

Completed Dose Escalation From the First-in-human, Phase 1/2 Study of CD123 NK Cell Engager, SAR443579, in Relapsed or Refractory Acute Myeloid Leukemia or High Risk-Myelodysplasia

Sylvain Garciaz¹, Ashish Bajel², Abhishek Maiti³, Pinkal Desai⁴, Gerwin Huls⁵, Nicolas Boissel⁶, Mojca Jongen-Lavrencic⁷, Ioannis Mantzaris⁸, Stéphane De Botton⁹, David De Leeuw¹⁰, Shaun Fleming¹¹, Jennifer Saultz¹², Kyle Jensen¹³, Timothy Wagenaar¹³, Rahul Marpadga¹³, Yue Yang¹³, Samira Ziti-Ljajic¹⁴, Angela Virone-Oddos¹⁴, Marco Meloni¹⁴, Celine Nicolazzi¹⁴, Dobrin Draganov¹³, Giovanni Abbadessa¹³, Sarah Cooley¹³, Anthony Stein¹⁵

¹Institut Paoli-Calmettes, Aix-Marseille University, Marseille, France, ²Peter MacCallum Cancer Centre and Royal Melbourne Hospital, Melbourne, Australia, ³MD Anderson Cancer Center, Houston, TX, United States of America, ⁴Weill Cornell Medicine, New York, NY, United States of America, ⁵University Medical Center Groningen, Groningen, The Netherlands, ⁶Hôpital Saint-Louis, Paris, France, ⁷Erasmus University Medical Center, Rotterdam, The Netherlands, ⁸Montefiore Medical Center, Bronx, NY, United States of America, ⁹Institut Gustave Roussy, Paris, France, ¹⁰Amsterdam University Medical Center, Amsterdam, The Netherlands, ¹¹Alfred Health, Melbourne, Australia, ¹²Oregon Health & Science University, Portland, OR, United States of America, ¹³Sanofi, Cambridge, MA, United States of America, ¹⁴Sanofi Oncology Research, Vitry sur-Seine, France, ¹⁵City of Hope National Medical Center, Duarte, CA, United States of America

Disclosures

Sylvain Garciaz: Nothing to disclose.

Ashish Bajel: Speaker's bureau for Amgen; honoraria from Abbvie, Pfizer, Astellas, Novartis and Amgen.

Abhishek Maiti: Received research funding from Celgene and Lin BioScience.

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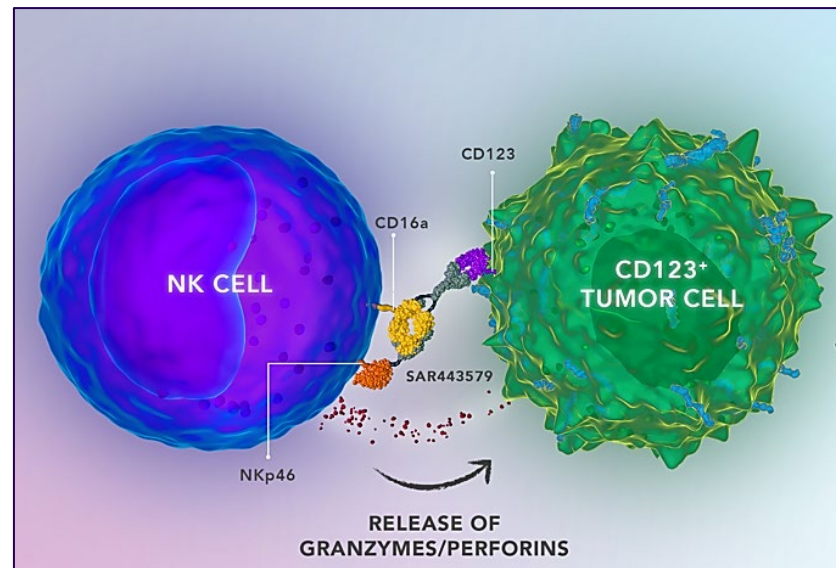
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Trifunctional SAR'579 Co-Engages NK Cells and CD123-Positive Tumor Cells

- CD123 is frequently overexpressed on leukemic blasts; high levels are associated with poor outcomes in various hematologic malignancies¹⁻⁹
- SAR'579 is an NKCE facilitating the formation of a cytolytic synapse between CD16 and NKp46 on NK cells and CD123 on tumor cells, leading to NK-cell activation and tumor death^{1,2}
- TCD17197 (NCT05086315) is an ongoing FIH phase 1/2 multicenter trial evaluating SAR'579 in patients with R/R AML, B-ALL or HR-MDS^{2,10}
- Early clinical results in R/R AML patients demonstrated that SAR'579 had a manageable toxicity profile up to 6.0 mg/kg/infusion QW; complete remissions were identified at a maximal target dose of 1.0 mg/kg/infusion^{2,11-12}



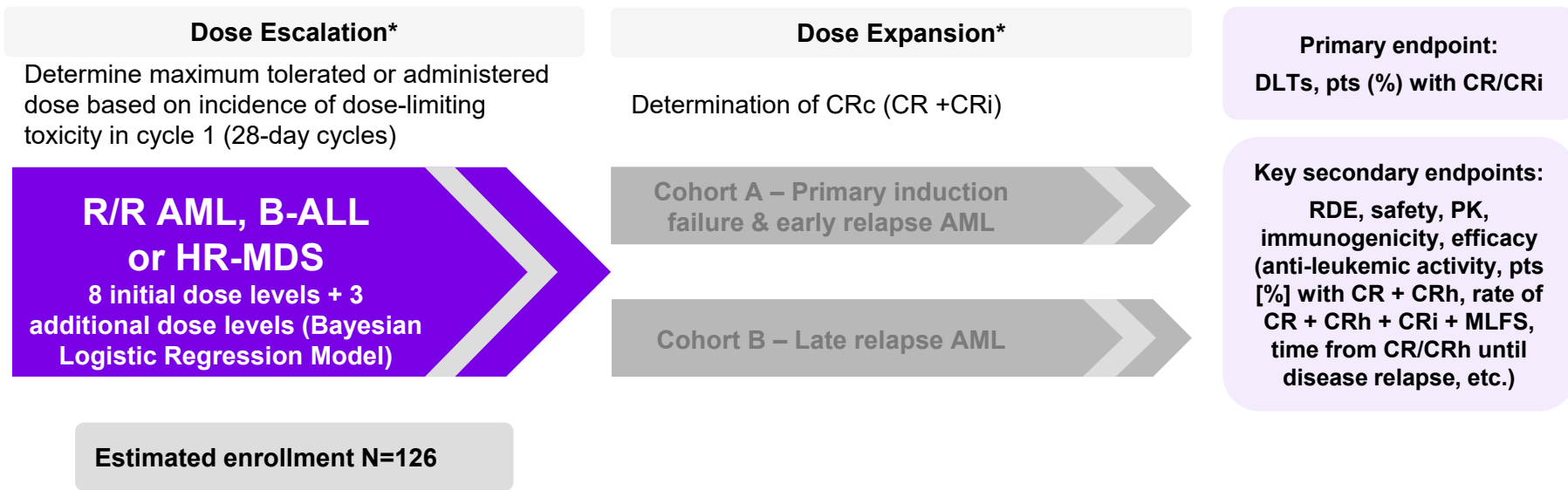
Here we report the results of SAR'579 from the completed phase 1 portion of TCD17197 in adults, as the foundation for selection of recommended doses for further development (phase 2 dose expansion)

AML, acute myeloid leukemia; B-ALL, B-cell acute lymphoblastic leukemia; CD, cluster of differentiation; DLT, dose-limiting toxicity; FIH, first-in-human; HR-MDS, high-risk myelodysplasia; NK, natural killer; NKCE, NK cell engager; QW, once weekly; R/R, relapsed or refractory; SAR'579, SAR443579.

Image modified from Bajel A, et al. Poster presented at the ASH Annual Meeting, San Diego, CA, December 9-12, 2023; #3474.

1. Gauthier L, et al. *Nat Biotechnol.* 2023;41(9):1296-1306; 2. Bajel A, et al. Poster presented at the ASH Annual Meeting, San Diego, CA, December 9-12, 2023; #3474; 3. Testa U, et al. *Biomark. Res.* 2014;2(1):4; 4. Jordan CT, et al. *Leukemia.* 2000;14(10):1777-84; 5. Testa U, et al. *Blood.* 2002;100(8):2980-8; 6. Lyapichev KA, et al. *Clin Lymphoma Myeloma Leuk.* 2021;21(4):e317-20; 7. Patnaik MM, et al. *Leuk Lymphoma.* 2021;62(11):2568-86; 8. Uckun FM, et al. *Front Aging.* 2021;2:757276; 9. El Achi H, et al. *Cancers (Basel).* 2020;12(11):3087; 10. ClinicalTrials.gov, <https://clinicaltrials.gov/study/NCT05086315> (accessed April 11, 2024); 11. Stein AS, et al. *J Clin Oncol.* 2023;41(Suppl 16): 7005; 12. Jongen-Lavrencic M, et al. *Ann Oncol.* 2023;34(Suppl_2): 823O.

Study Design^{1,2}: SAR'579 in R/R AML, B-ALL or HR-MDS



*Protocol has been amended to include three additional dose levels and a pediatric cohort for dose escalation/expansion.³

AML, acute myeloid leukemia; B-ALL, B-cell acute lymphoblastic leukemia; CR, complete remission; CRc, composite complete remission; CRh, complete remission with partial hematological recovery; CRi, complete remission with incomplete hematological recovery; DLT, dose-limiting toxicity; HR-MDS, high-risk myelodysplasia; HSCT, hematopoietic stem cell transplantation; MLFS, morphological leukemia-free state; PK, pharmacokinetics; pts, patients; RDE, recommended dose for expansion; R/R, relapsed or refractory; SAR'579, SAR443579.

1. Bajel A, et al. Poster presented at the ASH Annual Meeting, San Diego, CA, December 9-12, 2023; #3474. 2. ClinicalTrials.gov, <https://clinicaltrials.gov/study/NCT05086315> (accessed April 11, 2024); 3. TCD17197 Clinical Trial Protocol Amendment 5, Sanofi, data on file.

Presentation at the 2024 EHA Annual Meeting, Madrid, Spain, 13–16 June, 2024; S146

Oral Session 11:45-12:00 CEST; 16 June, 2024

Dose Escalation and Eligibility Criteria

SAR'579 IV Dose in mg/kg	DL1 n=3	DL2 n=4	DL3 n=4	DL4 n=4	DL5 n=4	DL6 n=8	DL7 n=8	DL8 n=8	DL9 n=6	DL10 n=6	DL11 n=4
Day 1	0.01	0.03	0.1	0.3	1.0	3.0	6.0	1.0	0.75	1.5	2.0
Day 4	0.03	0.1	0.3	/	/	/	/	/	/	/	/
Day 8	0.1	0.3	1.0	1.0	3.0	3.0	6.0	1.0	0.75	1.5	2.0
Day 11	0.1	0.3	/	/	/	/	/	/	/	/	/
Day 15	0.1	0.3	1.0	1.0	3.0	3.0	6.0	1.0	0.75	1.5	2.0
Day 22	0.1	0.3	1.0	1.0	3.0	3.0	6.0	1.0	0.75	1.5	2.0

Key Eligibility Criteria

- R/R AML, R/R HR-MDS, or R/R B-ALL
- Age ≥ 12 years
- Prior transplant allowed if relapse
- Steroids allowed if ≤ 10 mg/day of oral prednisone or equivalent
- Locally confirmed CD123+ for HR-MDS and B-ALL
- No active CNS leukemia
- No prior anti-CD123 directed agents
- WBC count $< 15 \times 10^9/L$

AML, acute myeloid leukemia; B-ALL, B-cell acute lymphoblastic leukemia; CD, cluster of differentiation; CNS, central nervous system; DL, dose level; HR-MDS, high-risk myelodysplasia; IV, intravenous; R/R, relapsed/refractory; SAR'579, SAR443579; WBC, white blood cell.

Baseline Characteristics

Characteristic	N=59
Age, median (range), years	67 (19–81)
18–65, n (%)	27 (45.8)
66–75, n (%)	22 (37.3)
>75, n (%)	10 (17.0)
Female, n (%)	20 (33.9)
ECOG PS, n (%)	
0	13 (22.0)
1	43 (72.9)
2	3 (5.1)
AML diagnosis, n (%)	58 (98.3)
HR-MDS diagnosis, n (%)	1 (1.7)

Characteristic	N=59
WBC at baseline,* median (range), 10⁹/L	2.6 (0–12)
Blast count in blood,† median (range), 10⁹/L	0.6 (0–29)
Blast proportion in bone marrow,‡ %, median (range)	47.5 (1.0–90.0)
Extramedullary disease,* n (%)	3 (5.2)
Prior lines of therapy, median (range)	2 (1–10)
1, n (%)	21 (35.6)
2, n (%)	14 (23.7)
≥3, n (%)	24 (40.7)
Prior allogenic HSCT, n (%)	18 (30.5)
Prior venetoclax, n (%)	52 (88.1)

*Reported in 58 patients. †Reported in 56 patients. ‡Reported in 54 patients.

AML, acute myeloid leukemia; ECOG PS, Eastern Cooperative Oncology Group performance status; HR-MDS, high-risk myelodysplasia; HSCT, hematopoietic stem cell transplantation; WBC, white blood cell.

Summary of Adverse Events

n (%)	DL1 0.1 mg/kg (n=3)	DL2 0.3 mg/kg (n=4)	DL3 1 mg/kg (n=4)	DL4 1 mg/kg (n=4)	DL5 3 mg/kg (n=4)	DL6 3 mg/kg (n=8)	DL7 6 mg/kg (n=8)	DL8 1 mg/kg (n=8)	DL9 0.75 mg/kg (n=6)	DL10 1.5 mg/kg (n=6)	DL11 2 mg/kg (n=4)	All (N=59)*
DLTs	0	0	0	0	0	0	0	0	1	0	0	1
TEAEs (all Grades)	3 (100)	4 (100)	4 (100)	4 (100)	4 (100)	7 (87.5)	8 (100)	8 (100)	6 (100)	6 (100)	4 (100)	58 (98.3)
Grade ≥3	3 (100)	3 (75.0)	3 (75.0)	3 (75.0)	3 (75.0)	5 (62.5)	3 (37.5)	3 (37.5)	6 (100)	5 (83.3)	3 (75.0)	40 (67.8)
Grade 5†	0	1 (25.0)	1 (25.0)	1 (25.0)	1 (25.0)	0	1 (12.5)	0	1 (16.7)	0	0	6 (10.2)
TE-SAEs	2 (66.7)	3 (75.0)	3 (75.0)	3 (75.0)	3 (75.0)	5 (62.5)	2 (25.0)	3 (37.5)	4 (66.7)	5 (83.3)	2 (50.0)	35 (59.3)
Leading to DC	0	0	0	1 (25.0)	0	0	1 (12.5)	0	2 (33.3)	0	0	4 (6.8)
TRAEs (all Grades)	2 (66.7)	4 (100)	2 (50.0)	4 (100)	3 (75.0)	4 (50.0)	6 (75.0)	7 (87.5)	3 (50.0)	3 (50.0)	1 (25.0)	39 (66.1)
TR-SAEs (Grade ≥3)	0	0	2 (50.0)	0	0	0	0	0	1 (16.7)	0	0	3 (5.1)
CRS,‡ [Grade]	0	0	0	1 (25.0) [1]	1 (25.0) [1]	0	0	0	1 (16.7) [3]	1 (16.7) [1]	0	4 (6.8)

- One DLT (Grade 3 CRS) was reported during C1D18 at a dose level of 0.75 mg/kg QW
- Grade 5 TEAEs were reported as unrelated to SAR'579 by the investigator and included cholestasis, hemorrhage intracranial, cerebellar hemorrhage, pneumonia, pulmonary sepsis, and urosepsis
- SAEs related to SAR'579 (Grade ≥3) included diverticulitis, infusion related reaction, and cytokine release syndrome
- No cases of immune effector cell-associated neurotoxicity syndrome (ICANS)

SAR'579 had a manageable toxicity profile, with one DLT and all Grade 5 TEAEs unrelated to study treatment

Cutoff date for analysis: March 04, 2024.

*Safety analyses includes 58 patients with R/R AML and 1 patient with HR-MDS. †Grade 5 TEAE occurring during the treatment period. ‡CRS is a Preferred Term and analyses are not pooled. C, cycle; CRS, cytokine release syndrome; D, day; DC, discontinuation; DL, dose level; DLT, dose-limiting toxicity; HR-MDS, high-risk myelodysplasia; QW, once weekly; R/R AML, relapsed or refractory acute myeloid leukemia; SAE, serious adverse event; TE, treatment-emergent; TEAE, treatment-emergent adverse event; TR, treatment-related; TRAE, treatment-related adverse event.

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TEAEs by Preferred Term Reported in >10% Patients* (All Grades)

n (%)†	DL1 0.1 mg/kg (n=3)	DL2 0.3 mg/kg (n=4)	DL3 1 mg/kg (n=4)	DL4 1 mg/kg (n=4)	DL5 3 mg/kg (n=4)	DL6 3 mg/kg (n=8)	DL7 6 mg/kg (n=8)	DL8 1 mg/kg (n=8)	DL9 0.75 mg/kg (n=6)	DL10 1.5 mg/kg (n=6)	DL11 2 mg/kg (n=4)	Any Grade (N=59)*	Grade ≥3 (N=59)‡
Infusion-related reaction§	1 (33.3)	3 (75.0)	2 (50.0)	4 (100)	2 (50.0)	4 (50.0)	6 (75.0)	7 (87.5)	2 (33.3)	2 (33.3)	1 (25.0)	34 (57.6)	1 (1.7)
Constipation	2 (66.7)	–	2 (50.0)	1 (25.0)	1 (25.0)	1 (12.5)	2 (25.0)	2 (25.0)	1 (16.7)	–	1 (25.0)	13 (22.0)	–
Febrile neutropenia	–	–	–	1 (25.0)	–	3 (37.5)	1 (12.5)	1 (12.5)	–	3 (50.0)	2 (50.0)	11 (18.6)	10 (16.9)
Arthralgia	1 (33.3)	1 (25.0)	1 (25.0)	2 (50.0)	1 (25.0)	–	1 (12.5)	–	2 (33.3)	2 (33.3)	–	11 (18.6)	2 (3.4)
Nausea	1 (33.3)	2 (50.0)	–	1 (25.0)	1 (25.0)	–	–	1 (12.5)	2 (33.3)	–	2 (50.0)	10 (16.9)	–
Pneumonia	–	–	1 (25.0)	–	1 (25.0)	1 (12.5)	–	1 (12.5)	2 (33.3)	2 (33.3)	–	8 (13.6)	6 (10.2)
Stomatitis	–	1 (25.0)	1 (25.0)	3 (75.0)	–	–	2 (25.0)	–	1 (16.7)	–	–	8 (13.6)	–
Vomiting	–	1 (25.0)	1 (25.0)	2 (50.0)	–	1 (12.5)	1 (12.5)	1 (12.5)	–	–	1 (25.0)	8 (13.6)	–
Fatigue	1 (33.3)	1 (25.0)	1 (25.0)	1 (25.0)	1 (25.0)	1 (12.5)	–	–	–	1 (16.7)	–	7 (11.9)	1 (1.7)
Decreased appetite	1 (33.3)	2 (50.0)	–	1 (25.0)	2 (50.0)	–	–	1 (12.5)	–	–	–	7 (11.9)	–
Diarrhea	1 (33.3)	1 (25.0)	–	–	–	–	2 (25.0)	2 (25.0)	–	1 (16.7)	–	7 (11.9)	–
Edema peripheral	–	1 (25.0)	–	1 (25.0)	1 (25.0)	–	–	2 (25.0)	–	2 (33.3)	–	7 (11.9)	–
Epistaxis	–	–	1 (25.0)	–	1 (25.0)	–	2 (25.0)	2 (25.0)	–	–	1 (25.0)	7 (11.9)	–
Non-cardiac chest pain	1 (33.3)	–	–	–	1 (25.0)	–	1 (12.5)	2 (25.0)	1 (16.7)	–	1 (25.0)	7 (11.9)	–

The most common TEAE was infusion-related reaction (57.6%)

Cutoff date for analysis: March 04, 2024.

*Threshold is based on total population and not individual groups. †Anxiety, dyspnea, abdominal pain, and back pain were reported in 6 (10.2%) patients each and were Grade 1/2.

‡Safety analyses includes 58 patients with R/R AML and 1 patient with HR-MDS. §IRR are not pooled analyses.

DL, dose level; TEAE, treatment-emergent adverse event.

Response per Investigator (per Modified IWG Criteria) in Patients with R/R AML

n (%)												All (N=58)
	DL1 0.1 mg/kg (n=3)	DL2 0.3 mg/kg (n=4)	DL3 1 mg/kg (n=4)	DL4 1 mg/kg (n=4)	DL5 3 mg/kg (n=4)	DL6 3 mg/kg (n=8)	DL7 6 mg/kg (n=8)	DL8 1 mg/kg (n=7)	DL9 0.75 mg/kg (n=6)	DL10 1.5 mg/kg (n=6)	DL11 2 mg/kg (n=4)	
CRc (CR + CRi)	0	0	2 (50.0)	1 (25.0)	0	0	0	2 (28.6)	0	0	0	5 (8.6)
CR	0	0	1 (25.0)	1 (25.0)	0	0	0	2 (28.6)	0	0	0	4 (6.9)
CRi	0	0	1 (25.0)	0	0	0	0	0	0	0	0	1 (1.7)

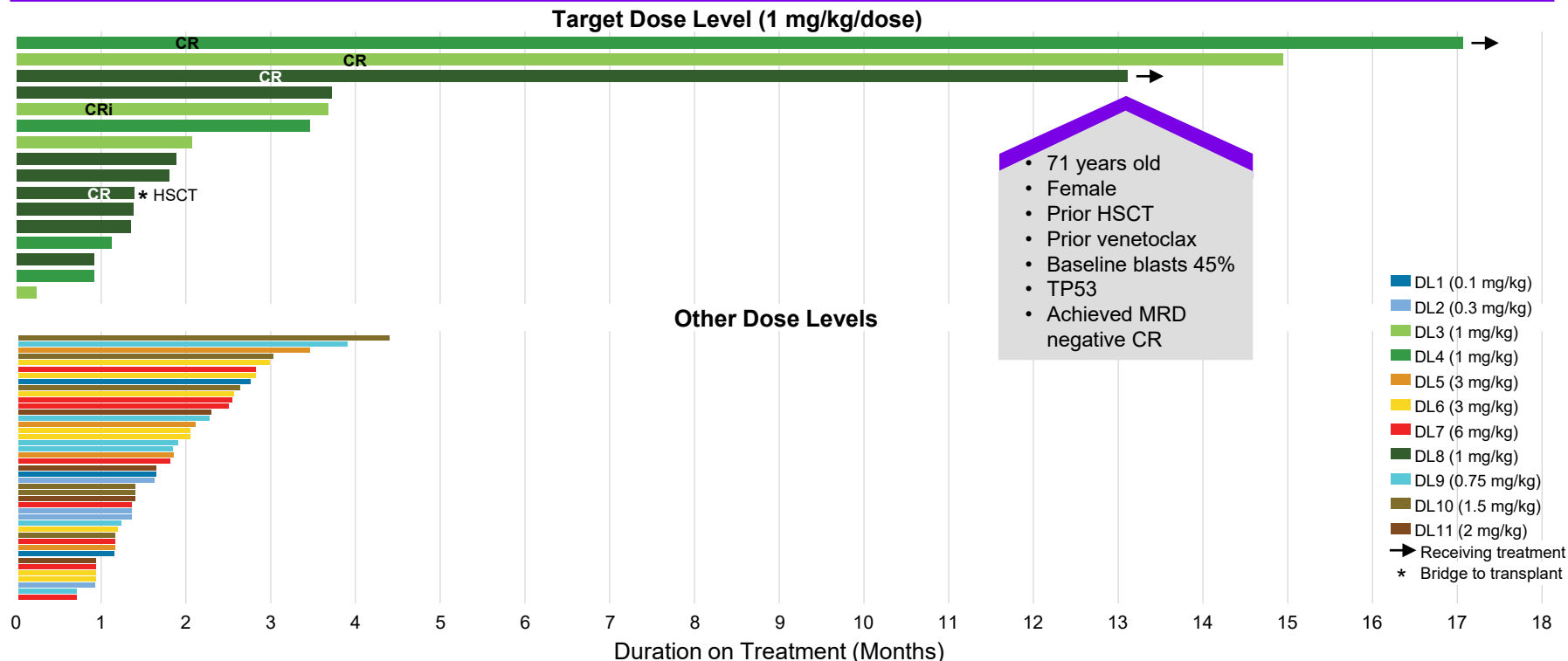
- Patients received a median of 2.0 cycles (range: 1–11) of treatment; median treatment duration was 7.9 weeks (range: 1–74)
- Median time to CR was 2 cycles (range, 1–4)

CR/CRi was reported in 5 of 15 (33.3%) patients with R/R AML treated at a final target dose of 1 mg/kg/infusion; 2 patients with CR are still receiving treatment

Cutoff date for analysis: March 04, 2024.

AML, acute myeloid leukemia; CR, complete remission; CRc, composite complete remission; CRi, complete remission with incomplete hematological recovery; DL, dose level; DOR, duration of response; R/R, relapsed or refractory.

Individual Patient Responses per Investigator



Durable CR (>10 mo) observed in 3 patients, with 2 remaining on maintenance

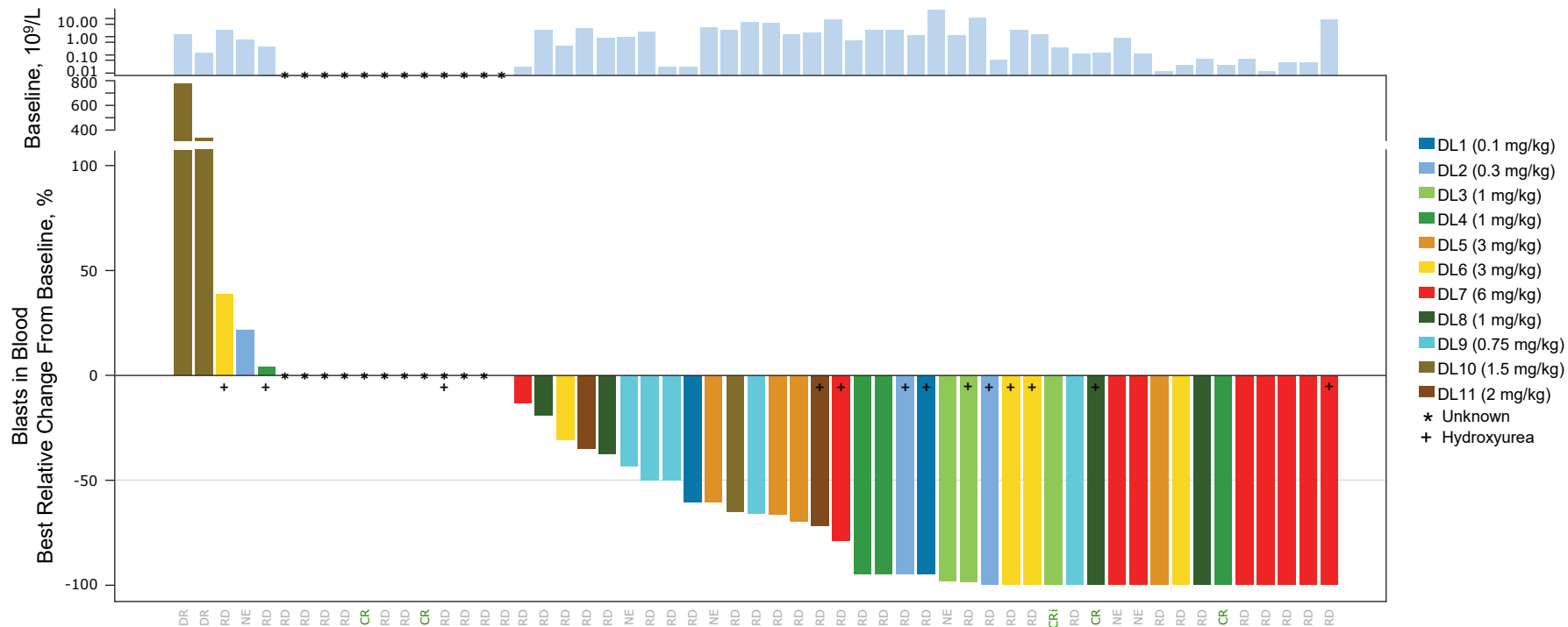
Cutoff date for analysis: May 21, 2024.

CR, complete remission; CRi, complete remission with incomplete hematological recovery; DL, dose level; HSCT, hematopoietic stem cell transplantation; MRD, minimal residual disease.

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Blast Count Best Relative Change (Peripheral Blood)



High baseline blast variability with observed reductions across all dose levels in peripheral blood

Cutoff date for analysis: May 31, 2024.

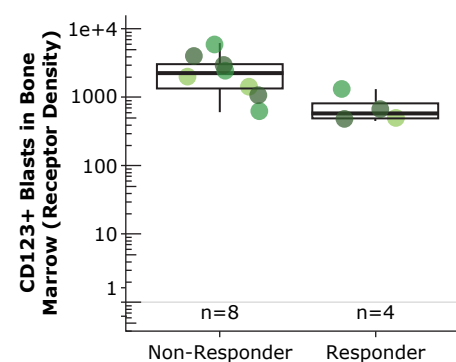
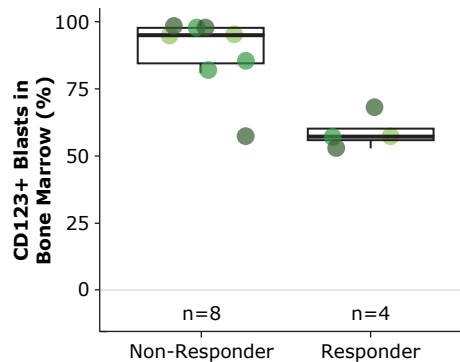
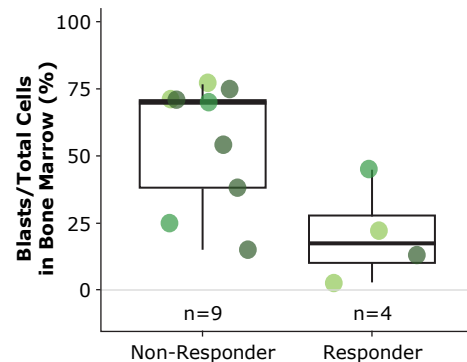
CR, complete remission; CRI, complete remission with incomplete hematological recovery; DL, dose level; DR, disease relapse; NE, not evaluable; RD, resistant disease.

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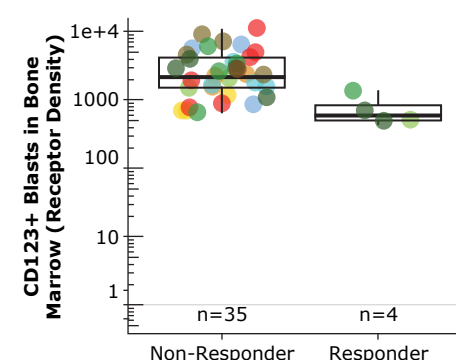
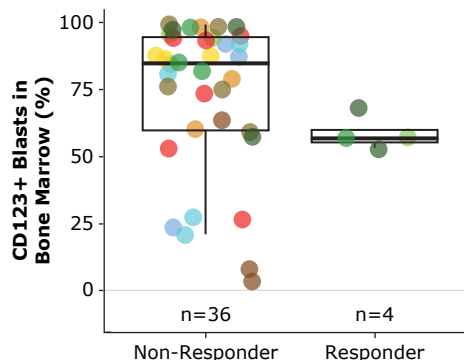
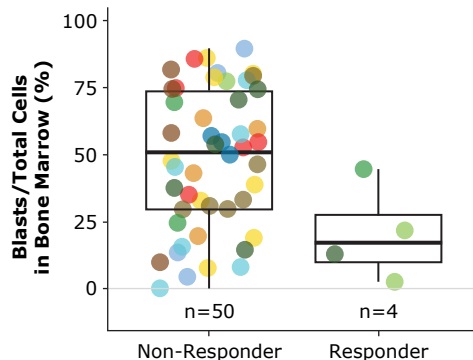
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Relationship Between Baseline Bone Marrow Blasts and Response

Patients
treated at final
target dose of
1 mg/kg
(DL3-4 & DL8)



Patients
treated at all
dose levels

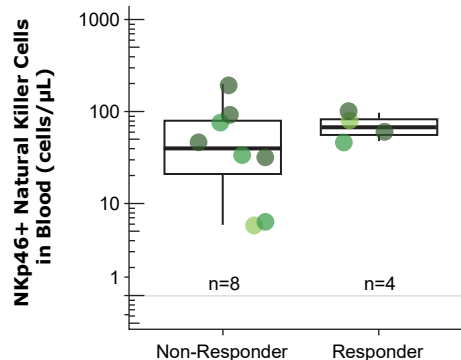
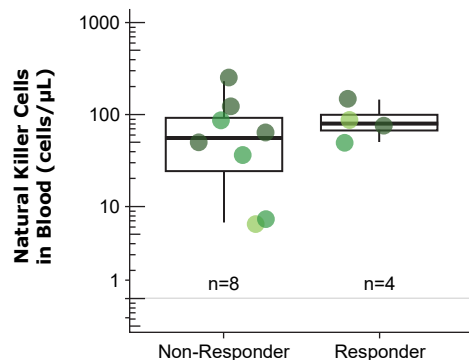


- DL1 (0.1 mg/kg)
- DL2 (0.3 mg/kg)
- DL3 (1 mg/kg)
- DL4 (1 mg/kg)
- DL5 (3 mg/kg)
- DL6 (3 mg/kg)
- DL7 (6 mg/kg)
- DL8 (1 mg/kg)
- DL9 (0.75 mg/kg)
- DL10 (1.5 mg/kg)
- DL11 (2 mg/kg)

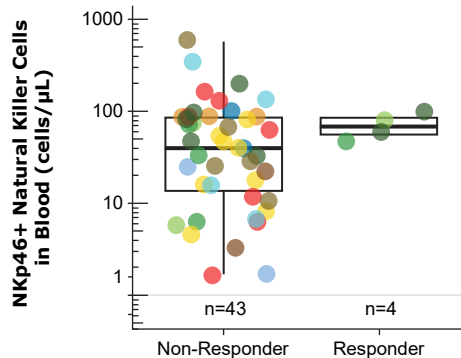
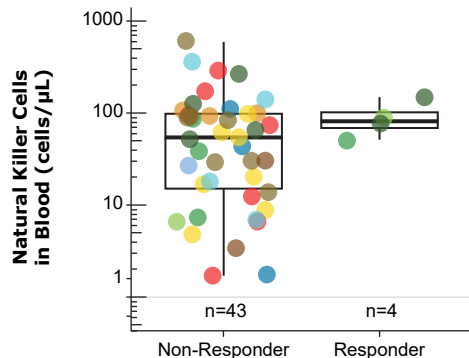
Central assessment cutoff date for analysis: May 31, 2024. All assessment by flow analysis of marrow aspirate.
CD, cluster of differentiation; DL, dose level.

Relationship Between Baseline Peripheral NK Cells and Response

Patients
treated at final
target dose of
1 mg/kg
(DL3-4 & DL8)



Patients
treated at all
dose levels



- DL1 (0.1 mg/kg)
- DL2 (0.3 mg/kg)
- DL3 (1 mg/kg)
- DL4 (1 mg/kg)
- DL5 (3 mg/kg)
- DL6 (3 mg/kg)
- DL7 (6 mg/kg)
- DL8 (1 mg/kg)
- DL9 (0.75 mg/kg)
- DL10 (1.5 mg/kg)
- DL11 (2 mg/kg)

Central assessment cutoff date for analysis: May 31, 2024.
DL, dose level; NK, natural killer.

Summary

Safety

- SAR'579 had a manageable toxicity profile up to a dose of 6 mg/kg QW in adult patients with R/R AML
- Low grade infusion-related reaction (Grade 1–2) was the most common TRAE
- CRS events were rare (one Grade 3) and no ICANS

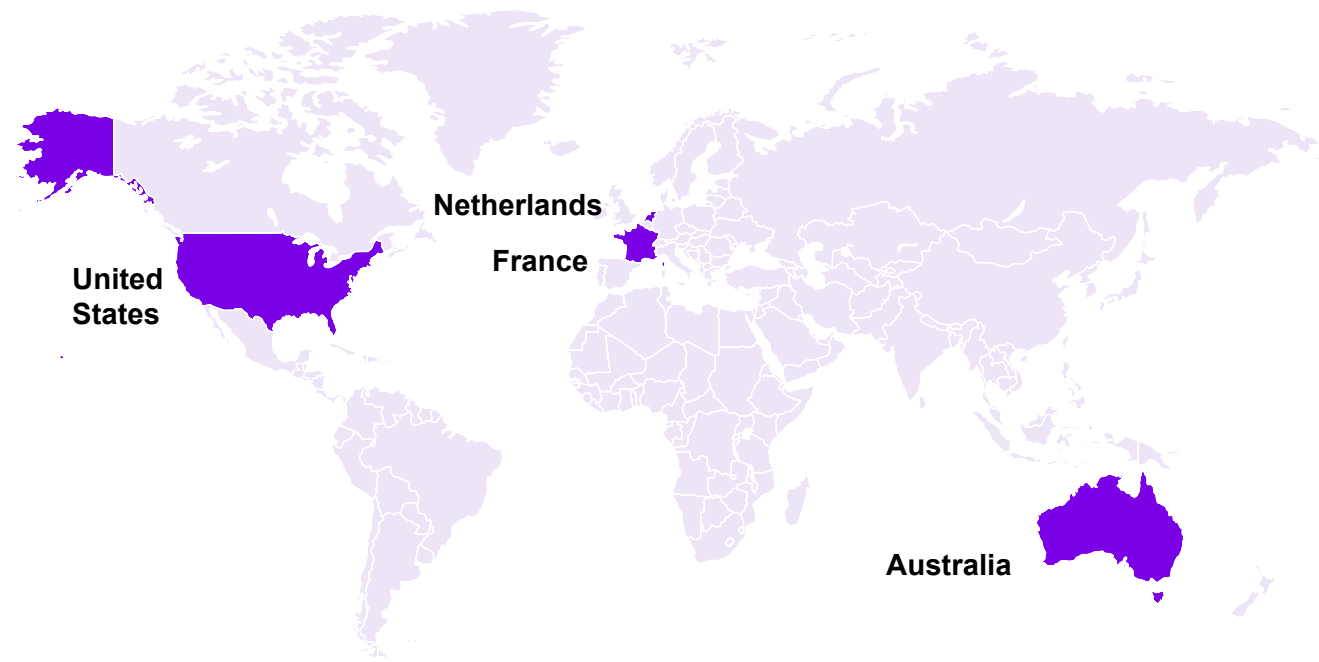
Antitumor Activity

- Among 15 AML patients treated at a target dose of 1 mg/kg, 5 (33.3%) achieved CR/CRi
- Durable remission >10 months in 3 of 5 responders
 - One responder proceeded to HSCT
 - Two remain on maintenance therapy
- Observed bell-shaped clinical dose-response curve consistent with preclinical findings (see Meloni M, et al. Poster #P475 for further details on the preclinical data)
- Recommended doses for development in the phase 2 portion of the study are still being explored

**SAR'579 received Fast Track designation by the US FDA,
and the dose optimization and expansion phase of TCD1719 is ongoing**

AML, acute myeloid leukemia; CR, complete remission; CRi, complete remission with incomplete hematological recovery; CRS, cytotoxic release syndrome; DLT, dose-limiting toxicity; DL, dose level; FDA, Food and Drug Administration; IRR, infusion-related reactions; QW, once weekly; R/R, relapsed refractory; SAE, serious adverse event; SAR'579, SAR443579; US, United States.

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