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New Medical Technology Development and Diffusion
Policy Challenges and Considerations

Christopher Lau
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New Medical Technology Development and Diffusion: Policy Challenges and Considerations

Christopher Lau

ABSTRACT

Over the past decade, the rapid pace of medical technology development has created a large array of drugs, medical devices and healthcare services, many of which have greatly expanded the scope of treatable diseases. The diffusion of these technologies in the U.S. has helped countless individuals realize substantial improvements in life expectancy and overall quality of life. However, the development and diffusion of new medical technology have also presented several challenges for U.S. policy makers. The three papers that comprise this dissertation examine three policy issues that center on some of these challenges. The first paper examines how markets can fail to incentivize the development of new medical technology that address a public need and explore the lessons learned from the implementation of four different policy solutions. The second paper examines the role of the National Institutes of Health in new medical technology development and the extent to which its research grant program has encouraged the formation of biopharmaceutical commercial alliances. The last paper examines the Centers for Medicare and Medicaid Service’s Accountable Care Organization program and whether its participants are changing the way that new medical technologies are used and adopted.
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Introduction

Medical technology comprises the range of products and services used to deliver medical care to individuals. Over the past decade, the rapid pace of medical technology development has created a large array of drugs, medical devices, and healthcare services, many of which have greatly expanded the scope of treatable diseases. The diffusion of these medical technologies in the U.S. has helped countless individuals realize substantial improvements in life expectancy and overall quality of life.
However, the development and diffusion of medical technology have also presented several challenges for U.S. policymakers. For example, medical technologies can offer considerable social benefits, but may go undeveloped due to the large risks and capital investments required of private firms and individuals. Consequently, policymakers have long sought ways to provide sufficient incentives for innovators who develop new medical technologies. At the same time, unproven medical technologies can threaten public safety. Concerns over these issues have pushed policymakers to consider policies that ensure that new medical technologies are reasonably safe and effective before entering the market. More recently, policymakers have been confronted with the contributions of medical technology to rising healthcare costs. Which policies will reduce wasteful spending on medical technology remains an unanswered question.

The central role played by medical technology development and diffusion across policy issues provides the primary motivation for the three papers that comprise this dissertation. In each paper, I will examine a policy issue that centers on the process of medical technology development or diffusion. The first paper will examine how markets can fail to incentivize the development of medical technology and explore the applicability of four policy alternatives that have historically been used to address these problems. The second paper will examine the role of the National Institutes of Health (NIH) in medical technology development and the extent to which its research grant program has encouraged the formation of biopharmaceutical commercial alliances. In the last paper, I will examine the Centers for Medicare and Medicaid Service’s (CMS) Accountable Care Organization (ACO) program and explore whether its participants are changing the way that medical technology is used and adopted.
Encouraging Medical Technology for Unmet Social Needs: Evidence and Lessons from Past Policy Interventions

Christopher Lau

ABSTRACT
Policymakers are occasionally confronted with public health and social issues that can only be addressed through the development of new medical products and services. A critical challenge for policymakers is determining how to select the best set of policies to encourage the production of these socially-desirable technologies. Using existing literature and empirical evidence, the following paper explores this policy problem, the solution set available to policy makers, and the lessons learned from previous implementations of four policy tools: federal research grants, federal R&D tax credits, expedited Food and Drug Administration (FDA) regulatory review, and FDA market exclusivity. This paper concludes with a discussion of what the current evidence implies for future policymakers’ efforts to encourage the production of specific medical technologies.
I. Introduction

The continual development of new medical technologies in the U.S. is the consequence of a complex and dynamic innovation system. The system generates medical products and services that can dramatically improve the quality of life for countless individuals. The innovation process involves private stakeholders who are searching for new ways to meet healthcare needs and are working to develop those ideas. Remarkably, much of the coordination required for medical innovation occurs without the guidance of a central planning authority. Instead, new drugs, medical devices, and healthcare services are developed in response to market forces.

Markets, however, can fail—and have failed in the past—to adequately incentivize the production of medical technologies that address a public health or social need. Take for instance, treatments for diseases that predominantly affect indigent populations. While these treatments can provide large social and public health benefits that extend beyond the relief provided to the individuals being treated, existing markets often do not offer sufficient profits to support the development costs for these innovations. Consequently, few innovators allocate the necessary time and resources to identify and develop new ways to address unmet medical needs for these populations.

Under these circumstances, policy interventions may be needed to redirect innovators’ efforts toward improving social welfare. Deciding which set of policy tools to apply presents a critical challenge for policymakers. Many policy interventions, ranging from federal research subsidies to expedited FDA review, are thought to encourage medical technology innovation. However, implementing these policies based on conventional wisdom alone can be problematic. The incremental effects of policy interventions can vary depending on the target technology and surrounding circumstances. In some cases, implementing the wrong policy interventions can lead to adverse and unintended consequences that undermine the policymakers’ original goals.

One way to guide policy making is to draw on lessons from past policy implementations. Since the 1980s, U.S. policymakers have made several attempts to encourage medical technology innovation in areas that address public needs. The response to the AIDS epidemic and the push to promote the development of orphan drugs are prime examples. In each case policies were implemented to modify critical government functions known to influence medical technology innovation. By modifying functions to favor certain technologies, the hope was that new incentives would induce innovators to develop medical products targeted by policymakers. Given these experiences, a natural question to ask
is: What lessons can be applied towards future policymaking? This paper aims to address that question by using existing literature to examine the effects and effectiveness of previous policy efforts.

This paper is organized into three major sections. In the first section, I review the medical technology innovation process and identify the basic problems that can constrain the development of technologies targeted by policymakers. The second section examines the major government functions that can be modified by policymakers to address deficiencies in the medical technology innovation process. In the third section, I summarize the current state of knowledge concerning four major policy tools that have been used in the U.S. to encourage the development of specific medical technologies: federal research grants, federal R&D tax credits, expedited FDA regulatory review, and FDA market exclusivity. This paper concludes by assessing what our current state of knowledge of various policy options implies for future strategies to encourage technology development.

II. Potential Problems in Medical Technology Innovation

A critical first step in developing any policy strategy is to understand the underlying causes of the problem at hand. Individual medical technologies may fail to reach healthcare markets for a number of reasons.
possible reasons. For instance, a new drug may fail to produce a level of efficacy that would be considered clinically valuable. Similarly, a new device may present unforeseen technological challenges that cannot be overcome. In contrast, the underdevelopment of new medical technologies is usually indicative of a handful of systematic problems in the innovation process. These problems most often originate during the search for new ideas or the evaluation of ideas for new medical technologies. See Figure 1.

A. Search for Ideas for Medical Technology

The first stage where problems can occur is while new ideas for medical products and services are being identified. The recognition of an idea is an important step in the development of medical technologies; all medical goods and services start as ideas. Ideas emerge as innovators recognize new ways to address unmet healthcare needs.1,3 Take for example the advent of remote patient monitoring for patients with diabetes. These systems provide diabetic patients with ways to manage their condition and wirelessly submit information about the patient’s condition directly to healthcare providers to help identify health problems early.4,5 Before these systems were developed, innovators first needed to recognize that a need existed among diabetic patients to better manage their disease. Secondly, innovators needed to recognize that existing mobile technologies could be used to provide real-time information that could address the needs of diabetic patient populations.

The identification of new ideas is driven by innovators’ prior knowledge. While the recognition of an individual idea can be characterized as random or serendipitous, the process is shaped by an individual’s own experience and their understanding of markets.6,7 The greater access an individual has to private and public sources of information, the more likely they are to identify ideas for new medical products and services. Two types of knowledge can shape the outcome of the discovery process. The first is knowledge of healthcare market needs. Understanding of market needs determines which problems medical innovators are likely to focus on and solve. The second type of knowledge is technical and scientific knowledge. An individual’s understanding of scientific and/or engineering concepts determines their capacity to generate solutions for unmet medical needs.

In the absence of suitable knowledge inputs, the discovery process for new ideas can stall. For instance, absent sufficient knowledge about an unmet healthcare need, fewer innovators will search for ideas to meet those needs. The more common concern among policymakers is a lack of scientific knowledge. For example, before treatments for a particular disease can be identified, the etiology and overall
scientific understanding of the disease must be well established. Furthermore, even after a prospective idea for a new medical product or service is identified, scientific knowledge inputs may be needed to develop the idea into a marketable medical technology. For example, knowledge of biomarkers that measure the progression of disease may be needed to measure the effectiveness of prospective biopharmaceutical products. Ultimately, if a lack of suitable knowledge inputs is linked to medical technologies targeted by policymakers, the scarcity could be responsible for the existing patterns of underdevelopment.

B. Evaluation of Ideas for Development

The second stage where problems can occur is during the evaluation of individual ideas. Innovators start with a set of ideas for new technologies to develop. Some of these ideas may be “new”—those that are identified during the initial identification process. Innovators may also consider “old” ideas—ideas for technologies that had not been developed based on previous evaluations. Irrespective of its origins, each idea is evaluated against several criteria. Some of these criteria may be idiosyncratic or tied to individual preferences, such as a desire to address a specific disease or broader social need. However, all innovators must account for an idea’s potential risk and returns. Even if an idea satisfies all of an individual innovator’s preferences, their financial backers will need to be assured that they will see returns on their investments. For this reason, ideas with lower risk and higher returns are more often selected for development.

In general, two features of an idea determine its risk and returns. The first feature is the steps needed to develop the idea. An innovator may need to complete a number of different tasks to develop an idea into a marketable medical product or service depending on the idea’s technological area and maturity. In the early stages of development, innovators may need to conduct additional research to establish a basic proof of concept and demonstrate that the original idea is feasible. Subsequently, clinical trials may be needed to demonstrate safety and efficacy for the FDA. Lastly, innovators may need to allocate funds towards marketing and reimbursement campaigns to establish a viable and profitable business for their technology. Collectively, these tasks introduce varying costs and uncertainty into the development process. As these costs and uncertainty rise, they decrease the expected returns and increase the risk that innovators anticipate, all else being equal.

Another key feature that determines an idea’s risk and return is its prospective profitability. An idea’s profitability can be thought of as the net present value of the total profits it would generate if the idea
for a new medical product reached the market. The potential profitability of a given idea is a function of a number of factors. One important factor is consumer demand in healthcare markets. Consumer demand determines how much an innovator can charge for their product and the number of products they are likely to sell. Competition from other medical services or products also factors into an innovator’s evaluation of profitability. Competition determines the innovator’s prospective market power and ability to command a price premium for their technology. Lastly, the innovator may need to consider existing federal regulations that directly affect profitability. For instance, tax policies can change the amount of profit that can be retained by innovators and investors. In general, factors that raise expected value and reduce the uncertainty surrounding profitability also raise an idea’s anticipated returns and reduce risk, all else being equal.

Ideas for medical technologies can go undeveloped if their prospective development costs and risks are high relative their profitability. If an idea’s development costs are high or uncertain, they can effectively raise the profitability needed to generate returns at a given level of risk. Similarly, if an idea’s profitability is low or uncertain, it can lower the maximum development costs and risk that an innovator may be willing to take on when investing in that idea. While these problems are often specific to an individual idea, they can affect an entire set of technologies targeted by policymakers. Under these circumstances, policies may be needed to alter the underlying profitability, development costs, or both to attract the attention of innovators and investors to invest in ideas that address the public need at hand.

III. Policy Solutions: Modifying Critical Government Functions

Once the underlying causes for underdevelopment are understood, public policy can be used to encourage innovators to develop technologies targeted by policymakers. Which policy options can be implemented will depend on the context and specific technology being targeted by the policymaker. However, most potential policy solutions are likely to involve modifying a handful of existing government functions.

The federal government serves many key functions that are intimately tied to medical technology innovation. See Figure 2. Some of these functions were created specifically to promote medical technology innovation, e.g. federal research funding. In other instances, these critical functions were developed in order to address other public needs (i.e. FDA regulation, public payer programs, taxes and
patent law). Policies that change these existing functions can help promote the development of technologies that address an unmet social need.

**FIGURE 2: CRITICAL GOVERNMENT FUNCTIONS IN MEDICAL TECHNOLOGY INNOVATION**

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**A. Federal Research Funding**

Perhaps the government function with the most direct impact on the medical innovation process is the government’s financial support of research. Many consider this function to be critical to the development of new medical technology which is one reason that policymakers have often relied on federal research funding to redirect the efforts of medical technology innovators.

Innovators rely on research in the health and life sciences to produce new knowledge. They apply this knowledge in innovative medical products and services. However, private firms and individuals tend to underinvest in research activities for several reasons. Scientific research is costly, but often produces knowledge that may be difficult to value or whose value is difficult to appropriate. Moreover, even when the value of the knowledge may be high, the associated risk and costs may be too great for an individual innovator or investor to accommodate.

Recognizing this fact, the federal government provides direct financial support for health and life science research through various grants, contracts, and programs. Most of this funding is administered by the National Institutes of Health (NIH). In 2011 alone, the NIH spent approximately $21 billion on research
grants and programs. Unlike privately funded research, the information generated by federally funded research is typically made freely available to the public. Innovators use this public knowledge to identify ideas for new medical technologies. For instance, pharmaceutical and biotechnology firms often cite research sponsored by public funds when developing new healthcare products.

One way policymakers can address low levels of technology development is by changing the types of research that the federal government funds. Increasing funding for one area of research can increase the production of scientific knowledge in that area. In turn, the increase in knowledge production can make it easier for innovators to identify opportunities and/or reduce innovators’ prospective development costs for new medical products related to the scientific field targeted by policymakers.

B. FDA Regulation

The activities carried out by the FDA constitute a second function that policymakers can leverage to redirect innovative efforts. Although new medical products and services help address unmet medical needs, each new technology can present risks of serious side effects. To ensure that these risks are minimized and not unduly taken, the federal government establishes standards to ensure that any new medical product marketed in the U.S. is relatively safe and efficacious.

Safety and efficacy standards are primarily established and enforced by the FDA. These standards apply to many types of medical technologies including drugs, biologics, and medical devices. The FDA upholds these standards through a rigorous review process which examines the evidence manufacturers generate to support claims about their products. Manufacturers of new technologies that fail to meet the agency’s standards of evidence are effectively excluded from selling their products in U.S. healthcare markets.

Policymakers can modify the FDA regulatory approval processes to encourage medical technology development. The FDA regulatory approval process imposes considerable development costs and risks for medical technology innovators. For pharmaceutical manufacturers, estimates have placed these development cost as high as $800 million per new drug. Changes to the FDA regulatory process can help lower some of these costs, improving the risk-return profile of targeted technologies. As we will see in later sections, the FDA’s role as a gatekeeper in healthcare markets can also help innovators establish short-term monopolies, increasing the returns for technologies that address a public need.
Public payer programs were created primarily to provide healthcare insurance coverage to the U.S.’s elderly, indigent, and veteran population. Collectively, these programs represent a third government function that exerts considerable influence over the behavior of innovators.

When determining which technologies to develop, medical innovators carefully evaluate the actions of health insurers because of the role they play in determining consumer demand. Each health insurer institutes its own set of rules and regulations that determine which medical technologies they will cover for their beneficiaries (i.e. individuals to whom they provide medical insurance) and how they will pay providers (physicians, hospitals etc.) for furnishing related services. When these policies provide favorable coverage or payment for a class of technology, they can help spur its adoption and increase demand by both patients and providers. Consequently, innovators are more likely pursue ideas for technologies that are likely to receive favorable treatment by health insurers, all else being equal.

Medical innovators pay close attention to the actions of public insurance programs for two reasons. First, the three main public insurance programs run by the federal government, the Veteran’s Health Administration, Medicaid, and Medicare have a relatively large market share. Together, these programs cover roughly 30 percent of the population in the U.S. and account for close to 40 percent of all U.S. healthcare spending. Second, among the three programs, Medicare is considered by most to be a bellwether insurer. The policies the Medicare program adopts are often followed by private insurers. This behavior is in part a function of the vast resources available to public insurance programs, resources which allow them to evaluate new technologies in ways private insurance companies cannot.

Because of the attention they draw from innovators, public insurance programs can be modified in ways that encourage the development of technologies targeted by policymakers. Policies can be instituted that change the types of technologies covered by public insurance programs or how healthcare providers are reimbursed for using different technologies. By providing more favorable treatment, policymakers can increase the prospective demand for certain technologies and by extension their prospective profitability. In turn, the increase in profitability can make targeted technologies more attractive to innovators and investors.

D. Patent Law

Another key federal government function in the innovation process is its enforcement of the rights conferred by patents. Patents are a core component of the innovation process across industries and
represent an area where policymakers can introduce incentives to redirect efforts to develop medical technology.

Patents grant innovators the right to exclude competitors from using their technology for a fixed period of time. When there are few substitutes for the technology, the market exclusivity rights conferred to patent holders allow them to raise prices and reap larger profits for their products. These profits are important for recouping development costs and compensating innovators for the risks they have taken when investing in an idea for a new technology. For drug and biotechnology firms, patents are often identified as being critical to their business model.15,16

Policymakers can modify aspects of patent law to encourage the development of specific medical technologies. Regulations regarding which technologies qualify for patents, what technologies can be excluded from patents, and how long patents last are elements of patent law that play a role in determining how much innovators can benefit from patenting. For example, extending the period that patent rights remain in effect for specific technologies can increase the profits the innovator can potentially recoup. When regulations are modified to benefit certain technologies, they can redirect the attention of innovators and investors toward those technologies.

E. Taxes on Profits

While taxation is not often associated with medical technology innovation, it is another government function that influences the behavior of innovators. As with for-profit firms across industries, medical technology innovators are subject to taxes on the profits they make on their products. For this reason, tax rates can influence the anticipated return on investment for a given idea and influence which ideas are selected for further development.

Policymakers can change tax policies and implement tax credits or tax deductions to encourage the development of specific medical technologies. Tax credits and tax deductions effectively reduce an individual or firm’s tax liabilities. When these tax credits are defined around specific technologies, policymakers can effectively increase the profitability of ideas in those technological areas. Ultimately, the increased profitability can attract the attention of additional innovators and investors.

IV. Lessons from Past Policy Implementations

Given a set of policy options, policymakers are likely to face the difficult task of determining which combination of reforms to implement. Selecting the best option involves choosing solutions that are
most likely to achieve policy goals with minimal social costs. However, this task is fraught with uncertainty—often it is not clear how effective a given policy solution will be. Fortunately, past U.S. policy experiences offer one source of information that may help reduce uncertainty. In particular, the U.S.’s experience with federal research grants, federal R&D tax credits, FDA expedited review, and FDA market exclusivity can provide useful insights for future policy efforts to promote specific medical technologies.

A. Federal Research Grants

Policy Motivation and Application

Federal research grants have often been used to encourage the development of medical technologies that address an unmet social need. As noted in Section II, scientific knowledge inputs are needed to both identify new ideas and develop those ideas. Gaps in key knowledge inputs can make it difficult for innovators to identify relevant ideas or prohibitively expensive to develop those ideas. Ultimately, large gaps in scientific knowledge can limit the production of new technologies being targeted by policymakers.

Federal research grants have been used to encourage the production of critical knowledge inputs when these gaps in scientific knowledge exist. In addition to funding government-run research facilities and commercial research contracts, research grants provide a channel through which the federal government can directly fund research. This research is conducted at non-government research facilities, like universities, which have the capacity to address research needs that may not be met by government research entities alone. Any new information produced by grant-sponsored research is made publicly available, typically through peer-reviewed publications and research reports. This important distinction allows all innovators to benefit from scientific discoveries generated by grant-funded research.

The use of federal research grants is predicated on a grant’s ability to increase the total production of critical knowledge inputs and ultimately the development of desired medical technologies. Once a knowledge gap has been identified, federal research grants can be defined to exclusively fund research areas likely to address these gaps. In theory, these grants can directly increase the production of critical knowledge inputs in two ways. First, the additional funding may help those already working in these research areas to increase the scale of their existing lines of scientific inquiry. Second, the new source of funding may induce researchers to pursue related lines of scientific inquiry that they would not have
pursued otherwise due the absence of funding. By increasing the number and scale of projects in key research areas, policymakers can effectively advance the scientific knowledge needed to generate new ideas for or to develop targeted technologies.

Federal research grants, however, can fail to achieve policymakers’ desired aims. Perhaps the biggest concern among critics is the potential for federal research grants to substitute for and displace existing innovation efforts. Consider scientific investigators who are conducting research in areas being targeted by policymakers. Absent federal research grants, they may have had sufficient financial resources and incentives to conduct the research needed to create the critical knowledge inputs and develop the technologies desired by policymakers. It is conceivable that the introduction of federal research grants may simply crowd out and substitute for the private financial resources that would have been used absent the policymaker’s intervention. Under these circumstances, while the source of research financing may change, the types and levels of research conducted by innovators may remain the same. Alternatively, when public monies are being allocated toward an innovator’s technological field, innovators may scale back their own private research efforts and attempt to “free-ride” grant-sponsored research since the knowledge produced will be made publicly accessible.17 If “free-riding” occurs, the total production of knowledge inputs from private and public sources may not increase as anticipated.

Evidence from Past Policy Implementations

The existing body of empirical evidence does not support strong generalizations about the effects and effectiveness of federal research grants. Researchers have long sought to quantify the impact of federal research grants on innovation and have used several different measures of innovation in their attempts. For example, some studies have examined research spending by private firms as a proxy for innovation, while others have examined research outputs such as peer-reviewed publications and patents.18 While the use of different outcomes has allowed policymakers and researchers to paint a fuller picture of the relationship between federal funding and innovation, it sometimes has made it difficult to compare the results of one study to another. Contributing to the confusion is the diversity in analytic strategies employed in each study. Although some researchers may find strong effects, limitations in their study design may not allow the study to fully address the possibility of confounding effects. For this reason, there remains some ambiguity about the net effect of federal research grants and, more broadly, research funding.19
Among the literature that examines the effects of federal research grants, the most useful insights can be derived from studies that examine the impact of NIH research funding. Recall from earlier sections that the NIH is responsible for a large portion of all federal research funding linked to medical technology innovation. In general, recent studies of the NIH and its use of research grants in the health and life sciences provide some evidence in support of federal research grant use. Studies have found that increases in total NIH funding for basic science increased the number of new molecular entity (NME) drug applications.\textsuperscript{20} A recent study also found that an increase in NIH spending on research grants for a specific disease area increased the number of Phase I trials conducted for treatments for that disease over the following 12 years.\textsuperscript{21} Studies of Small Business Innovation Research (SBIR) grants (research grants intended to encourage biomedical entrepreneurship) administered by the NIH also lend some support to the potential effect of research grants on encouraging technological development. For example, Toole & Czarnitzki (2007) found that firms linked to researchers that received the grant were more likely to receive follow-up funding from venture capital groups.\textsuperscript{22}

The studies that looked at the use of federal research grants to address public health and social needs have also found positive effects. However, most of these studies have been limited to descriptive analyses of research grant funding and indicators of innovation. Take for example, studies on the effect of the federal response to the HIV/AIDS epidemic. In the early 1980s, Congress began to earmark funds specifically for HIV/AIDS research in response to growing public concern about the disease.\textsuperscript{23} Between 1986 and 1990, funding for HIV/AIDS research had grown from 0.1 percent of the NIH’s budget to over 9 percent.\textsuperscript{23} Today the NIH spends approximately $3 billion for HIV/AIDS research alone, or roughly 10 percent of the NIH’s total research spending, much of which is allocated towards extramural research grants. In the decades since Congressional earmarks for HIV/AIDS began, major advances in prevention and improvements in the scientific community’s understanding of the disease have been traced back to research funded by federal grants.\textsuperscript{24} Researchers have also found that patents for HIV/AIDS therapies were much more likely to rely on publicly funded research than other therapies, as measured by citations of and coverage by public patents and publications.\textsuperscript{25} These studies are, however, mostly descriptive and only provide information about observed correlations between federal research grant funding and HIV/AIDS treatments. Ultimately, the limitations in scope and study design constrain our ability to draw stronger conclusions about the marginal contributions of federal research grants in HIV/AIDS research.
Federal research grants have been employed as part of other policy efforts; however, few have been able to isolate the effects of these research grants. In the same decade that HIV/AIDS funding increased, Congress passed the 1983 Orphan Drug Act (ODA) and created an FDA-administered grant program which provided funding to support clinical trials for orphan products. The law was passed to encourage the development of orphan drugs—drugs that target rare diseases—which were seen by many as an area of underinvestment on the part of the private sector. While the program has sponsored more than 500 studies and has an annual budget of approximately $14 million, evidence of the incremental effect of this grant program on the production of new technologies is limited. More recently, the 2007 Pediatric Medical Device Safety and Improvement Act created federal grants to support the formation of pediatric medical devices consortia. A Government Accountability Office (GAO) report found that the consortia had used these grants to assist 100 pediatric device projects between 2009 and 2010.26 Some studies have also attempted to anticipate how these reforms will influence technology innovation in the coming years.27 However, to date, the data about the program does not permit one to evaluate the incremental effects of the program.

B. Federal Research and Development (R&D) Tax Credits

Policy Motivation and Application

Tax credits represent another policy approach that has been used in the past to encourage the development of medical technologies. As cited in earlier sections, once an idea for a new medical product or service has been identified, innovators evaluate the idea to determine whether the expected returns are high enough to justify the risk involved in bringing the idea to market. These returns can be significantly diminished by a variety of factors including the presence of high development costs or the absence of sufficient market demand. Ultimately, if the prospective payoff for technologies that address a particular public health or social need is systematically insufficient, innovators and investors are likely to limit their efforts to develop ideas in these areas.

R&D tax credits can increase returns on investments in technologies that serve a public need. As in other industries, medical innovators pay taxes on the profits they generate from selling goods and services. R&D tax credits reduce this tax burden by an amount proportional to the cost of R&D activities related to the development of new medical technologies. Because the credits are proportional to R&D expenditures, the size of incentives are effectively proportional to the size and riskiness of the investment needed to develop that idea. When applied to specific technology areas, tax credits can
effectively increase the expected returns for investments in opportunities related to those technologies. In turn, the prospect of higher returns can attract more attention from innovators and investors.

When of R&D tax credits are employed, an immediate consequence is that existing firms working on technologies targeted by policymakers will enjoy greater returns. However, whether additional innovators and investors invest in developing new ideas in those same targeted technological areas depends on several factors. First, there must be ideas and opportunities to develop technologies being targeted by policymakers. Tax credits are likely to have minimal effects if the existing science does not allow innovators to identify opportunities to develop relevant technologies. Second, tax credits must raise enough prospective returns to make ideas for the desired technology as appealing as, or more appealing than, other opportunities for medical technology innovation.

R&D tax credits have been subject to a variety of criticisms. The central policy issue most frequently discussed is whether tax credits raise R&D spending rates enough to offset the cost of the tax credit. If spending rates increase by an amount less than the forgone tax revenue, it may be more efficient for the government to directly fund R&D spending, all else being equal. However, it is worth noting that this type of criticism has several shortcomings. First, it assumes that a federal agency would be able to allocate subsidies towards R&D activities as efficiently as private entities when given a tax credit—a relatively strong assumption. Moreover, criticisms based on tax elasticity ignore the social returns generated by R&D induced by the tax credit. It is conceivable that the R&D induced by tax credits may generate large social returns, particularly in situations where the R&D leads new scientific breakthroughs. These large returns could be enough to justify tax credits even if R&D spending increases less than the cost of the credit.28

Even when local aggregate R&D spending levels increase by more than the cost of forgone tax revenues, it is not necessarily the case that that global aggregate levels of R&D spending in key technological areas increase. Some have argued that tax credits induce firms to relocate their R&D activities to minimize their tax exposure rather than increase their level of investment in R&D. Particularly, in instances when innovation is constrained by the discovery of new ideas, it is possible that the credits may simply induce innovators to move their research facilities. Indeed, some evidence of this phenomenon has been recorded in previous studies of R&D tax credits across states.29

Evidence and Lessons from Past Policy Reforms

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There is still debate about the effects and effectiveness of R&D tax credits. In most cases, studies have found that tax credits are associated with increased R&D spending. However, there remains disagreement on the magnitude of this effect and whether it offsets the forgone tax revenue generated by the credit. For instance, using cross-country comparisons, Mansfield (1986) found that R&D spending rose by roughly a third of the cost of R&D credits.\(^\text{30}\) In contrast, panel studies of Organization for Economic Co-operation and Development (OECD) countries have found that, in the long run, R&D spending increases by approximately the same amount as the cost of the tax credits.\(^\text{28,31}\) Studies specific to the medical technology industry have also produced conflicting results. One study of California’s research tax credit for biopharmaceutical R&D found tax price elasticity in excess of one.\(^\text{32}\) However, studies of federal R&D tax credits on the pharmaceutical industry have produced results that were closer in line with the findings of Mansfield (1986).\(^\text{33}\)

Data and evidence on the use of R&D tax credits to encourage the development of specific technologies is relatively sparse. The most well-known use of R&D tax credits to increase the production of specific technologies was their application in the ODA. In addition to the federal research grants that were authorized as part of the ODA, innovators developing orphan drugs were given tax credits equal to 50 percent of their R&D expenditures.\(^\text{34}\) Between 2008 and 2010, more than $1.5 billion in credits were claimed under this program.\(^\text{35}\)

While several studies have looked at the impact of the ODA, there is little information about the effect of the tax provision it created. More than 300 orphan products have been approved since the law was passed, which is much more than the 34 products approved in the 17 years preceding the legislation.\(^\text{36,37}\) Researchers have also found that NME approval rates for orphan drugs increased substantially after the ODA.\(^\text{38}\) What remains unclear is to what degree the R&D tax credits were responsible for the observed increases in orphan drug production. The R&D tax credits were implemented alongside several other reforms targeting orphan products, making it difficult to distinguish which policies were primarily responsible for the increased production in medical technologies.\(^\text{39}\) One study has found that the ODA tax credits increased the number of clinical trials conducted for therapies targeting rare diseases.\(^\text{40}\) However, in order to draw these conclusions, the study had to ignore any potential impact of the other policy changes enacted as part of the ODA.

Recently, R&D tax credits in the 2009 Affordable Care Act (ACA) were approved to encourage research that could help develop new medical technologies that addressed an unmet medical need. Innovators that qualified for this program—known as the Qualifying Therapeutic Discovery Projects program—were
offered a choice between a tax credit and grant to cover 50 percent of their R&D costs. Most innovators opted to receive grants, but between 2009 and 2010, $18 million in tax credits had been approved as part of this program. This amounted to a little under 2 percent of the program’s total budget.\textsuperscript{41} Few conclusions can be drawn about the policy’s effect as any potential impact is likely to emerge over the coming years.

C. Expedited FDA Approval

_Policy Motivation and Application_

The provision of expedited FDA Approval represents a third policy approach that has been employed in recent decades to encourage technology development. As discussed in Section II, the production of medical technologies targeted by policymakers can be limited because of prohibitively high development costs and risks. After an idea for a new technology has been identified, innovators need to consider the R&D activities needed to bring the product to market. Some of these tasks are related to working out the technical details and scientific feasibility of an idea, while others may be oriented toward demonstrating a new product’s safety and efficacy. When innovators and investors anticipate high costs and risks for technologies targeted by policymakers, the unfavorable risk-return profile can lead to underinvestment in ideas in target areas.

Policymakers have tried to reduce the development costs and risks for socially desirable medical technologies by reducing the regulatory burden imposed by the FDA. Recall that the FDA is responsible for verifying that new medical products are safe and efficacious, and sets the threshold for evidence that must be met by innovators. The verification process and evidence threshold can be modified to make the FDA’s review less costly and less risky for technologies that may serve a public health or social need. By improving the return-risk profile for a given technology, modifications to the FDA regulatory review process can attract investments and the attention of prospective innovators and investors.

The FDA can expedite its review process and reduce the burden on innovators in a number of ways. First, the FDA can agree to accept surrogate endpoints in clinical trials for targeted technologies. Surrogate endpoints are alternative measures of efficacy that are more easily and rapidly collected than established endpoints. By accepting these as evidence, the FDA can reduce the length of time needed to demonstrate efficacy of a given technology. Another way the FDA can expedite the review process is to enhance its coordination and communication with innovators. When innovators have the opportunity to work more closely with the FDA, it can increase the chances that the evidence innovators produce will
meet the requirements of the FDA’s review process. Lastly, the FDA can accelerate its own review of the evidence submitted by innovators. This can be achieved by setting review benchmarks within the agency or allowing the innovator to provide data for their FDA application as it is produced.

In order for expedited FDA review to be effective in encouraging the development of new medical technologies, several conditions need to be met. As with tax credits, it must be the case that other limiting factors are not in play, such as the absence of basic scientific knowledge. Innovators and investors must also be able to anticipate which ideas will qualify for accelerated approval. If it is unclear which technologies will qualify, expedited review may not change which ideas innovators select for development. Lastly, if the reduction in cost and uncertainty is small relative to other sources of development costs and risks, accelerated FDA review may only have modest success in encouraging the development of medical technologies.

Perhaps the greatest concern about accelerated review is the potential trade-off between shorter development times and safety and efficacy. Critics have expressed concern that by accelerating the FDA review process, inadequate analysis may be conducted or insufficient safety data will be collected. This shortcoming could lead to ineffective medical products, or worse, products that are unsafe for the public.

Evidence and Lessons from Past Policy Reforms

The basis for using FDA expedited review to encourage innovation is the assumption that the FDA’s regulatory approval process imposes non-trivial costs on innovators. For the most part, this assumption has been supported by past studies. The modern form of the FDA and its review process were instituted as part of the 1962 Federal Food, Drug, and Cosmetic Act. Among other things, the law established the evidentiary requirement for drugs and pharmaceutical approval. Early studies found that the new law decreased R&D spending in the pharmaceutical industry by nearly 20 percent and reduced overall R&D productivity (i.e. the amount spent per new product). More recent studies have produced similar findings. One study found that the regulations reduced the research productivity in small firms as measured by the production of NMEs.

The capacity of expedited review to encourage medical technology is perhaps best demonstrated by the response to the 1992 Prescription Drug User Fee Act (PDUFA). The main provision of the law was that new review fees would be charged for new drug and biologic license applications. In 1993, the application fee was set at $100,000, which was small in comparison to the $10 million cost of an
additional one month of delay due to review.\textsuperscript{45} While the fees ostensibly increased the cost of development for many medical innovators, the law effectively reduced review times by allowing the FDA to allocate additional resources to its review process. Indeed, with the passage of the law, the FDA created a set of review timeline targets.

Most of the evidence to date suggests that PDUFA both accelerated new product review times and helped spur medical innovation. In the five years following the passage of the legislation, the average approval time for NMEs declined by almost 10 years. One study estimated that absent PDUFA, the approval times for several classes of drugs would have increased by upwards of 30 percent.\textsuperscript{46} As a result of these declines, studies have shown that innovators, particularly in the pharmaceutical industry, increased the amount of R&D spending.\textsuperscript{47} Similarly, some estimates have suggested that the law increased the returns to innovation by $11 billion for pharmaceutical producers.\textsuperscript{48} However, the reaction to PDUFA has not been entirely positive. In the decade following its passage, recalls of a handful of drugs caused some critics to question whether safety had declined in exchange for faster approvals.\textsuperscript{49}

For the most part, past implementations of FDA expedited review to encourage specific technologies have provided limited evidence of the effects of this policy approach. However, several important lessons can be drawn from these earlier experiences. One of the earliest attempts to expedite the review process for specific technologies was the FDA Accelerated Approval (AA) program. Initiated by the FDA in 1992, the program allowed the FDA to approve drugs that addressed serious conditions that fill an unmet medical need based on their ability to demonstrate efficacy on surrogate endpoints (e.g. tumor reduction for cancer treatments). The program was officially put into law in the 2012 Food and Drug Administration Safety Innovations Act. Overall, the AA program has produced mixed results. Some studies have found that the use of surrogate endpoints did not accelerate approval times or reduce the regulatory cost born by some drug developers.\textsuperscript{50} Moreover, some have criticized the program because it provided disincentives for innovators to conduct trials that demonstrated clinical benefit in a timely manner.\textsuperscript{51}

Another example of policymakers’ attempts to expedite the FDA review process for specific technologies was creation of the FDA’s Fast-Track program. Instituted by the 1997 FDA Modernization Act, the program was created to accelerate the development and approval of therapies that addressed an unmet medical need. The program offered qualified applicants, among other things, more frequent correspondence with the FDA and Rolling Review—a review process that allows applicants to submit their application in sections as data become available rather than wait to submit one final application.
To date, there is some evidence that the program has accelerated the development and approval times for the therapeutics that have entered into the program.\textsuperscript{38} However, much less is known about whether these programs have increased the production of technologies that prospectively would have benefited from the program.

Recent legislation has used reduced regulatory review times as an incentive to encourage the development of treatments for neglected tropical diseases. Known as the Priority Review Voucher (PRV) program, the provisions were approved as part of the 2007 FDA Amendment Act. Originally a proposal from faculty members at Duke University, the program rewards manufacturers that create drugs for neglected diseases with a voucher that guarantees a separate drug of their choosing a Priority Review designation. The FDA designation is tied to a review turnaround target of six months. In one article, proponents of the idea estimated that the vouchers could increase the value of sales for a blockbuster drug and could serve as a more than $300 million incentive.\textsuperscript{52} While it may be too early to evaluate the impact of these policies, the current data suggest that the policy may have only a modest effect. One of the first uses of the PRV program has been considered by many to be a failure. The first award recipient, Novartis, was able to obtain a voucher by exploiting a loophole in the program. The firm submitted an application for a drug that had been developed years earlier, but had not been registered in the U.S.—allowing it to qualify for a PRV.\textsuperscript{53} Moreover, when the firm used the voucher to obtain early approval for its drug Illaris, it experienced delays in its review due to FDA requests for additional data—offsetting the value of the PRV.\textsuperscript{54} One survey of manufacturers did find that the PRV program is often considered when making investment decisions. However, it also found that less than half of the firms interviewed considered the program to be a determining factor in their product development decisions.\textsuperscript{55}

The latest attempt to use FDA expedited review to encourage the development of specific technologies is the Breakthrough Therapy Designation program. Created as part of the FDA Safety and Innovation Act of 2012, the program extends many of the same benefits as the Fast-Track Program to drugs that treat a serious condition and demonstrate substantial improvement over existing therapies based on preliminary clinical evidence. The program also offers recipients guidance on efficient drug development and an organizational commitment from FDA senior managers. While some have expressed optimism about the program, it is too early to tell whether this program will induce innovators and investors to develop these technologies.\textsuperscript{56}

D. FDA Market Exclusivity
Policy Motivation and Application

FDA market exclusivity represents a second way the FDA’s functions have been modified to encourage innovation in specific technological areas. Like tax credits, FDA market exclusivity can be used when insufficient returns are primarily responsible for the under-development of technologies targeted by policymakers. Market exclusivity can be generally thought of as the ability to operate as the sole supplier of a technology for a market. The FDA can enforce market exclusivity for specific technologies in one of two ways. First, the agency can offer innovators in a particular technology area product exclusivity (sometimes simply referred to as market exclusivity). Under this guarantee, the agency agrees to not approve any similar drug or device for the U.S. market for a specified period of time. Secondly, the agency can offer technology developers data exclusivity. Data exclusivity prevents competitors from using data generated by an innovator for the FDA approval process. Provided that the data were costly to generate, the provision allows the FDA to confer a weaker form of market exclusivity.

FDA market exclusivity can encourage the development of specific technologies in a manner similar to what might be achieved through stronger patent rights. When an innovator is granted market exclusivity for a new technology, the absence of direct competitors allows them to charge higher prices for their product. These higher prices, in turn, can increase the expected profitability of and returns to a new medical technology. When a class of medical technologies has the potential to receive FDA market exclusivity, the higher expected returns can induce innovators to consider ideas in those fields more favorably when determining how to invest their time and resources.

The exclusivity conferred by the FDA differs from the rights conferred through patents on a couple of important dimensions. First, unlike patents, FDA market exclusivity is narrower in scope. FDA exclusivity typically prevents an interchangeable product from being approved for the same indication. An equivalent product may still be approved for another indication or alternative uses. Secondly, FDA market exclusivity differs from patents in that the benefits accrue passively as part of the FDA approval process. Once the FDA approves an application for a new drug or medical product, the FDA’s exclusivity provision is automatically enforced without additional action from the innovator.

One potential drawback of FDA market exclusivity is its potential to limit patient access and dramatically increase technology costs. A necessary tradeoff for the provision of market exclusivity to innovators is higher prices for new medical goods and services. These higher prices can limit patient access to new
technologies, particularly in cases when patients are price sensitive due to limited insurance coverage. While the tradeoff is expected, the provision may lead to net welfare loss if the expected profits granted by exclusivity rights exceed what is needed to induce investments in targeted medical technologies.

Evidence and Lessons from Past Policy Reforms

The importance of market exclusivity in medical technology innovation has been primarily established in the literature on patent law. Several studies have tried to examine the relationship between patent laws and R&D spending. In many cases, researchers have found that countries with well-enforced patent laws (and by extension market exclusivity rights) tend to have higher R&D spending rates. It is worth noting, however, that the effects of changing patent strength, particularly on the margins, have remained open to debate. Literature surveys and studies of global intellectual property rights regimes have found that current evidence does not support the conclusion that increases in patent strength can induce higher rates of innovation or R&D spending.

In general, studies of past implementations of FDA market exclusivity have offered limited evidence about the policy’s effects. Take for instance the use of FDA market exclusivity in the ODA. In addition to research grants and tax credits, market exclusivity was used as part of an effort to encourage the development of orphan products. As one of the earliest attempts to leverage the FDA’s role as gatekeeper, the legislation included provisions that allowed the FDA to provide orphan drugs with seven years of product exclusivity. Although some have attributed the increased production of orphan products to the FDA market exclusivity provisions, the existence of concurrent policy interventions prevents researchers from identifying what effects were uniquely linked to the FDA market exclusivity provisions. It is worth noting that some individuals have criticized the policy based on the large financial rewards that some firms have gained from developing orphan products. One study found that 9 percent of orphan drugs that were granted market exclusivity by the FDA achieved blockbuster status, generating more than $1 billion in revenue.

FDA market exclusivity was also used in the 1984 Drug Price Competition and Patent Term Restoration Act (Hatch-Waxman Act) to encourage the production of generic drugs. Prior to the Hatch-Waxman Act, there were public concerns that pharmaceutical prices were too high, in part due to the absence of generic competitors. In response to these concerns, the law authorized the FDA to provide 180 days of product exclusivity to the first manufacturer to file for FDA approval of a generic version of a branded drug following the branded drug’s patent expiration. Studies have found that in the years immediately

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following the law’s passage, many brand name drugs experienced increased generic drug competition. However, the degree to which these effects are attributable to product exclusivity is not clear. Around the same time the Hatch-Waxman Act was passed, many drug patents were set to expire. Existing data do not allow us to determine whether generic entrants in the late 1980s and early 1990s were responding to the policy or would have entered the market without additional incentives. In fact, studies of generic entry in the 1990s found that generic entry slowed during this period.

The Hatch-Waxman Act’s product exclusivity provision for generics has also been noted for its unintended and unwanted consequences. To qualify for the 180-day exclusivity provision, the first generic entrant was required to list all previous patents that were invalid, but potentially infringed upon by the generic entrant. In some cases, incumbent drug developers leveraged this requirement to challenge the FDA approval process for a prospective generic product/competitor. These delays sometimes led to outright settlements where prospective generic competitors agreed not to enter a market for a period of time in exchange for financial compensation from the incumbent firm. As a result, some products observed delayed generic entry, an outcome counter to the policy’s original intent. The passage of the Medicare Prescription Drug Act has closed some of the loopholes that facilitated delayed entry. However, some have argued that the existing legislation still maintains provisions that limit the degree to which branded drugs face competition from generic entrants.

More recently, FDA market exclusivity has been used to encourage the development of drugs for pediatric populations. Known as the Pediatric Exclusivity Program, the provision was created as part of the 1997 Food and Drug Administration Modernization Act and re-authorized in 2002 with the passage of the Best Pharmaceuticals for Children Act. As part of both laws, manufacturers could obtain an additional six months of FDA product exclusivity if they conduct clinical trials related to pediatric use for their products. Between 1998 and 2004, more than 250 pediatric studies were submitted to the FDA as part of the program. However, it is not clear which of the studies would have been conducted absent policy intervention. Furthermore, evaluations of the Pediatric Exclusivity Program have also found evidence that the cost of exclusivity may have outweighed the benefits. One study of the trials that were conducted as part of the pediatric exclusivity program found that some manufacturers received relatively high returns on their clinical trial investments, with returns exceeding 70 percent in some cases. Further examination of the trials that qualified for the program also found that the trials were conducted for drug entities that were infrequently used by children.
V. Discussion and Conclusion

Policymakers have a wide range of policy options that can be used to encourage the development of medical technologies that address public needs. Federal research grants, federal R&D tax credits, expedited FDA review, and FDA market exclusivity have been among the most frequently employed policy approaches in past U.S. policy efforts.

Based on my review of the existing literature, I find that there are still sizeable gaps in our understanding of the individual effects of these four policy approaches in previous applications. Empirical studies that looked at variations in policies over time and geography have offered useful insights about the marginal effects of policies like federal research grants and federal R&D tax credits. However, studies of previous implementations of these policy tools in the U.S. have been, for the most part, limited. Whether it has been federal research grants or FDA market exclusivity, each policy intervention has rarely been implemented in isolation. Consequently, most studies of past U.S. policy efforts to encourage technology development are unable to distinguish the effects of the individual provisions that comprise a piece of legislation. Moreover, most of these studies of past U.S. policy efforts have been descriptive in nature, relying primarily on trends over time to evaluate policy effects.

It is worth noting that past policy efforts have also offered limited evidence to support the primary criticism of individual policy approaches reviewed in this study. For instance, there is no evidence to suggest that the use of research grants in response to the HIV/AIDS epidemic and in the ODA significantly displaced any private R&D efforts. Similarly, it is not clear that changes in R&D spending insufficiently justified the use of R&D tax credits. And while the use of FDA market exclusivity has been linked to substantial profits for drug manufacturers, the evidence from policy efforts does not indicate that these profits have effectively restricted access to the new treatments. Lastly, despite the recall incidences following PDUFA, it is not clear that expedited FDA review processes have led to any decreases in safety for new medical technologies.

Despite our limited ability to infer the effects of individual policy approaches, past policy experiences do offer some important insights for policymakers. For example, past policy efforts have demonstrated that combinations of policy interventions can effectively increase the development of specific technologies. Efforts like the ODA, which combined several policy tools, have been successful in promoting the development of therapies for rare diseases. This conclusion has been supported by existing time trends as well as empirical studies using cross-country comparisons. However, an important caveat is that we
do not know how efficient these past policy efforts were. It is possible that the same effects could have been achieved using a different combination of policies.

Past experiences also shed light on potential policy pitfalls to avoid. For example, the procedures established for the 180-day market exclusivity provisions for generic drugs effectively undermined the original intent of policymakers. Future policymakers may be able to limit similar abuses by working more closely with stakeholders to develop improved administrative procedures. Similarly, the application of market exclusivity to encourage pediatric pharmaceutical clinical trials has exposed the problems that can emerge when provisions are inappropriately targeted. Instead of promoting safer dosing for drugs used by children, the design of the program has created incentives for manufacturers to conduct pediatric clinical trials for highly profitable pharmaceuticals which may not be used by pediatric populations. Fine-tuning the regulations to target only therapies that would benefit pediatric patients might have helped avoid these problems.

Looking ahead, policymakers can take several steps to ensure that future policy efforts to encourage medical technology innovation produce the desired effects. First, to the extent possible, policymakers should try to determine whether the problems that constrain the development of technologies being targeted are related to the identification of new ideas or the selection of new ideas. As the conceptual model presented in this paper suggests, policies like federal research grants may be more appropriate in the former case. Policies that affect the profitability of technologies may be more appropriate in the latter case. Secondly, once a policy approach has been selected, policymakers should acknowledge the potential for adverse consequences and take steps to mitigate these risks. For instance, when using FDA market exclusivity to promote specific technologies, steps should be taken to ensure that new policies include language to limit the potential for abuse.

Policymakers should also be conscious of the full breadth of policy interventions when targeting medical technologies. This paper’s review of policy tools was limited to those previously employed in the U.S.; however, these policies do not represent all of the potential options available. For example, innovation prizes have been touted by many researchers as a potential alternative to existing policy tools used to encourage technology innovation.73 Changes to public insurance program policies also have the potential to change where medical technology innovators focus their attention.74 For example, the ACA included several payment system reforms to the Medicare insurance programs. These reforms created financial incentives for providers to deliver more efficient care and adopt electronic medical records, both of which may spur the development of healthcare information technologies.
Ultimately, additional research is needed to improve policy efforts to encourage medical technology development. For federal research grants, R&D tax credits, expedited FDA review, and FDA market exclusivity, additional studies that leverage quasi-experimental designs to identify causal effects could help supplement existing case studies and provide a better characterization of the marginal effects of different policies. For other policy approaches, the experience of other countries employing these approaches and larger empirical studies may offer useful insights about how they might be applied in the future.

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Based on the experience from past policy reforms, it is not clear how effective individual policy interventions might be at encouraging the development of specific technologies. The most effective policy efforts to date have used a combination of several policy approaches. However, past successes do not necessarily reflect the most efficient outcomes. It is possible that other policy approaches may have achieved the same results at lower social costs. Policymakers looking to encourage medical technologies for future unmet social needs can take a number of steps to ensure that the best policy strategy is adopted. This includes ensuring that policy interventions address critical barriers as well as developing strategies to mitigate the possibility of unintended and adverse consequences linked to past policy efforts. Ultimately, additional research is needed to determine the appropriateness of the policy approaches covered in this paper, as well as promising policy approaches that have yet to be implemented in the U.S.
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The Effect of Federal Research Funding on the Formation of University-Firm Biopharmaceutical Alliances

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ABSTRACT

The National Institutes of Health (NIH) is the largest funder of health and life science research in the U.S. The research sponsored by the agency has continued to aid in the development of new biopharmaceutical therapies, many of which are commercialized via university and biopharmaceutical firm alliances. In this paper, we examine this commercialization pathway more closely and evaluate the effects of university research funding by the NIH on the formation of such alliances. Using an instrumental variable approach, we estimate that every $100 million in NIH research grant funding awarded allows a university to form an additional 2.68 alliances over a five-year period. Although the results of our study are sensitive to model specifications with different lags, the overall findings indicate that NIH research grants increase the number of commercial alliances formed by a university.

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I. Introduction

The National Institutes of Health (NIH) is the largest funder of health and life science research in the U.S. In 2011, the NIH spent over $21 billion to support research and development (R&D) projects, of which approximately $11 billion went to support extramural research at universities and other non-federal research institutions.\(^1\) The research funded by the NIH has led to many advances in the health and life sciences, including the development of several high-impact therapeutic drugs.[1]

While the NIH has garnered a high level of public support, it has not been insulated from recent political pressures to reign in discretionary federal spending. In 2013, the sequestration cuts authorized by the 2011 Budget Control Act (BCA) reduced the NIH’s total annual budget by 5 percent or $1.55 billion. [2] Critics from the scientific community as well as patient advocacy groups have argued that these cuts have threatened the future of the U.S. biomedical research enterprise. Due in part to these criticisms, Congress has been working to roll back budgets cuts for the NIH in 2014 and beyond. However, as of this writing, there is considerable uncertainty surrounding the level of public research the NIH will be able to fund in the coming years.

The cuts authorized by the BCA are the latest chapter in a long-standing debate over federal research funding. At the heart of the debate is the following question: how much funding should the federal government allocate toward research? In addition to the burden placed on taxpayers, every dollar spent by the NIH on research means one less dollar for other public needs like education and infrastructure. There is, therefore, a continuing pressure to evaluate the effectiveness of federal R&D funding, especially given the recent fiscal challenges and the need to identify high-return investments for scarce government dollars.

With this context in mind, we evaluated the effects of NIH extramural research grant programs on university-firm alliances in the biopharmaceutical industry. These alliances represent a common way by which basic health and life science research are commercialized to develop new healthcare products. Assuming that alliance formation rates respond to the quantity and quality of health and life science research conducted at a university, one can use the formation of commercial alliances as a measure of the effect of the NIH’s research grant program on research productivity.

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1 According to data reported on the NIH’s REPORT website: [http://report.nih.gov/index.aspx](http://report.nih.gov/index.aspx)
Using an instrumental variable approach and linear model for commercial alliance formation, we estimate that every $100 million in NIH research grant funding awarded allows a university to form an additional 2.7 alliances (P<0.10) over a five-year period. Estimates using a Poisson model also suggest positive effects. With the latter model, we find that every $100 million in NIH research grant funding increased the rate of university-firm alliance formation by 2.4 percent over a five-year period (P<0.10). Although the results of our study are sensitive to model specifications with different lags, the overall findings of our study indicate that NIH research grants may play a major role in commercial alliance formation. The economic implication of this role is substantial when one considers that each of these alliances is potentially a multi-million dollar transaction. Additional data and further research on NIH research funding and commercial alliances are needed to fully quantify these effects.

The rest of this paper proceeds as follows. In Section 2, we provide the background for this research and compare it to previous literature. The data and econometric methodology used in our analysis are presented in Sections 3 and 4, respectively. Section 5 presents the results, and in Section 6, we provide a more detailed discussion of these results. In Section 7, we summarize the main conclusions from our study.

II. Background and Prior Literature

A. The Effects of Federal Research Funding

Quantifying the effects of federal research funding presents a series of unique challenges. First, federal research funding can have far-reaching benefits, all of which are unlikely to be captured by a single outcome measure. Moreover, the marginal increase in research from federal funding can generate social benefits that are realized many years into the future. This lag between funding and effects makes it difficult to define an appropriate period of observation. Lastly, federal research grant funding is rarely randomly allocated across regions and individuals. The absence of random assignment limits the extent to which one can infer causal treatment effects. Together, these challenges have given rise to a diverse set of studies using different approaches and quasi-experimental designs to examine the impact of federal research funding.

Payne and Siow (2003) offers a notable example of earlier attempts to investigate the impact of federal research funding on research productivity. The authors examined the relationship between total U.S. federal research funding at 68 research universities and their productivity in terms of publications and
patents. To identify their models for research production, the study employed an instrumental variable based on university and geographic affiliation of Congress members sitting on key House and Senate appropriations committees. Using this approach, the authors found that a $1 million increase in federal research funding at a university resulted in 0.2 additional patents and 10 additional publications. [3]

With respect to the NIH’s research funding efforts, past studies have examined its effects on both university research and industry productivity. Studies conducted by Jacob and Lefgren are examples of the former. In Jacob and Lefgren (2011), the authors examined the effect of the NIH’s postdoctoral training grants on publication production. Using a regression discontinuity design, the authors find that the receipt of a postdoctoral fellowship led to one additional publication over five years. [4] In a second study, Jacob and Lefgren (2011, 2), the authors examined the effects of the NIH’s R01 research grant funding research on individual publication productivity. The authors again found a positive effect. Their results suggested that the receipt of an NIH R01 research grant led to a 7 percent increase in publication production. [5]

More recent research has tried to examine the effects of NIH research funding on productivity in the biopharmaceutical industry. For example, Toole (2012) examined the degree to which NIH funding contributed to the production of new drugs in the pharmaceutical industry. Using a model of knowledge stock and flows, the author found that NIH funding has positive effects on the number of new molecular entity (NME) drug applications, estimating that a 1 percent increase in public basic research funding increased the number of NME applications by 1.8 percent. [6] Blume-Kohout (2012), using a different approach, looked at the impact of changes in the allocation of NIH funding across diseases on the number of drugs entering clinical testing to treat those diseases. The study found that a sustained 10 percent increase in funding in a disease area increased the number of Phase I clinical trials for related therapies by 4.5 percent. [7]

B. University-Firm Biopharmaceutical Alliances

Models that connect NIH research grants to biopharmaceutical industry productivity intend to capture some type of knowledge transfer from research institutions to commercial interests. In order for the university research funded by the NIH to eventually influence the production of innovations like new pharmaceutical products, discoveries must be disseminated by universities and become known to commercial firms. This knowledge transfer can occur through a variety of channels, including peer-
reviewed publications and the training of skilled labor. Perhaps the most direct means of knowledge transfer is the formation of university-firm alliances.

The importance of university-firm alliances to the biopharmaceutical industry is widely recognized. Past studies have found that these alliances are closely linked to a commercial firm’s likelihood of success. For instance, Zucker (2002) explored the impact of these collaborations on firm success. The study found that firms with researchers that co-authored publications with a “star” scientist at a university produced a larger number of patents. [8] George et al (2002) found similar results. In their study, the authors found that firms that engaged in more university alliances were more productive and experienced lower R&D costs. [9]

The formation of university-firm alliances is closely tied to the productivity of university research. University-firm alliances are a common pathway by which university research is translated into innovations. [10] The alliances often transfer rights to research discoveries from universities to the participating companies in exchange for some form of financial support. [11] Firms also engage in these alliances because they provide access to their partners’ R&D facilities and expertise [12]. Consequently, if a university produces higher quantities and better quality research, one might expect to observe the university to form commercial alliances more frequently, all else being equal.

The relationship between alliances and university research productivity allows us to use alliance formation as an intermediate outcome measure for the effects of NIH funding. To date, most examinations on the impact on federal research grants on industry alliances have examined these effects at the level of individual researchers. For example, one study found that federal grants increase the degree of industry collaboration among academic researchers.[13] In this study, we extend this line of research and examine whether NIH research funding facilitates industry collaboration at the university level through biopharmaceutical alliances.

**III. Data & Sample**

**A. Data Sources**

The data used in this study was drawn from three sources. Data on the annual NIH funding for research grants received by individual universities was accessed through the NIH’s Research Portfolio Online Reporting Tools (RePORT). Data on university-firm alliances was extracted from Deloitte Recap’s “Deal
Builder” database. Lastly, data on congressional subcommittee membership representation was obtained through historical records maintained by the U.S. Congress.

The initial sample of universities in this study was constructed using administrative records of NIH funding for research project grants. In a given year, the NIH funds extramural research through newly awarded research grants and commitments from research grants that were awarded in one or more years prior. The NIH maintains a grant-year record for each year in which the NIH actively disburses funds for a research grant. Each record includes information about the amount of funding disbursed, the university and principal investigator, the NIH Institute or Center (NIC) that originated the research grant, and the number of years that have elapsed since the research grant was awarded. We extracted grant-year records for all NIH funding awarded to higher education institutions between the years 1992 and 2011.\(^2\) We restricted our sample to this period as it contained the years for which the NIH had complete funding data available at the outset of this study. In total, our study collected 13,880 grant-year records which included 682 unique university recipients of NIH funding through research grants.\(^3\)

The primary outcome variable for this study was university-firm alliance counts. The variable was constructed using records of pharmaceutical and biotechnology alliances contained in Deloitte Recap’s “Deal Builder” database. The records were created using a variety of methods including Freedom of Information Act requests and partial disclosures by private firms.[14] Each individual record includes information that identifies the licensor and licensees that form the alliance, the year in which the alliance was formed, the stage of development of the technology that forms the basis of the alliance, and information on the relevant disease space for the technology. Some records also included data on the value and terms of a partnership. However, since this information was limited for many alliances, it was not included in the analysis. To coincide with the NIH funding data, we extracted all records for alliances that were formed between the years 1992 and 2011. Our original sample of alliance data contained 3,810 unique pharmaceutical and/or biotechnology alliances.

Selected plots of the NIH and NIC funding and the commercial alliances formed in their respective disease areas are presented in Figures 1 through 5 in the Appendix. The data for total annual NIH

2The records were limited to funding that was awarded as part research project grants that were not affiliated with the Small Business Innovation Research (SBIR) and Non-Small Business Technology Transfer (STTR) programs. Funding tied to SBIR and STTR programs is intended to promote the development of university research spin-off companies and small businesses. The focus of this study was to examine the effect of the extramural funding that comprises the bulk NIH budget that is meant to promote

3 The original data included 694 unique university entries. However, for 12 of the university entries, the names were spelling variants of universities that were already accounted for.
funding loosely tracks the total annual number of university-firm alliances formed. The correlation between funding and alliance formation changes when the funding and alliance data are broken down by disease classification. For instance, we see that alliance formation in an NIC’s disease areas more closely tracks funding for the National Cancer Institute (NCI) funding and National Institute of Neurological Disorders and Stroke (NINDS). The relationship between funding and alliance formation is less clear for the National Institute of Allergy and Infectious Diseases (NIAIDS) and National Heart, Lung and Blood Institute (NHLBI).

B. Matching Alliances to NIH Funding

Data on pharmaceutical and biotechnology alliances were matched to universities identified in the NIH administrative records through a series of steps. We started by using a fuzzy matching algorithm to score matches between the names listed in the licensor field for each alliance record to the set of university names contained in our set of NIH funding data. Using the scores from the fuzzy matching algorithm in combination with manual reviews, we linked the NIH reported university names to alliances that involved the same university. In situations where the alliances involved multiple universities identified in the NIH records, we attributed the alliance to all of the relevant universities. When the alliance record identified the licensor as a university system without specifying the campus, the record was attributed to the largest campus of the university system that was listed in the NIH administrative data. The last attribution procedure that was applied was to assign alliances that were linked to a university’s research institutions, primary teaching hospitals, or research organization to that university. Two separate variants of the last alliance attributions rule were applied for sensitivity analyses. The first, more conservative, attribution rule excluded alliances from a university’s research institutions, primary teaching hospitals, or research organization. The second, less conservative, attribution rule included all potential university affiliates, including non-primary teaching hospitals.

Following the matching process, the NIH funding and biopharmaceutical alliance data were aggregated to create a university-year panel dataset. The panel covered the period between 2001 and 2011, the years that supported the lag structure employed in models in this study. We also excluded any university that lacked at least one alliance during the study period. In total, our panel covered 134 universities. Together, these universities represent 77% of all NIH extramural funding awarded to university institutions during this period. Table 1 provides the descriptive statistics for the study’s analytic dataset. We also included statistics for research grant funding data originating for selected NIC - NCI, NIAID, NHLBI, and NINDS - and the commercial alliances stratified by the disease classes most likely to
correspond to research sponsored by these institutes. Overall, the median and interquartile range (IQR) of our data suggests that while the levels of funding received by a university are higher, there is considerable variation between university-years. At the institute level, the level of variation is even larger. We also observe, perhaps not surprisingly, that the number of alliances formed by a university in a year is on average 0.5, suggesting that these alliances tend to be relatively rare events.

The data for our study panel were aggregated by year and organization and presented in Figures 1 and 2. While a clear pattern does not emerge between annual NIH research grant funding and commercial alliance formation, we do observe a relatively high correlation between the total amount of NIH research grant funding received during 2000-2011 and the number of commercial alliances formed by individual universities during that period \( \rho = 0.644 \). Our aim in the rest of the paper is to explore if there is a causal connection between NIH funding and alliance formation at the university level.

**IV. Methodology**

**A. Specification**

We modeled the relationship between NIH research grant funding and university-firm alliances in two ways. In our first model, we assumed a linear relationship between NIH research grant funding and the number of university-firm alliances formed. We used a linear model because it provided a simple base case and allowed us to generate easily interpretable results. However, in a given year, many universities may fail to form a commercial alliance altogether. Consequently, a linear estimator might be inefficient as it fails to account for the discrete and non-negative (or “count”) nature of university-firm alliance data. Therefore, we also used a Poisson model for university-firm alliance formation. In the second model, the key assumption is that NIH research grant funding and other covariates effect the annual rate of alliance formation through a log-linear relationship.

An important consideration for our study’s specification was the appropriate lag structure. The standard argument for including multiple lags for models of federal research funding is that often time the effects of research funding may not be realized instantaneously. For university-firm alliances, the effects of federal funding may not be realized until many years after funding has been received by a university. The delay may occur for several reasons. First, after a university has received funding, it takes time for the university to use the funding to conduct research and for that research to yield results that may form the basis of a university-firm partnership. Secondly, even after new discoveries have been
uncovered, it may take time for those discoveries to disseminate into the public domain and for firms to identify them as potential commercial opportunities.

Taking these factors into account, we included NIH research grant funding lags for the proceeding five years in our main specification. The lag structure was based on the grant guidelines for the NIH’s extramural research program. The NIH is congressionally mandated to keep the average length of research grants to four years.\(^4\) Including five years of lagged funding covariates would allow for our study to capture any potential effect from information that takes one year to disseminate and was published at the conclusion of a research grant where funding was awarded over four years. To test the robustness of our results, we examined our model specification with several alternative funding lag structures.

We also included in our main specification fixed effects for the year and university. Fixed effects were included to account for any bias resulting from secular trends or time invariant university characteristics that are correlated with both alliance formation and receipt of federal funding. For instance, universities with more faculty members or stronger research reputations may attract both greater federal funding and have a greater ability to form alliances. These omitted variables could yield a positive association between federal funding and alliance formation, even if no causal relationship exists.

The Generalized Least-Squares Model (GLM) link function for both the linear regression and Poisson regression are presented in Equations 1 and 2, respectively. The terms \(u\) and \(t\) denote the university and time for each observation. For the linear model, the left-hand side of the equation, \(E(Alliance_{u,t}|X_{u,t})\), is the expected number of alliances formed conditional on our model covariates. For the Poisson model, the left-hand side of the equation, \(AllianceRate_{u,t}|X_{u,t}\), can also be interpreted as the annual rate of alliance formation. The right hand side of the equations consist of the terms \(NIH\), \(v\), and \(\tau\) which represent NIH funding, university fixed effects, and time fixed effects respectively.

\[
E(Alliance_{u,t}|X_{u,t}) = \alpha_0 + \sum_{n=1}^{5} \beta_n \cdot NIH_{u,t-n} + v + \tau \tag{1}
\]

\[
AllianceRate_{u,t}|X_{u,t} = E(Alliance_{u,t}|X_{u,t}) = \exp[\alpha_0 + \sum_{n=1}^{5} \beta_n \cdot NIH_{u,t-n} + v + \tau] \tag{2}
\]

The marginal effects of NIH funding implied by the linear and Poisson model are presented in Equations 3 and 4, respectively. Parameter estimates for the NIH funding covariates in the linear model can be

\[^4\]See FAQ on Research Grant Length: [http://www.nigms.nih.gov/Research/Application/ResearchProjGrantDuration](http://www.nigms.nih.gov/Research/Application/ResearchProjGrantDuration)
interpreted as the average effect of a dollar spent on the expected number of commercial alliances. For the Poisson model, the parameter estimates can be interpreted as the average percentage effect of a dollar spent on the rate of commercial alliance formation.

\[
\frac{\partial E(\text{Alliance}_{u,t}|X_{u,t})}{\partial \text{NIH}_{u,t-n}} = \beta_n \\
\frac{\partial \text{AllianceRate}_{u,t}|X_{u,t}}{\partial \text{NIH}_{u,t-n}} = \beta_n
\]

\[
(3) \quad \quad (4)
\]

B. Identification Strategy

The quality and characteristics of NIH funded research opportunities are important potential sources of bias for our study’s models. To generate unbiased estimates, our model covariates need to be orthogonal to the unobservable factors that affect university-firm alliance formation. However, the quality and characteristics of the research opportunities funded by the NIH is an unobservable factor that potentially violates this assumption. For example, it may be the case that the NIH preferentially funds “high quality” research opportunities. If higher quality research opportunities lead to more commercial alliances, the estimates of our models will be biased upwards. Similarly, initiatives pursued by universities to increase alliance formation could have occurred simultaneously with increases in federal fundraising activity, causing us to erroneously conclude that federal funding caused growth in alliance formation. On the other hand, if the NIH preferentially award research grants to abstract research projects with limited commercial applications, we might expect a downward bias in our model estimates.

We used an instrumental variable approach to control for bias from unobserved factors that vary over time and across universities. The primary instrument we used was based on prior work by Blume-Kohout et al (2009). [15] The instrument consisted of university-year predicted NIH funding levels that were based on information about each university’s institutional specialization and growth in the NIH’s individual NIC budgets. The predicted NIH funding level for a university in a given year was calculated by taking the NIH funding received by a university in a base year, in our case 1995, and projecting it forward using a weighted growth rate. The weighted growth rate takes the annual growth rate for individual NIC budgets and weights them according to the proportion of the university’s NIH funding that came from that NIC during an earlier reference period. For our study, the reference period was the years 1992 to 1994. The formula for predicted NIH funding is presented in Equation 5.
To check the robustness of our study results, we employed a second instrument inspired by the work of Payne (2003) and Hegde (2008). [16] [17] In both studies, the authors explore the relationship between congressional subcommittee membership and the allocation of federal research funding. The authors found evidence that universities located in districts or states that had more representation on certain congressional subcommittees experienced higher levels of federal research funding. In particular, Hegde (2008) found that universities in states with an additional representative on the Labor, Health and Human Services, and Education and Related Agencies (LHHE) subcommittee in the House of Representatives experienced a 5.9 to 10.3 percent increase in federal research funding. [17] This sizeable effect suggests that state level representation on the LHHE subcommittee may potentially serve as an alternative instrument for NIH funding. We implemented this instrument with the help of historical membership records maintained by the House of Representatives.

In order for our instrumental variable approach to work, the instruments needed to satisfy two conditions. The first is the relevancy condition in which the instruments sufficiently predict our endogenous covariate, i.e. NIH research grant funding. This condition was tested by regressing NIH funding on the instrumental variables and evaluating the models’ F-statistic. The results are presented in Table 1 in the Appendix. For both instruments across all lags, the smallest F-statistic was 39.39, which is higher than the critical values proposed by Stock and Yogo (2002) to test for weak instruments when there are two instruments for a single endogenous variable. [18] These findings are consistent with the results presented by Blume-Kohout et al (2009) and Hegde (2008).

The second condition the instruments needed to satisfy is the exclusion restriction that the instruments are uncorrelated with the error terms in our models, i.e. the quality of university ideas that form the basis of firm alliances. However, the second condition cannot be tested empirically. Instead, we rely on intuitive arguments presented in previous studies. For predicted NIH funding, it is important to recognize that federal budgets determined for NICs are largely a function of the political process involving the President, Congress, and other political stakeholders. So while the quality of ideas at a university for a given year may increase the allocation of funding from an NIC, the quality of ideas are unlikely to be correlated with the aggregate NIC budgets. [15] For our congressional representation instrument, we rely on the fact that there is no clear relationship between the quality of university
research ideas and the degree to which a university’s congressional district and/or state is represented on congressional appropriations subcommittees. Any connection would suggest that universities with higher quality research ideas had some influence on congressional assignments or vice versa, which seems implausible.

C. Estimation

The parameters for the instrumented models were estimated using a two staged least squares procedure. In the first stage of the model, the NIH funding and lags were regressed on the instrumental variables of the corresponding period. The models used for the first stage of the procedure are presented in Equation 6 and 7. In the second stage, the number of university-firm alliances was regressed on the predicted values for NIH using the results from the first stage regression.

\[
NIH_{u,t-n} = \gamma_0 + \gamma_1 NIH_{u,t-n} + v + \tau + \delta_{u,t}, \quad \forall n \in [0, 5]
\]

(6)

\[
NIH_{u,t-n} = \gamma_0 + \gamma_1 NIH_{u,t-n} + \gamma_2 Rep_{u,t-n} + v + \tau + \delta_{u,t}, \quad \forall n \in [0, 5]
\]

(7)

Because the models involved multiple endogenous variables that corresponded to different lagged instruments, the standard errors could not be easily estimated using existing closed-form expressions. As a substitute, we bootstrapped our estimates to generate a first approximation of the standard errors for our parameters.

Consistency of the bootstrap estimates depends on two factors. First, our sample size needs to be sufficiently large. Our sample contains 134 university observations which is larger than the sample size threshold of 30 to 50 accepted by most researchers. [19] Second, our bootstrapped estimates need to use enough re-samples of the empirical population of data. For our study, we used 2,000 replications which is twice the number of replications commonly considered acceptable. [20]

V. Results

The main results for this study are presented in Table 2. Models (1) and (2) denote the non-instrumented linear and Poisson fixed effects models that include covariates for NIH research grant funding in the concurrent year and five-year lags. Models (3) and (4) present the same linear and Poisson models estimated using predicted NIH funding as an instrument and the 2SLS approach. In both sets of non-instrumented and instrumented models, there is general agreement between the sign and significance of parameter estimates produced by the linear and Poisson models for alliance formation.
The findings of the non-instrumented model suggest that the receipt of NIH funding modestly reduces the number and rate of commercial alliance formed over a five-year period. The linear model predicts that the receipt of $100 million in additional NIH research grant funding reduces the number of commercial alliances formed by 1.16 alliances (p<0.001). The Poisson model predicts that $100 million in additional NIH research grant funding decreases the rate of alliance formation by 1.16 percent (p<0.01). However, as previously noted, these estimates are likely to be biased due to the unobserved effects of the quality and characteristics of the ideas that serve as the basis for research grants and university-firm alliances.

After introducing predicted NIH funding as an instrument, we find that NIH funding has a positive effect on the number and rate of commercial alliance formed over a five-year period. Our estimates from the instrumented linear model suggest that a $100 million increase in NIH research grant funding increases the number of alliances formed at the university by 2.68 alliances over five years (p<0.10). The results of the instrumented Poisson model suggest that the same increase in NIH research grant funding increases the rate of alliance formation by 2.38 percent over five years (p<0.10).\(^5\)

For the most part, the results of our main model are robust to alternative alliance assignment procedures, as well as the inclusion of our congressional representation instrument. See Tables 3 and 4. Models (5) and (6) apply the same specification with the outcome variable defined by the most conservative alliance attribution procedure (the attribution rule excluded alliances from a university’s research institutions, primary teaching hospitals, or research organizations), while Models (7) and (8) correspond to the least conservative procedures (the attribution rule included all potential university affiliates, including non-primary teaching hospitals). The dual-instrumented specification is presented in Models (9) and (10). In all three alternative linear models, the estimates are similar to our main results in sign and significance, albeit the magnitude of the effect is diminished slightly. For the Poisson models, we also observe effects of the same sign across all three alternative models. However, our estimates again decrease in magnitude and we observe a loss in significance for the Poisson model using the less conservative alliance attributions, as well as in our dual instrument specification. The loss of significance, particularly for the less conservative alliance model, was in some ways expected as it potentially included alliances that were not connected to NIH funded research.

\(^5\) Marginal effects for the Poisson regression are presented as percentages because the link function for the Poisson model involves the exponentiation of the linear model.
Our results are, however, sensitive to the lag structure we assume for NIH research grant funding. As part of our robustness check, we generated estimates for two sets of alternative specification where we removed the lag for NIH funding that had relatively high p-values for both the linear and Poisson models. These results are presented in Table 4 (Models 11-14). In our models with four and five year lagged funding only, we observed that the magnitude and the significance of the effects differed from our main model. In fact, the results of these models suggest that increased NIH funding decreases the number of alliances formed over a five-year period. However, the estimates for the Poisson model with funding lags for years three through five were consistent with our main findings. Moreover, across all four models, our estimates for the coefficient for NIH funding when lagged by five years remained relatively stable.

VI. Discussion

The primary mission of the NIH is twofold: (1) to seek fundamental knowledge about the nature and behavior of living systems, and (2) to seek the application of that knowledge to enhance health, lengthen life, and reduce illness and disability.\(^6\) Consequently, to evaluate the effectiveness of the NIH’s extramural research grant program one needs to examine two features. First, do the programs increase the production of knowledge? Secondly, to what degree does this knowledge contribute to improvements in overall health? Our study has examined the NIH’s research programs in the context of the latter question by focusing on a critical stage in the creation of new healthcare innovations: the formation of university-firm alliances in the biopharmaceutical industry.

Our results suggest that NIH research grant funding promotes the formation of commercial alliances at individual universities. Assuming that our main study models reflect the true lag structure and our instruments are valid, our estimates can be interpreted as the local average treatment effects from increases in NIH funding due to long-standing historical institutional specialization. While the magnitude of the effects appears small, it is important to keep in mind that a single alliance can represent a multi-million dollar agreement for a university. More importantly, each alliance can lead to a new treatment for a disease and ultimately improve the lives of many individuals.

In some of the estimates we produced as part of our robustness checks, we did observe that the receipt of NIH funding reduced the expected total number of commercial alliances formed over a five-year period. While these results are not the focus of our study, it is worthwhile to discuss their implications. If

\(^6\) See NIH website: [http://www.nih.gov/about/mission.htm](http://www.nih.gov/about/mission.htm)
we consistently observed a negative relationship between NIH research grant funding and university-firm alliance formation, a simple model of commercial alliance formation might suggest that this is evidence that NIH research grant funding actually decreases the amount of total research conducted at universities and thus the number of commercial opportunities developed. However, based on the past literature concerning the effects of NIH research funding, this scenario seems unlikely. A more likely explanation is that NIH funding allows a university to focus more on research rather than alliance formation. The increase in the levels of research allowed by added NIH funding reduces the amount of time researchers spend searching for commercial alliance partners. So while overall research production may increase, the level of alliance formation may decrease.

In the context of ongoing policy debates about NIH budgets, our study and its results should be considered with several caveats. The research supported by the NIH can advance the life and health sciences in a number of ways and promote important scientific discoveries that may not be immediately commercialized. Our study examines only one of many possible pathways that NIH-sponsored research might produce economic and social returns. To the extent that NIH-funded research increases the production of knowledge on the margins, this knowledge may improve health and reduce illness by improving healthcare delivery systems, facilitating the creation of new start-ups, and inducing existing firms to explore new avenues of research, independent of universities. Indeed, past research has found that scientists engage in a wide variety of informal university technology transfer activities. [21] It is also worth noting that the knowledge produced by NIH-sponsored research may accrue to individuals and firms outside the U.S. All of these factors suggest that the results of our study alone offer a lower bound for the total social and economic effects of NIH research grant funding.

There are several important limitations in the data and models employed by our study that may be addressed in future research. The alliance data used in our study lacks information to identify the financial value of the commercial alliances and the relationship between commercial alliances. The financial value of an alliance would provide information to weight the importance of new commercial alliances and allow for an evaluation of the economic impact of NIH funding. Understanding the relationship between alliances would allow our study to disentangle whether multiple alliances observed in a given year are part of a single commercialization effort or represent attempts to commercialize multiple ideas developed at a university. Lastly, the bootstrapping methods used in combination with 2SLS models may not provide efficient standard error estimates. Future studies might employ GMM count models for panel data that allow for fixed effects and instrumental variables to
obtain more efficient results. However, these models may need to consider a slightly different system of equations to allow for estimation.

VII. Conclusion

The use of NIH funding to support research and development is grounded in economic theory. However, empirically, evaluating the effects of the NIH’s research grant funding program presents several challenges. Our study has shed light on one aspect of the economic impact of the NIH’s research funding by evaluating its effects on university-firm alliances. The results we have presented suggest that NIH research funding promotes the formation of university-firm commercial alliances, and by extension, the development of new health innovations. However, more research is needed to draw stronger conclusions about the magnitude of the effect. Future studies evaluating the effect of NIH funding on alternative commercialization pathways may help to complete our perspective of the economic and social impact of the NIH’s research funding programs.
References


14. Reuters, T. *Recap Factsheet*. 2013; Available from:
Table 1: Description and Summary Statistics for Panel Data of University-Firm Alliances and NIH Research Grant Funding

<table>
<thead>
<tr>
<th>Panel Data Description</th>
<th>Study Period</th>
<th>Number of University-Year Observations</th>
<th>Number of Universities</th>
<th>Number of Alliances</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study Period</td>
<td>2000-2011</td>
<td>1608</td>
<td>134</td>
<td>882</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Summary Statistics</th>
<th>Median</th>
<th>IQR</th>
</tr>
</thead>
<tbody>
<tr>
<td>NIH Research Grant Funding ($ Million)</td>
<td>34.9</td>
<td>90.3</td>
</tr>
<tr>
<td>National Cancer Institute</td>
<td>2.9</td>
<td>12.1</td>
</tr>
<tr>
<td>National Institute of Allergy and Infectious Diseases</td>
<td>3.4</td>
<td>11.1</td>
</tr>
<tr>
<td>National Heart, Lung and Blood Institute</td>
<td>2.4</td>
<td>11.9</td>
</tr>
<tr>
<td>National Institute of Neurological Disorders and Stroke</td>
<td>2.0</td>
<td>7.3</td>
</tr>
<tr>
<td>NIH Research Grant Awards (N)</td>
<td>96.0</td>
<td>230.0</td>
</tr>
<tr>
<td>Mean</td>
<td>0.5</td>
<td>1.0</td>
</tr>
<tr>
<td>SD</td>
<td>0.2</td>
<td>0.5</td>
</tr>
<tr>
<td>Cancer Related Research</td>
<td>0.1</td>
<td>0.3</td>
</tr>
<tr>
<td>Infectious Disease Research</td>
<td>0.0</td>
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</tr>
<tr>
<td>Cardiovascular Related Research</td>
<td>0.1</td>
<td>0.3</td>
</tr>
<tr>
<td>Central Nervous System Related Research</td>
<td>0.0</td>
<td>0.2</td>
</tr>
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</table>
Table 2: Effects of NIH Research Grant Funding on University-Firm Alliance Formation Counts and Rates, Non-instrumented and Single Instrumented Models

<table>
<thead>
<tr>
<th>NIH Research Grant Funding</th>
<th>No Instrument Linear (1)</th>
<th>Poisson (2)</th>
<th>Single Instrument Linear (3)</th>
<th>Poisson (4)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lag = 1</td>
<td>0.416</td>
<td>0.319</td>
<td>-0.600</td>
<td>0.701</td>
</tr>
<tr>
<td></td>
<td>(0.203)</td>
<td>(0.394)</td>
<td>(0.785)</td>
<td>(0.754)</td>
</tr>
<tr>
<td>Lag = 2</td>
<td>0.767*</td>
<td>0.759</td>
<td>2.799</td>
<td>0.159</td>
</tr>
<tr>
<td></td>
<td>(0.060)</td>
<td>(0.114)</td>
<td>(0.294)</td>
<td>(0.958)</td>
</tr>
<tr>
<td>Lag = 3</td>
<td>-1.157***</td>
<td>-1.158**</td>
<td>-2.327</td>
<td>-0.407</td>
</tr>
<tr>
<td></td>
<td>(0.005)</td>
<td>(0.025)</td>
<td>(0.304)</td>
<td>(0.860)</td>
</tr>
<tr>
<td>Lag = 4</td>
<td>0.321</td>
<td>0.489</td>
<td>-2.612</td>
<td>-2.725</td>
</tr>
<tr>
<td></td>
<td>(0.440)</td>
<td>(0.318)</td>
<td>(0.375)</td>
<td>(0.330)</td>
</tr>
<tr>
<td>Lag = 5</td>
<td>-0.000548</td>
<td>-0.0913</td>
<td>2.678*</td>
<td>2.379*</td>
</tr>
<tr>
<td></td>
<td>(0.998)</td>
<td>(0.773)</td>
<td>(0.083)</td>
<td>(0.082)</td>
</tr>
</tbody>
</table>

Observations: 1608 1608 1608 1608

p-values in parentheses: * p<.1 ** p<.05 *** p<.01
Table 3: Effects of NIH Research Grant Funding on University-Firm Alliance Formation Counts and Rates, Alternative Alliance Attribution

<table>
<thead>
<tr>
<th>NIH Research Grant Funding</th>
<th>Main Campus Only</th>
<th>All Affiliates</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Linear (5)</td>
<td>Linear (7)</td>
</tr>
<tr>
<td></td>
<td>Poisson (6)</td>
<td>Poisson (8)</td>
</tr>
<tr>
<td>Lag = 1</td>
<td>-0.145 (0.942)</td>
<td>-0.399 (0.871)</td>
</tr>
<tr>
<td></td>
<td>0.845 (0.693)</td>
<td>1.152 (0.617)</td>
</tr>
<tr>
<td>Lag = 2</td>
<td>2.665 (0.276)</td>
<td>2.402 (0.444)</td>
</tr>
<tr>
<td></td>
<td>0.611 (0.829)</td>
<td>-0.884 (0.779)</td>
</tr>
<tr>
<td>Lag = 3</td>
<td>-2.890 (0.111)</td>
<td>-2.316 (0.309)</td>
</tr>
<tr>
<td></td>
<td>-1.321 (0.543)</td>
<td>0.174 (0.938)</td>
</tr>
<tr>
<td>Lag = 4</td>
<td>-1.790 (0.474)</td>
<td>-2.149 (0.432)</td>
</tr>
<tr>
<td></td>
<td>-2.270 (0.395)</td>
<td>-2.274 (0.400)</td>
</tr>
<tr>
<td>Lag = 5</td>
<td>2.289* (0.089)</td>
<td>2.449* (0.076)</td>
</tr>
<tr>
<td></td>
<td>2.324* (0.067)</td>
<td>2.014 (0.112)</td>
</tr>
</tbody>
</table>

Observations
1536 1536 1620 1620

p-values in parentheses
* p<.1  ** p<.05  *** p<.01
Table 4: Effects of NIH Research Grant Funding on University-Firm Alliance Formation Counts and Rates, Dual Instrumented Models, Single Instrument and Alternative Lag Structures

<table>
<thead>
<tr>
<th>NIH Research Grant Funding</th>
<th>Dual Instrument Linear (9)</th>
<th>Poisson (10)</th>
<th>Lag 3-5, Only Linear (11)</th>
<th>Poisson (12)</th>
<th>Lag 4-5, Only Linear (13)</th>
<th>Poisson (14)</th>
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<tbody>
<tr>
<td>Lag = 1</td>
<td>-0.621</td>
<td>0.569</td>
<td></td>
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<tr>
<td></td>
<td>(0.751)</td>
<td>(0.796)</td>
<td></td>
<td></td>
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<tr>
<td>Lag = 2</td>
<td>2.810</td>
<td>0.327</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td></td>
<td>(0.229)</td>
<td>(0.908)</td>
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<td>Lag = 3</td>
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<td>-0.577</td>
<td>0.635</td>
<td>0.937</td>
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<tr>
<td></td>
<td>(0.200)</td>
<td>(0.780)</td>
<td>(0.613)</td>
<td>(0.516)</td>
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</tr>
<tr>
<td>Lag = 4</td>
<td>-2.185</td>
<td>-2.461</td>
<td>-3.179</td>
<td>-3.497</td>
<td>-2.130*</td>
<td>-1.942**</td>
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<tr>
<td></td>
<td>(0.396)</td>
<td>(0.361)</td>
<td>(0.212)</td>
<td>(0.178)</td>
<td>(0.061)</td>
<td>(0.039)</td>
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<tr>
<td>Lag = 5</td>
<td>2.431*</td>
<td>2.219</td>
<td>2.316</td>
<td>2.411*</td>
<td>1.838</td>
<td>1.705*</td>
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<tr>
<td></td>
<td>(0.086)</td>
<td>(0.108)</td>
<td>(0.152)</td>
<td>(0.096)</td>
<td>(0.102)</td>
<td>(0.067)</td>
</tr>
<tr>
<td>Observations</td>
<td>1608</td>
<td>1608</td>
<td>1608</td>
<td>1608</td>
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<td>1608</td>
</tr>
<tr>
<td>p-values in parentheses</td>
<td>* p&lt;.1</td>
<td>** p&lt;.05</td>
<td>*** p&lt;.01</td>
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</table>
Figure 1: Total Annual NIH Research Grant Funding and Commercial Alliances between the Years 2000 and 2011
Figure 2: Correlation between University NIH Research Grant Funding Received and Commercial Alliances Formed between 2000 and 2011

\[ \rho = 0.644 \]
Appendix A

Table 1: First Stage Regression of NIH Funding Variables on Corresponding Lagged Predicted NIH Funding and Congressional Representation Instruments

<table>
<thead>
<tr>
<th></th>
<th>NIH Funding, Lag 1</th>
<th>NIH Funding, Lag 2</th>
<th>NIH Funding, Lag 3</th>
<th>NIH Funding, Lag 4</th>
<th>NIH Funding, Lag 5</th>
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<tbody>
<tr>
<td><strong>Predicted NIH Funding</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
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</tr>
<tr>
<td>Lag 1</td>
<td>0.880***</td>
<td>0.877***</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>(0.000)</td>
<td>(0.000)</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Lag 2</td>
<td></td>
<td>0.865***</td>
<td>0.863***</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>(0.000)</td>
<td>(0.000)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lag 3</td>
<td></td>
<td></td>
<td>0.863***</td>
<td>0.861***</td>
<td></td>
</tr>
<tr>
<td></td>
<td>(0.000)</td>
<td>(0.000)</td>
<td></td>
<td>(0.000)</td>
<td></td>
</tr>
<tr>
<td>Lag 4</td>
<td></td>
<td></td>
<td></td>
<td>0.861***</td>
<td>0.859***</td>
</tr>
<tr>
<td></td>
<td>(0.000)</td>
<td>(0.000)</td>
<td></td>
<td>(0.000)</td>
<td></td>
</tr>
<tr>
<td>Lag 5</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>0.864***</td>
</tr>
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<td>0.862***</td>
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<td>(0.000)</td>
<td>(0.000)</td>
<td></td>
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<td>(0.000)</td>
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<tr>
<td><strong>Congressional Representation</strong></td>
<td></td>
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<tr>
<td>Lag 1</td>
<td>0.00972</td>
<td></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td></td>
<td>(0.370)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lag 2</td>
<td></td>
<td>0.00798</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td></td>
<td>(0.549)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lag 3</td>
<td></td>
<td></td>
<td>0.00720</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>(0.651)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lag 4</td>
<td></td>
<td></td>
<td></td>
<td>0.0135</td>
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</tr>
<tr>
<td></td>
<td>(0.553)</td>
<td></td>
<td></td>
<td>(0.553)</td>
<td></td>
</tr>
<tr>
<td>Lag 5</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>0.0123</td>
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<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>(0.601)</td>
</tr>
<tr>
<td><strong>F-Statistic</strong></td>
<td>44.90</td>
<td>42.20</td>
<td>44.23</td>
<td>41.49</td>
<td>43.81</td>
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<td></td>
<td>41.88</td>
<td>42.43</td>
<td>40.99</td>
<td>41.03</td>
<td>39.39</td>
</tr>
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</table>

P-values in parentheses: * p<.1 ** p<.05 *** p<.01"
Appendix B

Figure 1: Total Annual NIH Research Grant Funding and Commercial Alliances between the Years 1992 and 2011, Study Sample
Appendix C

Figure 2: Total Annual NCI Research Grant Funding and Cancer Related Commercial Alliances between the Years 1992 and 2011, Study Sample

[Bar chart showing annual NCI funding and cancer alliances from 1992 to 2011.]

Legend:
- NCI Funding
- Cancer Alliance
Appendix D

Figure 3: Total Annual NIAIDS Research Grant Funding and Infectious Disease Related Commercial Alliances between the Years 1992 and 2011, Study Sample
Appendix E

Figure 4: Total Annual NHLBI Research Grant Funding and Infectious Disease Related Commercial Alliances between the Years 1992 and 2011, Study Sample
Appendix F

Figure 5: Total Annual NINDS Research Grant Funding and Central Nervous System Related Commercial Alliances between the Years 1992 and 2011, Study Sample
Medical Technology Utilization Management under the Medicare Shared Savings Program

Christopher Lau

ABSTRACT

Recent research has shown that medical technology is regularly used either wastefully or inefficiently in the U.S. healthcare system. These trends have been attributed to a number of factors including the absence of adequate provider incentives. The recent formation of Accountable Care Organizations (ACOs) and associated risk sharing payment arrangements created in the Medicare Shared Savings (MSS) Program ostensibly address this problem by establishing new financial incentives that reward efficient care delivery. However, it remains unclear whether these new financial incentives will induce more efficient patterns of medical technology use. To address this gap in knowledge, this study seeks to characterize the medical utilization management strategies employed by ACOs participating in the MSS Program in California. Using a set of semi-structured interviews, this study finds there is some evidence that the MSS Program has spurred changes that align with policymakers’ goals. Some ACOs are responding to the MSS Program by implementing tools and programs to encourage providers to improve care and reduce costs. However, it is still too early to know whether these efforts will yield the cost savings or care improvements that were originally envisioned by policymakers.
I. Introduction

A. Medical Technology Utilization and Healthcare Spending

Medical technology comprises the products and services used to deliver medical care to patients. It not only encompasses the therapeutic drugs and devices that patients consume, but also the diagnostic capabilities and range of healthcare delivery models used to address medical needs. Over the past decade, the number and diversity of medical technologies has grown considerably. These advances have helped physicians tackle what were once considered to be incurable diseases—improving the quality of life for countless individuals.

While the benefits of medical technology are undeniably desirable, technology’s impact on healthcare spending growth is a growing concern. Researchers have long sought to uncover the underlying causes of healthcare spending growth. Newhouse (1992) was among the first to conclude that much of the rise in healthcare spending is likely due to medical technology. 1,2 Since his study, many have tried to quantify the degree to which technology has contributed to spending growth. Recent estimates have attributed as much as half of the growth in U.S. healthcare spending in past decades to the emergence of new medical technologies. 3 These and similar findings have given cause for policymakers and researchers to question the value of medical technology used in the U.S., particularly as the share of the U.S. economy captured by healthcare spending continues to exceed historic highs. 4

The impact of medical technology on healthcare spending is not necessarily problematic provided that patients receive benefits commensurate with the cost of the medical goods and services they receive. However, studies looking at patterns of medical technology adoption and use in the U.S. have found considerable evidence that this condition has not been met. Research has shown that medical goods and services are often used in a manner that is clinically inappropriate or of questionable value. 5,6 Moreover, studies have found that, relative to other countries, the U.S. uses expensive medical technologies more extensively and is more likely to adopt medical technologies with marginal or limited benefits. 7,9 When taken together, these findings suggest that changing existing patterns of use and adoption for cost-increasing medical technologies will be a necessary step to control long-term healthcare spending growth. 10

Identifying the root causes of inefficient medical technology utilization and developing policy solutions has been an active area of research. Several explanations have been posited for the persistence of
wasteful utilization patterns, including local practice norms and a lack of comparative effectiveness information. Although there are likely many contributing factors, perhaps the most widely cited explanation is the lack of appropriate financial incentives for providers to use technology more efficiently. Historically, many commercial and public payers have reimbursed for provider services on a fee-for-service basis. These payment systems tend to encourage resource-intensive care because providers are not exposed to the financial downside associated with more expensive care practices. The lack of exposure to financial risk also provides few incentives for providers to adopt technologies that would dramatically reduce healthcare costs.

B. The Medicare Shared Savings (MSS) Program and Accountable Care Organizations (ACOs)

Recognizing the misalignment in incentives in public payer programs, policymakers have made several changes to the Medicare program in the 2010 Patient Protection and Affordable Care Act (ACA). Medicare is the largest public insurance program, covering approximately 16 percent of the U.S. population. As part of ACA, the Centers for Medicare and Medicaid Services (CMS), the agency that oversees the Medicare program, was authorized to test out new payment systems that reward providers for delivering high value care to Medicare beneficiaries. For example, under the payment systems tested in the Bundled Payment for Care Improvement initiative, hospitals are rewarded for delivering lower cost care over an individual’s entire care episode, which may extend past an individual’s hospital stay. For physicians, efforts to encourage more efficient medical technology use are embodied in the MSS Program.

The MSS Program seeks to leverage a new healthcare delivery concept, the ACO. ACOs in the broadest sense are groups of providers that jointly agree to be financially accountable for the quality and cost of care delivered to a population of patients. The groups of providers are most often made up of primary care physicians, but may also include physician specialists, hospitals, and other healthcare providers. Through the contracts they form with payers, these providers assume some financial risk that is based on the cost of care delivered to their patients. The amount of risk born by the ACO varies, but all contracts offer the ACO financial incentives for delivering less costly care—provided they are able to meet certain quality standards.

The MSS Program offers ACOs the option to enter into contracts where they are financially accountable for the care delivered to traditional Medicare Fee-For-Service (FFS) beneficiaries (i.e. Medicare beneficiaries not enrolled in a Medicare Advantage plan). For the most part, the MSS Program is open to
any group of physicians that form an ACO and collectively provide primary care services to at least 5,000 Medicare FFS beneficiaries. Once approved for the program, the ACO is assigned a unique population of Medicare FFS beneficiaries by CMS. This population is intended to represent the Medicare FFS beneficiaries that receive the majority of their primary care services from the ACO and constitutes the individuals for whom the ACO will be financially accountable.

One of the key features of and motivations for ACOs to join the MSS Program is that ACOs who participate become eligible for a bonus payment. This payment depends on each ACO’s ability to reduce spending below a CMS determined benchmark. This benchmark is set according to the spending patterns of the ACO’s population of Medicare FFS patients and national growth in spending for the Medicare FFS beneficiaries. If, after a year, the realized spending growth for the ACO’s population of patients is lower than the benchmark by more than a minimum savings rate (between 2 to 3.9 percent), the ACO becomes eligible to receive a bonus payment. Depending on the “track” selected by the ACO, ACO size, and ability to satisfy certain quality benchmarks, ACOs can receive a bonus payment equal to 50 to 60 percent of the amount by which they have reduced spending above the minimum savings rate.17 In some cases, the ACO may also be liable for financial penalties if the costs of care for the patients they treat exceed their predetermined spending benchmark. Together, the new financial incentives are designed to reward the ACO for delivering more efficient (lower cost, higher quality) care.

C. Impact of the MSS Program on Medical Technology Utilization

Currently, it is not clear whether the MSS Program will promote more efficient use of medical technology. Commercial ACO programs like Blue Cross Blue Shield of Massachusetts’ Alternative Quality Contracts have produced promising results.18 Similarly, early reports from the MSS Program indicate that roughly a quarter of the first ACOs admitted to the program have qualified for shared savings after their first year of operation, giving additional reason for optimism.19 However, past experiences suggest that the program’s new financial incentives may not be enough to change physician behavior.20 For instance, the CMS’ Physician Group Practice Demonstration, the precursor to the MSS Program, generated mixed evidence about the ability of organizations similar to ACOs to reduce healthcare spending.21

Another reason for skepticism is that ACOs have limited tools to encourage patients to select more cost-effective care options. Unlike managed care programs where patient care is often directed by a single primary care physician, Medicare FFS beneficiaries retain the right to choose which providers they see
and possess a higher degree of freedom when choosing what services they receive. In fact, the MSS Program explicitly prohibits ACOs from requiring its physicians to exclusively refer patients to providers within the ACO.\textsuperscript{17} ACOs are also barred from implementing any rules that would potentially limit physician discretion when determining care for Medicare FFS patients. Despite these restrictions, ACOs remain accountable for the total spending of their assigned beneficiaries, including spending on care delivered by providers outside the ACO.

There are also concerns that the MSS Program may simply select for and reward more efficient provider groups without inducing real changes in physician behavior. Participation in the MSS Program is voluntary. Moreover, the program sets part of its shared-savings benchmarks using national spending growth rates. This feature allows provider groups in areas with historically low-cost spending growth rates to potentially benefit financially from the program without changing their behavior, and allows physician groups that are less efficient to avoid the program altogether. It is conceivable that the program only attracts providers that already deliver care efficiently. If this scenario were realized, the program would reward physician groups for “savings” that would have been generated absent the MSS program since participating providers would be limited to those that naturally exhibit lower than average cost growth for their Medicare patients. In turn, the Medicare program would in fact lose money and fail to induce the changes in behavior originally envisioned by policymakers.

One way to examine the effectiveness of the MSS program is to examine whether ACOs participating in the program are, in fact, developing strategies to encourage more efficient use of medical technology. To date, little data has been collected on how ACOs are responding to the MSS Program. One reason for the paucity of data is that many ACOs are relatively new—most having been established within the past two years. A handful of case studies have documented the experiences and challenges of early ACOs that formed outside the MSS Program.\textsuperscript{22,23} A notable example is the study by Larson et al. (2012) which recounts the early experience of four ACOs that participated in the Brookings-Dartmouth ACO Pilot program.\textsuperscript{24} The authors highlight, among other things, that there is considerable variation in the structure and background among ACOs. They also noted that each organization identified three common elements that have contributed to their early success: strong executive leadership, strong payer-provider relationships, and prior experience with performance based payment. What remains unclear, however, is whether these same experiences will manifest themselves among the wider population of ACOs. ACOs participating in the MSS Program may face a different set of challenges and constraints in their attempts to use medical technology more efficiently.
To address this gap in knowledge, this study seeks to characterize the medical utilization management strategies employed by ACOs participating in the MSS Program in California. Using a set of semi-structured interviews, data was collected to (1) determine what, if any, tools and programs are currently being employed by ACOs to encourage their physicians to use medical technology and resources more efficiently (2) whether these tools were developed in conjunction with ACO formation, and (3) identify the factors and challenges that have shaped the approaches these ACOs have taken.

II. Data and Methods

A. Respondent Sample

The sample frame of organizations for this study was the population of ACOs that were operating in California and actively participating in the MSS Program as of June 2013. The study sample frame included ACOs that were participating in the Advance Payment Model program.

Within each organization, this study solicited participation from an individual or groups of individuals with knowledge of care and utilization management strategies employed by their respective organizations. Individuals were initially identified by consulting the primary contacts listed by CMS for ACOs in MSS Program announcements. Contact information was cross-referenced with contact information produced by the California Healthcare Foundation and Cattaneo and Stroud.25 Respondents that participated in this study were medical directors, lead administrators, or chief executive officers of their respective organizations.

B. Instrument Design

Data was collected using semi-structured interviews. The main questions presented during each interview were used to identify the tools and programs employed by each ACO and adapted from a survey developed by Kerr et al. (1995).26 The questions focused on six potential utilization management tools employed by ACOs. The tools respondents were asked to discuss included: (1) prior authorization protocols for physician referrals or diagnostic examinations, (2) primary care physician utilization profiles including any reports that detail a physician’s use of various medical technologies, (3) written practice guidelines, (4) educational materials including orientation programs, seminars, or retreats, (5) decision support tools that address the use of diagnostic tests, physician referrals, uses of generic vs. branded drugs, and preventative and screening services, and (6) policies that governed the use of medical supplies. Figure 1 illustrates how these tools can influence medical technology utilization.
Note: The rectangle in green identifies all the potential uses for a medical technology. The bold rectangle delineates the applications that are covered based on local and national coverage determinations made by CMS. The dotted rectangle defines the applications of technology that are promoted by the ACO. Both CMS Coverage Policies and ACO Utilization Management Strategies aim to encourage the applications of medical technology where they achieve the highest value.

To understand whether the implementation of these tools coincided with the formation of each ACO, the interview guide included an additional set of follow-up questions for each utilization method. In situations where respondents confirmed the use of a utilization management method within their ACO, respondents were subsequently asked whether these (or similar) tools were in place in the 12 months prior to the interview. In most cases, this included the period prior to the ACO’s formation. If the ACO had formed earlier than one year prior, they were also asked whether the policies had been in place before the ACO had formed. In instances where the respondent indicated that a utilization management tool was not currently being employed, respondents were asked whether they had plans to implement such a tool in the coming year.

Lastly, the interview guide included an additional set of questions to examine what factors influenced the utilization management strategies employed by each ACO. To prompt responses, the interview
guide initially focused on four main areas: the ACO’s organizational history, local market factors, health information technology (IT) infrastructure, and access to cost-effectiveness research. These four factors were adapted from the logic model proposed by Fisher et al (2012) for the evaluation of ACOs. The guide also included a separate question to allow respondents to elaborate on other factors that have influenced their development. A copy of the complete interview guide has been included in the Appendix.

C. Data Collection

Responses were collected between July and October of 2013. Prior to each interview, respondents were advised that the interviews would be confidential and that no information identifying the individual or groups of individuals participating in the interview would be presented in the study. Respondents were also advised that their organizations would not be identified in the study without prior approval. Interviews were conducted over the phone and in person depending on the respondent’s preferences and availability.

III. Results

A. Study Population

All 17 ACOs participating in the MSS Program and located in California were contacted and asked to participate in the study. Representatives from 10 ACOs agreed to be interviewed. In general, the characteristics of the organizations that were included in the study were comparable to those of the population of California ACOs participating in the MSS Program. Half of this study’s respondents were partnered with a hospital as part of the MSS Program, which was slightly higher than the proportion of ACOs partnered with hospitals in California. In addition, a higher proportion of ACOs interviewed joined the MSS Program as part of the January 2013 wave. The average number of primary care physicians (PCPs) and average number of Medicare FFS beneficiaries attributed to the ACOs that were interviewed were comparable to the population averages for California’s ACOs participating in the MSS Program. A comparison of the size and date of participation of the ACOs is included in Table 1.
Table 1: Sample Frame and Respondent Characteristics

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<th></th>
<th>California MSS Program ACOs</th>
<th>Study Respondents</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of ACOS (N)</td>
<td>17</td>
<td>10</td>
</tr>
<tr>
<td>Partnered with Hospital</td>
<td>7</td>
<td>5</td>
</tr>
<tr>
<td>Admitted into the MSS Program, 2012</td>
<td>8</td>
<td>4</td>
</tr>
<tr>
<td>Admitted into the MSS Program, 2013</td>
<td>9</td>
<td>6</td>
</tr>
<tr>
<td>ACO PCP count, Mean (SD)</td>
<td>262 (337)</td>
<td>295 (399)</td>
</tr>
<tr>
<td>Beneficiaries Attributed to ACO, Mean (SD)</td>
<td>7,924 (3,839)</td>
<td>8,890 (4,556)</td>
</tr>
</tbody>
</table>

B. Utilization Management Strategies

Interviewees provided responses to the questions outlined in the interview guide in varying degrees of detail. In general, respondents were allowed to answer as they saw fit, but occasionally they were prompted to provide additional detail. Table 2 summarizes the core elements of the responses given when prompted to describe their organization and its use of six different utilization management tools. The first column denotes the number of respondents that indicated they employed the tool or program at the time of the interview. The second column reports the number of respondents that did not have a given tool or program employed at the time of the interview, but planned to employ the tool or program within the 12 months following the interview.

Table 2: Summary of Interview Responses

<table>
<thead>
<tr>
<th>Technology Utilization Management Tool or Program</th>
<th>Employed at Time of Interview</th>
<th>Not Employed, but Plan to Use</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Prior authorization protocols for physician referrals or non-urgent ambulatory tests</td>
<td>0/10</td>
<td>1/10</td>
</tr>
<tr>
<td>2. Utilization profiles of ACO’s primary care physicians</td>
<td>4/10</td>
<td>6/10</td>
</tr>
<tr>
<td>3. Practice guidelines for medical technologies (e.g. diagnostic imaging tests, screening)</td>
<td>6/10</td>
<td>1/10</td>
</tr>
<tr>
<td>4. Education and/or training on cost-effective primary care*</td>
<td>8/9</td>
<td>0/9</td>
</tr>
<tr>
<td>5. Decision support tools to promote cost-effective primary care</td>
<td>6/10</td>
<td>2/10</td>
</tr>
<tr>
<td>6. Policies about the selection of medical devices or the use of branded vs. prescription drugs</td>
<td>1/10</td>
<td>0/10</td>
</tr>
</tbody>
</table>

*One respondent was not able to comment on their organization’s educational programs
1. Prior Authorization for Physician Referrals and Non-urgent Ambulatory Tests

None of the respondents interviewed indicated that they currently have prior authorization procedures that require PCPs to consult the ACO before physician referrals are made or specific procedures are ordered. When asked whether they anticipated implementing prior authorization protocols in the future, almost all respondents (9 of 10) indicated that they had no plans to institute such programs in the next 12 months. Many respondents believed that the current restrictions imposed by the MSS Program prevented their ACO from intervening and directing the type of care received by Medicare FFS beneficiaries. However, more than one respondent indicated that they would consider prior authorization protocols if the current program rules were relaxed.

2. Utilization Profiles

Although less than half (4 of 10) of respondents had distributed utilization profiles for Medicare services to their PCPs at the time of the interview, all respondents that were not using utilization profiles (6 of 10) had plans to do so in the coming year. When asked about the frequency with which utilization profiles were expected to be distributed, responses ranged from twice-a-year to monthly. Among the respondents whose ACOs were actively using utilization profiles for Medicare services, half had an existing system that allowed physicians to access their utilization data or had distributed utilization profiles prior to the ACO’s formation. However, these profiles had been used primarily to guide care for managed care patients.

For the respondents that were planning to distribute utilization profiles, many (5 of 6) indicated that they had yet to receive the necessary data from CMS to allow them to profile their physicians. Some of these respondents also indicated that interpreting and operationalizing the data from Medicare was an ongoing challenge for the ACO.

3. Written Practice Guidelines

More than half of respondents (6 of 10) indicated that their PCPs received or were asked to follow (but not required to follow) some type of written practice guidelines that covered care for Medicare patients. However, based on the responses, it appears that many guidelines were not implemented specifically for the MSS Program. About half of those currently employing guidelines (3 of 6) indicated that the practice guidelines being used were already in place as part of an existing managed care infrastructure.
or would have been implemented irrespective of the MSS Program. Two respondents did indicate that new guidelines were put in place after their ACO was formed.

Among the respondents not currently using practice guidelines, only one indicated that they had plans to do so in the future. Some respondents expressed concern about implementing policies that would result in different treatment for Medicare patients and violate the rules of the MSS Program. The rest of the respondents indicated that they expected to rely more on performance evaluations and the structure of financial incentives as the main mechanisms to guide clinical care.

4. Education and Training

Most respondents (8 of 9) used some form of education or training to encourage physicians to use medical resources more efficiently, although there was considerable variation in the specific tools employed. More than half (5 of 8) of the respondents interviewed indicated that they distributed written materials that summarized new ways to improve quality or establish more cost-effective practices to their ACO’s physicians. Another common approach was to provide regular meetings or seminars to discuss cost-effective practices (7 of 8).

Of those who had some form of education to encourage cost-effective care, less than half indicated that the new education programs had been developed and implemented specifically for the ACO (3 of 8). In most instances, the education programs had already been in place for managed care patients. Some respondents indicated that the programs had been implemented previously as part of a larger system-wide effort to encourage cost-effective care. One respondent indicated that they could not comment on their ACO’s education program.

5. Decision Support

More than half of the respondents indicated that some of their ACOs had physician support tools – tools used to improve decision-making at the point of care - in place (6 of 10). Many of these respondents indicated that they had either written or computer generated reminders about patient follow-up, screening, or preventative care. Half of these respondents indicated that some of these tools had been in place or were put in place independently of the ACO (3 of 6). Half of these respondents also indicated that at least some tools had been put in place as part of forming an ACO.

Among the respondents that did not have any decision support tools in place for the ACO, some respondents explained that some of their individual physicians had decision support tools for their
individual practices (2 of 4). However, these tools were not implemented by the ACO and available to their entire system of physicians. Half of these respondents also indicated that they were actively working to have some type of decision support system put in place in the coming year (2 of 4).

6. Medical Supplies and Vendor Policies

When asked about policies used to guide physician decisions about medical device selection and the prescription of branded vs. generic drugs, only one respondent indicated that they had some type of policy in place for their Medicare FFS beneficiaries. Most respondents indicated that these policies had not been implemented, nor did they have plans to implement similar policies in the future. Some respondents indicated that these initiatives had been considered for other populations of patients, but nothing specific to the ACO had been put in place.

C. Factors Influencing Medical Utilization Strategies

Organizational History

Certain elements in each ACO’s history appeared to impact how they formed their utilization and care management strategies. Several organizations (4 of 10) indicated that their past experiences with managed care or capitation had helped with their transition into an ACO. Their managed care experience both helped with developing their utilization strategy and achieving physician buy-in. Some respondents also indicated that the leadership of their core physicians was helpful in facilitating the formation of their respective ACOs.

One key pattern that emerged from the interviews was how responses varied depending on the origins of the ACO. The ACOs that were interviewed varied in the number and types of the provider organizations that initially formed them. At one end of the spectrum were ACOs that emerged from largely unaffiliated independent physician practices. At the other end of the spectrum were ACOs that formed out of established integrated healthcare systems and hospital-based physician groups with stronger pre-existing relationships. For some questions, responses varied depending on who formed the ACO. For example, among the ACOs that formed out of established healthcare systems, respondents indicated throughout the interview that many of the tools they had in place were implemented as part of prior efforts to improve efficiency. Moreover, when asked about their utilization tools and programs, the respondents indicated that many would have put them in place absent the creation of their ACO and participation in the MSS Program.
The origins of the ACO also seem to influence interviewee responses when asked about the types of challenges they faced. ACOs that formed out of established integrated healthcare systems tended to have highly developed health IT infrastructures and decision support tools. Consequently, and perhaps not surprisingly, these organizations tended to express concerns about larger program design issues associated with the MSS Program, such as the patient attribution process and the ability of the ACO to implement new utilization strategies within the constraints of CMS’s rules. In contrast, ACOs that emerged from independent physician groups appeared to be confronted with a different set of challenges. Access to data and analyzing data tended to be much larger issues for these respondents. Also, these ACOs were more likely to cite education among their physicians and patients as a challenge for their organization.

*Local Market Factors*

Responses from study participants indicated that local market factors did not appear to play a critical role in shaping utilization management strategies. When asked about the role of local market factors, like competition from other providers and the presence of commercial payers, most respondents (7/10) did not perceive these features as having a major impact on their utilization management approach. A small number of respondents (3 of 10) indicated that the existence of commercial payer programs made the ACO more likely to adopt tools and programs that also aligned with commercial value-based contracts. Although competition was not seen as a driving force behind utilization management strategies, some respondents (2 of 10) indicated that competition was a driving force behind forming an ACO.

It is worth noting that the ACOs in California participating in the MSS Program are clustered geographically, servicing primarily urban areas. Most organizations were centralized in three major metropolitan areas—the San Francisco Bay Area, greater Los Angeles area, and the greater San Diego area. One ACO is based in Sacramento.

*Health IT Infrastructure*

The impact of ACO health IT systems on utilization management strategies was not clear; however, many respondents expressed some concern about being able to develop an IT infrastructure for their ACO. More than half of the respondents (6 of 10) indicated that developing or updating their health IT infrastructure for the ACO was a challenge. Most of these respondents indicated that their organization had some form of electronic medical record (EMR) system in place prior to forming an ACO. However,
they also indicated that updating their system or unifying different platforms used by their ACO’s physicians required or would require substantial time and resources.

Access to Cost-effectiveness Research

Almost all ACOs (7 of 8) indicated that they did not feel that access to cost-effectiveness research was an issue. Some ACOs relied more heavily on external resources for developing new utilization strategies, while others looked internally to identify clinical practices that could be applied to the ACO at large. Two ACOs indicated that credibility was an issue when attempting to implement new clinical practices designed to improve efficiency. The respondents indicated that their physicians remained skeptical of new care practices designed to improve the cost-effectiveness of the care they delivered and found that their physicians were more receptive to practices when supported by research conducted by credible third-party organizations (e.g. NICE, Cochran Review, etc.). Within the sample, two respondents were not able to comment on the subject.

Additional Factors Influencing ACO Behavior

When asked about other factors that have influenced their utilization strategy, respondents commonly identified two issues: accessing utilization data for Medicare patients and awareness about the MSS Program. As part of the MSS Program, CMS provides each participating ACO with claims data that captures the services received by the Medicare beneficiaries for which each ACO is accountable. The data include claims for services not delivered by the ACO’s providers. Almost all respondents indicated at some point in the interview that they had some trouble either accessing this data or using the Medicare claims data to understand the utilization patterns of their providers. More than one respondent commented that their impression was that CMS was not providing sufficient guidance and feedback on how to use the data effectively.

Awareness among patients and providers about the MSS Program was also cited as a major challenge. Multiple respondents (3 of 10) indicated that there was a general lack of knowledge among patients and providers about ACOs and what they might imply for patient care. These ACOs indicated that addressing patients’ concerns was often time-consuming. Furthermore, one respondent indicated that they have had to spend considerable resources on education to recruit prospective providers into their ACO—some of whom are pre-occupied with other ongoing healthcare reforms.
Several other issues emerged when respondents were asked to elaborate about their organization’s experience thus far as an ACO. Some ACOs that were interviewed (2 of 10) cited major concerns about the attribution process. CMS assigns the population of Medicare beneficiaries for which an ACO is accountable based on that ACO’s provision of primary care services. If a Medicare beneficiary receives the majority of their primary care services from the providers of an ACO, they are assigned to that ACO. Some respondents indicated that they had a hard time anticipating when patients would be attributed to the ACO. More than one ACO also expressed the view that lack of control over where patients went for care was expected to be a challenge going forward. Moreover, when discussing the issue of utilization management, respondents expressed some reluctance to exercise more control over the care delivered by their providers to Medicare patients. While most respondents were aware of limitations related to physician referrals and procedures, more than one respondent suggested that CMS barred any sort of special treatment of Medicare patients by ACOs.

IV. Discussion

The responses that were collected by this study suggest that ACOs have implemented or are working to implement a diverse combination of tools and programs to improve the care efficiency. Among the different strategies, almost all were using or planning to use Medicare data and utilization profiles to encourage more efficient use of medical resources. For many, access to Medicare data has created a new opportunity to hold providers accountable for care they deliver to Medicare patients. Education and decision support tools were also common feature in the utilization management strategies for our respondents. However, these tools were more likely to have been in place prior to the ACO’s formation and were not necessarily induced by the MSS Program. Nonetheless, their application towards the treatment of Medicare patients suggests ACOs are heading in the right direction. Taken together, the prevalence of new tools and programs to encourage more efficient care delivery are positive signs for the MSS Program.

Perhaps not surprisingly, many of the tools and programs currently employed by the ACOs represented in this study have been shaped by past experiences with risk-sharing payment arrangements. Early studies of ACO formation have found that ACOs have emerged in areas where hospital risk-sharing contracts are prominent. Similarly, case studies have shown that experience with risk-bearing contracts were an important facilitator for ACO formation. One possible explanation for this pattern is that experiences with risk-sharing contracts make it easier for certain physician organizations to adapt
to the MSS Program. This explanation is in part reflected in the responses collected in this study. It is too early to tell whether experience with managed care is a critical factor for the development of effective utilization management strategies. But if there is a causal link, the correlation between managed care experience and ACOs may foreshadow challenges for ACOs in the MSS Program located in areas with low rates of managed care penetration and little experience with risk-sharing payment arrangements.

Several challenges lie ahead for the ACOs in the MSS Program as they attempt to encourage more efficient medical technology use among their providers. One of the most prominent is the ability to access and leverage Medicare data to change practice patterns. As part of the MSS Program, CMS has agreed to provide participating ACOs with access to claims data for the population of Medicare patients for which they are accountable. These data are supposed to allow ACOs to evaluate the performance of their physicians, assess the types of care delivered to its patients, and ultimately develop new strategies to encourage more efficient use of medical technologies. Many respondents indicated that their organization had some trouble obtaining the data from CMS or obtaining guidance on how to interpret these data. While data access issues may be resolved as the MSS Program matures, policymakers should work to address existing problems quickly. One respondent indicated that the inability to access the claims data that CMS was supposed to provide was sufficiently severe that they were considering leaving the MSS Program. Moreover, it is clear that, early on, Medicare data will be used to provide each ACO’s physicians with more information about their performance on utilization and quality measures. However, it is not known whether greater awareness among physicians alone will be enough to change practice patterns. Finding ways to effectively use the newly available Medicare data to change medical technology utilization patterns will be critical to the success of ACOs in the MSS Program.

Another challenge identified by many ACOs that were interviewed was provider and patient awareness about the program. Although the lack of knowledge among physicians and patients about the MSS Program did not directly impact the development of an effective utilization management strategy, respondents indicated that efforts to raise awareness among providers and patients had consumed considerable resources that could have been used elsewhere. The problem was more apparent for groups of independent physicians that coalesced to form ACOs than for ACOs that formed from a more established hospital or provider system. Policymakers looking to help promote the formation of ACOs
may consider providing more information about the MSS Program to providers and patients to increase their familiarity and acceptance of ACOs as a new care model.

In the long-term, a challenge that lies ahead for ACOs will be learning to innovate within the constraints of the MSS Program. Early efficiency gains for ACOs are likely to be achieved by tackling “low hanging fruit”—wasteful practices that are easily identified and corrected. However, these opportunities are finite. At some point ACOs will need to develop new solutions to encourage efficient technology use. While some solutions may be borrowed from managed care experiences, ACOs will likely need to experiment with alternative approaches due to CMS’ prohibition against implementing tools and procedures that may limit physician discretion and/or patient choice in the Medicare FFS program. One way policymakers can help ACOs looking to innovate is to clarify what types of ACO programs and tools are admissible or provide safe harbors for ACOs to try new approaches to changing healthcare delivery. If existing policies are interpreted conservatively, some ACOs may shy away from novel approaches because of the perceived risk of violating the current rules established for Medicare FFS patients. Indeed, during interviews a handful of respondents expressed concerns about their ability to perform given the current attribution process and constraints of the MSS Program.

This study has several important limitations that prevent it from drawing stronger conclusions. First, this study involved a relatively small sample of ACOs, limiting its ability to infer trends to the broader population of ACOs in the MSS Program. Future studies that seek to examine utilization management strategies may consider conducting surveys similar to Kerr et al (1995).26 Second, due to the nature of the interview format, there was considerable variation in the level of detail to which respondents answered interview questions. Consequently, this study was only able to provide summary data on the types of utilization management strategies being employed. In-depth case studies, similar to those generated by Larson et al (2012), may be more appropriate to gather information about what specific programs and tools are being developed by ACOs.24 Third, while the interviews suggest that some ACOs have implemented tools and programs to encourage efficient use of medical technology, we still do not know how effective these strategies will be. The impact of ACOs on medical technology utilization will be known after more data on utilization patterns has become available. However, this study has some insights on what to expect in the coming years. Lastly, this study did not examine the policies and tools of provider groups that have not formed an ACO. It is conceivable that the efforts made by this study’s respondents to encourage more efficient use of medical technologies for Medicare patients are also being made by physicians not enrolled in the MSS Program. Understanding the similarities and
differences in behavior between physician groups that have and have not chosen to enroll in the MSS Program would help complete our understanding of the overall program effects.

V. Conclusion

ACOs and the MSS Program are an important component of CMS’s strategy to promote more efficient healthcare systems and have the potential to change how efficiently medical technology is used. Based on the interviews conducted in this study, there is some evidence that the MSS Program has spurred changes that align with the agency’s goals. Some ACOs are responding to the MSS Program by implementing tools and programs to encourage providers to improve care and reduce costs. Overall, these signs are encouraging as they represent real changes in provider organizations. However, it is still too early to know whether these efforts will yield the cost savings or care improvements that were originally envisioned by policymakers.
References


Appendix A: Interview Guide

Background

In our discussion today, we will be asking a series of questions to learn more about your Accountable Care Organization and its efforts to encourage more efficient care for Medicare beneficiaries. We are particularly interested in identifying any policies, programs or solutions that your organization has instituted, how these features may have changed over the past year and the challenges you may have faced under the Medicare ACO program. The results of the study will be used to identify trends in utilization and care management strategies employed by physician organizations in California and to develop recommendations for policy makers on ways to help ACOs and other physician organizations in their efforts to improve quality and reduce healthcare waste.

Consent Language

The information you provide will be used for research purposes only and will be aggregated into summaries. The discussion will last no more than one hour. Notes will be taken during the discussion and with your permission, an audio recording will be used to supplement the notes taken. No one outside of the study team will be allowed to see the notes or listen to the tapes. We will destroy all information that identifies you or your organization within a year of the end of the study.

Your participation in this study is voluntary. Your nonparticipation will not be reported to anyone. If you do choose to participate, we can stop the discussion at any time for any reason and you should feel free to decline to discuss any topic that we raise. (1) Do you have any questions about the study? (2) Do you agree to participate? (YES/NO) (3) Do you give your permission for us to tape the call? (YES/NO) (4) Would you be willing to have your organization listed in an appendix of the final report as one that we interviewed? (YES/NO)

We have sent you a copy of this explanatory statement, in case you have any questions or concerns about the study or your participation in it. If you have any questions or concerns, please contact Christopher Lau, Principal Investigator, by phone at (310) 393-0411, ext. 7246 or by email at clau@rand.org.
SECTION I: UTILIZATION AND CARE MANAGEMENT QUESTIONS

1. Presently, are there instances under which your ACO’s primary care physicians must obtain preauthorization from the ACO or consult representatives of the ACO before a Medicare patient is referred for non-urgent ambulatory consultations with one of the following types of physician specialists? [If no to all, Skip to question 2a; Otherwise, Skip to question 2b]
   - Cardiologists
   - Dermatologists
   - Ophthalmologists
   - Orthopedist
   - Oncologist

2. 
   a. Are there plans to institute preauthorization/consultation protocols for Medicare patient referrals to physician specialists within the next 12 months? [Skip to question 4]
   b. Were the preauthorization or consultation protocols for Medicare patient referrals different 12 months ago? [If no, Skip to question 4]

3. When compared to the preauthorization/consultation protocols used 12 months ago, do the current preauthorization protocols for Medicare patient referrals apply to the same types of physician specialist? If no, please explain how the protocols differ.

4. Presently, are there instances under which your ACO’s primary care physicians must obtain preauthorization from the ACO or consult representatives of the ACO before one of the following non-urgent ambulatory tests and procedures is ordered for a Medicare patient? [If no to all, Skip to question 5a; Otherwise, Skip to question 5b]
   - Electrocardiogram
   - MRI
   - Echocardiogram
   - Chest x-ray
   - CT Scan
5.  
   a. Are there plans to institute preauthorization/consultation protocols for non-urgent ambulatory tests and procedures for a **Medicare patient** within the next 12 months? [Skip to question 7]  
   b. Were the preauthorization/consultation protocols for non-urgent ambulatory tests and procedures different 12 months ago? [If no, Skip to question 7]  

6. When compared to the preauthorization/consultation protocols used 12 months ago, do the current preauthorization protocols for non-urgent ambulatory tests and procedures cover the same types of tests and procedures? If no, please explain how the protocols differ.

7. Approximately how often do your ACO’s primary care physicians receive a profile of their utilization patterns for **Medicare patients**? For example, a profile might include the number of specialty referrals or certain tests that the physician ordered over a specified period of time. [If never, Skip to question 8a; Otherwise, Skip to question 8b]

8.  
   a. Are there plans to regularly distribute utilization profiles to your ACO’s primary care physicians for care delivered to **Medicare patients** within the next 12 months? [Skip to question 10]  
   b. Were the utilization pattern profiles of your ACO’s primary care physicians used or distributed to primary care physicians differently 12 months ago? [If no, Skip to question 10]

9. When compared to the utilization management system in place 12 months ago, are utilization pattern profiles distributed to primary care physicians with the same, lesser, or greater frequency?

10. Are your ACO’s primary care physicians asked to follow **WRITTEN EXPLICIT PRACTICE GUIDELINES** that cover the following treatment decisions for **Medicare patients**? [If no to all, Skip to question 11a; Otherwise, Skip to question 11b]  
    - Diagnostic imaging for back pain  
    - Screening for cervical cancer for women older than 65 years of age  
    - Screening for colon cancer for men older than 75 years of age
• EKGs, cardiac stress tests, or other coronary artery disease screening exams

11.  
  a. Are there plans to distribute practice guidelines (that cover treatment decisions for Medicare patients) to your ACO’s primary care physicians within the next 12 months? [Skip to question 13]
  b. Were the practice guidelines used by your ACO’s primary care physicians different 12 months ago? [If no, Skip to question 13]

12. When compared to the practice guidelines used 12 months ago, do the current practice guidelines cover the same types of conditions and procedures? If no, please explain how the guidelines differ.

13. Do your ACO’s primary care physicians have access to the following educational tools to help practice cost-effective primary care for Medicare patients? [If no to all, Skip to question 14a; Otherwise, Skip to question 14b]
   • Orientation program (at least two hours) for new physicians that discuss cost-effective care
   • Written document(s) that summarize recent cost-effective research
   • Periodic seminars or lectures focusing on cost-effective practice
   • Periodic retreats (at least one full day) which focus on the practice of cost-effective medicine

14.  
  a. Are there plans to develop educational tools to help your physicians practice cost-effective primary care for Medicare patients within the next 12 months? [Skip to question 16]
  b. Were the educational tools that cover cost-effective primary care in place 12 months ago? [If no, Skip to question 16]

15. When compare to the educational tools used 12 months ago, does the current set of education tools require a comparable, lesser, or greater degree of time commitment from your ACO’s practicing physicians?
16. Do your ACO’s primary care physicians rely on any of the following decision support tools to practice cost-effective primary care for **Medicare patients**? [If no to all, Skip to question 17a; Otherwise, Skip to question 17b]
   - Manual or computer-generated reminders or recommendations to conduct preventative care or screening services
   - Computer-generated recommendations concerning the use of diagnostic tests and exams
   - Computer-generated recommendations concerning physician specialist referrals
   - Computer-generated recommendations concerning the prescription of generic vs. branded drugs

17.
   a. Are there plans to implement decision support tools to help your physicians practice cost-effective primary care for **Medicare patients** within the next 12 months? [Skip to question 19]
   b. Were the set of decision support tools that your ACO’s primary care physicians rely upon different or employ differently 12 months ago? [If no, Skip to question 19]

18. When compared to the decision support system employed 12 months ago, do the current decision support system to issue physician recommendations or reminders for the same set of conditions?

19. Does your ACO have any policies that address which type or brand of comparable medical devices or drugs is used during medical procedures for **Medicare patients**? [If no, Skip to question 20a; Otherwise, Skip to question 20b]

20.
   a. Are there plans to implement policies that address which type or brand of comparable medical devices or drugs is used during medical procedures for **Medicare patients** within the next 12 months? [Skip to Section II]
   b. Were the policies concerning the choice of medical device or drug used by your ACO’s physicians different 12 months ago? [If no, Skip to Section II]
21. When compared to the policies employed 12 months ago, do the current policies cover the same types or brands of medical devices and/or drugs? If no, please explain how the policies differ.
SECTION II: ACO/PHYSICIAN GROUP QUESTIONS

1. Do the physician groups that comprise your ACO operate as a financial subsidiary of a larger integrated healthcare system?

2. Is your ACO partnered with a hospital as part of the Medicare ACO Program?

3. Do all or some of the physician that comprise your ACO operate as part of an ACO with a non-Medicare patient panel?

4. Approximately what percentage of the patients that your ACO’s physicians see are Medicare patients?

5. Does your ACO or its physician groups distribute bonuses or financial compensation based on your ACO’s performance on cost/utilization measures for its Medicare patients?
SECTION III: FACILITATORS AND BARRIERS QUESTIONS

1. Are there elements of your physician organization’s past experience, culture, or leadership that have been particularly influential in how your ACO approaches care and/or utilization management today? If so, can you explain?

2. Have any of the following local market factors influenced your decisions to adopt different utilization management strategies? If so, how?
   a. Private payor programs or initiatives
   b. Programs and policies implemented by other physician groups
   c. Competition from other physician groups

3. To what degree has your existing Health IT infrastructure enhanced or limited your physician’s group to deliver more efficient care? Please explain.

4. Where do you obtain most of your information about cost-effective practices that you would potentially implement in your physician group? Are there any barriers to accessing or acting on the information that you receive on cost-effective practices?

5. Overall, what are some of the most difficult challenges your organization has faced while adapting to the Medicare Shared Savings Program? Are there any policy changes that you believe would allow your organization to deliver more efficient care to Medicare patients?
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