WEBINARS

Webinar: Mark Bear discusses fragile X syndrome, from bench to bedside

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Here's how Bear describes what he will discuss in this webinar:

"Genetics plays a major role in autism and associated intellectual disability, but many kinds of genetic variation can lead to autism. A critical question for the field is to what degree these varied etiologies converge onto shared processes. Research in animals suggests that one such mechanism is altered synaptic regulation of protein synthesis in neurons. This has been most clearly established in fragile X syndrome, a condition caused by mutations in the gene FMR1. Studies show that protein synthesis is elevated in fragile X animal models — and suggest that treatments that tamp down protein synthesis ease fragile X traits. The same approach seems to ease autism features in other genetic mouse models, suggesting that a treatment that works for fragile X might also work for other developmental conditions.

"However, fragile X treatments that showed promise in mice — namely mGluR5 inhibitors and the GABA-B receptor agonist R-baclofen — failed to produce a significant benefit in clinical trials on the chosen primary endpoints. These first human trials faced numerous unknowns, including what the dosage should be, how long to give the treatment and whether the chosen endpoints are valid. In this webinar, I will discuss the need to apply the knowledge gained in these pioneering efforts to future clinical trials. By improving trial design and drug selection, we can be optimistic about drug development for fragile X syndrome."