

PARTNERING PROGRAM

Focused Ultrasound Foundation

Lead PI: Neal Kassell, MD

Organization and Team Overview

The Focused Ultrasound Foundation is a medical technology research, education and advocacy organization (501(c)(3)) created to improve the lives of millions of people with serious medical disorders by accelerating the development and adoption of focused ultrasound. Focused ultrasound is an early-stage, non-invasive therapeutic technology with the potential to transform the treatment of many medical disorders by using ultrasound energy to target tissue deep in the body without incisions or radiation.

The Foundation leverages its intellectual and financial capital to expedite the pace of research towards clinical trials, through FDA review, and ultimately to the point of focused ultrasound becoming a widely accepted standard of care. We support translational preclinical and early clinical research that validates the potential for new applications and attracts follow-on funding from industry and other philanthropies. We develop collaborative partnerships to enable us to achieve more with our resources, expanding the number of patients who benefit from our work.

The Foundation is dedicated to ensuring that focused ultrasound finds its place as a mainstream therapy for a range of neurological disorders, including Parkinson's disease, brain tumors and epilepsy as well as uterine fibroids, cancer and other life-threatening conditions within years, not decades.

Since its establishment in 2006, the Foundation has become the largest non-governmental source of funding for focused ultrasound research. We at the Foundation have identified Parkinson's disease as a major target of opportunity for focused ultrasound because the disease takes a terrible toll on patients and their families; the development pathway is defined and straightforward; and the technology is well-suited to improve quality of life in significant ways.

Opportunity Overview

The Foundation is seeking philanthropic investment and other partnerships to support our programs to advance the development and clinical adoption of focused ultrasound as a noninvasive approach for the treatment of Parkinson's disease.

The fundamental principle of focused ultrasound is analogous to using a magnifying glass to focus beams of sunlight on a single point to burn a hole in a leaf. With this procedure, multiple intersecting beams of ultrasound energy, guided by magnetic resonance (MR) imaging, converge on a target deep in the body with extreme precision and accuracy. Where each of the individual beams passes through the tissue, there is no effect. But, at the focal point, the convergence of the multiple beams results in many important biological effects, creating the possibility of treating a variety of medical disorders.

MR-guided focused ultrasound has the potential to reduce Parkinson's symptoms (tremor and/or dyskinesia) for many patients who no longer find drug therapy effective and/or tolerable. Focused ultrasound treatment is performed with the patient awake, and involves no anesthesia, incisions in the skull or insertion of electrodes into the brain. This could be an effective option for patients who are not candidates for surgery or do not wish to undergo an invasive procedure. This t



PARTNERING PROGRAM

improve motor symptoms for the patient with minimal discomfort, rapid recovery and fewer complications.

The Foundation has defined a roadmap of clinical trials to study the safety and effectiveness of focused ultrasound ablation for treating Parkinson's. We are about midway through this roadmap and seek additional funding towards completing the research steps needed to validate the potential of this treatment. A Foundation-funded pilot trial has already demonstrated the potential of focused ultrasound to treat essential tremor (ET), and a pivotal trial for FDA approval of the treatment is ongoing. Another pilot study is currently assessing the feasibility of focused ultrasound to alleviate Parkinson's tremor. Based on the promising ET results, the Foundation secured funding from the Michael J. Fox Foundation to support a pilot clinical trial using focused ultrasound to target the globus pallidus of the brain to treat Parkinson's dyskinesia, a common side effect of Parkinson's drug therapy.

Additionally, the Foundation aims to support a burgeoning field using focused ultrasound in combination with ultrasound contrast agents (microbubbles) to temporarily and reversibly make the blood brain barrier more permeable to drugs and genes. This could enable more efficient delivery of these molecules to the desired region of the brain to promote effective treatment of Parkinson's disease.

The Foundation's ability to provide further support for focused ultrasound ablation and drug delivery preclinical and clinical studies, as well as regulatory and reimbursement initiatives, and hence advance the treatment options for Parkinson's patients, will require additional philanthropic investment.

Details of MJFF Grant

The grant from MJFF provides support for focused ultrasound treatment of a total of 15 Parkinson's patients with dyskinesia, divided over three sites around the world.

The effectiveness of drug therapy in alleviating Parkinson's symptoms often diminishes over time, and side effects such as dyskinesia (involuntary movements) can become prevalent and burdensome. The destruction of a small volume of tissue near the center of the brain (pallidotomy) can often reduce the dyskinesia, yet typical procedures require surgery or a high dose of focused radiation. This study will investigate the safety and effectiveness of MR-guided focused ultrasound as a completely noninvasive method of pallidotomy.

Results and Potential Next Steps

The MJFF-funded Parkinson's dyskinesia pilot trial is currently ongoing with several patients treated to date. Results will be available once the patients have been assessed at 12 months post treatment. We expect to see significant, immediate and durable improvement in patient symptoms. We also expect to see very minimal and non-significant complications following focused ultrasound, comparable to other ablative techniques such as radiofrequency ablation, with no infection, bleeding or neurological deficits. If the results of this pilot trial are encouraging, it will lead to a larger pivotal trial to gain full regulatory approval and reimbursement.

Intellectual Property Status

The Foundation itself does not hold IP, yet we can facilitate connection to and partnerships with the device manufacturers and universities involved in the development of focused ultrasound ablation and drug delivery treatments who do hold the IP.

