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Working Paper Series
La Follette School Working Paper No. 2009-021
http://www.lafollette.wisc.edu/publications/workingpapers

Governance and Evidence-Based Medicine: Lessons from the Organ Procurement and Transplantation Network

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Based on research presented in David L. Weimer,
Medical Governance: Values, Expertise, and Interests in Organ Transplantation
(Washington, DC: Georgetown University Press, forthcoming 2010).

November 2009

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Abstract

One of the clearest demonstrations of the consistent implementation of evidence-based medicine in the United States is the use of statistical evidence and tacit knowledge within the Organ Procurement and Transplantation Network (OPTN), a congressionally created private organization with responsibility for the design of organ allocation rules. Could a governance arrangement with the OPTN features be created to "bend the cost curve" for Medicare through evidence-based medicine? An answer to this question is given through a sketch of MedSAVE, an organization that would set reimbursement rates and reporting requirements for surgeries paid for by Medicare.

Keywords: Organ transplantation, medical governance, evidence-based medicine, Medicare

An army of health service researchers, clinicians, and social scientists do research that produces evidence-based findings about how well specific medical practices work in various circumstances. Although these findings usually do not systematically compare a full range of possible practices within fields of medicine, they provide a rich source of ways medicine could be practiced better and often at lower cost to patients, society, and third-party payers. Yet, in most circumstances, such findings only haphazardly and belatedly influence actual medical practice.[1] The governance of the U.S. health care system does not effectively promote and utilize evidence-based research.

Despite the failings of current governance arrangements in the health care system, few researchers have focused their attention on how these arrangements could be improved. Perhaps this is not surprising in view of the difficulty of assessing the effectiveness of alternative arrangements.[2] Only a small number of institutions are available for comparative study within the United States. Expanding the empirical base to include arrangements in other countries suffers from a slew relevant differences in political systems, cultures, and histories.[3] Assessing the effectiveness of governance arrangements generally requires in-depth case studies of particular institutions over an extended period, a labor intensive project that does not fit well within either the very focused quantitative studies done by health services researchers or the very broad work by political scientists on the politics of institutional choice. Consequently, although the success of the current health reform proposals will depend critically on the governance arrangements they employ, we have little empirical research to guide the design of these arrangements.

I report here on a particular form of governance, private rulemaking,[4] as it has been employed for more than two decades to make policy concerning the allocation of cadaveric organs for transplantation. Created by Congress in 1984, the Organ Procurement and Transplantation Network (OPTN) engages stakeholders, mainly representatives of transplant centers, laboratories, and organ procurement organizations, in the use of evidence developed from data in the Scientific Registry of Transplant Recipients (SRTR) and in the application of their clinical experience to develop allocation rules with literally life and death consequences. After posing the puzzle of private rulemaking and briefly arguing that the OPTN, as a private rulemaker, has been exemplary in its use of evidence in rule development, I identify the institutional features that I believe make its success possible. Finally, I consider how the governance of Medicare payments for surgery could be arranged to employ the OPTN features to implement evidence-based medicine and "bend the cost curve" for this important category of health expenditure.

Why Private Rulemaking?

Private rulemaking, which has been employed in the United States for such diverse tasks as implementing agricultural cartels and allocating internet domain names, poses a puzzle: Why would Congress delegate important decisions about the substance of rules to stakeholders rather than a federal agency? Both normative and positive explanations seem plausible.[5]

Normatively, private rulemaking offers technical efficiency when stakeholders have essential information that cannot be gathered easily or on a timely basis by an agency. For example, agricultural marketing boards consisting primarily of local growers can more easily

make assessments of seasonal production than could the Department of Agriculture with any plausible staffing. This informational advantage is likely to be particularly important in situations, such as medicine, that are characterized by rapidly changing technology. Agencies, because of civil service rules, often find it difficult to keep up with the expertise of the stakeholders employing the new technologies. Of course, technical efficiency does not necessarily imply social desirability——agricultural marketing boards may effectively implement cartels that on net are economically inefficient. Whether or not the potential benefits of technical efficiency make private rulemaking socially desirable requires a full assessment of all the social values at stake.

The credit and blame calculus of politicians provides a positive explanation for private rulemaking. Considerable evidence from cognitive psychology suggests that those who suffer a loss are more likely to perceive it, feel affected by it, and act on that feeling than those who obtain a comparable gain.[6] Consequently, politicians are likely to see the opportunity for claiming credit as more than offset by the risks of accruing blame in situations of zero-sum allocation. In the political calculus of "credit claiming" and "blame avoidance," the latter is likely to dominate.[7] Delegating authority to a regulatory agency tends to shift the focus for debate from the legislative arena to the agency and the courts. In cases involving direct allocation of things of value, however, primary stakeholders who do not receive satisfactory allocations may lobby the legislature or the executive to change the rules, returning the issue to the political agenda. Further, legislators may anticipate pressure from special interests to micro-manage in dysfunctional ways,[8] and therefore seek to tie their hands and those of their successors to limit the politicalization of what they may believe to be matters best addressed by professionals. In order

to avoid blame and reduce the temptation to micro-manage, politicians may seek to insulate themselves with clear delegations of rulemaking authority to non-governmental organizations with internal procedures for resolving, or at least accommodating to some extent, stakeholder conflicts.

The attractiveness of blame avoidance is likely to be particularly great in medical governance, especially when individuals perceive that decisions have direct implications for their own morbidity or mortality. As identifiable "victims"of proposed rules, those who would be disadvantaged would make dramatic witnesses at congressional hearings and attract human interest media coverage, further raising the prospect that opportunities for politicians to claim credit would be far exceeded by their risks of accruing blame.

Evidence-Based Medicine in the OPTN

The OPTN, administered under a contract between the Department of Health and Human Services (HHS) and the United Network for Organ Sharing (UNOS), develops the rules that govern the allocation of solid organs made available for transplant from deceased donors. Although de jure the HHS must issue OPTN rules as federal regulations to give them the force of law, de facto the requirement that transplant centers be members of the OPTN to receive Medicare and Medicaid funds makes the OPTN rules binding. Indeed, the only OPTN rules issued as federal regulations by the HHS concern data reporting; no allocation rules have been formalized in the more than 20 years of OPTN operation.

In order to assess OPTN rulemaking, especially in terms of the use of evidence and expertise, I conducted case studies of the evolution of liver, kidney, and lung allocation rules

through document research, interviews, and direct observation of OPTN committee meetings over a more than ten year period. Specifically, I considered the very public controversy over the geographic basis of liver allocation during the 1990s, the incremental response to concerns about the tradeoff between the medical benefits of antigen matching and racial disparity in kidney waiting times since the early 1990s, the fundamental change from medical urgency to medical net benefit in the rules for the allocation of lungs in 2005, and the ongoing effort to reformulate the kidney allocation system to be based on medical net benefit.[9] On the basis of these case studies, I offer the following assessment of OPTN rulemaking.

First, the initial rules adopted by the OPTN have been frequently but incrementally modified. Incremental change is most obvious in the various modifications made to the kidney allocation system in an effort to lessen racial disparity in waiting times by reducing the weight placed on antigen matching in the allocation formula for kidneys. Nonetheless, a similar process operated before, during, and after the liver controversy, which was prompted by efforts of several large transplant centers to enlist HHS to force the OPTN to remove local priority (first access to livers donated in the local area to patients on the waiting lists at transplant centers in that area) in liver allocation. Amidst an extended HHS rulemaking, a congressional moratorium on the implementation of the HHS rule, a congressionally mandated study by the Institute of Medicine, and legislation and law suits by state governments, the OPTN continued to make incremental changes to the allocation system that provided a more confident assessment of medical need, separate assessment systems for adult and juvenile patients, rearrangement of priority categories, and moves toward regional rather than local sharing.[10]

Second, rule changes have been evidence-based. The OPTN was created in tandem with

the SRTR, an exceptionally comprehensive data base in terms of both universal and longitudinal coverage. The combination of tacit knowledge and the availability of data to support scientific investigation give the OPTN great potential for applying evidence-based medicine. The cases show this potential being substantially realized over the entire history of the OPTN. The committees that take the lead in rule development routinely review statistical and simulation evidence developed in response to questions posed at prior meetings and make requests for additional analyses. They have also promoted variances to allow for the accumulation of evidence directly relevant to alternative allocation rules through local experiments. As a result, there is a continuous flow of evidence into the OPTN rulemaking process. The evidence has been important in showing the declining medical importance of antigen matching, the validity of statistical models of medical urgency for liver patients, and the viability of allocating kidneys across some blood types, to name just a few examples.

Although not as obvious as the role of scientific evidence, the tacit knowledge of stakeholders also influences the policy development in important ways. Transplant professionals often raise questions based on their experiences that could be investigated with SRTR data. For example, they often raise concerns about specific types of patients who might inadvertently be disadvantaged by rule changes. In this way, they contribute to a level of sophistication that would otherwise be difficult for analysts removed from clinical experience. Transplant professionals' clinical experiences also enable them to anticipate the behavioral responses to proposed rule changes. This has shaped policy in a variety of ways, such as: an understanding of how qualitative classifications of patients could, and were, being manipulated propelled the move to quantitative classifications based on objective data for liver patients before moving to regional

sharing and for lung patients as part of the new system of allocation based on net benefit; careful setting of default values for missing data in classification systems to reduce the opportunity for manipulation through delaying the provision of up-to-date data; and modification of the current kidney allocation proposal to counter the reduced incentive that the emphasis on net benefits would provide for young patients to seek live donors.

The amount and technical sophistication of scientific evidence used in rulemaking appear to have increased somewhat since the resolution of the controversy over liver allocation and the separation of the administration of the OPTN and the SRTR. The former helped set higher expectations about the level of evidence that would be needed to justify rule changes and made the possibility of reversal of rules by HHS somewhat more credible. The latter promoted some independence in establishing the analytic agenda. Overall, these changes further strengthened the already strong role of evidence-based medicine within the OPTN.

Third, rulemaking within the OPTN is value overt. Transplant professionals bring with them norms that tend to align their preferences closely to the interests of patients. The strongest of these norms for the physicians, who are most influential in the OPTN committees, is promoting the welfare of their own patients. However, they also share norms about the desirability of promoting health and the fairness in the distribution of health care——observers note that, since creation of the OPTN, "references to justice and equitable access have become part of the everyday vocabulary of those involved with transplantation"[11]——as well as the appropriate use of evidence in assessing medical treatments, though they may bring with them differing perspectives that affect these assessments. Congress set expectations that these norms would be influential by charging the OPTN to employ medical criteria in matching patients with organs. Therefore, it is

possible that, even in the presence of strong interests, the coincidence of these interests with professional norms, or their moderation by professional norms, would tend to align technical efficiency with patient interests and other social values.

Fourth, reliance on evidence and professional norms does not eliminate interests. Conflicting interests among stakeholders was most apparent in controversy over the role of geography in liver allocation. A large transplant center led the effort to eliminate local priority in the face of opposition from many smaller centers. As representation within the OPTN did not privilege the larger centers, the smaller centers dominated the board of directors, a majority rule body. Frustrated by the failure to move toward elimination of local priority, especially when the board failed to adopt committee recommendations for regional sharing, the large center took the issue to the President and the HHS secretary, setting off the public controversy. The smaller centers were able to mobilize support in Congress, partly because of their presence in many congressional districts but also because many members of Congress were skeptical about the desirability of HHS imposing rules on the transplant community, and the implications that would have for them in terms of credit and blame in their oversight.

In summary, the OPTN appears to be a governance arrangement that facilitates the use of evidence and expertise, harnesses professional values, and accommodates conflicting stakeholder interests. Indeed, it is exemplary in implementing evidence-based medicine. Although the choice of institutional form must be specific to the context, the OPTN provides a model of governance for promoting evidence-based medicine that is at least worthy of consideration in other applications.

OPTN Features Facilitating Effectiveness

With the caveat that, in the context of institutional design, the whole is always more than the sum of the parts, it is nonetheless useful to identify key features that appear to contribute to OPTN effectiveness in producing continuous informed adjustments to policy aimed at furthering desirable goals. Eight features seem particularly important in the operation of the OPTN: meaningful stakes, professional engagement, continuity, decision-making by voting, specialization with consultation, transparency, data for creating evidence, and strategic oversight.

Meaningful Stakes

The time and energy of people with scarce expertise typically has high opportunity costs. These experts, or their employers, are more likely to be willing to bear the costs of participating in governance when they perceive the stakes to be meaningful. For the stakes to be meaningful, they must involve something of value, like access to scarce transplant organs, that can be influenced through their participation in governance. Participation in the governance of things of value becomes more meaningful when it is consequential, connecting participation in some direct way to outcomes. Clear delegations of authority to organizations for the development of particular aspects of policy make participation in the organizations consequential. Relatively narrow and sharply circumscribed authorities are likely to be most consequential because they reduce the chances of competition with alternative authorities within the larger polity.

The OPTN has clear authority to develop policy concerning cadaveric organs, which have great value because they are the scarcest resource in organ transplantation. Although the availability of cadaveric organs can be affected by a variety of policies, such as those related to

the determination of deceased-donor eligibility, the opportunities for live donations, and the incentives for transplant centers to expend effort in securing donations, their short-run supply is effectively fixed. Consequently, allocation rules are very consequential. Transplant surgeons and other highly skilled professionals contribute many hours to service on committees with reimbursement only for travel and lodging expenses. They are probably motived to some extent by altruism and the benefits of professional association. However, the substantial commitments they make almost certainly reflect their perceptions that the rules being made are very relevant to their ability to help their own patients and realize the financial gains that flow from doing so.

The direct connection between expertise and policy in OPTN governance that makes the stakes for participants meaningful tends to be muted for regulatory advisory committees for two reasons. First, advisory committees are often given the task of determining fact independently of policy development. That is, the committees are often asked to separate science from policy and politics. Sheila Jasanoff[12] and Bruce L. R. Smith,[13] based on their comparative studies of federal advisory committees, conclude that committees are likely to be most effective when they do not limit their advice to questions of fact. Second, committee members rarely know the extent to which the advice they offer will influence agency decisions. The more tenuous the connection between their advice and policy, the less consequential they are likely to view their efforts.

Professional Engagement

Expertise and practice-related values distinguish professions. Professionals typically enjoy a high status that reflects the investments in knowledge and craft skills they must master and the commit they make to particular values. Professional norms, which express these values in

terms of the duties professionals owe to those whom they serve, typically arise through self-regulation but often become formalized in law. Engaging professionals in policy making not only offers the potential for tapping their expertise but also for giving voice to their norms.

Doctors, nurses, epidemiologists, and other medical professionals have essential substantive expertise needed for effective medical governance. These medical professionals also share a common background in science that gives them at least some familiarity with making inferences from empirical evidence. Engaging them in rulemaking not only taps their substantive expertise, but also brings to the table people with generally greater methodological sophistication than samples of the general public or some other professions, such as law. The shared methodological sophistication does not eliminate disputes over inference, but it does provide a framework for focusing debate and identifying relevant questions that could be answered empirically. In other words, it facilitates an evidence-based approach.

Beyond their contributions of expertise and expression of norms, professionals may also bring legitimacy to the rulemaking process. To the extent that the public and their elected representatives trust professionals to pursue particular values consistent with social welfare, they may be more willing to defer to the professionals, making them authoritative in their domains of expertise.

Rulemaking by the OPTN relies heavily on the participation of transplant professionals who bring relevant expertise to the table. The norm of promoting patient welfare, as well as a shared understanding of science, helps shape deliberations about allocation rules to be value-overt and evidence-based.

Continuity

The OPTN is an institution by virtue of both its formally delegated authorities and the generally realized expectations about how it will exercise those authorities. Its internal organization and practices create and maintain these expectations. Specifically, standing committees with clear jurisdictions, multi-year memberships with staggered terms, and assigned support staff give continuity to policy development and implementation.

The continuity enables the committees to deal effectively with many inherently complicated issues by providing an opportunity for members to learn about them over their two-year terms. The opportunity to learn is most important for those who do not have expertise directly relevant to the issue at hand, but it is also important for enabling those who do have such expertise to relate it directly to problems with existing policies and proposed alternatives for addressing the problems. Learning about the other committee members through mutual participation may also increase trust in the information that they provide. When major changes in policy are being developed, often over an extended period, committees may be allowed to grow in size so that existing members who have accumulated issue-specific knowledge can be reappointed as new members are added. The UNOS staff assigned to support committees contribute to learning by providing extensive pre-meeting materials, post-meeting minutes, information about deliberations in other committees, and guidance on organizational procedures for making policy changes.

The nature of this continuous engagement with policy issues differs from that found in conventional rulemaking. Although a regulatory agency may continuously assess policy in a particular area, only the most interested stakeholders are likely to communicate with it in the

absence of a notice of proposed rulemaking. Following a notice of proposed rulemaking, more stakeholders may volunteer information, but primarily in response to the particular proposed rule rather than related to the nature of the problem, or other problems perhaps of more importance, or alternative rules.

The continuous involvement of stakeholders is most important when they have tacit knowledge relevant to the design of effective rules. Engaging a full range of stakeholders increases the chances that relevant information that could be drawn from their tacit knowledge will be brought to the process. Engaging them as long-term participants in the decision-making process may increase the likelihood that they will be willing to reveal information that does not support the rules that they prefer, especially as they may have to respond to problems that actually arise during implementation.

Decision-making by Voting

Voting serves the very important instrumental purpose of allowing a decision to be made in the absence of consensus. In the development of complex policies, voting may also be useful in facilitating, at least temporarily, the specification of components needed to assemble a complete proposal and therefore move toward a final decision. Although voting can sometimes be sophisticated in the sense of people voting against their true preferences at the early stages of the process to obtain a more favorable final outcome, the proportions voting for and against a proposal generally convey information about the degree of consensus on the proposal.

The OPTN board of directors approves policies and bylaws through majority rule voting. In addition, committees routinely vote on questions related to the development of policies and

bylaws. Particularly in the development of complicated policy proposals, these votes allow the policy development process to move forward, resolving some issues so that attention can be focused on those that remain. Voting in committees also facilitates the process of continuous incremental change to existing policies. It does so by enabling committees to achieve "small wins," a series of concrete outcomes of moderate importance that may "attract allies, deter opponents, and lower resistance to subsequent proposals."[14] More mundanely, the small wins may help keep committee members engaged by making them feel more efficacious.

Relying on majority rule voting may cause instability if the distribution of votes does not reflect the distribution of interests. In the case of the OPTN, the equal representation of small and large transplant centers gives the larger centers less influence than they would likely have in conventional regulation. This appears to have been the case with respect to liver allocation during the 1990s: the University of Pittsburgh Medical Center attempted to achieve its goal of national allocation of livers through intervention by HHS when its efforts within the OPTN were frustrated. This risk to stability must be weighted against the various advantages of majority rule voting by heterogeneous memberships of organizations in the design of governance arrangements.

The extensive use of voting in the OPTN contrasts with its use in conventional regulation. Independent regulatory commissions take votes, but usually only on fully developed policy proposals——the actual development of the policy proposals, as with agency regulation, typically does not proceed through recorded votes. Regulatory agencies may engage advisory committees that record votes. However, as noted above, these committees usually advise on scientific and technical matters, rather than specifically on policy development, so that their votes rarely focus on specific policy choices and, in any event, are not binding on the agency.

Specialization with Consultation

Policy development often involves a tension between expertise and the representation of the full range of relevant values. Designing policies that can be effectively implemented in complex circumstances and predicting their consequences generally requires the tacit knowledge of specific experts. If these experts share very similar experiences, then they may also hold similar values. Consequently, it is usually valuable to broaden the policy design process to include a wider range of knowledgeable participants who may bring values to the process that might otherwise be ignored or only surface as controversy over the ultimately proposed policy.

In the case of the OPTN, the main locus of policy development is typically the standing committee with recognized "jurisdiction." Most importantly, specific committees take primary responsibility for allocation of the various types of organs. These organ allocation committees, which also include patient representatives and ex officio representatives of the SRTR and HHS, have a majority of members with substantial experience with that organ's transplantation, as surgeons or other transplant professionals. Policy changes usually emerge from these committees as proposals to the board of directors.

The organ committees consult regularly with other committees, including those that have "jurisdictions" over aspects of transplantation generally, such as patient affairs, minority affairs, and ethics. Consultation among committees occurs through a number of mechanisms, including representatives from these general jurisdiction committees serving as members of the organ allocation committees, presentations to these committees by members of the organ allocation

committees, sharing of information and analyses, and requests to the general jurisdiction committees for comments on specific proposals. These mechanisms promote extensive consultation during policy development.

As with public rulemaking, the OPTN also seeks public comment on proposed policies once they have been developed. In both cases, the comments tend to come from the most interested stakeholders or from individuals mobilized by them. The responses to OPTN rules are sometimes quite strong, particularly when groups of patients already on transplant waiting lists perceive policies as threatening their priorities. The board of directors may respond to strongly negative comments by rejecting the proposal or sending it back to the committees for redesign. Interestingly, in many cases where policy changes were vocally opposed but nonetheless adopted, the controversy did not continue once implementation began.

Transparency

Some degree of transparency in governance is a prerequisite for accountability and perhaps for legitimacy as well. The OPTN deliberates about policy in public. With the exception of meetings to handle membership issues, anyone can attend committee meetings as observers. Although there is some variation by committee, meeting summaries and reports to the board of directors are routinely posted on the OPTN website as are executive summaries of the minutes of board meetings. Those interested in participating in OPTN policy development can add their names to an e-mail list of people who are sent requests for comments on proposed policies. The OPTN is a very transparent form of governance.

The transparency of OPTN governance prevents stakeholders from being surprised by

policy decisions. The deliberative process provides many avenues for involvement in policy development—participation in committees, contact with committee members, and comments on proposed policies. At times, stakeholders have mobilized potentially interested parties, such as patients with a particular disease or organizations with missions related to specific diseases, to send comments. Consequently, the board of directors is rarely surprised by reactions to policies. Especially as policies can have life and death implications for patients on transplant waiting lists, the very transparent deliberations during policy development almost certainly add to the legitimacy of the policies that result, perhaps at the cost of greater difficulty in making non-incremental changes.

The transparency of policy development within the OPTN contrasts with the largely opaque process of policy development through conventional rulemaking.[15] The primary source of information on rule developments underway in federal agencies is the Semiannual Regulatory Agenda (Unified Agenda), which has been assembled and published by the Office of the Federal Register since 1983 and made available electronically since1994. Until 2007, when the criteria for listing were narrowed to focus on rules with significant economic impact on a large number of small entities, agencies were required to list all the proposed and final regulations they expected to issue during the next year. Monitoring the Unified Agenda allows interested parties to communicate with the agency during rule development. Indeed, Susan Webb Yackee finds evidence that interested parties do engage in influential ex parte communications with the agencies, but that this process favors resource-rich interests.[16] However, as the title of Yackee's paper, "The Hidden Politics of Regulation," makes clear, the rule development process is far from transparent. Indeed, only in the case of negotiated rulemaking, which directly involves

stakeholders in rule development, is the process nearly as transparent as it is within the OPTN.

Data for Creating Evidence

In the 1990s, evidence-based medicine arose as the "most important contemporary initiative committed to reshaping biomedical reason and practice.[17] Systematic research to assess the effectiveness of medical interventions requires data linking the interventions to meaningful outcomes. Data generated through, or as a byproduct of, clinical practice is almost always inferior to data from well-planned and well-executed experiments that guard against self-selection or steering into treatment modalities. Nonetheless, observational data from clinical practice can still be of great value, especially if they comprehensively cover relevant groups of patients and include high quality measures of outcomes. Clinical data may provide the only practical way to gain sufficiently large samples to find rare but significant effects or to find effects that manifest only after long periods of time.

The Scientific Registry of Transplant Recipients is an exemplary clinical data base. It includes a near universe of transplant candidates and it follows transplant recipients over an extended period of time——until death. The OPTN contributes to the quality and appropriateness of the data in the registry through the policies it sets concerning reporting by transplant centers. Analyses using the data, primarily statistical and simulation models developed by SRTR staff, play an important role in policy development and evaluation efforts of the OPTN committees—— the data provide systematic evidence to complement the tacit knowledge of their members. Arguably, because of the registry data and its use by the SRTR and the OPTN, organ transplantation is at the forefront of evidence-based medicine. Absent data to create relevant

evidence, one can wonder if the OPTN committees would be able to achieve sufficient agreement to continue to engage effectively in continuous policy revision.

Strategic oversight

Delegation of authority within representative government demands some sort of oversight to guide and monitor those exercising the authority. The most familiar delegations flow from legislatures to public agencies. Legislatures oversee agencies in a variety of ways ranging from routine monitoring, so called police patrol, to event-triggered or interest group prompted scrutiny, so called fire alarms.[18] Public agencies also often delegate, either by choice or mandate, to other entities, including other levels of government and private organizations. In these delegations, the agencies provide the primary oversight. Effective governance requires that oversight be appropriate for the delegation. Of course, it should be strategic in the sense of anticipating the reactions of the overseen, but it should also be strategic in the sense of promoting broad social goals rather than prescribing specific policies.

Congress created the OPTN as a distinct entity but made it subject to oversight by HHS. The oversight role of HHS with respect to organ procurement and allocation policies was initially ambiguous. The initial follow-up legislation seemed to strengthen the independence of the OPTN. Nonetheless, HHS asserted its authority over making OPTN rules binding, establishing a distinction between de jure rules that have the force of law and de facto rules that actually govern transplantation.

Aside from accepting, rejecting, or requiring OPTN actions through rulemaking, HHS exercises oversight through its management of the contracts for administration of the OPTN and

the SRTR. Although the OPTN and the SRTR each has a legislative basis, and the OPTN effectively has its own "taxing" power through its setting of fees for placing patients on waiting lists, HHS selects the organizations that administer them. Initially, they were both administered by UNOS, the administrator of the voluntary network of transplant centers that predated the creation of the OPTN. Later HHS took the contract for administering the SRTR away from UNOS, putting in place the current arrangement of separate administrators. The ex officio membership of HHS personnel on OPTN committees facilitates ongoing monitoring of policy development and implementation. These memberships reciprocally provide committees with information about what to expect from the otherwise largely opaque processes operating within HHS.

Since the resolution of the controversy over liver allocation, HHS has engaged in what might be termed "strategic oversight"——sparing and selective interventions to guide or direct the OPTN and SRTR. The HHS rule that ultimately emerged from the controversy requires review of allocation rules in terms of general goals, but it does not specify how those goals are to be met. Most importantly, HHS has not attempted to formalize or overturn *any* of the OPTN substantive allocation rules, but rather allows them to operate de facto. In contrast, it has acted to make OPTN rules concerning data reporting by transplant centers and organ procurement organizations legally binding so that OPTN efforts to ensure compliance with them have the force of law. HHS has also been strategic in directing the SRTR to develop new simulation models for use in assessing changes in organ allocation policies.

These selective and facilitating interventions contrast with the blunt effort during the liver controversy to push the OPTN toward national sharing of livers and other organs. That effort was

politically rebuffed by a congressional moratorium and largely scientifically rebuffed by the Institute of Medicine study mandated by Congress. It elicited court challenges and played out over a number of years before the less intrusive version of the HHS rule ultimately emerged. Although the controversy was extraordinary in many respects, it nonetheless gives a sobering view of how contentious organ allocation policy making could be if it were to be routinely done through conventional rulemaking. The possibility of overturning or preempting OPTN policies, or even replacing the OPTN contractor, gives HHS considerable leverage over the OPTN. However, as these actions would likely have costly consequences for all parties, they are most effective when they remain threats rather than actions. In game theoretic terms, they constitute a punishment path that helps keep HHS and the OPTN on an equilibrium path of cooperation.

Bending the Curve: MedSAVE

In the United States, surgeries are numerous, expensive, often lack solid evidence regarding their efficacy, and usually have not been assessed in terms of their cost-effectiveness. In 2005, the physician services payment system employed by Medicare Part B paid about $58 billion to physicians for their medical services, including over $8.5 billion to 13 surgical specialties.[19] With Medicare Part A spending roughly two dollars for in-patient hospital services for each Part B dollar, the total Medicare costs of surgery would have been about $25 billion annually. With a ratio of four-to-one for total surgeries to surgeries for those 65 years and older, and Medicare reimbursing at roughly 80 percent of the private rate, the total surgical bill in the U.S. in 2005 was probably about $120 billion.

Assessing the appropriateness of the large annual expenditures on surgeries requires

knowledge about the efficacy and cost-effectiveness of the various types of surgeries in the contexts in which they are used. Unlike new pharmaceuticals, which are regulated by the Food and Drug Administration, surgical procedures do not require "well controlled studies" to demonstrate their effectiveness and safety before they enter mainstream practice. As noted by Alan Gerber and Eric Patashnik in their account of arthroscopic surgery to treat osteoarthritis of the knee, at least since the 1970s, medical critics have noted the weak evidentiary basis typically advanced in support of new surgical procedures.[20] Gerber and Patashnik note the lack of research on the effectiveness of even widely adopted surgical procedures as well as failure of the surgical professions and the Medicare program to make use of the evidence that is available.

A recent study of angioplasty illustrates the problem of the failure to follow established guidelines for surgery. Percutaneous coronary intervention, commonly referred to as angioplasty, is used to treat coronary artery disease. However, it appears not to be more effective in terms of death or myocardial infarction than less expensive treatments involving drugs and life-style changes. As studies have shown that its use in patients with minimal symptoms appear on average to produce adverse outcomes, guidelines jointly issued by three relevant medical societies recommend that moderate or severe ischemia in stable angina patients be documented through noninvasive tests, primarily stress tests, before proceeding with angioplasty. Grace Lin and colleagues found that only about 45 percent of stable patients with Medicare fee-for-service coverage had stress tests within 90 days prior to their angioplasties. In other words, it appears that a majority of procedures were performed in contradiction to evidence-based guidelines.[21] George Diamond and Sanjay Kaul note that one reason for the underutilization of stress tests prior to angioplasty may be that the guidelines lack clarity.[22] Nonetheless, Diamond and Kaul

recommend that Medicare payments for angioplasties performed on stable patients without stress tests be reimbursed at a lower rate than those performed following stress tests that document the moderate to severe ischemia called for in the guidelines.

The differential payment proposal applies the strategy of fee-for-benefit rather than fee-for-services.[23] In view of its failure to follow up on the findings of the controlled trial of arthroscopic knee surgery, however, it does not seem likely that the Centers for Medicare & Medicaid Services (CMS) would take steps on its own to implement the fee-for-benefit strategy. More generally, despite the increasing efforts of the CMS, the Agency for Healthcare Research and Quality, the National Institutes of Health, and the Department of Veterans Affairs to generate information on the comparative effectiveness of medical and surgical interventions, the Medicare Payment Advisory Commission (MedPAC) has concluded that there is currently insufficient credible "information available to patients, providers, and payers to make informed treatment decisions."[24] MedPAC goes on to raise several possibilities for creating a more effective governance arrangement to support the generation and assessment of information on comparative effectiveness, including a public-private entity, like the Federal Reserve, or a purely private entity. The purpose of this exercise is to sketch briefly how an entity modeled after the OPTN could play this role for Medicare surgery, perhaps providing a mechanism for heeding the call of Miriam Laugesen for "greater direct responsibility by physicians for higher quality and medically effective services and greater oversight of what Medicare pays for, perhaps informed by comparative effectiveness."[25] For ease of reference, I call this entity the Medicare Surgery Assessment Volunteers for Effectiveness (MedSAVE).

Like the OPTN, MedSAVE would be created through legislation. Its primary function

would be to assess the effectiveness and cost-effectiveness of surgeries. The stakes would be made meaningful for participants by combining an overall annual budget for surgical services paid through Medicare Part B with the authority to set payment rates for specific surgical services through majority voting by a board of directors. Membership would be open to organizations representing surgeons and the surgical specialities, other organizations representing those concerned about specific diseases and the elderly, medical ethicists, and interested individuals drawn from the general public, patients, and the professions. Funding would be provided to support a full-time staff, a companion registry of surgeries like the SRTR, and participation expenses for individual members of MedSAVE through a small surtax on surgeries paid for by Medicare.

Participants and General Governance Structure

MedSAVE would require explicit and systematic representation of the surgical specialties. The board of directors, which would have responsibility for approving surgical payment schedules, would be broadly representative but also include members selected by the American College of Surgeons, the American Academy of Ophthalmology, the American Academy of Orthopaedic Surgeons, the American Academy of Otolaryngology, the American Association of Neurological Surgeons, the American College of Obstetricians and Gynecologists, the American Pediatric Surgical Association, the American Society of Colon and Rectal Surgeons, the American Society of Plastic Surgeons, the American Urological Association, the Society for Vascular Surgery, and the Society of Thoracic Surgeons. Other organizations representing the medical professions, those interested in specific diseases, the elderly, and taxpayers would be

invited to nominate people to participate in MedSAVE committees and the board of directors. So, for example, the board of directors might consist of the representatives appointed by the surgical societies and an equal number of people selected by CMS to represent broader medical and societal interests.

MedSAVE would create standing committees to advise the board of directors with recorded votes. Some of these committees would likely focus on specific surgical specializations while others would address cross-cutting issues, such as standards for evidence, ethical concerns, and the development of coherent fee schedule proposals. A professional staff, funded from the surgery surtax, would support the work of the committees and the board of directors. The surtax would also provide funds for a sister organization, like the SRTR, that would assemble a database to help assess surgical effectiveness, analyze its data as requested by the MedSAVE committees, and also make it available to researchers generally.

HHS would exercise strategic oversight in several ways. First, CMS would appoint the non-surgical specialists to the board of directors. Second, CMS would have ex officio representation on the board of directors and the committees, and it would be responsible for ensuring that decision-making within MedSAVE remained transparent. Third, the HHS secretary would have the option of overruling the fee schedule adopted by MedSAVE and forcing across-the-board reductions. This last form of oversight employs what Bradley Karkkainen calls a "regulatory penalty default," a provision that imposes harsh terms on regulated parties who fail to produce acceptable alternatives voluntarily.[26] It would be the "sword on the wall" that could be used if MedSAVE failed to carry out its responsibility to promote efficacy and efficiency in the provision of surgery to Medicare patients.

Money on the Stump: The Surgical Budget Constraint

Health care systems around the world have used four types of targets to control costs: price, volume, capacity, and appropriateness.[27] MedSAVE would employ three of these targets. A combination of price and volume targets would set a "global" maximum on expenditures for surgical services similar to the one currently employed for all physician services under Medicare Part B, and the members of MedSAVE would have an incentive to assess the appropriateness of types and circumstances of surgeries and discourage inappropriate surgeries through the fee schedule. The global budget would implement the advice offered by Alice Rivlin, the first director of the Congressional Budget Office: "[T]he best rule for politicians for dealing with generals, admirals, and doctors may be this: put the money on the stump and run."[28]

Originally, Medicare reimbursed physicians at rates that were "customary, prevailing, and reasonable." The Social Security Amendments of 1972 (PL 92-603) sought to slow the growth in Medicare expenditures by limiting the rate of increase in the fees paid for physician services to an index based on the average increase in physicians' costs of doing business. However, expenditures continued to grow rapidly as physicians increased the volume of services they provided to Medicare patients. The Omnibus Budget Reconciliation Act of 1989 (PL 101-239) authorized the setting of fee schedules for physicians providing services to Medicare beneficiaries based primarily on physician time, skill, intensity, practice expenses, malpractice insurance costs, and geographic area. It also imposed volume standards, limits on the growth in the number of procedures, intended to control the annual increase in expenditures. The fee schedules, which

went into effect in 1992, were accompanied by annual volume standards for surgical, primary care, and non-surgical services that sought to limit the increase in total expenditures for Medicare Part B.

The Balanced Budget Act of 1997 (PL 105-33) replaced the volume standards with the sustainable growth rate (SGR) system, which set both annual and cumulative targets for Medicare Part B spending. The SGR itself depends on several factors: the estimated percentage change in physician fees, the estimated percentage change in the number of fee-for-service Medicare beneficiaries, the estimated percentage growth in real gross domestic product per capita (10-year moving average), and the estimated percentage changes resulting from changes in laws and regulations. Beginning in 2002, the SGR target required across-the-board reductions in physician fees. The reduction called for in 2002, 5.4 percent, was actually implemented. However, in each of the following five years Congress intervened to block the implementation of reductions, instead allowing small increases in total expenditures to occur.

Why has the SGR system been ineffective in controlling expenditure growth? As noted by Rick Mayes and Robert Berenston in their review of the Medicare prospective payment system, under the SGR "prudent physicians are penalized financially, while profligate ones are rewarded" because, from the perspective of the individual physician, gains from over-provision of services are larger than losses due to the contributions of those services to the overall fee reduction.[29] Of course, this is just a tragedy of the commons, a classic common property resource problem in which individuals base their decisions on their private marginal benefits and costs, ignoring the contribution of their decisions to the average costs to all users of the resource. The most familiar approaches for countering the over-consumption of a common property resource are

regulation and self-governance. With respect to the Medicare expenditure problem, regulation involves CMS taking steps to limit service use, which have been largely ineffective and often elicit opposition from the medical profession. Self-regulation involves the resource users, physicians receiving Medicare payments, taking actions to limit their collective over-utilization.

MedSAVE would provide the governance arrangements in which at least the surgical professions would have an incentive to self-regulate. The primary incentive would be the imposition of a separate SGR for surgeries. It would be implemented by calculating current total payments for surgeries under Medicare Part B as the starting point. In subsequent years the SGR would be used to calculate the new expenditure targets for surgery. Actual expenditures in excess of the target amount would trigger proportional reductions of all surgical payments in the subsequent year. However, MedSAVE would have the authority to adjust the resource-based payments for surgeries so as to discourage inappropriate surgeries and reduce actual expenditures. The threat of across-the-board reductions in surgical fees, and the opportunity to influence the determination of inappropriate surgeries would provide the meaningful stakes to encourage participation in MedSAVE by the most relevant stakeholders, the surgical specialties.

Authorities: Payment Rates and Data Requirements

The primary authority of MedSAVE would be to apply downward reductions to the resource-based fee schedule for surgeries currently employed in the Medicare Part B prospective payment system——what one can think of as full payment for purposes of discussion. MedSAVE could reduce the full payment to signal uncertainly about the effectiveness of a surgical procedure, or reduce it to zero to remove it from Medicare coverage entirely. MedSAVE would

also have the authority to specify the conditions necessary for full payment. For example, returning to the angioplasty example, MedSAVE could choose to specify that the full payment would be made in the case of stable angina patients only if moderate or severe ischemia is documented through a stress test prior to the procedure.

MedSAVE would also have the authority to require that specific data be collected before and after surgery as a condition of full payment. This authority could be employed to produce data to provide better assessments of the effectiveness and safety of surgical procedures. Data requirements would be potentially very useful in assessing both commonly used procedures of questionable effectiveness and new procedures that are beginning to be used.

Finally, MedSAVE would have the authority to use some of the financial resources it receives from the surtax on surgeries to fund research on surgical procedures. The research funded could include controlled experiments, perhaps employing "sham surgery" methods where ethically appropriate, as well as observational studies based on administrative and required data. These studies would create evidence to support decision-making by MedSAVE

Potential Benefits (and Risks) of MedSAVE. If MedSAVE functioned as well as the OPTN, then the benefits could be substantial. Most importantly, MedSAVE would provide a framework for implementing evidence-based surgery. It would do so by tapping the tacit knowledge of surgeons and creating incentives for the development of data necessary to produce evidence about effectiveness. The driving force would be the opportunity to apply the scalpel to cut out inappropriate surgeries to avoid having the hatchet applied to fees across the board.

MedSAVE would thus offer the potential of actually controlling the growth in a substantial component of Medicare Part B spending.

A successful MedSAVE would likely produce a number of desirable spillover effects. Reductions in unnecessary, ineffective, and dangerous surgeries would reduce hospitalization costs covered by Medicare Part A as well as physician costs. Many private insurers would take advantage of the signals sent by fee schedule adjustments made by MedSAVE in setting their own reimbursement rates and coverages. MedSAVE would become a model for efforts to tie the search for appropriate medical practice to expenditures controls in other categories of physician services.

Adopting MedSAVE would not be without risks. The OPTN formalized an existing network of a community of transplant surgeons that had experience voluntarily sharing organs among their transplant centers. The creation of MedSAVE would represent a much more dramatic move beyond CMS advisory committees and MedPAC. It would require cooperation across heterogeneous surgical specialties rather than within a single surgical community. The liver allocation controversy that engulfed the OPTN shows the potential vulnerability that MedSAVE would face if it failed to accommodate powerful surgical specializations that could attempt to overturn unfavorable decisions with appeals to HHS or the Congress.

Perhaps the most relevant difference between MedSAVE and the OPTN is the scarcity that creates the high stakes. MedSAVE would require the imposition of a budget constraint that, unlike the natural constraint of a limited number of cadaveric organs, is legislatively created and therefore subject to legislative change. Can Congress credibly commit to a fixed budget for surgery or for any other set of medical services for which we seek to induce evidence-based

assessment? If the answer is no, then the various interests may put resources into lobbying Congress instead of implementing evidence-based medicine. Rather than conflicting with the promotion of good medical practice, more stringent cost control may actually be a prerequisite for more effective evidence-based medicine.

Conclusion

In our complicated medical system, traditional governance, involving federal agencies assisted by advisory committees, does not offer great hope for controlling costs and implementing evidence-based medicine. Many current health reform proposals recognize the problems inherent in our current governance arrangements. Some also recognize the advantages of insulating governance to some extent from interference. However, it is not clear how agencies can be kept independent in the face of lobbying by various stakeholders. We have at least one example of governance, private rulemaking of organ allocation rules, in which isolation has been generally effective and contributed to the implementation of evidence-based medicine in an area of extremely high stakes. No governance arrangement is perfect——indeed, it is not clear that well intentioned observes could agree on the definition of perfection——and one can easily find instances in which the OPTN moved slowly or took missteps in changing organ allocation rules. Nonetheless, its implementation of evidence-based medicine is substantial, sustained, and consequential.

Is the OPTN form of governance *sui generis* or can it be applied in other medical contexts? I have tried to answer this question by sketching MedSAVE, a private rulemaker that could potentially control costs and induce evidence-based medicine for Medicare surgery. The

prerequisite for an effective MedSAVE is creating meaningful stakes through delegated authority to allocate a scarce resource. The meaningful stakes, in turn, can secure continuous professional and other stakeholder engagement, providing both expertise and the possibility of keeping rulemaking isolated from legislative politics. A rich internal committee structure facilitating specialization and consultation, the reliance on majority rule voting to make decisions and move proposals forward, and creation of databases to produce evidence facilitate the effective use of expertise. Transparency and strategic oversight would provide democratic accountability.

Notes

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