

MYOCONNECT *Summit*

August 2026

What Patients Should Know About Emerging Therapies

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Disclosures

P.C.G. has served as an investigator or data/safety officer for multiple industry-partnered clinical trials in myositis (including Argenx and Cabaletta Bio).

He has received past honoraria from: Boehringer Ingelheim, Howard Hughes Medical Institutions (HHMI), Janssen Kezar Life Sciences, National Institutes of Health (NIH), Rheumatology Society of DC, The Myositis Association (TMA).

We have Slides but No “to-do” List

Our Hot Take:

Myositis is one of the most important families of autoimmune diseases right now

What is a Clinical Trial ?

Formal method(s) for assessing an intervention for a particular disease(s)

Cases/Case-series are anecdotal

Negative cases often not reported

Limited safety and tolerability information

Need to evaluate the safety as well as efficacy of intervention

Different designs or options depending on study needs

General Categories of Clinical Trials (i.e., “Phases”)

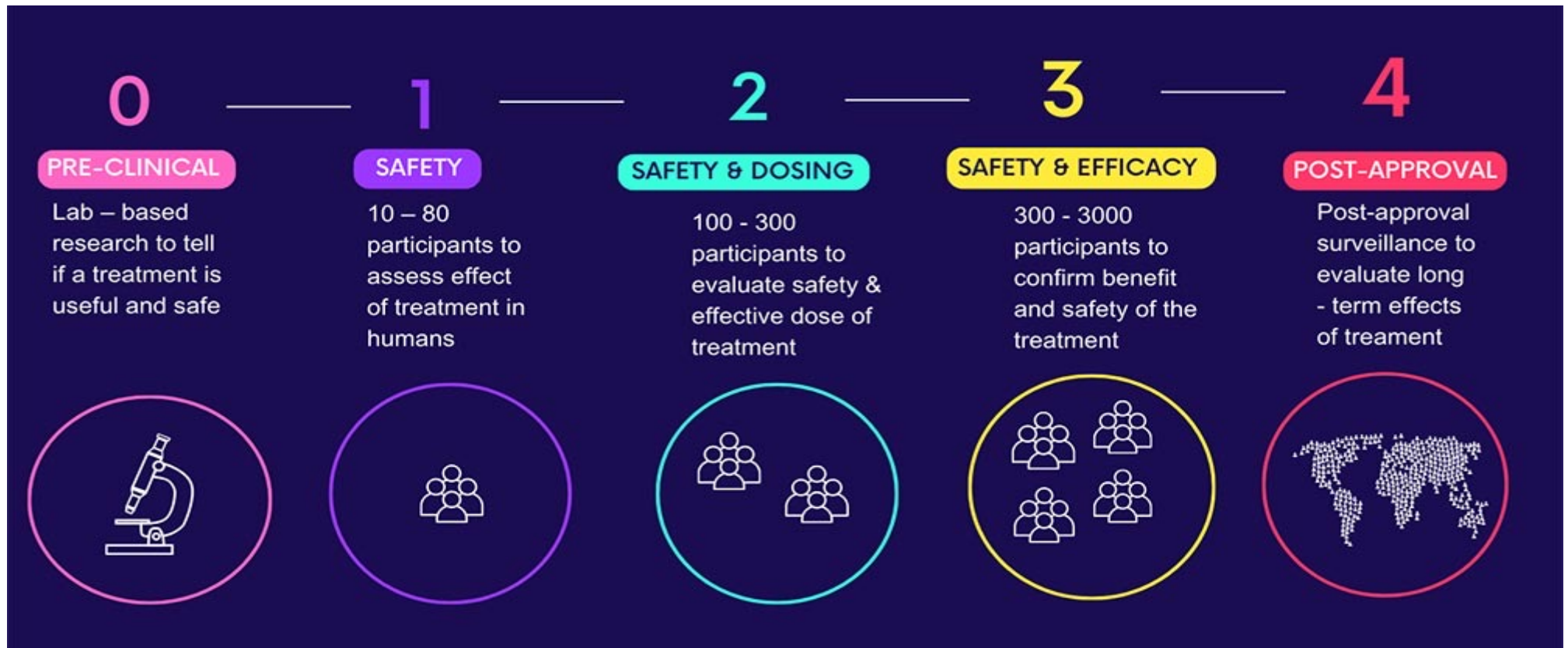


Image from Rare Disease Clinical Network

Why do Clinical Trials ?

Still MANY unanswered questions and unmet needs in myositis

- Are there Rx that work better than what we have?
- What patients most likely to respond to what interventions?
- How do we best use the Rx we already have?



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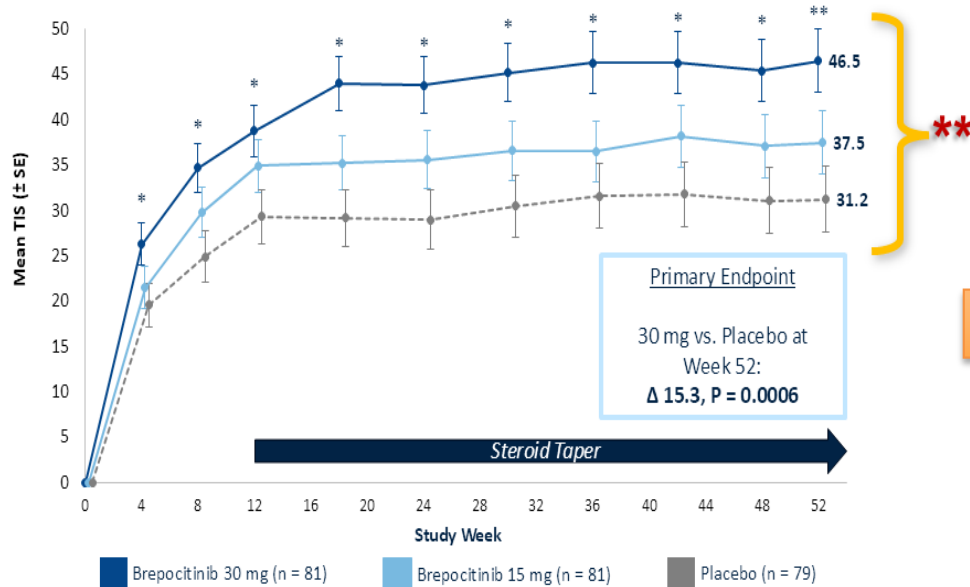
Part 1:

New Medications from Phase 3 Trials !

Brepocitinib (VALOR)

Oral JAK1/TYK2 tested in Dermatomyositis

VALOR showed durable statistically significant benefit in the Total Improvement Score and greater corticosteroid reduction compared to placebo



Aug 27, 2026

Brepocitinib (Lisraya) 30 mg now FDA-approved for dermatomyositis in adults !



Efgartigimod alfa (ALKIVIA)

FcRN blocker tested in DM/JDM, ASyS, IMNM

Phase 2 (24-week study, 89 pts) showed benefit



Phase 3 (52-week study, ~150 pts) completed in 2026

Phase 3 stage (52 weeks), visits Q4W, n≈150 (ongoing)¹⁻³



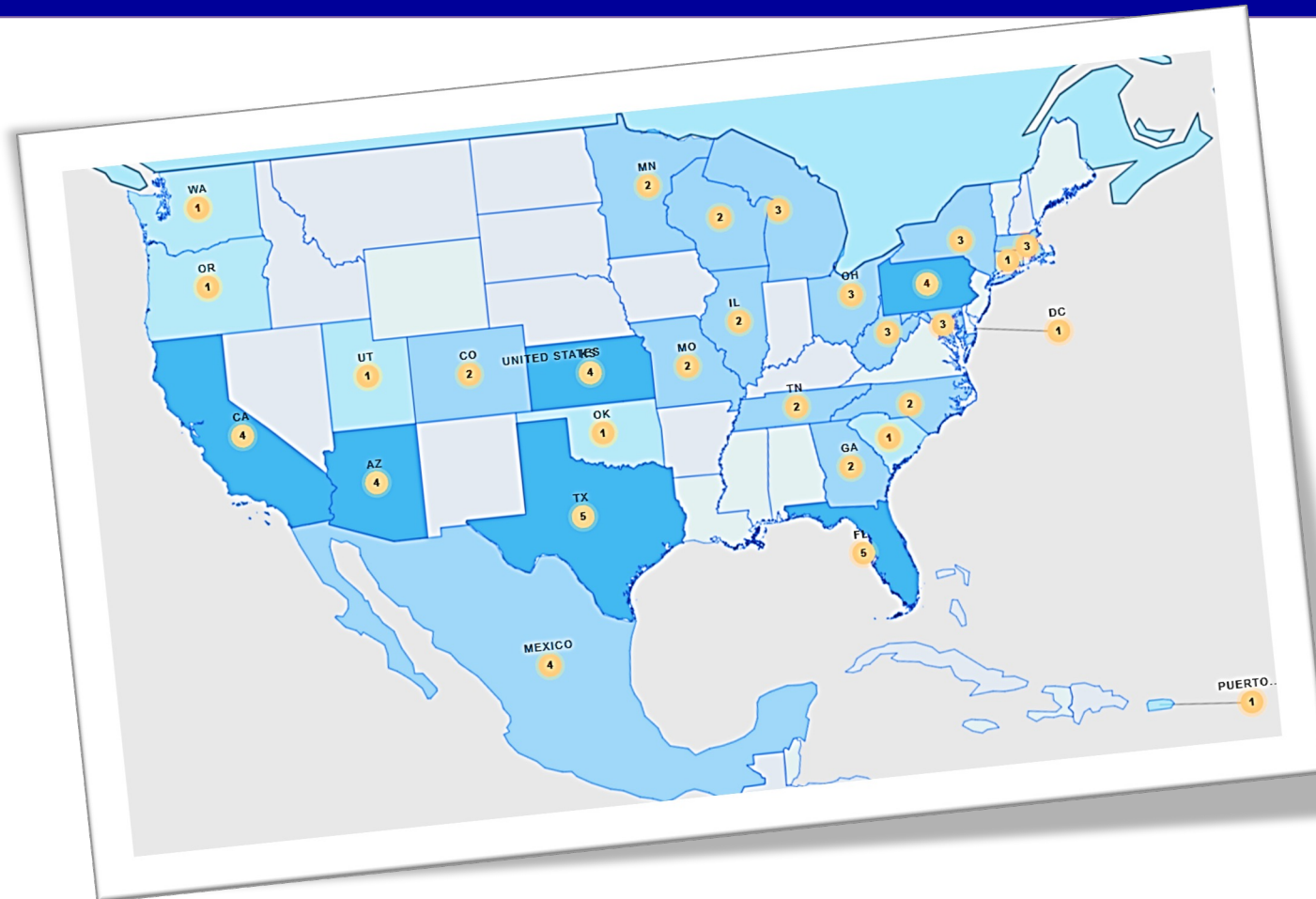
Aug 17, 2026

Topline Results positive !

Improved TIS compared to placebo
IMNM and DM sub-groups had benefit

More details pending

US Cities with Active Phase 3 Myositis Trials (Aug 2026)





Upcoming NIH-Supported Investigator Trials

- **MiGHT Trial**

Exploratory Clinical Trial of IVIG in Anti-HMGCR Immune Mediated Necrotizing Myopathy

- **SUMMIT Trial**

Study of Ublituximab in Early, Active, Autoantibody-Positive, Immune-Mediated NecroTizing Myopathy



National Institute of
Arthritis and Musculoskeletal
and Skin Diseases



National Institute of
Allergy and
Infectious Diseases

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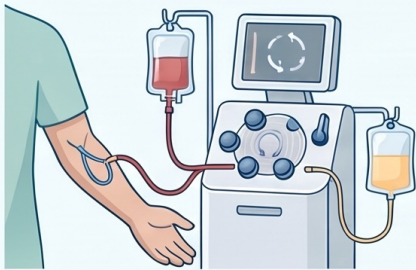
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Part 2: **Cellular Therapies !**

Understanding CAR-T Therapy: Your Step-by-Step Treatment Journey

CAR-T therapy uses a patient's own immune cells to fight disease. This process involves collecting T cells, "reprogramming" them in a lab to find specific targets, and returning them to the patient to perform an "immune reset."

Part 1: Manufacturing Your Cells



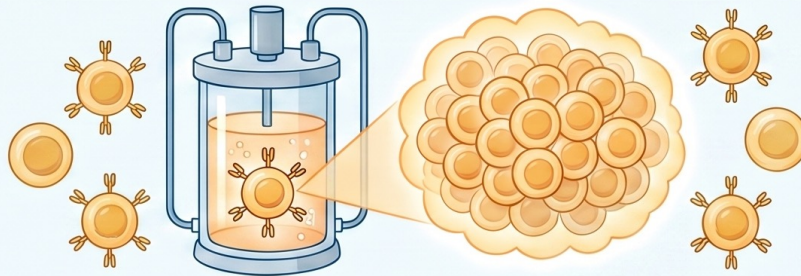
1. Cell Collection (Leukapheresis)

Healthcare providers collect blood to separate and remove your T cells.



2. Genetic Engineering

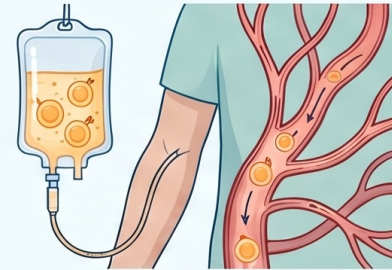
In a lab, a "GPS" receptor (CAR) is added to your cells.



3. Cell Multiplication

These modified cells are grown until they number in the millions.

Part 2: Your Treatment Process



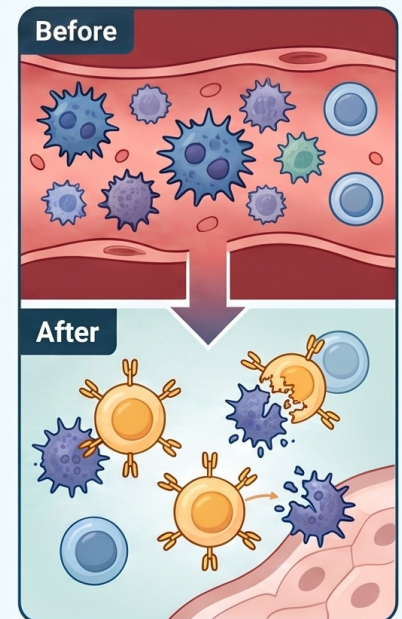
5. Cell Infusion

Your "supercharged" T cells are returned to your bloodstream via IV.



4. Body Preparation

Brief chemotherapy is given to clear space for the new cells.



6. The Immune Reset

The cells seek out and destroy disease-driving B cells to restart your immune system.

Typical CAR-T	Anti-CD-19 CAR-T, anti-BCMA CAR-T, Dual anti-CD19/BCMA CAR-T	Aimed at deep B-cell depletion and "immune reset" by targeting B cells and plasmablasts.
Allogeneic CAR-T	UCAR-T, FT819 (iPSC-derived)	"Off-the-shelf" platforms using donor cells or gene-editing to provide standardized availability and bypass manufacturing delays
In vivo CAR-T	CAR-encoding payloads (e.g., lipid nanoparticles)	Engineering circulating T cells directly within the body to bypass ex-vivo manufacturing for faster, more scalable treatment.
CAR-Natural Killer (CAR-NK)	anti-CD19 CAR-NK; multi-target CAR-NK platforms	Utilizes the innate cytotoxicity of NK cells; offers potential for off-the-shelf use with a lower risk of CRS/ICANS.
Chimeric Autoantibody Receptor (CAAR-T)	CAAR-T strategies	Selective elimination of autoreactive B-cell clones while sparing global humoral immunity.
CAR Regulatory T cells (CAR-Tregs)	CAR-Tregs	A strategy aimed at restoring immune tolerance rather than inducing cytotoxic depletion.
Next-generation CAR platforms	anti-BAFF-R CAR-T, TRUCKs (4th gen), "Armored CARs"	Incorporates inducible cytokines, novel modules (e.g., anti-BAFF-R), or BiTE-secreting modules to modulate the immune environment.
BiTEs	Bispecific T-cell engagers	Advanced antibody constructs that redirect cytotoxic T cells toward antigen-expressing target cells.
TriTEs	Trispecific T-cell engagers	Trispecific constructs designed to simultaneously bind T-cell antigens and one or two separate targeted cell-associated antigens.
BiKEs	Bispecific killer cell engagers	Constructs that activate and bridge NK cells to target cells, often through CD16 engagement, though specificity can be limited.
TriKEs	Trispecific killer cell engagers	NK-cell engagers designed to address the specificity and antigen escape limitations of BiKEs.

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Part 3:
What About Me ?

Should I Take Part in a Clinical Trial ?



Should I Take Part in a Clinical Trial ?



- How do you feel about taking part in a Particular trial?
- What are the other options?
- Think about participation early in treatment
- Patient resources for the trial

What Kinds of Questions Should I Ask ?

- What is known about the investigational Rx?
- What are my other options?
- Is it placebo-controlled? If so, what is the design and allocation?
- What types of assessments are done?
- What does the follow-up schedule and length look like?
- What are limitations/logistics (e.g., Need to stay nearby? Limitations on exposures?)
- What about the Open-Label Extension?
- What patient support is included in the trial?



Why Is Important for me to Know about Myositis Clinical Trials?



Knowledge is power. Sharing knowledge is the key to unlocking that power.

✦ MARTIN UZCHUKWU
UGWU



- **Know your options**
- **Help the Community**
- **Educate Doctors and Patients**

Where to Get More Info about Current Myositis Clinical Trials?

ClinicalTrials.gov

 **National Library of Medicine**
National Center for Biotechnology Information

PRS Login

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My Saved Studie

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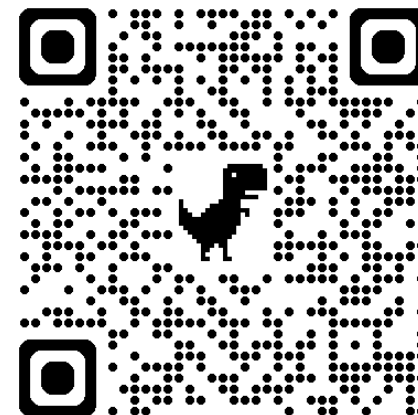
Condition/disease ⓘ

Other terms ⓘ

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Feedback





Thank you