

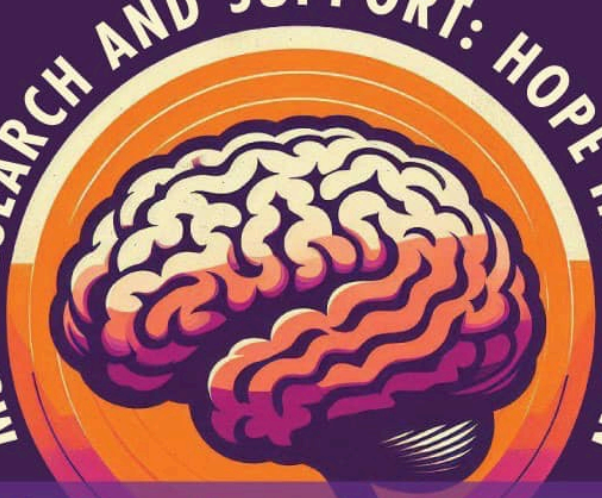
NEWS & VIEWS: AUGUST 2024

FREE CONFERENCE - REGISTER NOW

https://registration.crowdcomms.com/defeatmsa

September 7-8, 2024

MSA RESEARCH AND SUPPORT: HOPE IN ACTION



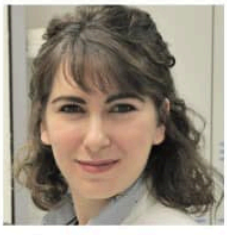
MULTIPLE SYSTEM ATROPHY RESEARCH UNITED

5th Multilingual Free All-Community

MSA Virtual Conference

Live Q & A with Experts • New Research Updates
Therapy Topics: Physical, Speech and Occupational

Spotlight Speakers:



Alessandra Fancuilli, MD, PhD
Innsbruck, Austria



Edwin George, MD, PhD
Movement Disorders, Drug Evaluation (FDA)



Elizabeth Coon, MD
Mayo, MN



Henry Houlden, MD
UCL, UK

Theravance Biopharma

AskBio

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REGISTER NOW FOR OUR ANNUAL MSA CONFERENCE

https://registration.crowdcomms.com/defeatmsa

- Also, Get A Free Awareness Package with your Conference Registration!

(Sorry, due to shipping costs, US residents only, one per patient & while supplies last)

SHOE FLOAT MAKES IT INTO WORLD FAMOUS ARTPRIZE 2024!





We are pleased to announce that the **MSA Awareness Shoe Float** was accepted into the world renown ART PRIZE - a bi-annual arts festival in Grand Rapids, Michigan. The float is a supersized clone of the **Official MSA Awareness Shoe (tm)**, a

series of 7 actual globetrotter shoes traveling the world, in honor of all our MSA warriors, people fighting MSA every day now and also in memory of those we have lost to the beast, called MSA.

The ART PRIZE festival and competition was founded in 2009 and is considered one of the most well-attended "big ticket" art events in the US, with over a million visitors throughout a 2 week run. This year, the art festival exhibit entries (approximately 1000 art pieces), will be competing for cash prizes, totaling 500K by a public voting opportunity and by a jury of art experts.

The float will be judged in the 3D category. The concept, purpose and promotional objects associated with the Shoe Float (and all of the official MSA Awareness shoes too) was developed by the volunteers connected to the US nonprofit, Defeat MSA Alliance (501c3).

This is the second time, the **Official MSA Awareness Shoe** (which will appear with the Float) will be visiting Grand Rapids. The Shoe was previously hosted by the **Van Andel Research Institute** in Grand Rapids, Michigan in 2018. So one could say, this will be a return Grand Rapids engagement. Researchers at the **Van Andel** have conducted MSA related research for many years.

The **Supersized MSA Awareness Shoe Float** will be hosted at the outdoor entrance of Cornerstone Church in the Heritage Hill district. The **Float is the world's first MSA Awareness Float.** It has already appeared in a major parade (in Detroit) and is scheduled to start a world tour in 2025. Learn about the Shoe on its website: www.msashoe.org and if you want to help organize the world tour, please do reach out: info@msashoe.org

For information on the Art Prize, visit: https://www.artprize.org/

European Academy of Nuerology

DEFEAT MSA VISITS FINLAND (at least our graphics did)



Photo Credit: Theravance Biopharma

Defeat MSA Alliance advocated for the MSA community at the **European Academy of Neurology (EAN)** Annual Conference in Helsinki, Finland this past June. No Defeat MSA volunteers were able to attend in person.

EAN is a non-profit, independent organization representing more than 45,000 neurology professionals, as well as 48 European national neurology related societies. As a medical society the EAN's mission is to promote excellence in the practice of neurology throughout Europe, leading to improved patient care.





START A FUNDRAISER OR DONATE NOW - HELP US DEFEAT MSA NOW!

4 Ways to Support Our Efforts:

1) Create a Facebook Fundraiser:
https://www.facebook.com/fund/DefeatMSA/

2) Create a Virtual Fundraiser (dedications to a loved one are possible too):
https://www.justgiving.com/defeatmsaalliance

3) Donate Directly to Our Secure Website (dedications to a loved one are possible too):
https://www.defeatmsa.org/donate-to-us/

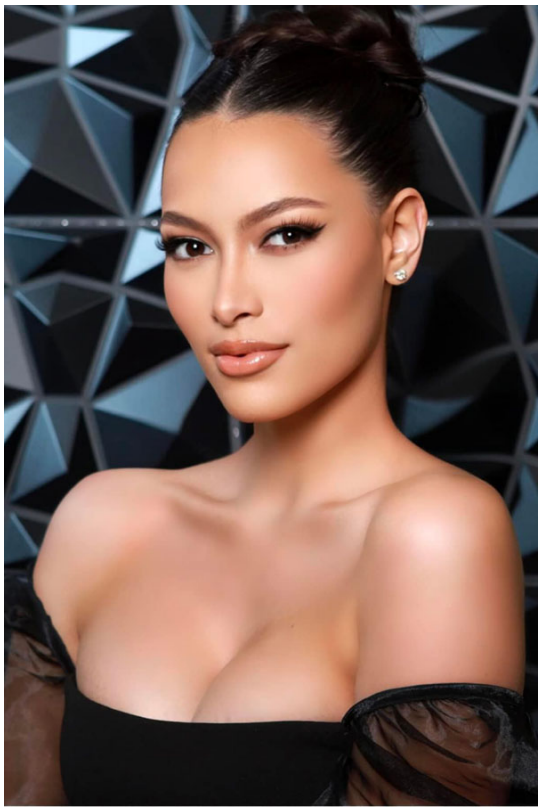
4) Via Check, Send to:
Defeat MSA Alliance, 29924 Jefferson Avenue, Saint Clair Shores, Michigan, 48082, USA

CHECKOUT OUR MONTHLY ADVOCATE SPOTLIGHT

Our June & July Advocates

Be sure to be on the lookout for our monthly MSA advocates!

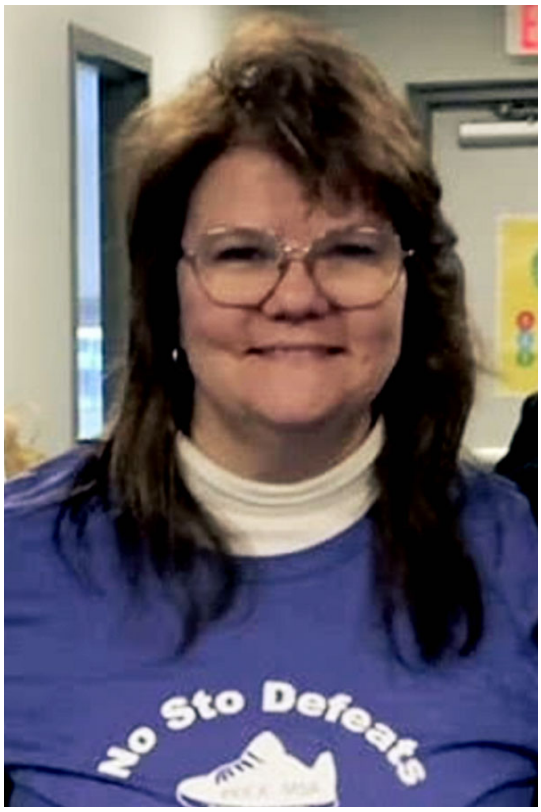
If you or someone you know would like to featured as a monthly difference maker, please send an email to jennifer.frost@defeatmsa.org.



JUNE ADVOCATE

Arianna Siefiert (Bustos)

Our first **MSA Awareness Advocate, Arianna Siefiert, Miss Las Vegas 2024**, continues the fight in honor of her late father.



JULY ADVOCATE

Emily Knowles

Emily, founder of the **NoStoDefeatsMSA5K,** continues her fight in honor of her late sister, Victoria Lynn Davis Schnieder.

SIGN UP TO JOIN THE 2024 NO-STO-DEFEATS-MSA-5K RACE HERE!



THE RESEARCH RADAR

Biomarkers in MSA Clinical Trials: What's New?

Multiple system atrophy (MSA) is a rare neurodegenerative disease that currently has no cure, with treatment focusing on managing symptoms[1,2]. But research is underway in hope of finding new and effective disease-modifying treatments—therapies that provide a "meaningful and lasting change in the clinical course and the underlying pathophysiology of the disease"[3,4]. Biomarkers can play a key role in developing new treatments – as well as managing disease – as effective biomarkers can help diagnose a disease, assess the severity of a disease and its progression, and monitor how effective a treatment is[5,6]. Here, we round up some of the recent clinical trials for MSA, with a focus on studies investigating biomarkers.

Biomarkers

A biomarker is a "biological molecule found in blood, other body fluids, or tissues that is a sign of a normal or abnormal process, or of a condition or disease. A biomarker may be used to see how well the body responds to a treatment for a disease or condition"[35]. Diagnosis of MSA can be difficult as patients can have a variety of symptoms or share symptoms or clinical features with other diseases, such as Parkinson's disease or progressive supranuclear palsy. And semi-quantitative methods to assess how well a treatment is working – such as the unified multiple system atrophy rating scale – can have limited reliability. Consequently, finding effective MSA biomarkers are needed for a range of uses, such as helping diagnose disease, determining treatment, and monitoring the effects of a

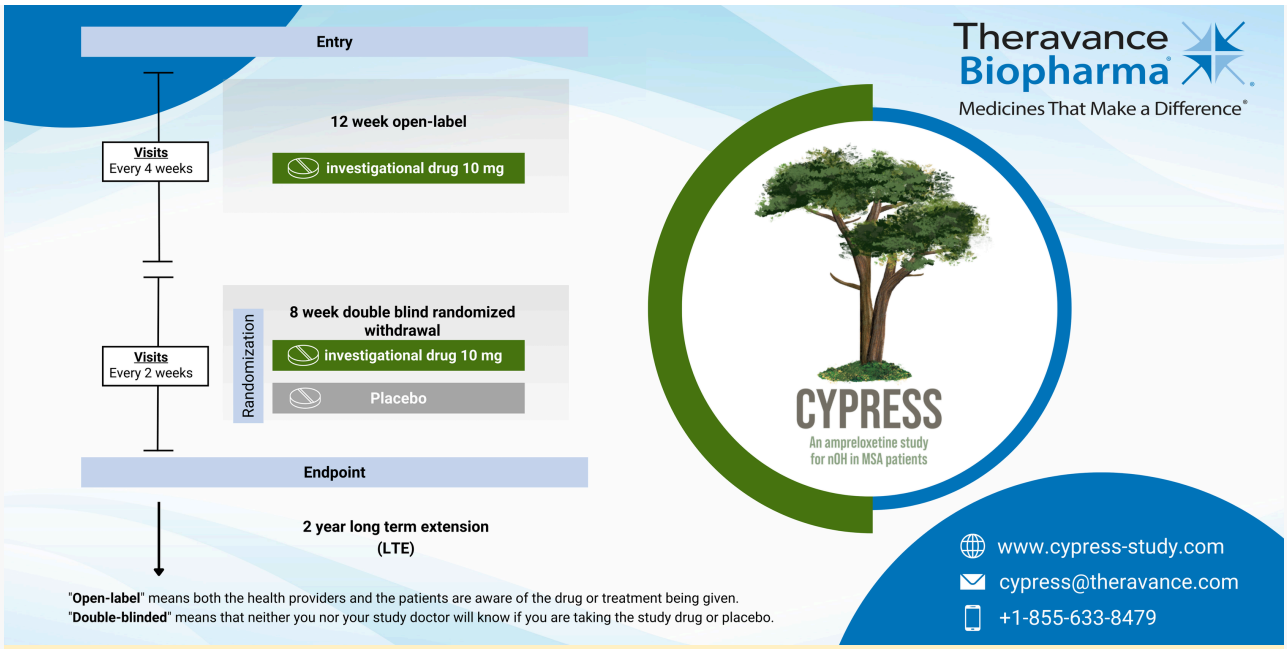
treatment[2,5,6]. A variety of biomarkers are currently undergoing testing for MSA, such as:

- **Alpha-synuclein** – for example, a clinical trial ("**Misfolded Proteins in the Skin of People With Parkinson's Disease and Other Parkinsonism**"; NCT04518059) is assessing whether the levels of misfolded alpha-synuclein from skin biopsies can be used to determine the type of parkinsonism a patient has.
- **Digital speech analysis** – a clinical trial ("**Differential Diagnosis Between Parkinson's Disease and Multiple System Atrophy Using Digital Speech Analysis - Part 2 (Voice4PD-MSA)**"; NCT05807373) is the second part of a study that has been looking to develop a digital voice-based tool to help discriminate between MSA (specifically MSA-P) and Parkinson's disease.
- **PET imaging** – for example, a clinical trial ("**PET Imaging Evaluation of [11C]SY08**"; NCT06098612) is examining whether PET imaging (using 18F-MFBG as a tracer) can be used to discriminate between MSA and Parkinson's disease, as well as discriminate dementia with Lewy bodies from Alzheimer's disease. PET imaging involves administration of a tracer (a radioactive molecule) into the patient and then scanning the body to detect where the tracer lands or is taken up, and how intensely. For example, in this study, researchers will look at heart uptake of the PET tracer (18F-MFBG) in participants —and also compare the test to another form of imaging ([123I]-MIBG SPECT).
- **Unstructured eye tracking** – a clinical trial ("**Unstructured Eye Tracking as a Diagnostic and Prognostic Biomarker in Parkinsonian Disorders**"; NCT05638477) is looking at whether measuring eye movements and pupil changes can be used to discriminate Parkinson's disease from atypical parkinsonism (such as MSA).

For more information on clinical trials related to biomarkers and disease-modifying treatments, as well as symptomatic treatments, visit www.defeatmsa.org/msa-research/pipelines

And see Defeat MSA's latest *Research Radar* newsletter for further information on alpha-synuclein targeting therapies.

For the Complete List of Blog References, contact Defeat MSA Alliance (info@defeatmsa.org)



THERAVANCE BIOPHARMA - NEW MSA DRUG TRIAL - FOR ORTHOSTATIC HYPOTENSION

Start the year on the right foot, Theravance Biopharma continues to enroll participants in the Phase 3 CYPRESS study.

Consider joining the study as they take another critical step toward the goal of managing symptoms of nOH for people with MSA.

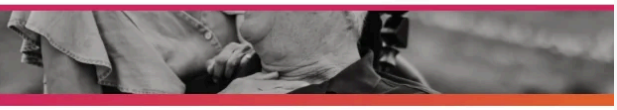
If you have been diagnosed with MSA and have blood pressure problems, contact them! They may be able to help.

For more information, including the locations of study centers, please email them at: cypress@theravance.com, or call **1-650-808-3737**

or visit the study website at www.cypress-study.com

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Find a Study



Multiple System Atrophy | Part 1: ION464

Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of ION464 Administered to Adults With Multiple System Atrophy (HORIZON)

This is a first-in-human, randomized, blinded, placebo-controlled, multiple-ascending-dose (MAD) study (Part 1) to evaluate the safety, tolerability, PK, and PD of ION464 in adult participants diagnosed with MSA with a long-term extension (LTE) (Part 2). The study will include up to approximately 40 participants. Part 1 of the study consists of a Screening Period of up to 6 weeks, a Treatment Period of 12 weeks, and a Follow-up Period of 24 weeks. The study duration for each participant in Part 2 will be approximately 96 weeks, which consists of a 72-week Treatment Period and a 24-week Follow-up Period.

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NEW MSA DRUG TRIAL INFORMATION - STUDY LOCATIONS: UK, PORTUGAL, FRANCE, AUSTRIA AND GERMANY

- If you are interested, do reach out to DEFEAT MSA ALLIANCE, we will help connect you: info@defeatmsa.org

IONIS WEBSITE: https://ionistrials.com/?s=msa

AskBio

A Bayer Cell & Gene Therapy Platform Company

Now Recruiting

AskBio MSA Phase 1/2 Clinical Program

Learn more about our actively recruiting study at Multiple System Atrophy (MSA) Clinical Trial - AskBio, or connect with us directly at askfirst@askbio.com.

At AskBio, bringing the potential for life-changing advanced gene therapeutics to patients with diseases that have a high unmet medical need fuels our research and development pipeline.

AskBio's approach to potentially treating multiple system atrophy (MSA) uses a glial cell-line neurotrophic factor (GDNF) gene therapy that takes advantage of the brain's natural production of the GDNF protein, which is required for the development and maintenance of dopamine brain cells. These brain cells are typically lost in MSA patients. Our goal with AB-1005 (also known as AAV2-GDNF-MSA) is to potentially promote the survival and function of dopamine producing brain cells, which may lead to significant motor function recovery for MSA patients.

MSA-101 is a randomized, Phase 1/2 clinical trial evaluating the safety and potential effects of AB-1005 in people with multiple system atrophy-parkinsonian type (MSA-p).



- AB-1005 is a one-time gene therapy delivered surgically into the brain to provide a continuous expression of the GDNF protein.
- Eligible participants have a 2 out of 3 chance of receiving active treatment versus placebo.
- Participants randomized to placebo will undergo minimal surgery and may be offered the gene therapy product after the main part of the study.

AB-1005 is an investigational therapy and has not been approved by the U.S. Food & Drug Administration (FDA) or any other health authority.

ASK BIO is NOW RECRUITING for their AAV2-GDNF-MSA study.

Click here for the study website for more information

SHOP MSA AWARENESS APPAREL!

One way to help us defeat MSA is to purchase official **DEFEAT MSA MERCH!** Proceeds go towards our research and awarenss efforts.

Help spread awareness by posting your merch haul to social media with hashtags: **#defeatmsa** & **#kickmsa**



KICK MSA T-Shirt



Distressed Dad Hat

MUGS, HATS, AND MORE AT OUR OFFICIAL STORE

All the charities affiliated with **MSA United International Consortium** are staffed entirely by volunteers - every single donation goes directly into our great cause.

We are the WORLD'S ONLY MAJOR CHARITY still operated entirely by volunteers. That means, we do not use public donations to pay high salaries of executives and staff!

Lastly, **MSA United International Consortium** is the world's only research consortium of MSA organizations.

Help Us Defeat MSA Now and Forever!

Donate - USA

Donate - Canada

Donate - New Zealand

Donate - Denmark

Donate - Italy

You can donate directly using

https://www.msaunited.org/donate-to-us/

DEFEAT MSA ALLIANCE & MSA UNITED CONSORTIUM

29924 Jefferson Ave,

St. Clair Shores, Michigan, 48082
USA

855 542-5672 (855 Kick-MSA)
info@defeatmsa.org

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