

MSA Clinical Trials: Where We Are Today

Dr. McFarland, neurologist and movement disorder specialist at the University of Florida's MSA Center of Excellence, presented an overview of the current state of Multiple System Atrophy (MSA) clinical trials — where the field has been, where it's going, and what patients and families should know. Watch the full webinar [here](#).

CURRENT TRIAL LANDSCAPE

Today, MSA treatment focuses primarily on **symptom management** (blood pressure, motor symptoms, sleep, GI issues). The field's main goal is finding **disease-modifying therapies** — treatments that slow or stop the underlying brain pathology, ideally as early in the disease as possible.

KEY TRIALS TO WATCH

Immunotherapy (targeting alpha-synuclein protein)

- **Aphotope (APHRIS)** — A vaccine targeting pathologic alpha-synuclein; Phase 2 completed with encouraging Phase 2 results; potential Phase 3 ahead
- **MASCOT (Lundbeck / emlanatig)** — Passive immunotherapy via monthly infusion; Phase 3 enrollment closed, results pending

Protein Aggregation Inhibitors

- **ATH434 (Alterity Therapeutics)** — Targets protein misfolding and brain iron; Phase 2 showed improvement in daily activities and brain scans; Phase 3 in development
- **ONO-2808 (Deciphera)** — Phase 2 complete; Phase 3 under consideration
- **Topaz / Arusomin (Teva)** — Ongoing Phase 2 trial

Anti-Inflammatory Therapies

- **Yota (Dasher Neuroscience)** — Ongoing Phase 2
- **Brilunab (Tisiana Life Sciences)** — Nasal drug targeting microglial inflammation; ongoing Phase 2

Neuroprotective & Other Approaches

- **Ubiquinol (Coenzyme Q)** — Early Japanese Phase 2 showed a signal; larger trial may follow
- **Gene therapy** — Targeting alpha-synuclein production (Ionis Pharmaceuticals) and GDNF growth factors; early-stage research
- **Stem cells (mesenchymal)** — Early studies suggest possible benefit, but larger controlled trials are needed; mechanism still not fully understood

HOW TO GET INVOLVED IN A TRIAL

- Visit [**clinicaltrials.gov**](https://clinicaltrials.gov) and search for active, enrolling MSA studies
- Ask your neurologist: "Am I a good candidate? Is this the right time? What would I need to stop or change?"
- Contact the trial's research coordinator — ask about purpose, visits, drug delivery, side effects, and caregiver requirements
- Participation is **voluntary**, and you can withdraw at any time

MISSION MSA RESOURCES

- Watch Dr. McFarland's full presentation [here](#).
- [Guide: How to Use clinicaltrials.gov](#)
- [Guide: What to Ask When You're Interested in a Clinical Trial](#)
- [Video: Clinicaltrials.gov walkthrough](#)

For questions or resources, contact Mission MSA at info@missionmsa.org.