

Benjamin Greenberg, M.D., M.H.S.
Director, Neurosciences Translational Research Center
UT Southwestern
Dallas, Texas

The Research Process

At its core, research is about asking and answering questions.

Why do we do research?

Proving cause and effect is important for understanding diseases and identifying effective treatments

What happens without research?

- Therapies that are not effective are used
 - Bloodletting
- Therapies that are dangerous are used
 - Cocaine
- Therapies that are not effective AND dangerous are used
 - Mercury

Research saves us from ourselves: The minocycline story in ALS

- Minocycline is safe molecule currently used as an antibiotic
- ALS is a uniformly fatal, neurodegenerative condition.
- In cell culture models and animal studies, minocycline helped with neuronal survival.
- So for a terminal condition, why wouldn't we put every ALS patient on this safe, approved, medicine????

Research saves us from ourselves: The minocycline story in ALS

 ALSFRS-R score deterioration was faster in the minocycline group than in the placebo group (-1.30 vs -1.04 units/month, 95% CI for difference -0.44 to -0.08; p=0.005). Patients on minocycline also had non-significant tendencies towards faster decline in FVC (-3.48 vs -3.01, -1.03 to 0.11; p=0.11) and MMT score (-0.30 vs -0.26, -0.08 to 0.01; p=0.11), and greater mortality during the 9month treatment phase (hazard ratio=1.32, 95% Cl 0.83 to 2.10; p=0.23) than did patients on placebo.

How do we do research?

Retrospective

Epidemiologic

Observational

Interventional

How do we do research?

Hypothesis Generation

Preclinical

Phase 1

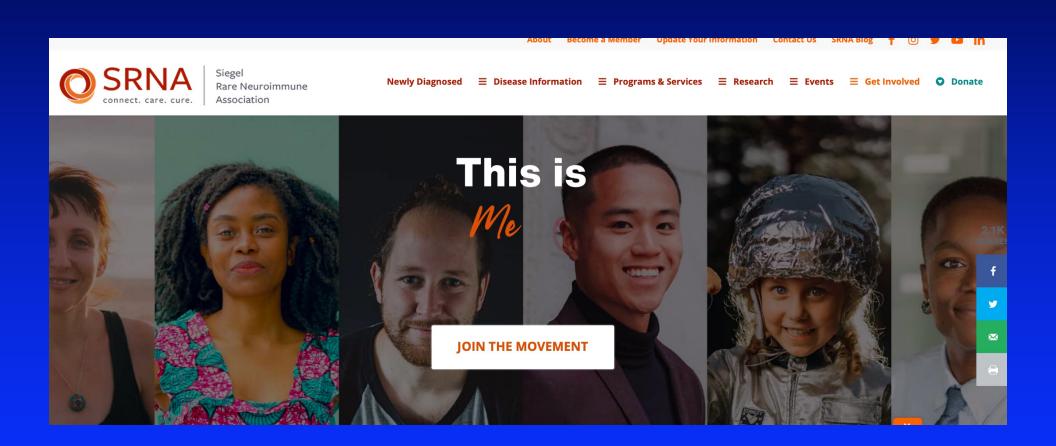
Phase 2

Phase 3

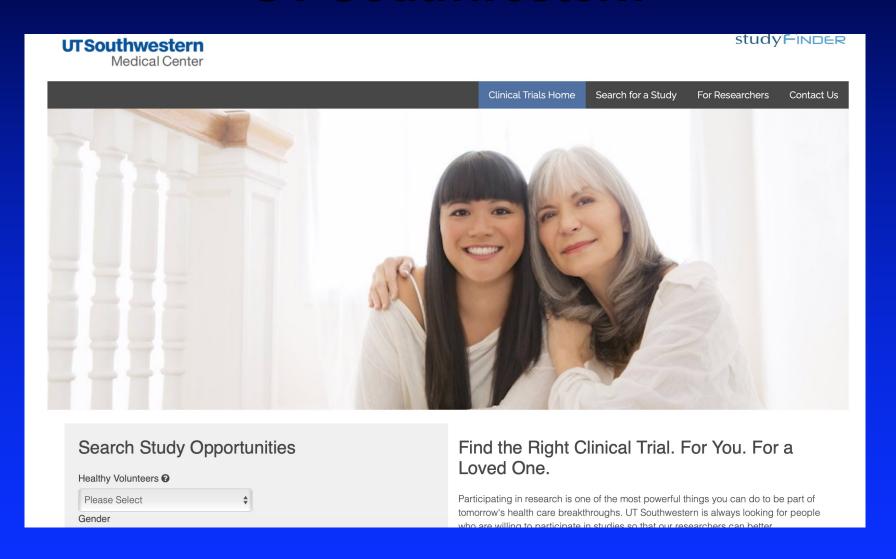
Opportunities to Assist in Research

- Registries
- Surveys
- Samples
- Prospective observational
- Open label interventional
- Randomized active comparator
- Randomized placebo controlled

The SRNA and Research



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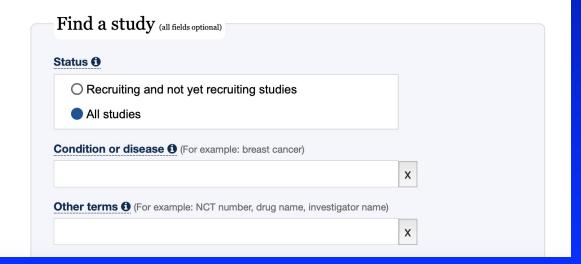
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IMPORTANT: Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our <u>disclaimer</u> for details.

Before participating in a study, talk to your health



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