Private Sector Risk-Sharing Agreements in the United States: Trends, Barriers, and Prospects

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ABSTRACT

Objectives: Risk-sharing agreements (RSAs) between drug manufacturers and payers link coverage and reimbursement to real-world performance or utilization of medical products. These arrangements have garnered considerable attention in recent years. However, greater use outside the United States raises questions as to why their use has been limited in the US private sector, and whether their use might increase in the evolving US healthcare system.

Study Design: To understand current trends, success factors, and challenges in the use of RSAs, we conducted a review of RSAs, interviews, and a survey to understand key stakeholders' experiences and expectations for RSAs in the US private sector.

Methods: Trends in the numbers of RSAs were assessed using a database of RSAs. We also conducted in-depth interviews with stakeholders from pharmaceutical companies, payer organizations, and industry experts in the United States and European Union. In addition, we administered an online survey with a broader audience to identify perceptions of the future of RSAs in the United States.

Results: Most manufacturers and payers expressed interest in RSAs and see potential value in their use. Due to numerous barriers associated with outcomes-based agreements, stakeholders were more optimistic about financial-based RSAs. In the US private sector, however, there remains considerable interest—improved data systems and shifting incentives (via health reform and accountable care organizations) may generate more action.

Conclusions: In the US commercial payer markets, there is continued interest among some manufacturers and payers in outcomes-based RSAs. Despite continued discussion and activity, the number of new agreements is still small.

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Risk-sharing agreements (RSAs) between drug manufacturers and payers—also called performance-based risk-sharing arrangements (PBRSAs), managed entry agreements, patient access schemes, and coverage with evidence development (CED), among other terms—link coverage and reimbursement levels to real-world performance or utilization of medical products.[1] These arrangements have garnered considerable attention in recent years: a recent analysis of the University of Washington Performance-Based Risk-Sharing (PBRS) Database,[2] including 148 arrangements over the last 2 decades, concluded that although the overall pace of adoption seems to be slowing, several new countries have begun to implement RSAs.[3] In the United States, the largest number of arrangements has been in the Medicare program; the uptake of private sector RSAs seems stagnant despite growing interest in the general principle of "paying for performance." The aim of this study was to assess the state of and prospects for private sector RSAs in the United States, considering by comparison experiences from other countries, most commonly involving public payers.

RSAs offer a number of potential advantages,[1,4-8] including: 1) reducing the risk to payers of making a suboptimal purchase; 2) providing earlier access to medications for patients; 3) creating international pricing efficiency, especially in a world with external reference pricing and parallel trade; and 4) generating evidence on what works in the real world. These potential advantages, coupled with the higher volume of use outside the United States, raise questions as to why they have seen limited use in the United States,[9,10] as well as what arrangements might gain traction with the new emphasis on accountable care organizations (ACOs), which operate under stronger incentives to manage patient care efficiently.

Previous work in this area has characterized the types of RSAs, trends in their adoption, economic incentives and rationale, and the drivers and barriers for adoption, but has

not focused on current thinking in the US private sector. A broad distinction has been made between "outcomes-based" RSAs and "financial-based" RSAs. For the former, the manufacturer provides or agrees to rebates, refunds, or price adjustments if their product fails to meet agreed-upon clinical outcome targets. For the latter, reimbursement is tied to financial measures (eg, total sales) or to utilization. Here, we focus on the US private sector marketplace and particularly on the level of interest in outcomes-based agreements; specifically, we sought to understand the barriers to and drivers of developing private sector RSAs in the United States. We also explored whether the current situation reflects a broader demand by regulators and payers for greater real-world data and comparative evidence.

Take-Away Points

This research assessed whether risk-sharing agreements between US commercial payers and pharmaceutical companies may become of greater interest with improved data systems and shifting incentives (via health reform and accountable care organizations). Key findings:

- While such agreements offer numerous advantages to payers, pharmaceutical companies, patients, and society, their growth remains stagnant in the United States, due in large part to the additional effort required to implement and adjudicate risk-sharing agreements as compared to traditional rebates.
- In the US private sector, however, there remains considerable interest: improved data systems and shifting incentives may generate more action.

METHODS

To assess the validity of the general perception of the lack of uptake of RSAs in the United States, we conducted a review of the University of Washington's PBRS Database to assess the number of US agreements and trends in the number of agreements over time. For comparison, we also examined the number of agreements for the rest of the world. At the time of the study, the database included information on RSAs initiated from January 1993 to December 2013, with information on the parties participating in the arrangement, the type of arrangement, and details of the arrangement and/or outcomes, as available.

To understand current trends, success factors, and challenges in the use of RSAs, we conducted key informant interviews and an online survey to understand key stakeholders' experiences with RSAs both inside and outside the United States as well as lessons for the US private sector situation. We used purposive sampling to identify 16 key stakeholders from pharmaceutical companies, payer organizations, and industry experts in the United States and European Union. We developed the interview guide and survey based on previous work in the area[1,2] and through iterative review and revision by a group of content experts. The key informant interviews consisted of in-depth, 1-hour telephone interviews conducted between October 2013 and May 2014. Interview topics included: experience with RSAs, motivating factors, administrative considerations, effectiveness of agreements, and potential for use in the future (**eAppendix A**, available at **www.ajmc.com**). To identify themes from the interviews, we assessed the number of interviewees who stated or generally agreed with key statements across all interviews.

The survey was administered via the Web in May 2014, and consisted of 8 questions that asked respondents about their expectations for the future of RSAs in the United States and to identify and rank key barriers to their use (**eAppendix B**). We identified themes by assessing the number of respondents who provided similar answers to the surveys. We invited a convenience sample of 37 individuals to complete the survey. Given the small sample size, we summarized responses using descriptive statistics.

RESULTS

Our review of the PBRS Database identified 148 RSAs worldwide from the late 1990s and 2013. Only 18 of the 148 total arrangements in the University of Washington Database, or 12.2%, represent US RSAs: 11 of these were public sector coverage with evidence development schemes, while only 7 were private sector RSAs. A review of the number of agreements annually suggests a little growth in the number of agreements in the United States. To date, RSAs have been more frequently employed in single-payer systems across Europe, Canada, and Australia.

Of the 16 individuals targeted for our key stakeholder interviews, we were able to schedule and conduct interviews with 14 (87.5%). The 14 in-depth interviews included 9 interviews with US stakeholders and 5 interviews with EU stakeholders. Of these, 7 were from pharmaceutical companies, 5 were currently or previously employed by payer/government organizations, and 2 were subject matter experts. We received 15 responses to our survey (response rate of 40.5%), including 10 responses from individuals from pharmaceutical companies, 3 from payers/pharmacy benefit managers, and 2 from other nonprofits and consultancies. The majority of respondents (93.3%) were from the United States.

In general, our interviews suggest that while there is interest in RSAs, there has been limited use in the United States due to difficulties in implementing and carrying

out such agreements. The majority of interviewees (12 of 14) were cautiously to mildly optimistic about the future potential for outcomes-based RSAs to be adopted in the United States. To illustrate the basis of this position, selected comments and quotations from interviews are shown in Figures 1, 2, and 3, and are organized to represent comments ranging from more optimistic, to cautious, to more pessimistic. Roughly half of manufacturers and payers expressed interest in outcomes-based RSAs and see value in their use; almost all were optimistic about the use of financial-based RSAs. In general, due to the difficulty in implementing and executing outcomes-based RSAs, interviewees indicated an interest in more financial-based RSAs (eg, utilization or financial capitation) but less interest in clinical and health outcomes-based RSAs.

Outcomes-based agreements, while attractive to some, were perceived by interviewees to be difficult to execute and as having high transaction costs. Interview respondents were skeptical about being able to use outcomes-based RSAs, citing challenges in implementing and executing outcomes-based RSAs that would mitigate their potential in the United States, particularly given the fragmented payer system with patient movement across plans, as well as the current lack of data infrastructure that limits feasibility and, to some extent, interest in measuring long-term outcomes. Interview respondents indicated that simpler, financial-based agreements, on the other hand, have had demonstrated success. The survey responses corroborated the general support for financial-based RSAs seen in the interviews: 80% of respondents stated that the use of financial-based RSAs in the United States would grow.

Reasons to Use RSAs

The interviews suggest that the perceived value of an RSA versus a traditional discount mechanism depends on the product, disease area, and availability of the necessary data infrastructure. The manufacturers we interviewed stated that they would use RSAs as a way to differentiate and demonstrate the effectiveness of their product versus competitors. RSAs are typically not used in a US setting when a product is first to market or the market leader, but are more attractive to manufacturers when there is competition. The manufacturer seeks to secure beneficial formulary placement or gain market share. They can also be a mechanism to increase patient compliance.

Based on our interviews, US payers leverage—or would like to leverage—RSAs as a way to reduce uncertainty about a product's clinical value, performance, or budget impact, as they allow payers and patients to gain experience with the medication. As payers and regulators are often interested in different types of data, at the time of launch, a product's impact on costs and comparative effectiveness is often not well understood. Ultimately, payers expressed interest in RSAs as they allow payers to ensure that the price of a drug is more closely aligned to its value. During our interviews, payers indicated interest in RSAs for products that are more costly (eg, specialty drugs, biologics, combination products) and for disease areas for which cost consequences are substantial. Payers are not interested in engaging in RSAs for undifferentiated products where there is no real or perceived advantage.

Barriers to Using RSAs

Figure 4 lists potential barriers to implementing RSAs that were identified during the interviews. The implementation and execution of RSAs is perceived to present a significant administrative burden that requires a substantial time investment. Further, outcomes-based agreements require significant payer and provider infrastructure: if payers lack the databases required to track individual patients, outcomes-based RSAs are difficult. Additional challenges that were mentioned include a) the need for adequately trained staff, b) the risk to pharmaceutical companies associated with being responsible for outcomes when they cannot control the way a drug is prescribed or used, c) the management of consequences in terms of changing preferred drugs and denying coverage for drugs, and d) the identification of outcomes that are meaningful but measurable within a reasonable time frame.

We tested the barriers identified during the interviews with a broader audience in the survey by asking respondents to rate and rank from the list of barriers provided in Figure 4 and any additional barriers not listed. The interview themes were consistent with the top barriers identified in the survey (Figure 5). The significant additional effort associated with RSAs was selected as the number 1 barrier to the use of RSAs in the United States by 33% of respondents and as the number 2 barrier by 27% of respondents. Inadequate data infrastructure was cited as the number 1 barrier by 27% of respondents and as the number 2 barrier by 33% of respondents. Other barriers that were selected as one of the top 3 hindrances to conducting RSAs in the United States included: federal (Medicaid) best price (40% ranked this as one of the top 3 barriers), significant resources/costs of adjudication (40%), challenges in measuring relevant outcomes (27%), and difficulty in reaching contractual agreement (27%).

Logistical Considerations

While not necessarily barriers, there were several logistical challenges highlighted during our interviews that may

Figure 1. Fifteen Optimistic Comments and Quotes About RSAs

1. Anticipate more agreements with ACOs: look for ways to demonstrate that a product reduces costs—eg, second admit—because there will not be payment for avoidable visits. *(Health Economics & Outcomes Research Organization, Pharma Company)*
2. RSAs are a way for pharmaceutical companies to show their belief in product and commitment to the value they are going to bring to patients and payers. *(Health Economics & Outcomes Research Organization, Pharma Company)*
3. We routinely assess whether an RSA would be useful for every product coming through the pipeline. *(Health Economics & Outcomes Research Organization, Pharma Company)*
4. RSAs for products with outcomes that are near-term have worked well. US payers are not interested in agreements greater than 18-24 months; prefer short time horizons (<12-18 months). *(Health Economics & Outcomes Research Organization, Pharma Company)*
5. For hepatitis C, we intend to try to put these agreements in place for the superior products. There seem to be a couple of front runners. If the other products are without agreements, they will be disadvantaged from a coverage perspective. *(US Payer)*
6. We need the risk-adjustment system over-hauled. Plans with sicker patients get more money. Is the government willing to do risk adjustment for specialty drugs? Solves the problem of worrying about how many high-risk patients you get. *(US Payer)*
7. A lot of these agreements are in process. Currently don't have any agreements in place that are based on medical/clinical outcomes, but are working toward agreements of that nature. *(National Medical Director, US Payer Organization)*
8. We have shared savings contracts with device makers in development, and have recently implemented coverage with evidence development programs for drugs that would otherwise not be a covered benefit. Our purpose will be to develop the economic data to make a coverage decision in the future. *(National Medical Director, US Payer Organization)*
9. These [RSA] agreements are becoming prevalent. In the small molecule area, generics have created a lot of issues for branded products because payers have been pretty successful in promoting generics; for this reason, pharmaceutical companies are looking for better ways to contract. *(National Medical Director, US Payer Organization)*
10. "If somebody can help reduce risk, take some of the variability out of the equation, or can actually help you manage some of those medical costs, then that's very attractive and that's more attractive than just getting a discount." *(National Medical Director, US Payer Organization)*
11. "It allows us to actually get experience using the medication or our members using the medication but it takes some of the risk off us." *(National Medical Director, US Payer Organization)*
12. "With these new regulatory pathways we don't think that all of our questions are getting answered, so a risk-based contract for a new pharmaceutical that's entered through an alternative pathway might be a way to get it covered for the pharmaceutical firm and a way for us to get experience with it without having to take the entire plunge." *(National Medical Director, US Payer Organization)*
13. In the US market, there is the ability to have differentiated prices and to negotiate on a plan-by-plan basis. This is a favorable environment for RSAs. *(EU Pharma Company)*
14. Medicare 5-star bonus structure is important in this market: payers get stars according to meeting different quality measures (Medication Possesson Ratio for 3 oral categories: hypertension, cholesterol, diabetes). They can receive a significant bonus to increase compliance in 3 categories ($14 PMPM). Potential win-win-win. Product with ease/convenience, believe you can improve compliance significantly. *(US PBM)*
15. A lot hinges on the Affordable Care Act. "ACOs could evolve in an interesting way. If in fact systems of care and payment reform change, if the ACO concept catches on, if there are more and more integrated delivery networks, risk-bearing entities could change the landscape and make risk-sharing a much more appealing proposition, particularly if we are able to get past some of the constraints from both the compliance side of things as well as the best price issues." *(Pricing & Payer Strategy, Pharma Company)*

ACO indicates accountable care organization; PBM, pharmacy benefit manager; PMPM, per member per month; RSA, risk-sharing agreement. Text not in quotation marks is a paraphrasing that attempts to capture the spirit of the interviewee's response to a specific question.

be a consideration for both payers and pharmaceutical companies when evaluating opportunities for RSAs. The number of agreements in place with a given payer or for a given class of drugs is a key consideration. While payers expressed interest in having multiple agreements for competing products, pharmaceutical manufacturers indicated this becomes a complex arrangement that they are reluctant to enter. Further, only large payers are likely to have the capability for multiple, simultaneous agreements, given the burden of negotiation, data collection, monitoring, and adjudication of RSAs. Another key consideration is duration, as payers and manufacturers agree that short-term deals are not desirable given the considerable investment in evidence development. On the other hand, they state that long-term deals are also not desirable given the costs and risks involved. Both parties acknowledge that medium-term deals (18-36 months) are the right balance trading off the sizable up-front investment and a preference to execute agreements reasonably fast.

What Works

Access to clinical data was stated as a key barrier to the use of RSAs; scenarios where clinical data are more readily available make RSAs more feasible. For instance, interviewees in-

Figure 2. Fifteen Cautious Comments and Quotes About RSAs

1. Have had discussions but no deals in place. *(Pricing & Payer Strategy, Pharma Company)*
2. "Payers can be fairly arbitrary—they back-calculate into the rebate they want. If you start with that assumption, it's a slippery slope in terms of what construct of a deal makes any clinical sense. This is not just a deal to back-calculate the rebate, it's a deal that has to be grounded in evidence, and if in fact your evidence doesn't fit up with what the payer wants, it's a challenge." *(Pricing & Payer Strategy, Pharma Company)*
3. "It's a challenge in the US because of some of the same reasons as in Europe, and then quite different ones. One of the things that transcends all payers is the desire for a more immediate rebate or payback, and so it makes risk-sharing less attractive because of the somewhat definitionally longer time between when they do the deal and when they get their money." *(Pricing & Payer Strategy, Pharma Company)*
4. Not all payers have the capability to execute these agreements. Therefore, it becomes a mixed model where some payers interested and have the infrastructure to do this, while others continue to leverage regular reimbursement approaches. *(Health Economics & Outcomes Research Organization, Pharma Company)*
5. There may be more potential in the ACO environment, but it will depend on what the ACOs look like and how pharmaceuticals are valued in the context of those organizations. *(Health Economics & Outcomes Research Organization, Pharma Company)*
6. The only way these will get done in the United States is if payers grab the ball on this and push it across the goal line. Don't think there is a lot of incentive for manufacturers to truly do this the way we've been talking about, to want to come to the table and truly hold themselves accountable to end points and doing what the products say. *(US Payer)*
7. I have spoken to manufacturers who will agree to an RSA as long as patients are compliant on their medicine. Manufacturers don't get the right to declare that, because that's a risk we need to co-create and co-own. *(US Payer)*
8. Getting the data is not easy, but it is doable, at least in the places where we have integrated medical systems. PDPs, drug-only benefits are challenging because don't have access to those data by law. *(US Payer)*
9. "A lot of times the Medicaid best price is too high to make the risk transfer to them [manufacturers] work. They are not taking enough risk in other words." *(National Medical Director, US Payer Organization)*
10. "The biggest challenge is measuring the outcomes and finding a fair way to get to the right outcomes or to make the connection between the outcome and the pharmaceutical firm. The drug has to be used in the right way and has to be the primary determinant of the outcome." *(National Medical Director, US Payer Organization)*
11. "I think it's a longer transition than that. There are therapeutic areas and drugs where outcomes-based or economic risk-based contracts will make sense. And there are some situations in which they will make sense for certain kind of providers: ACOs, patient-centered medical homes. And there are some situations—particular drugs or a particular disease state—where it's never going to happen because you really don't think the overall costs are going to change. I don't think one model is going to replace everything." *(National Medical Director, US Payer Organization)*
12. "The concept of ACOs is having more impact than the ACOs themselves. At this point, they are still getting organized. They really aren't spending that much time thinking about pharmaceuticals except they want to make sure patients use a lot of generics. I think it's going to be awhile before they really get it together and figure out what they want to do in terms of pharmaceuticals generally. They will be in the same position health plans are. It's not just about managing pharmaceutical costs, it's managing the pharmaceuticals to get the overall costs to the right place." *(National Medical Director, US Payer Organization)*
13. "If you're the first out in a new class where there is no competition, in the United States you don't need a risk-sharing agreement as long as you have good, comparative data." *(US PBM)*
14. "All this sounds good until you start looking at the real-world data and start seeing the real-world implication of this guarantee, what's the gap we're trying to solve here and why is it so much better with this guarantee." *(US PBM)*
15. Currently, ACOs are busy getting established and learning how to take risks; it is too early to engage them in RSAs. At this point, they are too small and distracted with implementing IT, systems, etc. There is potential in 3 to 5 years. *(US PBM)*

ACO indicates accountable care organization; IT, information technology; PDP, prescription drug plan; PBM, pharmacy benefit manager; PMPM, per member per month; RSA, risk-sharing agreement.
Text not in quotation marks is a paraphrasing that attempts to capture the spirit of the interviewee's response to a specific question.

dicated it may be easier to measure health outcomes for drugs that are administered in settings where there are more immediate clinical data available (eg, the hospital setting), where drugs are administered in person, or when data are already being collected as part of ongoing processes (eg, performance measurement via the Healthcare Effectiveness Data and Information Set). Complex outcomes might be more easily measured where there is an active provider (eg, patient-centered medical homes). Similarly, interviewees stated outcomes-based RSAs can be most successful where the infrastructure is robust to collect such data, such as in single payer or closed settings including integrated delivery networks. Drug cost as a share of total episode cost can also be a factor.

A key success factor is the need for manufacturers to understand the amount of risk they are undertaking when entering into an RSA. Manufacturers feel they should be able to reasonably predict plausible outcomes of the agreement and assess the level of risk; for example, what level

Figure 3. Fifteen Pessimistic Comments and Quotes About RSAs

1. "Setting up individual agreements with all these individual players, and without the benefit of large populations, economics of scale, or large data sets, it is very difficult to enact a financial agreement that makes sense without a straightforward rebate. Or if you try to get into the more complicated clinical outcomes-based agreements, payers just aren't sophisticated enough at this point to have the kind of databases and track and follow patients with enough time to be able to make those agreements reasonable. It's a combination of the fragmentation of the market but it's also a very fluid market as well." *(Pricing, Reimbursement & Market Access Organization, Pharma Company)*
2. "Pharma doesn't necessarily want to take on the risk of not necessarily being able to control the way the drugs are being prescribed or used but still be responsible for an outcome." *(Pricing, Reimbursement & Market Access Organization, Pharma Company)*
3. "For the most part, the US market doesn't have the level of data and the continuity of data to be able to follow through a lot of these kinds of agreements in a way that makes pharma not be solely responsible for things that are beyond our control." *(Pricing, Reimbursement & Market Access Organization, Pharma Company)*
4. Have seen some other payers' agreements. These are interesting but didn't get to the core of outcomes/end points. At a minimum, it is a good marketing strategy from a commercial line of business perspective. But didn't hit the point of talking about outcomes or pay for performance. *(US Payer)*
5. Manufacturers will go to payers from time to time; but the complexity involved in these agreements ends up killing the conversation. *(US Payer)*
6. There is a need for government support. For Medicare, if you have a specialty tier, can't take a drug above $600 out of the specialty tier. Even without the law, it would not be in the plan's best interest—the government premium is insufficient to allow preference for those drugs and the possibility of causing positive selection such that people taking the high-cost drug will pick your plan. *(US Payer)*
7. There is inertia with manufacturers; they like the theory, but the desire to take accountability is limited. *(US Payer)*
8. Have had a couple of agreements converted from outcomes-based contract to good rebate because of best price issues. *(National Medical Director, US Payer Organization)*
9. If you start an RSA with the third drug entering the market, you may lose the rebate agreement with first 2 drugs because they don't want to be co-preferred. *(US PBM)*
10. There has been a lot of conversation about RSAs, but they are not always done or are not published. "I can't even remember not having a conversation in the last 10 years with a manufacturer about the notion [of] what could it look like. And then it always fell apart when it started getting into the measurement." *(US PBM)*
11. "Beyond the basic price protection, etc, if you're talking more complicated financial risk-shares or outcomes-based risk-shares, very few payers are interested." *(Managed Markets, US Pharma)*
12. "Because there are so many factors that go into it, it's very, very hard to do an outcomes contract in diabetes or any condition that's multifactorial. You really would need to look at conditions where it either works or it doesn't and there are no confounding factors." *(Managed Markets, US Pharma)*
13. Payers think access rebates are easier, and there is less work for manufacturer and customer. *(Managed Markets, US Pharma Company)*
14. For large PBM customers, their business is predicated on administrative fees—they don't have direct access to medical data that is necessary for an outcomes study. For them, the simplest model is very transactional. You're removing a huge chunk of US pharmaceutical sales when you take PBMs out of the equation—they're at least two-thirds of the business. *(Managed Markets, US Pharma Company)*
15. [ACOs] Make things complicated. There are a number of ACOs that contract with the payer to administer their formularies, but they take on no risk. Our perspective would be that a direct contract with an ACO is outside of the discounts they harbor because they are not operating under any risk in terms of the pharmacy benefit. *(Managed Markets, US Pharma Company)*

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of compliance is required and to what extent the clinical trial population differs from the real-world population.

What Doesn't Work

There are a few scenarios that are considered prohibitive to successful RSAs. Both parties indicated that trust is a critical component of agreements between pharmaceutical companies and payers. It is imperative that both parties trust the data: clear agreements on data validation and analysis are important to create trust in the data.

Certain types of agreements can also be problematic. For instance, manufacturers expressed that population-based agreements are perceived to be risky because there are many unknowns around compliance, prescribing, and so forth. Manufacturers are reluctant to take on risk when they cannot predict how their product will be used in the population. Agreements that involve the manufacturer paying for nonpharmaceutical costs are difficult to execute, as payers do not generally have the systems or data to support such agreements. Both parties shy away from agreements in disease areas where there are many different treatment paradigms or the relevant outcome is an intermediary outcome, because it can be challenging to attribute the outcome to the product in question.

Figure 4. Potential Barriers to RSA Use in the United States

1. Significant additional effort required to establish/execute RSAs (eg, compared to traditional rebates/discounts)
2. Challenges in identifying/defining meaningful outcomes
3. Challenges in measuring relevant real-world outcomes
4. Data infrastructure inadequate for measuring/monitoring relevant outcomes
5. Difficulty in reaching contractual agreement (eg, on the selection of outcomes, patients, data collection methods)
6. Implications for federal (Medicaid) best price
7. Payer concerns about adverse patient selection
8. Fragmented multi-payer insurance market with and significant patient switching among plans
9. Challenges in assessing risk upfront due to uncertainties in real-world performance
10. Lack of control over how product will be used
11. Significant resources and/or costs associated with ongoing adjudication

RSA indicates risk-sharing agreement.

DISCUSSION

Future Prospects: Barriers, Health Reforms, and Policy

This study confirms that very few private sector RSAs have been implemented in the United States; while payers and pharmaceutical companies remain interested in these types of agreements, various challenges restrict the types of situations in which they can be feasibly employed. Also, because of the effort required, payer and pharmaceutical respondents are less enthusiastic about outcomes-based agreements and more optimistic about simpler, financial-based agreements.

Although the global growth in RSAs seems to have slowed, several countries maintain robust programs, particularly Italy and Sweden, and interest is growing in Latin America and Asia. There has also been a broad shift to financial-based agreements for several reasons. Our research suggests that public payers, especially outside the United States, are attracted to making arrangements with manufacturers, first and foremost, to obtain an effectively lower price in a world where reference pricing and the prospect of parallel trade make differential (or tiered) pricing difficult. And, of course, this can benefit patients by providing earlier and broader market access. Second, few countries have an adequate data infrastructure to operate outcomes-based schemes efficiently.

The history of RSAs in the United States is dominated by the Medicare experience with CED. Although the interest remains, it seems to have waned. The growth in visible private RSAs in the United States seems to have stalled, but our interviews suggest growing interest and some new activity. There are multiple barriers in the United States—most prominently, the additional effort needed to negotiate and maintain these agreements relative to traditional rebates. The challenge of measuring individual-level outcomes in a claims-oriented data infrastructure makes operationalizing RSAs difficult because of the lack of relevant clinical outcomes contained in these data. Medicaid best price (and the similar 340B Drug Pricing Program) provisions, which link mandated discounts for public sector programs to private sector prices, are considered complications, but would seem to be manageable in a well-designed contract; notwithstanding, several interviewees regarded this as a major barrier.

If a medicine covered by an RSA does not perform as anticipated by the manufacturer and a price discount ensues, the reduced prices could be perceived as a new best price and made available to all Medicaid purchasers. In the case of Medicare, we were told that provisions around the Medicare 5-star rating program, which ties payment to achievement of certain quality metrics in health plans, have made adherence programs—a kind of outcomes-based program—attractive to payers. The preference for a short-term horizon of 18 months to 3 years being the most practicable arrangement in the US private health system, because it reduces long-term liability for manufacturers and payers, and limits the feasibility of RSAs in some disease areas.

Our interviewees were generally pessimistic as to whether the growth of ACOs would have any positive impact in the near term, since many ACOs are in the initial development and market experimentation phase[11]; however, they may well bring a new orientation to risk-sharing that will support the future development of pharmaceutical RSAs.

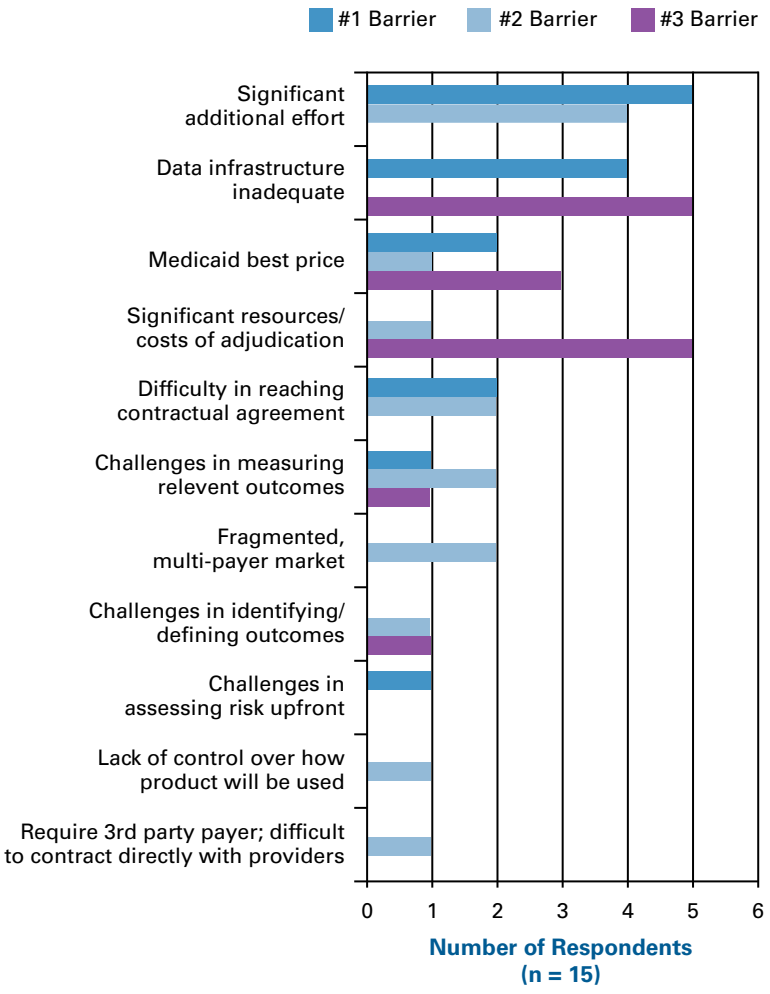
The ISPOR PBRSA Task Force emphasized that better information about what works in medicine is a global public good,[1] but it still appears that the RSAs being developed in the United States will be confidential ones, with the knowledge gained only shared inadvertently or sporadically, rather than by design. Both payers and manufacturers have competitors, and they have some incentive to be "free riders" rather than developing innovative pricing schemes that yield knowledge on best medical practice. One of our payer interviewees—a national medical director for a major US payer—made this revealing and somewhat encouraging comment:

"If we learn something about a pharmaceutical by looking at our own data and we decide that we are going to use that information to make coverage determinations, it is our policy that we make that public (publish in a peer-reviewed journal). If the coverage decision is based on an economic analysis, that may be kept private; however, if the decision is based on something clinical, it has to be available for physicians and patients to evaluate."

This suggests an important dichotomy between clinical and economic evidence, though they clearly become related when combined in a cost-effectiveness ratio or other assessment of value. It is not clear if this distinction is sustainable given that the recent joint American Heart Association and American College of Cardiology Task Force on Value and Cost called for consideration of cost-effectiveness in establishing clinical treatment guidelines in cardiology.[12]

The growing emphasis on comparative effectiveness research in the United States, including the establishment of the Patient-Centered Outcomes Research Institute,[13] are testimony to the fact that the country is investing more in generating real-world evidence on clinical outcomes and their related economic implications. In principle, RSAs will generate some of this information, but if the results remain confidential, the new knowledge will, at best, slowly diffuse through a healthcare system that aspires to become a "learning healthcare system." This raises the question of the need for greater public policy intervention. The following question was not part of our remit in this study, but we should be asking: Are there other policy tools or incentives that could be used to encourage and leverage US private sector RSAs to reveal their lessons for the greater good of the system and all patients?

Figure 5. Survey Findings of Top Barriers to the Use of RSAs in the United States



RSA indicates risk-sharing agreement.

Limitations

There are a number of limitations to this study. First, there is the possibility that the database reviewed may not include the entirety of RSAs due to the confidential nature of agreements, resulting in an underestimation of the total number of RSAs in the United States. However, our interviews did not suggest a significant growth in the number of RSAs that would contradict the findings of the database review.

Additionally, the interviews and survey had a relatively small sample size, and we leveraged a convenience sample for both approaches; as a result, there is the possibility that our findings may not be generalizable to the entire US market. We attempted to mitigate this risk by including individuals from a number of organizations that might bring varying perspectives.

Due to the confidential nature of agreements and limited number of agreements in the United States, we have few data points to learn from. Thus, it remains to be seen how some of the challenges and opportunities identified will be realized in actual practice.

Finally, we sought to assess the potential for increased use of RSAs in the United States given the shifting incentives in the healthcare system with healthcare reform and the growth of ACOs. However, many of the forthcoming changes have yet to take effect, and until these have been fully implemented, it is challenging to assess whether RSAs may in fact have a great role in the future.

CONCLUSIONS

In summary, there is continued and even growing interest on the part of both manufacturers and payers in RSAs in the United States. Yet, the number of new agreements is still small—mostly exceptional situations. We can ask, "What are the necessary conditions for RSA adoption in the United States?" and the answer seems to be that more robust infrastructure and optimized RSA development and monitoring processes are needed. There is a lot of talk, but improved data systems and changed incentives (via health reform and ACOs) may generate more action.

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REFERENCES

1. Garrison LP Jr, Towse A, Briggs A, et al. Performance-based risk-sharing arrangements-good practices for design, implementation, and evaluation: report of the ISPOR Good Practices for Performance-Based Risk-Sharing Arrangements Task Force [published online July 10, 2013]. *Value Health*. 2013;16(5):703-719.

2. Performance-Based Risk-Sharing Database. University of Washington website. https://depts.washington.edu/pbrs/index.php. Accessed January 2014.

3. Carlson JJ, Gries KS, Yeung K, Sullivan SD, Garrison LP Jr. Current status and trends in performance-based risk-sharing arrangements between healthcare payers and medical product manufacturers. *Appl Health Econ Health Policy*. 2014;12(3):231-238.

4. de Pouvourville G. Risk-sharing agreements for innovative drugs: a new solution to old problems? *Eur J Health Econ*. 2006;7(3):155-157.

5. Hutton J, Trueman P, Henshall C. Coverage with evidence development: an examination of conceptual and policy issues. *Int J Technol Assess Health Care*. 2007;23(4):425-432.

6. Carlson JJ, Sullivan SD, Garrison LP, Neumann PJ, Veenstra DL. Linking payment to health outcomes: a taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. *Health Policy*. 2010;96(3):179-190.

7. McCabe CJ, Stafinski T, Edlin R, Menon D; Banff AED Summit. Access with evidence development schemes: a framework for description and evaluation. *Pharmacoecon*. 2010;28(2):143-152.

8. Towse A, Garrison LP Jr. Can't get no satisfaction? will pay for performance help? toward an economic framework for understanding performance-based risk-sharing agreements for innovative medical products. *Pharmacoecon*. 2010;28(2):93-102.

9. Carlson JJ, Garrison LP Jr, Sullivan SD. Paying for outcomes: innovative coverage and reimbursement schemes for pharmaceuticals. *J Manag Care Pharm*. 2009;15(8):683-687.

10. Neumann PJ, Chambers JD, Simon F, Meckley LM. Risk-sharing arrangements that link payment for drugs to health outcomes are proving hard to implement. *Health Aff (Millwood)*. 2011;30(12):2329-2337.

11. Douven R, McGuire TG, McWilliams JM. Avoiding unintended incentives in ACO payment models. *Health Aff (Millwood)*. 2015;34(1):143-149.

12. Anderson JL, Heidenreich PA, Barnett PG, et al. ACC/AHA statement on cost/value methodology in clinical practice guidelines and performance measures: a report of the American College of Cardiology/American Heart Association Task Force on Performance Measures and Task Force on Practice Guidelines. *J Am Coll Cardiol*. 2014;63(21):2304-2322.

13. Mestre-Ferrandiz J, Deverka P, Pistollato M, Rosenberg E. The current drug development paradigm: responding to US and European demands for evidence and comparative effectiveness and relative effectiveness. Center for Medical Technology Policy website. http://www.cmtpnet.org/docs/resources/Current_Drug_Development_Paradigm_Mestre-Ferrandiz_2014.pdf. Office of Health Economics and Center for Medical Technology Policy Occasional Paper. Published April 2014. Accessed December 11, 2014. ■

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eAppendix A. Interview Guide

Thank you for agreeing to speak with us regarding your experiences with and perspectives on risk-sharing agreements (RSAs) for pharmaceuticals and other medical products. Our objective is to gain an understanding of the types of agreements that have been used to date, the purpose for which these agreements have been employed, what has worked and what hasn't, and how these arrangements are evolving.

We will be taking notes during the interview, and with your permission we would also like to record the session. This helps ensure our notes are accurate and that we capture all of the themes that you discuss; all recordings will be deleted after completion of thematic review. You and your organization will not be identified with individual responses in any of the public presentations that we make about this project.

Questions:

1. How do you define RSAs?

2. Please describe your experience and/or your organization's experience with RSAs to date.

3. Based on your/your organization's experience with RSAs:
    a. For what types of medical technologies have RSAs been used (pharmaceuticals, biopharmaceuticals, medical devices, diagnostics)?
    b. What types of RSAs are used?
    c. What is the primary goal of the RSAs that have been employed?
    d. Are these arrangements linked to other mechanisms that facilitate the appropriate use of the technology (e.g., tiered drug formularies, prior authorization requirements)?
    e. How are outcomes selected? What drives the selection of broad vs. narrow outcomes?
    f. What is the timeframe for these arrangements?
    g. Who is responsible for administering these arrangements?
    h. Who initiated these RSAs?

4. How effective have the RSAs you have experience with been?
    a. In your estimation have the arrangements been successful?
    b. What has worked well?
    c. What has not worked well?
    d. What challenges are involved in implementing RSAs?

5. What factors drive the value of a RSA?

6. What time horizon do you consider most meaningful for the implementation of RSAs?

7. What are key considerations when developing an exit strategy for an RSA?

8. Do you believe that RSAs have been successful in other organizations? Why or why not?

9. How do you see the use of RSAs evolving in the future? Do you think their use will increase or decrease?
    a. Globally?
    b. In the U.S.?
10. Do you have any contacts that you believe it would be useful for us to speak with about this topic?

eAppendix B. Web Survey

This brief survey is being conducted on behalf of the National Pharmaceutical Council by VeriTech Corporation to understand opinions about the use of risk-sharing agreements (RSAs) in the United States.

Our objective is to gain an understanding about how these arrangements are evolving and expectations for their use in the future. We are specifically interested in the use of these agreements in the U.S. private health insurance market.

All responses will be kept confidential, and findings will be reported in aggregate only.

Thank you for your participation!

1. In which segment of the healthcare industry are you currently employed?
Pharmaceutical/Biotech Company
- o Commercial Payer
- o Government Payer
- o Pharmacy Benefit Manager (PBM)
- o Accountable Care Organization (ACO)
- o Other (please specify)

2. Please indicate whether you have had any direct experience with RSAs. (Select all that apply)
- o Experience assessing the feasibility or potential for RSAs
- o Experience designing RSAs
- o Experience implementing RSAs
- o No direct experience with RSAs

3. Please indicate whether your experience with RSAs has been with pharmaceuticals or medical devices. (Select all that apply)
- o Pharmaceutical products
- o Medical devices
- o N/A - I do not have direct experience with RSAs

4. In your opinion, how will the use of financial risk-sharing agreements (RSAs) in the U.S. private market change in the next 5-10 years?
- o The use of financial RSAs will grow.
- o The use of financial RSAs will remain constant.
- o The use of financial RSAs will decline.

5. In your opinion, how will the use of outcomes-based risk-sharing agreements (RSAs) in the U.S. private market change in the next 5-10 years?
- o The use of outcomes-based RSAs will grow.
- o The use of outcomes-based RSAs will remain constant.
- o The use of outcomes-based RSAs will decline.

6. The following factors have been identified as potential barriers to the use of risk-sharing agreements. Please rank the extent to which you believe each factor is a barrier to the use of RSAs in the U.S.

	Not a Barrier	Minimal Barrier	Moderate Barrier	Major Barrier	Almost Prohibitive Barrier	Unsure
A. Significant additional effort required to establish/execute RSAs (e.g., compared to traditional rebates/discounts)	O	O	O	O	O	O
B. Challenges in identifying/defining meaningful outcomes	O	O	O	O	O	O
C. Challenges in measuring relevant real-world outcomes	O	O	O	O	O	O
D. Data infrastructure inadequate for measuring/monitoring relevant outcomes	O	O	O	O	O	O
E. Difficulty in reaching contractual agreement (e.g., on the selection of outcomes, patients, data collection methods)	O	O	O	O	O	O
F. Implications for Medicaid best price	O	O	O	O	O	O
G. Payer concerns about adverse patient selection	O	O	O	O	O	O
H. Fragmented, multi-payer health insurance market with significant patient switching	O	O	O	O	O	O

I. Challenges in assessing risk upfront due to uncertainties in real-world performance	O	O	O	O	O	O
J. Lack of control over how product will be used	O	O	O	O	O	O
K. Significant resources and/or costs associated with ongoing adjudication	O	O	O	O	O	O

7. If there are additional barriers to the use of risk-sharing agreements in the U.S. private health insurance market that were not noted above, please list them below:

L. _____
M. _____
N. _____

8. Please rank the top 3 barriers to the use of RSAs in the U.S. private market.
Use the letter associated with each barrier in Questions 6 and 7 to indicate your rankings (e.g., enter "A" in the first row if you believe the significant additional effort required to establish/execute RSAs is the top barrier).

#1 Barrier _____
#2 Barrier _____
#3 Barrier _____