develop new uses of old drugs. The problem of off-label uses, on the other hand, is one of safety and efficacy: off-label uses are risky because they aren't supported by the same level of evidence as approved uses. While descriptively accurate, this is not the only accurate description. Each of these problems is also one of information—a lack of information about the safety and efficacy of prescribing approved drugs for unapproved uses. Because all new uses of approved drugs are off-label uses, gathering information about the safety and efficacy of off-label uses, in effect, produces safety and efficacy information for many new uses. Not only that, but some off-label uses may be new: physicians may innovate by prescribing drugs off-label. Reframing these two seemingly disparate problems in terms of a common information deficit enables a single, information-based solution. This solution—which draws on the existing suite of innovation policy levers—incentivizes providers, rather than pharmaceutical companies, to generate the post-market information needed to address both problems.

Introduction 2
I. The Problem of Off-Label Uses & the Problem of New Uses 6
A. The Problem of Off-Label Uses 6
1. Off-Label Use 6
2. The Problem of Off-Label Uses as an Informational Problem 9
B. The Problem of New Uses 10
1. Patent Law 11
2. Market & Data Exclusivity 13
3. The Problem of New Uses as an Informational Problem 14
C. The Common Informational Overlap 15
II. Solving the Problems of New and Off-Label Uses 20
A. Providers as a Source of Information 22
1. Data Collection and Electronic Health Records 22
2. Institutional Bodies & Expertise 23
3. Institutional Know-How & Experience 25
B. Incentives 25
1. Building Out Existing Government Programs 27
2. New Government Programs: Market Inclusivity 29
3. Tax Credits 34
4. Modified Market Exclusivity, Royalties, and Sua Sponte Label Expansions 38
Conclusion 40

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