(S114) SAFETY AND EFFICACY OF UCART22 IN HEAVILY PRE-TREATED PATIENTS WITH RELAPSED OR REFRACTORY CD22+ B-CELL ACUTE LYMPHOBLASTIC LEUKEMIA (B-ALL): RESULTS OF THE PHASE 1 BALLI-01 TRIAL

Topic: 02. Acute lymphoblastic leukemia - Clinical

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Background

UCART22 is an allogeneic donor derived CD22 directed CAR T cell product with TALEN® technology driven TRAC and CD52 knockout to minimize the risk of GvHD and allow for use of an anti-CD52 antibody for optimal lymphodepletion (LD). Prior data demonstrated high response rates with externally manufactured UCART22 (P1) (Boissel N EHA 2023) and Cellectis manufactured product (P2) (Jain N, ASH 2023). We now report final PhI results including all patients (pts) treated with UCART22 P2, establishing the recommended Ph2 dose (RP2D).

Aims

Report results of a dose escalation study of UCART22 in CD22+R/R B-ALL

Methods

The primary endpoints are safety and determination of the RP2D of UCART22. Additional endpoints are efficacy, UCART22 expansion and impact of alemtuzumab on LD. Eligibility criteria include ages 15–70y, CD22 expression ≥70%, and ≥2 prior treatments. After LD with fludarabine+cyclophosphamide (FC), +/- alemtuzumab, pts received UCART22 at 4 dose levels (1X105 to 5X106 cells/kg). A sub-study to investigate weight-based dosing of alemtuzumab was also performed.

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Results

As of Feb 2026, 45 pts were enrolled (safety set), 43 received UCART22, P1=18, P2=25, (efficacy set). Median prior lines of tx (including Blina, Ino, HSCT and CAR-T) were 4 (Table 1). 53% of the pts received prior CD19 CAR T. CRS occurred in 32 pts (71%; 16 G1, 14 G2, 2 G3). ICANS occurred in 8 pts (18%; 2 G1, 4 G2, 2 G3). IEC-HS occurred in 2 pts (4%; 1 G2,1 G4). All CRS, ICANS, and IEC-HS resolved. AESIs include Infusion related reaction G4 in 1 pt, GvHD G2 in 1pt and DIC G3 in 1 pt.
Serious adverse events were reported in 31 pts (69%) including 6 (13%) bacterial infections, 6 (13%) fungal infections and 7 (16%) febrile neutropenia. Adverse event of CMV reactivation occurred in 6 pts.
In P1 cohorts, ORR (CR, CRi, CRh and MLFS) and CR/CRi were 33% and 28%, respectively. In P2 cohorts, ORR and CR/CRi were 64% and 36%, respectively.

Highest response rates were seen in the 15 pts treated with 5 x 10^6 UCART22 cells/kg P2 (RP2D) with FC + alemtuzumab, with ORR and CR/CRi rates of 73% and 40%, respectively. Of the 6 pts who achieved a CR/CRi, 5 (83%) pts achieved MRD-ve status (<0.01%) by flow cytometry.

In the 7 pts age ≤50 y who received P2 at 5 x 10^6 cells/kg (target Ph 2 population) with alemtuzumab (60 mg), ORR was 100% and 4/7 (57%) achieved CR/CRi (3/4 MRD-ve). All 7 pts went for a subsequent transplant.

UCART22 Dose		**RP2D (P2)**		**All Doses**
	Age 15 to 70 (n=15)	**Age ≤ 50 (n=7) 60 mg Alemtuzumab**	**Age ≤ 50 (n=5) Lower dose Alemtuzumab**	**(n=45)**
Age, median (range)	28(16-66)	23(16-30)	39(23-45)	28(16-68)
Prior tx, median (range)	5(2–11)	5(4– 6)	5 (3 –11)	4(2 –11)
HSCT, n (%)	6(40)	3(43)	3(60)	22(47)
Inotuzumab, n (%)	9(60)	3 (43)	4(80)	25(56)
Blinatumomab, n (%)	14(93)	7(100)	4(80)	37(82)
CD19 CART, n (%)	8(53)	3 (42)	4(80)	24(53)
Screening Blasts % (range)	67(14 –98)	63(14-73)	80(50-98)	67(1-99)
Efficacy				(n=43)
ORR	11(73)	7(100)	3(60)	22(51)
CR/CRi	6(40)	4(57)	2(40)	14(33)
% CR/CRi MRD-ve	5(83)	3(75)	2(100)	9(64)

Summary/Conclusion

Results from the BALL1-01 trial indicate that UCART22 delivers high response rates in heavily pre-treated patients enabling a majority of the targeted Ph2 patient population to proceed to transplant. UCART22 was generally well tolerated, with a favorable safety profile in comparison to approved CAR-T products. The Ph2 trial is currently recruiting (NCT#04150497).

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