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The Potential Benefits of Deferred Payment for a Hypothetical Gene Therapy for Congestive Heart Failure: A Cost-Consequence Analysis

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Abstract

BACKGROUND—The emergence of potentially curative pharmacologic treatments that deliver long-term clinical benefits with a limited number of doses is may create short-term budget challenges for payers as their unit price can be high.

OBJECTIVES—This paper tests clinical and financial properties of a deferred payment model (DPM) in a hypothetical therapy for congestive heart failure (CHF) from the perspective of payers, manufacturers and patients.

METHODS—We present an empirical analysis of longitudinal data for cardiovascular admissions and mortality using a Markov transition model for patient progression under different payment scenarios. The model calculates life-years gained and avoided cardiovascular admissions under status quo and deferred payment and a hypothetical budget constraint. We track over 91,000 Medicare fee-for-service beneficiaries over a period of 5 years (2009–2014) using MedPAR 5% data files.

RESULTS—We find that DPM is associated with earlier treatment and a consequent improvement in clinical outcomes. A 25% down payment is associated with the highest relative improvement and reduces hospital admissions by 0.52% (by 2,611 vs. 2,071 cases) and mortality by 0.29% (by 799 vs. 648 cases), both relative to status quo payment. Deferred payment results in limited financial gains for payers or manufacturers, primarily because of the small share of

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expected cost savings on the total cost of therapy. Our results are robust to changes in relative risk for cardiovascular admissions and a change in the cost of therapy.

CONCLUSIONS—DPM may result in faster access to a CHF gene therapy and may thus reduce hospital admissions and mortality in contrast to status quo payment with the same budget constraint. Although financial benefits of DPM in a CHF gene therapy are limited, it is possible that deferred payment will show greater promise for treatments with higher cost offsets.

Introduction

The emergence of “curative” therapies that deliver long-term clinical benefits with a limited number of doses, possibly including some gene and cell therapies, is creating challenges for payers as the unit price of those products tends to be very high. Even if these treatments meet conventional thresholds for value for money, short-term budget constraints can lead to obstacles to access, such as in the case of the directly acting antiviral for Hepatitis C (1, 2).

New payment approaches have been proposed to address this challenge, ranging from outcome-based agreements to reinsurance schemes and installment-based payment (3–6). Recently, multiple states have adopted a subscription-based model to ensure wider access to high-cost cures of Hepatitis C (7), dubbed the ‘Netflix model’ (8). In cases of curative and regenerative therapies, deferred (installment) payments are thought to have an advantage in shifting the financial profile of such payments to that of chronic treatments, and could even include a performance component to link payment to clinical outcomes in individual patients or patient cohorts (3).

As we show in earlier work, regenerative therapies are currently under development for numerous indications potentially targeting large numbers of people (9). In this paper, we study one potential design of an outcomes-based, deferred payment model (DPM) and use gene therapy for congestive heart failure (CHF) as case study. By studying the financial and clinical properties of this payment model for payers, manufacturers and patients, we provide an empirical assessment of the financial and clinical properties this payment model produces relative to existing (status quo) payment model, generally associated with upfront payment.

The choice of CHF as case study is motivated by the potentially large treatment-eligible population, as around 6.5 million people suffer from the disease in the United States alone (10). As the prognosis under standard of care remains poor – a 5-year mortality of nearly 50% (11) – a substantial share of these patients could be eligible for treatment. Specifically, several regenerative therapies have been under development for CHF, including a Phase IIb trial by Celladon of Mydicar, a gene therapy aiming at SERCA2a protein which is downregulated in a failing heart, which experienced a vector failure in 2015 (12), and more promising Phase II results reported in 2017 by Renova Therapeutics for RT-100, its adenylyl cyclase type 6 (AC6) gene transfer (13). A phase III trial of RT-100 is expected to commence soon. RT-100 is a single-dose gene therapy delivered via an intracoronary injection during an outpatient procedure with the potential to improve cardiac function (13).

This paper tests the clinical and financial properties of a deferred payment model for a hypothetical CHF gene therapy relative to status quo payment. It focuses on the perspective

of three stakeholders: payers, who minimize per-patient spending, manufacturers, who maximize product revenue, and the patient population, which maximizes clinical benefits. In addition, we test the sensitivity of clinical and financial outcomes on payment design choices.

Methods

The analytical approach of this paper consists of three components: an empirical analysis of longitudinal data for cardiovascular admissions and mortality, a Markov transition model for patient progression under different payment scenarios, and a discounted cash flow forecast model.

A separate empirical analysis of CHF readmissions and patient mortality by the authors (14, 15) was used to populate Markov chain probabilities in our model. That analysis, using Medicare Provider and Analysis Review (MedPAR) 5% data files, calculated 30-day probabilities of death and repeat CHF admission. The analysis was limited to patients admitted with a primary diagnosis of CHF (ICD-9-CM 428.xx) who did not die during the admission and had not been admitted for CHF in the preceding 12 months. The study ultimately tracked more than 91,000 Medicare fee-for-service beneficiaries over a period of 5 years (2009–2014), from the index admission until death, disenrollment, or the end of the study period.

The Markov transition model for patient progression under status quo payment and deferred payment calculates life-years gained and avoided cardiovascular admissions under DPM relative to status quo payment with an identical (hypothetical) budget constraint. The model consists of annual time steps and assumes an identical gene therapy would be offered under existing (status quo) and deferred payment models. We model different levels of payment deferral by varying the size of a down payment relative to the cost of the therapy. In our simulation, down payment ranges from 25% to 75% of the total expected cost of therapy, which is estimated at US\$ 200,000 (16).ⁱ Our payment simulation is based on the assumption that avoided cardiovascular hospitalizations contribute to an increased annual budget for CHF patients (such cost offsets are designed to be the only change to otherwise fixed annual budgets, to ensure comparability with status quo payment), allowing more people to receive treatment. The average cost of cardiovascular hospitalizations avoided of US\$ 16,000 is based on Kilgore, Patel (17). The average cost of therapy is identical under both DPM and status quo payment on a per-patient basis over a period of 3 years. A longer payment model may not be practical due to a large attrition of patients with CHF given high mortality rates both with and without treatment, as we show later. Disenrollment (loss to follow-up) commonly denotes patient switching to Medicare Advantage plans, and is assumed constant under both payment models.

For treated patients, we assume a relative risk of any future cardiovascular admission of 0.70 compared to untreated patients (rounded downwards from the relative risk of a first heart

ⁱWe do not consider down payments smaller than 25% due to feasibility issues, and do not expect any down payment to exceed 75% of the total cost of care for practical purposes.

failure admission achieved by sacubitril/valsartan (Entresto), a new therapy for CHF (18). Moreover, we assume an identical relative risk of mortality to that of Entresto for cardiovascular death, 0.80 (18).

Payment under DPM consists of a down payment of differing amounts and monthly installments for each period when a patient is ‘stable’ (our payment model is outcome-based: it is suspended for a month if a patient experiences a cardiovascular hospitalization and is halted once a patient dies). At the baseline, we assume an annual, hypothetical budget constraint of US\$ 1 billion – the amount associated with “potential short-term affordability and access challenges” estimated by the Institute for Clinical and Economic Review (19) – which translates to about 15,000 patients treated in the first 3 years under fee-for-service payment arrangements. This is only a small fraction of the potential patient pool but shows the magnitude of the potential benefit relative to the cost of therapy for a cohort of any size. Likely spending on a novel gene therapy for CHF would be higher given the burden of disease, and we would not expect an explicit budget constraint in the Medicare program.

Figure 1 shows the payment design as evaluated in this study and Table 1 indicates our parameters and sensitivity ranges tested.

Third, we assess the financial effects of a DPM for manufacturers with a discounted cash flow forecast model. We calculate the cash flow as discounted treatment revenue using a discount rate of 5% and compare the cash flow resulting from status quo payment and different down payment scenarios under DPM, both with identical budget constraints, cost of therapy and relative risk reduction. The cash flow model assumes that all payments for the therapy accrue to the manufacturer as revenue, and discounts future revenues to present-day dollars.

Results

Figure 2 shows monthly event probabilities for patients, who have been discharged following a CHF diagnosis under standard of care. Patients are at the highest risk of cardiovascular admissions in the first several months following the index event, with monthly risk ranging from 14.7% in the first month to 5.1% one year after the index event. Similarly, mortality risk is the highest in the first several months following the index event, ranging from 8.4% in the first month to 2.3% a year after initial discharge. Total mortality in the first, second and third years are 33.6%, 20% and 19.5%, respectively.

The number of patients treated monthly under different payment scenarios is presented in Figure 3. Under all scenarios, deferred payment results in slightly more patients from the same cohort receiving gene therapy (ranging from 15,043 patients treated under a 25% down payment to 15,000 under status quo payment)ⁱⁱ, and those patients receiving therapy sooner than under status quo.

In Table 2, the effect of individual payment models relative to no treatment is presented. The highest number of admissions and deaths are avoided under the lowest down payment of

ⁱⁱNumbers of patients treated monthly in Figure 3 are rounded.

25%, which translates into a 26.1% increase of cardiovascular admissions avoided relative to status quo payment (or a 0.52% reduction in the simulated patient pool) and a 23.3% increase of deaths avoided over a three-year period (or a 0.29% decrease in mortality in the simulated patient pool), largely due to patients receiving treatment sooner than under status quo. Our results are conditional on the fact that only about 16.5% of eligible patients receiving treatment. At an admission cost of US\$ 16,000 and annual budget constraint of US \$ 1 billion, the highest potential cost-savings due to admissions avoided under DPM relative to status quo payment are US\$ 8.64 million at the baseline.

Relative to status quo payment, a 25% down payment in DPM under our base budget constraint (US\$ 1 billion annually) results in 345.2 life-years gained, an increase of 51.6%. The gain with a 75% down payment relative to status quo is 73.3 life-years gained, an increase of 11%. As a result of being treated earlier under DPM, fewer patients die in the 3-year period and societal benefits are derived from patients with CHF living longer beyond savings due to avoided admissions (a full summary of clinical benefits observed is presented in Table A.1 in the Appendix).

Finally, the results of a discounted cash flow analysis indicate small differences in total cash flow to the manufacturer under different payment scenarios and for different discount rates over a period of 3 years. Under the highest discount rate of 5%, the relative difference between DPM with the smallest down payment and status quo payment is just 0.31% (US\$ 2.859 billion for status quo, US\$ 2.868 billion for a 25% down payment). A 50% down payment produces a discounted cash flow of US\$ 2.864 billion. These results are primarily driven by model design: the difference in cash flow results from more patients being treated due to savings in hospital admissions under DPM relative to status quo.

Our results show consistent trend following changes to relative risk for cardiovascular admissions and a change in the cost of therapy (as shown in the Appendix, Figures A.2 and A.3). An improvement in the relative risk of cardiovascular admission to 0.50 produces a 28.5% increase in the admissions avoided under the lowest down payment in DPM relative to status quo payment (Figure A.2.), and a decrease in the cost of therapy by 50% to US\$ 100,000 per patient allows over twice as many patients to receive treatment, resulting in 26.9% more cardiovascular admissions avoided under a 25% down payment relative to status quo (Figure A.3). In case of a higher treatment effectiveness for cardiovascular admissions ($RR=0.50$), the total cost of admissions avoided in our model is US\$ 16.9 million, and in case of a reduced price of therapy (US\$ 100,000 per patient), the cost of admissions avoided is US\$ 17.8 million.

Discussion

Previous research has indicated that implementing novel payment arrangements for prescription drugs, including performance-based payment, has been difficult (20). Yet, the arrival of cures and other regenerative therapies is continuing pressure on payers to come up with new payment models that would enable ‘rapid adoption’ of high-cost treatments (6), and payers including the Centers for Medicare and Medicaid Services have been exploring “innovative pricing systems” that could achieve that goal (21). In this paper, we present the

first empirical assessment of both clinical and financial outcomes of deferred payment for patients, payers and manufacturers, using a hypothetical gene transfer in congestive heart failure as a test case.

We find that deferred payment does not result in substantial financial gains for payers or manufacturers, primarily because of the small share of expected cost savings on the total cost of therapy. In our model, nearly 13 admissions must be avoided for one additional patient to receive treatment and benefit from a lower relative risk of cardiovascular hospitalization. The absolute impact on hospital admissions and mortality resulting from DPM is small (both less than 1% (Table A.1.)), given our hypothetical annual budget constraint of US\$ 1 billion and the small share of treated patients on all patients in the sample, with the cost of admissions avoided of less than US\$ 9 million over a period of 3 years under the most favorable scenario. Net gains of DPM may be eliminated once the costs of implementing the model are considered. In neither of our scenarios does DPM present a significant cash flow advantage to the manufacturer: under the most favorable conditions, the maximum cash flow gain is just over 0.3%.

The benefits of deferred payment in CHF would be mostly felt by patients: we find that DPM is associated with earlier treatment and a resulting improvement in clinical outcomes. The smallest down payment under DPM is associated with the highest improvement and reduces the number of admissions by 0.52% and deaths by 0.29% relative to status quo payment, while spending is held constant. Given our budget constraint and the size of the simulated cohort, approximately 16.5% of theoretically eligible patients would be treated over a period of 3 years: the absolute improvement in mortality and admissions directly correlates with how many of eligible patients ultimately receive treatment (see Table A.1 in the Appendix for our base case scenario). DPM may also be appealing to payers as it rewards positive outcomes in patients who remain enrolled in their plans since payment is discontinued when a patient dies and suspended during readmissions.

While we find that financial benefits of a DPM in a CHF gene therapy are relatively limited, it is possible that deferred payment will show a greater promise for treatments with higher cost offsets, such as a potential cure of hemophilia, a disease with significant maintenance therapy costs (22). Moreover, indications with large prevalent populations relative to incident cases may benefit more significantly from DPM as the budget impact of treating all eligible patients is concentrated into a shorter time horizon and could prevent a larger share of patients from receiving timely access to a new therapy (in contrast, CHF is a disease with a large number of incident cases). Our results, nevertheless, suggest that providing patients with earlier access to a high-cost therapy is a key benefit of deferred payment in CHF, and that the presence of budget impact considerations in coverage decisions may increase plans' interest in innovative payment models, such as DPM, for high-cost therapies.

Limitations

Our analysis may be affected by several limitations. First, the patient population in our model is based on a sample of Medicare beneficiaries (commonly age 65 and older) – a sample which does not capture the full spectrum of CHF patients in clinical practice. In

addition, the data includes patients with any heart failure, irrespective of their ejection fraction stage. Some of the treatments in late-stage trials aim at reduced ejection fraction heart failure patients who could experience different outcomes than the general CHF patient population. Third, we do not address implementation challenges, such as regulatory issues or the cost of implementing DPM in clinical practice. As a result, we are not able to determine the net costs and benefits of DPM relative to status quo payment. Fourth, our outcome of interest, cardiovascular admissions, is an imperfect proxy for the effectiveness of a gene therapy in CHF, given that patients with the disease suffer from multiple comorbidities and may be admitted for a number of conditions. We conclude, however, that relying on CHF admissions alone could underestimate the benefit of the therapy given the imperfect distinction between cardiovascular and heart failure admissions in claims data. Fifth, our data originates from 2009–2014, prior to the introduction of sacubitril/valsartan (Entresto), which has likely changed the underlying mortality and admissions risk for patients who have received it. As a result, our model may overestimate the relative benefits of a hypothetical CHF gene therapy. Sixth, our model assumes a hypothetical gene therapy, which is not yet approved. Seventh, we assume providers are ‘perfect agents’ for patients, which does not take into account possible incentive misalignment between providers and patients. Finally, we do not assume any prioritization in treatment by disease severity, thus assuming homogeneous risk reduction due to treatment across patients in our sample. In reality, it is possible that patients expected to benefit the most from treatment (such as those who would experience the largest improvement in survival or reduction in hospitalizations) would be treated first, thus increasing both clinical and financial gains of deferred payment observed in the real world relative to our findings.

Conclusions

While conceptually promising, deferred payment may not be a universal solution in payment for high-cost treatments such as a gene therapy in CHF. We find, however, that under the proposed design, DPM may result in faster access to a restorative treatment for CHF, which may produce societal benefits, including a reduction in hospital admissions and mortality in contrast to status quo payment with the same, hypothetical budget constraint.

Deferred payment could, in addition, generate greater clinical and financial benefits if implemented in indications with high per-patient or cohort-level cost offsets due to receiving treatment sooner – ranging from indications with costly standard of care to those with large prevalent patient populations relative to incident cases observed. Empirical research should be conducted to test these hypotheses, particularly focusing on treatments with a combination of substantial budget impact and cost offsets, and show under which conditions a DPM would produce greatest clinical and financial benefits.

We conclude that deferred payment in CHF gene therapy is a creative solution that may expedite access to high-cost therapies and improve clinical outcomes, but its acceptance in clinical practice hinges on resolving challenges related to its economic viability.

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Appendix

Table A.1:

Events observed under different payment schemes (base scenario)

Treatment and Payment Scheme	Variable	DPM	Life-Years Gained (Relative to status quo payment)
No Treatment	Deaths	52,116	-
	Loss to Follow up	24,852	
	Admissions	103,744	
	Patients Treated	0	
Status Quo Payment	Deaths	51,468	-
	Loss to Follow up	25,026	
	Admissions	101,673	
	Patients Treated	15,000	
75% Down Payment (US\$ 150,000)	Deaths	51,430	73
	Loss to Follow up	25,042	
	Admissions	101,541	
	Patients Treated	15,011	
62.5% Down Payment (US\$ 125,000)	Deaths	51,406	121
	Loss to Follow up	25,053	
	Admissions	101,457	
	Patients Treated	15,017	
50% Down Payment (US\$ 100,000)	Deaths	51,378	181
	Loss to Follow up	25,067	
	Admissions	101,358	
	Patients Treated	15,025	

Treatment and Payment Scheme	Variable	DPM	Life-Years Gained (Relative to status quo payment)
37.5% Down Payment (US\$ 75,000)	Deaths	51,346	256
	Loss to Follow up	25,084	
	Admissions	101,242	
	Patients Treated	15,034	
25% Down Payment (US\$ 50,000)	Deaths	51,317	345
	Loss to Follow up	25,102	
	Admissions	101,133	
	Patients Treated	15,043

Figure A.2: Clinical outcomes under improved efficacy (risk reduction in admissions of 0.50)

NOTE: This model assumes existing (baseline) assumption for the cost of therapy per patient (US\$ 200,000), and annual budget of US\$ 1 billion.

Figure A.3: Clinical outcomes under lower cost of therapy per patient (US\$ 100,000)

NOTE: This model assumes existing (baseline) assumption for risk reduction in admissions (RR = 0.70) due to treatment, and annual budget of US\$ 1 billion.

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Key Points for Decision Makers

- Deferred payment models can improve access to treatment.
- Simulating such a model using the example of a hypothetical gene therapy for heart failure suggests that it can facilitate access and improve clinical outcomes.

Figure 1:
Structure of a Deferred Payment Model for Congestive Heart Failure

Figure 2: Post-discharge event probability for CHF patients with no treatment, by month since index event

Note: Monthly data based on a 5% sample from based on Medicare Provider and Analysis Review (MedPAR) data (2009 – 2014).

Figure 3:

Average monthly number of patients treated by year, by payment scenario (annual budget: US\$ 1 billion)

Table 1:

Model Parameters and Sensitivity Ranges

Parameter	Parameter Value	Sensitivity Test	Reference
Down payment	25%, 37.5%, 50%, 62.5% and 75%	-	Authors' choice
Length of Deferred Payment Model	36 months	-	Authors' choice (based on high patient attrition beyond 36 months)
Cost of therapy	US\$ 200,000	US\$ 100,000	Baseline value based on Kish (2017) [16]
Cost of cardiovascular admission	US\$16,000	-	Kilgore, Patel et al. (2017) [17]
Relative risk of mortality	0.80	-	Comparable to sacubitril/valsartan (Entresto) (McMurray et al., 2014) [18]
Relative risk of cardiovascular admission	0.70	0.50	Baseline value comparable to sacubitril/valsartan (Entresto) (McMurray et al., 2014) [18], hypothetical reduction to 0.50
Budget constraint	US\$1 billion	-	Approximation based on Institute for Clinical and Economic Review (2019) [19]
Discount rate	5%	-	Authors' choice

Table 2 4:

Clinical outcomes for congestive heart failure patients relative to no treatment, by payment scenario (annual budget: US\$ 1 billion)

	Admissions avoided	Deaths avoided	Admissions reduction	Mortality reduction
Status Quo	2,071	648	2.00%	1.24%
75% down payment	2,203	686	2.12%	1.32%
62.5% down payment	2,287	710	2.20%	1.36%
50% down payment	2,386	738	2.30%	1.42%
37.5% down payment	2,501	770	2.41%	1.48%
25% down payment	2,611	799	2.52%	1.53%

Note: Under Status Quo, 15,000 patients would receive treatment, while a 25% down payment would enable the treatment of 15,043 patients. Please see Table A.1 in the Appendix for a summary of deaths and admissions avoided as well as life-years gained under individual payment schemes.