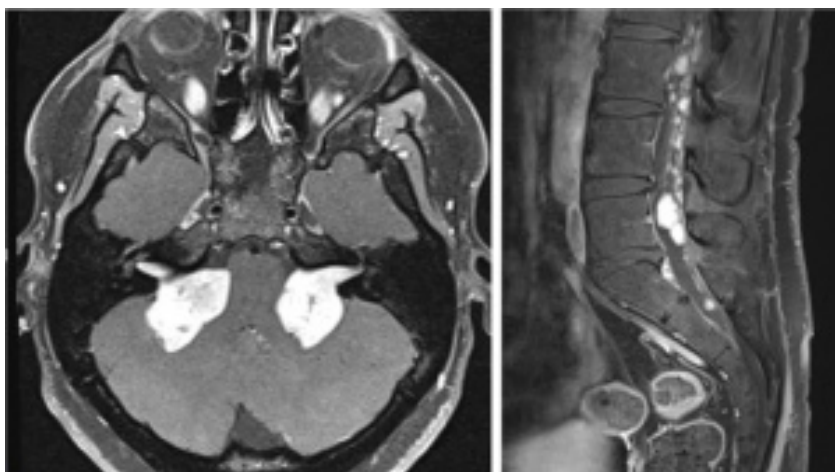




# Miller School Selected for National NF2 Tumor Clinical Trial

A University of Miami Miller School of Medicine researcher is hoping an innovative multicenter clinical trial will lead to new therapies for neurofibromatosis type 2 (NF2), a devastating disease causing multiple types of tumors involving the brain, spine, and peripheral nerves.

“Our goal is to accelerate the process of identifying effective drugs that will address the unique needs of this challenging patient population,” said Christine Dinh, M.D., associate professor of otolaryngology, otology, neurotology, and lateral skull base surgery.



Treatment of NF2 (seen here in diagnostic imaging) is challenging because patients can have multiple types of tumors that affect different nerves.



Dr. Dinh is the principal investigator for the University of Miami site in the Innovative Trial for Understanding the Impact of Targeted Therapies in Neurofibromatosis Type 2 (INTUITT-NF2). The sponsor site, Massachusetts General Hospital, is led by Scott Plotkin, M.D., Ph.D., renowned NF2 researcher, neuro-oncologist, and Giovanni Armenise – Harvard Professor of Neurology.

Other sites include Johns Hopkins Hospital, New York University Langone Medical Center, University of California Los Angeles Medical Center, and the Mayo Clinic Hospital in Rochester, Minnesota. The initiative has been supported by the Children's Tumor Foundation's visionary Synodos Consortium for NF2 research collaborative.

“Joining this very exclusive INTUITT-NF2 clinical trial gives national recognition to our NF2 team, our multidisciplinary skull base tumor program at UHealth,” said Fred F. Telischi, M.E.E., M.D., chairman of otolaryngology, professor of neurological surgery and biomedical engineering, and the James R. Chandler Chair in Otolaryngology. “This trial is so incredibly important because it is the last step of a comprehensive translational research pipeline bringing novel and new therapies from the bench to the bedside.”

## **A Challenging Condition**

About one in 25,000 individuals has NF2, which arises from a genetic mutation, Dr. Dinh said. However, treatment is challenging because NF2 patients can have multiple types of tumors that affect different nerves, resulting in hearing loss, balance problems, and other neurological sequelae such as paralysis.



“While some NF2 tumors can be removed with surgery, there is a risk of irreversible neurological injury,” Dr. Dinh said. “Others can be treated with radiation, which may slow tumor growth but doesn’t result in complete removal.”

## An Innovative Trial

Dr. Dinh and the NF2 team at University of Miami are now recruiting patients for the INTUITT-NF2 clinical trial, which involves the administration of the drug brigatinib for treatment of four different types of tumors. New drugs that show promise in preclinical models will be added to the trial on an ongoing basis, rather than starting another trial several years in the future.



INTUITT-NF2 trial team (from left): Drs. Michael Ivan, Christine Dinh, Fred Telischi, and Rita Bhatia

“Most clinical trials involve one drug and one type of tumor,” Dr. Dinh said. “With the INTUITT-NF2 trial, we can potentially fast-track new medications studied in the lab to NF2 patients and see effect in different tumor types. We will get results



more quickly with this approach, which hopefully will lead to new treatments.”

## **A Collaborative Approach**

Dr. Dinh has been studying NF2 therapies for the past six years, in collaboration with Cristina Fernandez-Valle, Ph.D., Pegasus Professor, Divisions of Neuroscience and Cancer Research at the University of Central Florida College of Medicine in Orlando. Dr. Fernandez-Valle played an important role in the initial research that led to the clinical trial.

“I’m glad INTUITT-NF2 found a home in Miami,” she said. “I have collaborated with Dr. Dinh on several grants from the National Institutes of Health, and we are working together on identifying other potential drugs for NF2 tumors.”

More than 20 clinicians, researchers, and staff make up the NF2 trial team, and members involve the departments of otolaryngology, neurosurgery, neuro-oncology, pediatric hematology/oncology, and neuroradiology.

“Having instituted an important tissue bank for NF2 tumors, Dr. Dinh and her colleagues can investigate other molecules as potential medical therapies to control schwannomas and related NF2 tumors,” Dr. Telischi added.

## **A Center for Clinical Care**

Dr. Telischi said the new clinical trial complements the substantial work in NF2 already taking place at UHealth.

“For example, we have established one of only a handful of the technically challenging Auditory Brainstem Implant (ABI) programs in the nation to help these patient have some access



to sound lost to the NF2 tumors involving nerve of hearing and balance (aka vestibular schwannomas).” He added that Dr. Dinh and her team have conducted seminal research to elucidate the effects of radiation therapy on these tumors.

“Because of these and other accomplishments, UHealth has become a regional destination for individuals and families afflicted with neurofibromatosis,” Dr. Telischi said. “Ultimately, our hope is that these clinical trials and the related basic research will lead to more effective treatments for our NF2 patients.”

Dr. Dinh’s research partners and co-investigators at the Miller School include Maria del Pilar Guillermo Prieto Eibl, M.D., assistant professor of neurology, division of neuro-oncology at Sylvester Comprehensive Cancer Center; Michael Ivan, M.D., associate professor of neurosurgery and director of research at Sylvester’s Brain Tumor Initiative; and Aditi Dhir, M.D., assistant professor of pediatrics, division of pediatric hematology/oncology.

NF2 patients with growing tumors may be eligible to participate in the study. For more information, call 305-243-7339.

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