

NEW DIGS

FoCUS

Financing and Reimbursement
of Cures in the US

WHITE PAPER



Survey Results

Payer Perspectives on Financing and Reimbursement of One-time High-cost Durable Treatments

October 11 2019

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KEY TAKEAWAYS

- Payers have a heightened concern regarding the financial risk and sustainability of high cost one-time durable treatments
- BOTH high upfront cost of individual treatments and cumulative impact of multiple treatments are a concern
- Payers are interested in implementing new financing approaches – with high priority and urgency, yet different payer segments have different challenges and may be more closely aligned with different financing models.
- Payers are most closely aligned with the benefit of paying for what works
- Multiple barriers must be resolved: Regulatory issues must be addressed at the structural systems level

BACKGROUND

The reimbursement of emerging one-time high-cost durable/potentially curative cell and gene therapies is likely to be a challenge for US payers. The pricing of currently approved treatments and the cumulative cost impact as pipeline agents are approved are likely to challenge payers’ ability to provide affordable health coverage. In addition, the lack of long-term clinical durability data could mean that payers will be reluctant to cover these types of therapies. This would have a serious negative impact on patient access.

The Financing and Reimbursement of Cures in the U.S. (FoCUS) project was launched in 2016 by MIT’s NEWDIGS Initiativeⁱ with the objective of further elucidating the challenges and financial impact created by durable/potentially curative therapies and providing implementable financial models to manage the cost burden on the US healthcare system. This survey is the second payer survey performed for the FoCUS project to inform payer perspectives regarding current and future management of high-cost durable therapies with one-time administration.

The first FoCUS payer survey was conducted in August-September 2017 as the first chimeric antigen receptor (CAR) T-cell therapies, tisagenlecleucel (Kymirah/Novartis) and axicabtagene ciloleucel (Yescarta/Kite Pharma) were being approved. It consisted of telephone interviews with 15 payers across multiple payer segments.^{1,2} At that time, payers had variable awareness and readiness to manage the new cost of the therapies in question, with one-third newly aware and learning about these therapies, 40% watchfully waiting and 27% engaged in active management. Payers were open to financing mechanisms with 47% expressing a willingness to engage in innovative financing models, performance-based annuities and risk-pooling.

Additional payer surveys were published by the Alliance for Regenerative Medicine (ARM) and the National Association of Managed Care Physicians (NAMCP) of 36 respondents in March-June 2017³ and the National Pharmaceutical Council (NPC) of 21 respondents in February to March 2018⁴. These surveys reported a progression in awareness and concern regarding management of durable therapies, with 10% and 100% of payers respectively, reporting having started to consider coverage or operational issues associated with these treatments.

The goal of this online survey was to update the results of the initial FoCUS survey and broaden the number of payers surveyed. Areas of focus included interest in considering new financing approaches, the priority and timing of action, and considerations for adoption.

ⁱ See <http://newdigs.mit.edu/> for further details.

METHODS

An online 15-question survey was conducted with clinical and financial health plan leaders from US payers between September 2018 and April 2019. The survey was focused on assessing payer perspectives regarding current and future management of high-cost durable therapies with one-time administration. Over 400 individuals from various payer segments, including commercial plans, self-insured employers, Medicare and Medicaid were invited to participate by email. Results were compiled overall and by payer segment.

A total of 77 payers completed the online survey. They reported representing 153 commercial fully-insured, Medicaid, Medicare Advantage, and self-insured employer plans. Payers ranged in size from less than 5,000 insured lives to upwards of 50 million and in total covered over 280 million lives (Appendix Table 1). The survey did not control for more than one person from the same plan completing the survey. Results from intermediaries such as pharmacy benefit managers were excluded to the extent they could be identified.

Respondents were distributed among roles in pharmacy (53%); medical (31%); human resources and benefits (9%); and finance and actuary (7%).

THREE MAJOR FINDINGS

1. Payers are concerned about financial risk and impact of high-cost durable therapies.

Payers have a heightened level of concern regarding the impact of high-cost durable therapies. All payers have some level of concern related to the financial risk and impact of these treatments. 80% of payers rate their concern as high or extremely high.

When viewed by payer segment, commercial fully-insured plans expressed the highest levels of concern with 93% reporting a high or extremely high concern and 7% reporting a moderate concern (Figure 1). 50% of self-insured employers reported a high or extremely high concern and 50% reported a moderate or low concern.

Payers cited a number of reasons for their high or extremely high concern. These include:

- The total cost is material for the plan (98%)
- Product performance risk (effectiveness and durability) (91%)
- Actuarial risk (likelihood of encountering an orphan case) (91%)
- Payment timing relative to benefit realization (offsets may not cover the high cost of treatment) (84%)

Other reported reasons for high concern include:

- Burden of multiple high-cost therapies
- Concern about what this will represent in 3-5 years with respect to total cost
- Potential off-label use
- Operational management of larger pipeline
- Adverse selection

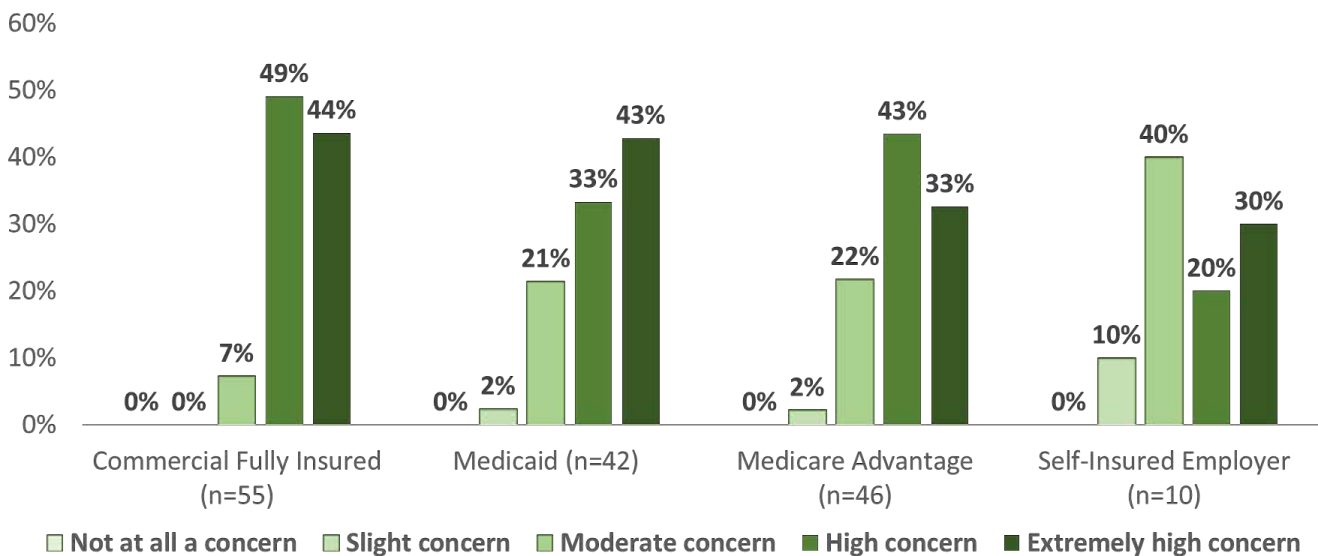


Figure 1. Level of concern regarding managing the financial risk and impact of high-cost durable therapies, by payer segment (n=153).

Payers are currently managing high-cost durable therapies with various strategies. The majority of payers surveyed (70%) have either stop-loss or reinsurance to cover high-cost cases.ⁱⁱ Thresholds vary from greater than \$2 million to \$100 thousand or less (Appendix Figure 2).

The vast majority of payers surveyed (99%) currently cover one-time high-cost durable therapies; 46% cover all treatments, while 53% cover some. For those covering high-cost durable therapies, payers are evenly divided regarding utilization management practices. 48% cover high-cost durable therapies as specified in the FDA-approved label. 49% apply utilization management coverage with more restrictions than the FDA-approved label. 3% cover with less restrictions than the label.

Commercial fully-insured payers are the most restrictive with 62% managing coverage with more restrictions than the label. Medicare Advantage is the least restrictive with 67% managing consistent with the label and 31% more restrictive. Case management is used by 82% of payers and centers of excellence (COE) are required for 64% of payers surveyed. 30% restrict use of all high-cost durable treatments to COE, while 34% restrict some of these treatments to COE.

2. Payers are motivated to manage the financial risk associated with high-cost durable one-time treatments differently, making this a high priority over the next 2 years.

In contrast to high-cost *chronic* treatments such as SPINRAZA (nusinersen) or ONPATTRO (patisiran), the majority of payers reported they were moderately, very, or extremely likely to change how they manage the financial risk associated with *one-time* high-cost durable treatments (Figure 3).

Payers were somewhat mixed regarding the importance of high upfront cost per patient and cumulative per member per month (PMPM) to their organization’s serious consideration of alternative approaches to manage financial risk of one-time high-cost treatments. 39% rated both factors as equal. 31% reported high upfront therapy cost per patient is much or somewhat more important, while 26% reported high total PMPM impact is much or somewhat more important.

Neither factor would trigger serious consideration of an alternative payment model for 5% of those surveyed.

Figure 4 demonstrates 57% of payers expect to implement a new management strategy in the next 1-2 years, while 13%

ⁱⁱ Reinsurance is typically offered by one insurance company to protect another insurance company. Self-funded employers (who usually are NOT insurance companies) purchase stop loss.

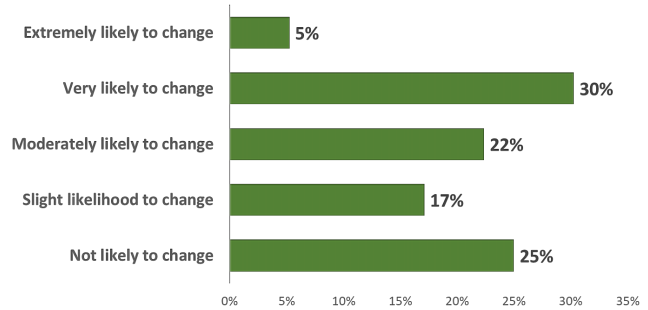


Figure 3. Payers are inclined to manage financial risk associated with high-cost one-time therapies differently (n=76).

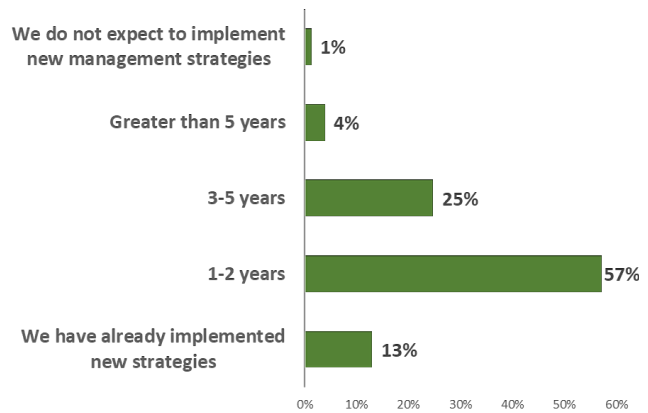


Figure 4. Responses to the question, “When would you expect to implement a new management approach?” (n=77).

already have. Figuring out the best way to finance new high-cost durable therapies is a high or very high priority for 76% of payers surveyed.

New strategies that payers reported have already been implemented included value-based or outcome-based agreements and changes in provider contracting, including payment of a third party and global case rate payments.

Various financing solutions were of interest for future management of high-cost durable therapies. Overall, payers were most interested in short-term milestone-based contracts, defined as contracts of less than 2 years duration where the therapy is paid for up-front and the plan receives refunds tied to performance (Table 2). Self-insured employers were most interested in population risk-pooling, stop-loss/reinsurance over the broader population.

Approach	Commercial Fully-Insured (n=55)	Medicaid (n=42)	Medicare Advantage (n=46)	Self-Insured Employer (n=10)	All Payer Segments (n=153)
Population risk-pooling	69%	40%	52%	50%	55%
Short-term MBC	75%	50%	61%	20%	60%
Long-term MBC	58%	36%	43%	30%	46%
Annuity: Spread payments over >2 years	33%	14%	24%	20%	24%
Performance-based annuity: spread payment over >2 years/tied to performance	47%	21%	35%	30%	35%

Note: Milestone-Based Contract (MBC): Pay for therapy upfront and receive refunds tied to performance over the short-term (<2 years) or the long-term (≥2 years)

Table 2. Financing solutions of interest for future management of high-cost durable therapies.

3. Addressing contract terms and barriers will matter.

Payers see multiple benefits of alternative financing approaches:

- Reducing upfront budget impact of the new therapy by smoothing payments over time
- Aligning the timing of the therapy costs with its benefits, and
- Only paying for therapy that works by including performance-based requirements for initial or continued payment

Of the three, payers see the most benefit in paying for what works, with 83% identifying this factor as extremely or very beneficial (Figure 5).

The majority of payers identified a number of elements of multi-year performance-based agreements as very important or a deal breaker. These include:

- The inclusion of performance-based requirements for payment (72%)
- Termination of payment obligation with the death of the patient (67%)
- Access to data on specific measures (66%)
- Term: The number of years over which the payouts are stretched (65%)
- Ability to track performance even if the patient has left the plan (62%)

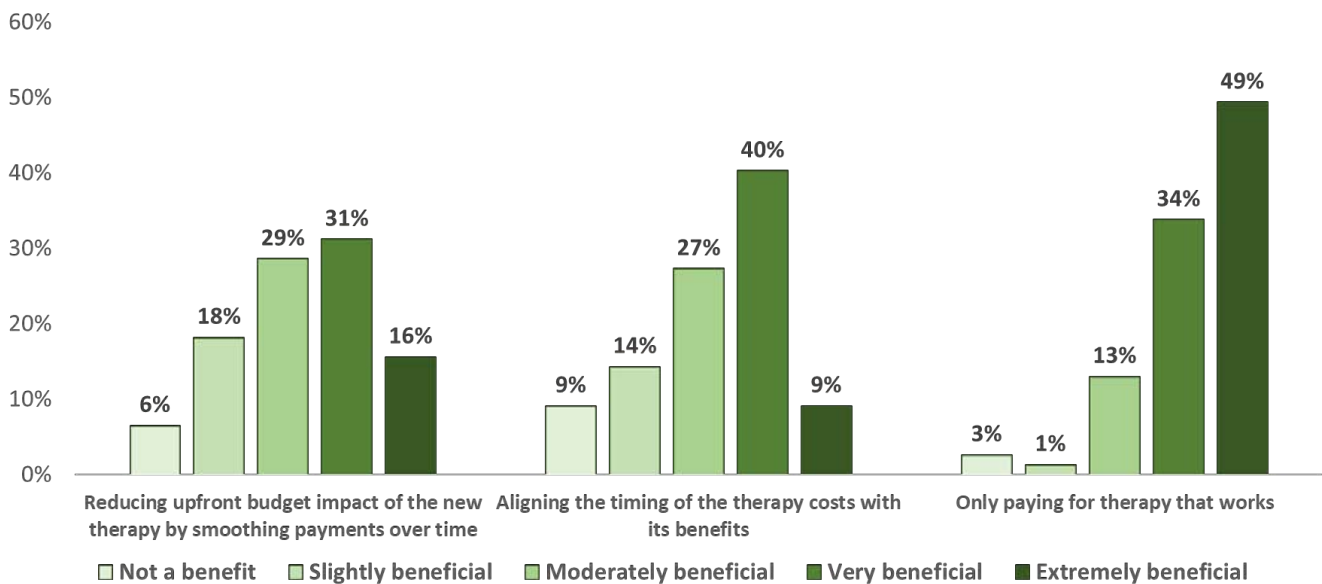


Figure 5. Benefits of alternative financing approaches (n=77).

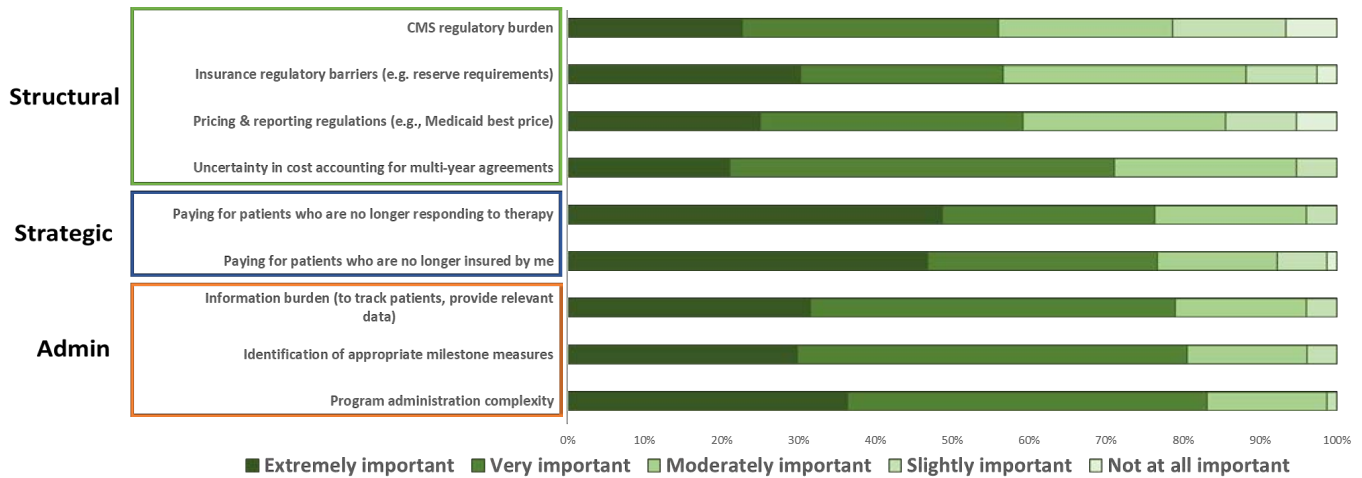


Figure 7. Payer barriers to alternative financing, by importance (n=77).

The length of time a patient is typically enrolled in the plan varies by payer segment from less than 2 years for 38% of Medicaid members to over 5 years for 70% of self-insured employer patients (Appendix Figure 6). This highlights payer concerns regarding the mismatch between payment and benefit, and the concern regarding ability to track patients over time for longer term contracts.

Finally, payers rated a number of barriers to alternative financing approaches by level of importance (Figure 7).

Using the two top levels of importance, extremely important and very important, roughly 80% of payers identified administrative barriers as the highest importance.

These included:

- Program administration complexity (83%)
- Identification of appropriate milestone measures (81%)
- Information burden for tracking patients and providing relevant data (79%)

Top rated strategic barriers included:

- Paying for patients who are no longer insured by the plan (77%)
- Paying for patients who are no longer responding to therapy (76%)

Top rated structural barriers included:

- Uncertainty in cost accounting for multi-year agreements (71%)
- Pricing & reporting regulations (e.g., Medicaid best price) (59%)
- Insurance regulatory barriers (e.g., minimum reserve requirements) (57%)
- CMS regulatory burden (56%)

CONCLUSIONS / DISCUSSION

Payers have a heightened level of concern regarding the impact of high-cost durable therapies with one-time administration. This level of concern has increased over time as more treatments have entered the market and clarity regarding the pipeline and likely approvals over time has grown.^{5,6} Commercial payers report the highest degree of concern, while self-insured employers reported the least concern. Whether this is a product of less awareness, reliance on intermediaries for plan administration or the result of having stop-loss coverage to protect against high-cost claims is unknown.

Payer concerns were consistent with the financing challenges FoCUS has identified in previous white papers,⁷ namely actuarial risk, therapeutic performance risk and payment timing relative to benefit gained. In addition, payers emphasized the impact of the total cost of treatment, both for an individual patient and the burden of multiple high-cost therapies.

These concerns further highlight the risk to long-term sustainability for individual payers and the US health care system as a whole. As a result, payers have already started to manage these treatments using many of the current management strategies they currently employ for other high-cost treatments.

And, payers are interested in managing the financial risk and impact associated with these high-cost one-time therapies differently. Payers are open to multiple different approaches, although most favor short-term milestone-based contracts

where therapy is paid for upfront and potential refunds are tied to failure to achieve performance metrics over the first 2 years following treatment.

This focus on a short-term, performance-based approach is consistent with payers' emphasis on paying for what works balanced with the administrative complexities of tracking patients for measurement over time and the increased risk over time that the patient may no longer be enrolled in the plan.

Compared with the prior FoCUS survey and the 2017 ARM/NAMCP survey that reported just over 10% of medical directors had started to consider coverage or operational issues associated with these treatments,³ payers now have a sense of urgency. Implementation of new management approaches is on the short-term horizon for the majority of payers and is a high priority for them. Both factors increase the likelihood that action will be taken.

Those actions will face a number of barriers. Payers will need to work through administrative issues and strategic positioning to facilitate alternative financing approaches. Of somewhat less importance to the plan, but high interest to other stakeholders, are issues related to regulation. Flexibility in current price reporting, such as Medicaid best price, and other insurance regulatory and accounting requirements will need to be addressed at a structural, systems level to enable implementation of these innovative models.

ABOUT FOCUS

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to collaboratively address the need for new, innovative financing and reimbursement models for durable and potentially curable therapies that ensure patient access and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by these therapies leading to system-wide, implementable precision financing models. This multi-stakeholder effort gathers developers, providers, regulators, patient advocacy groups, payers from all segments of the US healthcare system, and academics working in healthcare policy, financing, and reimbursement in this endeavor.

Please cite using MIT NEWDIGS White Paper: *A Survey on Payer Perspectives—2019F210v044*

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APPENDIX

	Commercial Fully-Insured	Medicaid	Medicare Advantage	Self-Insured Employer	Total/Range
<i>Payer segments n; (percent of participants)</i>	55 (36%)	42 (27%)	46 (30%)	10 (7%)	n=153
<i>Range of covered lives (000's)</i>	10 – 54,000	10 – 13,200	5 – 8,000	3 – 400	3 – 54,000
<i>Lives Covered</i>	184,335,447	59,379,661	36,508,957	933,854	281,157,919

Note: Survey participants reported engaging in more than one business segment.

Table 1. Participation by segments and covered lives (n=77).

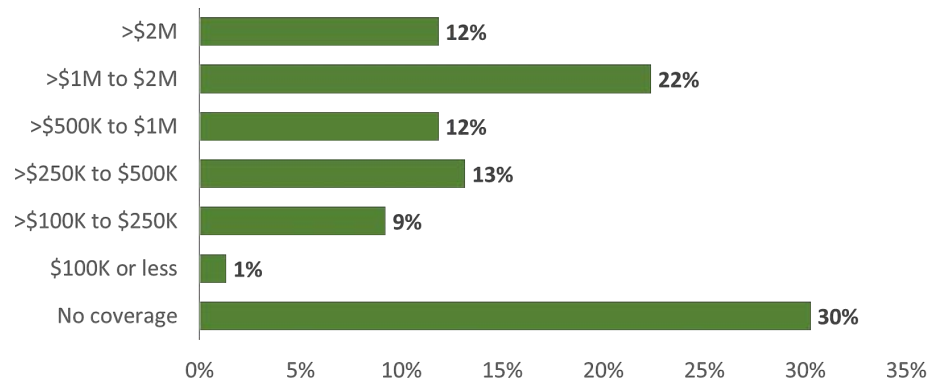


Figure 2. Threshold for stop-loss / reinsurance coverage (n=76).

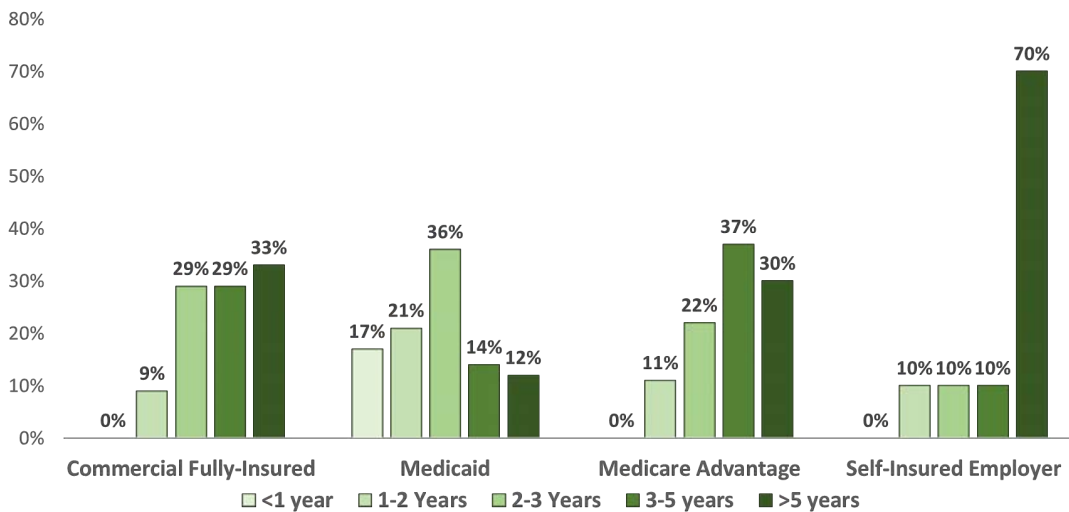


Figure 6. Length of time patients typically stay in the plan, by line of business (n=151).

MORE INFORMATION
 Supplemental figures are also available to download online at <http://bit.ly/PayersSurveyPlus>