UC Davis MIND Institute Distinguished Lecturer Series

January 12, 2021 | 11:30-12:45p.m.

Randi J. Hagerman, M.D.

The Fragile X Spectrum and new Targeted Treatments for FXS and ASD

Presentation

Fragile X Spectrum Disorders (FXSD) include Fragile X syndrome (FXS), the most common single gene cause of autism spectrum disorder (ASD) and the most common heritable cause of intellectual disability, and several premutation disorders characterized by the premutation of *FMR1*. Premutation disorders have become more widely recognized over the last decade and they include the most common genetic cause of early ovarian failure and infertility, FXPOI, the fragile X-associated neuropsychiatric disorders (FXAND) and a neurodegenerative disorder of older carriers called the fragile X-associated tremor ataxia syndrome (FXTAS).

The Clinical Trials Program at the MIND Institute has led many trials of targeted treatments for FXS and other conditions and the commonalities across disorders will be emphasized because one medication can be beneficial for several disorders. The neurobiology of why cannabidiol (CBD) is helpful for FXS and for ASD in addition to the benefits of metformin and bumetanide will be reviewed. The need for treatments that improve mitochondrial function, such as Anavex 2-73 can make a difference for both neurodevelopmental disorders and neurodegenerative disorders including Rett syndrome, FXS, Parkinson Disease, Alzheimer Disease and FXTAS. Future new treatments for FXSD will be reviewed including genetic interventions.

About the Speaker

Randi Hagerman is a developmental and behavioral pediatrician, a Distinguished Professor of Pediatrics and the Medical Director of the MIND Institute at UC Davis. She is internationally recognized as both a clinician and researcher in the fragile X field. Hagerman received her M.D. from Stanford University, where she also carried out her Pediatric residency. She completed a Fellowship in Learning and Disabilities and Ambulatory Pediatrics at UC San Diego, then led Developmental and Behavioral Pediatrics at the University of Colorado for 20 years. She co-founded the National Fragile X Foundation in 1984. In 2000, Hagerman joined the MIND Institute. There, she and her team discovered the Fragile X-associated Tremor/Ataxia Syndrome (FXTAS). Hagerman's research involves genotype-phenotype correlations in fragile X and she collaborates on this research with her husband, Paul Hagerman. Hagerman has written over 400 peer-reviewed articles and numerous book chapters on neurodevelopmental disorders, as well as edited several books on fragile X including a 2020 book edited by Hagerman and Hagerman titled Fragile X Syndrome and Premutation Disorders published by MacKeith press, London. Hagerman has received numerous awards for her research including the Jerrett Cole Award from the National Fragile X Foundation for unselfish dedication in working with fragile X children and adults, the Bonfils-Stanton Foundation Award for Science including Medicine, the IASSID Distinguished Achievement Award for Scientific Literature, the 2005 and 2014 Distinguished Scholarly Public Service Award from UC Davis. In 2004, to honor both Randi and Paul Hagerman in recognition of their work in FXTAS, the National Fragile X Foundation established the Hagerman Award that recognizes research accomplishments in the field of FXTAS and is given at the bi-annual International Conference on Fragile X. In 2008, the National Fragile X Foundation again honored Hagerman with a Lifetime Achievement Award. In 2014 she received the C Anderson Aldrich Award for Outstanding Career Achievement from the AAP; in 2014 the International Sisley-Jerome Lejeune Award in France and in 2017 the Emil M. Mrak International Award from the Cal Aggie Alumni Association. Hagerman has worked internationally to establish fragile X clinical programs and research programs throughout the world. She is currently carrying out multiple targeted treatment trials in FXS including a CBD trial, a metformin international trial, and an AFQ056 NeuroNext study in addition to an Anavex 2-73 trial in Rett syndrome.

Event information

To view this presentation, please register for the ZOOM webinar:

https://zoom.us/webinar/register/WN_G3QyMfreRbaUrgTuasLBfQ

All questions to the speaker will be answered through the Q&A function after the presentation is completed.

This webinar will also be recorded and added to the MIND Institute's YouTube channel at a later date.

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