References

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Table S1 Summary of clinical trials using CAR T-cells in children and adolescents with relapsed and refractory B-acute lymphoblastic leukemia

Clinical trial	Antigen target	Co-stimulatory domain	Enrolled (N)	Treated (N)	Median age in years (range)	CR/CRi (% of treated)	MRD negative CR (% of CR)	Relapse (% of CR)	Antigen negative relapse (% of relapse)	HSCT after treatment (% of CR)	Duration of B-cell aplasia	Event-free survival	Overall survival	Median follow-up, months (range)
CART19* (NCT01626495, NCT01029366) (9)	CD19	4-1BB	NA	30	14 [5–60]	27 [90]	22/25 [88]	7 [26]	3 [43]	3 [11]	Up to 1 year in responders	67% at 6 m	78% at 6 m	7 [1–24]
ELIANA (first report)** (NCT02435849) (13)	CD19	4-1BB	92	75	11 [3–23]	61 [81]	61 [100]	20 [33]	15/16 [94]	8 [13]	83% at 6 m	50% at 12 m	76% at 12 m	13.1
ELIANA (second report)** (NCT02435849) (14)	CD19	4-1BB	97	79	11 [3–24]	65 [82]	64 [98]	24[#] [37]	19/21 [90]	11 [17]	59% at 24 m	44% at 36 m	63% at 36 m	38.8
NCI (first report)* (NCT01593696) (10)	CD19	CD28	NA	21	14 [5–27]	14/20 [70]	12 [86]	2 [14]	2 [100]	10 [71]	13/14 lost BCA around d28	NA	51.6% at 9.7 m	10
NCI (second report)* (NCT01593696) (11)	CD19	CD28	NA	53	13.5 [4.3–30.4]	31/50 [62]	28 [90]	12 [39]	3/6 [50]	21 MRD negative CR [75]	NA	38% at 6 m	Med 10.5 m	57.6 [42–86.4]
huCART19* (NCT02374333) (19)	CD19 (humanized)	4-1BB	43	41 CAR naïve	10.3 [1.7–29.1]	39/39 [100]	39 [100]	12 [31]	6 [50]	4 [10]	85% at 6 m	72% at 24 m	88% at 24 m	34.6 [11.6–49.9]
			37	33 prior CD19 CAR exposure	12.6 [4.4–24.8]	21 [64]	18 [86]	8 [38]	1 [12.5]	1 [5]	42% at 6 m	37% at 24 m	55% at 24 m	21.2 [8.9–55.0]
PLAT-02 (NCT02028455) (15)	CD19	4-1BB	45	43	12.2 [1.3–25.3]	40 [93]	40 [100]	18 [45]	7 [39]	11 [28]	Med exp 3 m	50.8% at 12 m	69.5% at 12 m	9.6 [2–28]
ZUMA-4 (NCT02625480) (12)	CD19	CD28	31	24	13.5 [3–20]	16 [67]	16 [100]	NA	NA	14 [88]	No CAR gene at 3 m	NA	72.7/87.5 at 24 m based on cell dose	36.1 [24–53.9]
NCI* (NCT02315612) (30)	CD22	4-1BB	64	58	17.5 [4.4–30.6]	40/56 [73]	35 [88]	30 [75]	20/27 [74]	13 [33]	NA	Med 3.2 m	Med 13.4 m	9.7 [1.1–43.9]
China (ChiCTR2000032211) (18)	CD19, CD22 (co-administration)	4-1BB	232	194 hematological or combined R/R	7.6 [0.8–19.6]	192 [99]	192 [100]	43 [22]	17 [40]	78 [41]	59.8% at 6 m	73.5% at 12 m	87.7% at 12 m	11 [0.1–32.4]
				31 isolated extramedullary relapse	7.6 [1.4–15.5]	31 [100]	NA	3 [10]	NA	NA	NA	68.6% (CNS) 95% (Testis) at 12 m	NA [2 deaths]	13.3 [NA]

*, some studies included patients with T-ALL with CD19 expression, chronic myeloid leukemia with ALL blast crisis, or B-cell malignancies other than B-ALL. However, responses and outcomes are focused on the patients with ALL. **, ELIANA study is discussed in this Editorial Commentary. #, when patients who relapsed after further anticancer therapies and those who did not qualify for prespecified definition of response are included, the number of relapses was 33 as shown *Tab. S1* of the original manuscript (14). CAR, chimeric antigen receptor; N, number of patients; CR, complete remission; CRi, complete remission with incomplete bone marrow recovery; MRD, minimal residual disease; HSCT, hematopoietic stem cell transplantation; NCT, National Clinical Trial number; m, months; BCA, B-cell aplasia; NCI, National Cancer Institute; NA, not available; med, median; med exp, median expected duration; R/R, relapse/refractory; d, days; CNS, central nervous system; ALL, acute lymphoblastic leukemia. Fractions (a/b) represent the incidence (a) out of evaluable patients (b).

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