



# Highlighting New Data from Our Neuroimmunology Franchise at AAN

April 22, 2026

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# Today's Agenda

- Solidifying our Leadership in Autoimmune CAR T
- Stiff Person Syndrome (SPS) – Primary Analysis Review
- Generalized Myasthenia Gravis (gMG) – Phase 2 Data Update
- Advancing Valuable Market Opportunity in SPS
- Q&A

## Speakers



**Warner Biddle**  
Chief Executive Officer



**Naji Gehchan, M.D., MSc, MBA**  
Chief Medical & Development Officer



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University of Colorado Anschutz  
Céline Dion Foundation Endowed Chair



**Sri Muppidi M.D.**  
Stanford Medicine

# Positioned to Deliver the First Approved Autoimmune CAR T, Miv-cel



**Lead indications, SPS and gMG, addressing significant unmet medical need**



**Transformative clinical results** reinforce potential to change the treatment paradigm by delivering **drug-free, disease-free remission with a single dose**



**BLA submission preparations underway for SPS, a valuable commercial opportunity**



**Phase 3 gMG trial** paves way to significant market opportunity underpinned by miv-cel's **differentiated clinical profile**

# New Data Reinforce Kyverna's Differentiated Neuroimmunology Franchise Opportunity

## SPS Registrational Primary Analysis

*Further supports path to approval & confidence in launch*

- ✓ **Statistically significant, durable clinical benefit across all endpoints, with reversal of disability scores**
- ✓ **New secondary endpoint results and translational data demonstrate full spectrum of miv-cel clinical benefit**

## gMG Phase 2 Longer-Term Follow-Up

*Further supports confidence in Phase 3 Trial*

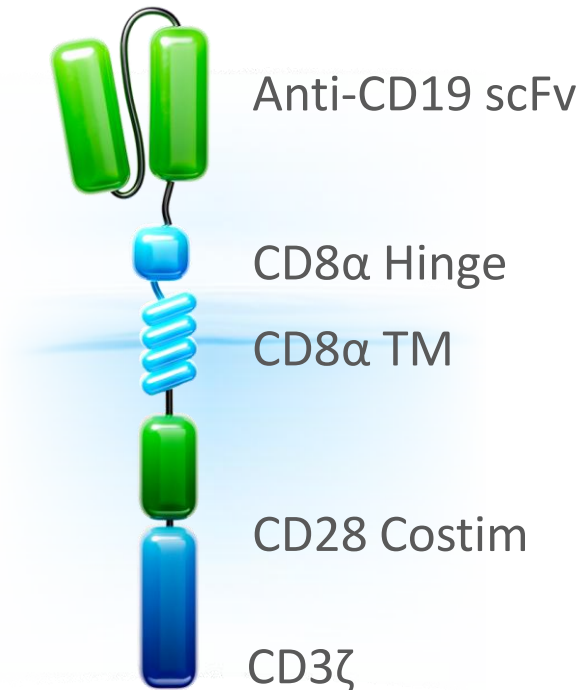
- ✓ **Even deeper responses as data mature, with durability out to 52 weeks**
- ✓ **100% of patients achieved clinically meaningful response across MG-ADL, QMG, and MGC**
- ✓ **Majority of patients achieved MSE**

**100% free of immunotherapies and a well-tolerated safety profile**

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

## Mivocabtagene Autoleucel (miv-cel)<sup>1,2</sup>

Fully Human Autologous CD19 CAR T  
With CD28 Costim



- **More than 100** patients dosed with miv-cel across multiple indications<sup>3</sup>
- **Deep and broad depletion of peripheral- and tissue-resident B cells to support broad immune reset and durable remission<sup>4,5</sup>**
- **No high-grade CRS or ICANS<sup>3</sup>**
- First SPS and gMG patients treated with a single dose of miv-cel achieved **durable efficacy beyond 24 months without the need for chronic immunotherapies<sup>6</sup>**

# Patient Perspective from KYSA-8: Before and After Treatment with Miv-cel

[View Video](#)





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# SPS – Primary Analysis Results from KYSA-8 Registrational Trial

Naji Gehchan, M.D., MSc, MBA – Chief Medical and Development Officer

Amanda Piquet, M.D., FAAN – University of Colorado Anschutz, Céline Dion Foundation Endowed Chair

# 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<sup>1,2</sup>



Symptoms characterized by **muscle stiffness** and **painful muscle spasms**, impacting mobility<sup>1-3</sup>



**Inadequate response** with off-label symptomatic and immunomodulatory therapies<sup>1,2,5</sup>

## Devastating Impact on Patients

**80% of patients lose mobility**, needing walking aid assistance or wheelchair<sup>1-3</sup>

**Only ~19% of patients remained able to work** after 4 years<sup>4</sup>

**“Freezing attacks” and sudden falls** requiring ER care<sup>1,2</sup>

Risk of **permanent disability** and **increased mortality**<sup>3</sup>

# SPS Natural History Study Reinforces Significant Unmet Medical Need

**Large, multicenter, retrospective study assessing T25FW in patients with SPS (n=153)**

## Key Takeaways


- Majority of patients had **no or limited (<20%) improvement in T25FW**
- **Disability (mRS) did not improve** over time
- **Walking aid use increased** over time
- **All patients treated** with off-label immunomodulators or symptomatic medication

**Study Contextualizes Transformative Miv-cel Data and Supports the T25FW as a Valid Longitudinal Measure of Mobility in SPS**

# Registrational Trial Designed to Support Path to BLA

## *Received Both ODD and RMAT Designations*

### 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  $\geq 2$

**Miv-cel**  
Low-Dose Cy/Flu  
lymphodepletion  
+  
Single infusion of  
 $1 \times 10^8$  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**

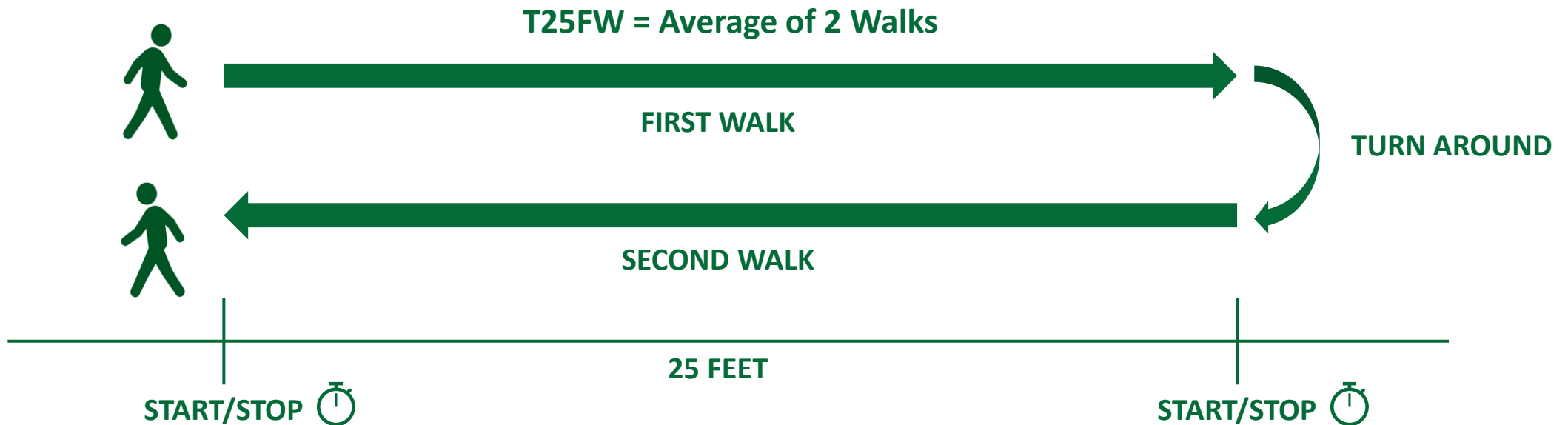
**Rapid Clinical Enrollment Underscores Significant Unmet Need and Kyverna's Ability to Execute**

# Primary Endpoint Outcome Assesses Impact of SPS on Walking Ability

## Timed 25-Foot Walk (T25FW)

- Validated tool to assess walking ability<sup>1</sup>
- Used to evaluate stiffness and loss of mobility in SPS<sup>2</sup>
- Healthy adults can perform the T25FW in ~4-5 seconds<sup>3</sup>

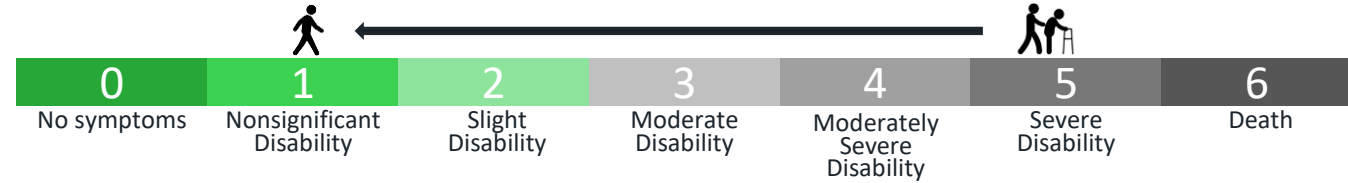
20% improvement  
considered clinically  
meaningful<sup>1</sup>



# Secondary Endpoint Outcomes Assess Extent of Disability and SPS-Specific Symptoms

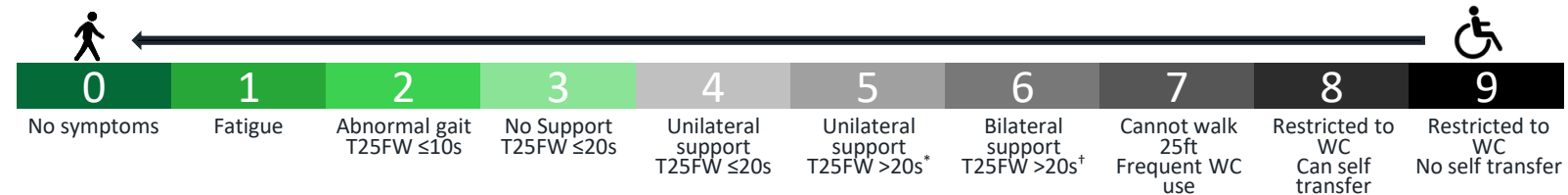
## Modified Rankin Scale (mRS)

Degree of disability<sup>1</sup>



## Hauser Ambulation Index (HAI)

Time and degree of assistance to complete T25FW<sup>2</sup>



## Distribution-of-Stiffness Index (DSI)

Muscle stiffness across body regions<sup>3,4</sup>

1 point for each stiff body region (0-6)



## Heightened Sensitivity Scale (HSS)

Number of triggers of muscle spasms<sup>3,4</sup>

1 point for each trigger/stimulus (1-7)



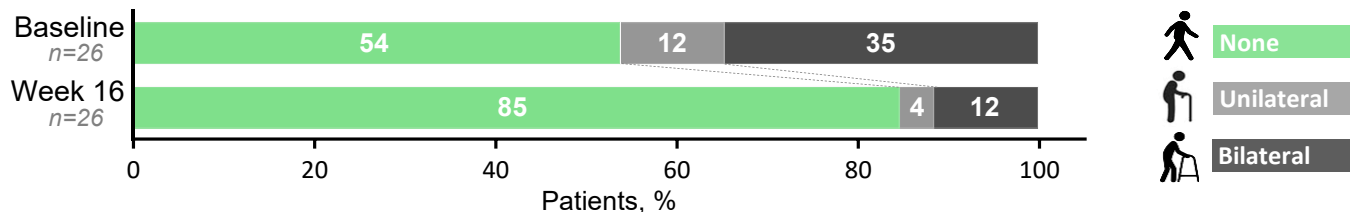
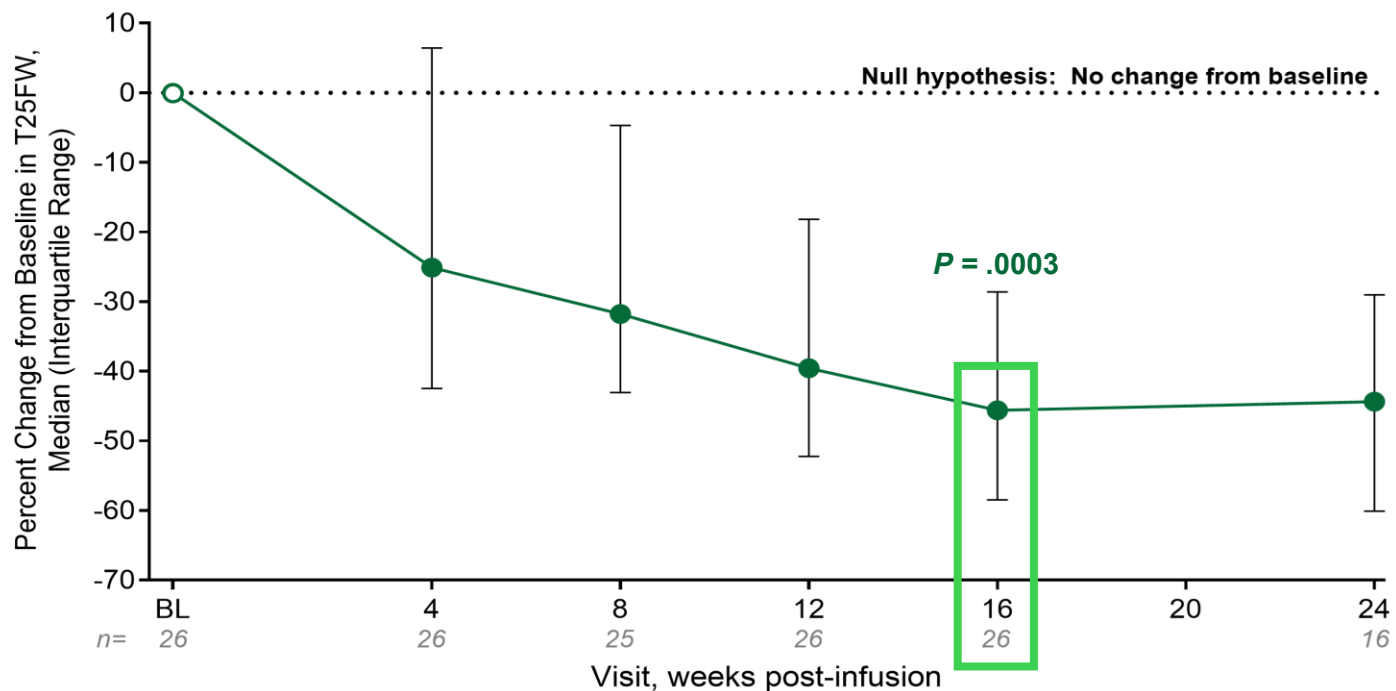
T25FW, timed 25-foot walk; WC, wheelchair.

1. van Swieten JC, et al. *Stroke*. 1988; 19(5): 604-607. 2. Hauser SL, et al. *New Engl J Med*. 1983; 308(4): 173-180. 3. Dalakas MC, et al. *N Engl J Med*. 2001; 345(26): 1870-1876. 4. Dalakas MC, et al. *Ann Neurol*. 2017; 82(2): 271-277.

# Primary Endpoint Met: Significant Improvement in T25FW

## 46% Median Improvement at Week 16

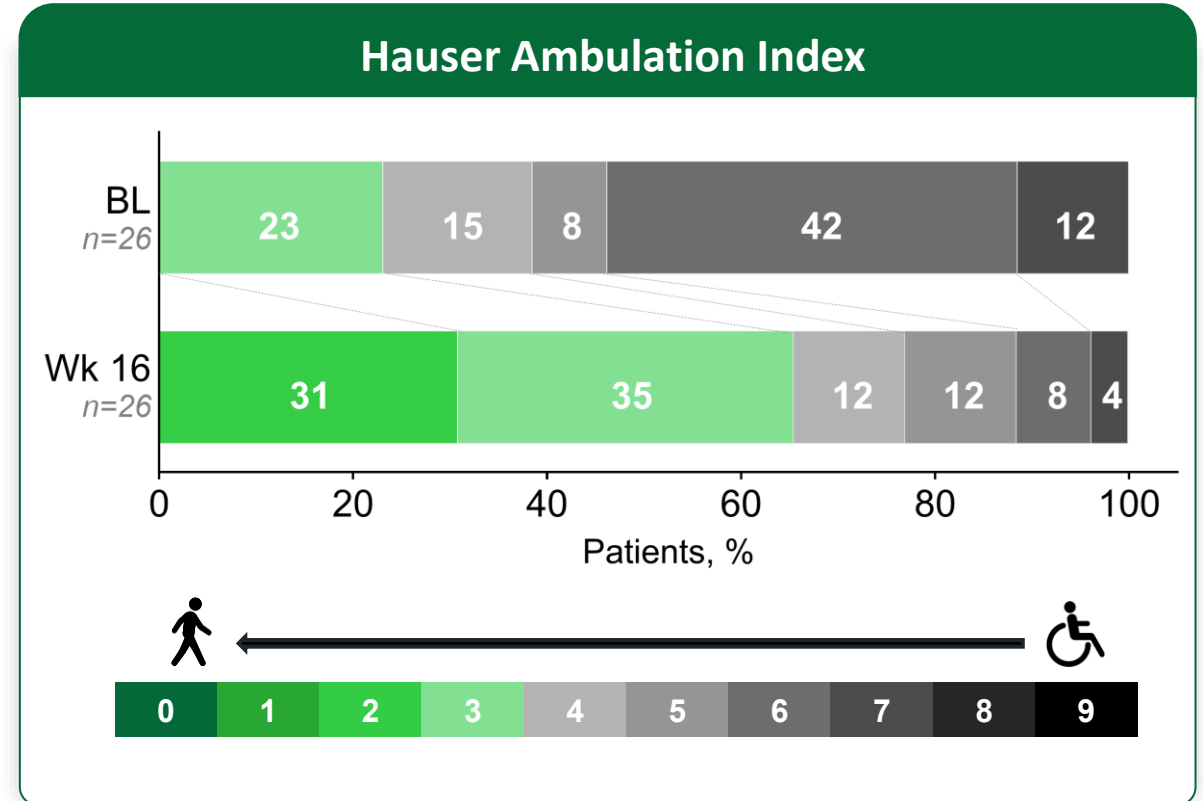
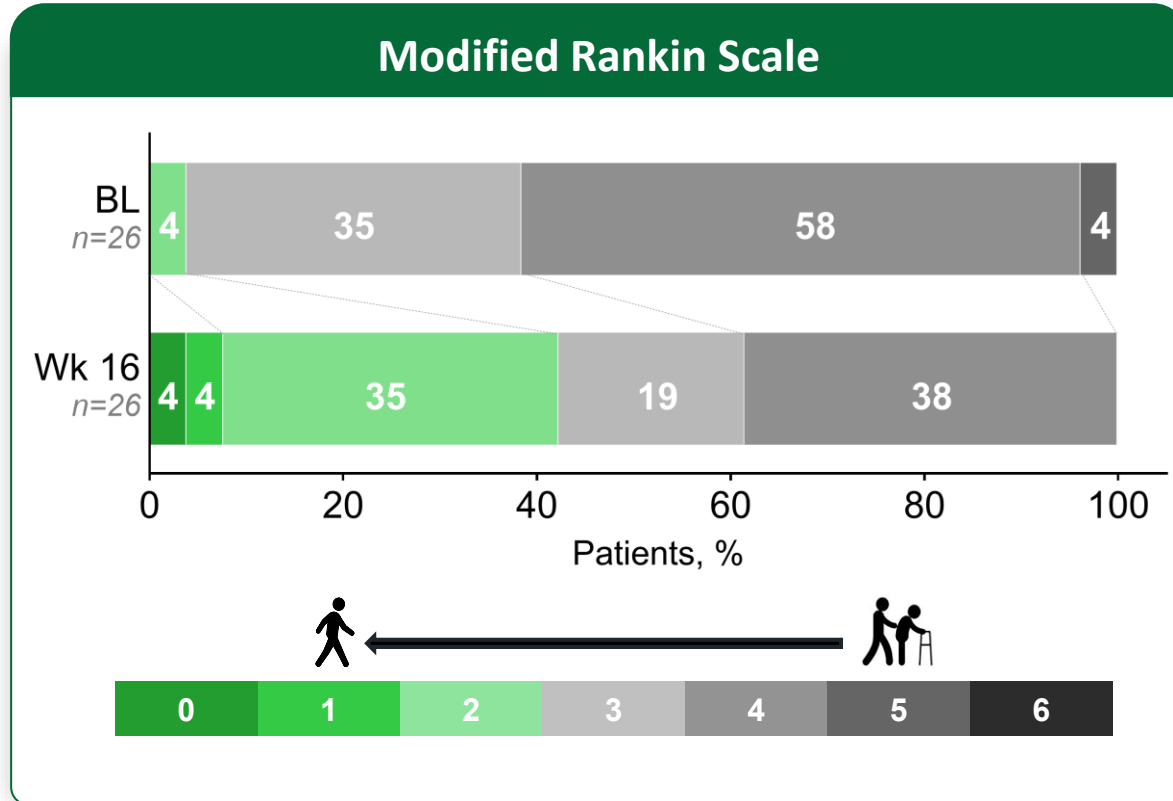
### Significant T25FW Improvement and Reduced Walking Aid Use



- 81% of patients achieved **clinically meaningful improvement** ( $\geq 20\%$  reduction from baseline)<sup>1</sup>
- 31% completed T25FW in  $< 5$  seconds; **typical time for healthy adults**<sup>2</sup>
- 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 ( $\geq 20$  mg/day) for SPS symptoms.  
 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.

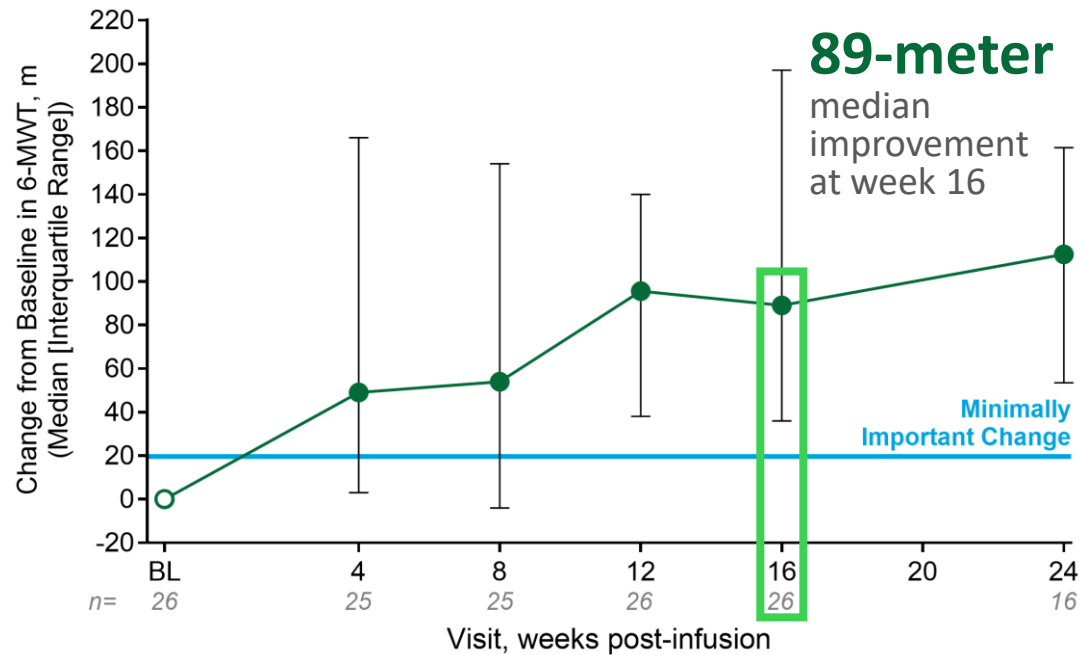
# Secondary Endpoints Met: Miv-cel Achieved Significant ( $P < .0001$ ) Improvements in Disability, Mobility, Stiffness, and Hypersensitivity



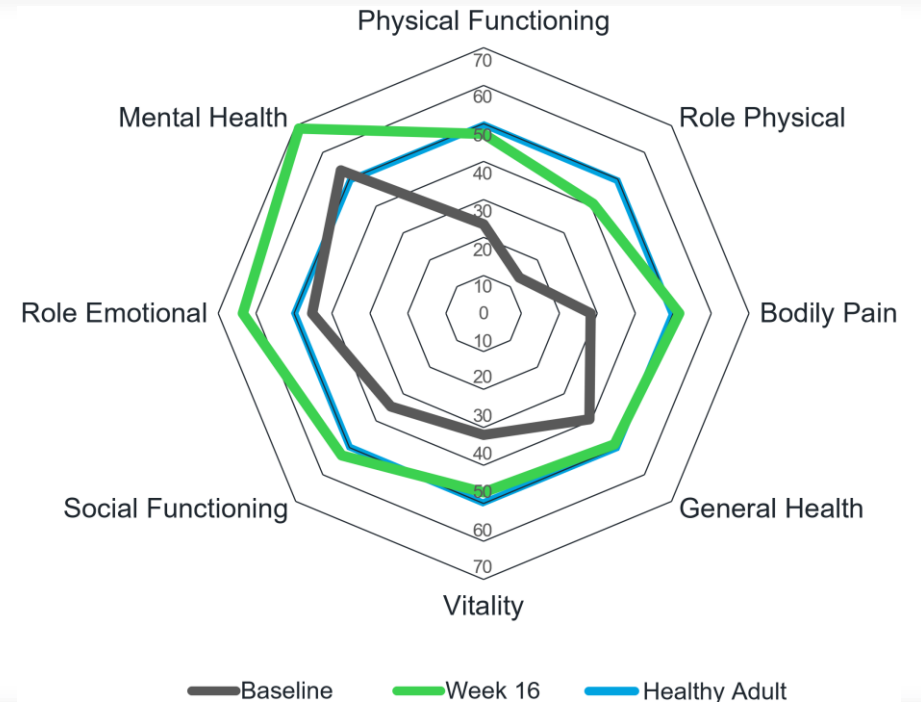
- Significant ( $P < .0001$ ) mean improvements in mRS and HAI of -0.8 (SD, 0.86) and -1.6 (1.13) and SPS-specific measures, DSI and HSS, of -1.5 (1.75) and -3.2 (2.01), respectively
- 96% of patients (25/26) had improvement in  $\geq 1$  primary or secondary efficacy endpoint

# Additional Efficacy Measures: Substantial Improvements in Physical and Mental Functioning

## 6-Minute Walk Test (MWT): >4-fold improvement over clinical minimally important change<sup>1</sup>

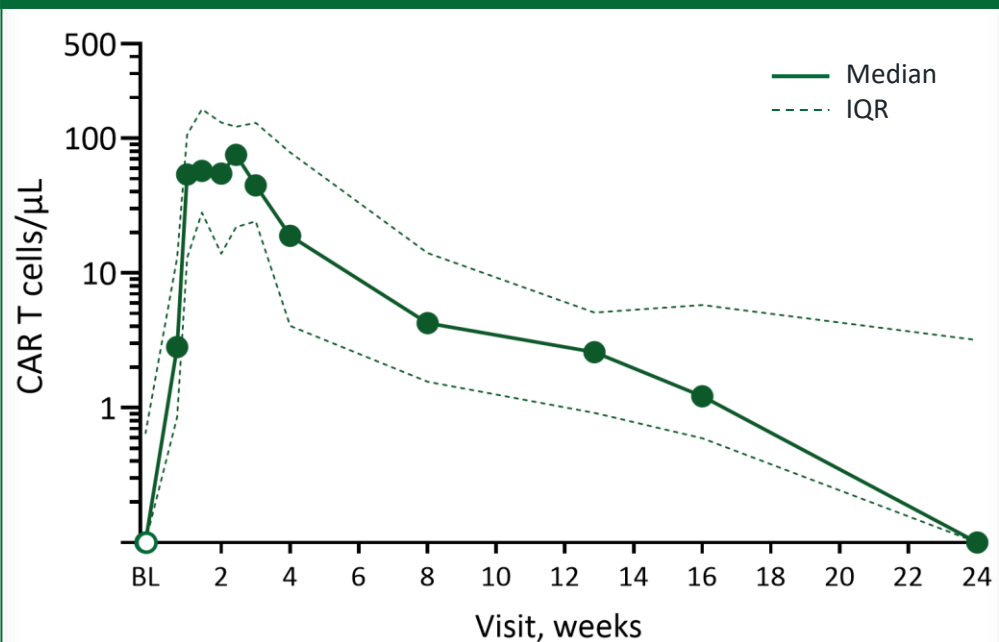


## 36-Item Short Form Health Survey (SF-36): Week 16 scores comparable to healthy adults for most domains<sup>2,3</sup>



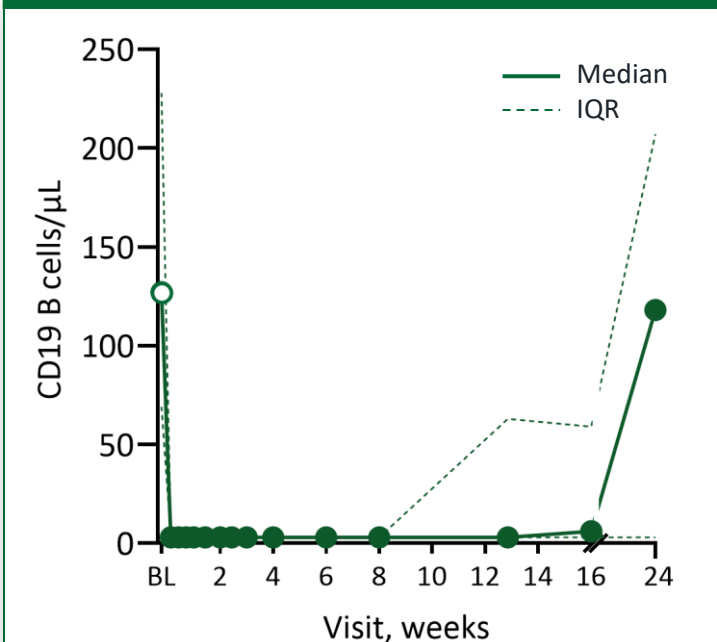
# Robust Miv-cel Expansion Led to Complete Peripheral B-cell Depletion and Significant Reductions in Autoantibody Titers

## Robust CAR T-cell Expansion



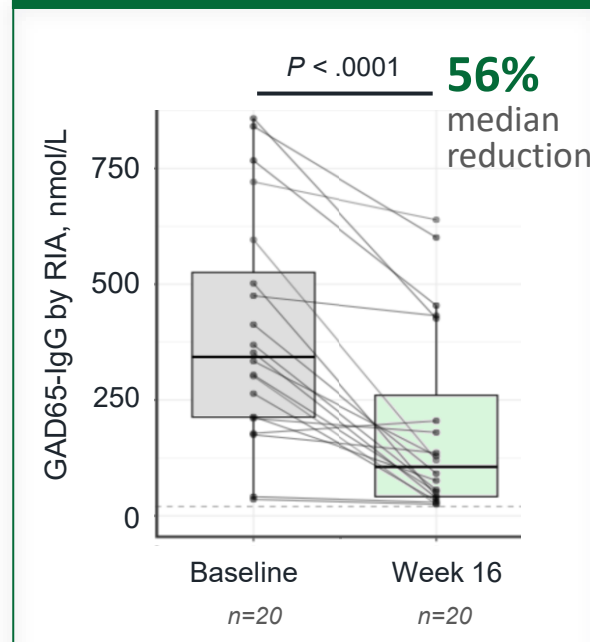
- CAR-positive T cells peaked by day 14

## Deep B-cell Depletion



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

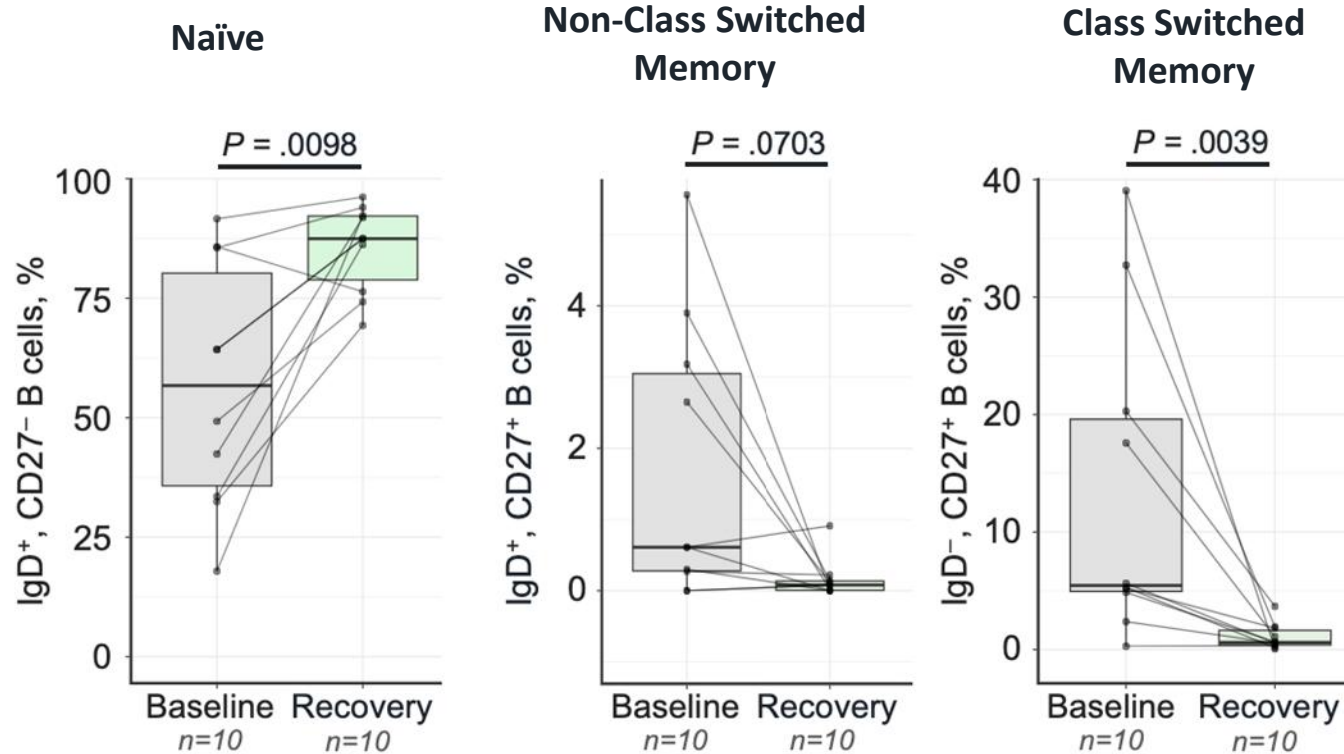
## Reduced GAD65-IgG



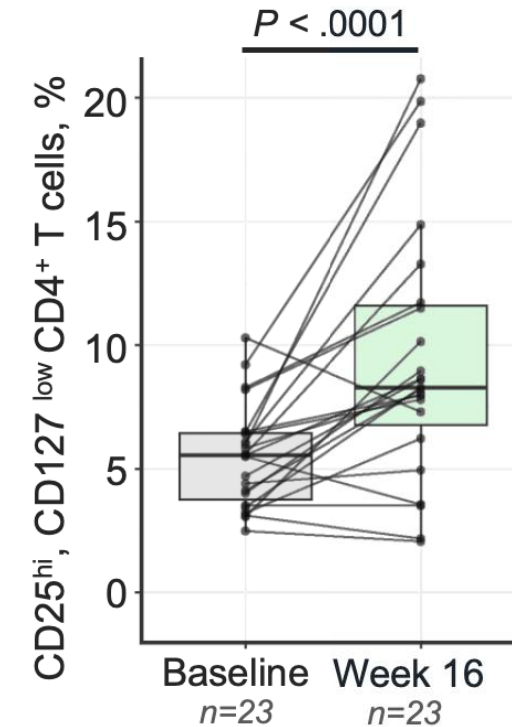
- GAD65-IgG was reduced in 19/20 patients with  $\geq 20$  nM GAD65 at baseline

# Miv-cel Treatment 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
- Most common treatment-related AEs were CRS (92%), fatigue (54%), diarrhea (38%), and headache (31%)
- 4 patients had Grade 3/4 neutropenia, an expected AE with lymphodepletion and CAR T-cell therapies
  - All events were manageable with treatment including G-CSF, fully resolved in 3 patients (median duration of 85 days), and was ongoing in 1 patient
  - No serious infections associated with neutropenia
- Treatment-related serious AEs occurred in 3 patients; all fully resolved without sequelae

# Potential to Achieve Durable, Drug-Free, Disease-Free Remission and Reverse Disability

**In KYSA-8 trial, a single dose of miv-cel resulted in:**



Significant, robust, rapid improvements in mobility, disability, stiffness, and hypersensitivity



100% free of immunomodulatory or immunosuppressive therapies for SPS as of last follow-up



A consistent, well-tolerated, and manageable safety profile, with the potential for outpatient administration



Sustained clinical benefit following deep B-cell depletion and broad immune reset



# gMG – Updated Data from Phase 2 KYSA-6 Trial

Naji Gehchan, M.D., MSc, MBA – Chief Medical and Development Officer

Sri Muppidi, M.D. – Stanford University

# Patient Perspective from IH Pathway: Before and After Treatment with Miv-cel

[View Video](#)



# Despite Available Treatment Options, High Disease Burden Remains in Generalized Myasthenia Gravis

- gMG is a B-cell and antibody-mediated neuromuscular autoimmune disease that causes fluctuating muscle weakness and fatigue<sup>1,2</sup>

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

## Current State of Treatment for Patients With gMG



Inadequate symptom control<sup>3,4</sup>



Few reach minimal symptom expression (MSE)<sup>1,5-6</sup>




Majority require ongoing immunosuppressant therapy<sup>1-4</sup>



Costly and chronic treatment options<sup>1,7</sup>

# KYSA-6: Phase 2/3 Study of Miv-cel in gMG

## Phase 2 design: Open-label, single-arm, multicenter study

 **N = 7**

- Age 18 to 75 years
- Diagnosis of gMG, Class IIB-IV per MGFA criteria
- Autoantibodies to AChR, MuSK, or LRP4
- MG-ADL  $\geq 6$
- Failed  $\geq 2$  immunosuppressive/immunomodulatory therapies OR failed  $\geq 1$  immunosuppressive therapy and required chronic plasmapheresis or IVIg to control symptoms

### Miv-cel

Low-Dose Cy/Flu lymphodepletion  
+  
Single infusion of  $1 \times 10^8$  CAR T cells

**MG immunotherapies are discontinued**

### Primary endpoints

- MG-ADL at 24 weeks
- Adverse events

### Key secondary endpoints

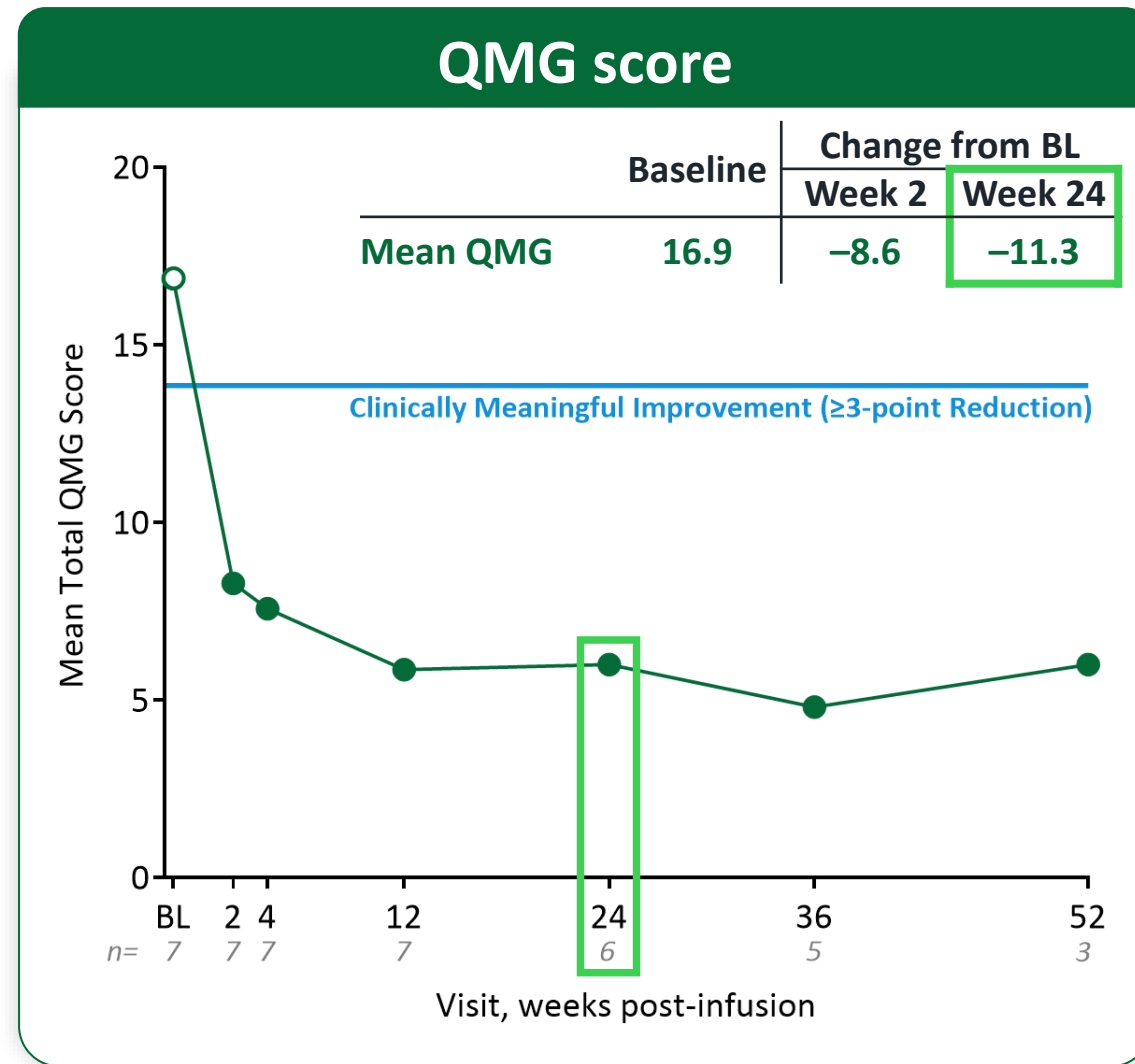
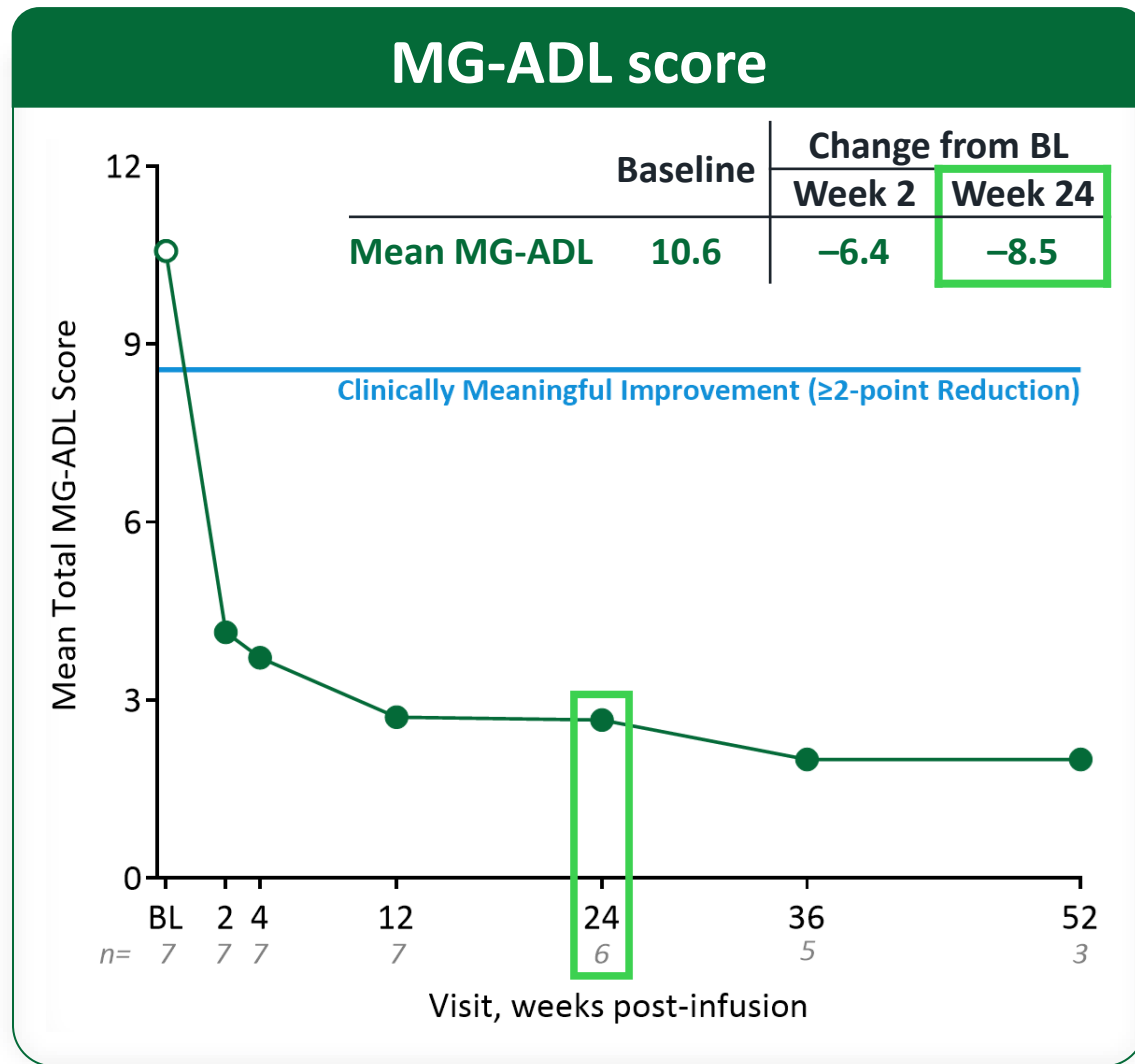
- QMG and MGC scores
- PK/PD



**18-month follow up**

**Longer-Term Follow-Up Data Cut Off as of February 25, 2026**

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



# After a Single Dose of Miv-cel, Patients Achieved Substantial and 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) <sup>a</sup>	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
  - 1 patient had transient Grade 4 neutropenia that resolved within 10 days post-infusion
  - 1 patient had Grade 4 neutropenia was manageable with G-CSF, and resolved within 2 months post-infusion

Data cutoff: February 25, 2026.

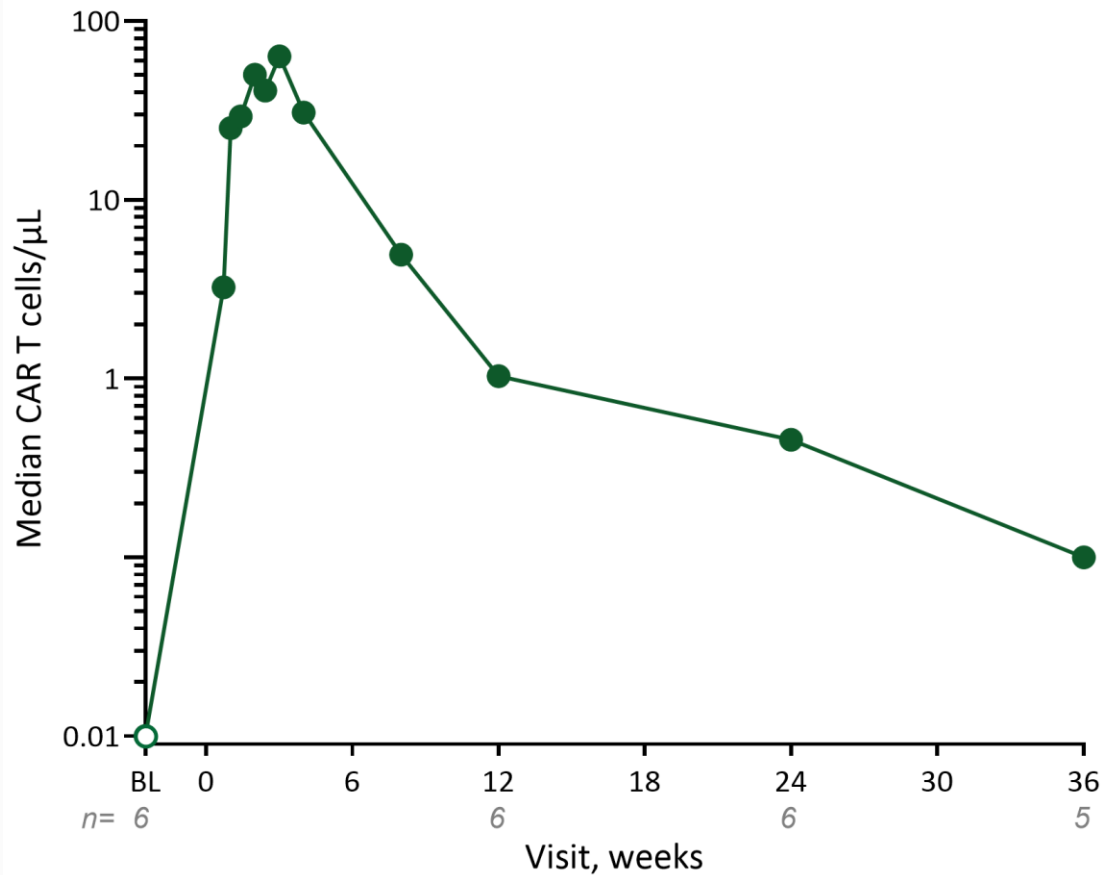
<sup>a</sup>After the prior data cut (October 3, 2025), a previously reported treatment-related SAE was reclassified by the Investigator as 'not serious', reflecting his clinical assessment of the AE per protocol-defined seriousness criteria.

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

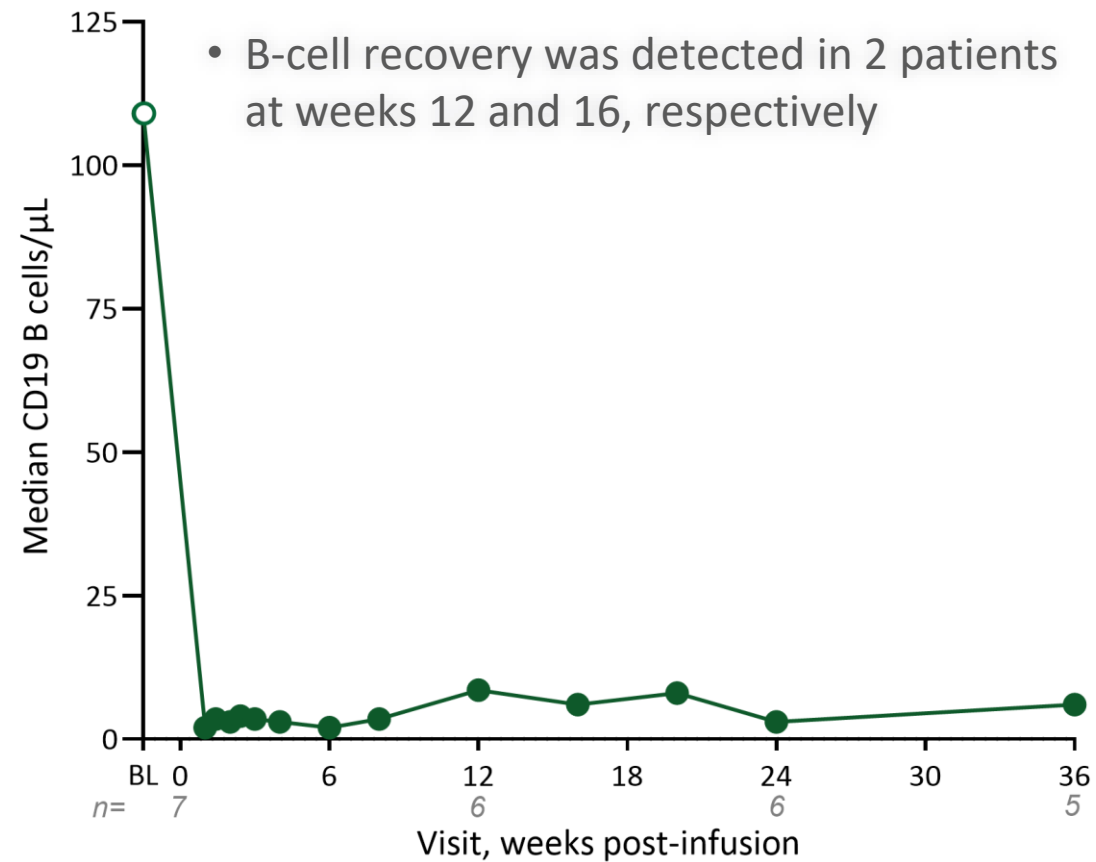
AE, adverse event; ASTCT, American Society for Transplantation and Cellular Therapy; CAR, chimeric antigen receptor; CTCAE, Common Terminology Criteria for Adverse Events; CRS, cytokine release syndrome; DLT, dose-limiting toxicity; G-CSF, granulocyte colony-stimulating factor; ICANS, immune effector cell-associated neurotoxicity syndrome; LD, lymphodepletion; SAE, serious adverse event; TEAE, treatment-emergent adverse event.

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

## Robust CAR T-cell expansion

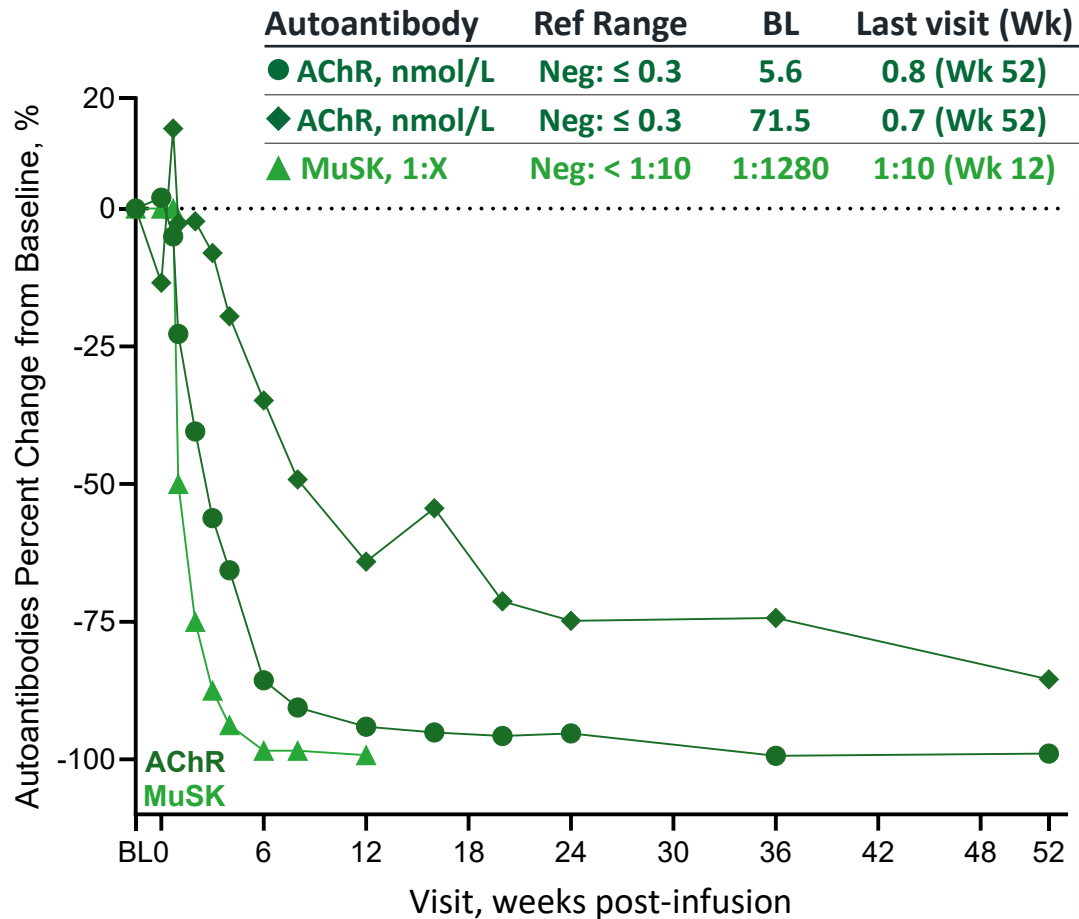


## Deep B-cell depletion

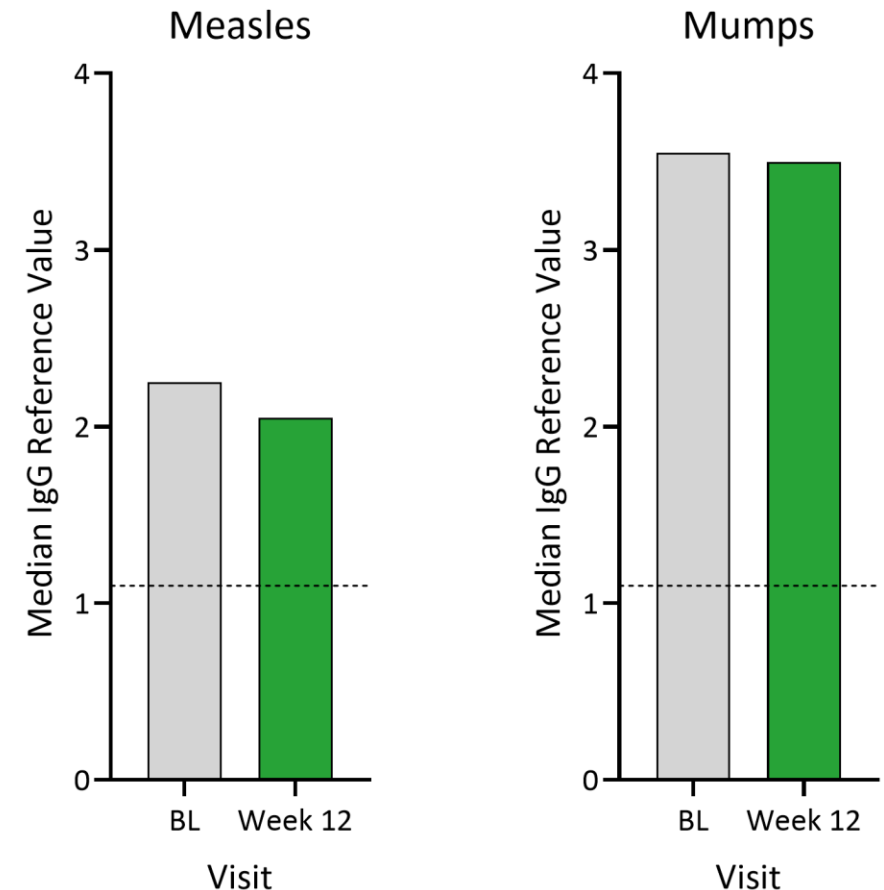


# Miv-cel Reduced Autoantibody Levels While Preserving Humoral Immune Responses

## Reduction of autoantibodies



## Preservation of humoral immunity



# Potential to Change the gMG Treatment Paradigm by Delivering Durable, Drug-Free, Disease-Free Remission

**In KYSA-6 trial, a single dose of miv-cel resulted in:**



Robust, rapid, and sustained improvements regardless of prior biologic exposure



100% free of immunotherapies, including NSISTs, high-dose steroids (>10 mg), and FcRn and complement inhibitors up to 24 weeks



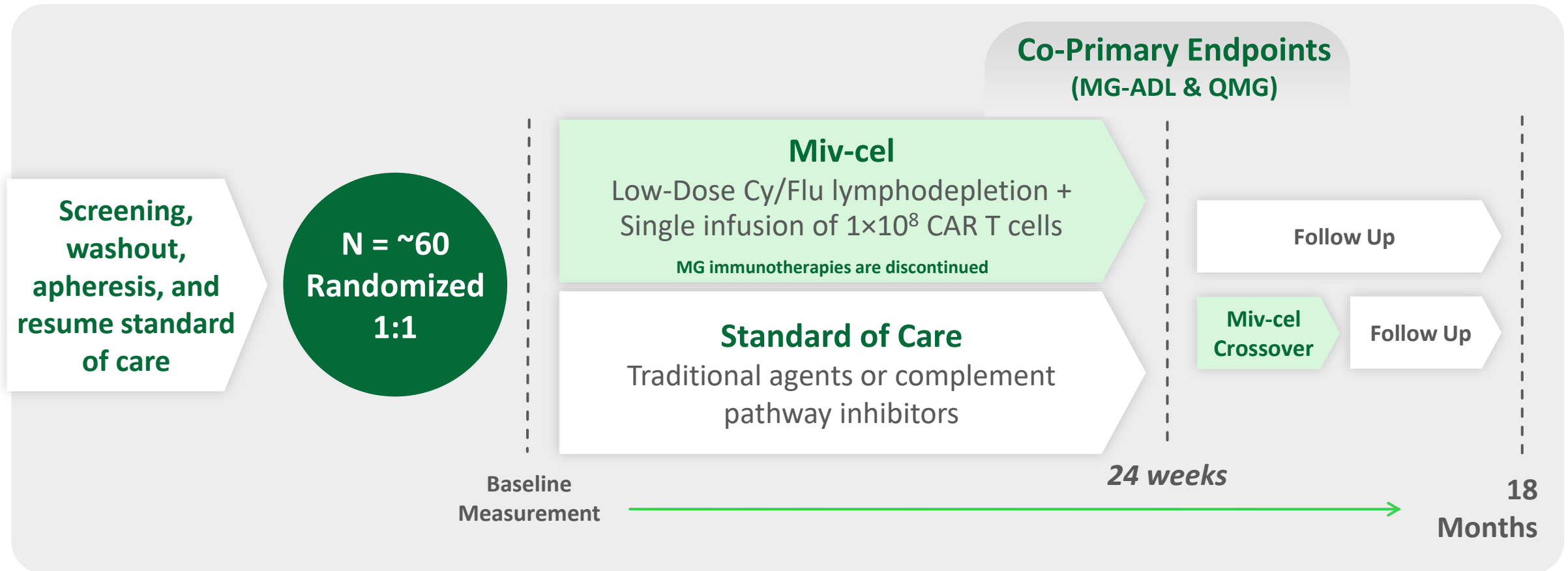
Consistent, well-tolerated, and manageable safety profile with no high-grade CRS or ICANS



Evidence of immune reset and preserved humoral immunity

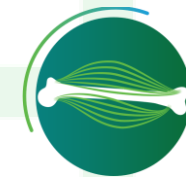
# Phase 3 Trial Advancing with First Patients Enrolled and 14 Sites Activated Globally

~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.*

# Single Dose Miv-cel Achieved Unprecedented gMG Clinical Outcomes



**All other therapies require chronic background immunotherapies**

		Approved			Investigational*	
		FcRn Inhibitor <sup>1</sup> VYVGART <sup>®</sup>	Complement Inhibitor <sup>2,3</sup> ULTOMIRIS <sup>®</sup>	CD19 mAb <sup>4</sup> UPLIZNA <sup>®</sup>	BCMA mRNA CAR T <sup>5</sup> Descartes-08	<b>Miv-cel CD19 CAR T (KYSA-6, n=6)</b>
<b>Primary Endpoint</b>		4 weeks	6 months	6 months	3 months	<b>6 months</b>
<b>Depth of Response</b> <i>Mean reduction from baseline to primary endpoint (non-placebo adjusted)</i>	<b>MG-ADL Reduction</b>	~4.6	3.1	4.2	4.1	<b>8.5</b>
	<b>QMG Reduction</b>	~6.2	2.8	4.8	3.9	<b>11.3</b>
<b>% Responders</b> <i>Patients with ≥3-point MG-ADL improvement from baseline to primary endpoint (non-placebo adjusted)</i>		~73%	~57%	69%	64%	<b>100%</b>
<b>Achieve Minimal Symptom Expression (MSE)</b> <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>	<b>57%</b> <i>At any point before primary endpoint</i>

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

BCMA, b-cell maturation antigen; FcRn, neonatal fragment crystallizable receptor; mAb, monoclonal antibody; MG-ADL, myasthenia gravis activities of daily living; mRNA, messenger RNA; QMG, quantitative myasthenia gravis score.

\*Under investigation in gMG.

1. Howard Jr JF, et al. *Lancet Neurol.* 2021;20(7):526-536. 2. Vu T, et al. *NEJM Evid.* 2022;1(5):EVID0a2100066. 3. AstraZeneca. ULTOMIRIS<sup>®</sup> 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.



# Advancing Valuable Market Opportunity in SPS

Warner Biddle – Chief Executive Officer

# Miv-cel: Potential to Be First and Only Approved Therapy in SPS with Patients Ready for an Effective Treatment Option



**~6k**

**U.S. Diagnosed  
SPS Patients<sup>1,2</sup>**



**Miv-cel  
Addressable Market<sup>2,3</sup>**

**Initial  
Priority**

**~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*

\*Immunotherapy defined as off-label immunosuppressants (e.g., prednisone), rituximab and/or IVIg, 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).

# SPS Treators Show Strong Enthusiasm for Early Adoption of Miv-cel



Survey of 20 high volume  
SPS treators 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 Targets ~10 High-Value SPS Centers



## SPS Leadership

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



## Addressable Patients

- Existing patients ready at center
- Strong referral network



## CAR T Expertise

- Commercial CAR T experience
- Accreditation



## Economic Potential

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

**Meaningful Portion of Immediately Addressable Patients at ~10 Centers**

# Kyverna: Delivering the Curative Potential of CAR T in Autoimmune Diseases



## **CAR T Leadership**

Potentially first approved autoimmune CAR T

## **Best-in-Class Profile**

Demonstrated durable drug-free, disease-free remission

## **Focused Strategy**

Neuroimmunology-led franchise

## **Valuable Commercial Opportunity in SPS**

Immediately addressable market and premium pricing potential

## **Pipeline-in-a-Product**

Clinical data supports expansion into broader indications

## **Strong Financial Position**

Supporting SPS BLA submission, anticipated commercial launch and gMG Phase 3 trial



# Q&A



**Warner Biddle**  
Chief Executive Officer



**Naji Gehchan, M.D., MSc, MBA**  
Chief Medical  
& Development Officer



**Marc Grasso, M.D.**  
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**Amanda Piquet, M.D., FAAN**  
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