Article

Delinking Reimbursement

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INTRODUCTION

Recently, scholars and policymakers on both sides of the aisle have become interested in the legal and regulatory structures surrounding pharmaceutical approval and reimbursement in this country. Scholars focusing on the Food and Drug Administration (FDA) have considered the ways in which it ought to regulate emerging technologies,[1] debated the optimal level of evidence required for approval,[2] and explored the ways in which

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1. *See, e.g.*, W. Nicholson Price II, *Regulating Black-Box Medicine*, 116 MICH. L. REV. 421 (2017) (proposing a disclosure-based regulatory regime for medical algorithms); Rachel E. Sachs & Carolyn A. Edelstein, *Ensuring the Safe and Effective FDA Regulation of Fecal Microbiota Transplantation*, 2 J.L. BIOSCIENCES 396 (2015) (examining the challenges of regulating fecal microbiota transplantation as a biologic drug); Patricia J. Zettler et al., *Closing the Regulatory Gap for Synthetic Nicotine*, 59 B.C. L. REV. (forthcoming 2018) (discussing the need for regulation to keep up with new, synthetic substances).

2. *Compare* Aaron S. Kesselheim & Jerry Avorn, *Approving a Problematic Muscular Dystrophy Drug: Implications for FDA Policy*, 316 J. AM. MED. ASS'N 2357 (2016) (arguing that the FDA approved the drug eteplirsen prematurely), *with* Vahid Montazerhodjat & Andrew W. Lo, *Is the FDA Too Conservative or Too Aggressive?: A Bayesian Decision Analysis of Clinical Trial Design* (Nat'l Bureau of Econ. Research, Working Paper No. 21499, 2015) (using Bayesian analysis to demonstrate that the FDA can be overly conservative regarding drugs that treat life-threatening illnesses).

pharmaceutical companies seek to game various FDA requirements to extend their patent monopolies.[3] Scholars focusing on reimbursement have argued that existing payment systems do not provide optimal incentives to payers or providers,[4] and have examined the relationship between insurance regulation and patient costs for new drugs.[5]

In the policy arena, efforts like the 21st Century Cures Act[6] claim to modernize the FDA, encouraging agency officials to think carefully about the development of new healthcare technologies in an age of personalized medicine.[7] At the same time, concerns about the ever-increasing prices of drugs have led to a host of proposals for reform, some wholesale[8] and some piecemeal.[9] Although legislators have yet to take meaningful action to lower drug prices on the federal level, state legislators have

3. *See, e.g.*, Michael A. Carrier & Carl Minniti, *Citizen Petitions: Long, Late-Filed, and at-Last Denied*, 66 AM. U. L. REV. 305 (2016) (exposing the potential for citizen petitions to be used to extend brand monopolies by delaying generic approval); Robin Feldman & Evan Frondorf, *Drug Wars: A New Generation of Generic Pharmaceutical Delay*, 53 HARV. J. ON LEGIS. 499 (2016) (describing how pharmaceutical companies take advantage of nuances in the Hatch-Waxman Act to hold off generic competition); Jordan Paradise, *REMS as a Competitive Tactic: Is Big Pharma Hijacking Drug Access and Patient Safety?*, 15 HOUS. J. HEALTH L. & POL'Y 43 (2015) (describing how FDA risk evaluation and mitigation strategies are used to inhibit generic competition).

4. *See, e.g.*, MEDICARE PAYMENT ADVISORY COMM'N, REPORT TO THE CONGRESS: MEDICARE AND THE HEALTH CARE DELIVERY SYSTEM 119 (June 2016), http://www.medpac.gov/docs/default-source/reports/june-2016-report-to-the-congress-medicare-and-the-health-care-delivery-system.pdf?sfvrsn=0 [hereinafter MEDPAC] (proposing adjustments to Part B payments to change incentives); Rachel E. Sachs, *Prizing Insurance*, 30 HARV. J.L. & TECH. 153 (2016) (discussing the potential for prescription drug insurance to remedy distortions in the patent system that have led to the underdevelopment of drugs).

5. *See, e.g.*, Stacie B. Dusetzina et al., *Association of Prescription Drug Price Rebates in Medicare Part D with Patient Out-of-Pocket and Federal Spending*, 177 J. AM. MED. ASS'N INTERNAL MED. 1185, 1185–86 (2017); Stacie B. Dusetzina et al., *Out-of-Pocket and Health Care Spending Changes for Patients Using Orally Administered Anticancer Therapy After Adoption of State Parity Laws*, J. AM. MED. ASS'N ONCOLOGY (Nov. 9, 2017), https://jamanetwork.com/journals/jamaoncology/fullarticle/2661763.

6. 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016).

7. *21st Century Cures*, ENERGY & COM. SUBCOMMITTEE, https://energycommerce.house.gov/cures (last visited June 18, 2018) (claiming that the 21st Century Cures Act updates an outdated regulatory apparatus).

8. Improving Access to Affordable Prescription Drugs Act, S. 771, 115th Cong. (2017) (proposing reforms to all aspects of the innovation and access process).

9. Creating and Restoring Equal Access to Equivalent Samples (CREATES) Act of 2017, S. 974, 115th Cong. (proposing reforms to the process by which generic drug companies access samples to demonstrate bioequivalence).

aimed to fill the void, advancing eighty bills on the topic in thirty states in 2017 alone.[10]

Yet too often, those who focus on the FDA and those who focus on reimbursement fail to appreciate the links between the two programs. At least in the United States, FDA approval and insurance reimbursement for prescription drugs are tightly linked by law, in a way that affects policy choices on both sides of the equation.[11] It is critical that scholars and policymakers come to understand this linkage. Understanding the relationship between approval and reimbursement is key to effective policymaking. Lawmakers must seek to ensure that policies are actually capable of having their intended effect, and that they do not also have significant unintended consequences. This Article considers the ways in which approval and reimbursement are linked in the United States and envisions a system in which the two are delinked, if only partially.

Part I provides an overview of the legal relationship between FDA approval and insurance reimbursement. In the United States, federal law requires Medicare and Medicaid to cover most, and in many cases all, FDA-approved drugs.[12] Private payers are typically subject to regulation as well, either through state-level coverage mandates for particular sets of drugs or through the Affordable Care Act's (ACA) essential health benefits requirements for plans sold on the individual and small-group markets.[13] Part II explores the ways in which this legal linkage affects our policy choices. Existing proposals that would require the FDA to approve drugs on the basis of less (or less robust) evidence would statistically result in the approval of more unsafe, ineffective drugs[14]—and Medicare and Medicaid would need to pay for all of them. Reform of the FDA's approval system without accompanying reform to insurance reimbursement would be more likely to increase costs, rather than decrease them. Similarly, it is not productive as a policy matter to permit Medicare to negotiate the price of prescription drugs if the government cannot walk away from the deal a pharmaceutical company is offering.

10. AARON BERMAN ET AL., YALE GLOB. HEALTH JUSTICE P'SHIP, CURBING UNFAIR DRUG PRICES: A PRIMER FOR STATES 3 (2017), https://law.yale.edu/system/files/area/center/ghjp/documents/curbing_unfair_drug_prices-policy_paper-080717.pdf.

11. *See infra* Part II.

12. *See infra* Part I.A.

13. *See infra* Part I.B.

14. *See infra* note 101 and accompanying text.

Part III envisions a thought experiment, considering what the potential policy impacts of strongly delinking approval and reimbursement might be. Specifically, what would be the implications for both innovation and access if payers like Medicare and Medicaid were not required to cover these products? There are at least three potential consequences, although their precise reach undoubtedly depends on the scope of revisions made to existing law. First, there would likely be some reduction in access to these medicines. If payers are not legally required to cover certain drugs, they will no longer choose to.

Second, if pharmaceutical companies know that coverage is not automatic—that they must earn coverage, perhaps by demonstrating their product's efficacy over competing drugs—then they may innovate more thoughtfully, in ways that are socially valuable. For instance, we may gain additional information as a society about the comparative costs and benefits of different drugs in a particular class.

Third, strong delinkage would help address the drug pricing problem, precisely because of both of the above considerations. A government which can credibly follow through on the threat not to cover a particular product can extract greater discounts in agreeing to cover it.

Part IV examines three real-world delinkage models to evaluate the potential likelihood that each of these policy outcomes would be realized. In the United States, the Department of Veterans Affairs (VA) is permitted to construct a formulary, unlike Medicare and Medicaid. This delinkage has resulted in lower drug spending, but it has also decreased access to medicines by some amount.[15] The model deployed in many European countries has displayed similar results, with national payers or regulators negotiating on behalf of their citizens.[16] However, in neither case have policymakers observed the development of relevant data about the comparative effectiveness of drugs in a given class. Another American delinkage model, our system of approving and covering medical devices,[17] illustrates some of the policy concerns that might arise for drug companies if the two regulatory systems are delinked.

Part V considers policy options short of full delinkage that might help achieve key benefits of delinkage while avoiding some of its most concerning impacts. Focusing on the theoretical

15. *See infra* Part IV.A.
16. *See infra* Part IV.B.
17. *See infra* Part IV.C.

justifications for the structure of both the FDA approval system and public insurance system, Part V links theories of regulation and innovation with specific policy options. The traditional theory of the FDA as a consumer protection agency might counsel in favor of a carefully designed partial delinkage approach like the one recently considered by the state of Massachusetts.[18] The more modern theory that understands the FDA as an innovation-focused, information-producing agency might encourage collaboration between the FDA and the Centers for Medicare and Medicaid Services (CMS) to accomplish mutual goals.[19] In addition, more recent scholarship that has considered the role of CMS as an innovation agency in its own right reveals a range of solutions targeted at modernizing our reimbursement system.[20]

I. THE RELATIONSHIP BETWEEN FDA APPROVAL AND INSURANCE REIMBURSEMENT

Although there is not always perfect agreement between the set of FDA-approved drugs and the drugs payers are required to cover, in general there is significant overlap. Particularly for public payers, this robust coverage of prescription drugs is required by federal law. Although private payers are often less constrained, many of them provide similarly comprehensive prescription drug coverage pursuant to federal and state laws. This Part presents these various legal regimes and considers the ways in which they are expressed across a range of particularly relevant examples.

A. PUBLIC PAYERS

In the United States, CMS provides insurance to over 100,000,000 Americans through Medicare and Medicaid.[21] These two programs were enacted together, as part of the Social Security Amendments of 1965,[22] but the two differ along a range of

18. *See infra* Part V.A.

19. *See infra* Part V.B.

20. *See infra* Part V.C.

21. *See* CTRS. FOR MEDICARE & MEDICAID SERVS., U.S. DEP'T OF HEALTH & HUMAN SERVS., FISCAL YEAR 2016 JUSTIFICATION OF ESTIMATES FOR APPROPRIATIONS COMMITTEES 109 (2015), https://www.cms.gov/About-CMS/Agency-Information/PerformanceBudget/Downloads/FY2016-CJ-Final.pdf [hereinafter FISCAL YEAR 2016 JUSTIFICATION OF ESTIMATES].

22. *Key Milestones in Medicare and Medicaid History, Selected Years: 1965–2003*, 27 HEALTH CARE FIN. REV. 1, 1 (2005), https://www.cms.gov/Research-Statistics-Data-and-Systems/Research/HealthCareFinancingReview/downloads/05-06Winpg1.pdf.

dimensions. A first key point of distinction is the division of governing responsibilities the programs create between the states and the federal government. Medicare is exclusively federally run and administered, while Medicaid is a classic cooperative-federalism program,[23] jointly administered between the federal government and the states.[24] States are statutorily empowered to seek waivers to Medicaid's general framework, allowing them to expand coverage to new populations[25] or to experiment with new delivery systems.[26] As such, although the broad strokes of the program remain consistent across states, every state's program differs in the details of its implementation.[27]

The programs also differ in terms of the populations they cover. Medicare was designed to cover essentially all Americans beginning when they reach the age of sixty-five.[28] By contrast, Medicaid was initially conceived of as providing health insurance to the "deserving poor,"[29] including children, pregnant

23. Theodore W. Ruger, *Of Icebergs and Glaciers: The Submerged Constitution of American Healthcare*, 75 LAW & CONTEMP. PROBS. 215, 224 (2012).

24. Abbe R. Gluck, *Intrastatutory Federalism and Statutory Interpretation: State Implementation of Federal Law in Health Reform and Beyond*, 121 YALE L.J. 534, 562, 577 (2011).

25. *Id.* at 563.

26. *See* Public Welfare Amendments of 1962, Pub. L. No. 87-43, tit. I, § 122, tit. XI, § 1115, 76 Stat. 172, 192 (1962) (codified as amended at 42 U.S.C. § 1315 (1988)) (providing for experimental project waivers).

27. *Policy Basics: Introduction to Medicaid*, CTR. ON BUDGET & POL'Y PRIORITIES, https://www.cbpp.org/research/health/policy-basics-introduction-to-medicaid (last visited June 18, 2018) ("Because the federal guidelines are broad, states have a great deal of flexibility in designing and administering their programs. As a result, Medicaid eligibility and benefits can and often do vary widely from state to state.").

28. For background on the original design and implementation of Medicare, see PAUL STARR, THE SOCIAL TRANSFORMATION OF AMERICAN MEDICINE 368–70 (1982).

29. David Orentlicher, *Medicaid at 50: No Longer Limited to the "Deserving" Poor?*, 15 YALE J. HEALTH POL'Y L. & ETHICS 185, 185–86 (2015). *See* STARR, *supra* note 28, at 372–74 (describing the impetus for creating access to health care for the poor).

women, parents of minor children, the elderly,[30] and disabled individuals.[31] The ACA attempted to impose a mandatory Medicaid expansion that would have covered everyone below 138% of the poverty line,[32] but the Supreme Court effectively held that the Medicaid expansion must be optional for states.[33] At present, thirty-three states have opted into the expansion,[34] meaning that in many states, nondisabled, childless adults still have little or no Medicaid coverage.[35]

30. Seniors whose income and assets are sufficiently low qualify for both Medicare and Medicaid. There are nearly ten million of these "dual eligibles." KATHERINE YOUNG ET AL., KAISER FAMILY FOUND., MEDICAID'S ROLE FOR DUAL ELIGIBLE BENEFICIARIES 1 (2013), https://kaiserfamilyfoundation.files.wordpress.com/2013/08/7846-04-medicaids-role-for-dual-eligible-beneficiaries.pdf.

31. Nicole Huberfeld, *The Universality of Medicaid at Fifty*, 15 YALE J. HEALTH POL'Y L. & ETHICS 67, 70 (2015).

32. *See Affordable Care Act Eligibility*, MEDICAID.GOV, https://www.medicaid.gov/affordable-care-act/eligibility/index.html (last visited June 18, 2018) (stating that the eligibility for Medicaid under the ACA is expanded to individuals with incomes up to 133% of the poverty line); *Medicaid Expansion & What it Means for You*, HEALTHCARE.GOV, https://www.healthcare.gov/medicaid-chip/medicaid-expansion-and-you (last visited June 18, 2018) (pointing out that in most cases the calculation of income results in a 138% threshold).

33. *See* Nat'l Fed'n Indep. Bus. v. Sebelius, 567 U.S. 519, 585 (2012). The Court held that although the Secretary of Health and Human Services could not constitutionally condition existing Medicaid funds on a state's failure to expand Medicaid, she could offer additional funds to states choosing to expand Medicaid. *Id.*; *see also* Gillian E. Metzger, *To Tax, To Spend, To Regulate*, 126 HARV. L. REV. 83, 108 (2012) ("[States] do not have an obligation to expand their Medicaid programs . . . .").

34. *Current Status of State Medicaid Expansion Decisions*, KAISER FAMILY FOUND., http://www.kff.org/health-reform/slide/current-status-of-the-medicaid-expansion-decision (last updated Apr. 5, 2018). In the November 2017 elections, the citizens of Maine voted to expand Medicaid, but as of this writing, their recalcitrant governor has refused to do so. Abby Goodnough, *Maine Voters Approve Medicaid Expansion, a Rebuke of Governor LePage*, N.Y. TIMES (Nov. 7, 2017), https://www.nytimes.com/2017/11/07/us/maine-medicaid-healthcare.html; Kevin Miller, *Groups Press LePage To File Medicaid Expansion Plan as Time Runs Out*, PORTLAND PRESS HERALD (Apr. 5, 2018), https://www.pressherald.com/2018/04/03/maine-hits-the-federal-deadline-for-medicaid-expansion-plan.

35. As with the original passage of the Medicaid statute, however, this process is likely to take some time. The last state to join Medicaid the first time, Arizona, did so seventeen years after the law's passage. Nicole Huberfeld, *Federalizing Medicaid*, 14 U. PA. J. CONST. L. 431, 445 n.69 (2011). *See generally* KAISER FAMILY FOUND., A HISTORICAL REVIEW OF HOW STATES HAVE RESPONDED TO THE AVAILABILITY OF FEDERAL FUNDS FOR HEALTH COVERAGE 2–6 (Aug. 2012), https://kaiserfamilyfoundation.files.wordpress.com/2013/01/8349.pdf (describing the varying Medicaid implementation timelines for different states and examining the effect of federal funds on states' decisions).

Within Medicare, prescription drugs are primarily covered under two different sections of the program: Part B and Part D. Medicare Part B primarily covers physician services in the outpatient setting,[36] but in doing so it also covers prescription drugs that are administered in doctors' offices and outpatient settings.[37] These drugs—typically large biologics used for the treatment of conditions like cancer,[38] arthritis,[39] or macular degeneration[40]—can cost thousands of dollars per dose, with many doses needed over the course of a year.[41] Part B spending on drugs totaled nearly twenty-five billion dollars in 2015,[42] and half or more of this total comes from anticancer drugs.[43]

Part B coverage of prescription drugs is governed chiefly by the same standard that governs coverage of services under that program: whatever is "reasonable and necessary for the diagnosis or treatment of illness or injury."[44] However, "reasonable and

36. 42 U.S.C. § 1395k(a)(2) (2012).

37. *Id.* § 1395u(o)(1).

38. In 2015, Part B spent $1.25 billion on pegfilgrastim, a drug used in conjunction with chemotherapeutic agents to stimulate the production of white blood cells. *Medicare Drug Spending Dashboard 2015*, CTRS. FOR MEDICARE & MEDICAID SERVS., https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Dashboard/2015-Medicare-Drug-Spending/medicare-drug-spending-dashboard-2015-data.html (last visited June 18, 2018); *Pegfilgrastim*, NAT'L CANCER INST. (Dec. 22, 2016), https://www.cancer.gov/about-cancer/treatment/drugs/pegfilgrastim.

39. In 2015, Part B spent $1.24 billion on infliximab, a drug used to treat rheumatoid arthritis and other autoimmune conditions. *Infliximab (by Injection)*, NAT'L LIBR. MED., https://www.ncbi.nlm.nih.gov/pubmedhealth/PMHT0010708 (last visited June 18, 2018); *Medicare Drug Spending Dashboard 2015*, *supra* note 38.

40. In 2015, Part B spent $1.8 billion on aflibercept, a drug used to treat age-related macular degeneration and other related conditions. *Aflibercept Injection*, NAT'L LIBR. MED.: MEDLINE PLUS, https://medlineplus.gov/druginfo/meds/a612004.html (last updated Feb. 15, 2017); *Medicare Drug Spending Dashboard 2015*, *supra* note 38.

41. *See, e.g.*, Tracy Staton, *Eylea May Beat Lucentis on Price, but What of Avastin?*, FIERCEPHARMA (Nov. 21, 2011), http://www.fiercepharma.com/pharma/eylea-may-beat-lucentis-on-price-but-what-of-avastin (describing per-dose costs of $1850 to $2000 and yearly treatments ranging from $16,000 to $24,000).

42. The twenty-five billion dollar figure was calculated using data provided by CMS. *Medicare Drug Spending Dashboard 2015*, *supra* note 38.

43. MEDPAC, *supra* note 4 ("In 2014, Medicare spending for anticancer drugs accounted for about 55 percent of the nearly $21 billion spent on Part B drugs . . . .").

44. 42 U.S.C. § 1395y(a)(1)(A) (2012).

necessary" is not defined by the statute or regulations,[45] and as such CMS has set up extensive coverage-determination procedures.[46] In practice, Part B drug coverage is quite broad and is limited primarily by the structure of the program. That is, Part B coverage is restricted to drugs which are not self-administered and are provided in the course of a physician's service.[47] But Part B cannot decline to cover an effective FDA-approved drug simply because it is expensive,[48] and the Part B payment system is even structured to encourage physicians to prescribe more expensive products.[49]

Although the broader Medicare program has existed since 1965, Medicare did not provide a standard pharmacy benefit plan to seniors until 2003,[50] when Medicare Part D was created.[51] Total expenditures on drugs under the Part D program are much higher than under Part B, with 2015 spending under the program exceeding $135 billion.[52] The drugs with the highest expenditures under the Part D program tell a slightly different story than the drugs with the highest expenditures under Part B. To be sure, the expensive multiple-myeloma drug Revlimid cost Part D just over two billion dollars in 2015, for the treatment

45. *See* Isaac D. Buck, *Furthering the Fiduciary Metaphor: The Duty of Providers to the Payers of Medicare*, 104 CAL. L. REV. 1043, 1068–69 (2016); Peter J. Neumann & James D. Chambers, *Medicare's Enduring Struggle to Define "Reasonable and Necessary" Care*, 367 NEW ENG. J. MED. 1775, 1775–76 (2012).

46. *See Medicare Coverage Determination Process*, CTRS. FOR MEDICARE & MEDICAID SERVS., https://www.cms.gov/Medicare/Coverage/DeterminationProcess/index.html (last updated Mar. 6, 2018). *See generally* Eleanor D. Kinney, *Medicare Coverage Decision-Making and Appeal Procedures: Can Process Meet the Challenge of New Medical Technology?*, 60 WASH. & LEE L. REV. 1461, 1471–89 (2003) (describing Part B coverage determination and appeals processes).

47. MEDPAC, *supra* note 4, at 121.

48. *See* 42 U.S.C. § 1395y(a)(1)(A).

49. MEDPAC, *supra* note 4, at 118, 127. *See infra* text accompanying notes 244–48 for a fuller explanation of this point.

50. *See* JANET LUNDY, KAISER FAMILY FOUND., PRESCRIPTION DRUG TRENDS 5 (2010) ("[A]bout one-quarter (27%) of seniors age 65 and older, and one-third of poor (34%) and near-poor (33%) seniors, had no drug coverage in 2003 [when Congress passed Part D]."); *see also* Dana Gelb Safran et al., *Prescription Drug Coverage and Seniors: Findings from a 2003 National Survey*, HEALTH AFF. (Web Exclusive) (Apr. 19, 2005).

51. Medicare Prescription Drug, Improvement, and Modernization Act of 2003, Pub. L. No. 108-173, 117 Stat. 2066 (codified in scattered sections of 21, 26, and 42 U.S.C.).

52. *Medicare Drug Spending Dashboard 2015*, *supra* note 38 (using the Part D spreadsheet to calculate).

of roughly 30,000 beneficiaries.[53] But the program also spent nearly $2.9 billion providing Crestor, a high cholesterol drug, to more than 1.7 million beneficiaries, at a much lower cost per patient.[54]

Part D's coverage requirements are specified quite clearly in both statute and regulation. By law, plans must cover at least two FDA-approved[55] drugs per therapeutic class,[56] although plans generally cover more than two.[57] And for six classes of drugs—anticonvulsants, antidepressants, antineoplastics (cancer drugs), antipsychotics, antiretrovirals (for the treatment of HIV/AIDS), and immunosuppressants (for the treatment of transplant rejection) Medicare must cover essentially all FDA-approved drugs.[58] There are two primary reasons for the protection of these six classes. First, CMS wanted to prevent discrimination against beneficiaries with these conditions, as might be expected for patients with high-cost preexisting conditions.[59] Second, CMS aimed to "mitigate the risks and complications associated with an interruption of therapy for these vulnerable populations."[60]

Medicaid's system of prescription drug coverage is somewhat simpler. The federal government does not require that state Medicaid programs cover outpatient prescription drugs,

53. *Id.*

54. *Id.*

55. 42 C.F.R. § 423.100 (2017) defines "Part D drug" for the purposes of the program by reference to Social Security Act section 1927(k)(2)(A), the Medicaid statute, which is linked to drugs approved under the FDA statute. *See* 42 U.S.C. § 1396r-8(c)(1)(C)(i) (2012) (referencing section 505(c) of the Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 355(c) (2012)).

56. 42 C.F.R. § 423.120(b)(2)(i).

57. NAT'L COUNCIL ON AGING, MEDICARE PART D DRUG PLANS: WHAT THEY MUST, MAY, AND CANNOT COVER 1 (2017), https://www.ncoa.org/resources/medicare-part-d-plans-what-they-must-can-cannot-cover.

58. 42 U.S.C. § 1395w-104(b)(3)(G)(iv).

59. *See* CTRS. FOR MEDICARE & MEDICAID SERVS., U.S.DEP'T OF HEALTH & HUMAN SERVS., MEDICARE PRESCRIPTION DRUG BENEFIT MANUAL ch. 16, § 30.2.5 (2016) [hereinafter MEDICARE MANUAL], https://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovContra/Downloads/Part-D-Benefits-Manual-Chapter-6.pdf; *see also* Douglas B. Jacobs & Benjamin D. Sommers, *Using Drugs to Discriminate—Adverse Selection in the Insurance Marketplace*, 372 NEW ENG. J. MED. 399, 400 (2015) (describing how some insurers structure prescription drug benefits to deter high-cost patients from enrolling in their plans).

60. MEDICARE MANUAL, *supra* note 59.

but all states have chosen to do so.[61] That choice comes with a set of coverage obligations. States must cover all FDA-approved drugs with a few classes of exceptions,[62] such as drugs used for cosmetic purposes.[63] To be sure, Medicaid programs are permitted to use formulary management tools like prior authorization or step therapy to steer patients toward less-expensive products, at least at first.[64] But where these tools are used in a way that goes beyond treatment guidelines, patients have sued and obtained access to rationed products.[65]

Medicaid's coverage requirements come with preferred-pricing benefits for the states. By law, pharmaceutical companies must remit to Medicaid a rebate for each unit of a drug they sell to the program, and these rebates can be quite substantial. Innovator drug companies must remit at least 23.1% of a drug's Average Manufacturer Price (AMP),[66] and states are empowered to seek additional rebates on top of that.[67] If the company offers an even bigger discount to another payer, Medicaid is entitled by law to that "best price" provided to another entity for the drug.[68] Medicaid is also insulated from price increases in existing drugs that outpace the inflation rate,[69] and more than half of Medicaid rebates are estimated to be due to this provision.[70]

61. JULIA PARADISE, KAISER FAMILY FOUND., MEDICAID MOVING FORWARD 4 (Mar. 9, 2015), https://www.kff.org/health-reform/issue-brief/medicaid-moving-forward.

62. 42 U.S.C. § 1396r-8(k)(2).

63. *See id.* § 1396r-8(d)(2)(C); *see also id.* § 1396r-8(d)(2).

64. *Id.* § 1396r-8(d)(1)(A), (d)(4).

65. *E.g.*, Ed Silverman, *Washington State Told To Lift Restrictions on Hepatitis C Medicines*, STAT (May 27, 2016), https://www.statnews.com/pharmalot/2016/05/27/washington-state-hepatitis-drug-prices; Joseph Walker, *Arkansas Reaches Settlement in Cystic Fibrosis Drug Suit*, WALL ST. J. (Feb. 5, 2015), https://www.wsj.com/articles/arkansas-reaches-settlement-in-cystic-fibrosis-drug-suit-1423162197.

66. *See* 42 U.S.C. § 1396r-8(c)(1)(B)(i)(VI); *see also* Patient Protection and Affordable Care Act, Pub. L. No. 111-148, § 2503(a)(2), 124 Stat. 310 (2010) (codified as amended at 42 U.S.C. § 1396r-8(k)(1)(A)) (redefining AMP).

67. Generic companies must remit thirteen percent of the AMP per unit. 42 U.S.C. § 1396r-8(c)(3)(B)(iii).

68. *Id.* § 1396r-8(c)(1)(A)(ii)(I). There are some exceptions to this. Prices paid by Medicare Part D plans or the Veterans Administration, for instance, are excluded. *Id.* § 1396r-8(c)(1)(C)(i).

69. *Id.* § 1396r-8(c)(2)(A).

70. U.S. DEP'T OF HEALTH & HUMAN SERVS., OFFICE OF INSPECTOR GEN., OEI-03-13-00650, MEDICAID REBATES FOR BRAND-NAME DRUGS EXCEEDED PART D REBATES BY A SUBSTANTIAL MARGIN 8 (2015).

Importantly, Medicaid coverage seems to be required regardless of the FDA pathway the drug in question takes to approval. Before 1992, drugs approved by the FDA took a fairly standard path to approval, proceeding through three phases of clinical trials designed to demonstrate safety and efficacy.[71] Although many innovators still make use of this traditional pathway today, more and more innovator companies are taking advantage of a set of expedited development programs to speed their path to market.[72]

Some of these programs offer primarily procedural benefits. For example, the Fast Track and Breakthrough Therapy Designations give qualifying sponsors more opportunities to meet and work with FDA officials in ways that ensure trials are designed efficiently and carefully from the beginning.[73] However, the Accelerated Approval program is more substantive. It permits sponsors to obtain FDA approval on the basis of a surrogate endpoint or an intermediate clinical endpoint that is reasonably likely to predict the drug's clinical benefit.[74] This program is intended to address unmet medical needs "for a serious or life-threatening disease or condition."[75] In theory, drugs approved under this program are subject to required postapproval clinical trials, to confirm and support their effectiveness.[76] However, too often these trials are not completed.[77]

Drugs approved under the Accelerated Approval program are not subject to the same standards before FDA approval as

71. PETER BARTON HUTT ET AL., FOOD AND DRUG LAW 628 (3d ed. 2007).

72. Martin Kwok et al., *Expedited Programs for Serious Conditions: An Update on Breakthrough Therapy Designation*, 37 CLINICAL THERAPEUTICS 2104, 2104 (2015).

73. *See* 21 U.S.C. § 356(a)–(b) (2012); *see also* FDA, GUIDANCE FOR INDUSTRY: EXPEDITED PROGRAMS FOR SERIOUS CONDITIONS—DRUGS AND BIOLOGICS 7–8 (2014), http://www.fda.gov/downloads/Drugs/Guidances/UCM358301.pdf (comparing FDA programs for expediting drug approval).

74. *See infra* text accompanying notes 151–55 for a more in-depth discussion of surrogate endpoints.

75. 21 U.S.C. § 356(c)(1)(A).

76. Huseyin Naci et al., *Characteristics of Preapproval and Postapproval Studies for Drugs Granted Accelerated Approval by the US Food and Drug Administration*, 318 J. AM. MED. ASS'N 626, 627 (2017).

77. *Id.* at 634 (noting that of twenty-four indications approved under the Accelerated Approval program, five years after approval eight indications still had not fulfilled their postmarket requirements).

drugs subjected to the standard three-phase clinical trial process.[78] As such, there may be a question about whether Medicaid should be legally required to cover them, if coverage is viewed as a reward for successful completion of the thorough FDA review process. The FDA has taken the position that "because drugs approved under the accelerated approval process meet the statutory standards for safety and effectiveness, they would be eligible for reimbursement under State Medicaid programs or other third-party plans."[79] It is not clear why this would be the FDA's interpretive decision to make (rather than CMS's decision), but to date, it does not appear that CMS has formally advanced a contrary position.[80]

B. PRIVATE PAYERS

The statutes and regulations governing coverage through private payers are more complex. Private insurance is also regulated at the state level, and there are often state-level coverage mandates for particular conditions. For instance, forty-two states require payers to cover all FDA-approved cancer therapies.[81] Forty-six states have laws mandating diabetes coverage, including at least a subset of relevant medications.[82]

Private plans that are marketed under the ACA are jointly regulated at the federal and state level.[83] Federal regulations require plans sold in the individual and small-group insurance markets to cover ten essential health benefits, one of which is

78. New Drug, Antibiotic, and Biological Drug Product Regulations, Accelerated Approval, 57 Fed. Reg. 58,942, 58,942 (Dec. 11, 1992) (codified at 21 C.F.R. pts. 314, 601).

79. *Id.* at 58,945.

80. A 2015 presentation from the Executive Director of the National Association of Medicaid Directors seemed to confirm this view, noting that Medicaid currently "requires coverage regardless of approval pathway." Matt Salo, *High-Cost Drugs: Impacts on the Medicaid Program* at slide 7 (Apr. 9, 2015), http://www.csrxp.org/wp-content/uploads/2015/04/CSRxP-Congressional-briefing8.pdf.

81. Lee N. Newcomer, *Those Who Pay Have a Say: A View on Oncology Drug Pricing and Reimbursement*, 21 ONCOLOGIST 779, 779 (2016).

82. Although the states differed as to how many medication options they provided to patients, they all offered coverage of at least a dozen brand-name drugs. *Diabetes Pharmaceuticals State Mandates*, NAT'L CONF. OF STATE LEGISLATURES (Oct. 10, 2016), http://www.ncsl.org/research/health/diabetes-pharmaceuticals-state-mandates.aspx.

83. *See* David Blumenthal & Sara R. Collins, *Health Care Coverage Under the Affordable Care Act—A Progress Report*, 371 NEW ENG. J. MED. 275, 281 (2014).

prescription drug coverage.[84] At a minimum, these plans must cover at least one drug per therapeutic class.[85] But, at present, the federal government has delegated the choice of a minimum benchmark plan to each state,[86] and these benchmark plans often require more expansive coverage.[87]

To the extent that private plans are legally required to cover fewer drugs than Medicare or Medicaid are, they may have more freedom to negotiate prices than do public payers. However, private payers' ability to demand those discounts may be practically limited. Medicaid's statutory best-price rule requires that pharmaceutical companies providing large discounts to private payers extend those discounts to Medicaid as well.[88] For drugs whose indications have a relatively high prevalence among the Medicaid population, it is easy to imagine their manufacturers limiting discounts to private payers to prevent triggering the best-price rule.

Importantly, just because a payer is legally required to cover a particular product does not mean it will be affordable to the patient in question. Medicare and private payers often impose significant out-of-pocket cost sharing, although Medicaid copayments are tightly regulated by the government.[89] Medicare Part D enrollees may need to pay thousands of dollars out of pocket, particularly for expensive specialty drugs.[90] And for the growing proportion of privately ensured patients who are enrolled in a

84. 42 U.S.C. § 18022(b)(1)(F) (2012).

85. 45 C.F.R. § 156.122(a)(1)(i) (2017).

86. *See* Nicholas Bagley, *Legal Limits and the Implementation of the Affordable Care Act*, 164 U. PA. L. REV. 1715, 1716–17 (2016).

87. *See* Joshua P. Cohen et al., *Complying With State and Federal Regulations on Essential Drug Benefits: Implementing the Affordable Care Act*, 20 AM. J. MANAGED CARE 153 (2014) (explaining how HHS implies broader coverage of prescription drugs, as it would require state coverage of either at least one drug in each therapeutic class, or the number of drugs that the benchmark plan offers, whichever is more); *State Insurance Mandates and the ACA Essential Benefits Provisions*, NAT'L CONF. OF STATE LEGISLATURES (Apr. 12, 2018), http://www.ncsl.org/research/health/state-ins-mandates-and-aca-essential-benefits.aspx#EHB_Rx ("The benefits and services included in the benchmark health insurance plan selected by the state would be the essential health benefits package. Plans could modify coverage within a benefit category so long as they do not reduce the value of coverage.").

88. 42 U.S.C. § 1396r-8(c)(1)(C).

89. *Cost Sharing Out of Pocket Costs*, CTRS. FOR MEDICARE & MEDICAID SERVS. (2013), https://www.medicaid.gov/medicaid/cost-sharing/out-of-pocket-costs/index.html (describing out-of-pocket costs imposed by states under Medicaid).

90. *See supra* note 5.

high-deductible health plan,[91] they may similarly be exposed to thousands of dollars in cost sharing before their insurance coverage kicks in.[92] These up-front costs may dissuade or prevent patients from accessing even covered products.[93]

II. LINKAGE AFFECTS POLICY CHOICES ABOUT BOTH APPROVAL AND COVERAGE

The legal link between FDA approval and insurance reimbursement has implications for policy proposals in both areas. This Part considers policy initiatives that scholars and policymakers have proposed on both sides of the issue and explains how those initiatives would be affected by the legal relationship between approval and reimbursement. In short, initiatives that would alter the FDA's approval process would likely have significant unintended consequences. And initiatives that would seek to affect drug pricing and overall drug spending would be rendered toothless.

A. ALTERING THE FDA APPROVAL PROCESS

Over the last several years, academics and policymakers have proposed a number of initiatives that would permit or require the FDA to approve drugs on the basis of less (or less robust) evidence. Some of these proposals are quite extreme, such as proposals to approve drugs on the basis of safety data alone, rather than requiring proof of efficacy.[94] Others are much more

91. *See 2015 Employer Health Benefits Survey*, KAISER FAMILY FOUND. (Sept. 22, 2015), https://www.kff.org/report-section/ehbs-2015-summary-of-findings (noting that in 2015, twenty-four percent of workers were enrolled in a high-deductible plan, up from four percent in 2006).

92. *See 2016 Employer Health Benefits Survey: High-Deductible Plans With Savings Option*, KAISER FAMILY FOUND. (Sept. 14, 2016), https://www.kff.org/report-section/ehbs-2016-section-eight-high-deductible-health-plans-with-savings-option.

93. *See, e.g.*, ROBIN A. COHEN & MARIA A. VILLARROEL, STRATEGIES USED BY ADULTS TO REDUCE THEIR PRESCRIPTION DRUG COSTS: UNITED STATES, 2013, NCHS DATA BRIEF 2 (Jan. 2015) (detailing strategies patients used to reduce prescription drug costs, including skipping doses or delaying filling a prescription). Professor Amy Monahan has looked closely at the ways in which ACA plans have implemented the Act's essential health benefit requirements, including for prescription drugs like those for the treatment of hepatitis C, in ways that impose significant out-of-pocket costs on patients. *See generally* Amy B. Monahan, *Undermining the ACA: How the Regulatory Failure to Define Essential Health Benefits Allows Strategic Insurer Behavior*, 44 AM. J.L. & MED. (forthcoming 2018).

94. *See, e.g.*, Joseph V. Gulfo, *A Trumpian Cure for the FDA's Chronic Lethargy*, WALL ST. J. (Nov. 22, 2016), https://www.wsj.com/articles/a-trumpian

moderate, such as the provision in the 21st Century Cures Act requiring the FDA to consider the potential use of "real-world evidence," rather than randomized, controlled trials, in the approval of secondary indications for existing drugs.[95] Still others lie in between, such as the proposed Reciprocity Ensures Streamlined Use of Lifesaving Treatments (RESULT) Act, which would require the FDA to speed review of drugs that are already approved for marketing in a particular list of foreign countries.[96]

Proponents of these and other initiatives argue that many of the requirements the FDA imposes on manufacturers seeking to bring new drugs to market are mere bureaucratic "red tape."[97] In their view, if we could only tear down the barriers the FDA imposes throughout the regulatory process, there would be enormous benefits to the system. Drug approvals would happen much more quickly,[98] Americans would be able to access life-saving drugs and devices which are already available elsewhere,[99]

-cure-for-the-fdas-chronic-lethargy-1479773883 (suggesting returning the FDA to its original role of ensuring that approved drugs have demonstrated biological activity in fighting a disease and can be labeled for safe use); Ed Silverman, *Trump Is Considering a Radical To Lead FDA*, STAT (Dec. 12, 2016), https://www.statnews.com/2016/12/12/donald-trump-fda-oneill (describing Jim O'Neill's views on approval of prescription drugs). Importantly, at some level this proposal is incoherent. Safety cannot be assessed independently of efficacy. The FDA review process assesses the safety of drugs only in comparison to their efficacy for a particular indication. A safety profile that may be acceptable in the context of a drug that is effective at treating late-stage cancer may be entirely unacceptable for a vaccine administered to an otherwise healthy individual. If a drug turns out to have high efficacy, more safety concerns might be tolerated.

95. *See* 21st Century Cures Act, H.R. 34, 114th Cong. § 3022 (codified at 21 U.S.C. § 355g (2012)). The impact of this provision remains to be seen, as the FDA Commissioner has not yet established a draft framework for considering such evidence, as required by the Act. 21 U.S.C. § 355g(c)(1).

96. *See* RESULT Act, S. 2022, 115th Cong. (2017); *see also* Erika Lietzan, *Thoughts on "Reciprocal Marketing Approval,"* OBJECTIVE INTENT (Nov. 3, 2017), https://objectiveintent.blog/2017/11/03/thoughts-on-reciprocal-marketing-approval (describing why she calls the RESULT Act the "Send All of the FDA Employees Home Act of 2017").

97. Press Release, Senator Ted Cruz Press Office, Cruz, Lee Introduce the RESULT Act (Dec. 11, 2015), https://www.cruz.senate.gov/?p=press_release&id=2554; Gulfo, *supra* note 94.

98. Gulfo, *supra* note 94.

99. Senator Ted Cruz Press Office, *supra* note 97.

and drug prices might even go down.[100] The veracity of these predictions aside,[101] this Section focuses on another effect of these initiatives and others like them.

These initiatives would lead the FDA to approve more unsafe, ineffective drugs. Importantly, this is not meant pejoratively. It is meant as a statistical observation about the kind of question the FDA must answer when it approves a drug. The FDA must consider how to balance Type I and Type II errors in the approval process. As a matter of policy, one option would be for the FDA to focus on minimizing the number of unsafe or ineffective drugs that it approves (minimizing Type I errors). On this view, the FDA should not put its stamp of approval on drugs that harm patients or that do not work.[102] Over time, too many approvals of unsafe or ineffective drugs could erode public trust in the FDA as a tool for consumer protection.[103] More generally, this is the entire reason the FDA possesses the legal authority to screen pharmaceuticals for safety and efficacy. Scandals involving unsafe or ineffective drugs prompted Congress to give the FDA more and greater powers over the years, in large part to prevent such products coming to market in the first instance.[104]

Alternatively, a second option would be for the FDA to focus on minimizing the number of safe, effective drugs it *fails* to approve (minimizing Type II errors).[105] On this view, it is worse for

100. Gulfo, *supra* note 94.

101. The FDA already approves most drugs more quickly than its developed world counterparts (Europe, Canada, and Japan), so the set of drugs to which this applies is small. *See, e.g.*, Nicholas S. Downing et al., *Regulatory Review of Novel Therapeutics—Comparison of Three Regulatory Agencies*, 366 NEW ENG. J. MED. 2284, 2284 (2012); Matthieu Larochelle et al., *Assessing the Potential Clinical Impact of Reciprocal Drug Approval Legislation on Access to Novel Therapeutics in the USA: A Cohort Study*, 7 BMJ OPEN 1, 1 (2017).

102. *See* DANIEL CARPENTER, REPUTATION AND POWER 1–32 (2010) (describing the role of the FDA as gatekeeper).

103. *Id.* at 11.

104. *Id.* at 73, 228 (detailing the elixir sulfanilamide and thalidomide tragedies and their contribution to the enactment of legislation giving the FDA new powers).

105. Importantly, this is not truly an either or issue. It is consistent to require vaccines or other preventive interventions to undergo strict testing, as they are administered to healthy people, and at the same time speed drugs to market for deadly conditions where patients have no other treatment options. As discussed in Part I, *supra* text accompanying notes 73–80, there are already accelerated-approval systems in place to help accomplish this latter goal today, systems which may account for a larger percentage of the unsafe, ineffective

the FDA to deny patients access to a drug that is safe and effective than it is for the FDA to approve a drug that later is shown to be unsafe or ineffective. This view might still permit the FDA to screen out drugs with significant safety signals or reject drugs with no plausible mechanism of action, and this view might require postmarket surveillance studies. However, in general, this view holds that the FDA ought to be enabling sick patients to access drugs more quickly. This view of the FDA's role places greater responsibility on insurers, physicians, and patients to gather, process, and act on information about a drug's safety and efficacy.

Over the last few decades, the FDA has generally chosen to err on the side of minimizing the number of unsafe, ineffective drugs it approves (minimizing Type I errors).[106] Importantly, under this view the right number of approved unsafe, ineffective drugs is still *not zero*. The FDA certainly makes mistakes, and so although the "right" number in our current system is something small, it is not zero. These policy proposals envision a system in which the FDA approves many more drugs, the efficacy of which has not yet been tested in the real world or has been tested on a limited basis. They thus envision a system in which the right number of approved unsafe, ineffective drugs is much higher than it is right now, and certainly far higher than zero.

This position is entirely defensible. Proponents might argue that Type II errors are more visible and therefore fixable, as approved drugs can be studied further to examine potential safety signals, while unapproved drugs cannot be studied as easily.[107] When expressed publicly, however, defenders usually do not consider the full consequences the policy would create, precisely because of the link between FDA approval and insurance reimbursement. Insurers cannot easily sort out the efficacy of these unproven drugs and they will have no ability to demand additional information from manufacturers because they cannot decline to cover the drugs, even though their efficacy has not been demonstrated.

drugs approved at present. *See* Jonathan J. Darrow et al., *New FDA Breakthrough-Drug Category—Implications for Patients*, 370 NEW ENG. J. MED. 1252, 1253–54 (2014).

106. *See* Montazerhodjat & Lo, *supra* note 2, at 3. ("[T]he current standards of drug-approval are weighted more on avoiding a Type I error (approving ineffective therapies) rather than a Type II error (rejecting effective therapies).").

107. It is possible that drugs erroneously denied approval by the FDA applying strict safety and efficacy standards might be approved in other countries, providing opportunities for such study.

As a result, not only would these proposals lead to the approval of more unsafe, ineffective drugs—but Medicare and Medicaid would be required by law to cover nearly all of them. The idea that these proposals will somehow *decrease* drug spending is, therefore, difficult to understand. Reform of the FDA approval system without accompanying reform to insurance reimbursement would likely increase spending, not decrease it.[108]

B. CURBING DRUG PRICES AND SPENDING THROUGH MEDICARE AND MEDICAID

Similarly, policy proposals aiming to control drug prices and spending through government-run insurance programs overlook the linked nature of approval and reimbursement and would therefore not have the desired policy impact. On the Medicare side, the idea that permitting Medicare to negotiate drug prices will significantly reduce costs has captivated policymakers on both sides of the aisle.[109] And within the Medicaid program, some policymakers have contended that per-capita caps or other efforts to limit Medicaid spending will enable states to save on drug spending.[110] Neither of these arguments standing alone is accurate.

Policy arguments about permitting Medicare to negotiate for lower drug prices have their origin in a provision of the law establishing the Medicare Part D program that prohibits such conduct. Often referred to as the noninterference clause, the statute provides that the Secretary of Health and Human Services (HHS) "may not interfere with the negotiations between drug manufacturers and pharmacies and [Prescription Drug Plan] sponsors" and "may not require a particular formulary or institute a price structure for the reimbursement of covered part

108. This is somewhat of a perverse result, as those proposing such initiatives (like Senators Ted Cruz (R-TX) and Mike Lee (R-UT), sponsors of the RESULT Act) typically favor less federal spending on health care, rather than more.

109. *See* Juliette Cubanski & Tricia Neuman, *Searching for Savings in Medicare Drug Price Negotiations*, KAISER FAMILY FOUND. (Jan. 2017), http://files.kff.org/attachment/issue-brief-searching-for-savings-in-medicare-drug-price-negotiations ("In response to higher drug spending growth and heightened attention to drug prices, some policymakers have proposed allowing Medicare to negotiate the price of prescription drugs—a proposal supported by 82 percent of the public, including a majority of Democrats (93%), Republicans (68%), and Independents (85%).").

110. *See, e.g.*, Salvador Rizzo, *MacArthur Faces the Wrath of New Jersey*, OBSERVER (May 11, 2017), http://observer.com/2017/05/macarthur-faces-the-wrath-of-new-jersey.

D drugs."[111] Essentially, this section prohibits HHS from negotiating or setting prices in Medicare Part D.[112] The policy argument is therefore simple: if we permitted Medicare to negotiate on behalf of its fifty million enrollees,[113] it would be able to negotiate deeper discounts than the program is currently able to demand. President Obama continually proposed to repeal the noninterference clause at least in part in his proposed budgets,[114] and President Trump has suggested he would like to implement this policy as well.[115]

However, these arguments either do not appreciate or willfully ignore the Medicare coverage requirements set out in Part I—requirements which severely limit the program's bargaining power. Medicare might be able to achieve some savings where there is already market competition and where Medicare is permitted to cover two drugs in that class, although it is difficult to see why private plans have not negotiated such deals already. But for the six protected classes in which Medicare must cover all products, or for expensive new drugs with few, if any, substitutes, Medicare cannot walk away from the table if it does not like the deal companies are offering. This is why the Congressional Budget Office (CBO) estimated that providing Medicare with negotiating authority by itself "would have a negligible effect on Medicare drug spending."[116]

Importantly, negotiation authority could be coupled with other powers that would have such an impact. The CBO suggests that the "authority to establish a formulary"[117] is one such power. In other words, if Medicare was permitted to decline to

111. 42 U.S.C. § 1395w-111(i) (2012).

112. Importantly, this section does not apply to private entities who design and administer Part D plans. CONG. BUDGET OFFICE, COMPETITION AND THE COST OF MEDICARE'S PRESCRIPTION DRUG PROGRAM 25 (2014). They do negotiate prices, although their patient populations may be small relative to the pool of Medicare enrollees more generally. The idea is that Medicare, negotiating on behalf of all of its enrollees, would be able to leverage more bargaining power.

113. *Total Number of Medicare Beneficiaries (2015)*, KAISER FAMILY FOUND., https://www.kff.org/medicare/state-indicator/total-medicare-beneficiaries (last visited June 18, 2018).

114. Cubanski & Neuman, *supra* note 109.

115. *See* Alison Kodjak, *Medicare Should Leverage Buying Power to Pull Down Drug Prices, White House Says*, NPR (Feb. 7, 2017), http://www.npr.org/sections/health-shots/2017/02/07/513945538/white-house-says-medicare-should-leverage-its-buying-power-to-pull-down-drug-pri.

116. Letter from Peter R. Orszag, Director, Cong. Budget Office, to Ron Wyden, Senator 2 (Apr. 10, 2007), https://www.cbo.gov/sites/default/files/cbofiles/ftpdocs/79xx/doc7992/drugpricenegotiation.pdf.

117. *Id.*

cover a product when a pharmaceutical company refuses to deal fairly with Medicare in negotiations, it might be able to achieve savings.[118] Of course, this would mean delinking FDA approval and insurance reimbursement, potentially depriving at least some patients of access to drugs that would otherwise have been available to them.

A similar argument has been made in the context of the Medicaid program. Since its creation, Medicaid has been structured as an open-ended entitlement program. If states enroll more people in the program or provide them with more benefits in a particular year, the federal government will continue to pay for its share of the program. Particularly during economic downturns, when more individuals may lose their jobs and become eligible for Medicaid, the program expands to meet their needs.[119]

More recently, a number of Republican legislators have proposed funding Medicaid through finite block grants or per-capita caps, which would provide the states each year with either a fixed pot of money or a pot of money that is fixed on a per-enrollee basis.[120] The buzzword here is flexibility.[121] The thinking is that states faced more explicitly with finite resources will make more efficient choices about how to allocate their funding, perhaps cutting wasteful services or cutting rates on particular

118. This is not the only way to achieve such savings. The CBO also suggests that prices may simply be set administratively. *Id.* Scholars have suggested using binding arbitration as another option. *See, e.g.*, Richard G. Frank & Joseph P. Newhouse, *Should Drug Prices Be Negotiated Under Part D of Medicare? And If So, How?*, 27 HEALTH AFF. 33, 39–41 (2008) (discussing possible methods for setting drug prices, including arbitration).

119. David Gamage, *Preventing State Budget Crises: Managing the Fiscal Volatility Problem*, 98 CAL. L. REV. 749, 760 (2010) ("[M]ore state residents generally qualify for Medicaid during downturns . . . ."); Benjamin D. Sommers & Arnold M. Epstein, *Why States Are So Miffed About Medicaid—Economics, Politics, and the "Woodwork Effect,"* 365 NEW ENG. J. MED. 100, 100 (2011).

120. *See, e.g.*, American Health Care Act (AHCA) of 2017, H.R. 1628, 115th Cong. § 121; Better Care Reconciliation Act (BCRA) of 2017, Senate Amendment to H.R. 1628, 115th Cong. § 133.

121. *See, e.g.*, Eugene Scott, *Burgess Defends GOP Health Care Bill*, CNN (May 4, 2017), http://www.cnn.com/2017/05/04/politics/michael-burgess-health-care-bill-cnntv/index.html (quoting Representative Burgess saying state governors have asked Congress for flexibility); Bruce Westerman, *Medicaid Block Grants Give States More Freedom*, THE HILL (Mar. 21, 2017), http://thehill.com/blogs/congress-blog/healthcare/325097-medicaid-block-grants-give-states-more-freedom.

products or services.[122] Legislators have suggested that such caps would permit states to achieve savings on drug costs.[123]

It is difficult to see why this would be so. If Medicaid is required by law to cover essentially all FDA-approved drugs, it lacks the bargaining power to demand better prices on particular products. Perhaps some states would increase their use of step therapy or prior-authorization tactics and achieve some savings. But as states have been employing these efforts for decades, it is difficult to imagine they could achieve more than a marginal additional level of savings through these techniques.[124] Where proposed caps would cut Medicaid funding levels by twenty-five percent or more over the next decade,[125] no program can absorb such cuts through incremental gains.

Since prescription drugs are an optional category of coverage for Medicaid, states could decline to cover them entirely,[126] although that would be an extreme solution. More likely, states would pressure Congress to permit them to set formularies in Medicaid, using partial delinkage to create bargaining power, but also limiting access to such drugs.[127]

The link between FDA approval and insurance reimbursement is also the reason a number of state-level ballot initiatives attempting to control drug costs would be ineffective. California and Ohio have considered (and ultimately rejected) ballot initiatives that propose to cap what drug manufacturers can charge to public payers in the state (including Medicaid) at the price paid

122. Aaron E. Carroll, *How Would Republican Plans for Medicaid Block Grants Actually Work?*, N.Y. TIMES (Feb. 6, 2017), https://www.nytimes.com/2017/02/06/upshot/how-would-republican-plans-for-medicaid-block-grants-actually-work.html.

123. Rizzo, *supra* note 110 (quoting Representative Tom MacArthur that Republican proposals would help drive down drug prices).

124. *But see* David Dranove et al., *A Dose of Managed Care: Controlling Drug Spending in Medicaid* 3 (Nat'l Bureau of Econ. Research, Working Paper No. 23956, 2017) (finding privatization of state Medicaid drug programs greatly reduced costs).

125. CONG. BUDGET OFFICE, LONGER-TERM EFFECTS OF THE BETTER CARE RECONCILIATION ACT OF 2017 ON MEDICAID SPENDING 1 (2017) (concluding that BCRA would lower Medicaid spending by twenty-six percent in 2026, a number which would increase to thirty-five percent by 2036).

126. *See* Peter R. Orszag, *One Nightmare Scenario in Senate Bill: Drug Rationing*, BLOOMBERG VIEW (June 28, 2017), https://www.bloomberg.com/view/articles/2017-06-28/one-nightmare-scenario-in-senate-bill-drug-rationing (discussing how states may need to ration drug access to control costs).

127. *Id.*

by the VA.[128] But the VA is permitted to establish drug formularies and decline to cover drugs that are too expensive.[129] State Medicaid programs are legally obligated to cover the relevant products and so do not clearly have the bargaining power to demand that they pay the same prices as the VA. The VA can get up and walk away from the table—Medicaid cannot.[130]

III. HYPOTHESIZING DELINKAGE

If academics and policymakers would like to implement policy changes of the type described in Part II but are unable to do so because of the link between these policy proposals and required drug coverage,[131] one possibility is to delink the programs. It is important to consider both the positive and negative potential implications if approval and reimbursement were delinked. This Part considers three main policy consequences that might be expected to result from delinkage, although their precise reach undoubtedly depends on the scope of revisions made to existing law and the relative sizes of the markets at issue.

A. REDUCING ACCESS TO MEDICINES

The first and potentially most important consequence that might result from delinkage is a reduction in access to certain medicines. The concern is that if insurers (especially Medicare and Medicaid) are no longer legally required to cover certain drugs, they will no longer choose to. Whether this result is of real social concern depends on how valuable these excluded drugs are

128. Renee Hickman, *Ohio Takes Drug Price Measure to Voting Booth*, BLOOMBERG BNA (July 19, 2017), https://www.bna.com/ohio-takes-drug-n73014461943.

129. Austin B. Frakt et al., *Should Medicare Adopt the Veterans Health Administration Formulary?*, 21 HEALTH ECON. 485, 487 (2012).

130. There is also a first-order barrier to the implementation of these initiatives. Capping state prices at VA prices seems to require the state to know what the VA is paying for a drug. However, the prices paid by the VA are generally not public. *See* Mike McCaughan et al., *Health Policy Brief: Veterans Health Administration*, HEALTH AFFAIRS (Aug. 10, 2017), https://www.healthaffairs.org/do/10.1377/hpb20171008.000174/full. Nothing in either state initiative requires pharmaceutical companies to disclose these prices to the relevant state actors, and it is not clear that the states would have the ability to access the information otherwise.

131. The sense in which policymakers would be unable to implement these changes is different for the two sets of reforms. In the case of policymakers who seek to speed the FDA approval process, they may or may not seek to limit public spending on these newly approved drugs. In the case of policymakers who seek to enable Medicare to use more market-based tools to control prices, those initiatives will not be possible without delinkage.

to individual patients. Although it is difficult to specify with certainty what insurers might do in such a case, we can glean some potential concerning outcomes from existing insurer efforts to exclude drugs from coverage.

First, expensive orphan drugs are likely to be a target for exclusion, even where only a small number of patients rely on them.[132] Consider the case of Kalydeco, a drug approved for the treatment of cystic fibrosis in a subset of patients with a particular genetic mutation.[133] Kalydeco may significantly improve the disease's symptoms in that small group of patients, but its list price is over $300,000 per patient per year[134]—for a drug that patients may take for their entire lives. Although Medicaid programs are entitled to significant discounts off of this list price,[135] it is easy to see how state budgets can be strained by a few patients needing expensive drugs like this one.

After Kalydeco's approval, many state Medicaid programs aimed to limit the patients who could obtain the drug beyond the genetic limitations already imposed by the FDA. For instance, the programs required patients to demonstrate first that they had failed to respond to older, less expensive therapies. Patients objected, and three patients even sued Arkansas for denying them access to Kalydeco.[136] The parties settled the case, with Arkansas changing its eligibility criteria for Kalydeco.[137] But we

132. As defined by the Orphan Drug Act, these are drugs approved for the treatment of a disease or condition which "affects less than 200,000 persons in the United States." 21 U.S.C. § 360bb(a)(2) (2012).

133. FDA, PRESCRIBING INFORMATION: KALYDECO 2 (Feb. 2017), https://www.accessdata.fda.gov/drugsatfda_docs/label/2017/203188s022l_207925s003lbl.pdf (listing the mutations patients may have for which Kalydeco is approved).

134. *See* Matthew Herper, *For Vertex Pharmaceuticals, Can One Billion-Dollar Breakthrough Beget Another?*, FORBES (Aug. 8, 2017), https://www.forbes.com/sites/matthewherper/2017/08/08/vertex-pharmaceuticals-and-the-price-of-inspiration.

135. 42 U.S.C. § 1396r-8(c)(1)(B)(i)(VI) (2012) (setting the "minimum rebate percentage" for rebate periods after December 31, 2009 at 23.1%).

136. Arkansas has said that "[c]ost alone was not the determining factor" in imposing these restrictions on Kalydeco. But internal state emails showed that state officials expressed concern about the cost. *See* Joseph Walker, *Costly Vertex Drug Is Denied, and Medicaid Patients Sue*, WALL ST. J. (July 16, 2014), https://www.wsj.com/articles/costly-drug-vertex-is-denied-and-medicaid-patients-sue-1405564205.

137. Arkansas had actually changed its eligibility criteria for Kalydeco after the initiation of the lawsuit but before the settlement occurred, and they technically agreed to maintain those new eligibility criteria. Walker, *supra* note 65.

might expect states to exclude these drugs going forward if coverage is not required.[138]

Second, payers might balk at covering even less-expensive cures, if the aggregate budgetary impact is sufficiently high. The recently developed hepatitis C cures are the primary example here. The first of these drugs to be approved, Sovaldi, retailed for $84,000 per course.[139] Although the drug cures hepatitis C in the vast majority of patients, making the drug a one-time expenditure rather than a chronic expense, states could not afford to provide the drug to all of their hepatitis C patients enrolled in Medicaid.[140] Medicare experienced a similar spike in spending once the drugs were introduced.[141]

Most state Medicaid programs initially restricted access to these drugs beyond what was deemed medically necessary. For instance, many states required patients to demonstrate particularly severe levels of liver disease or to demonstrate their abstinence from the use of illegal substances for particular periods of time before providing access to the medications.[142]

As in the case of Kalydeco, these restrictions were met with lawsuits or demand letters in a number of states,[143] with the

138. *See, e.g.*, Letter from Thomas J. Betlach, President, Nat'l Ass'n of Medicaid Dirs. (NAMD), & John B. McCarthy, Vice President, NAMD, to Fred Upton, Representative & Frank Pallone, Representative 2 (Apr. 8, 2015), http://www.medicaiddirectors.org/wp-content/uploads/2015/08/namd_letter_to_congress_21st_century_cures.pdf.

139. NAT'L ACADS. OF SCI. ENG'G & MED., A NATIONAL STRATEGY FOR THE ELIMINATION OF HEPATITIS B AND C: PHASE TWO REPORT 151 (Gillian J. Buckley & Brian L. Strom eds., 2017). Again, importantly, state Medicaid programs were able to obtain these drugs at significant discounts, especially once competition was introduced into the market with the approval of Viekira Pak months later. *See id.* at 165.

140. *See* Joshua Sharfstein et al., *We Have a Cure for Hepatitis C. but the Neediest Can't Afford It. Louisiana Wants to Change That.*, VOX (Sept. 27, 2017), https://www.vox.com/science-and-health/2017/9/27/16350562/hepatitis-c-drug-prices-louisiana.

141. Charles Ornstein, *New Hepatitis C Drugs Are Costing Medicare Billions*, WASH. POST (Mar. 29, 2015), https://www.washingtonpost.com/national/health-science/medicare-spent-45-billion-on-new-hepatitis-c-drugs-last-year-data-shows/2015/03/29/66952dde-d32a-11e4-a62f-ee745911a4ff_story.html.

142. *See* CTR. FOR HEALTH LAW & POLICY INNOVATION, HEPATITIS C: THE STATE OF MEDICAID ACCESS 5–9 (2016).

143. *See, e.g.*, JoNel Aleccia, *Lawsuit Targets Medicaid Policy that Limits Spendy Hepatitis C Drugs*, SEATTLE TIMES (Feb. 17, 2016), https://www.seattletimes.com/news//lawsuit-targets-medicaid-policy-that-limits-spendy-hep-c-drugs; *ACLU Files Class Action Lawsuit Against Colorado Medicaid over Unlawful Hepatitis C Treatment Restrictions*, ACLU COLO. (Sept. 19, 2016), https://aclu-co.org/aclu-files-class-action-lawsuit-colorado-medicaid-unlawful-hepatitis-c-treatment-restrictions; Jen Rini, *State Changes Hep C Medication*

cases resolved in the patients' favor.[144] Drugs like these may also be excluded, or at least significantly limited, if coverage is not required.

Finally, in the private-insurance market, we may expect companies to engage in business practices designed to attract healthy, low-cost individuals to their plans and discourage sicker, high-cost individuals from enrolling. This practice, referred to as "cream skimming," has been known in the literature for decades.[145] The ACA made it more difficult for insurers to engage in these practices, through the essential health benefits requirement and the nondiscrimination provisions of the Act.[146]

But discrimination has persisted. Recent lawsuits alleged that insurance companies were discriminating against patients with HIV/AIDS in an effort to discourage the patients from signing up for their plans. Specifically, the insurers would place most of the drugs needed for this condition in the highest cost-sharing tier, requiring patients to pay far more out of pocket for their treatment.[147] Alternatively, they would decline to cover a sufficient number of drugs within each category, preventing physicians from providing their patients with the most effective treatment options.[148] Practices like these would likely increase if coverage were not required on nondiscriminatory terms.

Importantly, in none of these three cases is there a question about whether the drugs are effective. There may be questions about whether the efficacy produced by these drugs is worth their price, but in each case, there is clear evidence to support the use of these drugs in at least some patient populations.[149] As

Guidelines, Avoids Lawsuit, NEWS JOURNAL (June 7, 2016), https://www.delawareonline.com/story/news/health/2016/06/07/state-changes-hep-c-medication-guidelines-avoids-lawsuit/85554396.

144. *See, e.g.*, JoNel Aleccia, *Judge Orders Washington Medicaid To Provide Lifesaving Hepatitis C Drugs for All*, SEATTLE TIMES (May 28, 2016), https://www.seattletimes.com/seattle-news/health/judge-orders-apple-health-to-cover-hepatitis-c-drugs-for-all.

145. *See, e.g.*, Joseph P. Newhouse, *Cream Skimming, Asymmetric Information, and a Competitive Insurance Market*, 3 J. HEALTH ECON. 97, 97 (1984).

146. Patient Protection and Affordable Care Act, 42 U.S.C. § 18116 (2012).

147. Michelle Andrews, *Seven Insurers Alleged to Have Discriminated Against HIV Patients*, NPR (Oct. 18, 2016), https://www.npr.org/sections/health-shots/2016/10/18/498427561/7-insurers-alleged-to-have-discrimated-against-hiv-patients.

148. *See, e.g.*, Office for Civil Rights Administrative Complaint at 5–7, *In re* Anthem Silver Level QHPs in Wisconsin (Sept. 6, 2016), https://www.chlpi.org/wp-content/uploads/2013/12/WI-ANTHEM.pdf.

149. *See* Andrews, *supra* note 147; Herper, *supra* note 134; Sharfstein et al., *supra* note 140.

such, insurer practices like these should give us pause about the idea of delinking approval and reimbursement without appropriate safeguards. If Medicare or Medicaid had been aiming to exclude a subset of drugs which have been approved with insufficient evidence of their efficacy,[150] that might produce less concerning results. But if we could expect payers to exclude or discriminate against patients where the drugs are highly effective, we ought to be concerned about that potential policy outcome from an access perspective.

B. ENCOURAGING INFORMATION PRODUCTION

Other potential policy implications of delinkage are more positive. Perhaps most usefully for future innovation, if approval and reimbursement were delinked, pharmaceutical companies would know that they must earn insurance coverage. As such, they might choose to run their clinical trials differently, to produce more socially valuable information. This is likely to be true in at least two senses. First, we might gain more information about the social value of particular drugs in an objective sense. And second, we might gain more information about the comparative efficacy of particular products within a class.

First, public payers in particular might decline to pay for FDA-approved products which have not demonstrated sufficient evidence of safety and efficacy. More specifically, they might decline to pay for products that were approved on the basis of questionable surrogate endpoints. "A surrogate endpoint . . . is a laboratory measurement or a physical sign used as a substitute for a clinically meaningful endpoint that measures directly how a patient feels, functions, or survives."[151] A classic example of a surrogate endpoint is cholesterol. Drugs may be tested based on their ability to lower a patient's level of cholesterol, a surrogate endpoint, rather than on their ability to decrease the risk of death from heart disease, the true endpoint. If drugs approved on the basis of their ability to lower cholesterol levels do not actually lower the risk of death from heart disease,[152] payers may

150. *See infra* text accompanying notes 215–26 (discussing Massachusetts's proposed section 1115 waiver).

151. *See* Robert J. Temple, *A Regulatory Authority's Opinion About Surrogate Endpoints*, *in* CLINICAL MEASUREMENT IN DRUG EVALUATION 3, 4 (Walter S. Nimmo & Geoffrey T. Tucker eds., 1995); *see also* 21 C.F.R. § 314.510 (2017) (explaining that a new drug may be given market approval if the drug impacts a surrogate endpoint).

152. *See, e.g.*, Brendan M. Everett et al., *Reducing LDL with PCSK9 Inhibitors—the Clinical Benefit of Lipid Drugs*, 373 NEW ENG. J. MED. 1588, 1589–90

be reticent to pay for new drugs which do not come with evidence of the true endpoint.

There are some surrogate endpoints which may have value on their own. Consider cancer drug approvals. Two-thirds of cancer drugs are now approved on the basis of surrogate endpoints, such as whether solid tumors have shrunk, or how long the patient was able to survive without their cancer progressing or recurring.[153] Yet often there is no evidence as to whether these drugs actually enable patients to live longer—and in many cases there is evidence that the drug has no overall survival benefit.[154] It may be that cancer patients value progression-free survival even if there is no overall survival benefit.[155] But payers, patients, and physicians would benefit from knowing both pieces of information about a particular product.

Second, in a delinked reimbursement world we may gain additional information about the comparative costs and benefits of different drugs in a particular class. Today, this information is scarce. Fewer drugs are approved on the basis of clinical trials involving competing products, either direct or indirect competitors. For rare conditions and particularly for rare cancers, it is increasingly common for products to be approved on the basis of a single-arm trial,[156] in which the effects of the therapy to be tested are not compared to the effects of any other intervention, either placebo or comparator. In such circumstances, it may be difficult for physicians to decide which new products they should prescribe for their patients, where a choice is permissible.[157]

(2015).

153. Chul Kim & Vinay Prasad, *Cancer Drugs Approved on the Basis of a Surrogate End Point and Subsequent Overall Survival: An Analysis of Five Years of U.S. Food and Drug Administration Approvals*, 175 J. AM. MED. ASS'N INTERNAL MED. 1992, 1992 (2015).

154. *Id.* at 1993.

155. *See* Lesley J. Fallowfield & Anne Fleissig, *The Value of Progression-Free Survival to Patients with Advanced-Stage Cancer*, 9 NATURE REVIEWS CLINICAL ONCOLOGY 41, 45 (2012) (discussing an end-of-life care study in which seventy-two percent of patients preferred symptom-directed therapy over life-extending therapy).

156. *See, e.g.*, Himabindu Gaddipati et al., *Rare Cancer Trial Design: Lessons from FDA Approvals*, 18 CLINICAL CANCER RES. 5172, 5176 (2012).

157. INST. FOR CLINICAL & ECON. REVIEW, POLY ADP-RIBOSE POLYMERASE (PARP) INHIBITORS FOR OVARIAN CANCER: EFFECTIVENESS AND VALUE 38 (2017), https://icer-review.org/wp-content/uploads/2017/02/MWCEPAC_OVARIAN_FINAL_EVIDENCE_REPORT_10112017.pdf.

Companies also have little incentive to demonstrate the safety and efficacy of their products relative to potential competitors, as long as insurers must cover both. Currently, in order to demonstrate the comparative benefit of their product, a company may need to run a particularly large, expensive trial, powered to detect potential differences between two similar products.[158] Further, the company runs the risk that their trial may show no benefit relative to their competitor, or that their competitor may even emerge superior.[159] Thus companies are unlikely to expend the time and money to conduct these trials where the result may harm, not help, their market share.

Giving payers more control over the choice of products they cover and the organization of their formularies may improve pharmaceutical companies' incentives to produce more information about their products and to differentiate between products in a class through the development of comparative-effectiveness data. Payers might decline to cover drugs approved on the basis of more novel surrogate endpoints, or might manage to strike innovative contracting deals (such as money-back requirements) to cover such drugs until sufficient data is produced. Further, payers would likely give preferred formulary placement to drugs which can demonstrate superior safety or efficacy. Evidence-based physicians are likely to support these efforts, as it would enable them to determine which drug in a particular class would best fit the needs of their patients.

C. ADDRESSING THE DRUG PRICING PROBLEM

Third, delinkage also would likely help address the problems of high drug prices and spending, precisely because of both of the above considerations addressed in this Part. There is general agreement that drug prices are too high,[160] although, to be sure, there is much less agreement as to *which* drug prices are

158. *See generally* Daniel Garrun, *Clinical Trial Delays: America's Patient Recruitment Dilemma*, DRUG DEV. TECH. (July 18, 2012), http://www.drugdevelopment-technology.com/features/featureclinical-trial-patient-recruitment (discussing the obstacles clinical trials face in both expense and volunteer recruitment).

159. Amy Kapczynski & Talha Syed, *The Continuum of Excludability and the Limits of Patents*, 122 YALE L.J. 1900, 1923–28 (2013) ("[T]here are asymmetrical incentives to provide positive and negative information about new drugs.").

160. *See* FDA Reauthorization Act of 2017, Pub. L. No. 115-52, § 609, 131 Stat. 1005, 1051 (expressing the need for Congress to take action to "lower the costs of prescription drugs"); Cubanski & Neuman, *supra* note 109.

too high, and whether it is individual or aggregate costs that are more problematic. For instance, some products may not be considered worth their high prices because they do not provide significant social value, while others may be worth their prices but impose budgetary concerns in the aggregate.[161] Nevertheless, pharmaceuticals make up a significant share of overall healthcare spending, and pharmaceutical spending is growing quickly,[162] such that more serious efforts to lower either unit prices or overall spending may soon be necessary.

Delinkage can help address these problems. A payer that can credibly follow through on the threat not to cover a particular product can likely extract greater discounts in agreeing to cover it. Alternatively, the payer could nudge patients toward cheaper but similarly effective products through formulary management. Relatedly, our ability to distinguish between high-value and low-value pharmaceuticals may improve with an increased amount of comparative-effectiveness research. This research may enable payers to offer better quality of care at the same prices they had previously been paying, and to promote optimal treatment incentives among physicians and patients.

IV. CONSIDERING ALTERNATIVE MODELS

A logical question to ask at this point is whether there are systems in which delinkage has occurred that might serve as models to interrogate this thought experiment. Specifically, we might look at different models of delinkage and consider whether these predictions have been met. There are at least three potential delinkage models to consider: (1) the U.S. Department of Veterans Affairs (VA); (2) the pharmaceutical approval and reimbursement system in a number of European countries; and (3) the system of medical device approvals in the United States. These models reveal that delinkage is likely to result in decreased access but also decreased prices, as predicted in Part III.

161. Ari B. Friedman & Janet Weiner, *What's the Story with Drug Prices?: The Plot Thickens*, LEONARD DAVIS INST. HEALTH ECON. (May 30, 2016), https://ldi.upenn.edu/healthpolicysense/what's-story-drug-prices.

162. *National Health Expenditures 2016 Highlights*, CTRS. FOR MEDICARE & MEDICAID SERVS., https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/downloads/highlights.pdf (last visited June 18, 2018) (noting that retail prescription drug spending grew by 8.9% in 2015, to encompass ten percent of overall health expenditures). Importantly, this is an underestimate, as it does not account for drugs administered through the Part B program.

However, the potential for using delinkage to develop more information about drugs may be more difficult to realize.

Importantly, none of these three models provides perfect information about what is likely to happen in the event of delinkage. The VA example exists within the United States, but its overall magnitude and interaction with the rest of the healthcare system may impair its generalizability. The European example approaches a similar scale, but the different regulatory culture and market incentives may limit its applicability as well. Finally, the medical-device approval system is premised on a very different view of the FDA than is the drug approval system, limiting its relevance. As a result, the fact that we fail to see increased information production in any of these three cases may not be dispositive.

But these results still matter for policy-based assessments of delinkage's impact. At its core, delinkage by necessity will have some negative impact on access, even if it is narrowly tailored, and will likely also have a salutary impact on drug pricing. This tradeoff is one that policymakers in the United States have so far been unwilling to make.[163] Where the relative magnitude of these potential changes is unknown, there is understandable concern about making this tradeoff. However, if there is an additional social benefit to delinkage in the form of increased information production about these therapies, that benefit might embolden policymakers to take steps toward delinkage. If we have reason to doubt that there would be such an additional benefit, that doubt is similarly important to policymakers as they make decisions about drug pricing policy.

163. In part, their unwillingness is also driven by concerns about the relationship between price and innovation. When faced with the prospect of policy reforms that have the potential to lower their revenues, pharmaceutical companies respond by arguing that their ability to innovate and to develop new drugs will be impaired. *See, e.g.*, Jay Taylor, *Government-Imposed Price Controls Threaten Innovation and Access*, PHRMA: THE CATALYST (May 9, 2017), https://catalyst.phrma.org/government-imposed-price-controls-threaten-innovation-and-access. This Article does not grapple directly with their arguments, other than to present them obliquely *infra* in Part IV.C, but it is reasonable to think that the scope and content of any proposed price reform would matter in the innovation calculus.

A. THE DEPARTMENT OF VETERANS AFFAIRS

In the United States, many veterans are entitled to publicly funded health care through the VA.[164] Like Medicaid, the VA is entitled by law to a large statutory discount—twenty-four percent—off of the nonfederal AMP for the product.[165] But unlike Medicaid, the VA is further entitled to create formularies and exclude particular drugs from coverage.[166] Because these formularies are created at the national level,[167] the VA can leverage its purchasing power to obtain greater discounts on particular products.

The VA's program clearly bears out two of the three hypotheses described in Part III. First, the VA's program lowers prices significantly as compared to Medicare and Medicaid. Estimates suggest that the VA pays on average sixty percent of the prices paid by Part D plans.[168] And although the large statutory discounts available to Medicaid bring its prices closer to the range paid by the VA, estimates suggest that even Medicaid pays more than the VA for a significant minority of drugs.[169]

Second, the VA's program does lead to some decrease in access. One study noted that although private Medicare Part D plans cover on average eighty-five percent of the top-selling 200 drugs in the country, the VA national formulary covers only fifty-nine percent of these drugs.[170] As noted above, whether this decrease in access is a problem depends on the type and value of drugs being excluded from the formulary.[171] But the fact that

164. Most veterans must meet a minimum duty requirement first. *Health Benefits: Veterans Eligibility*, U.S. DEP'T VETERANS AFF., https://www.va.gov/healthbenefits/apply/veterans.asp (last visited June 18, 2018).

165. 38 U.S.C. § 8126(a)(2) (2012).

166. Austin B. Frakt et al., *Controlling Prescription Drug Costs: Regulation and the Role of Interest Groups in Medicare and the Veterans Health Administration*, 33 J. HEALTH POL. POL'Y & L. 1079, 1081 (2008).

167. *Id.* at 1087.

168. Austin B. Frakt et al., *Should Medicare Adopt the Veterans Health Administration Formulary?*, 21 HEALTH ECON. 485, 487 (2012).

169. Thomas J. Hwang & Aaron S. Kesselheim, *Public Referendum on Drug Prices in the US: Will It Bring Relief?*, 355 BRITISH MED. J. 1, 2 (2016) (estimating that Medicaid likely pays more than the VA for thirty-three percent of drugs by thirty percent on average). The VA is statutorily excluded from the calculation of the Medicaid best-price rule, as discussed throughout this Article. 42 U.S.C. § 1396r-8(c)(1)(C)(i)(I) (2012).

170. Frakt et al., *supra* note 168, at 490–91.

171. At least initially, the VA imposed restrictions on access to the new hepatitis C drugs similar to what has been observed in the Medicaid context. Patricia Kime, *VA Expands Hepatitis C Treatment to All Patients with the Virus*, MILITARY TIMES (Mar. 9, 2016), https://www.militarytimes.com/veterans/

roughly one-third of VA patients with Medicare report having additional prescription drug coverage through Part D plans suggests that they are using such coverage to supplement the VA's more restrictive formulary.[172]

However, it does not appear that the VA's delinkage has had much of an impact on the development of information about the comparative effectiveness of different products approved for the same indication. Importantly, the VA is hoping to change this. In mid-2017, the agency announced a partnership with the Institute for Clinical and Economic Review (ICER) to better enable the agency to develop and use information about comparative clinical effectiveness and cost effectiveness in their formulary management process.[173] Further, the VA is a relatively small program as compared to Medicare and Medicaid, serving 8.9 million Americans.[174] In spending terms, the VA estimates that it will spend just under seven billion dollars on drugs in 2017,[175] far less than either Medicare or Medicaid.[176] It may be that the size of the delinked market is insufficient to spur companies to produce information that could then be used in the larger, linked market.

B. EUROPEAN DELINKAGE MODELS

A second model of delinkage exists in most European countries. In these systems, approval by the European Medicines Agency (EMA) or national regulator does not dictate coverage requirements or terms on a national level. Consider the United Kingdom's system, perhaps the most well-studied model. Once a

2016/03/09/va-expands-hepatitis-c-treatment-to-all-patients-with-the-virus; Rini, *supra* note 143.

172. GRACE HUANG ET AL., 2016 SURVEY OF VETERAN ENROLLEES' HEALTH AND USE OF HEALTH CARE 47 (2017), https://www.va.gov/HEALTHPOLICYPLANNING/SoE2016/2016_Survey_of_Veteran_Enrollees_Health_and_Health_Care.pdf. VA patients who have private insurance overwhelmingly report (eighty-two percent) that their private insurance includes a prescription benefit. *Id.* at 46.

173. Press Release, Institute for Clinical and Economic Review, The Institute for Clinical and Economic Review To Collaborate with the Department of Veterans Affairs' Pharmacy Benefits Management Services Office (June 27, 2017), https://icer-review.org/announcements/va-release.

174. HUANG ET AL., *supra* note 172, at 2.

175. U.S. DEP'T VETERANS AFF., MEDICAL PROGRAMS AND INFORMATION TECHNOLOGY PROGRAMS VHA-207 (2017), https://www.va.gov/budget/docs/summary/fy2018VabudgetVolumeIImedicalProgramsAndInformationTechnology.pdf.

176. *See Medicare Drug Spending Dashboard 2015*, *supra* note 38.

drug is approved by the Medicines and Healthcare Products Regulatory Agency (MHRA) or the EMA, the National Institute for Health and Care Excellence (NICE) conducts "technology appraisals"[177] on new drugs and makes recommendations to the United Kingdom National Health Service (NHS) regarding reimbursement and use of the drug.[178] NICE considers not just the clinical evidence for the drug, but also its economic evidence—does the drug represent good value for money? NICE is likely to recommend drugs for coverage by the NHS where the cost per quality-adjusted life year (QALY) is between £20,000 and £30,000 per QALY gained.[179]

NICE's technology-appraisal system, coupled with the lack of a coverage mandate, means that nearly all branded drugs are less expensive in the United Kingdom than they are through Medicare.[180] But this decrease in price does come with a decrease in access. NICE does not recommend that all drugs be covered, and for certain types of drugs—particularly expensive cancer drugs—this lack of coverage has created political problems for the program.[181] In 2011, the NHS created the Cancer Drugs Fund, devoting over £200 million to provide cancer drugs not covered by the NHS.[182] While the Fund was originally scheduled to end in 2014, it was extended until 2016 with its expenditure during 2015–2016 amounting to £466 million.[183] The United Kingdom has now implemented a new model for appraising and

177. *Technology Appraisal Guidance*, NAT'L INST. FOR HEALTH & CARE EXCELLENCE, https://www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-technology-appraisal-guidance (last visited June 18, 2018).

178. *Id.*

179. Of course, if the incremental cost-effectiveness ratio is less than £20,000, NHS would prefer that. NICE has even created a fast track appraisal (FTA) process for the most cost-effective treatments, where "the company's base-case incremental cost-effectiveness ratio (ICER) is less than £10,000 per quality-adjusted life year (QALY) gained." *Our Processes*, NAT'L INST. FOR HEALTH & CARE EXCELLENCE, https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/process (last visited June 18, 2018).

180. *See* Jeanne Whalen, *Why the U.S. Pays More than Other Countries for Drugs*, WALL ST. J. (Dec. 1, 2015), https://www.wsj.com/articles/why-the-u-s-pays-more-than-other-countries-for-drugs-1448939481.

181. *See, e.g.*, *Health Economics: The Cancer Drugs Cost Conundrum*, CANCER RES. UK (Aug. 10, 2016), http://www.cancerresearchuk.org/funding-for-researchers/research-features/2016-08-10-health-economics-the-cancer-drugs-cost-conundrum.

182. NHS ENGLAND CANCER DRUGS FUND TEAM, APPRAISAL AND FUNDING OF CANCER DRUGS 5 (July 2016), https://www.england.nhs.uk/wp-content/uploads/2013/04/cdf-sop.pdf.

183. *Id.*

reimbursing all cancer drugs under the Fund,[184] which includes a new category of recommendations when NICE considers there to be "plausible potential for a drug to satisfy the criteria for routine commissioning, but where there is significant remaining clinical uncertainty."[185]

This new program is designed to encourage the development of additional clinical information about these products even after they come to market. Once NICE recommends a drug as a Fund candidate under this new category, the drug's final coverage through the fund depends on the pharmaceutical company accepting the requirements of the Fund under a Managed Access Agreement,[186] which includes the Fund Commercial Agreement.[187] This Agreement is "a confidential agreement between NHS England and the pharmaceutical company, with input from NICE," and determines the level of reimbursement during the managed-access period.[188] When there is sufficient data to address the original clinical uncertainty, the drug begins the process of exiting the Fund.[189] NICE then reappraises the drug resulting in a positive or negative recommendation for routine commissioning.[190] With this new model of appraisal and funding for cancer drugs, NHS hopes to provide patients with increased access to these medications, to "drive stronger value for money for taxpayers in drugs expenditure," and to offer "a new fast-track route to NHS funding" for pharmaceutical companies willing to price drugs responsibly.[191]

To date, the delinked systems present throughout much of Europe have not produced the kind of additional information about comparative effectiveness that might be the result of delinkage, as hypothesized in Part III.C.[192] The United Kingdom in particular has produced a wealth of *cost-effectiveness* information about these products, but comparative effectiveness data about drugs in a particular class are still lacking. It may be that a more carefully designed system, on a broader scale, would be needed to produce that information.

184. *Id.*
185. *Id.* at 6.
186. *Id.* at 16.
187. *Id.* at 17.
188. *Id.* at 18.
189. *Id.* at 22–23.
190. *Id.*
191. *Id.* at 6.
192. *See supra* Part III.C.

C. MEDICAL DEVICE REGULATION

In the United States, there has historically been little or no link between FDA approval and insurance reimbursement for medical devices, particularly through CMS. As a result, medical-device companies have had to navigate two separate regulatory systems: they must both obtain FDA approval and proceed through CMS's national-coverage determination to secure Medicare reimbursement for their device.[193] But the FDA and CMS apply different legal standards to those determinations,[194] resulting in substantial uncertainty for device companies about whether the information generated in the FDA approval process will be sufficient to support a CMS coverage determination.[195] Even where a company has produced sufficient information, the additional time required to go through the CMS coverage determination process after FDA approval is costly both for the company and for patients who may want to access the device in question.

The system of medical-device approval and coverage in the United States is in some ways too distinct from our regulatory structure around drugs to compare directly the impact of this delinkage on price, access, and the development of comparative-effectiveness information. Many devices are regulated only lightly[196] or using an abbreviated follow-on pathway, in a way

193. LIZ RICHARDSON, HEALTH POLICY BRIEF: ALIGNING FDA AND CMS REVIEW 2 (Aug. 27, 2015), https://www.healthaffairs.org/do/10.1377/hpb20150827.132391/full/healthpolicybrief_143.pdf.

194. The FDA ensures that devices are "safe and effective." 21 U.S.C. § 360e(c)(1)(A) (2012) (providing that applicants for medical device premarket approval must show "whether or not such device is safe and effective"). CMS covers products that are "reasonable and necessary." *See* 42 U.S.C. § 1395y(a)(1)(A) (2012) (providing that "no payment may be made . . . for any expenses incurred for items or services . . . not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member"); *see also* 21 U.S.C. § 393(b)(2)(B) (describing part of the FDA's mission as "ensuring that . . . human and veterinary drugs are safe and effective").

195. RICHARDSON, *supra* note 193 ("This can lead to cases in which the FDA approves a product that is subsequently denied Medicare coverage because the evidence collected in pivotal clinical trials does not meet the 'reasonable and necessary' bar.").

196. Medical devices are regulated under a risk-based framework. Under this system, low-risk (designated as Class I) devices, such as tongue depressors, are subject only to "general controls," such as reporting and adherence to good manufacturing practices. 21 U.S.C. § 360c(a)(1)(A). By contrast, high-risk (designated as Class III) devices, such as artificial hearts, are subject to more stringent controls, typically including premarket approval requirements. 21 U.S.C. § 360c(a)(1)(C); *see also* Rachel E. Sachs, *Innovation Law and Policy: Preserving*

that does not resemble the pharmaceutical approval pathway.[197] Many laboratory diagnostics (which arguably fall under the statutory definition of "device")[198] are essentially unregulated by the FDA,[199] and CMS even sets rates for their reimbursement.[200] This divergent approval structure means that it is difficult to determine whether we should expect to observe the development of comparative-effectiveness information in the device context. However, there is still an important lesson to be gleaned from the system of medical-device approval and coverage.

Specifically, the medical-device system provides a window into how the regulated industry may react when faced with two separate regulatory systems they must satisfy. Medical-device companies do not like having to deal with separate regulators. They complain about the cost and uncertainty of the process, and they argue that it makes attracting venture capital funding for innovation difficult.[201] They would undoubtedly prefer to have FDA approval automatically trigger insurance reimbursement.

Pharmaceutical companies worry even today about the burdens placed on them by the FDA approval process. If they must

the Future of Personalized Medicine, 49 U.C. DAVIS L. REV. 1881, 1894–95 (2016) (explaining the FDA's risk classification system).

197. *See* Food, Drug, and Cosmetic Act § 510(k), 21 U.S.C. § 360(k) (2012). *See generally* Jeffrey K. Shapiro, *Substantial Equivalence Premarket Review: The Right Approach for Most Medical Devices*, 69 FOOD & DRUG L.J. 365 (2014) (discussing § 510(k) and suggesting that substantial equivalence is a sound approach for most medical devices).

198. The Federal Food, Drug, and Cosmetic Act gives the FDA the authority to regulate any medical device, defined as "an instrument, apparatus, implement, machine, contrivance, implant, in vitro reagent, or other similar or related article, including any component, part, or accessory, which is . . . intended for use in the *diagnosis of* disease or other conditions, or in the cure, mitigation, treatment or prevention of disease." 21 U.S.C. § 321(h) (emphasis added).

199. FDA, DRAFT GUIDANCE FOR INDUSTRY, FOOD AND DRUG ADMINISTRATION STAFF, AND CLINICAL LABORATORIES: FRAMEWORK FOR REGULATORY OVERSIGHT OF LABORATORY DEVELOPED TESTS 5 (Oct. 3, 2014), http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM416685.pdf.

200. The Clinical Laboratory Fee Schedule sets the rates at which Medicare will reimburse outpatient laboratory testing services. *Clinical Laboratory Fee Schedule (2014)*, CTRS. FOR MEDICARE & MEDICAID SERVS., https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/ClinicalLabFeeSched/Clinical-Laboratory-Fee-Schedule-Files-Items/14CLAB.html (last visited June 18, 2018).

201. INNOVATION COUNSELLORS, A FUTURE AT RISK: ECONOMIC PERFORMANCE, ENTREPRENEURSHIP, AND VENTURE CAPITAL IN THE U.S. MEDICAL TECHNOLOGY SECTOR 25 (Oct. 2016), https://www.advamed.org/sites/default/files/resource/a_future_at_risk_advamed_october_2016.pdf.

satisfy both regulators, we can expect their concern with the system to increase accordingly. As such, we might consider whether programs that have been developed to lower the cost and uncertainty of development in the medical-device context might be extended to pharmaceuticals in the event that a delinkage proposal is implemented. These programs still permit the two regulatory systems to function independently, but ameliorate some of the additional regulatory burden they might impose.

At least two such systems have already been developed. The first is a parallel review program, allowing medical-device product sponsors to request that CMS begin the coverage-determination process while the product is still under review by the FDA.[202] The idea is to partially collapse the two review timelines and permit product sponsors to anticipate and develop the data needed by both agencies. The program was formally made permanent in 2016,[203] although in its first five years of operation, just a single device was approved through the program—Cologuard, a noninvasive colorectal-cancer screening test.[204] If used more widely,[205] the parallel review program should reduce the cost and uncertainty faced by medical-device companies in dealing with separate regulatory bodies.

The second such system is coverage with evidence development (CED). The idea is to permit CMS to provide reimbursement for particular technologies on the condition that the manufacturers continue to gather clinical data about the technologies.[206] That data can later be used to evaluate more

202. Program for Parallel Review of Medical Devices, 81 Fed. Reg. 73,113 (Oct. 24, 2016).

203. *Id.*

204. RICHARDSON, *supra* note 193, at 4. It is worth noting that at least one other product, from Medtronic, failed to demonstrate efficacy in its Phase III trial and thus did not complete the program. *Id.*

205. It is unclear why medical device companies have not yet embraced the program. Reports supported by AdvaMed, the trade organization representing the industry, state only that the parallel review program "has limitations and would require modification[s]" to achieve its goals. INNOVATION COUNSELLORS, *supra* note 201. However, it is unclear what modifications they would like to see. *Id.*

206. CTRS. FOR MEDICARE & MEDICAID SERVS., U.S. DEP'T OF HEALTH & HUMAN SERVS., GUIDANCE FOR THE PUBLIC, INDUSTRY, AND CMS STAFF, NATIONAL COVERAGE DETERMINATIONS WITH DATA COLLECTION AS A CONDITION OF COVERAGE: COVERAGE WITH EVIDENCE DEVELOPMENT (July 12, 2006), https://www.cms.gov/Medicare/Coverage/DeterminationProcess/downloads/ced.pdf. Coverage with evidence development is also available for services (not only technologies), but for my purposes I have focused on its relationship to health care technology products. *Id.*

fully the technology for an official coverage determination. CMS has implemented CED protocols roughly twenty times,[207] but in only two cases has CMS used the process to revise its coverage determination.[208] More rigorous use of CED to review the coverage of promising but ultimately investigational devices might provide a sample framework for use in the drug context as well.[209]

V. THEORIZING INTERMEDIATE SOLUTIONS

Policymakers wishing to achieve the potential benefits of delinkage while avoiding the potential social costs may wish to design guardrails more purposefully to achieve these outcomes. Simply removing the requirements that payers cover particular classes of drugs may achieve some cost savings, but it is likely to have a detrimental effect on access in a way that is not necessarily evidence-based, and it may be unlikely to lead to the development of comparative-effectiveness information on its own. This Part considers three potential intermediate solutions, each of which would balance the benefits and costs of delinkage.

Importantly, each of these intermediate solutions is motivated by a different theoretical model of the purpose of the FDA and its relationship to insurance reimbursement. On the view that the FDA's central purpose is to approve safe, effective drugs and CMS's central purpose is to provide access to those drugs, partial delinkage models that focus on evidence development may be attractive. On the view that gives primacy to the role of the FDA as an information-generating agency, solutions that provide CMS with an opportunity to direct the development of the relevant evidence may be helpful. And on a view of both agencies as driven to promote socially valuable innovation, realigning payment incentives with the development of clinical trial information would be important.

207. RICHARDSON, *supra* note 193, at 3. Examples may be found at *Coverage with Evidence Development*, CTRS. FOR MEDICARE & MEDICAID SERVS., https://www.cms.gov/Medicare/Coverage/Coverage-with-Evidence-Development (last visited June 18, 2018).

208. RICHARDSON, *supra* note 193, at 3.

209. *See, e.g.*, Rebecca Eisenberg & Harold Varmus, *Insurance for Broad Genomic Tests in Oncology*, 358 SCIENCE 1133, 1134 (2017).

A. CONSUMER PROTECTION AND PARTIAL DELINKAGE

One traditional view of the FDA emphasizes its consumer protection function.[210] On this view, the public must be able to trust that when they purchase a prescription drug, the FDA's stamp of approval means that the product is safe and effective. The Agency's reputation matters.[211] On the other side, a traditional view of payers, and of Medicare and Medicaid in particular, is to help ensure that patients can access and afford needed medical care.[212] Whether at their creation in 1965 or their expansions decades later, politicians have emphasized the importance of these programs in providing not just care, but financial stability.[213]

For those holding strong versions of both of these views (consumer protection and access), a strong legal link between FDA approval and insurance coverage may be logically attractive. If the FDA only approves products that have been shown to be safe and effective, and if the purpose of insurance is to help patients afford needed medical care, surely insurers should cover a wide range of therapies. This perspective does not logically require the precise system Medicare and Medicaid have now, but it might be consistent with a requirement to cover a certain number of drugs per class, or to prevent discrimination on the basis of particular health conditions.

However, this linkage only holds true if the FDA is approving only products that have been proven to be safe and effective. As described in Parts I and II above, the FDA now approves a whole range of drugs that have not yet been shown to be effective for their target indication. When the FDA is approving drugs on the basis of surrogate endpoints with labels stating that "[a] clinical benefit . . . has not been established,"[214] does paying for such products really serve the purpose of ensuring access to care? It

210. Rebecca S. Eisenberg, *The Role of the FDA in Innovation Policy*, 13 MICH. TELECOMM. & TECH. L. REV. 345, 367, 387 (2007).

211. CARPENTER, *supra* note 102, at 10–11 (discussing the regulatory power of the FDA gained through reputation).

212. *See* STARR, *supra* note 28, at 367; Ruger, *supra* note 23, at 220.

213. *See, e.g.*, Nancy-Ann Min DeParle, *Celebrating 35 Years of Medicare and Medicaid*, 22 HEALTH CARE FINANCING REV. 1, 1 (2000) (quoting President Johnson signing Medicare and Medicaid into law); Dan Collins, *Bush Signs Medicare Bill*, CBS (Oct. 23, 2003), https://www.cbsnews.com/news/bush-signs-medicare-bill (quoting President Bush at the signing ceremony for Medicare Part D as helping seniors "find affordable medical care in the later years of life").

214. *Highlights of Prescribing Information: EXONDYS 51*, FDA (2016), https://www.accessdata.fda.gov/drugsatfda_docs/label/2016/206488lbl.pdf.

does not obviously do so in a system of limited resources. Similarly, it may undermine trust in the FDA as a consumer regulator.

These dual goals might therefore be served by partial delinkage, where the delinkage is limited to certain classes of drugs or to drugs with particular characteristics. A thoughtful recent example of such a proposal comes from Massachusetts, whose Medicaid program has applied for a waiver of the prescription drug coverage requirements.[215] Specifically, Massachusetts is seeking to make two kinds of changes to its program. First, to adopt a closed formulary of the type used by private payers and Medicare Part D plans to the extent they are permitted to select two drugs per class to cover.[216] Second and more novel, to exclude from the formulary entirely drugs "with limited or inadequate evidence of clinical efficacy."[217]

In requesting this second set of exclusions, Massachusetts is expressing concern about the interaction between the Medicaid coverage requirements and accelerated approval, as discussed above in Part I.[218] Massachusetts is particularly worried about drugs coming to market through the accelerated approval pathway which "have not yet demonstrated clinical benefit and have been studied in clinical trials using only surrogate endpoints."[219] Importantly, Massachusetts does not seek to exclude such drugs entirely or indefinitely. Drugs that have demonstrated "incremental clinical value relative to peer drugs" in their class would still be covered.[220] But drugs that have yet to demonstrate such a benefit would be candidates for potential exclusion from the formulary.

This second set of exclusions does not single out drugs on the basis of disease or even necessarily expense, although expense is certainly central to Massachusetts's decision to ask for the waiver in the first place.[221] Instead, it is focused on drugs approved on the basis of comparatively weaker evidence.[222] As

215. *See* COMMONWEALTH OF MASS., MASSHEALTH SECTION 1115 DEMONSTRATION AMENDMENT REQUEST (Sept. 8, 2017), https://www.medicaid.gov/medicaid-chip-program-information/by-topics/waivers/1115/downloads/ma/ma-masshealth-pa3.pdf.

216. *Id.* at 8–9.

217. *Id.* at 9.

218. *See supra* text accompanying notes 72–80.

219. COMMONWEALTH OF MASS., *supra* note 215, at 9.

220. *Id.* at 10.

221. *Id.* at 8.

222. *Id.* at 9.

such, Massachusetts's waiver application is designed to maximize the benefits and minimize the costs of delinkage. Massachusetts is clearly hoping to achieve some cost savings as a result of this delinkage, both directly (by excluding drugs for which there is little evidence of efficacy) and indirectly (by increasing their negotiation leverage over the drugs that remain). Massachusetts's program also seems designed to lead to the production of information about the comparative effectiveness of new drugs.[223]

At the same time, there would be some reduction in access to medicines. Massachusetts has thought seriously about ways to minimize the therapeutic effects of decreased access,[224] but policymakers should carefully consider the patients who are likely to be impacted. If the reduction in access falls disproportionately on historically marginalized patients with certain diseases (such as cancer or orphan conditions), policymakers might try to create other options for patients that impose financial risk on the drug companies, not the states. For instance, states implementing proposals like these might be incentivized to strike outcomes-based deals with drug companies.[225] The companies would agree to provide their products to particular patients, but they would be paid only if follow-on clinical trials demonstrated clear efficacy.[226]

B. INFORMATION PRODUCTION AND INTERAGENCY COLLABORATION

A more modern view of the FDA, represented most clearly by the work of Professor Rebecca Eisenberg, conceives of the FDA as an information-producing, innovation-focused agency.[227] As she has written, "If a century ago the goal of drug regulation was to protect people from poisons, today drug regulation guides

223. If adopted by Massachusetts alone, the threat of a formulary exclusion may not be sufficient to encourage companies to produce the relevant data sets. As noted in Part IV, *supra*, the size of the market may be too small to encourage companies to develop the information.

224. *See* COMMONWEALTH OF MASS., *supra* note 215, at 9 (detailing the proposed exceptions process for patients who need access to excluded products).

225. *Cf.* Rachel E. Sachs et al., *Innovative Contracting for Pharmaceuticals and Medicaid's Best-Price Rule*, 43 J. HEALTH POL. POL'Y & L. 5, 10 (2018).

226. *See, e.g.*, Katie Thomas & Charles Ornstein, *Considering the Side Effects of Drugmakers' Money-Back Guarantees*, N.Y. TIMES (July 10, 2017), https://www.nytimes.com/2017/07/10/health/prescription-drugs-cost.html ("Italy now asks drug companies to provide some of their products for free—at first. Manufacturers are only paid once results are demonstrated.").

227. Eisenberg, *supra* note 210, at 348.

the development of information that turns poisons, used advisedly, into drugs."[228] Eisenberg portrays the FDA's clinical trial requirements as a tool to force pharmaceutical companies to produce information about the safety and efficacy of their products—information they would otherwise be unlikely to produce on their own.[229]

Similarly, more modern views of insurers (particularly public insurers) consider them not only as a means of providing access to care for patients, but also as tools to encourage evidence- and value-based care. On this view, insurers can and should aim to compensate providers on the basis of the quality, not the quantity, of the care they provide. In this vein, HHS is aiming to continue to increase the amount of reimbursement that is based on quality or value rather than volume.[230] Unfortunately, to date this initiative has largely been limited to hospital and physician services.[231] The coverage mandates described herein likely limit CMS's ability to extend these new payment models from healthcare services to healthcare technologies.

Under this set of views about the agencies' purposes, it is not clear that there is a strong logical need to link approval and coverage as a matter of law. If the FDA is appropriately channeling companies toward the production of information that is needed to enable insurers and physicians to make evidence- and value-based decisions about care, a coverage mandate would not obviously be necessary. A coverage mandate might even get in the way of payers' attempts to obtain value-based prices for new medicines. That is, if FDA clinical-trial results show that a drug is likely to lead to a particular level of benefit, requiring insurers to cover that drug may permit its manufacturer to charge a price that is out of proportion to the value it provides.

228. *Id.* at 347.

229. *See id.* at 370; *see also* Kapczynski & Syed, *supra* note 159, at 1922.

230. *Better, Smarter, Healthier*, U.S. DEP'T OF HEALTH & HUMAN SERVS. (Jan. 26, 2015), http://wayback.archive-it.org/3926/20170127185400/http://www.hhs.gov/about/news/2015/01/26/better-smarter-healthier-in-historic-announcement-hhs-sets-clear-goals-and-timeline-for-shifting-medicare-reimbursements-from-volume-to-value.html (setting goals "of tying 85 percent of all traditional Medicare payments to quality or value by 2016 and 90 percent by 2018").

231. *Cf.* Sylvia M. Burwell, *Setting Value-Based Payment Goals—HHS Efforts to Improve U.S. Health Care*, 372 NEW ENG. J. MED. 897, 897 (2015) (emphasizing provider "teamwork and integration" and "effective coordination of providers across settings"); *see infra* text accompanying notes 247–48 (discussing the Administration's cancellation of the Part B pharmaceutical demo).

A solution that adopts these perspectives of agency purpose might address the issue not necessarily by giving CMS or other payers the ability to decline to pay for FDA-approved drugs, but by giving them input into the FDA approval process to begin with. This can be done either procedurally or substantively, in a way that provides CMS with more or less power over the process. A procedural intervention which is merely exhortatory might involve CMS in the process by which the FDA decides whether a potential new drug would be eligible for the Accelerated Approval program. As discussed in Part I, the program is intended to address "serious or life-threatening diseases or conditions and unmet medical needs."[232] But how is the FDA to know whether a particular condition qualifies as serious or whether the medical need is unmet? These determinations can be informed by evidence possessed by CMS, in its role as insurer for over 100,000,000 Americans.[233]

Regulators might also imagine a program more akin to the parallel review program described above that the agencies have developed in the device context.[234] Under this more substantive intervention, delinkage would be coupled with CMS involvement in the clinical-trials process. CMS would be able to recommend to pharmaceutical companies just beginning clinical trials the kinds of clinical evidence they would need to produce to achieve a formulary placement, or even a favorable one. At that point, companies could choose whether to complete the recommended trials. If they choose not to, they may obtain FDA approval—with full knowledge that there may be little payer appetite for their products. And if they do complete the trials, FDA approval will bring the release of clinical-trial information that is far more useful to payers.[235]

The impact of this intervention on the three potential outcomes described above likely depends on the level at which the intervention is adopted. A program resembling parallel review has the potential to increase the production of information that is truly useful to the healthcare system, such as comparative-effectiveness information, because such information is requested

232. 21 U.S.C. § 356(e)(1) (2012).

233. *See* FISCAL YEAR 2016 JUSTIFICATION OF ESTIMATES, *supra* note 21, at 109–10.

234. *See supra* text accompanying notes 202–05.

235. *Cf.* Rebecca S. Eisenberg & W. Nicholson Price, II, *Promoting Healthcare Innovation on the Demand Side*, 4 J.L. BIOSCIENCES 3, 15–24 (2017) (exploring potential opportunities for payers and examining the role health insurers may play separately in the development of such information).

ex ante and payers retain the ability to decline to pay for products approved without such information. A more procedural intervention in which CMS provides assistance to the FDA in determining which products should proceed through the accelerated-approval pathway, but which still requires CMS to cover such products once approved, would be unlikely to have an effect on the development of comparative-effectiveness evidence.

One potential concern about this set of interventions is that they might decenter the role of the FDA in the drug approval process, elevating the role of CMS by comparison. Even as scholars have come to view the FDA as serving this valuable information-forcing function, to most patients it still holds its respected consumer protection position.[236] CMS has not needed to cultivate public trust in the same way, and arguably it has become a site of more political strife since the passage of the ACA. Injecting CMS into the supposedly apolitical FDA approval process[237] might, in the eyes of some members of the public, taint that process. As such, policymakers might be especially cautious before requiring measures that go beyond information sharing.

C. ALIGNING INCENTIVES IN AN INNOVATION ECOSYSTEM

A third set of policy proposals comes from recognizing not only the FDA's centrality in the development of innovative new therapies, but CMS's role in the process as well. There are a number of FDA initiatives designed to promote innovation in socially valuable areas that may be understudied by the private sector. For instance, the four expedited review programs described above encourage companies to invest in new therapies for serious illnesses lacking existing treatments.[238] As another example, the Orphan Drug Act provides extra incentives for companies to study diseases affecting few Americans,[239] which might

236. CARPENTER, *supra* note 102, at 10–11.

237. *See, e.g.*, Rachel E. Barkow, *Insulating Agencies: Avoiding Capture Through Institutional Design*, 89 TEX. L. REV. 15, 47 (2010) ("[T]he FDA is relatively more independent than other executive agencies, with its heads often advocating for drug regulation regardless of the position of their appointing president.").

238. *See supra* text accompanying notes 73–80.

239. *See* 21 U.S.C. § 360cc(a) (2012) (conferring seven years of market exclusivity on orphan products); 26 U.S.C. § 45C(a) (2012) (conferring a fifty percent tax credit for eligible clinical trial expenses). However, Congress's 2017 tax overhaul reduced the credit to twenty-five percent. P.L. No. 115-97, 115th Cong., § 13401(a) ("Modification of Orphan Drug Credit"). *See also* Daniel J. Hemel & Lisa Larrimore Ouellette, *Beyond the Patents—Prizes Debate*, 92 TEX. L.

not otherwise be serious topics of study.[240] The process is not perfect, and there is much work left to do. But policymakers clearly understand the potential benefits of implementing innovation-related policies through the FDA approval process.

As of yet, policymakers have largely not viewed insurance generally, and CMS more specifically, as capable of advancing these policy goals.[241] This is a mistake. As scholars have recognized, prescription drug insurance closely resembles prize systems that have been theorized to provide incentives for the development of new medicines.[242] Pharmaceutical companies who know that insurers must pay for their products can rely on a certain level of rewards, and they may redirect their innovative activities accordingly. For example, scholars have studied the creation of the Medicare Part D program, finding that

REV. 303, 379 (2013) (describing how the Act increased government grants, market exclusivity, and tax credits for companies studying rare diseases).

240. Some have questioned whether the Orphan Drug Act has gone too far, however. *See, e.g.*, Sarah Jane Tribble, *Senator Grassley Launches Inquiry Into Orphan Drug Law's Effect on Prices*, NPR (Feb. 10, 2017), https://www.npr.org/sections/health-shots/2017/02/10/514373480/sen-grassley-launches-inquiry-into-orphan-drug-laws-effect-on-prices (stating that officials have become interested in revisiting Orphan Drug legislation); Nicholas Bagley, INCIDENTAL ECONOMIST, *The Benefits and Costs of Promoting the Development of New Orphan Drugs*, pt. 3 (2017), https://theincidentaleconomist.com/wordpress/wp-content/uploads/2017/02/2.12-orphan-drug.pdf (outlining the costs and problems surrounding Orphan Drug legislation).

241. Congress's one foray into this area, Medicare's New Technology Add-on Payment, directs CMS to create a procedure to identify new medical technologies and provide additional payments to encourage their use. *See* 42 U.S.C. § 1395ww (2012) (outlining the determination of costs and payments within hospital services settings); *see also* Alexandra T. Clyde et al., *Experience With Medicare's New Technology Add-On Payment Program*, 27 HEALTH AFF. 1632, 1632–33 (2008) ("Without appropriate payment to the hospital at the point of use, technologies that provide value to patients and the health care system over time might not be available to patients.").

242. *See, e.g.*, William Fisher, *Intellectual Property and Innovation: Theoretical, Empirical, and Historical Perspectives*, at 12 (May 2, 2001), http://cyber.law.harvard.edu/people/tfisher/Innovation.pdf (discussing rewards as ways to stimulate innovation); *see also, e.g.*, Kevin Outterson, *The Legal Ecology of Resistance: The Role of Antibiotic Resistance in Pharmaceutical Innovation*, 31 CARDOZO L. REV. 613, 645–55 (2010) (discussing insurance reimbursement broadly, finding that they "are a key policy lever for antibiotic effectiveness . . . ."); Arti K. Rai, *The Ends of Intellectual Property: Health as a Case Study*, 70 LAW & CONTEMP. PROBS. 125, 128–29 (2007) (stating that there is little difference between universal health-insurance schemes based on purchasing and those based on prizes); Benjamin N. Roin, *Intellectual Property Versus Prizes: Reframing the Debate*, 81 U. CHI. L. REV. 999, 1012–13 (2014) (describing government rewards for new drugs as similar to a prize system); Sachs, *supra* note 4, at 178 (finding that "prescription drug insurance strongly resembles a prize system").

pharmaceutical companies engaged in increased investment into drug classes with higher consumption among the Medicare population when more seniors had access to comprehensive prescription drug coverage.[243] And yet to date, health insurance has traditionally been viewed by policymakers as a tool only for promoting access to healthcare technologies.

Recalibrating our view of insurance as a tool for promoting innovation as well as access reveals ways in which policymakers might realign reimbursement more closely with the FDA and innovation incentives. This is true for both relatively weak and relatively strong interventions, and those along a spectrum in between. A relatively weak intervention would involve reforming the way in which physicians are reimbursed for prescribing and administering drugs under Medicare Part B. As discussed in Part I, many expensive biologics are administered in physicians' offices and reimbursed under Part B.[244] When the physician is reimbursed for providing the drug to her patients, she is reimbursed not based on the value of the drug she provides but instead receives a fee based on a percentage of its price.[245] Many scholars and policymakers have argued that this system may encourage physicians to prescribe and administer more expensive drugs than may be medically necessary.[246] Providing a flat fee instead of a percentage would help nudge providers in the right direction from a prescription perspective. Relatedly, toward the end of the Obama Administration, CMS sought to implement

243. *See* Margaret E. Blume-Kohout & Neeraj Sood, *The Impact of Medicare Part D on Pharmaceutical R&D* 12–13 (Nat'l Bureau of Econ. Research, Working Paper No. 13857, 2008). *But see* Daron Acemoglu & Joshua Linn, *Market Size in Innovation: Theory and Evidence from the Pharmaceutical Industry*, 119 Q.J. ECON. 1049, 1084 (2004) (describing the effects profit incentives have on innovation as requiring further research); David Dranove et al., *Pharmaceutical Profits and the Social Value of Innovation* 6–7 (Nat'l Bureau of Econ. Research, Working Paper No. 20212, 2014) (qualifying the findings of Blume-Kohout and Sood by noting that truly innovative activity takes longer to emerge). Relatedly, Professor Amy Finkelstein has discovered that several policies designed to increase the uptake of vaccines (including Medicare's 1993 decision to cover the flu vaccine) resulted in an increase in clinical trials for new vaccines. Amy Finkelstein, *Static and Dynamic Effects of Health Policy: Evidence from the Vaccine Industry*, 119 Q.J. ECON. 527, 556–57 (2004).

244. MEDPAC, *supra* note 4, at 121.

245. *See id.* at 117.

246. *See id.* at 118 (explaining that the reimbursement might incentivize physicians, noting that "a higher priced drug generates more revenue for the provider"); Patricia M. Danzon et al., *Alternative Strategies for Medicare Payment of Outpatient Prescription Drugs—Part B and Beyond*, 11 AM. J. MANAGED CARE 173, 173 (2005) (describing generally how reimbursement may result in higher prices for private and public purchasers).

such a program as a demonstration project.[247] Industry groups, especially the oncologists that prescribe many of these drugs and the pharmaceutical companies that make them, objected vociferously and the program was never implemented.[248]

An intermediate intervention may be implemented in the Medicaid program. Recall that Medicaid is entitled by law to large statutory discounts off of the average manufacturer price for a drug, or, if lower, the best price available to a specified group of payers.[249] This is a sign of a program that is designed for access. The goal here is to spend as little as possible on each drug, to use scarce resources efficiently, and to care for as many people as possible. But at present, Medicaid likely dampens incentives to develop drugs primarily for low-income Americans. Pharmaceutical companies know that if they choose to develop products with high Medicaid market share, their potential revenue will be lower than if most patients with the disease in question are on Medicare or private insurance.[250]

Happily, Medicaid's reimbursement system can be recalibrated to balance incentives for both innovation and access and provide additional incentives to companies who choose to invest in developing drugs for diseases prevalent among low-income Americans. One option would be to equalize down the rates that private payers and Medicare pay for these products, removing the innovation distortion in favor of diseases of affluence, while

247. *Medicare Program: Part B Drug Payment Model*, 81 Fed. Reg. 13230 (Mar. 11, 2016) (to be codified at 42 C.F.R. pt. 511).

248. *See, e.g.*, *CMS Formally Withdraws Medicare Part B Demo*, AM. SOC'Y OF CLINICAL ONCOLOGY (Oct. 3, 2017), https://www.asco.org/advocacy-policy/asco-in-action/cms-formally-withdraws-medicare-part-b-demo ("ASCO has opposed the demo since its release in March 2016, citing a flawed premise, experimental nature, and lack of important patient protections."); Allison Funk, *Medicare Monday: 3 Things to Know About the Government's Medicare Payment Change*, PHRMA (Mar. 14, 2016), http://catalyst.phrma.org/medicare-monday-3-things-to-know-about-the-governments-medicare-payment-change ("[T]his proposal . . . could discourage investment in future treatment advances . . . .").

249. 42 U.S.C. § 1396r-8(c)(1)(A)(ii)(I) (2012).

250. This is not merely a problem within the U.S. market. Tiered pricing of the type used here is typically viewed by economists as a potential win-win strategy for producers seeking profits and low-income patients seeking access to medicines. *See, e.g.*, Jens Plahte, *Tiered Pricing of Vaccines: A Win-Win-Win Situation, Not a Subsidy*, 5 LANCET INFECTIOUS DISEASE 58, 59–60 (2005) (arguing that tiered pricing contributes to the overall welfare of the involved parties, creating a "win-win-win" situation). But where the primary patient market is a low-income one, tiered pricing alone may be insufficient to encourage the development of such products in the first instance.

at the same time helping mitigate our problem of drug spending.[251] Another option would be to equalize up the rates that Medicaid pays for a particular set of products,[252] providing bonuses to companies who choose to invest in products primarily for low-income Americans.[253] The simplest way to implement such an incentive would be to waive at least a portion of the required Medicaid rebate these companies must pay. A 23.1% or more[254] increase in reimbursement for any particular product may well make a difference to companies choosing to invest in the first instance.

The two previous interventions can be accomplished within the framework of our existing system, maintaining the linkage between approval and reimbursement in public programs. A third option would be to move toward a model like that in the United Kingdom as described in Part IV, going even further in involving payers to incentivize innovation. Paying for drugs based on the social value they provide, rather than on the price the manufacturer can demand in a linked market, might push companies toward different areas of research than they are currently prioritizing. This intervention would require not only delinkage, but also a more robust policy conversation about what our society is willing to pay for than we have had so far. But from

251. Proposals suggesting that Medicare Part D adopt the provision that insulates the Medicaid program when drug prices rise faster than inflation fall into this category. *See* U.S. DEP'T OF HEALTH & HUMAN SERVS., *supra* note 70, at 8–9 (finding that total rebates were higher under Medicaid than Medicare Part D, Medicaid's net unit costs were lower, and one half of manufacturer-owed rebates could be attributed to add-on, inflation-based rebates). Proposals that would reimburse drugs for dually eligible patients at Medicaid rates would accomplish this for a subset of the Medicare population. Gretchen Jacobson et al., *Summary of Medicare Provisions in the President's Budget for Fiscal Year 2016*, KAISER FAMILY FOUND. (Feb. 3, 2015), http://kff.org/medicare/issue-brief/summary-of-medicare-provisions-in-the-presidents-budget-for-fiscal-year-2016.

252. The choice between equalizing down or up, and in either case how far to do so, is ultimately an empirical question that depends on a range of considerations. *Compare* Alan M. Garber et al., *Insurance and Incentives for Medical Innovation* 5 (Nat'l Bureau of Econ. Research, Working Paper No. 12080, 2006) (arguing that pharmaceutical companies possess excessive incentives for innovation), *with* Darius Lakdawalla & Neeraj Sood, *Insurance and Innovation in Health Care Markets* 25 (Nat'l Bureau of Econ. Research, Working Paper No. 11602, 2005) (arguing that incentives for innovation are insufficient).

253. I explore this proposal in more detail in Sachs, *supra* note 4.

254. Given that states are empowered to seek supplemental rebates beyond the required 23.1%, the percentage change may be even greater. U.S. DEP'T OF HEALTH & HUMAN SERVS., STATES' COLLECTION OF OFFSET AND SUPPLEMENTAL MEDICAID REBATES 5 n.19 (2014).

the perspective of not just providing access to medicines, but also encouraging innovation into the most needed technologies, an intervention along these lines may be more useful.

CONCLUSION

Scholars and policymakers are rightly interested in opportunities for reform of both the FDA and health-insurance system in this country. Simultaneously, there is broad agreement about the need to take action to address the problems of drug pricing. Yet the failure to appreciate the linkage between FDA approval and insurance reimbursement has thus far stalled the development of potential policies to solve both problems. This Article's evaluation of that linkage presents options for scholars and policymakers to pursue going forward.