CARSGEN
THERAPEUTICS

CARsgen Therapeutics (HKEX: 02171)

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Making Cancer Curable

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Data Sharing

Recent Highlights:

- We presented clinical data on the BCMA-targeted allogeneic candidate CT0596 at the 2025 ASH Annual Meeting:

  A First-in-Human Study of CT0596, an Allogeneic CAR T-Cell Therapy Targeting BCMA, in Patients with Relapsed/Refractory Multiple Myeloma

- We also shared preliminary clinical data on the CD19/CD20-targeted allogeneic candidate CT1190B for the treatment of relapsed/refractory B-cell non-Hodgkin's lymphoma.

CT0596: An Allogeneic BCMA-Targeting CAR-T Cell Therapy (THANK-u Plus™)

THANK-u Plus™ Platform

- THANK-u Plus™ demonstrates significantly enhanced expansion compared to THANK-uCAR®
- THANK-u Plus™ sustains expansion regardless of NKG2A expression levels in NK cells

CT0596

- Based on the THANK-u Plus™ platform, the allogeneic BCMA-targeting CAR-T product **CT0596** has been developed for the treatment of **R/R MM or PCL**

Clinical Development Progress and Plans

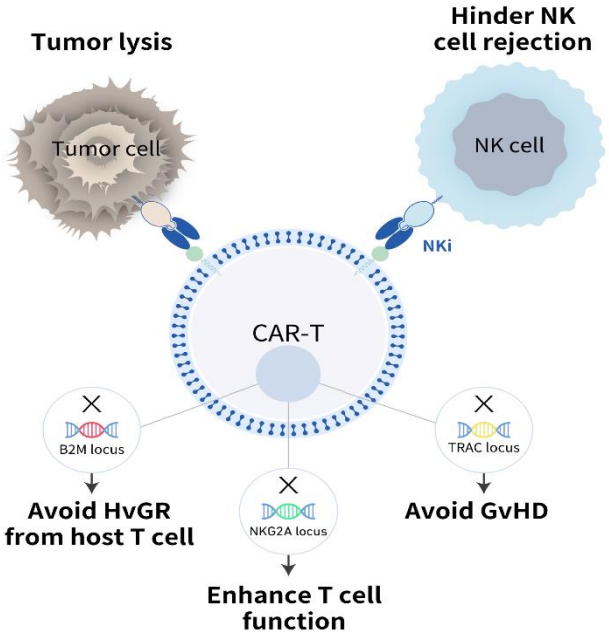
- An Investigator-Initiated Trial (IIT) of CT0596 for R/R MM or R/R PCL is ongoing:
  - ✓ As of May 6, 2025, 8 R/R MM patients have been infused
- We plan to explore applications in other plasma cell neoplasms and autoimmune diseases
- IND submission for multiple myeloma is anticipated in H2, 2025

CT0596 is Designed to Overcome the Typical Challenges of Allogeneic CAR-T

Multiple Advantages of Allogeneic/Universal CAR-T (uCAR-T)

1. More standardized and lower risk of manufacturing failure
2. Not limited by the quality or quantity of patient's T-cells
3. Can be manufactured in batches for timely infusion

However, there are several challenges associated with uCAR T, CT0596 is designed to overcome these challenges:



Challenges	Potential Solutions in CT0596
Graft vs Host disease (GvHD)	Knockout TRAC (T-cell receptor)
Host vs Graft Reaction or T cell rejection	Knockout B2M (HLA-I)
uCAR-T fratricide (NKG2A-related)	Knockout NKG2A in uCAR T cells
Additional gene editing	To prevent rejection mediated by host NK cells

Study Design

An open-label, single-arm, phase 1, first-in-human trial was conducted in China (NCT06718270).

Key Inclusion Criteria

- Age ≥ 18 years
- Patients with R/R MM who have received at least 3 prior lines of therapy. Patients with R/R pPCL had received at least 1 prior line of therapy.
- MM patients have progressive disease following or during the last treatment.
- Patients must have measurable disease.
- ECOG 0-1

Primary Endpoint

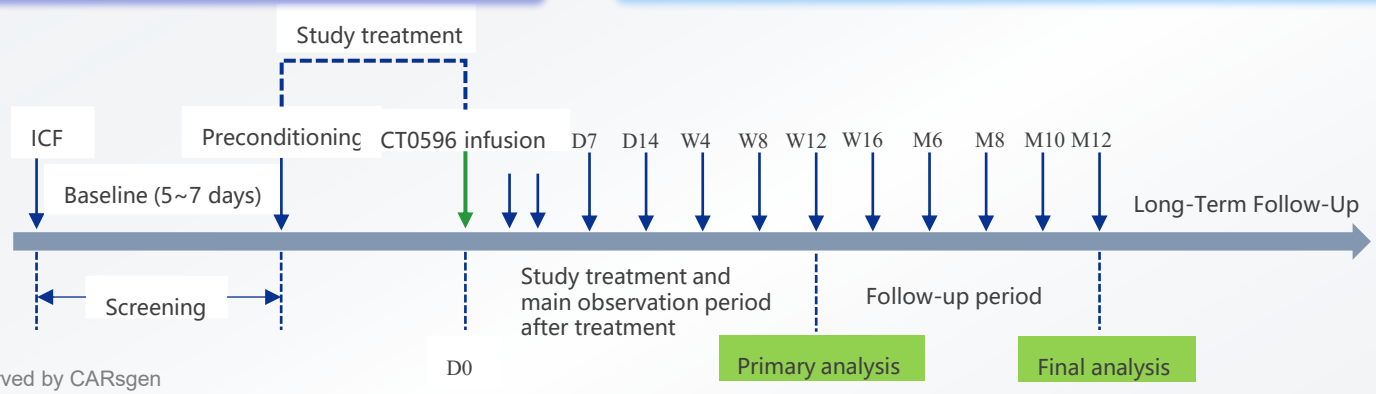
- Safety and Tolerability

Secondary Endpoints

- Pharmacokinetics
- Preliminary Efficacy

Lymphodepletion Regimen:
Fludarabine: 30 mg/m²/day for 3 consecutive days
Cyclophosphamide: 500 mg/m²/day for 3 consecutive days

i3+3 design
Doses: 150×10^6, 300×10^6, 450×10^6, 600×10^6 CT0596 cells
Re-infusion was allowed.



Patient Baseline Characteristics

Data Cut-off: June 24, 2025

	Patients (N=8)
Multiple Myeloma	8 (100%)
Median Age	63.5
Immunoglobulin Type at Initial Diagnosis, n (%)	
IgG	2 (25.0%)
IgA	4 (50.0%)
κ Light Chain	2 (25.0%)
R-ISS Stage, n (%)	
I	0
II	5 (62.5%)
III	3 (37.5%)
High-Risk Cytogenetics, n (%)	
Yes	1 (12.5%)
No	3 (37.5%)
Missing	4 (50.0%)
Extramedullary Disease, n (%)	1 (12.5%)
Median Prior Lines of Therapy	4.5
Prior Autologous Stem Cell Transplantation	5 (62.5%)

- **Lymphodepletion Dose**

  - 6 patients received the full-dose lymphodepletion regimen (i.e., fludarabine 30 mg/m²/day and cyclophosphamide 500 mg/m²/day for 3 consecutive days, as per protocol).
  - 2 patients had their lymphodepletion doses adjusted due to investigator concerns about potential severe hematological toxicity or pre-existing impaired creatinine clearance. The specific adjustments were as follows:
    - ✓ 1 patient: cyclophosphamide dose reduced by 30% only, with fludarabine dose unchanged.
    - ✓ 1 patient: both fludarabine and cyclophosphamide doses reduced by 25%.

- **Cell Infusion Dose**

1.5 × 10^8 cells dose cohort: 1 patient

3.0 × 10^8 cells dose cohort: 5 patients

4.5 × 10^8 cells dose cohort: 2 patients

CT0596 Demonstrated a Manageable Safety Profile

- Cytopenias were reported in all 8 patients.
- Grade ≥3 treatment-related cytopenias:
  - ✓ Lymphopenia: [n=8]
  - ✓ Neutropenia: [n=7]
  - ✓ Leukopenia: [n=8]
  - ✓ Thrombocytopenia: [n=3]
- Four patients experienced Grade 1 CRS, with no Grade 3 or higher CRS observed.
- The time to CRS onset was 2 (range: 1-8) days post-infusion, with a median duration of 6 (range: 2-10) days.
- No cases of ICANS or GvHD were observed.
- No DLTs, no study discontinuation due to AE, no deaths due to AE.

	N (%)
TEAEs	8 (100.0)
SAEs	2 (25.0)
≥Grade 3 AEs	8 (100)
Treatment-related TEAEs	
≥Grade 3 Lymphopenia	8 (100)
≥Grade 3 Leukopenia	8 (100)
≥Grade 3 Thrombocytopenia	3 (37.5)
≥Grade 3 Neutropenia	7 (87.5)
≥Grade 3 Anemia	2 (25.0)
≥Grade 3 Infections	0
CRS	4 (50.0)
ICANS	0
GvHD	0
AEs leading to study discontinuation	0
AEs leading to death	0
DLT	0

CT0596 Induced Deep and Durable Responses

- All 8 infused patients were all evaluable for efficacy, with the median follow-up of 2.56 months (range: 0.9-5.9).
- 5 patients achieved PR or above: 3 CR/sCR (all 3 received full lymphodepletion dose), 1 PR, and 1 VGPR.
- 4 out of 6 patients with full lymphodepletion dose achieved PR or above. 6 patients achieved MRD-negativity at Week 4.
- Hematological responses deepened over time in MRD-negative patients. No patients got progression disease. Furthermore, Patient 01 has maintained sCR and MRD-negative for nearly 6 months.

Summary of CT0596 in Multiple Myeloma

- Among the 8 infused patients, the 3 previously reported patients who achieved CR or better have maintained their responses. Furthermore, one patient initially achieving PR subsequently improved to VGPR, indicating that patient responses are deepening and approaching CR.
- The lymphodepletion dose and cell dose significantly influence treatment response. Standard lymphodepletion doses and higher cell numbers are associated with improved response rates and CR rates.
- Dose escalation is ongoing. The lymphodepletion dose has been finalized, and higher cell doses are being explored to determine the recommended dose.
- Efforts will continue to fully accelerate the IND plan for CT0596, focusing on clinical studies for the intended commercial indication.

CT0596 Achieved sCR in Two R/R pPCL Patients

As of the data cutoff date (Oct 17, 2025), two patients with relapsed/refractory pPCL had been enrolled.

	pPCL-01	pPCL-02
Patient	62-year-old male, IgG-λ type	70-year-old male, κ light chain type
Prior Therapies	ASCT + triple classes of drugs (PI, IMiD, CD38 mAb)	Triple classes of drugs (PI, IMiD, CD38 mAb)
CAR-T Treatment	Two infusions, ~2 months apart	Single infusion
Safety	Grade 2 CRS, Grade 4 cytopenia, lung infection	Grade 1 CRS, Grade 4 neutropenia and thrombocytopenia
Pharmacokinetics	C_{max}: **161,971** copies/μg gDNA; Maintained at 10^3 by Week 8	C_{max}: **151,654** copies/μg gDNA
Efficacy	Achieved **sCR** at Week 4 & 8; bone marrow MRD-negative ($<10^{-6}$) at Week 4	Achieved **sCR** at Week 4, 8, & 12; bone marrow MRD-negative ($<10^{-6}$) at Week 4 & 12

- CT0596 has exhibited **robust and rapid anti-tumor efficacy** in heavily pretreated patients with rapidly progressive relapsed/refractory pPCL
- Aside from expected CAR-T-associated toxicities such as CRS and hematologic adverse events, no significant organ toxicities were observed, indicating a **manageable safety profile**.

pPCL: Primary Plasma Cell Leukemia; sCR: Stringent Complete Response; ASCT: Autologous Stem Cell Transplantation; PI: Proteasome Inhibitor; IMiD: Immunomodulatory Drug; CRS: Cytokine Release Syndrome; MRD: Minimal Residual Disease

CT0596 Program Summary

- CT0596 is a BCMA/NKG2A dual targeting CAR T with additional gene editing, designed with triple gene knockout (TRAC/B2M/NKG2A) to overcome the challenges of allogeneic CAR T-cell therapy.
- In the first-in-human study of CT0596:
  - ✓ CT0596 has a manageable safety profile.
  - ✓ 5 out of 8 Multiple Myeloma patients achieved PR or above. Among the 6 patients receiving full-dose lymphodepletion, 4 achieved PR or better, including 3 achieving CR or better.
  - ✓ Both pPCL patients achieved sCR.
  - ✓ Significant CAR-T cell expansion was observed in patients who achieved responses.
- Larger, multi-center studies are planned to further evaluate the efficacy and safety of CT0596 at higher cell dose levels.

CT1190B: An Allogeneic CD19/CD20-Targeting CAR-T Cell Therapy (THANK-u Plus™)

THANK-u Plus™ Platform

- THANK-u Plus™ demonstrates significantly enhanced expansion compared to THANK-uCAR®
- THANK-u Plus™ sustains expansion regardless of NKG2A expression levels in NK cells

CT1190B

- Based on the THANK-u Plus™ platform, the allogeneic CD19/CD20 -targeting CAR-T product **CT1190B** has been developed for the treatment of **B-cell malignancies or autoimmune diseases.**

Clinical Development Progress and Plans

- An Investigator-Initiated Trial (IIT) of CT1190B for relapsed/refractory B-cell non-Hodgkin's lymphoma is ongoing.
- Products based on this platform are also being investigated in autoimmune diseases.

Enrollment

- A total of 14 patients have been enrolled:
  - ✓ 3 with Follicular Lymphoma (FL)
  - ✓ 3 with Mantle Cell Lymphoma (MCL)
  - ✓ 8 with Diffuse Large B-Cell Lymphoma (DLBCL)
- The dose-escalation study has been completed, establishing the lymphodepletion regimen and preliminarily determining the recommended cell dose.

Lymphodepletion Dose Exploration Phase:

- 3 FL patients (Cell dose: 3.0×10^8: 1 patient; 4.5×10^8: 2 patients)
- 2 DLBCL patients (Cell dose: 1.5×10^8: 1 patient; 4.5×10^8: 1 patient)
- 1 MCL patient (Cell dose: 4.5×10^8: 1 patient)

Recommended Lymphodepletion Dose: Fludarabine 30 mg/m²/day for 3 days + Cyclophosphamide 1000 mg/m²/day for 2 days

- 2 MCL patients (Cell dose: 6.0×10^8)
- 6 DLBCL patients (Cell doses: 3.0×10^8: 1 patient; 4.5×10^8: 1 patient; 6.0×10^8: 4 patients)

Efficacy and Safety

Data cut-off: October 17, 2025. The primary safety signals were CRS, cytopenias, and infections. No DLTs were observed, and no other adverse reactions such as ICANS or GvHD were reported.

➢ Lymphodepletion Regimen: Fludarabine 30 mg/m² × 3 days + Cyclophosphamide 500 mg/m² × 3 days

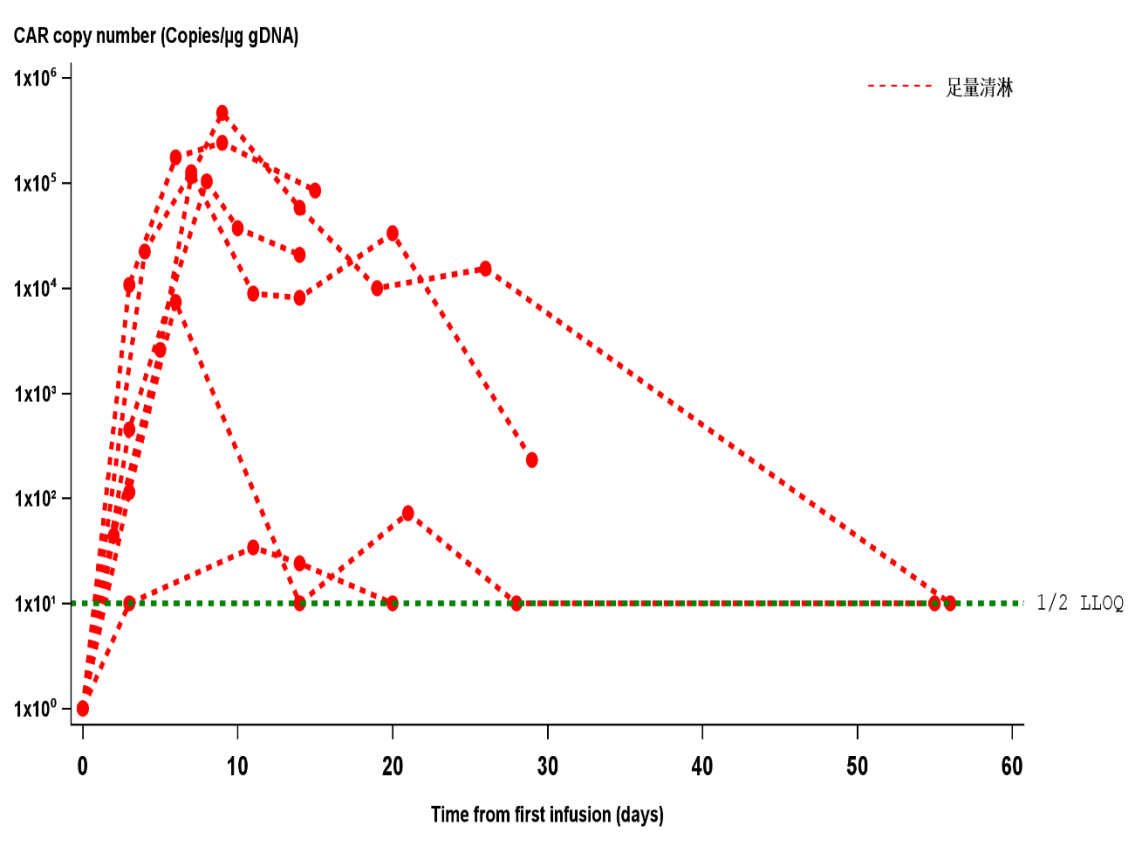
- All three FL patients achieved CR, resulting in an ORR of 100% and a CRR of 100%. One FL patient had failed immunochemotherapy, a PI3K inhibitor, chemotherapy + autologous HSCT, and CD3/CD20 bispecific antibody therapy. Another FL patient had failed immunochemotherapy + autologous HSCT and CD19 CAR-T therapy. The peak expansion copy number reached 10^3-10^4 copies/μg gDNA.

➢ Lymphodepletion Regimen: Fludarabine 30 mg/m² × 3 days + Cyclophosphamide 1000 mg/m² × 2 days

- 8 patients were enrolled under this regimen, including 2 MCL patients (cell dose: 6×10^8) and 6 DLBCL patients (cell doses: 3×10^8: 1 patient; 4.5×10^8: 1 patient; 6×10^8: 4 patients).
  - ✓ 6 patients were evaluable for efficacy, showing an ORR of 83.3% and a CRR of 66.6%, including 4 CR and 1 PR. Two DLBCL patients infused with 6×10^8 cells had not reached the efficacy assessment timepoint.
  - ✓ Both MCL patients achieved CR. Among the DLBCL patients: 2 achieved CR, 1 achieved PR (this patient had failed autologous CD19 CAR-T manufacturing), and 1 had PD. The two DLBCL patients not yet evaluable for efficacy showed a peak expansion of 10^5 copies/μg gDNA.
  - ✓ In the 6×10^8 cell dose cohort (4 patients), 3 achieved CR.

Pharmacokinetics at the Recommended Dose

Product	Indication	Mean or Median Cmax (copies/ug)
CT1190B (allogeneic)	NHL	114564.5 (RD)
ALL-501 (allogeneic)	LBCL	1688
relma-cel (autologous)	LBCL	25214.5~29693.5
Kymriah (autologous)	LBCL	5210.33~6450



At the recommended dose (full-intensity lymphodepletion and cell dose of 6 × 10^8), involving 6 patients (4 DLBCL, 2 MCL), the median Cmax of CT1190B reached 10^5 copies/μg gDNA. This significantly exceeds the levels observed with currently approved autologous CAR-T products (typically 10^3-10^4) and other investigational allogeneic CAR-T products (around 10^3).

Significant Clinical Value of Allogeneic CAR-T

After undergoing multiple lines of therapy, a patient's own T-cell function may be compromised. Consequently, treatments that rely on activating the patient's own T cells and mobilizing their anti-tumor capacity will have significantly diminished efficacy compared to their effect in patients with intact immune function. In contrast, allogeneic CAR-T products are derived from T cells of healthy donors, offering this patient population an opportunity to receive T-cell immunotherapy.

Response to cilta-cel

	Full cohort N = 20	ADC exposed* N = 13	Bispecific exposed* N = 7
Overall response rate,† % (95% CI)	60.0 (36.1-80.9)	61.5 (31.6-86.1)	57.1 (18.4-90.1)
Best response, rate, n (%)			
Stringent complete response	1 (5.0)	1 (7.7)	0
Complete response	5 (25.0)	4 (30.8)	1 (14.3)
Very good partial response	5 (25.0)	3 (23.1)	2 (28.6)
Partial response	1 (5.0)	0	1 (14.3)
Minimal response‡	1 (5.0)	0	1 (14.3)
Stable disease	3 (15.0)	2 (15.4)	1 (14.3)
Progressive disease	3 (15.0)	3 (23.1)	0
Not evaluable‡,§	1 (5.0)	0	1 (14.3)
≥VGPR	11 (55.0)	8 (61.5)	3 (42.9)
Median duration of response (95% CI), mo	11.5 (7.9-NE)	11.5 (7.9-NE)	8.2 (4.4-NE)

- The CARTITUDE-2 study investigating cilta-cel found that patients treated with autologous CAR-T therapy after prior exposure to T-cell engagers (TCEs) showed reduced efficacy, depth of response, and duration of response compared to other treatment approaches. The applicability of treatments relying on activating and mobilizing a patient's own T cells after TCE exposure is therefore limited by prior treatment choices. This is particularly relevant in the field of hematological malignancies, where TCEs are increasingly moving into earlier lines of therapy, highlighting a growing unmet medical need for these patients.

Adam D Cohen,et al.blood. 2023 Jan 19;141(3):219-230. doi: 10.1182/blood.2022015526.

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