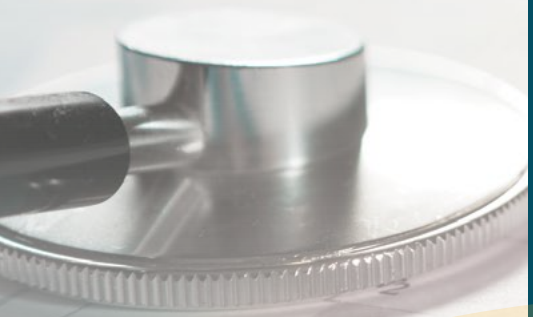
CASE STUDY

Using real-world data to assess the cost and clinical effectiveness of CAR-T therapy



OVERVIEW

There is an increased demand for European health technology accessors (HTAs) to make accurate assessments of the long-term value and performance of novel therapies from smaller datasets and shorter outcomes. As such, discovering new approaches to reduce the time and cost of clinical development are of the utmost importance.

Data from initial clinical trials investigating chimeric antigen receptor T-cell therapies (CAR-T) yield promising results[1,2] with two CAR-T therapies already being approved to treat acute lymphoblastic leukemia (ALL); however, the extent to which current CAR-T therapies perform compared to the current standard of care (SOC) remains to be fully determined.

CHALLENGE

Orphan conditions, such as ALL, concern inherently small patient populations. In such cases, clinical trials designed to determine the efficacy and safety of new therapies are not feasible - especially considering that CAR-T therapy is only indicated as a second-line 'salvage' treatment in patients displayed no response to chemotherapy and those who experienced a relapse following the SOC. Therefore, pharmaceutical companies and HTAs are faced with the challenge of determining the efficacy of new treatments in a time-efficient manner.

In the current study, Nashville Biosciences partnered with Vital Transformation to conduct a retrospective study to mine existing electronic health records (EHRs) from patients that had participated in CAR-T clinical trials and compare the results with patients treated with the SOC for ALL.

Nashville Biosciences

VitalTransformation

CAR-T Accuracy

BENEFITS

Novel Approaches to Modern Medicine: Nashville Biosciences developed an analysis procedure utilizing small sample sizes that can reliably link real-world data on clinical effectiveness, disease severity and treatment cost analysis. This is essential for informing payers of the total cost and long-term value effectiveness compared with conventional therapy, especially in small disease populations.

Efficiency: Our approach expedites the process of delivering new therapies to the market by saving both time and the cost of conducting long-term clinical trials. Performing retrospective analysis on existing EHRs could improve R&D efficiency and also identify novel candidates who could benefit from new therapies.

Nashville Biosciences®

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Leveraging Vanderbilt University Innovation™

SOLUTION AND RESULTS

Our goal was to demonstrate a novel methodology harnessing a longitudinal real-world dataset extracted from EHRs of a medical center serving as a CAR-T clinical trial site and to develop an accurate analysis of the performance of CAR-T compared to the SOC. In order to accomplish this, Nashville Biosciences utilized a database containing de-identified versions of live EHRs with nearly 3 million patient records. Although inherently small patient populations for this orphan indication exist, our approach allowed us to mine EHR data from patients that had participated in CAR-T clinical trials, enabling us to build up a longitudinal record with a median of 3 years of outcomes. Nashville Biosciences conducted a retrospective analysis to provide anonymized, subject-level data on demographics, diagnoses, tumor histology, and relapse-free survivability, as well as the total average costs of hospital procedures and medicines per patients for treating ALL with CAR-T and the SOC.

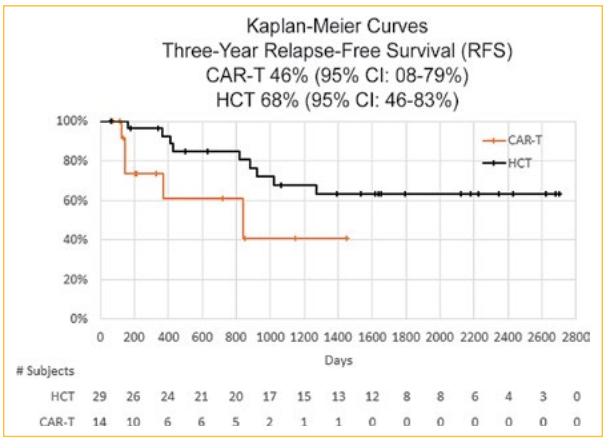

Figure 1.* Kaplan-Meier curves and 95% CIs for relapse-free survival in the CAR-T and HCT cohorts. Data censoring (slash) represents the end of available data. CAR-T, chimeric antigen receptor T-cell; HCT, haematopoietic cell transplant.

Table 1*

	CAR-T cohort: average annual costs (US$)					
	Infusion	Year 1	Year 2	Year 3*	Year 4*	NPV 3% total costs
Hospital procedures	50676	41740	14022	4711	1582	106926
Medicines (excluding price of CAR-T)	5542	6351	507	40	3	11870
Total treatment cost (excluding price for CAR-T)	56217	48091	14529	4751	1586	118795
Total treatment cost (including price for CAR-T)	531217	48091	14529	4791	1589	579999
	HCT cohort: average annual costs (US$)					NPV 3%
	Infusion	Year 1	Year 2	Year 3	Year 4	total costs
Hospital procedures	17241	9687	2617	22589	24007	69043
Medicines	135196	74465	32040	2223	1480	234022
Total treatment cost	152437	84152	34657	24812	25487	303065

*The CAR-T infusion period covers the time period from 8 weeks before to 6 weeks after the CAR-T infusion date: year 1 data excludes infusion costs. The HCT period cover the time period from 15 weeks before to 8 weeks after the date of the procedure.

For years 3 and 4, CAR-T costs were extrapolated

CAR-T, chimeric antigen receptor T-cell; HCT, haematopoietic cell transplant; NPV, net present value

Our approach involves the creative use of real-world data by providing actual (not estimated or extrapolated) cost assessments and outcomes measures of a new orphan therapy. This work presents a novel approach to quantifying the comparative value and effectiveness of new therapies to help meet the increasing challenges that are being faced by payers and HTAs.

References:

*BMJ Evid Based Med. 2019 Jul 17. pii: bmjebm-2019-111226. doi: 10.1136/bmjebm-2019-111226. [Epub ahead of print]

[1]Maude SL, et al. Tisagenlecleucel in Children and Young Adults with B-Cell Lymphoblastic Leukemia. N Engl J Med 2018;378:439-48.

[2]Neelapu SS, et al. Axicabtagene Ciloleucel CAR T-Cell Therapy in Refractory Large B-Cell Lymphoma. N Engl J Med 2017;377:2531-44.