

# METHODOLOGICAL ANALYSIS OF PHARMACOECONOMIC STUDIES IN CAR-T: A SYSTEMATIC REVIEW

I.Aldalur, J.M. Guiu, C.F. Lastra, P. Modamio, E. Mariño

Consorci de Salut i Social de Catalunya (CSC), Barcelona, España

Clinical Pharmacy and Pharmaceutical Care Unit, Faculty of Pharmacy and Food Sciences, University of Barcelona, Barcelona, España

## BACKGROUND AND IMPORTANCE

Chimeric antigen receptor T-cell therapies (CAR-T) are based on the ex-vivo modification of T-lymphocytes for the expression of an antigen receptor that provides the specific union with tumour cells for their consequent destruction. CAR-T introduction into clinical practices present challenges from a clinical and economic perspective. Traditional pharmacoeconomic studies may be limited in their ability to act as a valid decision-making tool in the access management of CAR-T and alternative methodological approaches may have to be considered.

## OBJECTIVE

Elaboration of a systematic review of CAR-T pharmacoeconomic studies to determine if traditional pharmacoeconomic studies represent a valid tool for decision-making in the access management of CAR-T.

## METHODOLOGY

### Systematic search in:

- Scopus
- Pubmed
- Cochrane Library

### Using terms related with:

- CAR-T
- Pharmacoeconomics

### Quality evaluation by:

- CHEERS
- Drummond

### Excluded:

All other scientific documents

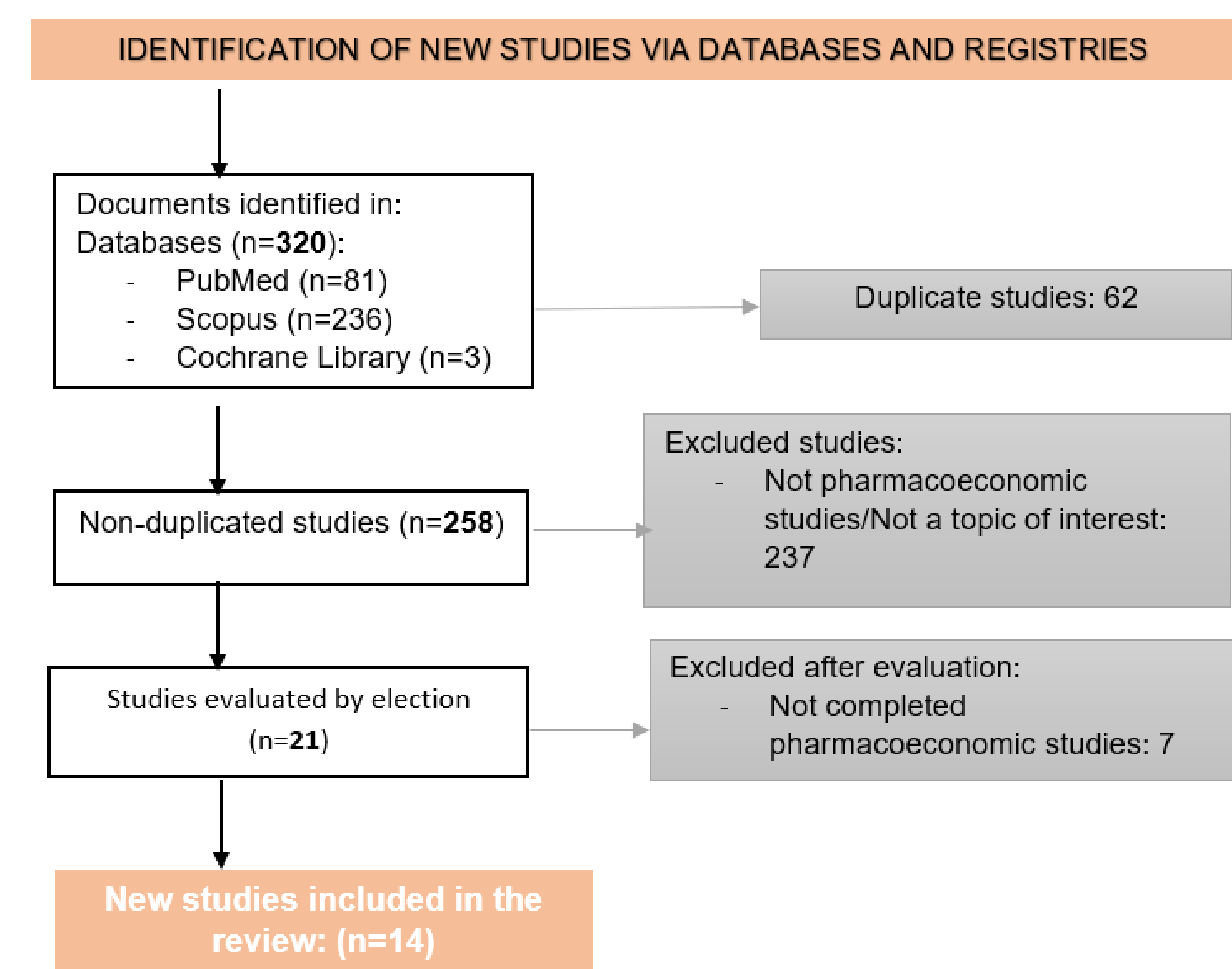
### Included:

Published articles and accepted manuscripts written in English or Spanish until 15 August 2021

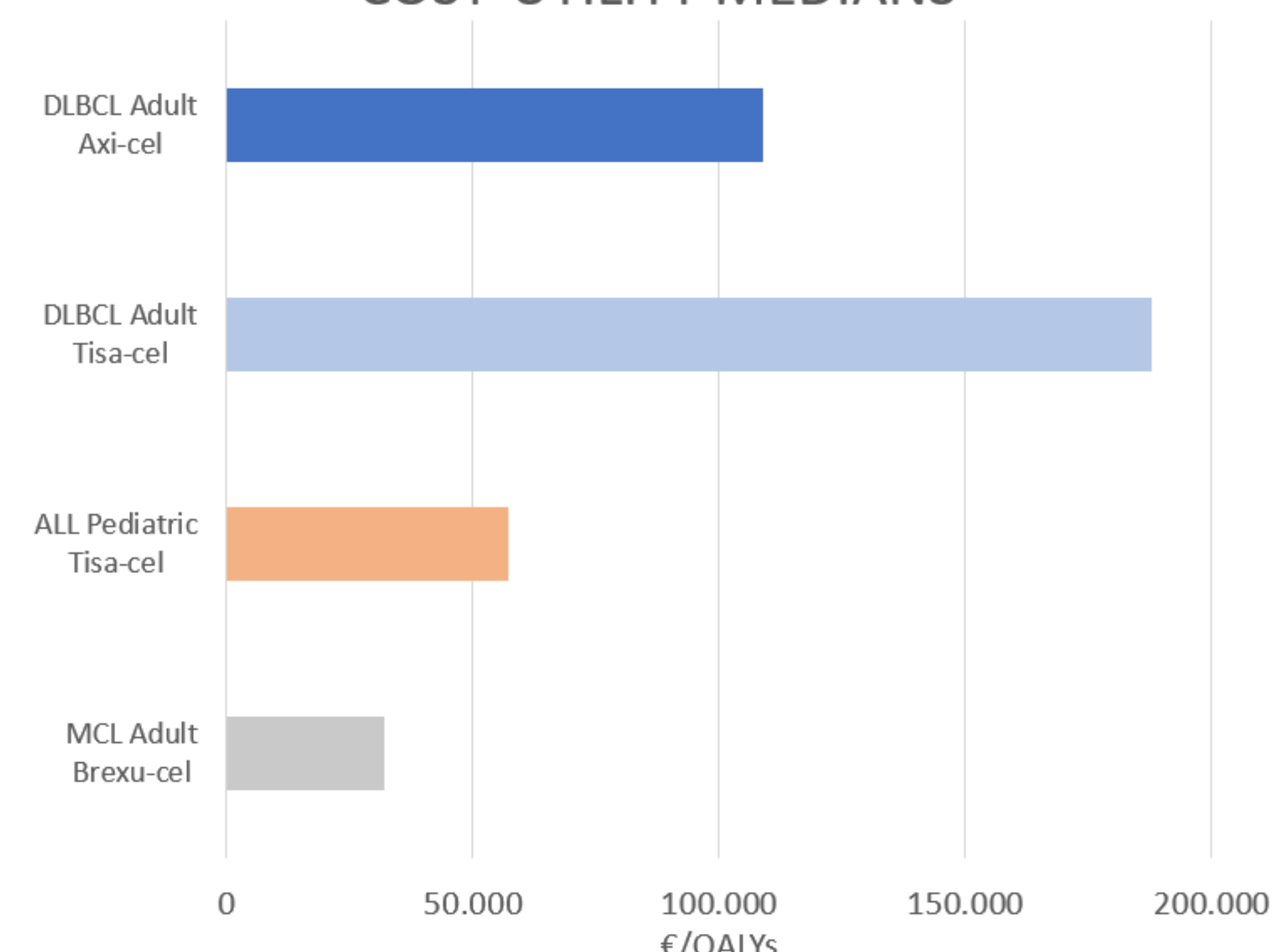
## RESULTS

The most studied CAR-T drug was tisagenlecleucel for diffuse large B-cell lymphoma in adults, with a median cost per QALY of 291924,51€. CAR-T therapies represent a clinically and potentially cost-effective therapeutic alternative. The quality of the identified studies was good according to the quality assessment scores.

| AUTHOR YEAR OF PUBLICATION | COUNTRY       | CLINICAL INDICATION | POPULATION                | CAR-T                |
|----------------------------|---------------|---------------------|---------------------------|----------------------|
| Wakase et al, 2021         | Japan         | ALL r/r             | Pediatric and young adult | Tisa-cel             |
| Ribera et al, 2020         | Spain         | ALL r/r             | Pediatric and young adult | Tisa-cel             |
| Thielen et al, 2020        | Netherlands   | ALL r/r             | Pediatric                 | Tisa-cel             |
| Furzer et al, 2020         | Canada        | ALL r/r             | Pediatric                 | Tisa-cel             |
| Whittington et al, 2018    | United States | ALL r/r             | Pediatric                 | Tisa-cel             |
| Sarkar et al, 2018         | United States | ALL r/r             | Pediatric                 | Tisa-cel             |
| Lin et al, 2018            | United States | ALL r/r             | Pediatric                 | Tisa-cel             |
| Wakase et al, 2021         | Japan         | DLBCL r/r           | Adult                     | Tisa-cel             |
| Cher et al, 2020           | Singapur      | DLBCL r/r           | Adult                     | Tisa-cel             |
| Lin et al, 2019            | United States | DLBCL r/r           | Adult                     | Tisa-cel<br>Axi-celo |
| Liu et al, 2021            | United States | DLBCL r/r           | Adult                     | Axi-celo             |
| ●Whittington et al, 2019   | United States | DLBCL r/r           | Adult                     | Axi-celo             |
| ●Whittington et al, 2019   |               |                     |                           |                      |
| ●Whittington et al, 2019   |               |                     |                           |                      |
| ●Whittington et al, 2019   |               |                     |                           |                      |
| ●Whittington et al, 2019   |               |                     |                           |                      |
| Roth et al, 2018           | United States | DLBCL r/r           | Adult                     | Axi-celo             |
| Simons et al, 2021         | United States | MCL                 | Adult                     | Brexu-cel            |



## COST-UTILITY MEDIANS



## CONCLUSION

Cost-effectiveness of CAR-T therapies depends on its long-term results, the duration of the study conducted, and the cure rate used of the clinical study. Because of that, pharmacoeconomic studies in CAR-T exhibit certain limitations and could not be robust tools for decision making solely based on their findings. There is a need to develop pharmacoeconomic methods that can avoid the uncertainty of many assumptions and incorporate more data, including real-life data.

## ABBREVIATIONS

QALYs: Quality-Adjusted Life-Year; MCL Adult Brexu-cel: Results of brexucabtagene autoleucel for mantle cell lymphoma in adult population; ALL Pediatric Tisa-cel: Results of tisagenlecleucel for diffuse acute lymphoblastic leukemia in pediatric population; DLBCL Adult Tisa-cel: Results of tisagenlecleucel for diffuse large B-cell lymphoma in adult population; DLBCL Adult Axi-cel: Results of axicabtagene ciloleucel for diffuse large B-cell lymphoma in adult population