

First-in-Class BCMA CAR-MILs for Multiple Myeloma

VCB-1201 (BCMA CAR-MILs)

1. Market
2. Mode of Action
3. Target Product Profile
4. Technical Feasibility
5. Business strategy

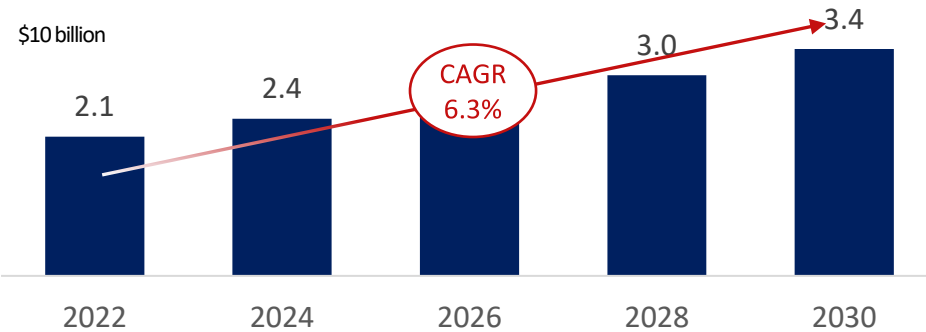




01 Multiple Myeloma(MM) Global and Domestic Market

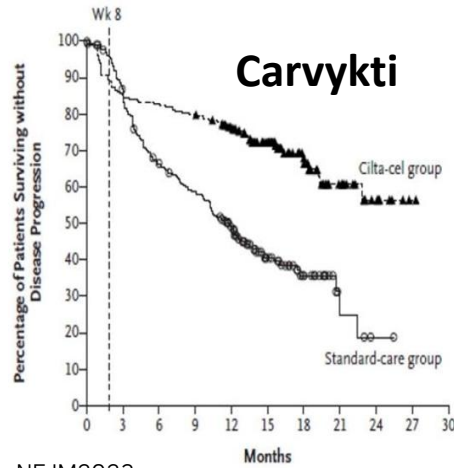
 Potential for superior efficacy and cost advantage over existing CAR-T therapies

Global Multiple Myeloma Therapeutics Market¹

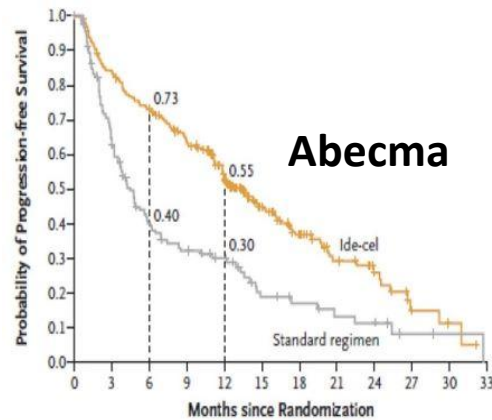


Status of CAR-T Therapies for Multiple Myeloma^{2,3}

Product Name	Approvals	Cost
Abecma (BMS) Carvykti (J&J)	Approved (Not Yet Launched in Korea)	\$455K ⁴ \$411K ⁴



NEJM2023



- Relapse & resistance over time
(antigen loss, immune evasion, CAR-T exhaustion)
- Adverse events: Cytokine release syndrome (CRS), Neurotoxicity, etc.



01 MM Competition Status in Global and Domestic

 The only BCMA-CAR-MILs in the world

Pipeline	Antibody	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Approval	Status
VCB-1201	BCMA CAR-MILs	Relapsed/Refractory Multiple Myeloma						• Ongoing preclinical trial
Abecma (BMS) Carvykti (J&J)	BCMA CAR-T	Relapsed/Refractory Multiple Myeloma						• 2021.03 FDA approval • 2022.02 FDA, approval
Fucaso(IASO)	BCMA CAR-T	Relapsed/Refractory Multiple Myeloma						• 2023. 06 China approval • 2025. 10 GC cell license in KOR
Rimqarto (Curocell)	CD19 CAR-T	Relapsed/Refractory Large B-cell lymphoma						• 2024.04 KFDA approval filing

Competitiveness

- Minimize off-target side effects with precise targeting
- Reduces the immune avoidance and recurrence thru multiple tumor antigen recognition
- Possible application of MIL platform-based antigen extension
- Structural design to cope with multiple myeloma antigen heterogeneity
- Selection of the KDDF Project in April 2025

02 Mode of Action

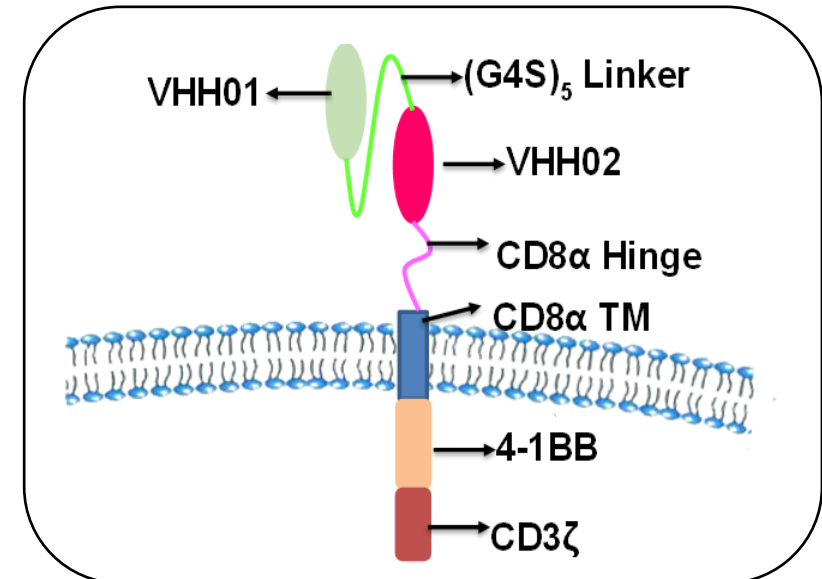
VCB-1201 (BCMA CAR-MILs)

MILs (Marrow Infiltrating Lymphocytes)

- Cell Source: Autologous Marrow Infiltrating Lymphocytes (MILs)
- Cell Type: Tumor antigen-specific Central Memory T cells
- Expansion: Proprietary ex vivo activation/expansion platform
- Key Features: Bone marrow homing, long-term persistence, targeted cytotoxicity

BCMA CAR-MILs

- Target: BCMA (B-cell maturation antigen)
- CAR Design: Dual-epitope nanobody (VHH01, VHH02)
- Structure: CD8 α hinge & transmembrane domain
- Signaling Domains: 4-1BB (co-stimulatory), CD3 ζ (activation)



02 Mode of Action

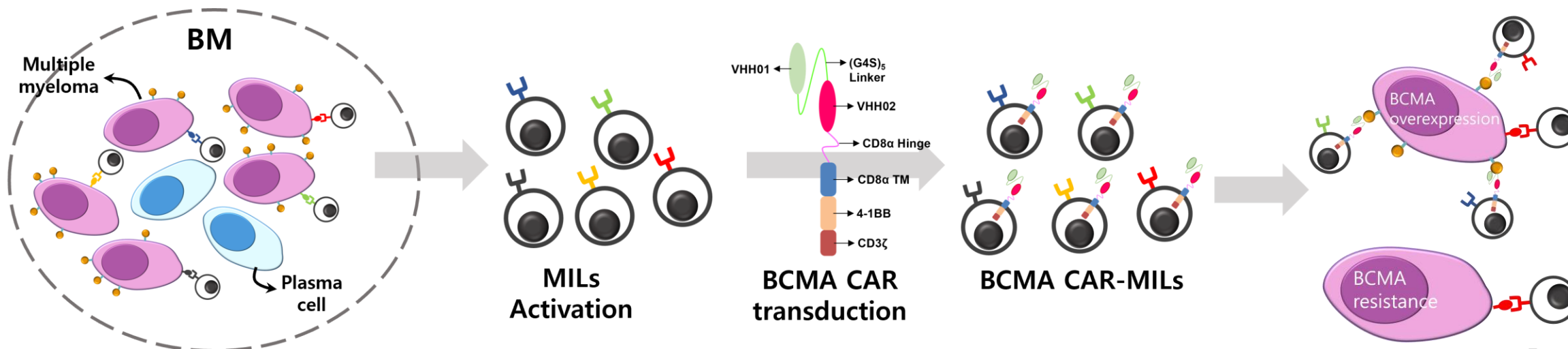
VCB-1201 (BCMA CAR-MILs)

BCMA CAR-MILs

- Specifically recognizes and kills BCMA-expressing multiple myeloma cancer cells.

BCMA CAR-MILs

- Specifically recognizes and kills various multiple myeloma tumor antigen cancer cells.
- Increased targeted homing to bone marrow / Long-term persistence in the body.





03 Target Product Profile

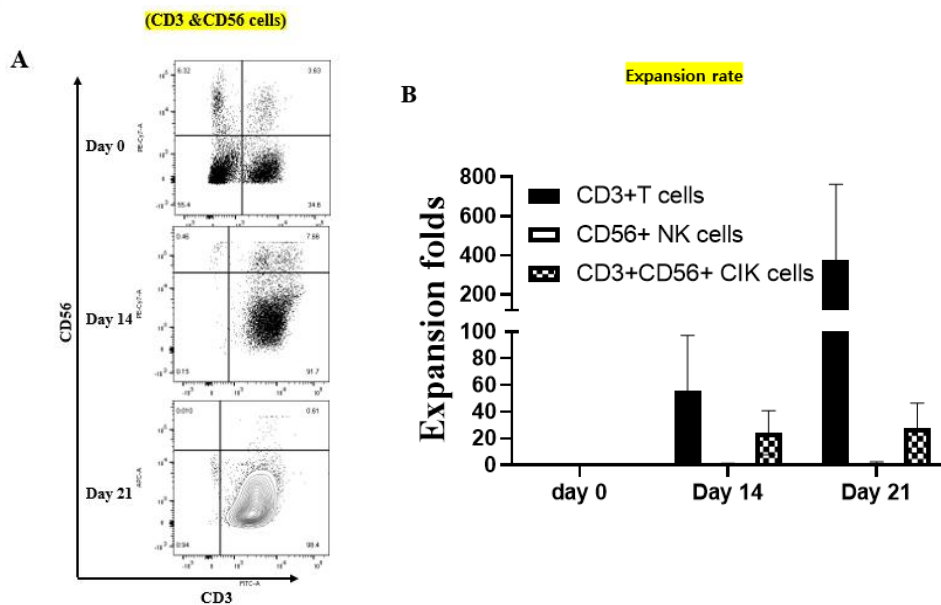
 The only BCMA-CAR-MILs in the world

Name	VCB-1201 (BCMA CAR-MILs)
Primary Indication	Relapsed/Refractory Multiple Myeloma
Patient Population	MM patients who do not respond to at least 4 previous lines of treatment Patients expressing BCMA antigen Multiple myeloma patients who have developed resistance to CAR-T
Delivery Mode	Intravenous Injection
Dosage Form	Patient-derived cell and gene therapy
Regimen	Single administration (To confirm safety, efficacy, and long-term persistence)
Efficacy	Objective Response Rate (ORR) $\geq$ 90% (Minimum $\geq$ 70%) Complete Remission $\geq$ 40% (Minimum $\geq$ 20%)
Side Effects	Cytokine Release Syndrome (CRS) $\leq$ 30% Neurotoxicity Incidence $\leq$ 5%

04 Technical Feasibility

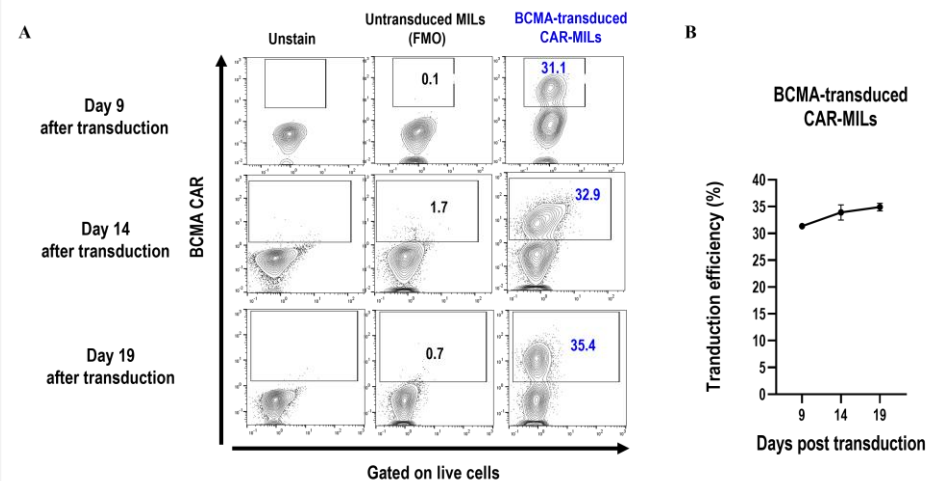
VCB-1201 (BCMA CAR-MILs) Preclinical Trial Results

Ex Vivo Expansion Rate of MILs



- 400× expansion by Day 21 (proprietary platform)
- Overcomes low CAR-T expansion in MM

CAR Transduction Efficiency

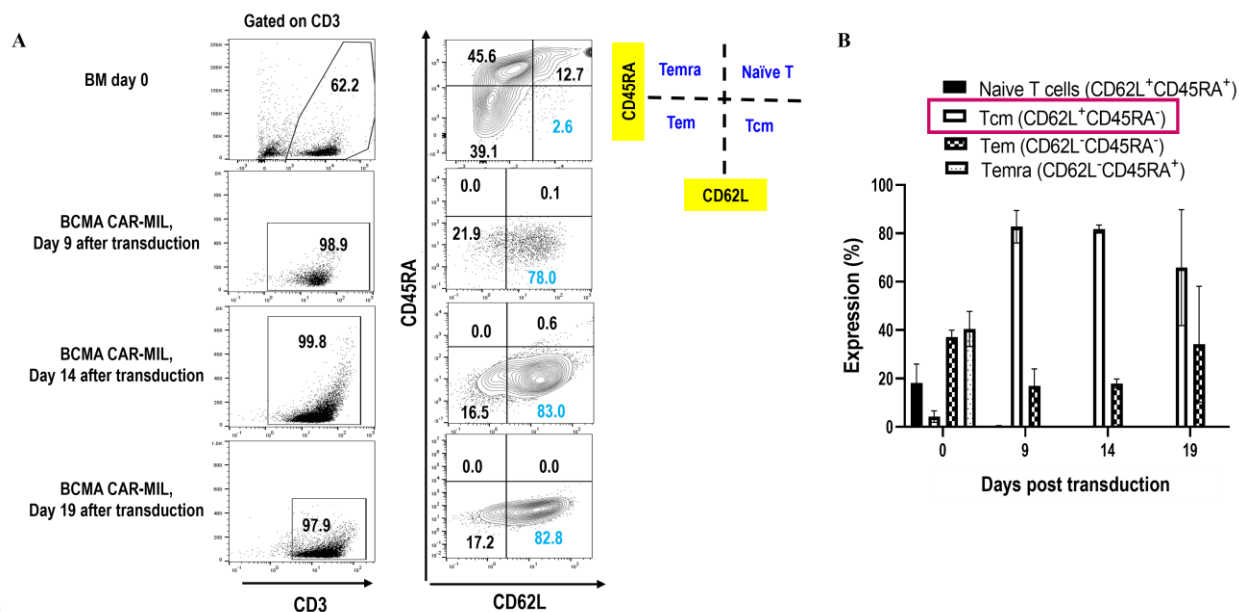


- 30% CAR transduction efficiency
- Stable efficiency over 3-week culture

04 Technical Feasibility

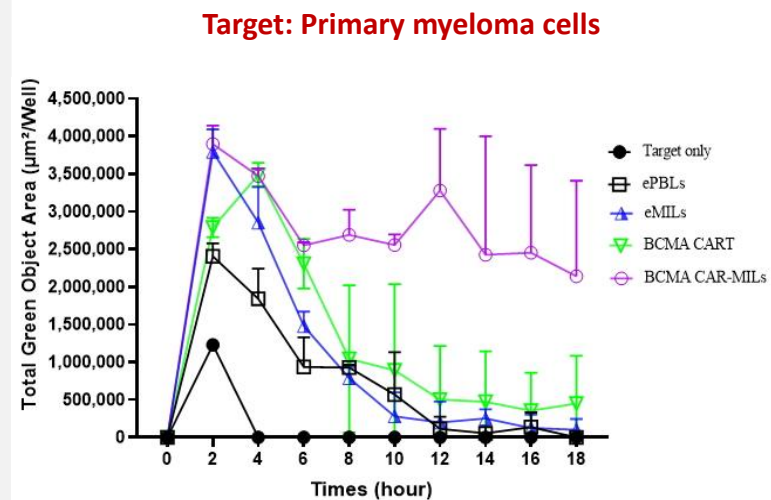
VCB-1201 (BCMA CAR-MILs) Preclinical Trial Results

High Proportion of Central Memory T Cells (Tcm) Post-Expansion



- 80% Tcm expansion and maintenance
- Long-term persistence via antigen-specific memory function

Cytotoxicity: CAR-MILs vs. CAR-T

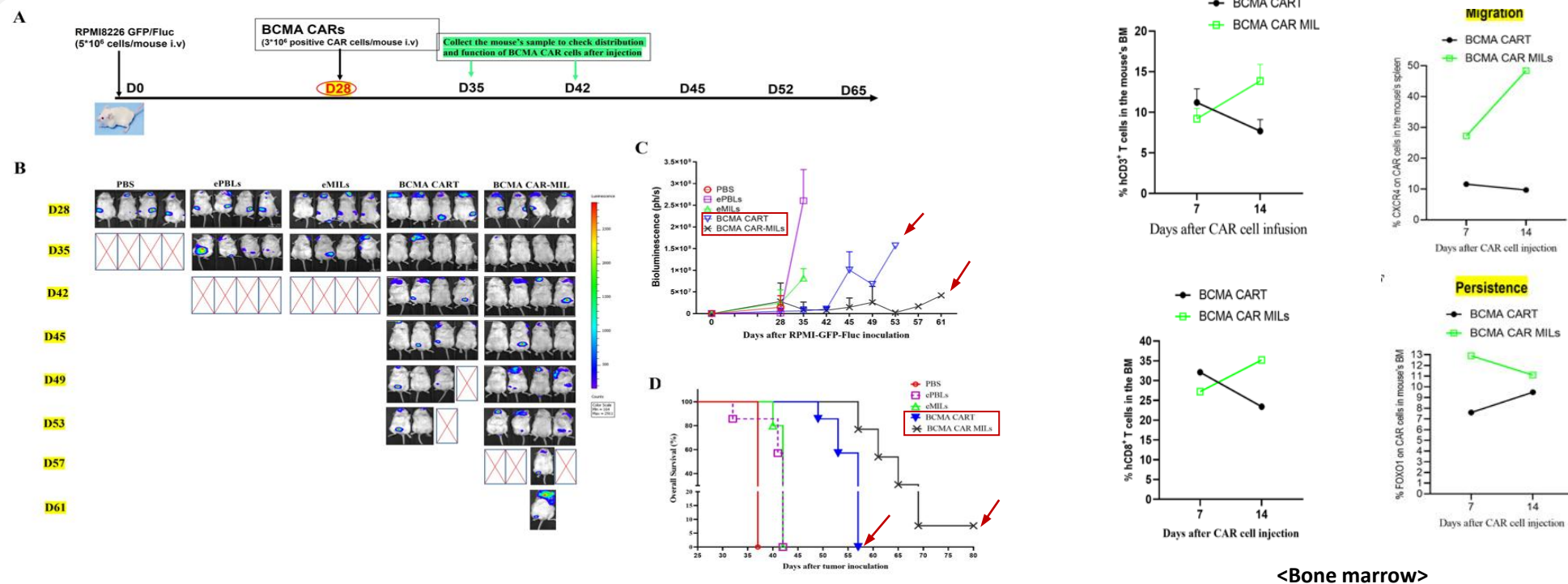


- Specific cytotoxicity to BCMA+ cell lines
- Sustained activity against primary MM cells

04 Technical Feasibility

VCB-1201 (BCMA CAR-MILs) Preclinical Trial Results

Disease Model Comparison – Multiple Myeloma disease models (RPMI8226-Luc), CAR-MILs vs. CAR-T

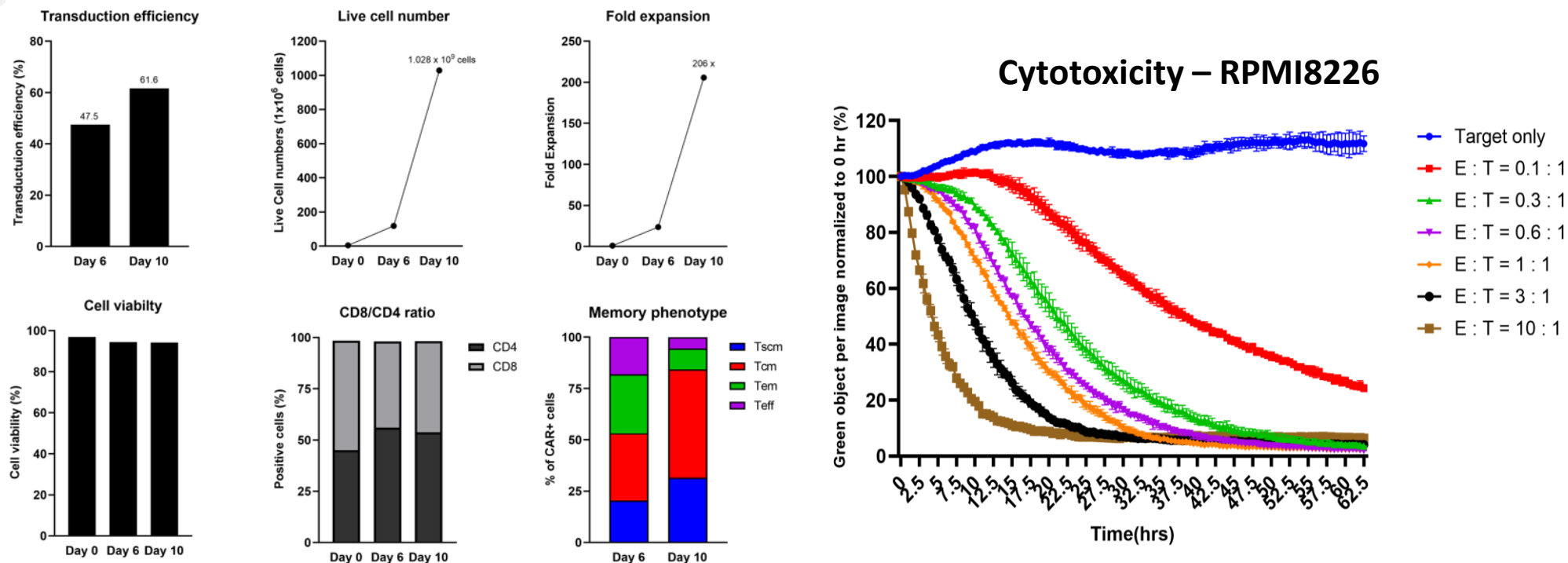


- Superior tumor suppression and survival vs. CAR-T
- Enhanced bone marrow homing and persistence vs. CAR-T

04 Technical Feasibility

VCB-1201 (BCMA CAR-MILs) Preclinical Trial Results

Establishment of Scalable Manufacturing Process for CAR-MILs

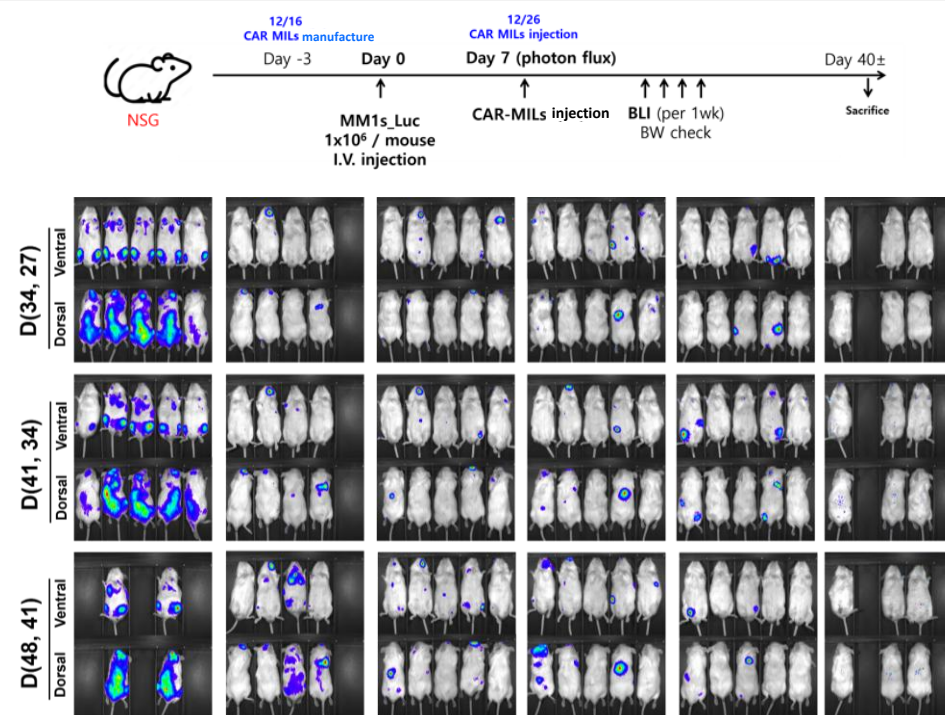
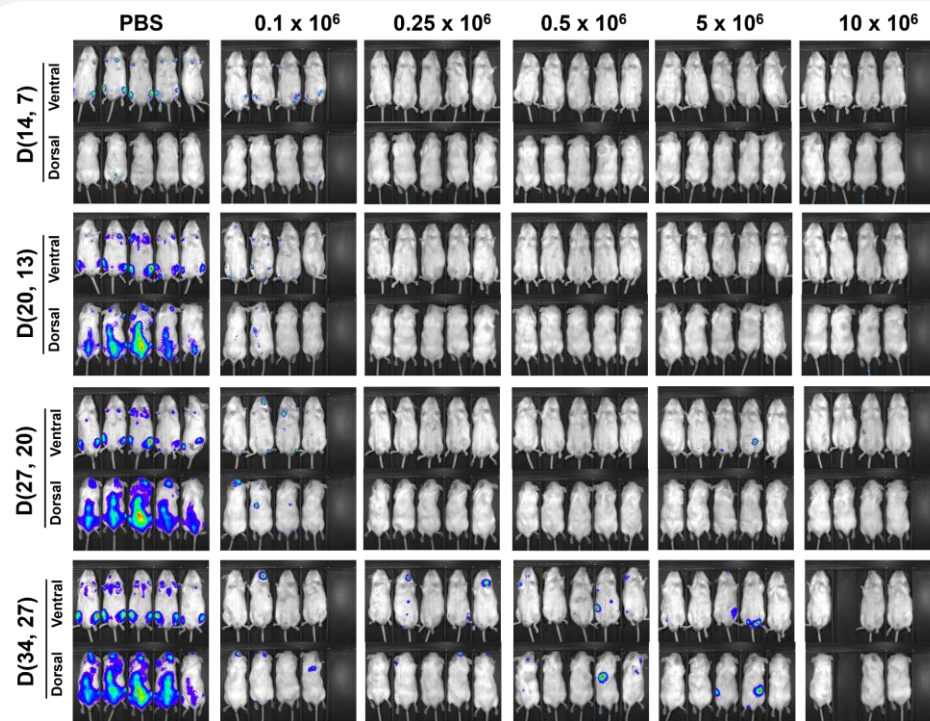


- 10-day manufacturing process; ~1x10⁹ CAR-MILs yield
- CAR transduction improved to ~60%
- 80% Tcm enrichment & Strong cytotoxicity vs. RPMI8226 MM cells

04 Technical Feasibility

VCB-1201 (BCMA CAR-MILs) Preclinical Trial Results

Dose-Dependent Efficacy & Preliminary Toxicity of BCMA CAR-MILs in MM1.S-Luc Model



- Tumor inhibition observed from 0.1x10⁶ dose
- No observable toxicity at 5x10⁶ dose

05 Business strategy

VCB-1201 (BCMA CAR-MILs) Intellectual Property

특허청
출원번호통지서

출원일자: 2023.11.13
특기사항: 심사청구(무) 공개신청(무)
출원번호: 10-2023-0156399 (접수번호 1-1-2023-1252025-40)
(DAS접근코드: J38)
출원인명칭: 전남대학교산학협력단(2-2004-036577-5)
대리인성명: 최은선(9-2003-000221-0)
발명자성명: 이재준, 배만공, 정성훈
발명의명칭: 신규한 골수 침윤 림프구 집단 및 이의 제조방법

특 허 청 장

== 안내 ==

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Manufacturing
Method of MILs

특허청
출원번호통지서

출원일자: 2024.01.24
특기사항: 심사청구(무) 공개신청(무)
출원번호: 10-2024-0011223 (접수번호 1-1-2024-0096321-45)
(DAS접근코드: A379)
출원인명칭: 주식회사 박셀바이오(1-2015-049273-5) 외 2명
대리인성명: 최은선(9-2003-000221-0)
발명자성명: 이재준, 배만공, 정성훈, 왕민
발명의명칭: BCMA 특이적 키메라 항원 수용체를 포함하는 신규한 골수 침윤 림프구 집단 및 이의 제조방법

특 허 청 장

== 안내 ==

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Manufacturing
Methods of BCMA CAR-MILs

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접수일:	28 February 2024																		
수리관청:	Korean Intellectual Property Office																		
광조기호:	VCB23P-0001-WO																		
출원인:	주식회사 박셀바이오																		
출원인 수:	3																		
발명의명칭:	BCMA 특이적 키메라 항원 수용체를 포함하는 신규한 골수 침윤 림프구 집단 및 이의 제조방법																		
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제출자: 최은선 씨 (고객 아이디: user_KR_주식_박셀_001_1_1_1_6393)
접수 타임스탬프: 28 February 2024 17:32 UTC+9 (KST)
제출에 관한 공식 요약: B6:8F:E8:69:1E:27:AD:ED:64:DC:23:05:52:DC:97:34:DF:61:6A:BB

/RO/KR/

(PCT) Manufacturing
Methods of BCMA CAR-MILs

- Patent applications for the manufacturing methods of MILs and BCMA CAR-MILs have been completed
- PCT application has been filed for national phase entry



05 Business strategy

Development status and commercialization strategy

Pipeline	Timeline	2026	2027	2028	2029	2030	2031
VCB-1201 (BCMA CAR-MILs)	Preclinical study						
	CAR-T Manufacturing, Quality control, Safety						
	IND Approval						
	Clinical Study Phase I, II						
	Regulatory Approval & Commercialization						
	Collaboration, Commercialization, Licensing-out						



Thank You

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