Department of Biomedical Engineering
UNIVERSITY OF WISCONSIN–MADISON

Spring 2026 Seminar Series

SPEAKER CHANGE

Gene Therapy for Rare Neurodegenerative Diseases: RARE will get us there

Wally Block, PhD
Professor
Department of Biomedical Engineering
Department of Medical Physics
University of Wisconsin-Madison

Rare monogenic neurological diseases affect about 0.5% of Americans at birth and are estimated to account for up to 40% of the workload in hospital pediatric practice. Current drug delivery methods struggle to overcome the Blood Brain Barrier (BBB), with 98% of small molecule drugs and 99% of monoclonal antibody therapies failing to cross the BBB. This barrier, while protecting the brain, creates significant challenges for drug delivery and patient treatment.

Convection Enhanced Delivery (CED) is emerging as a promising solution, circumventing the BBB with direct, minimally invasive catheter-based infusion. Current CED surgical protocols distribute gene therapies are transforming the outlook for Huntington's disease where only perhaps 1% of the brain needs to be altered genetically. In most rare neurodegenerative diseases however, much larger volumes of the brain require will require treatment.

New government initiatives like ARPA-H THRIVE are making a 9-figure investment in genetic correction, many of which will be focused on genetic correction for rare brain disorders. This talk will provide an overview of the biophysics technology being developed across a consortium centered at UW-Madison to get from 1% to 100% of brain coverage. The talk will present an argument why solving rare diseases will accelerate efforts to treat genetic approaches to much higher prevalence diseases such as Parkinson's or Alzheimers.

ABOUT the SPEAKER

I worked as a systems engineer on some of the first commercial magnetic resonance imaging (MRI) scanners at GE Healthcare. I then returned for his doctorate at Stanford, specializing in fast acquisition MR methodology. As a faculty member in Biomedical Engineering at UW-Madison since the year 2000, my laboratory develops, implements, and analyzes novel MRI acquisition, reconstruction, and visualization strategies that dramatically shorten and simplify MRI. My lab's primary focus today is in using MRI to guide transformative, minimally invasive treatments in the brain, primarily using intraparenchymal delivery to bypass the blood brain barrier. This work utilizes aspects of signal processing, machine learning, mechanical engineering, biophysics, and image processing.

Monday, February 16 at Noon
1003 Engineering Centers (Tong Auditorium)