



Transforming the Treatment Paradigm in Neurologic Autoimmune Diseases

August 2026

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Pioneering Differentiated Therapies with Curative Potential to Free People from Life-long Autoimmune Diseases and Chronic Therapy



Transformative Impact of Miv-cel

A **differentiated single-dose CAR T** therapy designed to **reset the immune system** and deliver the potential for **durable, drug-free, disease-free remission**

Focused Neurologic Autoimmune Strategy

First-to-market opportunity with a focused **neuro franchise strategy**, enabling efficient market entry with SPS and future scale for gMG, and beyond

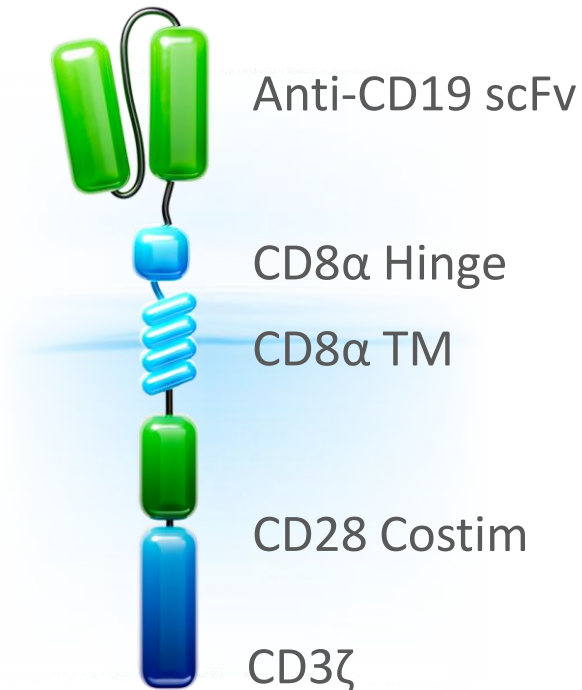
Innovating to Expand Access

Extend leadership by **broadening access and choice for patients** (e.g., indication expansion, no/alternative preconditioning regimens, manufacturing enhancements)

Miv-cel: Potential First- and Best-in-Class CAR T Designed for Potency & Tolerability

Mivocabtagene Autoleucel (miv-cel)^{1,2}

Only Fully Human Autologous CD19
CAR T With CD28 Costim

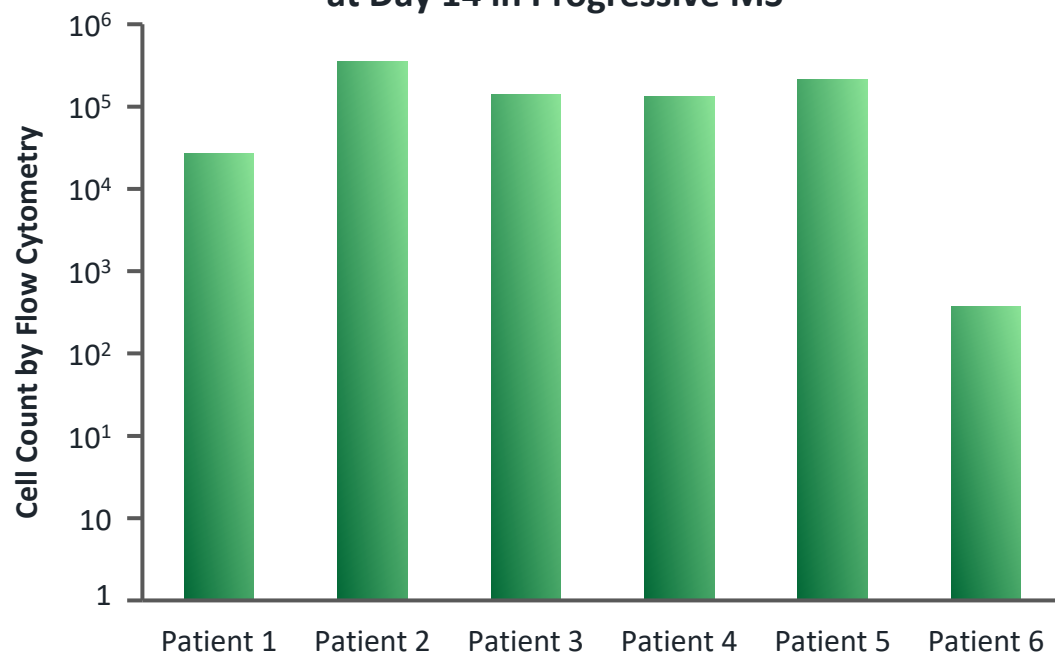


- **More than 100** patients dosed with miv-cel across multiple indications³
- **Deep and broad depletion of peripheral- and tissue-resident B cells to support broad immune reset and durable remission^{4,5}**
- **No high-grade CRS or ICANS³**
- First SPS and gMG patients treated with a single-dose of miv-cel achieved **durable efficacy beyond 24 months without the need for chronic immunotherapies⁶**

Miv-cel's Profound MOA and Ability to Penetrate the CNS

CNS Penetration and Expansion

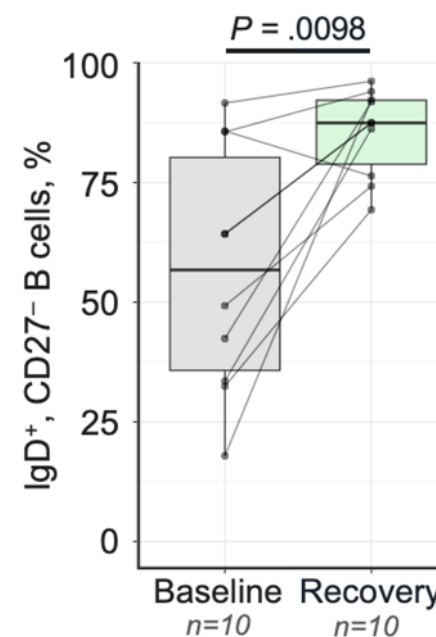
CAR⁺ Miv-cel Expansion Detected in CSF at Day 14 in Progressive MS^a



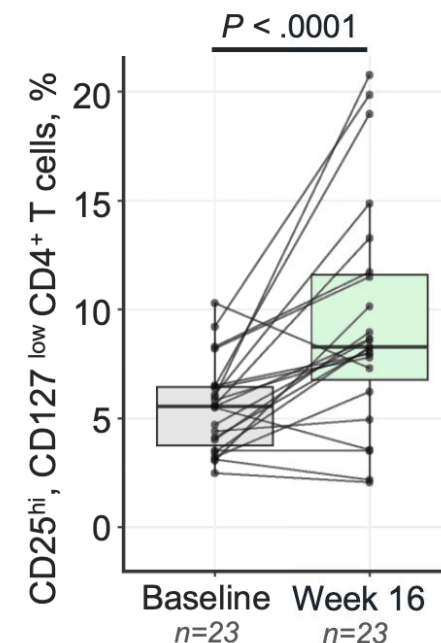
Acts directly on cells in the CNS at the site of disease¹

Broad Immune Reset

Naïve B Cells in SPS



Regulatory T Cells in SPS



Resets B cells and upregulates regulatory T cells, important suppressors of CNS disease inflammation²⁻⁴

^aAbsolute numbers of CAR T cells in CSF assumed a volume of 140 mL and 150 mL for female and male, respectively.

CAR, chimeric antigen receptor; CNS, central nervous system; CSF, cerebrospinal fluid; miv-cel, mivocabtagene autoleucel; SPS, stiff person syndrome.

1. Dunn J, et al. Presented at the ACTRIMS Forum 2026; February 5-7, 2026; San Diego, CA. Poster 112. 2. Piquet A, et al. Presented at the AAN Annual Meeting 2026; April 18-22, 2026; Chicago, IL. LBA Poster 8. 3. Goverman JM. *N Engl J Med.* 2021;384:578-580. 4. Harkins AL, et al. *Crit Rev Immunol.* 2022;42:1-27.

Significant Market Opportunity for Miv-cel in SPS and gMG



Stiff Person Syndrome

- **First-to-market** opportunity with highly efficient infrastructure
- **~6K U.S. diagnosed patients**¹
- **Progressive and debilitating rare disease with no approved therapies**
- **High-cost burden** (~\$0.7 to \$1.5M 3-yr cost per patient)²
- **Immediately addressable** patients
- **Highly concentrated** treatment network



Generalized Myasthenia Gravis

- **~80K U.S. diagnosed patients**^{3,4}; growing market
- **Significant unmet need** with current SOC
- **High-cost burden** (~\$2M 3-yr cost per patient)^{5,6}
- Opportunity to **change the treatment paradigm**
- **Strong commercial synergies** with SPS enables efficient scaling

**Miv-cel Could Deliver Significant Value to Patients and Healthcare System,
Supporting Premium Pricing Potential**

SOC, standard of care.

1. Crane PD, et al. *Neurology*. 2024;103(12):e210078. 2. Merative 2025 HCRU Analysis of Commercial Chronic Immunotherapy SPS patients.

3. Rodríguez E, et al. *Muscle. Nerve*. 2024;69(2):166-171. 4. Hendricks TM, et al. *Am J Ophthalmol*. 2019; 205:99-105. 5. ICER Report on MG 2021. 6. Global Data Pricing database.

Robust Near-Term Catalysts for Prioritized Neurologic Pipeline Indications

Indication	Anticipated Milestones
Stiff Person Syndrome RMAT, ODD	+ Complete rolling BLA submission in Q4 2026 + Report one-year follow-up data in Q3 2026
Generalized Myasthenia Gravis RMAT, ODD*, FTD†	+ Report longer-term follow-up Phase 2 data in Q3 2026 + Complete enrollment in Phase 3 expected mid-2027
Progressive Multiple Sclerosis RMAT for naSPMS	+ Share development strategy by early 2027 + Report additional data from Phase 1 IIT in 2H 2026



Stiff Person Syndrome (SPS)

SPS is a Debilitating, Progressive Autoimmune Disease with No FDA-Approved Therapies



SPS impacts the inhibitory signaling pathways, which are the body's braking system and the **target of autoantibodies** produced by B cells in SPS^{1,2}



Symptoms characterized by **muscle stiffness** and **painful muscle spasms**, impacting mobility¹⁻³



Inadequate response with off-label symptomatic and immunomodulatory therapies^{1,2,5}

Devastating Impact on Patients

80% of patients lose mobility, needing walking aid assistance or wheelchair¹⁻³

Only ~19% of patients remained able to work after 4 years⁴

“Freezing attacks” and sudden falls requiring ER care^{1,2}

Risk of **permanent disability** and **increased mortality**³

KYSA-8 Patient Perspectives: Before and After Treatment with Miv-cel



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[View Video](#)



[View Video](#)

Potential for Miv-cel to Quickly Set a New Treatment Standard in SPS with >2,000 Patients Immediately Addressable



~6k

**U.S. Diagnosed
SPS Patients^{1,2}**



**Miv-cel
Addressable Market^{2,3}**

**Immediately Addressable
Market**

~2.0 to 2.5k Patients

*30-40% of total diagnosed
Patients treated with
off-label immunotherapy*

**Total Miv-cel
Addressable Market**

~5.5k Patients

*90% of total diagnosed
Patients treated with
symptomatic therapies*

Expect to Capture Total Addressable Market Over Time Due to Progressive Nature of Disease

Immunotherapy defined as off-label immunosuppressants (e.g., prednisone), rituximab and/or intravenous immunoglobulin.

1. Crane PD, et al. *Neurology*. 2024;103(12):e210078. 2. Analysis of 2024 Komodo U.S. Claims Data.

3. Kyverna Patient Journey and Demand Study (data on file).

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SPS Treaters Show Strong Enthusiasm for Early Adoption of Miv-cel



Survey of 20 high volume
SPS treaters in U.S.



Product profile based on
miv-cel topline data

80% view efficacy data
and one-time treatment
as key attributes

90% view profile as
compelling versus current
treatment options

85% would use miv-cel
for moderate-to-severe
patients at launch

Focused Launch Strategy Targeting ~10 High-Value SPS Centers



Neuro Leadership

- Thought leaders / SPS high-volume treaters
- Institutional support for miv-cel



Addressable Patients

- Meaningful portion of immediately addressable patients at ~10 centers
- Strong referral network



CAR T Expertise

- Commercial CAR T experience
- Accreditation



Economic Potential

- Robust inpatient and outpatient models
- Commercial payer and Medicare dynamics

Targeted Launch Expected to Enable Efficient Go-to-Market Approach

Flexible and Scalable Manufacturing Model



- ✓ **Dual-source** U.S. manufacturing strategy
- ✓ **>98% manufacturing success rate** driven by robust process, quality systems, and technical expertise
- ✓ Commercial agreement with Elevate **supports SPS and future commercial launches and clinical demand**
- ✓ Miv-cel cost of goods supports potential for **biologics-like margins**



elevatebio®



 **Minaris**
Advanced Therapies

SPS Registrational Trial – Rolling BLA Submission Underway;

On track to Complete Submission in Q4 2026



KYSA-8: Open-label, single-arm, multicenter study

N = 26

- Age 18 to 75 years
- Diagnosis of SPS
- Inadequate response to immunomodulatory therapy
- Stiffness index ≥ 2

Miv-cel

Low-dose Cy/Flu
preconditioning

+

Single infusion of
 1×10^8 CAR T cells

SPS immunotherapies are
discontinued

Primary endpoints:

- Change from baseline in T25FW at 16 weeks
- Safety

Secondary endpoints: change from baseline at 16 weeks

- Modified Rankin Scale (mRS)
- Distribution of Stiffness Index (DSI)
- Hauser Ambulation Index (HAI)
- Heightened Sensitivity Scale (HSS)



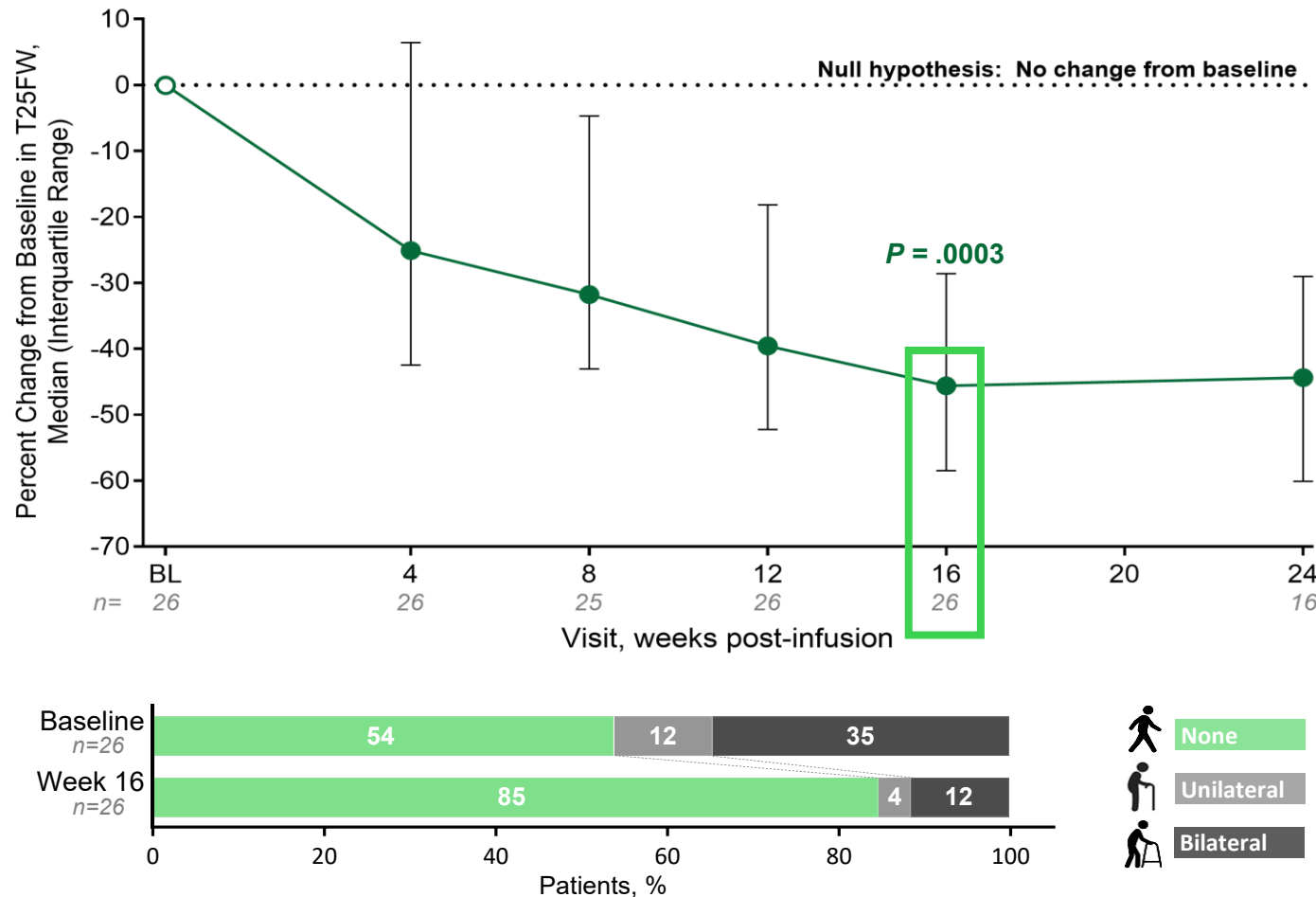
One-
year
Follow
Up

Miv-cel Granted ODD and RMAT Designations by FDA for SPS

Primary Endpoint Met: Single-dose Miv-cel Achieved Statistically Significant Improvement in T25FW - 46% Median Improvement at Week 16



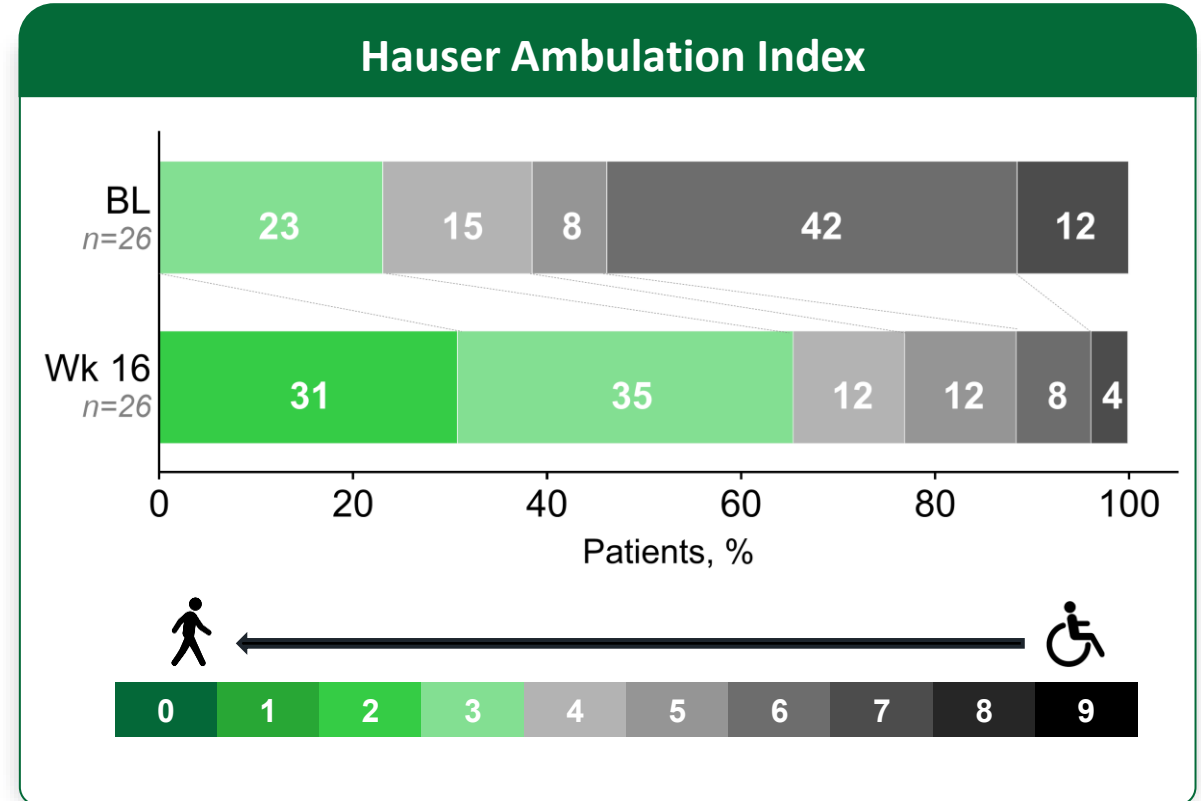
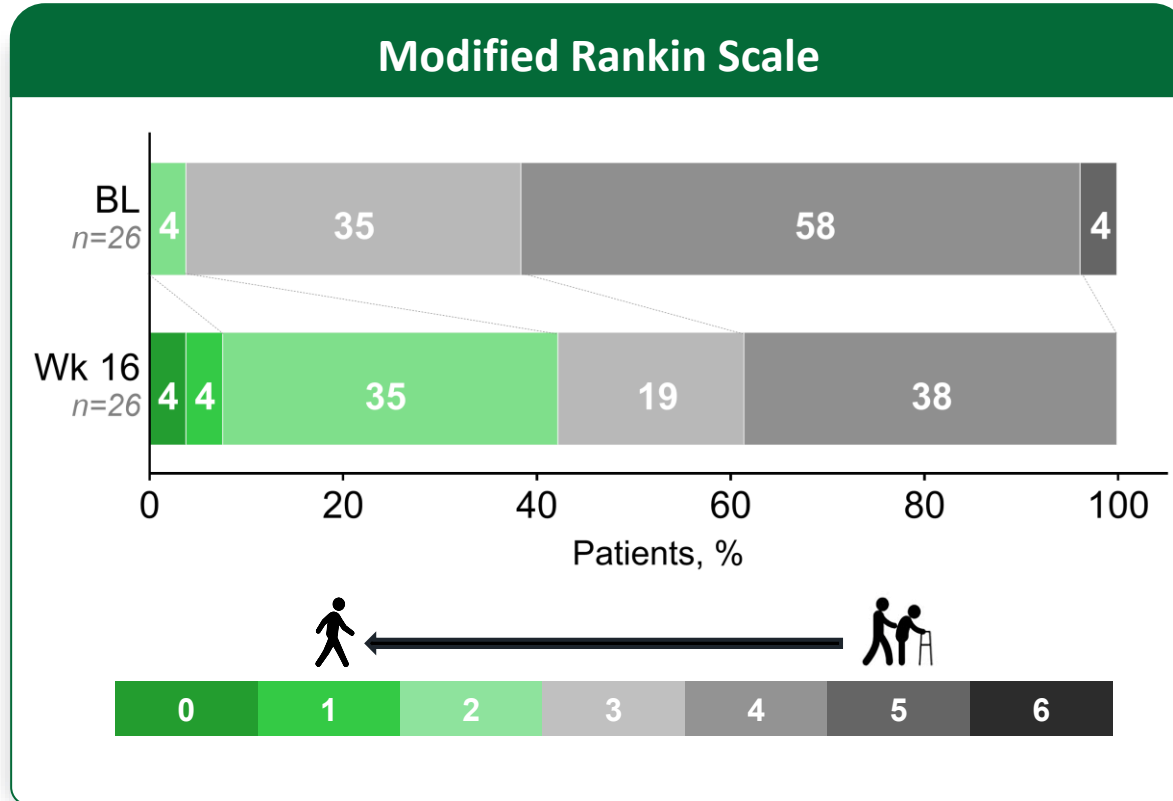
Significant T25FW Improvement and Reduced Walking Aid Use



- 81% of patients achieved **clinically meaningful improvement** ($\geq 20\%$ reduction from baseline)¹
- 31% completed T25FW in < 5 seconds; **typical time for healthy adults**²
- Of the 12 patients requiring a walking aid for T25FW at baseline, 67% (8/12) **no longer needed assistance** at week 16
- As of week 16 and through last follow-up, all 26 (100%) patients remained **free of immunomodulatory or immunosuppressant therapies** for SPS*

*Includes Includes IVIg/SCIg, PLEX, rituximab and/or prednisone (≥ 20 mg/day) for SPS symptoms.
N=26. Data cutoff: 26Nov2025. Percentages may total more than 100% due to rounding. BL, baseline; T25FW, timed 25-foot walk.
1. Hobart J, et al. *Neurology*. 2013;80(16):1509-17. 2. Motl RW, et al. *Mult Scler*. 2017;23(5):704-710.

All Secondary Endpoints Achieved Statistically Significant Improvements ($P < .0001$) in Disability, Mobility, Stiffness, and Hypersensitivity



- Significant ($P < .0001$) mean improvements in mRS and HAI of -0.8 (SD, 0.86) and -1.6 (1.13) and SPS-specific measures, DSI and HSS, of -1.5 (1.75) and -3.2 (2.01), respectively
- 96% of patients (25/26) had improvement in ≥ 1 primary or secondary efficacy endpoint

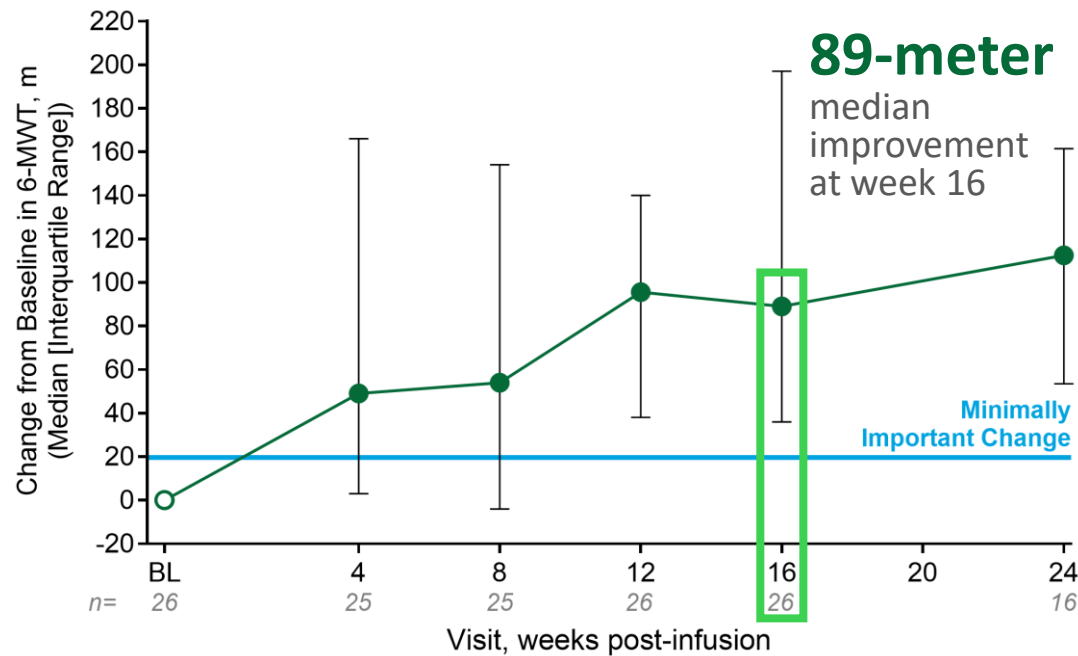
N=26. Data cutoff: 26Nov2025. Percentages may total more than 100% due to rounding.

BL, baseline; DSI, Distribution-of-Stiffness Index; HAI, Hauser Ambulation Scale; HSS, Heightened Sensitivity Scale; mRS, Modified Rankin Scale; wk, week.

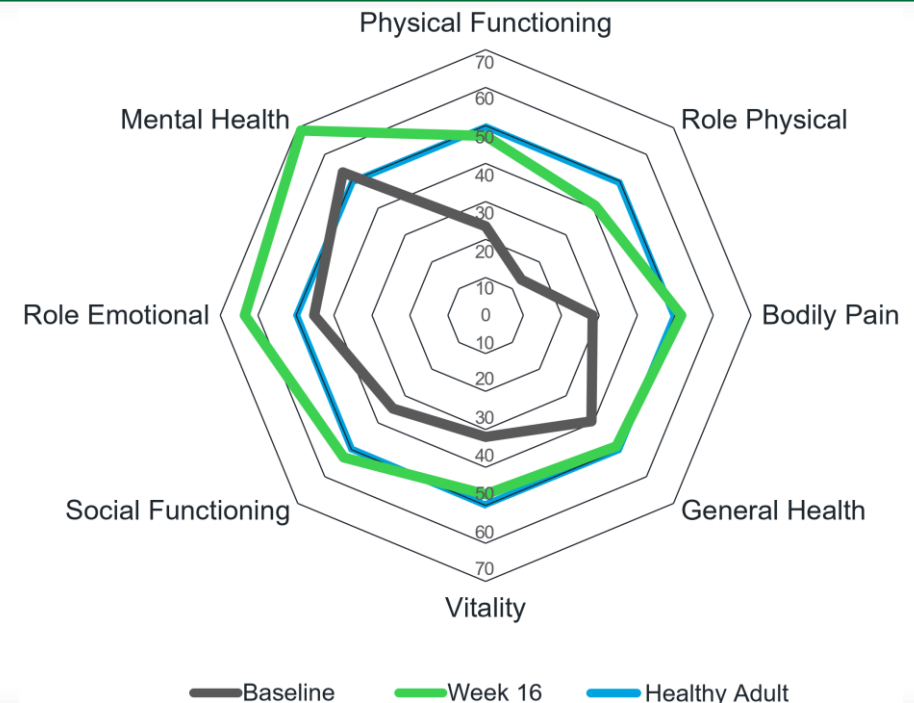
Single-dose Miv-cel Led to Substantial Improvements in Physical and Mental Functioning



6-Minute Walk Test (MWT): >4-fold improvement over clinical minimally important change¹



36-Item Short Form Health Survey (SF-36): Week 16 scores comparable to healthy adults for most domains^{2,3}



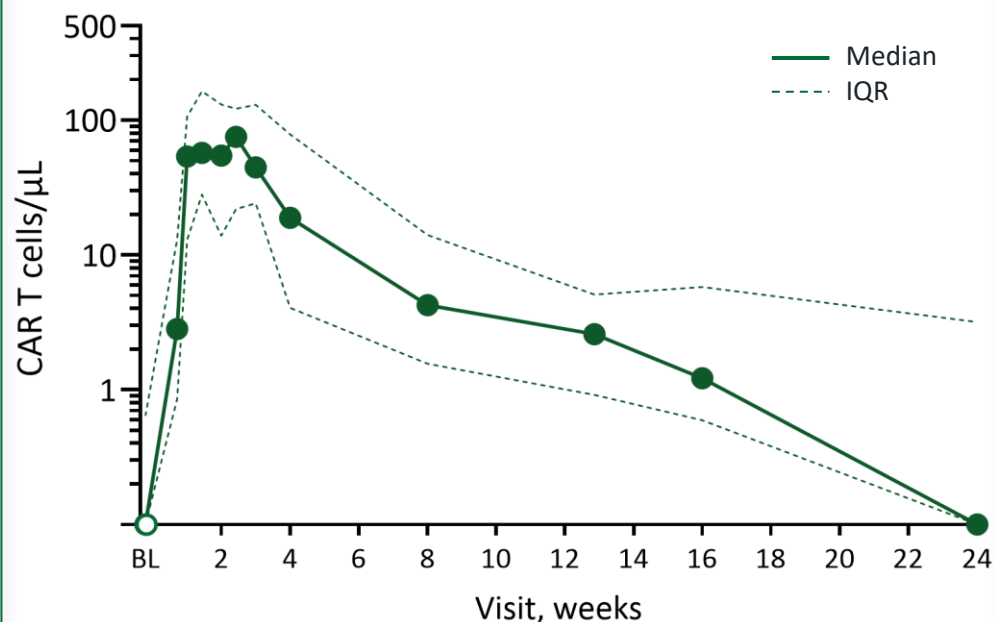
N= 26; Data cutoff: 26Nov2025.

1. Oosterveer DM, et al. Mult Scler Relat Disord. 2022;57:103438. 2. Maglinte GA, et al. J Clin Epidemiol. 2012;65(5):497-502. 3. Wu Q, et al. Medicine (Baltimore). 2023;102(24):e33979.

Robust Miv-cel Expansion Led to Complete Peripheral B-cell Depletion and Significant Reductions in Autoantibody Titers

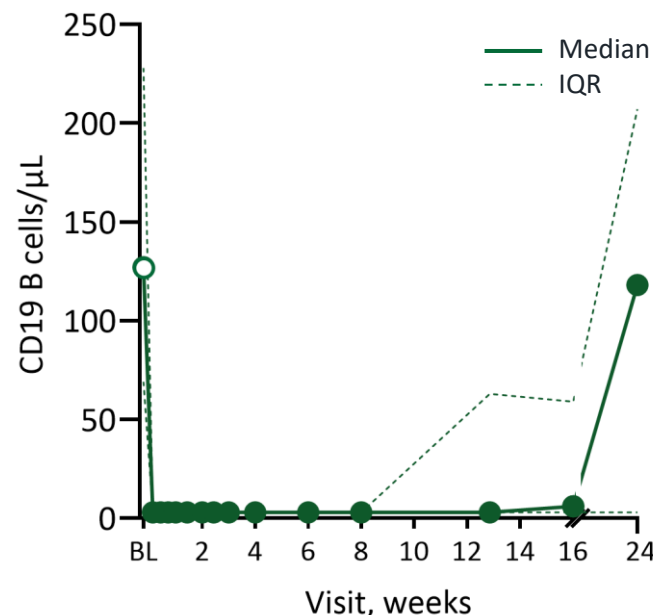


Robust CAR T-cell Expansion



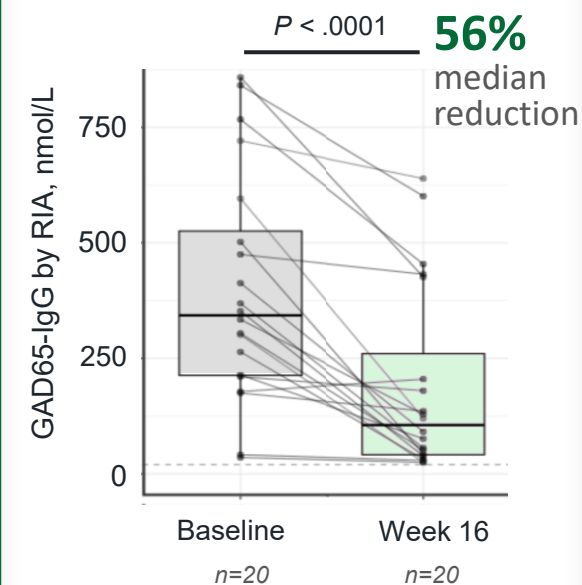
- CAR-positive T cells peaked by day 14

Deep B-cell Depletion



- 54% of patients had B-cell reconstitution by week 16
- Efficacy was maintained with B-cell reconstitution

Reduced GAD65-IgG

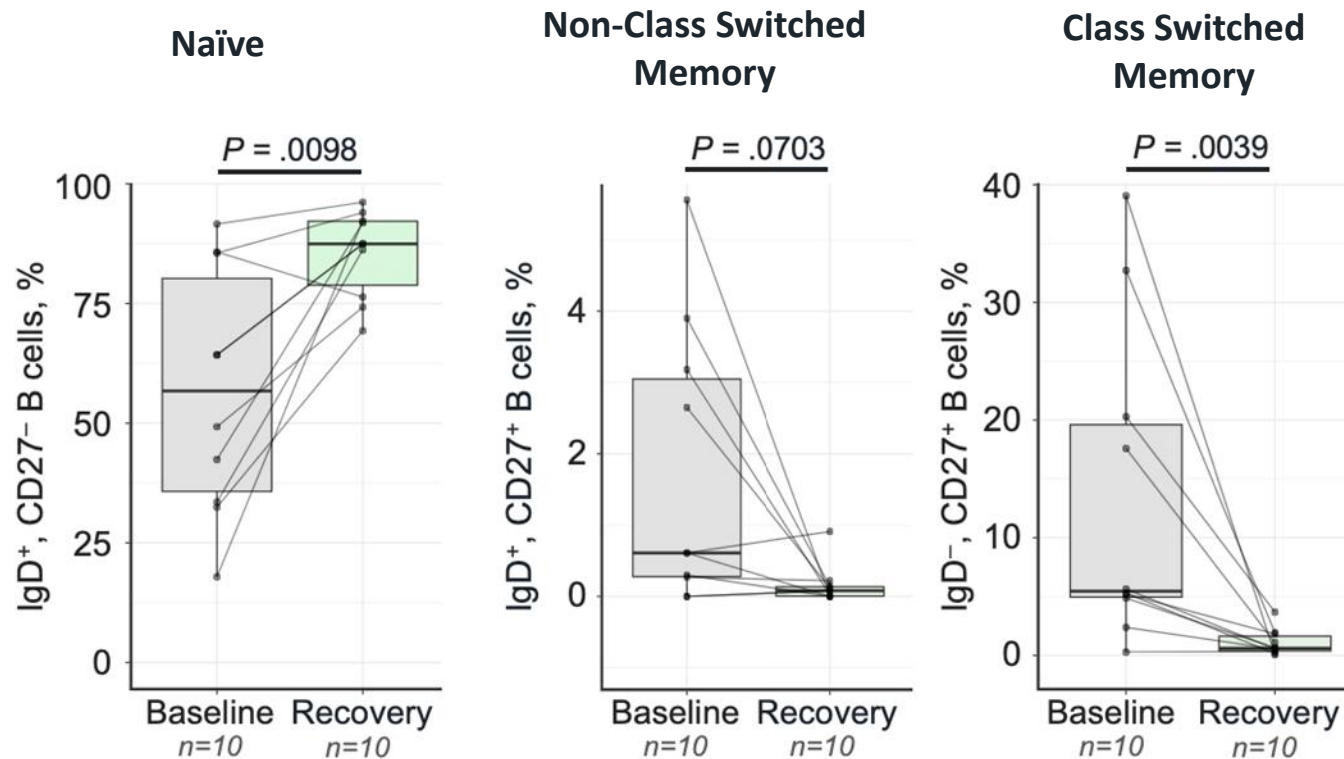


- GAD65-IgG was reduced in 19/20 patients with ≥ 20 nM GAD65 at baseline

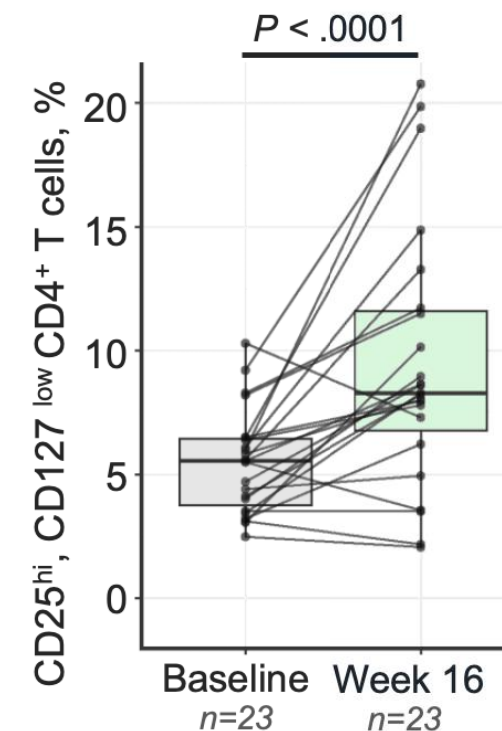
Single-dose Miv-cel Induced Markers of Broad Immune Reset



B-Cell Phenotypes



Regulatory T Cells



- Newly emerging B-cell population showed significantly increased naïve phenotype with concomitant decrease in memory phenotype
- Significant increase in regulatory T cells at week 16

Miv-cel Demonstrated a Well-Tolerated Safety Profile



Treatment-Related Adverse Events, n (%)	N=26
CRS (any Grade)	24 (92)
Grade 1	10 (38)
Grade 2	14 (54)
ICANS (any Grade)	3 (12)
Grade 1	3 (12)
Grade 3/4 neutropenia	4 (15)
Any treatment-related serious AE	3 (12)

- No high-grade CRS or ICANS observed
- 4 patients had Grade 3/4 neutropenia, an expected AE with lymphodepletion and CAR T-cell therapies; all events were manageable
- Treatment-related serious AEs occurred in 3 patients; all fully resolved

Natural History Study Contextualizes Transformative Miv-cel Data and its Opportunity to Change the Treatment Paradigm



Natural History Study

Large, multicenter, retrospective study assessing T25FW in patients with SPS (n=153) over 10-year period

Patient Population

Earlier in disease course
(mRS mean 2.6; 55% immunotherapy at index date*)

T25FW

✗ No or minimal improvement[†]

Walking Aid Use

↗ 73% increase over average 5 years

mRS score

✗ No improvement

Immunotherapy Use

↗ 81% had immunotherapy by last datapoint
(47% increased usage)

KYSA-8 SPS Registrational Trial

More severe
(mRS mean 3.6; all have failed immunotherapy)

✓ 46% improvement at 16 weeks, sustained at 24 weeks

↘ 67% decrease at 16 weeks

✓ Improvement in disability scores

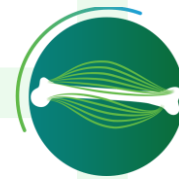
↘ No immunotherapy after single-dose of miv-cel

mRS, Modified Rankin Score; T25FW, Timed 25-foot Walk
Newsome SD, et al. Presented at the 2026 AAN Annual Meeting [abstract 966].
*Index date is the date of the first T25FW assessment. †Over average ~5 years.



Generalized Myasthenia Gravis (gMG)

Despite Available Treatment Options, High Disease Burden Remains



- gMG is a B-cell and antibody-mediated neuromuscular autoimmune disease that causes fluctuating muscle weakness and fatigue^{1,2}

Novel therapies are needed that minimize or eliminate symptoms of disease while reducing risks associated with chronic immunosuppression

Current State of Treatment



Inadequate symptom control^{3,4}



Few reach minimal symptom expression (MSE)^{1,5-6}



Majority require ongoing immunosuppressant therapy¹⁻⁴



Costly and chronic treatment options^{1,7}

Miv-cel's Potential: Durable, Drug-free, Disease-free Remission with Single Dose



~80k

**U.S. Diagnosed
gMG Patients^{1,2}**



**Miv-cel
Addressable Market^{1,3}**



**Initial
Priority**

~12k Patients

*15% of total diagnosed
Patients with inadequate
response to ≥ 1 biologic**

**Total Miv-cel
Addressable Market**

~40k Patients

*50% of total diagnosed
Patients with inadequate
response to immunosuppressants*

SPS Lays Foundation for Rapid and Efficient Market Entry in gMG



SPS

gMG

Target ~10 centers at launch

Expand to ~50-75 total centers at peak

- Meaningful portion of SPS patients at approximately 10 centers
- High-volume SPS treaters, including clinical trial sites
- Continue to activate more centers post SPS launch

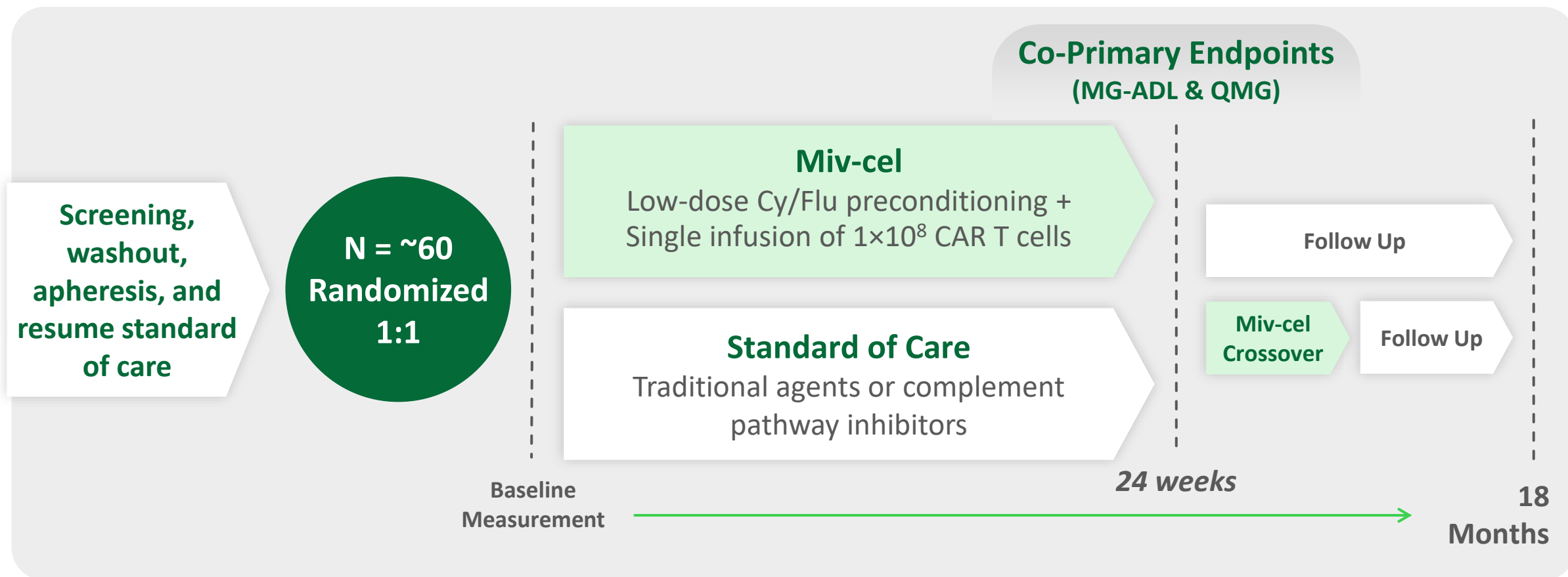
- MG/neuro centers of excellence and high-volume treaters
- Centers that also treat SPS patients
- Centers with existing referral networks for patients

..... ***Centers with Deep CAR T Expertise, Strong Neurology Partnership***
And Positive Site Economics Potential

Phase 3 Ongoing with Enrollment Expected to be Completed Mid-2027

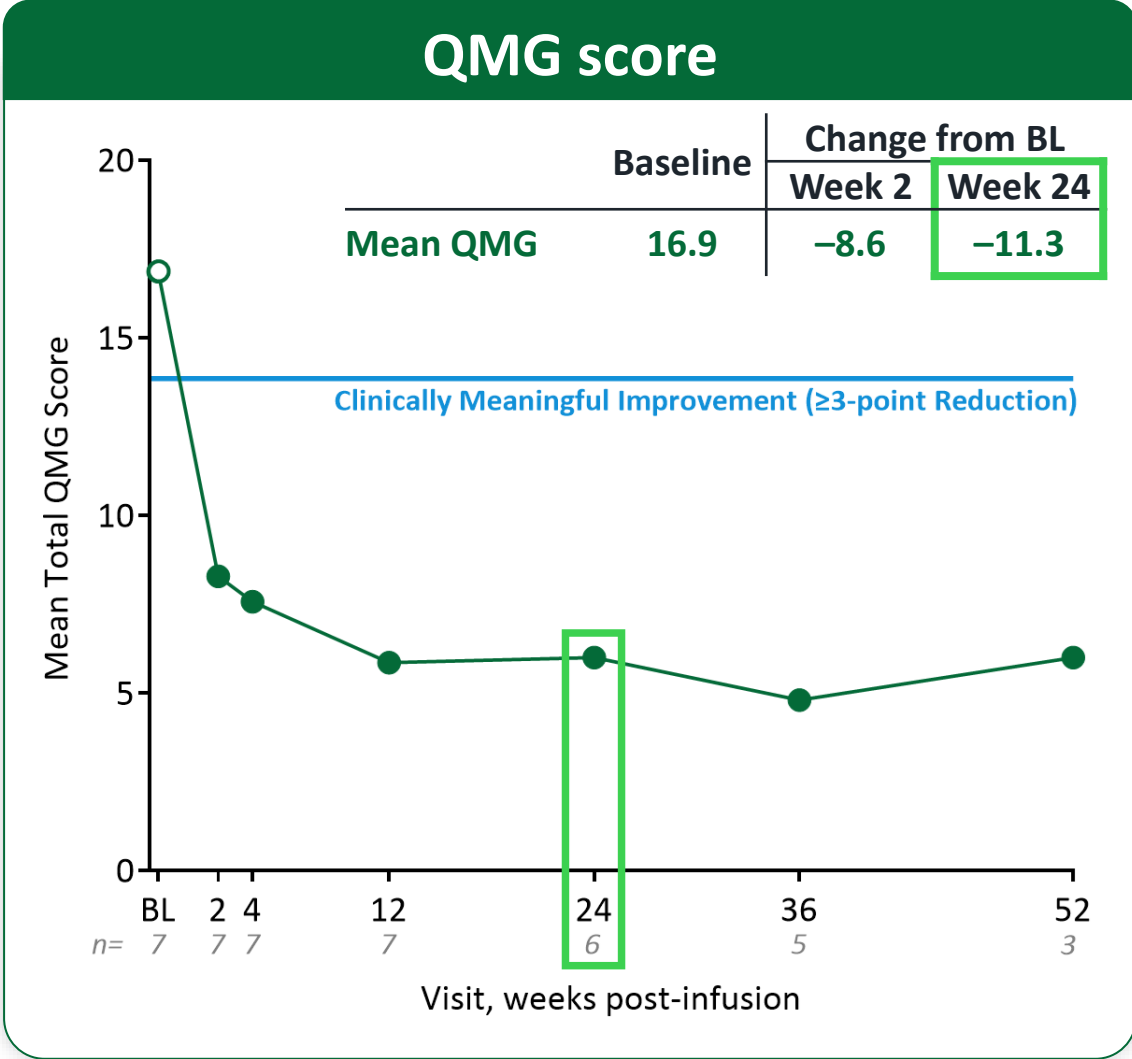
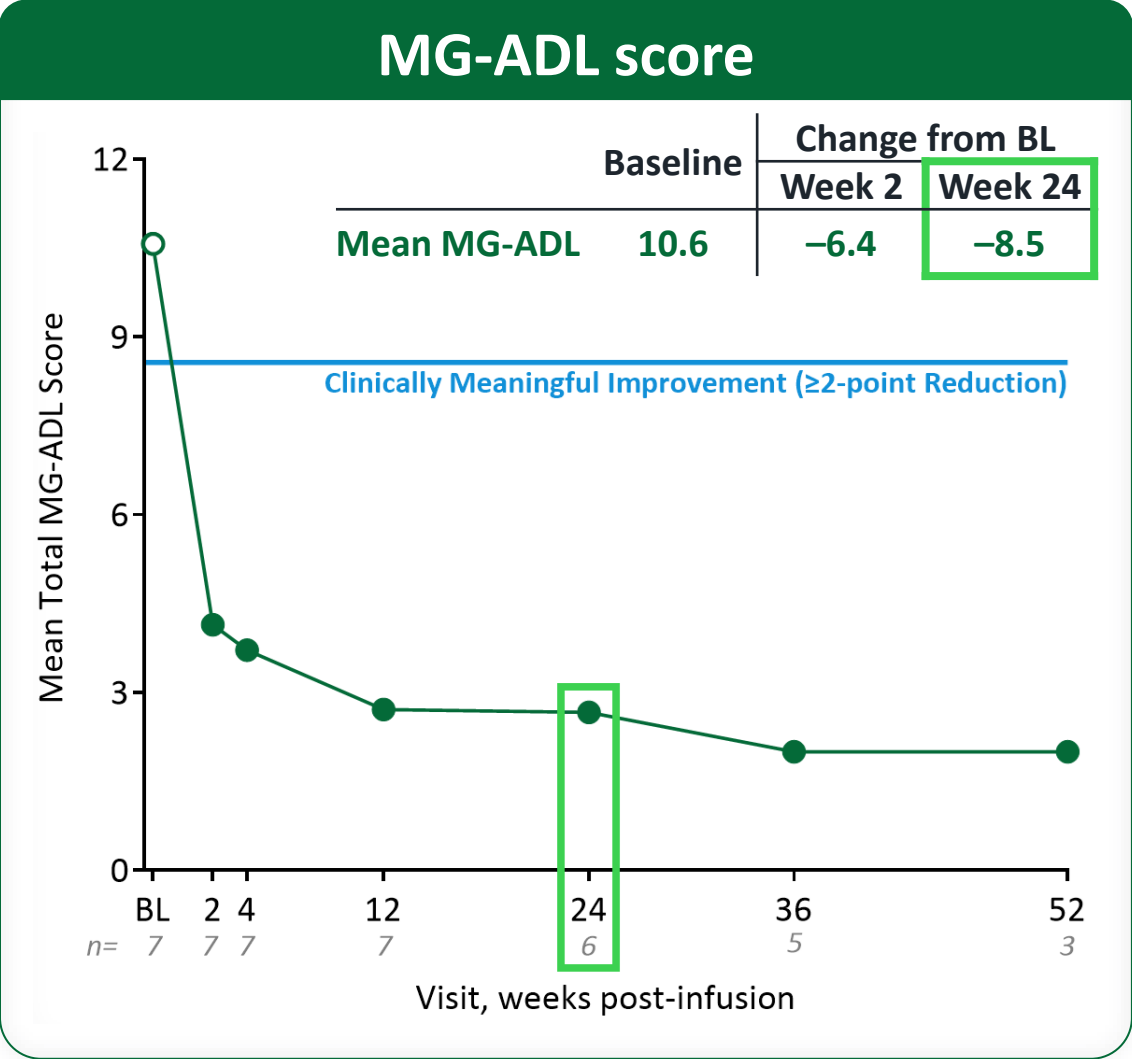


~60-patient, global, open-label, randomized controlled Phase 2/3 trial with crossover design



Standard of care may consist of traditional agents (e.g., prednisone, azathioprine, mycophenolate, methotrexate, chronic IVIg/PLEX) or complement pathway inhibitors (e.g., eculizumab, ravulizumab). Anti-CD20 or -CD19 monoclonal antibodies or FcRn inhibitors not allowed as defined in inclusion criteria.

Miv-cel Demonstrated Rapid and Robust Reductions in MG-ADL and QMG Sustained Out to 52 Weeks in Phase 2 Trial



N=7. Data cutoff: February 25, 2026.
BL, baseline; MG-ADL, myasthenia gravis activities of daily living; QMG, quantitative myasthenia gravis.

Single-Dose of Miv-cel Led to Clinically Meaningful Reductions in MG Outcome Scores and Treatment Burden



Substantially improved clinical outcomes

MG-ADL

100% had clinically meaningful response
(≥ 2 -point reduction vs baseline)

100% were responders
(≥ 3 -point reduction vs baseline)

57% reached MSE at last follow-up
(MG-ADL score of 0-1)

QMG

100% had clinically meaningful response
(≥ 3 -point reduction vs baseline)

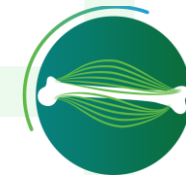
MGC

100% had clinically meaningful response
(≥ 3 -point reduction vs baseline)
-16.0 mean reduction at 24 weeks

Substantially reduced MG treatment burden

**100% free of immunotherapies, including NSISTs, high-dose steroids (>10 mg),
and FcRn and complement inhibitors up to 24 weeks**
6 of 7 patients remained free of these agents at last follow-up

Miv-cel Demonstrated a Well-Tolerated Safety Profile



Treatment-related AEs, n (%)	Patients (n=7)
CRS (any grade)	7 (100)
Grade 1	5 (71)
Grade 2	2 (29)
ICANS (any grade)	0 (0)
Grade 3/4 events	3 (43)
Neutropenia	2 (29)
Lymphopenia	1 (14)
Lymphocyte count decreased	1 (14)
SAE (any grade) ^a	0 (0)

- No high-grade CRS and no ICANS observed
- CRS was low-grade and manageable in all patients
 - 5 of 7 patients only experienced fever (grade 1 CRS)
- 2 patients with Grade 3/4 treatment-related AEs of neutropenia, an expected AE with lymphodepletion and CAR T-cell therapies; neither was associated with infections; all events were manageable and fully resolved

N=7. Data cutoff: February 25, 2026.

^aAfter the prior data cut (October 3, 2025), a previously reported treatment-related SAE was reclassified by the Investigator as 'not serious', reflecting his clinical assessment of the AE per protocol-defined seriousness criteria.

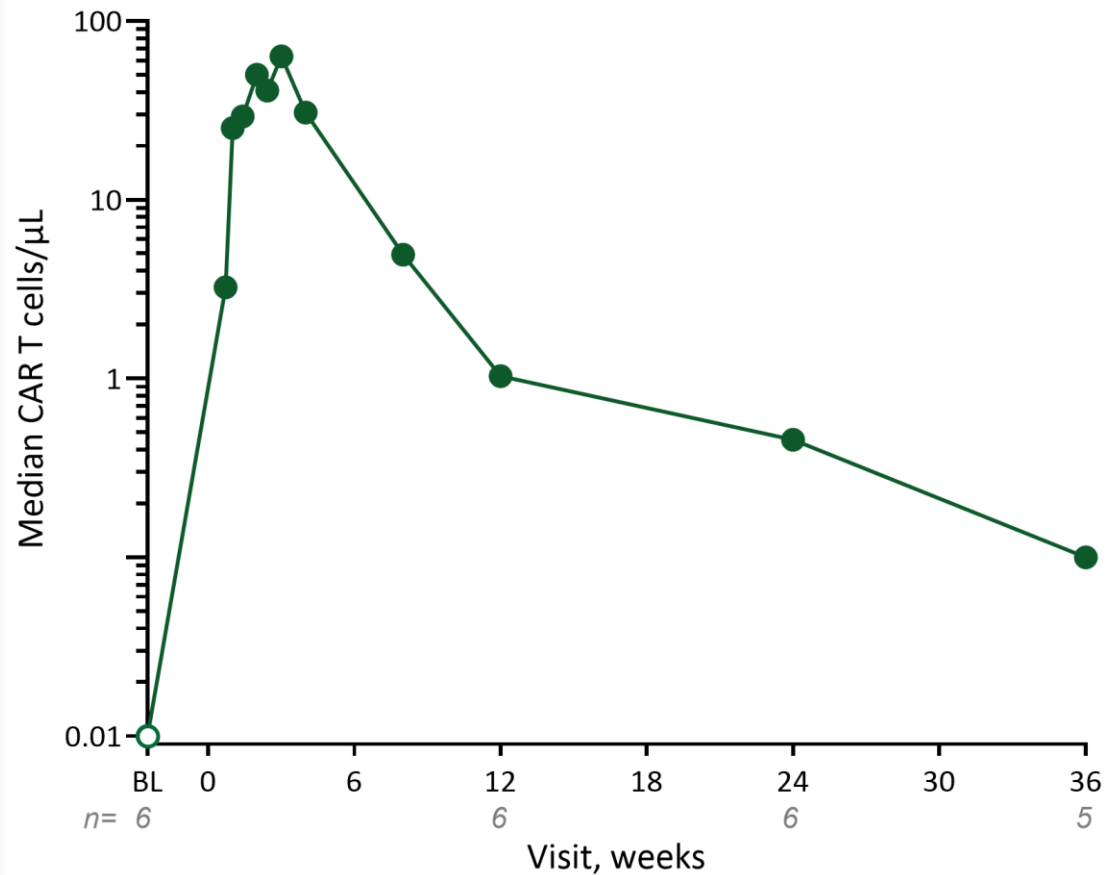
CRS and ICANS graded using ASTCT criteria; other AEs graded using CTCAE criteria.

AE, adverse event; ASTCT, American Society for Transplantation and Cellular Therapy; CTCAE, Common Terminology Criteria for Adverse Events; CRS, cytokine release syndrome; ICANS, immune effector cell-associated neurotoxicity syndrome; SAE, serious adverse event.

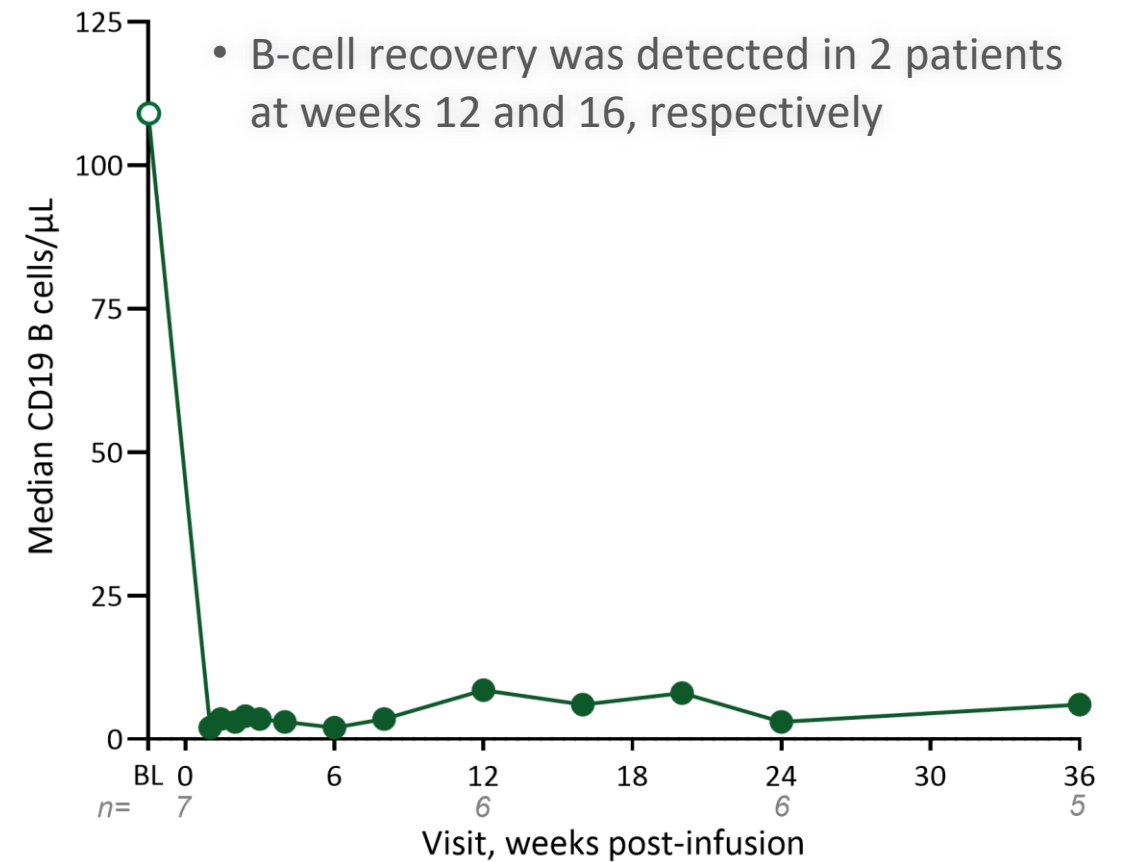
Robust CAR T-cell Expansion Led to Deep B-cell Depletion



Robust CAR T-cell expansion



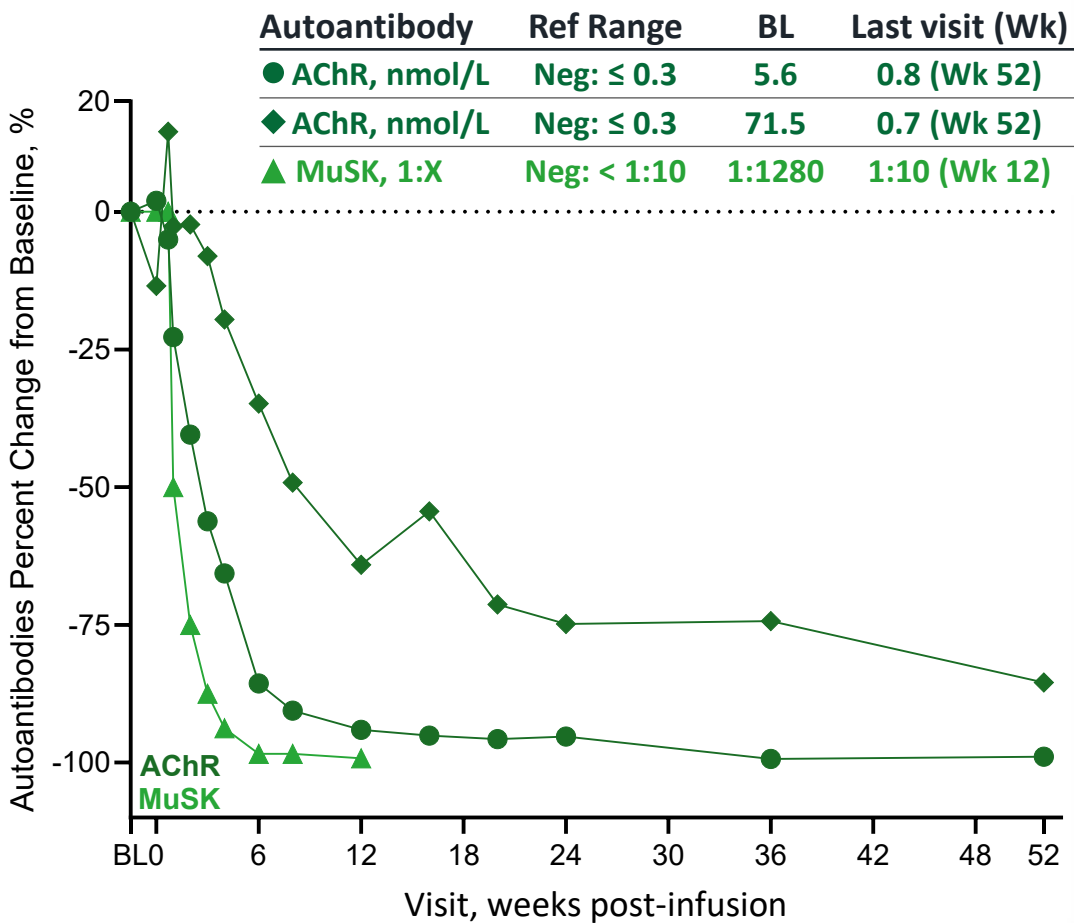
Deep B-cell depletion



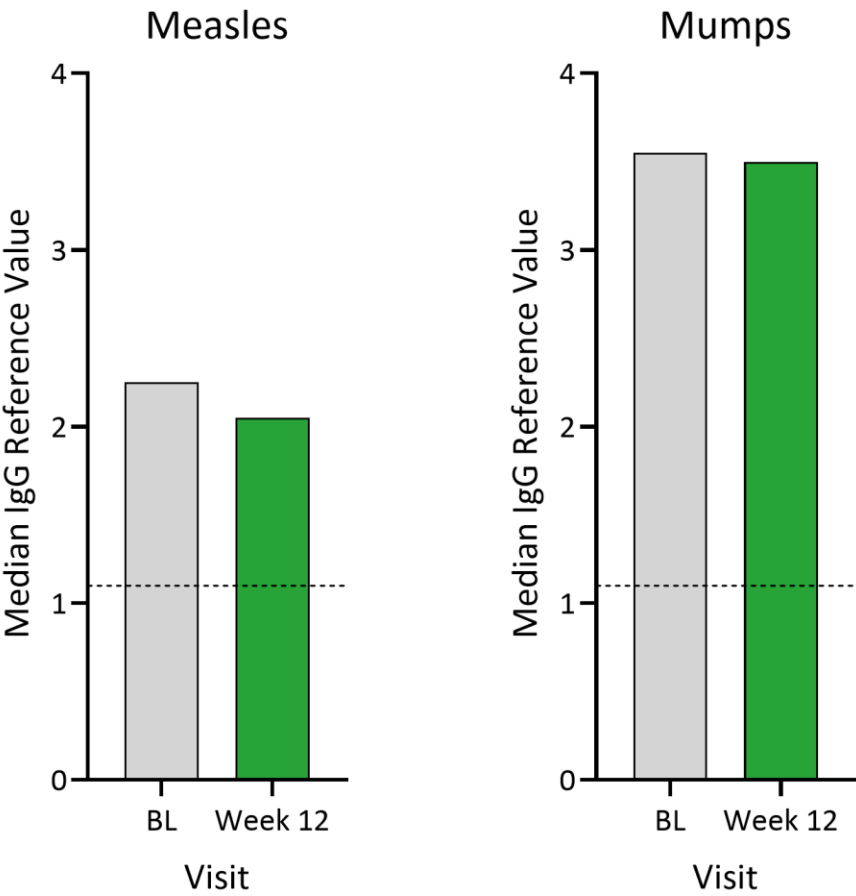
Miv-cel Reduced Autoantibody Levels While Preserving Humoral Immune Responses



Reduction of autoantibodies



Preservation of humoral immunity



Data cutoff: February 25, 2026. Humoral immunity data was not available for patient 7; dashed line on humoral immunity graph shows the threshold above which indicates prior exposure or vaccination. AChR, acetylcholine receptor; BL, baseline; IgG, immunoglobulin G; MuSK, muscle-specific kinase; neg, negative value; Ref, reference; Wk, week.

Single Dose Miv-cel Achieved Unprecedented gMG Clinical Outcomes



Note: These observations are derived from separate clinical settings; comparisons across trials are not based on head-to-head studies.

Note: These observations are derived from separate clinical settings; comparisons across trials are not based on head-to-head studies.		Approved			Investigational	
		FcRn Inhibitor ¹ VYVGART®	Complement Inhibitor ^{2,3} ULTOMIRIS®	CD19 mAb ⁴ UPLIZNA®	BCMA mRNA CAR T ⁵ Descartes-08	Miv-cel CD19 CAR T (KYSA-6, n=6)
Primary Endpoint		4 weeks	6 months	6 months	3 months	6 months
Depth of Response <i>Mean reduction from baseline to primary endpoint (non-placebo adjusted)</i>	MG-ADL Reduction	~4.6	3.1	4.2	4.1	8.5
	QMG Reduction	~6.2	2.8	4.8	3.9	11.3
% Responders <i>Patients with ≥3-point MG-ADL improvement from baseline to primary endpoint (non-placebo adjusted)</i>		~73%	~57%	69%	64%	100%
Achieve MSE <i>% of patients achieving MG-ADL of 0 or 1</i>		40% <i>At any point before primary endpoint</i>	43%	Not reported	33% <i>6 months to 1 year</i>	57% <i>At any point before primary endpoint</i>
Free from chronic background immunotherapies		✗	✗	✗	✗	✓

BCMA, b-cell maturation antigen; FcRn, neonatal fragment crystallizable receptor; mAb, monoclonal antibody; MG-ADL, myasthenia gravis activities of daily living; mRNA, messenger RNA; MSE, minimal symptom expression; QMG, quantitative myasthenia gravis score.
1. Howard Jr JF, et al. *Lancet Neurol.* 2021;20(7):526-536. 2. Vu T, et al. *NEJM Evid.* 2022;1(5):EVIDoa2100066. 3. AstraZeneca. ULTOMIRIS® efficacy data from CHAMPION-MG. <https://ultomirishcp.com/gmg/efficacy>. Accessed 20 Aug 2025. 4. Nowak RJ, et al. *N Engl J Med.* 2025;392(23):2309-2320. 5. Vu T, et al. *Nat Med.* 2026;32:1131-1141.



Progressive Multiple Sclerosis (PMS)

Progressive Multiple Sclerosis (PMS): Encouraging IIT Data Highlight Broad Opportunity with Miv-cel in Neuroimmunology



Phase 1 IIT Studies at Stanford (N=6) and UCSF (N=2)

Stanford uses alternative bendamustine lymphodepletion regimen

Biological Activity

- **Robust CAR T cell expansion** in blood and penetration into CNS
- Reconstitution of naïve B cells supportive of **immune reset**

Efficacy

- All patients with available data **showed improved or stable EDSS**
- All patients remained **off other immunotherapies**
- **Improvement in fatigue scores** from baseline in all patients with available data

Safety

- Reinforces **established tolerability profile**
- **No high-grade CRS/ICANS**

RMAT Designation Granted for naSPMS

Broader PMS Development Strategy Expected to be Shared by early 2027

Data from 8 patients treated at dose levels 33M (n=3) and 100M (n=5) CAR+ T cells

EDSS, expanded disability status scale scores; UCSF, University of California, San Francisco, Weill Institute for Neurosciences; CNS, central nervous system; CRS, cytokine release syndrome; ICANS, immune effector cell associated neurotoxicity syndrome; naSPMS, non-active secondary progressive multiple sclerosis.

Dunn J, et al. Poster Presentation at ACTRIMS 2026. ID# P112. Dunn, J, et al. Presentation at Stanford Blood and Marrow Transplantation and Cellular Therapy Symposium May 2026. Galleta K, et al. Poster Presentation at ACTRIMS 2026. ID#121 O027.

Pioneering Differentiated Therapies with Curative Potential to Free People from Life-long Autoimmune Diseases and Chronic Therapy

Miv-cel BLA Submission Underway for SPS

First autoimmune CAR T BLA; potential first approved treatment for SPS

Best-in-Class Profile

Miv-cel has demonstrated durable drug-free, disease-free remission with single-dose

Valuable Commercial Opportunity in SPS

Commercial readiness underway; immediately addressable patients; premium pricing potential

Neurologic Autoimmune Strategy

Focused franchise strategy prioritizing SPS, MG, and PMS

Innovation to Expand Access

Potential for new indications, and delivery mechanisms, including no or alternative preconditioning

Capitalized to Support Initial Miv-cel Launch

Operating runway into 2028 supporting SPS launch and gMG Phase 3 trial