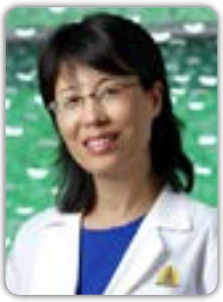

Wenzhen Duan

Johns Hopkins University, USA

Therapeutic target and biomarker development in Huntington's disease

Most neurodegenerative diseases, including Alzheimer's, Parkinson's, and Huntington's disease (HD), have converging pathogenesis, such as formation of abnormal protein aggregates and mitochondrial dysfunction in the nervous system. Unfortunately, despite tremendous efforts by many scientists and increasing knowledge about disease mechanisms, we still lack disease-modifying treatments for any of these diseases. While these diseases affect different areas of the brain and are distinct at the cellular and molecular levels, they share underlying similarities. Thus, development of treatment for any one disease has the potential to accelerate the path to treatment for related neurodegenerative diseases. Research into potential therapies for HD is particularly attractive because it is a genetically homogeneous disease for which numerous well-established animal and cell-based models exist. HD is an autosomal dominant disease caused by a CAG repeat expansion in exon 1 of the huntingtin gene. The HD gene encodes the protein huntingtin (Htt), whose polyglutamine expansion is believed to mediate the cytotoxic effects of HD. Therefore, HD serves a model for both neurodegenerative diseases and polyglutamine diseases. Our laboratory aims to develop therapeutic targets and biomarkers for neurodegenerative diseases, with a focus on HD. I will discuss our recently identified therapeutic targets as well as biomarkers which have high potentials to be translated into clinical application. Drug discovery has been revolutionized in the past decade. However, despite technological advances because of substantial investment, the number of new drug approvals remains stagnant and the cost of bringing a drug to market is higher than ever. This highlights the persistence of a model of drug development that has not adapted to changes in science and public perception of drug companies. I will use Huntington's disease as an example to discuss the challenges and opportunities in the translational neurobiology and drug development.

Speaker Biography

Wenzhen Duan is an Associate Professor of Psychiatry and Neuroscience, Johns Hopkins University School of Medicine, has completed her PhD in Neuropharmacology at Peking Union Medical University, China in 1998. She is the current Director of Laboratory of Translational Neurobiology at Johns Hopkins University School of Medicine. She is internationally known for her work on translational research in neurodegenerative diseases, particularly in Huntington's disease. She is a pioneer in developing multimodal micro-MRI biomarkers for preclinical studies of neuroprotective therapeutics. Her laboratory identified key molecular targets for therapeutic intervention for HD and conducted preclinical therapeutic trials. She has published over 70 research articles and reviews. She is recognized by national and international organizations and serves in Review Committee for science foundations in the USA such as NIH as well as outside US, including UK, Austria, Swiss, Italian, and other European science foundations.

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