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Defining and Managing High-Priced Cures: Healthcare Payers' Opinions

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ABSTRACT

Objectives: Recent regulatory approvals of potentially curative but high-cost treatments have made these therapies a focus of health policy discussions. Cures present new challenges to healthcare payers because they have high upfront costs but have life-long health benefits. The objectives of this study are to understand how healthcare payers define and manage cures. We investigated payers' views on key features of curative treatments and the affordability and value challenges they present.

Methods: We conducted semistructured interviews in 2016 with key informants in US payer organizations. Interviewees were directly involved in coverage determination for highly effective and curative therapies.

Results: We contacted 24 individuals and 18 participated. When asked what aspects of cures were important for coverage determination, an equal percentage of respondents (61% each) mentioned clinical and economic factors. In defining a cure, half of respondents included an economic element such as no downstream costs associated with the disease. When asked about challenges, 72% of respondents mentioned uncertainty regarding long-term outcomes and 56% mentioned membership churn and competition.

Conclusions: Payers expressed a novel definition of a cure—which we call a "healthcare cost cure"—that captures both the clinical and economic consequences of treatment. This definition may be more pertinent in fragmentary financing systems that unevenly distribute cure costs and benefits across payers. Overall findings indicate that decision makers desire evidence to ensure that the long-term real-world consequences of covering cures match the expected benefits. Future policies need to balance upfront acquisition costs with downstream financial benefits.

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Introduction

Recent regulatory approvals of several highly effective, potentially curative, but high-cost treatments have brought these therapies to the forefront of health policy discussions. Some treatments, such as the new generation of direct-acting antiviral therapy for hepatitis C, are major advances in both efficacy and tolerability from previously available treatments for a large population. Others such as the chimeric antigen receptor (CAR) T-cell therapies or gene therapies also represent innovative mechanisms for treating conditions that have the potential to be applied to many indications.[1–3] Further, based on the passage of the 21st Century Cures Act, the Food and Drug Administration (FDA) is designing several expedited development and review programs for regenerative medicine and advanced therapies, thereby further increasing the potential for a substantial number of new products to enter the medical marketplace with the ability to "cure" diseases.[4–8] Indeed, according to one recent industry estimate, there were 79 phase 3 clinical trials underway globally for such therapies.[9]

The list prices for the marketed curative treatments are often high as evidenced by the suggested $475 000 list price for the CAR T-cell therapy, tisagenlecleucel for acute lymphoblastic leukemia,[10] and $850 000 for the gene therapy voretigene nepavarac for inherited retinal disease.[11,12] When combined with highly prevalent conditions or expanding indications, these high prices can have substantial effects on overall health spending. For example, Medicare Part D and Medicaid spending (before confidential rebates) on 2 hepatitis C treatments in 2015 was $11 billion dollars or 6% of total drug spending by these organizations.[13] Another challenging aspect of potentially curative treatments is their uneven distribution of costs over time relative to benefits, with a somewhat unique feature of a large upfront cost, followed by health benefits that are accrued over the patient's full lifespan. Public and private health systems more commonly deal with treatments for acute and chronic conditions or low-cost vaccines

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Figure 1. Semistructured interview guide used for all interviews.

Section 1: Defining cures
I would like to start by discussing highly effective or curative therapies for severe diseases. 1. Can you think of any examples of curative therapies? 2. How do you define a cure? 3. What are the aspects of cures that are important for payer decision-making?
Section 2: Paying for cures
I would now like to discuss how you have handled and/or plan to handle paying for cures and other highly effective but often expensive therapies. 1. You previously gave some examples of therapies that you think are cures. In many cases, patients may enjoy the benefits of treatment for a long time, yet the cost is often upfront with the budget impact typically highest in the near-term. In this context, what are the unique challenges to paying for cures and other highly effective but expensive treatments? 2. Has your organization enacted or discussed any specific policies to deal with these challenges? 3. If you have discussed any alternative financing options: What are the pro's and con's to the alternative financing options considered?

that produce prompt health impacts, but not "one-time cures." This difficulty was illustrated in the case of alipogene tiparvovec—the first gene therapy treatment available in the Western world. It was indicated for a rare lipid storage disease and was approved for the European market with a price of approximately $1 million per patient.[14] The treatment was subsequently withdrawn by the manufacturer owing to low uptake, potentially because of the high upfront cost and uncertainty regarding long-term effectiveness.[14,15] Further, elucidating and defining what a cure is complicates matters. There are multiple definitions of a "cure" in the biomedical literature,[16–20] and it is unknown how US healthcare payers define a cure and what are the most salient factors to consider when faced with decisions about covering and paying for these products. The objectives of this study were to understand how healthcare payers define cures, their opinions regarding the key features of cures, the affordability and value challenges posed by these treatments, and how they manage these challenges.

Study Data and Methods

Participant Selection

We conducted in-depth, semistructured interviews between February and July 2016. Eligible participants were individuals in US payer or purchaser organizations who dealt directly with coverage determination for highly effective or curative therapies. Because many curative treatments approved to date have been covered under pharmacy benefits, we focused on recruiting interviewees who managed pharmacy benefits or both pharmacy and medical benefits. Potential interviewees were purposefully sampled to ensure inclusion of a broad range of organizations, including public and private insurance plans. We recruited contacts through our investigator network, with additional contacts gained through snowball sampling. We invited potential interviewees to participate via email with up to 3 follow-up attempts to solicit their participation. We continued to contact interviewees and conduct interviews until no new, relevant information emerged; at that point, we doubled the number of interviews that we had conducted to ensure theoretical thematic saturation.[21]

Interviews

All of the interviews were conducted by telephone, except one that was conducted in person. Each interviewee participated in a single recorded interview lasting approximately 1 hour. The interview was preceded by a survey to collect information on demographics (age, sex), position in company, educational attainment or credential, and plan characteristics (for profit, plan type, and plan size). The content of the interview itself was divided into 2 sections: (1) definition, examples, and key features of cures; and (2) affordability and value challenges and policies to handle the challenges. A single interviewer (KY) conducted each interview using a semistructured interview guide that was developed by the entire research team and pilot tested with 2 potential interviewees (not included in sample) to ensure comprehension (Figure 1). The interviewer was known to most interviewees as an academic researcher in pharmacoeconomics but had no ongoing clinical, operational, or financial relationship with interviewees.

Analysis

The interviews were professionally transcribed and uploaded into the qualitative analysis software, Dedoose version 8.0.35, a web-based qualitative analysis tool.[22] Transcripts were assigned an anonymous study ID before study analysis. We conducted the analysis using a modified grounded theory approach by continually allowing new data (ie, new transcripts) to revise our analysis (ie, collection of codes, subthemes, and major themes).[23,24] This method was appropriate for our study because we aimed to understand, from the payer's perspective, how various notions about cures were conceptualized without imposing an *a priori* framework. We developed a coding tree composed of major themes, subthemes, and codes in 3 phases. First, one investigator (K.Y.) developed multiple iterations of a working code dictionary by coding each new transcript line-by-line and then recoding previous transcripts based on the revised code dictionary. Revisions of the code dictionary may include additions, modifications, or deletion of codes, or merging of similar codes. This code dictionary was then discussed and revised by the entire research team. Second, each transcript was independently coded using this revised code dictionary by 2 members of the research team (ie,

Table 1. Payer interviewee characteristics (N = 18)

Individual Characteristics	
Age in years, mean (SD)	51 (14)
Male, n (%)	11 (61)
Position	
Pharmacy director, n (%)	7 (39)
Formulary manager, n (%)	4 (22)
Medical director/chief medical officer, n (%)	4 (22)
Pharmacy vice president, n (%)	3 (17)
Highest Education or Credential	
PharmD/RPh, n (%)	13 (68)
MD, n (%)	4 (21)
PhD, n (%)	2 (11)
Organization Characteristics	
Nonprofit, n (%)	10 (56)
Administration Type	
Health plan, n (%)	9 (50)
Pharmacy benefits management, n (%)	4 (22)
Purchaser, n (%)	3 (17)
Integrated health plan (delivery, insurer, and PBM), n (%)	2 (11)
Lines of Business*	
Commercial, n (%)	14 (78)
Medicare Part D, n (%)	15 (83)
Medicaid, n (%)	12 (67)
Other public,† n (%)	2 (11)
Beneficiary Size	
<1 million, n (%)	4 (22)
1 million to <2 million, n (%)	5 (28)
2 million to <5 million, n (%)	3 (17)
5 million to <10 million, n (%)	2 (11)
>10 million, n (%)	4 (22)

*Lines of business sum to greater than 100% because an organization may participate in multiple lines of business.
†Refers to public employees and Department of Corrections.

investigator triangulation; KY and KS), and any differences between coders were discussed and remaining discrepancies were adjudicated by a third member of the research team (JC). Revisions to the coding dictionary and recoding of transcripts were allowed to occur based on these discussions. Third, 2 members of the research team (KY and KS) grouped similar codes hierarchically into subthemes and major themes, and the entire research team discussed and revised the final coding tree. We used the consolidated criteria for reporting qualitative research (COREQ) checklist to ensure thorough reporting.[25] This study was approved by the Institutional Review Board at the University of Washington.

Results

Interviewee Description

We contacted a total of 24 individuals and interviewed 18, achieving a 75% response rate. Of the nonrespondents, 4 declined, citing time constraints, and 2 did not respond to email contact. The study participants were employees of plans that administrated benefits for a large population with wide variation in plan sizes from fewer than 1 million members to more than 10 million members (Table 1). Half of respondents held positions in organizations that administrated benefits for plans with 2 million or greater enrollees. Further, half of the interviewees worked for traditional health plans, and the remainder worked for pharmacy benefit management companies, integrated health plans, or purchasers that make coverage decisions. Of the respondents, 56% worked for nonprofit payers. Most of the respondents (78%) held pharmacy management positions, and the remainder held medical director or chief medical officer positions.

Definitions and Examples of Cures

Figures 2 and 3 present the hierarchical coding tree with the percentage of respondents endorsing each code. The majority of individuals defined a cure in clinical terms as having a sustained biologic response (83%) and requiring no further treatment (56%). One-half (50%) of interviewees also defined a cure in economic terms as eliminating the need for additional downstream costs. Regarding this concept, one respondent reported, "When I think of the term *cure*, I think that the patient doesn't have to take any more drugs, does not have to get any more medical care; is not gonna cost my health plan any more money." Similarly, one participant noted that treatments that required long-term monitoring would not be considered a cure, "So, most of us would say, economically, if you're perfectly healthy, but you have to get a $5000 scan once a year, for the rest of your life, that's not really—economically, that's not a cure."

Respondents most commonly named the newest generation of hepatitis C direct-acting antivirals (78%) as an example of a cure. The next 2 most commonly mentioned examples of cures (28% of respondents each) were antibiotics and cancer treatments—both of which, if administered early in the course of disease, can result in disease eradication and do not require further treatment. In contrast, 28% of respondents considered cancers that require continual treatment as not being cures. One interviewee described the reasoning for the classification as follows, "Let's take CML (chronic myelogenous leukemia) and the use of Gleevec (imatinib) and that class of drug, does that cure CML or just control it? I say 'control,' so I wouldn't call that a 'cure' … because it still requires that you still use a healthcare resource." Based on similar logic, 17% of respondents did not consider treatments for human immunodeficiency virus infections as curative. "When I think of medications, it would also mean to me that that medication wouldn't need to be taken long-term. So, it wouldn't be something where, for example, an HIV therapy where you take the therapy, and it suppresses your viral load, and you can essentially have no viral load. But in order to continue that, you would need to continue taking that therapy for the rest of your life. So it's really suppressing but not curing the disease."

Aspects of Cures Important for Payer Decision Making

Respondents mentioned with equal frequency the clinical (61%) and financial (61%) aspects of cures as important for decision making. The clinical aspects that were important included extension of life (50%), improvement in quality of life (39%), and requiring no further treatment (22%). The financial aspects that were important included incurring no additional costs for the disease (50%) and costs that represented good value for money (11%). Two participants' statements illustrate the importance of prevention of downstream costs as a result of cures: "We want to see the person healthy, with a normal expected future. And as I say, no significant downstream costs as a result." "What's important is, with a cure, you're hopefully avoiding downstream costs."

Challenges to Paying for Cures

Every interviewee mentioned affordability as a challenge to paying for cures. Respondents stated that pent-up demand, high prevalence, and high prices drive budget impact. Such budget effects can ultimately be returned to plan beneficiaries through higher premiums. "From a payer perspective, we try to predict actuarially what our premiums need to be to cover the cost to get the advances in technology, new treatments, and things like that.

Figure 2. Theme 1: Definitions, examples, and important aspects of cures.

Sub-themes	Percentage of Respondents Endorsing the Concept	Illustrative Quotes
1. Definitions		
a. Sustained biologic response	83%	"To me a cure is where you have no evidence of disease and you reach the point at which you've been free of disease long enough that there's a high probability that it's not coming back."
b. No further treatment	56%	"If it's a disease where they are requiring ongoing treatment, I wouldn't say it's a cure as much as controlling the disease."
c. No downstream costs	50%	"When I think of the term "cure," I think that the patient doesn't have to take any more drugs, does not have to get any more medical care; is not gonna cost my health plan any more money."
2. Examples considered cures		
a. Hepatitis C direct acting antivirals	78%	
b. Antibiotics	28%	
c. Cancer treatments leading to disease eradication and no further treatment	28%	"Cancer treatment where patients diagnosed with cancer and through drug therapies are eradicated, no detection of cancer cells, and go on to lead a normal life"
d. Vaccines	11%	
e. Gene therapy	6%	
3. Examples not considered cures		
a. Cancer treatments that require continual therapy	28%	"If you're looking at most oncology products, you stay on the medication until disease progression. I wouldn't necessarily define as a cure, more so holding the disease at bay."
b. Human Immunodeficiency Virus treatment	17%	
4. Important aspects of cures for payer decision making		
a. Clinical aspect	61%	
i. Life extension	50%	How much longer is this person expected to live? And that, in our mind, trumps the other concerns"
ii. Quality of life improvement	39%	
iii. No further treatment	22%	
b. Financial aspect	61%	"Overall reduction of medical costs, I think that's very important."
i. No downstream costs	50%	
ii. Costs that represent good value for money	11%	

It's difficult in this situation (with hepatitis C) to plan for this sort of a perfect storm because of pent-up demand and high cost."

A majority of interviewees mentioned uncertainty regarding long-term outcomes (72%) as a challenge. Interviewees questioned whether surrogate markers could reliably be used in place of outcomes of interest in clinical trials and whether these outcomes from clinical trials could project long-term cure without downstream clinical and economic sequelae. Regarding this, one participant mentioned, "You know, all clinical trials eventually have to end. And if we just have 2 years of data, there's uncertainty around, is this a lifetime cure? We don't know, right? I mean, it may look good. But what we know is the only data we have. So there's a lot of uncertainty there." Another participant described this differently, "So, if you're 50, and I cure you, that tells me something about what you look like till 57. It doesn't tell me what you're gonna look like at 77."

Interviewees also raised the concern that because of high upfront costs, long-term benefits, and the possibility of enrollees changing plans, it may not be in the plan's financial interest to cover cures if other plans are not covering them as well (56%). For example, if a certain plan covers a cure for an enrollee, the plan would expect to reap the clinical and economic benefits over the individual's life time; nevertheless, if the enrollee switches to a competitor's plan, the first plan would have paid for the cure entirely but not fully reap the benefits of that coverage. One respondent stated, "If a gene therapy's gonna cost $2 million to cure somebody, and you have a churn rate of a third of your people every year, well, why should Plan A pay for that when Plan B down the road doesn't have to pay—I mean, that's kind of stupid—they're kind of dumb arguments, but they're real." Another respondent stated, "If the premiums go up, it makes healthcare less affordable. And if other health plan's premiums don't go up, if they're more aggressive at managing how they keep their premiums down, they're gonna gain more membership."

Managing Cure Challenges

Plans have attempted to deal with the challenge of paying for cures by instituting utilization management methods such as prior authorization (89%) or step therapy (50%). Nevertheless, one-half (50%) of interviewees recognized the limitations of such approaches because these tools were intended to ensure appropriate utilization, not control affordability. Other interviewees discussed the constraints on coverage and cost-sharing policies owing to legislative or regulatory restrictions. One participant indicated, "If you look at Medicare and now

Figure 3. Theme 2: Cure challenges and policies to address the challenges.

Sub-themes	Percentage of Respondents Endorsing the Concept	Illustrative Quotes
1. Challenges in paying for cures		
a. Affordability	100%	"I think this is probably the single largest issue facing pharmacy managers today… some of these therapies are hundreds of thousands of dollars, and they have a relatively limited population and I understand the economics behind why the pharmaceutical manufacturers are pricing them as they are. But in many ways, it's abusive."
b. Uncertainty regarding long term outcomes	72%	"So, if you're 50, and I cure you, that tells me something about what you look like till 57. It doesn't tell me what you're gonna look like at 77."
c. Member churn and competition	56%	
d. Increased premiums	50%	
e. Lack of pricing transparency and value	22%	
2. Policies to address cure challenges		
a. Towards patients		
i. Prior authorization	89%	"Target the drugs to those patients for whom there's the highest value and the greatest clinical benefit. That can be done through step therapy. That can be done through prior authorization."
ii. Step therapy	50%	
iii. Increase cost-sharing	28%	
iv. Specialty pharmacy	28%	
b. Towards providers:		
i. Specialist restriction	28%	"We decided to keep it fairly tight to just people who were specialists in hepatology; they were the ones who were writing the guidelines."
ii. Prescribing guidelines/other education	17%	
iii. Accountable care organizations/shared savings models	17%	
c. Towards manufacturers:		
i. Performance-based risk-sharing	72%	"I think we're moving to a world where we want to see more accountability on the part of the pharmaceutical company. But that takes a dialogue. That also takes willingness to understand what does success look like? How do we measure it? At what point do we measure? Who's accountable for compliance and adherence, especially if you have to take a chronic drug? And then, the hard part, which is if the drug fails, how do you define failure? And then, how does pharma make you whole for those costs?"
ii. Price-volume arrangements	44%	
iii. Copayment coupons	22%	
iv. Indication-based pricing	17%	
3. Limitations of enacted policies		
a. Reduced patient access	33%	"You wanna treat those patients with the most severe liver disease and that would get the most benefit right now, because you can't afford to treat everyone. And so what we have is implicit rationing. It's not explicit."
b. Not in line with effectiveness or cost-effectiveness	11%	
c. Constraints on coverage and cost-sharing policies due to legislative or regulatory restrictions	39%	"if it's a payable drug by contract and if the doctor demonstrate that the patient needs medical necessity and have either tried alternatives or there are no alternatives, we're not in a position to say no"
4. Limitations of policies not yet enacted: Performance-based risk-sharing		
a. Outcomes not easy to measure and agree on	39%	"They're typically expensive to monitor and track and report on. So, it takes administrative resources from a health plan. Then you have to come to an agreement on a contract itself."
b. Laborious	17%	
c. Expensive	44%	
d. Data availability	33%	

Medicaid is following suit quickly. There are a lot of regulations around if you are a Part D benefit plan you will have the following formulary structure … . And the government goes so far to say and if you do not have certain drugs on your formulary here are the exceptions criteria you must apply to allow patient access to these drugs." Another noted, "So trying to find that

happy balance between trying to help member cost share, but at the same time, be accountable for drug costs. It's a very fine line, very fine balance. And to be very controversial, you have a lot of government agencies trying to implement copay maxes and how much you can charge for a drug (eg, state laws limiting maximum out-of-pocket costs[26])."

Interviewees were also cautious about new approaches. Whereas 72% of interviewees mentioned performance-based risk-sharing arrangements as a potential approach to deal with the challenges, 67% also recognized limitations of this approach. Some challenges mentioned were administrative burden with measuring outcomes, financing the measurement, and the need for manufacturers and payers to mutually agree on outcomes measures and terms of the contract. One respondent indicated, "They're complicated to administer, and often the outcomes that are observed takes times to observe. And they're typically expensive to monitor and track and report on. So, it takes administrative resources from a health plan or PBM (pharmacy benefit management) perspective, and it also impacts the base agreement with the drug company potentially. … Then you have to come to an agreement on a contract itself."

Discussion

In this study, we interviewed a diverse group of stakeholders mainly involved in administrating pharmacy benefits for a large number of beneficiaries. We solicited interviewees' opinions on their definitions and key features of cures, the affordability and value challenges posed by these treatments, and methods for addressing these challenges. We found that payers most commonly cited clinical criteria in their definition of a cure. Nevertheless, all respondents also agreed that affordability was a major challenge, and an equal number mentioned the financial aspect of cures being as important to payer decision making as the clinical aspect. Such concern was even present in defining the concept of cure because half of respondents defined a cure based on whether the intervention can eliminate future patient expenditure owing to the disease.

To our knowledge, such a concept, which we term a "healthcare cost cure" has not been explicitly developed or defined in the biomedical literature. This concept goes beyond prior cure definitions to include the elimination of downstream costs. It differs from 3 definitions of cures in the literature: absolute cure, functional cure, and statistical cure. An absolute cure is marked by complete and final termination of a specific instance of disease.[16,17] A statistical cure refers to a patient's mortality returning to the background rate of the general population.[18,19] One example cited in the literature for statistical cure is autotransplantation and long-term maintenance chemotherapy for multiple myeloma where individuals can have remission of disease for up to 15 years of follow-up from baseline.[27,28] A functional cure is one in which the individual's disease is brought under control to the degree that the individual no longer has any manifestations of the disease but the disease has not been eradicated. This term is used in the HIV literature to designate potential treatments where HIV genetic material still persists in patients but patients can discontinue antiretroviral therapy and have no clinical consequences.[20,29]

Although achieving a statistical or functional cure is important for clinical and patient-centered objectives, they do not emphasize whether the expenditures incurred by "cured" individuals return to baseline levels. As such, a healthcare cost cure represents a higher bar in that it considers both the cost and health effects of the intervention. For instance, after initial successful treatment, multiple myeloma patients require long-term posttransplantation maintenance therapy and annual re-evaluation.[27,28] More broadly, although oncology providers may consider individuals "cured" after 20 years of disease remission, they may continue to recommend annual oncology follow-up visits for these same patients.[30] Such instances could be considered statistical cures, but they would not be considered healthcare cost cures because payers specifically indicated that treatments that continue to require long-term surveillance or ongoing treatment would not be considered cures (for representative quotes, see Figure 2 "No further treatment," and see Appendix Table 1, quotes 4 and 8, in Supplemental Materials found at https://doi.org/10.1016/j.jval.2018.11.012). This distinction has important practical consequences because coverage of statistical or functional cures that are not also healthcare cost cures could cause plan premiums to increase or coverage of other services to decrease. This also raises questions, such as, if payers were to pursue a payment model whereby health plans take on debt to reimburse the costs of cures, would payers consider such treatments no longer curative, at least economically, because payments are amortized over time?[31,32]

Another concern raised by interviewees is membership churn and competition. Their concern seems to be consistent with the standard economic view which indicates that markets tend to undersupply goods that have a positive externality. Indeed, there may be scenarios in which it would be in the mutual interest of health plans that share a common pool of beneficiaries (flowing into and out of the different plans) to all offer high coverage for a treatment, but each individual plan has incentives not to so. Generally in these cases, government interventions such as subsidies[33] and mandatory health plan coverage (eg, for certain vaccines[34]) or broad-based health plan fees (eg, to generate comparative effectiveness research[35]) have been used to level the playing field. In the case of cures, others have proposed the concept of a potential tradable currency that would be backed by Medicare that guarantees payment to the private payer for each treated person entering the Medicare program.[36] This incentivizes private payers to invest in upfront coverage for cures because cured individuals would likely have lower morbidity, and the private payer can recoup value of the curative intervention when the member switches plans. A survey of managed care pharmacy stakeholders in a different setting suggested that such a concept was appealing economically but would require major structural and legislative change.[37] Another potential solution could be payments that are spread over the expected duration of benefit that would be paid by the plan which has the currently enrolled (previously treated) individual.[38] Nevertheless, appropriate design to avoid perverse incentives not to enroll treated individuals would be required. Finally, the concern with membership churn and firm competitiveness highlights the importance of careful consideration of the decision maker's perspective in economic evaluations of cures (as recommended by the Second Panel on Cost-Effectiveness in Health and Medicine).[39] In particular, to target decision makers in fragmentary financing systems, our study suggests that analysts should consider an additional multipayer system perspective that accounts for relevant time horizon and membership turnover factors.

Payers also expressed uncertainty regarding long-term outcomes of cures. Determining whether a patient is truly cured can in certain cases require many years of follow-up; nevertheless, most curative treatments are approved based on clinical trials that are much shorter in duration. Concern about the use of clinical trial data to extrapolate long-term real-world impacts is likely to increase with proposed changes to shorten FDA approval time[40] and the passage of the 21st Century Cures act, which establishes a review pathway that enhances use of biomarker evidence and lowers the evidentiary standard for certain new anti-infective agents.[41]

Although payers indicated that they were likely to continue applying utilization management methods and that they recognized the limitations of such methods in managing the affordability of potentially high-value drugs, they did not discuss many new approaches. Among those that were mentioned, performance-based risk-sharing arrangements were the most commonly mentioned approach. This interest aligns with the Center for Medicare and Medicaid Services' (CMS) stated interest in these arrangements.[42] Indeed, CMS had previously entered into an outcomes guarantee agreement with the manufacturer of the CAR T-cell treatment, tisagenlecleucel, where CMS would only pay for the treatment for acute lymphoblastic leukemia patients who respond by the end of the first month.[43] Nevertheless, similar to study respondents' discussions regarding the limitations of performance-based risk sharing, others have raised concern over the short duration of the tisagenlecleucel contract, given the uncertain long-term effectiveness of the cure, and the agreement has subsequently been canceled by CMS.[10,44,45] To address some of these concerns, the manufacturer of another treatment, voretigene gene therapy for inherited retinal disease, is developing performance-based contracts with short- and long-term effectiveness measures along with multiyear payment models.[46]

Limitations

Our study has several limitations. First, to obtain valid information regarding payer views on cures, our sample was restricted to individuals who hold positions in US payer or purchaser organizations and who deal directly with coverage determination for highly effective or curative therapies. We limited our sample to one representative individual per organization. Despite a high response rate, our sample was only 18 observations. Nonetheless, we did not find new themes after interviewing the ninth individual. We then doubled our sample to ensure thematic saturation. Second, our findings were based on a voluntary convenience sample and may not be generalizable to other payers. Nevertheless, the large number of beneficiary lives covered by the health plans represented by the interviewees would suggest that the results of this study are at least representative for the large number of beneficiaries. Third, we interviewed only one individual at each organization, and that individual may not adequately capture the opinions of the entire organization. This concern is mitigated somewhat by our choice of individuals who would likely be key informants, targeting individuals who explicitly dealt directly with coverage determination for highly effective or curative therapies. Finally, our interviews represent the opinions of the interviewees at a particular point in time, and respondents may be prone to responding based on recent experience, such as with the market approval of the new generation of hepatitis C direct-acting antivirals in late 2013-2014. Nevertheless, as suggested previously, the regulatory environment and drug development pipeline seem to suggest that new curative treatments are to be expected in the future.[47]

Conclusions

Curative treatments present new challenges for healthcare payers. With these treatments, payers are concerned with the prospect of reducing downstream costs. This pervades their concept, definition, and management of curative therapies. Further, payers struggle with the affordability of high prevalence cures, the large upfront costs, uncertainty of long-term outcomes, and how costs and benefits accrue to different stakeholders. In particular, the fragmentary US health insurance market introduces incentives to not cover such treatments. Stakeholders will need to consider policies that ensure that the costs of cures can be appropriately spread and covered overall.

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Supplemental Materials

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