How Are Curative Therapies Defined by United States (US) Payers?

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BACKGROUND

- Curative therapies are generally described as one-time, or short-term, treatments for patients with diseases that would otherwise require treatment for prolonged periods of time and often for the duration of a patient's life.
- Curative therapies often have high upfront costs that may strain short-term budgets, which creates a significant challenge for payers of healthcare services.[1]
  - Innovative payment models are a key component to accelerate patient access and maximize the potential long-term benefits of curative therapies.[2]
- Coverage decisions for curative therapies may differ due to the often unknown long-term benefit/risk profile of newly approved curative therapies.
- Given the recent increases in the number of curative therapies in the pipeline, the Institute for Clinical and Economic Review (ICER) published a "Value Assessment Methods and Pricing Recommendations for Potential Cures: A Technical Brief," which outlines considerations for the assessment of potential curative therapies.[3]

OBJECTIVE

- To evaluate familiarity with curative therapies, curative therapy decision patterns, and utilization of ICER's technical brief among US payers.

METHODS

- Data were collected from qualified users registered with FormularyDecisions (FD) using a double-blinded, 10-item survey. The survey was fielded in March 2020.
  - FD is an online platform that facilitates access to critical product evidence and supports a bidirectional exchange between payers and manufacturers via syndicated surveys and other innovative methods.[4]
  - FD includes over 2,100 registered healthcare decision makers (HCDMs) representing the US payer community, including managed care organizations, pharmacy benefit managers (PBMs), hospitals, and government entities. Effect of pre-approval information upload on HCDM activity was measured by comparing mean Product Page hits during a 24-month period before the Prep Kit upload date vs mean Product Page hits during a 24-month period after the Prep Kit upload date.

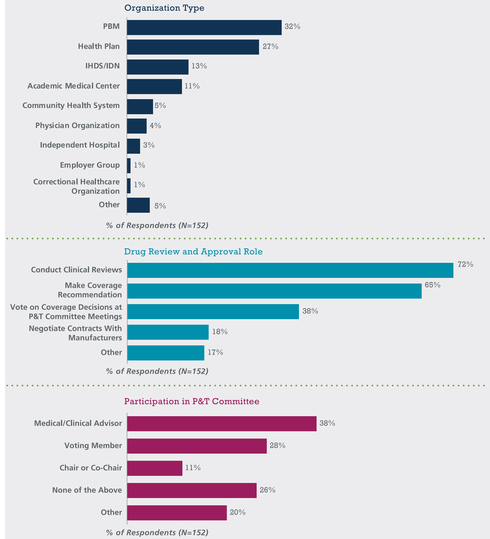
RESULTS

SUMMARY OF RESPONDENTS

- Respondents included 152 advisors. Among the most common respondent types were representatives of national (50%) and regional (50%) organizations, which included PBMs (32%), health plans (27%), and integrated health delivery system (IHDS)/integrated delivery networks (IDNs) (13%) (**Figure 1**).
- A majority of respondents were either clinical pharmacists (57%) or pharmacy directors (20%) within their organizations.
  - Roles of respondents in the drug review and approval process within their organizations included conducting clinical reviews (72%), making coverage recommendations (65%), and voting on coverage decisions (38%) (**Figure 1**).
- About a third (38%) of respondents served as medical/clinical advisors for Pharmacy and Therapeutics (P&T) Committees, while approximately another third (28%) were voting members of P&T Committees (**Figure 1**).

RESULTS (cont.)

Figure 1. Respondent Summary



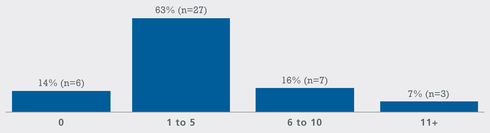
FAMILIARITY WITH CURATIVE THERAPIES AND COVERAGE DECISIONS TRENDS

- A majority (83%) of respondents reported being "extremely/very" or "somewhat" familiar and knowledgeable about curative therapies.
- Only 5% of respondents reported their organizations had a standard definition for curative therapies, while all other respondents reported their organizations either did not have a definition (51%) or they did not know if their organization had a definition (45%) for curative therapies.
  - The top reasons reported for not having a standard definition for curative therapies included only reviewing curative therapies on a case-by-case basis and/or discussions about curative therapies had only recently started (40%) (**Table 1**).
- A majority (72%, n=109) of respondents did not know the number of coverage decisions made for curative therapies by their organizations over the past year.
  - Of those who were aware (28%, N=43), the majority reported between 1–5 coverage decisions made in the last year (63%, n=27) (**Figure 2**).

Table 1. Top Reasons Respondents' Organization Does Not Have Standard Definition of Curative Therapies

Reasons for Lack of Definition for Curative Therapies	%, (n) N=77
Discussion started only recently/Case by case for now	40% (31)
Follow NCCN/ASCO/Clinical pathways, FDA, health plan definitions, compendia	10% (8)
Definitions are not needed/Policies don't require it/Hospital leadership review	9% (7)
Different definitions by disease states/cancers	8% (6)
Field too broad	3% (2)
Not a priority	3% (2)
Population too small for CT	3% (2)
No category for curative or not	3% (2)
Evidence-based medicine resources	<1% (1)
Curative may be defined to differing degrees	<1% (1)
Evaluate endpoints and long-term data	<1% (1)
Manufacturers will differentiate	<1% (1)
No guidance from regulatory bodies so far	<1% (1)
Too new	<1% (1)
Clients make own decisions	<1% (1)
Don't know/Not sure	13% (10)

Figure 2. Coverage Decisions for Curative Therapies

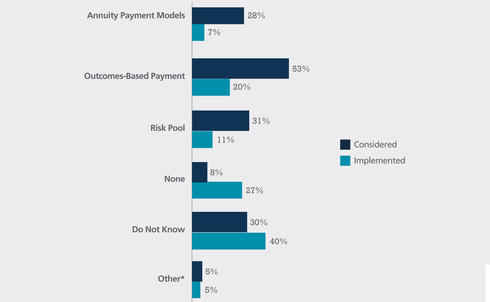


Total respondents: N=43

Q2. For approximately how many curative therapies has your organization made a coverage decision in the last year?

- Outcomes-based payment models were the most frequently considered (53%) and implemented (20%) alternate payment or reimbursement model for curative therapies (**Figure 3**).
- The 3 biggest drivers in decision making for curative therapies were cost-effectiveness research (77%), comparator data (75%), and real-world evidence post-launch (72%) (**Figure 4**).

Figure 3. Alternate Payment or Reimbursement Models



N=152

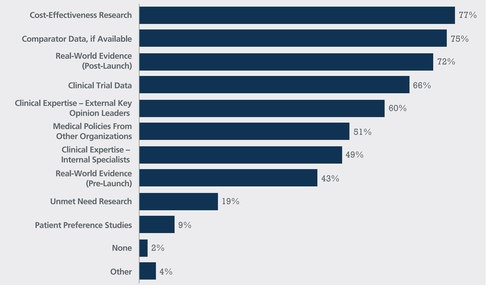
Q3: Which alternate payment or reimbursement models has your organization considered to mitigate the high costs of curative therapies?

Other: 340B pricing, gene therapy, global case rates, max cost for other rare disorders, reinsurance

Q4. Which alternate payment or reimbursement models has your organization implemented to mitigate the high costs of curative therapies?

*Other: global case rates, MUEs, coding reviews, reinsurance

Figure 4. Drivers of Decision Making for Curative Therapies



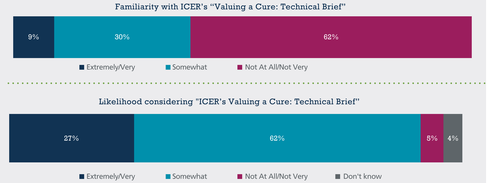
N=152

Q5. Which of the following would be useful to your organization to help drive decision making for curative therapies?

FAMILIARITY WITH ICER'S "VALUING A CURE: TECHNICAL BRIEF"

- The majority (62%) of respondents were "not at all/not very" familiar with ICER's technical brief; however, nearly all (89%) of respondents reported they would be "extremely/very likely" or "somewhat likely" to consider the brief to assess value of curative therapies (**Figure 5**).

Figure 5. Familiarity With, and Future Consideration of, ICER's "Valuing a Cure: Technical Brief"



N=152

Q6. How familiar are you with ICER's "Valuing a Cure: Technical Brief"?

Q7. How likely are you to consider using ICER's Valuing a Cure: Technical Brief" methodology to assess value of a curative therapy?

LIMITATIONS

- Caution should be taken when generalizing results from this survey; results are based on a small sample of recruited HCDMs and are primarily qualitative in nature.

CONCLUSIONS

- Although respondents were generally familiar with curative therapies, very few respondents reported having standardized definitions of curative therapies within their respective organizations.
- Manufacturers should work closely with payers to better develop definitions of curative therapies given these definitions may impact payer coverage decisions, consideration of alternate payment models, and value assessment methods.
- With an increasing number of curative therapies in the pipeline, future research should examine factors that impact payer decision making for curative therapies.

REFERENCES

1. Daniel G, Leschly N, Marrazzo J, McClellan MB. Advancing gene therapies and curative health care through value-based payment reform. *Health Affairs Blog*. 2017. https://www.healthaffairs.org/do/10.1377/hblog20171027.83602/full/.
2. Gottlieb S. Harnessing the curative potential of genomic technologies. US Food and Drug Administration. 2018. https://www.fda.gov/news-events/speeches-fda-officials/harnessing-curative-potential-genomic-technologies-09282018.
3. Chapman R, Kumar V, Samur S, Zaim R, Segel C, Pearson S. Value Assessment Methods and Pricing Recommendations for Potential Cures: A Technical Brief. Institute for Clinical and Economic Review. 2019. https://icer review.org/wp content/uploads/2019/08/ICER_TechnicalBrief_SSTs_080619-1.pdf.
4. FormularyDecisions. https://www.formularydecisions.com/global/dynamic.aspx.

***Formerly with Xcenda at time of abstract submission.**

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