Together We Can Make A Difference
ADVANCE RESEARCH: GET INVOLVED
Research Opportunities with the Emory Parkinson’s Disease and Movement Disorders Program
Working Together

Emory University is a leading clinical and basic research center for Parkinson’s Disease and other movement disorders including dystonia, essential tremor, Huntington’s Disease, and Tourette’s syndrome. As a part of the Emory School of Medicine, our Center serves as a major referral center for both adult and pediatric patients in Atlanta, the state of Georgia, and the Southeastern United States.

Our patient’s lives are improved from the synergy found in a setting like Emory. We bring together medical discoveries through research and provide the newest, most beneficial treatments available that range from diagnosis to rehabilitation. Our neurosurgeons, neurologists, psychologists and researchers work together to tailor treatment for the specific needs of each patient.

The twelve clinical faculty members in Emory Movement Disorder program see approximately 6,000 patients annually of which 3,000 have Parkinson’s disease or parkinsonism, making this probably the single largest specialty group for this condition in the world.

The program is internationally recognized for the pioneering work of Drs. Mahlon DeLong, Thomas Wichmann and others in the group who played integral part in the discovery of brain circuitry changes relating to Parkinson’s disease which lead to novel surgical therapies that were brought into clinical practice worldwide. The Emory program is also internationally recognized for its advanced work on the role of pesticides and other environmental influences in Parkinson’s disease, as well as being the central site for the Dystonia Coalition.

It would be impossible to list here all the ways we have enhanced our delivery of care to patients and family members. This work is possible with the help of individuals such as yourself. We’ve made outstanding progress, but we haven’t uncovered every opportunity. Each discovery brings us closer to unlocking the answers to neurological brain disorders. It’s gratifying to have your support and vote of confidence in our ability to make a difference. Our only hope for change is to work collectively on the issues we know no one can tackle alone.

Sincerely

Stewart Factor, DO
Vance Lanier Chair in Neurology
Director, Emory Parkinson’s Disease and Movement Disorders Program
What is a clinical research study?
A clinical research study is a carefully designed scientific evaluation of an investigational drug conducted by doctors. Clinical research studies help to answer important medical questions, such as how a new drug acts in the body, how it affects certain diseases or conditions, and whether or not it is safe for wider use. Because clinical studies are voluntary, participants are free to leave the study at any time for any reason.

Why are clinical research studies important?
Clinical studies are the only way new medications for diseases can become approved for widespread public use. They provide a way to test drugs so we will know if they are safe and effective. People who participate in clinical studies contribute to research that will further the knowledge about the treatment of diseases.

PARKINSON’S DISEASE

TOZ-PD STUDY
The TOZ-PD Study is divided into two parts. Part A will test the effect of tozadenant compared to a placebo over a six month time period. A placebo looks exactly like the medication being tested, but contains no active drug. During this part of the study, you will receive either tozadenant 60 mg, tozadenant 120 mg, or a placebo two times daily. Part A of the study evaluates the effects of the study drug. To evaluate the drug, you will be asked to maintain a home diary of when your medication is providing benefit, or when it has worn off. During this part of the study, neither you nor your study doctor will know if you are receiving tozadenant or placebo. Following Part A, subjects will continue into Part B and receive tozadenant for one year during which more safety data will be collected.

The TOZ-PD Study is for people with Parkinson’s Disease who are currently taking L-dopa and at least one other medication to control their Parkinson’s symptoms. The purpose of the study is to examine the safety and effectiveness of L-dopa in combination with a new investigational medication called tozadenant. Tozadenant acts on the part of the brain where dopamine controls movement.

The entire study will last a total of 86 weeks and require about 14 visits to your study doctor. The purpose of the study is to examine the safety and effectiveness of L-dopa in combination with a new investigational medication called tozadenant. Tozadenant acts on the part of the brain where dopamine controls movement.

ELIGIBILITY
30 to 80 years old
It has been at least 3 years since you were diagnosed with PD
You take L-dopa every day, as well as at least one other anti-PD medication
Experiencing periods when medication no longer has effects

CONTACT
Mary Louise M. Weeks, RN, BSN
mmusant@emory.edu
404-712-6999
BETTUR PD
8-week clinical trial with 6-month follow-up among those in the treatment group. This study is evaluating pelvic floor muscle exercise-based behavioral therapy to treat urinary incontinence (accidental urine loss) in persons with Parkinson disease. Participants will be randomly assigned to either the exercise-based behavioral treatment or to a behavioral control activity (involves a drawing exercise). Those in the control group will be offered the behavioral treatment after 8 weeks. After enrollment, clinic visits will occur every 2 to 4 weeks and last up to 90 minutes. All study visits are individually scheduled at the Atlanta VA Medical Center.

ELIGIBILITY
Diagnosed with Parkinson disease
Experience accidental loss of urine
Able to attend clinic appointments

CONTACT
Camille Vaughan, MD, MS
camille.vaughan@emory.edu
404-321-6111 ext 5080

BREATHE
The purpose of the study is to learn more about how the molecules in your breath can let us know about the development and progression of neurologic disorders in people. It will also determine if there is a potential for a PD-specific breath signature. For this study we are looking at people who have Parkinson’s disease, and family or community healthy controls.

ELIGIBILITY
25-85 years old
One time visit
Must be a non-smoker

CONTACT
Elaine Sperin, LPN
esperin@emory.edu
404-712-6988

DROXIDOPA
11-week clinical trial for Parkinson disease patients. Subjects will receive study drug droxidopa some of the time, and at other times, placebo. This study will assess benefit and safety of droxidopa in the treatment of Parkinson’s disease on freezing of gait, as well as possible beneficial effects on mildly cognitive impaired Parkinson disease subjects.

ELIGIBILITY
At least 30 years old
Minimum 3 months of typical freezing of gait symptoms
Mild difficulties with cognitive (thinking) abilities
On stable dose of carbidopa-Levodopa (Sinemet)

CONTACT
Cathy Wood-Siverio, MS
cwoodsi@emory.edu
404-712-6988
INTREPID
5-year clinical trial to evaluate the safety and effectiveness of Boston Scientific implantable deep brain stimulation (DBS) Vercise system for bilateral stimulation of the subthalamic nucleus (STN) as an adjunctive therapy for improving good symptom control and no troublesome dyskinesias (ON time) in advanced Parkinson disease subjects. Subjects will be randomized to either an active group or control group at baseline to the first 12 weeks. Implanted subjects in the active group will receive bilateral stimulation at therapeutic levels. Implanted subjects in the control group will not receive set therapeutic levels until after the week 12 visit. Subjects will have a 3:1 chance to be randomized to the active group.

ELIGIBILITY
22-75 years old
Persistent disabling Parkinson’s disease symptoms
Appropriate candidate for surgical procedures required for bilateral STN DBS

CONTACT
Greg Johnstone
gpjohns@emory.edu
404-778-7673
Cathy Wood-Siverio
cwoodsi@emory.edu
404-712-6988

Motor Training in PD
The purpose of this research study is to learn more about brain activity when individuals with and without Parkinson disease (PD) move their lower limbs. The investigators also want to see if and how two different types of partnered dance affect brain activity in individuals with and without PD. Testing will take place at the Atlanta VA Medical Center and at Emory University. The investigators expect to enroll about 140 people for this study over a five-year period.

ELIGIBILITY
40 years and older
Willingness to spend 1-h in a MRI scanner
Able to walk with or without an assistive device 10 feet
Best corrected/aided visual acuity in the better eye of 20/70
Absence of dementia or vascular cognitive impairment
Absence of primary memory deficits

CONTACT
Madeleine Hackney, PhD
mehackn@emory.edu
404-321-6111 ext. 5006
Aaron Bozzorg
Ariyana.bozorg@va.gov
404-321-6111 ext. 6277

Drooling Study—Open Label Study
Seeking treatment for troublesome drooling (Sialorrhea) for at least 3 months that is occurring secondarily for any disorder (Parkinson’s disease, cerebral palsy etc.)

ELIGIBILITY
In the treatment of troublesome Sialorrhea
Up to 4 treatment cycles

CONTACT
Elaine Sperin
404-712-7044
esperin@emory.edu
Freezing of Gait

3-4 visit study to evaluate freezing of gait in Parkinson’s patients. Assessments include PET scan, MRI (1 hour), motion analysis evaluation and cognitive testing assessments.

**ELIGIBILITY**
18-85 years of age
Parkinson’s disease patients willing to undergo the 4 assessments to evaluate freezing of gait.

**CONTACT**
Barb Sommerfeld, RN
404-712-6997
bsommer@emory.edu

CYNAPSUS

Why is this study important? Due to the frequency and negative impact of “OFF” states in people with Parkinson’s disease, patients need a medication that can rapidly and reliably reverse their “OFF” state in a more convenient way. The only approved drug to address this issue requires an injection, and this investigational drug is looking to see if a more convenient version of the medication can solve this problem.

Therefore, it’s important to research and evaluate additional treatment options that may offer quick and effective relief from “OFF” states, while being easy to administer. The results of this study will provide more information about the safety and effectiveness of the investigational drug. By taking part in this study, you will be making an important contribution to “OFF” state treatment research in Parkinson’s disease patients.

All study-related visits, tests, and medications will be provided to you at no cost. In addition, reimbursement for study-related time and travel may be provided if needed.

**ELIGIBILITY**
18 years of age or older
Have a medical diagnosis of Parkinson’s disease
Be receiving stable doses of levodopa/carbidopa or Rytary™
Experience at least one “OFF” state per day, with a daily total of 2 or more “OFF” hours during the day

**CONTACT**
Mary Louise M. Weeks, RN, BSN
mmusant@emory.edu
404-712-6999

SURE-PD3

Placebo controlled study to see if a treatment that raises urate levels can slow the rate of worsening in PD. Study involvement is 28 months.

**ELIGIBILITY**
PD less than 3 years since diagnosis.
Cannot be on any anti-parkinsonian medication within 60 days of Baseline or have been on medications in excess of 90 days.

**CONTACT**
Elaine Sperin, LPN
esperin@emory.edu
404-712-7044
INFUSION
Outpatient study to assess long-term safety and effectiveness of apomorphine continuous infusion in Parkinson’s disease patients who are unable to achieve adequate symptom control despite optimal treatment with noninvasive therapy.

**ELIGIBILITY**
- 30-80 years of age
- Parkinson’s disease
- On oral carbidopa/levodopa at least 4 doses/day
- Experience “off” periods of 3 or more hours/day

**CONTACT**
Cathy Wood-Siverio, MS
cwoodsi@emory.edu
404-712-6988

RESTORE
The purpose of this research study is to evaluate the effectiveness and safety of a study drug called droxidopa (also known by the trade name NORTHERATM) in people with symptomatic orthostatic hypotension. There are 15 visits over 36 weeks.

**ELIGIBILITY**
- Symptomatic orthostatic hypotension (feeling lightheaded when going from sitting to standing)
- A drop of at least 20 mmHg from sit to stand

**CONTACT**
Mary Louise Weeks, RN
mmusant@emory.edu
404-712-6999

DYSTONIA
Revance CD
Looking at a new formulation of botox that may last up to 6 months. 14 visits over 24 weeks

**ELIGIBILITY**
- Cervical dystonia with no prior injections or the last injection has been more than 6 months ago.

**CONTACT**
Mary Louise Weeks, RN, BSN
404-712-6999
mmusant@emory.edu
HUNTINGTON’S DISEASE

ENROLL
Is a longitudinal, observational, multinational study that will integrate two existing Huntington’s Disease (HD) registries, REGISTRY in Europe and COHORT in North America and Australia, while also expanding to include sites in Latin America and Asia.

ELIGIBILITY
18 years of age or older
Carriers: This group consists of individuals who carry the HD gene mutation.
Controls: This group consists of individuals who do not carry the HD expansion mutation.

CONTACT
Elaine Sperin, LPN
esperin@emory.edu
404-712-7044

NN 105
This study is looking at how well a study drug works on irritability in participants with early symptomatic HD over 12 weeks.

ELIGIBILITY
Age 18 or older
Family history of Huntington’s disease or cag >37
Available study partner to come with you to the visits
Stable medications for mood, behavior or neurologic symptoms for 30 days before baseline visit

CONTACT
Elaine Sperin, LPN
esperin@emory.edu
404-712-7044

TOURETTE’S SYNDROME

NBI-1501
Clinical trial to evaluate study medication in children (6-17 years old) with Tourette’s disease. Subjects will receive either study drug or placebo. This study will last approx. 11 weeks and assess the safety and improvement of Tourette’s disease.

ELIGIBILITY
6-17 years of age
Diagnosed w/ Tourette’s disease
Display moderate tic behavior
On stable medication

CONTACT
Jonna Seppa, BS
jseppa@emory.edu
404-727-1509
**NBI-1505**
Clinical trial to evaluate study medication in adults with Tourette's disease. Subjects will receive either study drug or placebo. This study will assess the safety and if it can improve the symptoms of Tourette's disease and last approx. 11 weeks.

**ELIGIBILITY**
- 18-64 years of age
- Diagnosed w/ Tourette's disease
- Display moderate tic behavior
- On stable medication

**CONTACT**
Jonna Seppa, BS
jseppa@emory.edu
404-727-1509

**ATAXIA**

**MOXiE**
This 2-part study will evaluate the efficacy, safety, and pharmacodynamics of RTA 408 in the treatment of patients with Friedreich’s ataxia.

Friedreich’s ataxia is impairment of antioxidative defense mechanisms, which play a major role in disease progression. Studies have demonstrated that nuclear factor erythroid-derived 2-related factor 2 (Nrf2) signaling is grossly impaired in patients with Friedreich’s ataxia. Therefore, the ability of RTA 408 to activate Nrf2 and induce antioxidant target genes is hypothesized to be therapeutic in patients with Friedreich’s ataxia.

The first part of this study will be a randomized, placebo-controlled, double-blind, dose-escalation study to evaluate the safety of RTA 408 at various doses in patients with Friedreich’s ataxia. The second part of this study is a randomized, placebo-controlled, double-blind, parallel study to evaluate the safety, efficacy, and pharmacodynamics of up to 2 dose levels of RTA 408 in patients with Friedreich’s ataxia. Eligible patients in Part 2 will be randomized 1:1:1 to receive RTA 408 (at one of 2 doses chosen from Part 1), or placebo.

Study drug will be taken for 12 weeks and there are 8 visits over 16-19 weeks (including a follow up visit 4 weeks after stopping the study drug). 1-2 of the visits may be completed by a home health nurse.

**ELIGIBILITY**
- Diagnosis of FA, confirmed by genetic testing
- Ability to pedal a recumbent bicycle
- Ambulatory for 25 ft (may use a cane or walker)
- Not currently taking antioxidant medications or supplements
- No significant cardiac dysfunction

**CONTACT**
Becky McMurray, RN
404-712-7013
rmcmurr@emory.edu
FA-COMS

This is an observational study of patients with Friedreich’s ataxia. Patients will be assessed on an annual basis to evaluate their symptoms over time. Physical exams, fine motor movements, visual acuity, cardiac evaluations (if ordered by a cardiologist) and patient questionnaires will be collected annually. Additionally, blood will be collected for DNA (baseline only) and RNA testing as well as frataxin levels and a cheek swab for frataxin levels will also be collected annually.

**ELIGIBILITY**
Diagnosis of FA by clinical exam or genetic testing

**CONTACT**
Becky McMurray, RN  
404-712-7013  
rmcmurr@emory.edu

Natural History of and Genetic Modifiers in Spinocerebellar Ataxias

Spinocerebellar ataxias (SCA) are genetic neurological diseases that cause imbalance, poor coordination, and speech difficulties. There are different kinds of SCA and this study will focus on types 1, 2, 3, and 6 (SCA 1, SCA 2, SCA 3, also known as Machado-Joseph disease and SCA 6). The diseases are rare, slowly progressive, cause increasingly severe neurological difficulties and are variable across and within genotypes. The purpose of this research study is to bring together a group of experts in the field of SCA for the purpose of learning more about the disease. Patients who enter this study will be evaluated every 6-12 months. Physical exams, fine motor testing and questionnaires will be collected. At study entry only, blood will be collected for DNA testing to confirm the diagnosis.

**ELIGIBILITY**
Diagnosis of SCA 1, 2, 3 or 6 by clinical exam or genetic testing

**CONTACT**
Becky McMurray, RN  
404-712-7013  
rmcmurr@emory.edu
FOR MORE INFORMATION:

- To make a clinical appointment call 404-778-3444
- APDA call Lynn Ross at 404-712-7091
- NPF contact Tammyjo Best at 404-712-6990
- For more information about community programs contact Cornelya Dorbin at 404-712-1416.