



FOCUSED ULTRASOUND FOUNDATION

2018 SUMMER REPORT

Highlights

- Foundation recognized as one of America's 10 Best Medical Research Organizations
- Alzheimer's disease clinical trial results presented at the Alzheimer's Association International Conference, published in *Nature Communications*
- Parkinson's dyskinesia clinical trial results published in *Journal of Neurosurgery*
- Foundation and Cancer Research Institute announce first co-funded grant award
- Governor of Virginia visits the Foundation
- 2018 State of the Field report published
- Record number of presentations to be featured at 6th International Symposium on Focused Ultrasound

Dear Friends,

The Foundation was honored to be recognized by Charity Navigator as one of [America's 10 Best Medical Research Organizations](#). This list highlights charities committed to funding cutting-edge research, finding breakthroughs for a spectrum of conditions and diseases, and dedicated to using donor funds wisely in their journey to find a cure. The field of focused ultrasound continues to evolve and mature at an accelerated pace, and we are transitioning rapidly from research activity to commercialization. The numbers tell the story: in just ten years, we have moved from three known mechanisms of action to 18; from three focused ultrasound indications to more than 100 and counting; from only five focused ultrasound manufacturers to more than 60. Worldwide, there are more than 300 clinical and preclinical focused ultrasound research sites and more than 20 regulatory approvals, of which five are in the US, and three are now receiving insurance reimbursement. We are also seeing awareness increasing across target audiences as media coverage shifts from industry publications to include mainstream, high-circulation outlets such as the Associated Press, *Forbes Magazine*, the Washington Post, *Huffington Post*, and NPR. But much work remains to be done.

Thank you for your continued support.

Be well,

Neal F. Kassell, MD



Creating Knowledge: Research Milestones

EXTERNAL RESEARCH AWARDS PROGRAM

The Foundation funded two new external research awards projects: **“Drug Delivery Strategies Using Drug-loaded Extracellular Vesicles Generated by Microbubble-assisted Ultrasound”** (Yuana Yuana, PhD, University Medical Center Utrecht, Netherlands) and **“Noninvasive Focused Ultrasound Ablation for the Treatment of Cholangiocarcinoma Liver Tumors”** (Eli Vlasisavljevich, PhD, Virginia Polytechnic Institute and State University). The total number of funded projects to date is now 69; approximately 30 percent of reviewed proposals have been funded, and 80 percent of funded projects have been completed. Of the completed projects, 96 percent have been presented at scientific meetings and 71 percent have been published in peer-review journals. Follow-on funding continues to increase and is now more than eight dollars to every one dollar of original Foundation support.

Completed external projects, cumulative



\$4.8M

Funding provided for completed projects



\$38.8M

Follow-on funding



x8

Factor by which the Foundation leverages donors' contributions

BRAIN PROGRAM

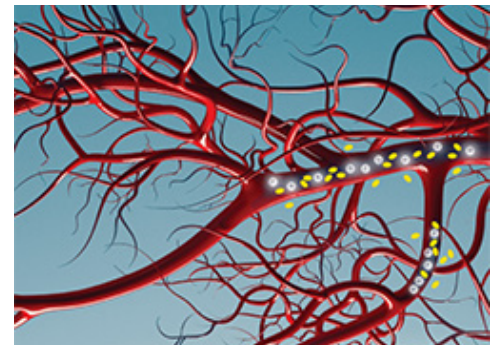
Preclinical Laboratory Studies

Three new brain preclinical studies have been initiated related to the use of focused ultrasound for the following: microbubble-enhanced mechanical ablation of brain tumors with focused ultrasound; focused ultrasound-mediated delivery of mitochondria or stem cells to improve outcomes following stroke; and treatment of cavernous malformations in the brain with focused ultrasound-activated drugs.

Clinical Trials

Alzheimer's Disease

Results of a **landmark trial at Sunnybrook Health Sciences Centre in Toronto, Canada** using focused ultrasound to temporarily open the blood-brain barrier in Alzheimer's patients were published in *Nature Communications* and presented at the Alzheimer's Association International Conference (AAIC) in Chicago, Illinois. The trial is the first small but critically important step in a process that could potentially lead to a novel approach in treating Alzheimer's by delivering drugs directly to the brain.





Creating Knowledge: Research Milestones

BRAIN PROGRAM/Clinical Trials (Cont.)

Depression

In a trial investigating the use of focused ultrasound to treat **medicine-refractory severe depression** at Sunnybrook Health Sciences Centre, four of six patients have been treated. This trial is targeting the anterior limb of the internal capsule in the brain.

Dystonia

In a **trial of focused ultrasound to treat dystonia of the hand** (also called musician's dystonia) at Tokyo Women's Medical University, Japan, all ten patients have been treated. Initial outcomes are encouraging, and follow-up patient evaluations are being completed.

Epilepsy

In a **trial of focused ultrasound to treat hypothalamic hamartoma** at the University of Virginia (UVA), the first of ten patients has been treated. A second trial using focused ultrasound to interrupt the progression of focal to complete seizures has begun recruiting patients at Ohio State University.

Obsessive-Compulsive Disorder (OCD)

In a **trial using focused ultrasound to treat medication-refractory OCD** at Sunnybrook Health Sciences Centre, six of ten patients have been treated.

Parkinson's Disease – Dyskinesia

Results of a ten-patient clinical trial using focused ultrasound to alleviate dyskinesia (involuntary movements) due to Parkinson's or the medicines that treat the disease were published in the *Journal of Neurosurgery*. In this trial at Yonsei University Medical Center's Brain Research Institute in Seoul, Korea, which is led by Dr. Jin Woo Chang, the target treatment area was the nucleus in the brain at the internal portion of the globus pallidus. There was significant improvement in the movement disorder measured at six months and one year, as well as improved quality of life. No patients experienced persistent adverse events.

A follow-up to this trial, launched in the United States, has treated six of 116 patients. Spearheaded by the University of Maryland, the goal of this **multicenter pivotal trial** is to enable FDA approval of focused ultrasound to treat Parkinson's. The target treated in this trial is the globus pallidus.

Another **trial using focused ultrasound** in the treatment of Parkinson's dyskinesia is ongoing at HM Hospital Universitario Puerta Del Sur. CINAC in Madrid, Spain; 23 of 40 patients have been treated. The target treated in this trial is the subthalamic nucleus.

At Tokyo Women's Medical University in Japan, **two of ten patients have been treated in a trial** using focused ultrasound to ablate the nerve pathways (tractotomy) that carry undesired nerve signals as a result of Parkinson's dyskinesia. The target treatment area in this trial is the pallido-thalamic tract.

Neuropathic Pain

In a **trial investigating focused ultrasound** to treat chronic craniofacial neuropathic pain at UVA, two of ten patients have been treated.





Creating Knowledge: Research Milestones

BODY PROGRAM

Pancreatic Cancer

Organization of the pancreatic cancer registry is progressing, with several international sites now part of the regulatory approval process. The registry will help to define the role of focused ultrasound in the treatment of this deadly disease. The first of eight sites is expected to begin enrolling patients in the fourth quarter of 2018.

Osteoid Osteoma

In a clinical trial of 56 patients comparing focused ultrasound to radiofrequency ablation in treating osteoid osteoma at the University of California, San Francisco and Stanford University, four additional patients have been treated, bringing the total number treated to eight.

Five Canadian patients have been enrolled in the **osteoid osteoma pediatric registry**. Additional sites worldwide are working through the regulatory process to join this 30-site international registry, which will collect data from 900 children treated for osteoid osteomas with nonsurgical methods. More than 200 of the participants have undergone focused ultrasound therapy.

Facetogenic Low Back Pain

In a clinical trial of focused ultrasound to treat facetogenic low back pain at McGill University in Montreal, Canada, seven of ten patients have been treated.

Knee Osteoarthritis

In a clinical trial using focused ultrasound to reduce osteoarthritic knee pain in patients at Kochi Medical School in Japan, 20 patients have been treated. Follow-up evaluations have been completed, and results are encouraging.

CANCER IMMUNOTHERAPY

The Cancer Research Institute (CRI) and the Foundation jointly selected a **focused ultrasound-enhanced cancer immunotherapy project** for co-funding. This is the first grant awarded since the two organizations established their formal cancer immunotherapy partnership. The recipient, Gavin Dunn, MD, PhD, a neurosurgeon at Washington University in St. Louis, will investigate which types of focused ultrasound best increase tumor exposure to the immune system and how soundwaves affect the immune cells within the tumor. The end goal is to use the data to refine clinical trials that combine ultrasound and specific immune-based treatments.



Gavin Dunn, MD, PhD,
Washington University
in St. Louis

VETERINARY PROGRAM

At the Oklahoma State University Center for Veterinary Health Sciences, a study investigating focused ultrasound to speed wound healing has enrolled three canine patients. Focused ultrasound is being assessed as a means of enhancing delivery of an antimicrobial agent (or drug) to treat hygromas, a condition where repeated pressure on a bony joint produces significant swelling.

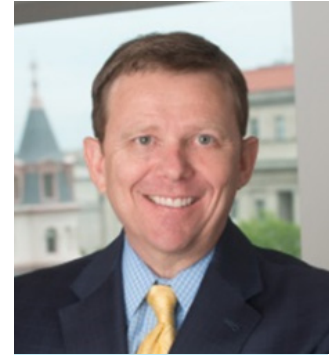
Convening the Community

2018 SYMPOSIUM



The **6th International Symposium on Focused Ultrasound** will be held October 21-25 in Reston Virginia. A record number of papers will be presented by researchers from more than 24 countries, including more than 130 oral presentations. An additional 100 researchers will take part in the poster session. Keynote and special lecture speakers include technology innovator Gary Shapiro, President and CEO of the Consumer Technology Association; healthcare advocate and policy expert, AdvaMed CEO Scott Whitaker; and Openwater founder and CEO Dr. Mary Lou Jepsen, who has been included in the “TIME 100”, the magazine’s list of the world’s 100 most influential people, and CNN’s list of top-ten thinkers, the “CNN 10”.

Registration is currently open.



AdvaMed CEO
Scott Whitaker

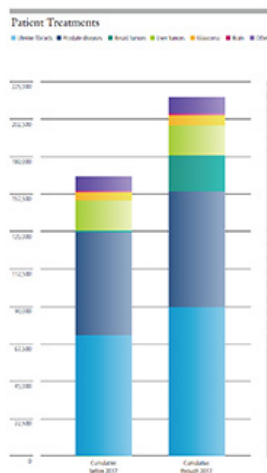
Fostering Collaboration

PARTNERSHIPS

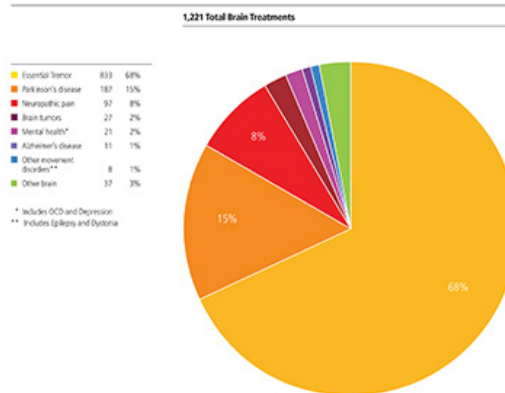
The Medical Imaging & Technology Alliance (MITA) and the Foundation have **formed a partnership** to raise awareness of focused ultrasound technology among policymakers, insurance payors, and medical specialty societies; advocate for common goals related to commercialization; and advance the field towards clinical adoption. MITA, a division of the National Electrical Manufacturers Association, is the collective voice of medical imaging equipment manufacturers, innovators, and product developers.

STATE OF THE FIELD 2018

The **State of the Field 2018 report**, published in June, documents the rapid growth in the number of indications under investigation for treatment with focused ultrasound, with well over 100 indications on the research and regulatory development landscape. The report counts more than 200,000 patients treated with focused ultrasound worldwide. Additionally, this year’s publication includes new charts and data summaries delineating research sites working with specific mechanisms of action and technical aspects of focused ultrasound.



Cumulative Brain Treatments by Indication



Cultivating the Next Generation

INTERNS AND GLOBAL SCHOLARS

The Foundation's **summer internship program** welcomed seven students who pursued a wide variety of projects, from graphic design and the newly launched FUS Partners Program to technical projects including 3D-printed acoustic lenses and software tools to automate and simplify research.

Additionally, the Foundation selected 20 Global Scholars this year, working at 14 academic institutions across five countries. Of note, 17 of the 41 2017/2018 Global Scholars had abstracts accepted for the upcoming Symposium on Focused Ultrasound.

Earlier this year, the Foundation's internship program – which encompasses both local and global interns – was named in memory of Board of Directors member Charles Steger, PhD. The Foundation's summer technical internships are generously funded by the Claude Moore Charitable Foundation.



2018 Summer Interns

Increasing Awareness

VIRGINIA GOVERNOR NORTHAM VISITS THE FOUNDATION



Ralph Northam, governor of Virginia and pediatric neurologist, **visited the Foundation in June** and met with staff, members of the Foundation's Board and Council, and donors. Also in attendance were Virginia House of Delegates Leader David Toscano, representatives from the University of Virginia, and members of the local media.

“This meeting was a great opportunity to update the governor on the latest developments in focused ultrasound and share what we feel are the next big things happening in the field,” said Dr. Neal Kassell. “With the governor's background in medicine and his passion for bettering the lives of those in Virginia, it is easy to see how a strong push toward focused ultrasound programs in the Commonwealth is a win-win for everyone.”

COMMUNICATIONS

The Foundation's communications team has disseminated a record number of press releases and newsletter stories in recent months, resulting in media coverage by more than a dozen news organizations including CNBC, *South China Morning Post*, and *American Veterinarian*. The presentation of first-in-human Alzheimer's trial data at the AAIC in Chicago garnered coverage by the Associated Press, the *Washington Post*, and hundreds of others. Additionally, more than 10,100 individuals now receive Foundation newsletters, and more than 22,000 additional people stay in touch with the Foundation and industry news via our major news announcements and progress reports.



Foundation Officers interview Nir Lipsman, MD, PhD at AAIC in Chicago



Aggregating and Sharing Knowledge

WEBSITE AND SOCIAL MEDIA

The Foundation’s website is the most comprehensive, up-to-date source of information for the field of focused ultrasound, and website traffic continues to grow. We are engaged in a new coordinated social media campaign to reach more people and engage them more thoughtfully and intentionally. Our audiences are expanding across all channels: Facebook, Twitter, YouTube, LinkedIn, Medium, and Instagram. Please be sure to follow us on these social media platforms.



SEMINAR SERIES

In May, the Foundation hosted a **webinar with Philip Bourne, PhD**, Stephenson Chair of Data Science and Director of the Data Science Institute at the University of Virginia, exploring data science in biomedicine. Bourne addressed successes and failures thus far, whether or not we are seeing a paradigm shift, and what organizations and researchers can do. The webinar was broadcast via Facebook Live.

Finances

FOUNDATION RECOGNIZED AS ONE OF 10 BEST MEDICAL RESEARCH ORGANIZATIONS

The Foundation was included in Charity Navigator’s recent list of **America’s 10 Best Medical Research Organizations**. The site notes, “these charities are committed to funding cutting-edge research and finding breakthroughs for a spectrum of conditions and diseases. They are also dedicated to using donors’ funds wisely in their journey to find a cure.” “We are honored to be recognized as one of the country’s best medical research organizations,” said Foundation chairman Neal F. Kassell, MD. “The success of the Foundation is related to our ability to amalgamate the hard work of our team, Board of Directors, Council members, and donors into an awesome force that is driving the focused ultrasound field forward.” **Charity Navigator** is the nation’s largest independent charity evaluator and leading donor advocate, and the Foundation has earned four stars, the highest rating awarded by the site.

FUNDRAISING

This summer the Foundation engaged a number of new high-profile donors and raised more than \$1.5 million in new cash and more than \$500,000 in pledges (from May 18 to August 31). The cash goal of \$1 million was exceeded; but we fell short of our \$1 million goal of pledges payable in 2019.

All donations are still being matched dollar-for-dollar under the terms of a generous pledge agreement funded by an anonymous donor. As of August 31, we have raised approximately \$7.8 million toward the match and expect to meet the match by the end of 2018. We hope you will consider helping us meet this goal, appreciating that your gift will be doubled.

FOUNDATION ORGANIZATION



In June the Foundation welcomed former CEO of Norfolk Southern and Amtrak **Charles W. “Wick” Moorman IV to the Board of Directors**. “His superb leadership ability and extensive operating experience, coupled with an innate understanding of technology, will complement the other members,” said Dr. Neal Kassell. In addition to the Foundation Board, Moorman serves on the Board of Directors of Chevron Corporation, Duke Energy, the Georgia Tech Foundation, the Nature Conservancy of Virginia, and Oracle.



If you would like additional information or want to discuss how you can support our mission, please contact:

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