SCIENTIFIC SEMINAR

Sonia Vallabh

Broad Institute of MIT and Harvard

How to prevent prion disease in our lifetimes

Prion disease is a rapidly progressive neurodegenerative disease that is universally fatal and currently untreatable. Pathogenesis is driven by the misfolding of a single causal protein, the prion protein (PrP, encoded by PRNP), into a self-propagating conformer. Genetic proofs of concept have long shown that reduction of PrP gene dosage is protective against prion disease, with full knockout conferring complete protection. This talk will describe efforts to develop PrP-lowering therapeutics across a range of paradigms: oligonucleotide and gene therapies, encompassing both established and novel modalities, developed through both pharmaceutical collaborations and academic-led efforts. Further, regardless of drug type, the central clinical challenge in this rapidly progressive dementia is treating individuals at risk preventively in order to preserve full quality of life. To this end we will also discuss biomarker development, natural history and preclinical studies, and how this work is guiding our clinical plans. This talk will be delivered from a patient-scientist perspective as Sonia Vallabh is both a prion disease researcher and herself a carrier of a high-penetrance pathogenic PRNP variant causal for genetic prion disease.

CIC bioGUNE
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EXCELENCIA SEVERO OCHOA

Monday
February 24
Atrio 800
12.00H

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