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RESET Myositis™ An Open-Label Study to Evaluate the Safety and Efficacy of Rese-cel (CABA-201) in Active Idiopathic Inflammatory Myopathy or Juvenile Idiopathic Inflammatory Myopathy

Courtney Little, BSN, MS (presenting on behalf of the RESET-Myositis study team)
Cabaletta Bio, PA, USA

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Background: Myositis and CAR T Therapy

- Idiopathic inflammatory myopathies (IIMs), including juvenile IIM (JIIM), are a group of systemic autoimmune diseases characterized by inflammation in several organs and muscle weakness[1]
- JIIM is driven by autoantibodies produced by disease-causing B cells[1]
- JIIM often requires long-term use of immunomodulatory therapies and steroids.
- Current therapies do not adequately control the disease in most patients. There are no FDA-approved treatments for JIIM.
- Medicines that target B cells have been effective, but responses may be incomplete, and relapses are common, likely due to incomplete depletion and return of autoreactive B cells[2]
- Chimeric antigen receptor (CAR) T cells may have the potential to achieve long-term, drug-free clinical responses through a one-time infusion leading to depletion of tissue resident B cells.

What is rese-cel?

- Rese-cel is an investigational, chimeric antigen receptor (CAR) T cell therapy designed to treat myositis and other autoimmune diseases by targeting B cells that may be causing the disease. It is being studied in the RESET clinical trial program across several autoimmune diseases, including systemic lupus erythematosus (SLE), IIMs (myositis), and systemic sclerosis (SSc).
- Rese-cel is made from the patient's own white blood cells (Figure 1).
- Initial data from the first 18 patients with autoimmune disease (including 8 adults with myositis) treated in the RESET clinical program showed **rapid B cell depletion in blood followed by return of B cells as early as 2 months after treatment with rese-cel**[*3-5]
- After receiving rese-cel, 7 out of 8 patients with adult myositis demonstrated clinical responses off immunomodulators and steroids (or while tapering steroids).[4*]
- Rese-cel was generally well tolerated across all 8 IIM subjects treated to date..
  - Four patients with myositis did not develop cytokine release syndrome (CRS) and 4 had Grade 1 (fever); no patients developed immune effector cell–associated neurotoxicity syndrome (ICANS)[4]
- In the first 18 patients with autoimmune disease treated with rese-cel in the RESET clinical program, 94% experienced either no CRS or Grade 1 CRS (fever) and 89% of patients experienced no (ICANS)[3-5*]

*As of 6 May 2025.

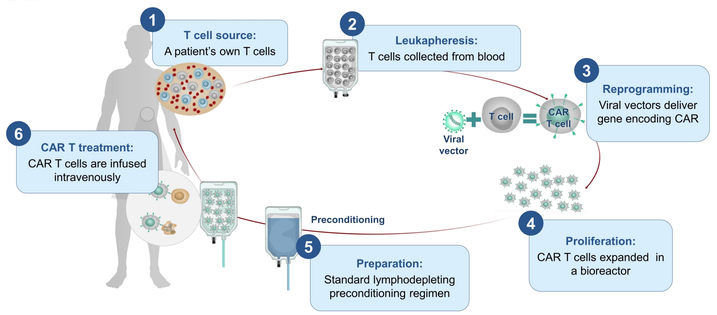

Figure 1. Rese-cel is made from the patient's own T cells and uses the patient's immune system to get rid of the disease-causing B cells and restore healthy B cells to the body. [6]

RESET-Myositis Study Design

RESET-Myositis (NCT06154252) is a Phase 1/2 trial evaluating the safety and efficacy of rese-cel in children and adults with IIM (Figure 2).

Key Inclusion Criteria (Must have all to be eligible)[7]

- A definite or probable diagnosis of IIM using 2017 EULAR/ACR classification criteria
- Evidence of active disease and muscle weakness despite prior or current treatment with standard of care
- **For adult cohorts**: Age ≥18 and ≤75 with a diagnosis of **antisynthetase syndrome, dermatomyositis, or immune-mediated necrotizing myopathy** based on presence of serum myositis-specific antibodies (MSA)
- **For juvenile myositis cohort:** Age ≥6 and ≤17 with presence of at least one MSA or myositis-associated antibody (MAA)

Key Exclusion Criteria (Must not have any to be eligible)[7]

- Cancer-associated myositis or malignancy within the last 5 years
- Significant lung or cardiac impairment
- Previous CAR T cell therapy and/or HSCT
- Treatment with B cell-depleting agent within prior ~6 months

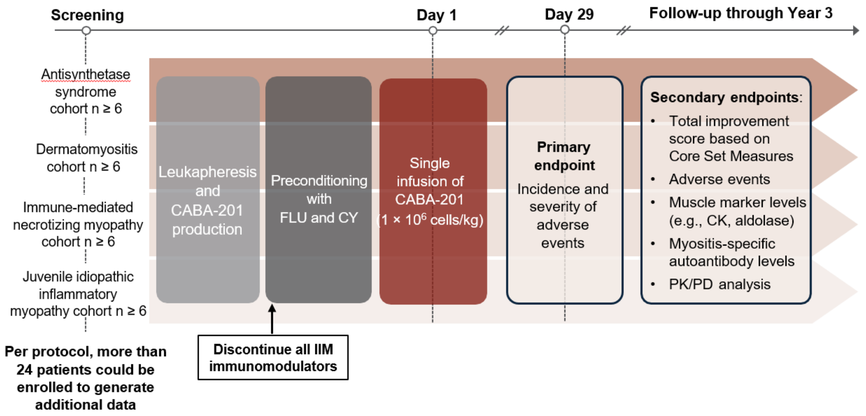

Figure 2. RESET-Myositis Study Design[7]

ACR, American College of Rheumatology; CAR T, chimeric antigen receptor T cells; CK, creatine kinase; CY, cyclophosphamide; EULAR, European Alliance of Associations for Rheumatology; FLU, fludarabine; HSCT, hematopoietic stem cell transplant; IIM, idiopathic inflammatory myopathy; JIIM, juvenile IIM; MAA, myositis-associated antibody; MSA, myositis-specific antibodies; PK/PD, pharmacokinetic/pharmacodynamic; rese-cel, resecabtagene autoleucel; RESET™, REstoring Self Tolerance;

Summary

- Rese-cel is an investigational, CD19-CAR T therapy made from a patient's own white blood cells.
- Rese-cel is designed to potentially reset the immune system by targeting the B cells that may be causing disease.
- RESET-Myositis (NCT06154252) is an ongoing Phase 1/2 trial evaluating the safety and efficacy of rese-cel in patients with myositis.
- Data from 18 adults with autoimmune disease who received rese-cel (**including 8 with myositis**) were recently presented at a scientific meeting.*
- Rese-cel was generally well tolerated across 8 IIM subjects treated in RESET-Myositis.*
- 7 of the first 8 adult patients in RESET-myositis have achieved a clinically meaningful response off all immunomodulatory medications and steroids (or while tapering steroids).*
- The study is actively enrolling patients with JIIM and adult IIM
- Cabaletta is planning to initiate two registrational adult myositis cohorts of ~15 patients each this year.

*as of 6May25

Actively Recruiting Participants

If you are interested in participating in the RESET-Myositis study, please discuss with your doctor.

The RESET-Myositis trial has sites across the USA and UK. As of June 2025, we have two sites accepting juvenile myositis patients. The full list of sites is here: www.clinicaltrials.gov/study/NCT06154252

For more information, visit www.cabalettabio.com or contact clinicaltrials@cabalettabio.com

Rese-cel is an investigational treatment which has not been approved by the US Food and Drug Administration (FDA) and has not been determined by the FDA to be a safe or effective treatment for any disease or condition.

References:
1. Lundberg IE, et al. Nat Rev Dis Primers. 2021;7(1):86. **2.** Liu T, et al. Clin Rev Allergy Immunol. 2025;68(1):40. **3.** Sheikh, S. RESET-SLE: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), a Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Non-Renal SLE and Lupus Nephritis. Oral Presentation presented at EULAR; June 12, 2025; Barcelona, Spain **4.** Tummala, R. RESET-Myositis™: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), a Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Idiopathic Inflammatory Myopathies. Oral Presentation presented at EULAR; June 13, 2025; Barcelona, Spain. **5.** Khanna, D. RESET-SSc™: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), a Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Systemic Sclerosis. Oral Presentation presented at EULAR; June 13, 2025; Barcelona, Spain. **6.** C&EN Oncology. 2024. Available at: https://cen.acs.org/pharmaceuticals/oncology/Controlling-CAR-T-scientists-plan/96/i19 (accessed May 2025). **7.** NCT06154252. Available online at: www.clinicaltrials.gov/study/NCT06154252 (accessed June 2025).