Will CAR-T cell therapies change how drugs are priced and reimbursed?

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Background

- The first two CAR-T cell therapies, Kymriah® (tisagenlecleucel) and Yescarta® (axicabtagene ciloleucel) received European marketing authorization in August 2018 (Kymriah® for ALL and DLBCL and Yescarta® for DLBCL/PMBCL)
- These therapies offer transformational benefits for patients with life-threatening cancers with few treatment alternatives from a single treatment administration
- However, there has been significant payer concern about the affordability of these therapies, which can be cost-effective at very high patient prices. This research aims to evaluate how payer bodies have assessed these therapies to date

Methods

- All publicly-available published HTA evaluations of Kymriah® and Yescarta® by NICE, SMC, G-BA, HAS, NT, and ZIN were screened and key information extracted (to 08/06/2019)

Results

- Seventeen completed HTA evaluations of CAR-T cell therapies in scope countries were identified (6 Kymriah® ALL, 5 Kymriah® DLBCL, 4 Yescarta® DLBCL/PMBCL, 1 Yescarta® DLBCL, and 1 Yescarta® PMBCL) which were completed at an average of 5.4 months after European marketing authorisation
  - NICE recommended both Kymriah® (ALL and DLBCL) and Yescarta® (DLBCL/PMBCL) via the CDF. An additional assessment for Yescarta® (MCL) was identified but omitted from the analysis as the appraisal is anticipated to begin in 2020
  - Only two negative appraisals were identified (SMC: Kymriah® in DLBCL and Yescarta® in DLBCL/PMBCL)
  - Under HAS, Kymriah® attained ASMR III (ALL) and ASMR IV (DLBCL), and Yescarta® an ASMR III (DLBCL/PMBCL)
  - The G-BA deemed that Kymriah® (ALL and DLBCL) and Yescarta® (DLBCL/PMBCL) offered an unquantifiable additional benefit, but this resolution was time-limited for 1 year
  - The NLT accepted Kymriah® (ALL) but delayed their recommendations for Kymriah (DLBCL) and Yescarta® (DLBCL/PMBCL) to the county council organizations for an unspecified period
  - ZIN accepted Kymriah® (ALL and DLBCL) and Yescarta® (DLBCL/PMBCL) under a reimbursement lock mechanism due to the anticipated high costs of these treatments

Table 1: HTA appraisal/P&R decision maker outcome of Kymriah® and Yescarta®

	KYMRIAH®		YESCARTA®	
EC-approval date	22nd August 2018		23rd August 2018	
Indication	ALL	DLBCL	DLBCL	PMBCL
HTA appraisal/P&R decision maker outcome				
NICE (England)	Recommended via CDF	Recommended via CDF	Recommended via CDF	
SMC (Scotland)	Accepted	Not recommended*	Not recommended**	
HAS (France)	SMR: Important ASMR: III	SMR: Important ASMR: IV	SMR: Important ASMR: III	
G-BA (Germany)	Unquantifiable benefit‡	Unquantifiable benefit†	Unquantifiable benefit†	Unquantifiable benefit†
NT (Sweden)	Accepted	Decision deferred	Decision deferred	Decision deferred
ZIN (Netherlands)	Accepted (reimbursement lock)	Accepted (reimbursement lock)‡	Accepted (reimbursement lock)‡	

* Publication of resubmission due 9th September 2019
** Publication of resubmission due Q3 2019
† Time-limited resolution: the G-BA requires Novartis and Gilead to submit new data on Kymriah® and Yescarta® for a new assessment in 2020
‡ Placed in a lock for expensive medicines due to expected high costs

KEY: = positive decision = restricted/conditional decision = negative decision = no decision

Conclusions

- Despite concerns over the affordability of CAR-T cell therapies, to date they have generally received positive assessments by European HTA bodies, which have enabled patient access
- Nevertheless, many of these positive assessments are temporary and conditional on future data generation (both G-BA assessment were time-limited and all three NICE assessments are via the CDF)
  - Whether the final subsequent NICE and G-BA appraisals will result in positive assessments or whether patient access will only be temporary still remains to be determined

ALL: Acute lymphocytic leukemia; ASMR: Amélioration du service médical rendu; CAR-T: Chimeric antigen receptor T cells; CDF: Cancer Drugs Fund; DLBCL: Diffuse large B-cell lymphoma; G-BA: Gemeinsamer Bundesausschuss; HAS: Haute Autorité de Santé; HTA: Health technology assessment; MCL: Mantle cell carcinoma; NICE: National Institute of Health and Care Excellence; NLT: New Pharmaceutical Product Therapies; PMBCL: Primary mediastinal large B-cell lymphoma; P&R: Pricing and reimbursement; SMC: Scottish Medicines Consortium; SMR: Service médical rendu; UK: United Kingdom

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