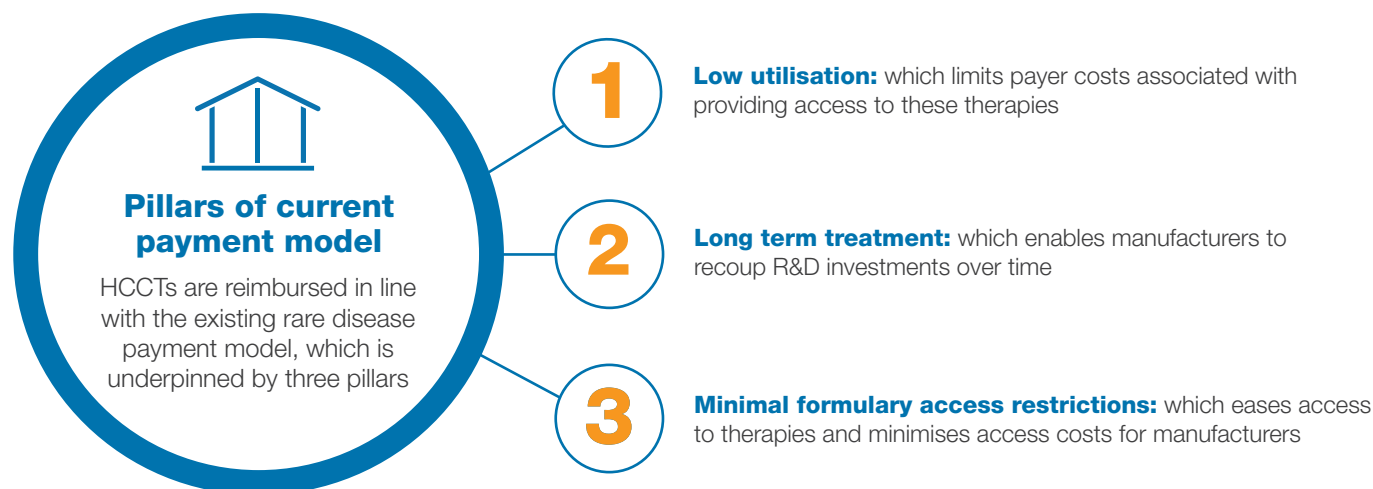


# Impact of High-Cost Curative Therapies (HCCTs) on Existing Business Models



Unlike traditional rare disease therapies,  
**HCCTs pose a different set of challenges to the existing payment models**



## Ultra High Upfront Costs

**Costs associated with HCCTs are typically high** (e.g., CAR-T therapies – Kymriah and Yescarta cost in excess of \$300,000 / patient)

**Immediate / upfront budget impact;** HCCTs tend to have ‘front-loaded’ budget impact (first year) that fades over time



## Increased Utilization

**Increase in number of patients on HCCTs** due to broader use of HCCTs and expected increase in availability (currently over 800 therapies in the pipeline)



## Cost-Effectiveness Assessment

**Difficulty assessing the curative value and cost-effectiveness of HCCTs** due to lack of long term safety / efficacy data

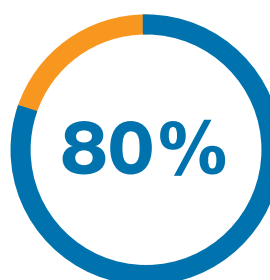
**HCCTs pose challenges that impact payers and manufacturers**  
in several different but related ways

## Key Research Takeaways\*



“Curative therapies have a significant 1st year budget impact that fades away in subsequent years”

■ Agree ■ Neutral/Disagree



“The potential budget impact of curative genetic therapies merits a new way of thinking about how we pay for HCCTs”

■ Agree ■ Neutral/Disagree

\*based on interviews with n=10 payers

## HCCT Challenges



### Payer Challenges

#### Front-Loaded Budget Impact

Budget impact of HCCTs is concentrated in the initial years of launch due to patient warehousing and the curative nature of HCCTs

Cost mitigation through increased premiums and patient cost-sharing is unsustainable

#### Difficulty Evaluating HCCT Benefit

70%\* of respondents felt manufacturers do not have or share robust long term data needed for evaluating the cost-effectiveness of HCCTs

Similarly, HCCT outcomes data is often inadequate to evaluate cost-effectiveness and long-term outcomes



### Manufacturer Challenges

#### Shorter Product Lifecycle

HCCTs typically tap into 'warehouse' of patients; thus peak sales occur much earlier in product lifecycle

Shorter commercial window increases the need for greater value capture at launch

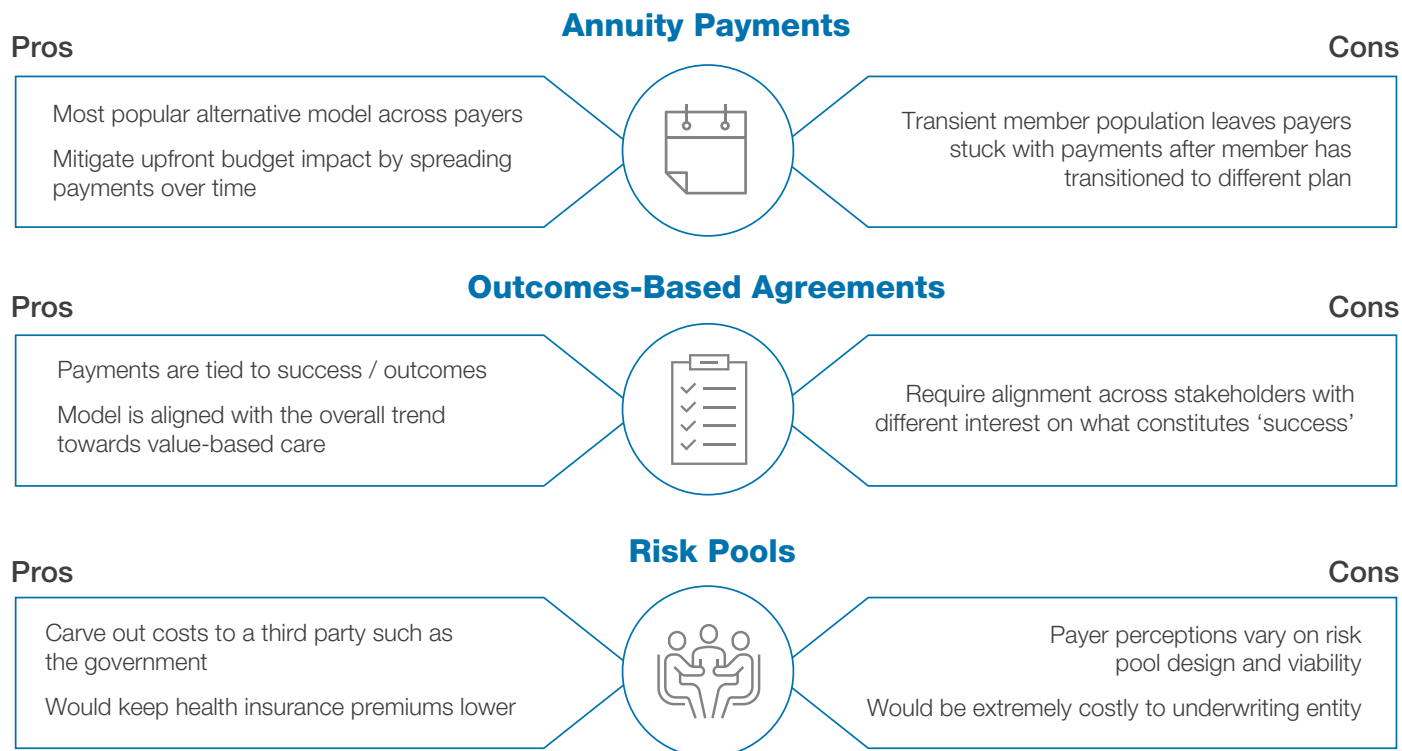
#### Higher Evidence Barrier

Given HCCT costs, manufacturers face greater burden in communicating 'curative' value story to payers

There is a need to fortify patient-level data management and HEOR capabilities, as well as leverage the support of patient advocacy groups

\*based on interviews with n=10 payers

**Payers suggested alternative payment models** that would potentially address challenges with the current model but also raise several new challenges



## HCCTs will force payers and manufacturers to reconsider their business models

Early and collaborative multi-stakeholder engagement focused on the design of a new model that maximizes the lifetime value of HCCTs or manufacturers and mitigates the budgetary impact of HCCTs to payers will facilitate sustainable long-term uptake of HCCTs