

## CAR-T Therapy in Haematological Malignancy: Update Bulletin #2

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## **Abstracts**

This edition presents leading US and EU key opinion leader (KOL) views on recent developments in the CAR-T therapy space. Topics covered include expert opinions on the FDA approvals of Novartis' Kymriah (tisagenlecleucel-T; CTL019) and Gilead/Kite's Yescarta (axicabtagene ciloleucel; KTE-C19) for the treatment of patients with paediatric acute lymphoblastic leukaemia (ALL) and adult diffuse large B-cell lymphoma (DLBCL) respectively. KOLs discuss the three-month data reported in the DLBCL setting for Juno Therapeutics' and Celgene's JCAR017 in the TRANSCEND study and also offer their candid insights on allogeneic CAR-T therapies such as Cellectis and Servier/Pfizer'sUniversal Chimeric Antigen Receptor T-cells (UCARTs) and the recent progress made in diseases such as multiple myeloma and acute myeloid leukaemia (AML).

**Business Questions:** 

According to the KOLs, how are Kymriah and Yescarta faring on the US market so far?

What are the experts' initial views on the cost of the two CAR-T therapies?

In terms of distribution and centre capacity, how do the KOLs view the roll-out of the two CAR-T therapies? Are there any major setbacks to successful roll-out?

What challenges lie ahead in terms of infrastructural and production capacity?

Do the experts have any remaining concerns about the manufacturing processes for CAR-T?



What challenges face the two CAR-T therapies if and when they are approved in Europe?

What do KOLs think CAR-T companies can do to help in terms of further training, support and education?

Are KOLs impressed with the three-month data for Juno and Celgene's JCAR017 in the TRANSCEND study?

How does JCAR017 match up to Kymriah and Yescarta so far in terms of efficacy and safety?

Do the benefits of a defined composition CAR-T product outweigh the potential disadvantages?

How do the KOLs view allogeneic CAR-T cells, in particular Cellectis/Servier/Pfizer's UCART programme?

What are KOLs' main concerns about 'off-the-shelf' allogeneic CAR-T approaches?

How do KOLs view the safety issues with UCART123 in patients with AML? Where does the opportunity for CAR-T exist in AML?

With Bluebird Bio reporting early data for anti-BCMA CAR-T cells in multiple myeloma, how do the experts view the recent progress made with this novel CAR-T approach?



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