IDENTIFYING THE KEY CHALLENGES AFFECTING THE REIMBURSEMENT OF CAR-T THERAPIES IN THE EU5 USING A WEB-BASED PORTAL TO ENGAGE PAYERS

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INTRODUCTION

Chimeric antigen receptors T-cell therapies (CAR-T) have demonstrated excellent remission rates in hematological cancers but are associated with a high treatment cost and logistical burden. In addition, regulatory approvals have been based on data from single-arm Phase II trials; payers therefore need to reconcile concerns related to uncertain clinical benefit and budget impact.

This research was completed to identify the key challenges CAR-T therapies pose to payers in the EU5 and how these challenges can be mitigated.

METHODOLOGY

A web-based survey was administered through the Rapid Payer Response™ online portal (RPR®) to 25 payers with experience in HTA and reimbursement decision-making for oncology treatments in France, Germany, Italy, Spain, and the UK; MAT engaged 5 payers per country.

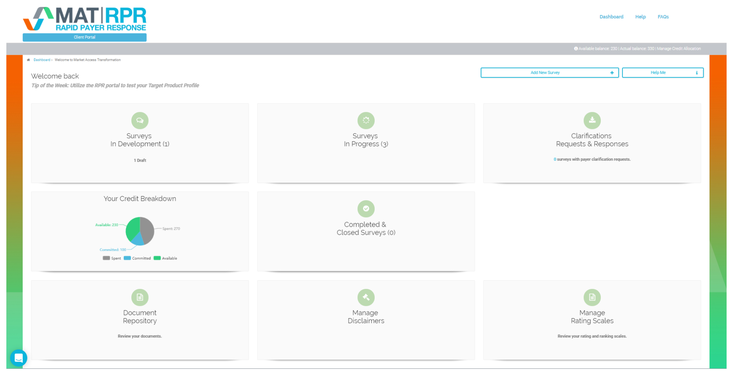

Figure 1: Rapid Payer Response™ portal

Payer profiles included former-members of NICE and the SMC in the UK, ex-CEPS and ex-TC payers in France, ex-GBA and SHI payers in Germany and both national and regional level payers in Italy and Spain. Responses were collected through RPR® in 5 days and analyzed via Microsoft™ Excel.

RESULTS

What are the key challenges to CAR-T reimbursement in Europe?

Payers agreed that the lack of comparative Phase III data, limited long-term outcomes data (particularly safety outcomes) and the high cost of treatment were the central challenges to reimbursement; furthermore, payers highlighted that on top of the cost of treatment, there are also significant costs associated with infrastructure, diagnostics, staffing and monitoring patients.

> *"The other major consideration is the companion costs associated with the therapy which will be significant - some in relation to establishing supporting staffing, infrastructure, diagnostics and monitoring."* ***Former Senior Advisor, SMC, UK***

How will payers mitigate the high treatment cost of CAR-T therapies?

Despite the initial cost burden, payers in Germany, France, Italy and the UK considered that up-front payment for CAR-T therapy was likely, given the simplicity in terms of administration; payers in the UK, Spain and Italy also considered outcomes-based models or risk-sharing to be a favorable option for CAR-Ts, especially since these models are already in place for expensive therapies. Lifetime lease and service model options were considered less likely to be utilized.

Reimbursement models	Advantages	Disadvantages
Lifetime lease	• *No upfront payment, simple implementation* • *Manageable budget-impact*	• *Lack of visibility on the total cost* • *Duration needs to be negotiated*
Risk-sharing and outcomes-based agreements	• *Currently already in use in many markets* • *Potential to reduce overall spend* • *Represents good return on investment; i.e. only the patients that benefit are paid for*	• *Requires monitoring and handling patient specific data* • *Requires alignment on clinical definitions such as full, partial, incomplete response*
Service model	• *Could lead to lower overall price*	• *Patient by patient basis* • *Timescales to confirm cost avoidance could be significant*
Full up-front payment	• *Clear process* • *Easy administration procedure*	• *High risk for payer* • *Substantial up-front cost*

Table 1: The main advantages and disadvantages of potential reimbursement models for CAR-T therapies

What role are European registries expected to play in tracking CAR-T therapy outcomes?

Payers across all markets acknowledged the value of registries to obtain supportive RWE on CAR-T outcomes. Payers considered that although it would be feasible to establish new registries (there are various registries across the EU5 already) this would require significant initial investment, in terms of infrastructure, protocol development and training, and require sustained funding to maintain. Payers also cautioned that manufacturers should not view RWE as a substitute for a robust comparative Phase III trial.

Registry data should not be viewed as an alternative to Phase III trial data

Registries require substantial funding to establish and maintain

Requirement for protocols to ensure consistency in data collection nationally across registries

Figure 2: Payer concerns regarding the use of registries to track CAR-T outcomes data

CONCLUSIONS

Despite providing a valuable therapeutic option, CAR-Ts present a unique challenge to both payers and manufacturers. Manufacturers will need to actively engage with payers and physicians to develop practical solutions to the logistical and cost-based challenges. Up-front payment and outcomes-based agreements are expected to be the most likely reimbursement models utilized in the EU5, the latter of which will require robust real-world outcomes data. Payers acknowledged that registries are a key source of RWE but that they require considerable resource and infrastructure to establish and maintain.

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