

NEWSLETTER

HD UPDATES


Fall 2021



Welcome to our inaugural *HD Updates* Newsletter! Fall is in the air (well, at least up in Northern Florida). While we don't quite share the colors above, the feeling is definitely on our minds as the Holiday season fast approaches. Here at the UF Health Norman Fixel Institute for Neurological Diseases, we send our heartfelt wishes for wonderful season. And to start off this season, we would like to (re-)introduce our HDSA Center of Excellence Team and our new clinic here at UF Health, opened in 2018 on the growing Williston road campus in Gainesville, FL. The new Fixel Institute is home to multiple scientists and clinicians who strive to provide state-of-art, comprehensive, and compassionate care and hope to individuals and families affected by neurological disorders like Huntington disease. Here we

provide access to a multidisciplinary team of health providers including HD experts, psychiatry, neuropsychology, neurosurgery, genetic counseling, rehabilitation specialists, dieticians, social work, and researchers under one roof. We believe that care and research should revolve around the patient, not the other way around. If not already part of our "family," we hope knowledge of our center and model of care entices you to seek us out. We are eager to help.

Sincerely,


Nikolaus McFarland, MD, PhD
Director, UF HDSA Center of Excellence



HD Experts



Nikolaus McFarland, MD, PhD

Associate Professor of Neurology, Director of the UF HDSA Center of Excellence

Dr. McFarland is an HSG Investigator and holds the Wright/Falls/Simmons Professorship in PSP/Atypical Parkinson's and is a member of the Fixel Institute and the Center for Translational Research in Neurodegenerative Disease (CTRND). In addition to HD, he directs a comprehensive multidisciplinary clinical-research program for Atypical Parkinson disorders, the CurePSP Center of Care, and the Fixel Biomarkers laboratory.

Meet the Team

Addie Patterson, DO

Assistant Professor of Neurology, Associate Residency Program Director

Dr. Patterson completed her Neurology residency and fellowship training here at UF. She received distinction as Chief Resident and was awarded the Outstanding Resident Teachers Award from the College of Medicine and the Kenneth M. Heilman Award for Excellence in Research. Her interests include teaching, HD, Parkinson's, and Palliative care.



Genetic Counseling



Lee Kugelmann, MSc, GC

Ms. Kugelmann is a relative new addition to our HD team. She received her Master's degree in Human Genetics and Genetic Counseling from Emory University's Genetic Counseling Training Program and joined the Fixel Institute team to build our neurogenetics program. She provides critical genetic counseling services.

Case Manager/Social Work

Jana Unislawski, SW

Jana has worked as Case Manager at the Fixel Institute for Neurological Diseases for nearly four years. She assists patients and families with Huntington disease, Alzheimer disease, Parkinson disease, and neuromuscular disorders. As a case manager, she refers patients and families to community assistance programs, provides information about disability, caregiver respite, local support groups, and organizes educational programs to increase community awareness.



HD Clinic Manager



Melissa Himes

Melissa is the clinic manager for the UF Fixel Institute for Neurological Sciences. She received her degree in business management from Saint Leos University and has worked in many clinical areas such as billing, payment posting, clerical duties, scheduling, and management.

Rehabilitation

Lisa Warren, OTD, MOT, OTR/L

Site Manager for Fixel Institute Rehab and Occupational Therapist

Lisa joined UF Rehabilitation at the Center for Movement Disorders Clinic in 2010 to develop a clinical outpatient rehab program for patients with movement disorders. In addition to her role as site manager, she also evaluates and treats patients in our interdisciplinary clinic and is actively involved in research projects.





Heather Simpson, MOTR/L

Occupational Therapist, Tics and Tourette's Clinical Coordinator

Heather received her Masters in Occupational Therapy from the University of Florida. She has worked in a variety of settings including outpatient neurology, inpatient physical rehabilitation and pediatric outpatient. As a part of the multidisciplinary team, Heather provides occupational therapy evaluations and treatment for patients with various movement disorders diagnoses, including HD. She also serves as clinical coordinator for the center's Tics and Tourette's program.

Alison Kraus PT, DPT, NCS

Physical Therapist

Alison Kraus graduated with a doctorate in physical therapy in 2013 from Ithaca College and a B.S. in Clinical Health Studies from Ithaca College in 2011. Alison was designated a Neurologic Certified Specialist in Physical Therapy in 2016. Since then she has worked in varying settings including acute care and outpatient neurology.



Nicole Herndon, MS, CCC-SLP

Speech Pathologist

Ms. Herndon is a graduate of Purdue University with Masters in Speech-Language Pathology. She is the primary SLP at the UF Fixel Institute where she specializes in outpatient care of persons with progressive neurological disorders. Her clinical interests include evaluation and treatment of motor speech disorders, cognitive-linguistic impairments, and swallowing dysfunction in persons with neurological disease (ie, Parkinson disease, Atypical Parkinsonism, Huntington disease, Dystonia, Essential Tremor, Ataxia, Multiple Sclerosis and ALS).



Dietary/Nutrition

Mathew Beke, MS, RDN, LDN

Mr. Beke is a Registered Dietitian Nutritionist and current Nutritional Sciences masters student at the University of Florida. As a Clinical Dietitian at Shands Lake Shore Regional Medical Center in Lake City, Florida, he has cared for the most vulnerable members of our community with compassion using the latest nutrition therapies.



Dawn Bowers, PhD

Professor of Neuropsychology

Dr. Bowers is a clinical neuropsychologist and cognitive neuroscientist. She has longstanding research and clinical expertise in cognitive and emotional changes associated with neurologic disease and aging, such as in HD. Her research focuses on emotion regulation and executive function, psychophysiological signatures of apathy and depression, and interactive effects of cognitive training and exercise in cohorts of elderly adults. She and her colleagues provide expert neuropsychological (both cognitive and emotional) evaluations and therapy in our HD patients.



Matt Farrer, PhD

Professor, Department of Neurology (Basic Science)

Dr. Farrer is a world-renowned researcher who work focuses on genetic and functional analysis of neurologic and neurodegenerative disorders. Molecular insights in disease pathogenesis provide the rationale and tools for novel therapeutic advances. With his help, we hope to build and provide an in-house HD genetic testing to patients.



News

GENERATION-HD1 Topline Results Announced!

Sponsored by Roche-Genentech, this study examined the effects of an antisense oligonucleotide (ASO) targeting the Huntington gene message, with a goal of reducing the Huntingtin protein (both mutant and normal HTT) and its effects on disease progression. The study was highly successful and enrolled nearly 900 HD participants worldwide. However, on March 21 (at 17 months) the study was prematurely stopped in all arms due to lack of benefit. GEN-EXTEND was also paused at the same time.

While the results are certainly disappointing, the study represents the richest dataset collected in HD to date. With this date researchers and clinicians, world-wide, hope to gain insights into future therapeutics for HD.

Wave Life Sciences

Around the same time, Wave Life Sciences Ltd announced similar disappointing results from their PRECISION-HD1 and PRECISION-HD2 phase 1/2 trials. These studies also tested ASO's targeting the HD gene message, but were designed to selectively reduce only the mutant Huntingtin protein (mHTT). Both WVE-120102 and WVE-120101 failed to show benefit or reductions in mHTT or NfL, a nonspecific protein associated with degeneration of the brain.


Not deterred, Wave Life Sciences has developed WVE-003 with modifications designed to enhance its potency and durability in central nervous system. A new phase 1/2 study, **SELECT-HD**, is planned to start enrolling later this year in the US. ([NCT05032196](#))

Additional Studies

uniQure (CT-AMT-130-01) is a phase I/II study, also involving "gene therapy," but which aims to reduce the Huntingtin protein using a virus to deliver sustained "knockdown" of the protein. The theoretical advantage is a one-time treatment; however, delivery of virus requires brain surgery (injection into brain areas affected by HD). Eligible patients must have early disease as brain volumes are critical for targeting. ([NCT04120493](#))

PROOFHD is a phase III trial of pridopidine in early stage HD aimed at maintaining functional independence. Enrollment has successfully completed! ([NCT04556656](#))

KINECT HD Valbenazine is a selective, orally active VMAT2 inhibitor (similar to tetrabenazine) developed by Neurocrine Biosciences, Inc. that is being investigated for the treatment of chorea associated with Huntington disease (HD). Valbenazine is FDA approved for adults with tardive dyskinesia (TD), but not yet for chorea. ([NCT04102579](#))

Enroll-HD  is a worldwide observational study that monitors how HD appears and changes over time in individuals who have HD or are at-risk. The goal of Enroll-HD is to accelerate the discovery and development of new therapeutics.

Future Studies

Branaplam is a novel, oral drug developed by Novartis that promises to mutant Huntingtin (and normal HTT protein). Stay tuned for future studies!

Calendar

Huntington Study Group (HSG)

Nov 4-6, 2021

UF Virtual HD Support Group

Third Thursday at 5:30PM (EST),

For info email:

jana.unilawski@neurology.ufl.edu

Caregiver Support Group

contact: sharon@help4hd.org

HDSA Webinars

[HDSA & ME: HD Parity Act & Advocacy](#)

Thursday, November 11, 2PM (EST)

[NYA Virtual Miniseries: Grief & Coping with the Holidays](#)

Saturday, November 13, 12PM (EST)

[HDSA Research Webinar: Ask the Scientists Anything with HDBuzz](#)

Tuesday, December 7, 12PM (EST)

More Info

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For information on studies at UF,
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