



PEPTALK

The Protein Science Week

JANUARY 18-22
SAN DIEGO 2016

Cambridge Healthtech Institute's Mary Ruberry spoke with Dr. Ruben Boado, Vice President of R&D at ArmaGen, Inc. about engineering enzyme therapeutics to penetrate the blood-brain barrier, the topic of his upcoming keynote presentation at the 12 Annual "Recombinant Protein Therapeutics" conference at PepTalk in San Diego.

MR: *How do you engineer enzyme therapeutics to penetrate the blood-brain barrier (BBB)?*

RB: Enzyme therapeutics are reengineered in a form of human IgG fusion proteins, wherein the human IgG is the transport domain of the fusion protein and targets BBB endogenous protein transport systems, like insulin and transferrin, to induce receptor-mediated transcytosis across the BBB and into the brain interstitial fluid and into brain cells. The therapeutic domain of the fusion protein is an enzyme for the treatment of lysosomal storage disorders of the brain.

MR: *Where do these efforts stand?*

RB: The genetic engineering and validation of IgG fusion proteins with iduronidase (IDUA) and iduronate-2-sulfatase (IDS) have been completed for the potential treatment of Hurler MPSI and Hunter MPSII, respectively. Toxicological studies in primates were also completed for these two applications.

MR: *What is the target of the First-in-human clinical trials that are in progress?*

RB: First-in-human clinical trials for both Hurler MPSI and Hunter MPSII are in progress in the US and in ex-US sites. The goal is to measure safety and peripheral pharmacodynamics in adult patients of the disease.

MR: *What will your Keynote Presentation reveal next January at PepTalk?*

RB: The Keynote Presentation will present an update on the development of human IgG fusion proteins for the treatment of lysosomal storage disorders.



Ruben Boado, Ph.D., Vice President of R&D at ArmaGen, Inc. will give the

Opening Keynote address at the upcoming 12th Annual Recombinant Protein Therapeutics conference in San Diego January 18, 2016.

Dr. Boado co-founded ArmaGen in 2004, following more than 25 years of academic experience in fields of molecular and cell biology of the BBB, and drug delivery to the brain. His leadership and expertise have been instrumental in the development of ArmaGen's extensive product pipeline, including potential biotherapeutic treatments for mucopolysaccharidosis, stroke, Alzheimer's disease and Parkinson's disease. Dr. Boado was the principal investigator in a number of Small Business Innovation Research (SBIR) programs granted by the National Institutes of Health to ArmaGen. Dr. Boado is also a co-inventor of the intellectual property that supports ArmaGen's pipeline. He was previously professor of Medicine at UCLA, and has published over 195 scientific peer-reviewed publications and book chapters related to the BBB.

To learn more about Dr. Boado's presentation and 12th Annual Recombinant Protein Therapeutics conference, visit
CHI-PepTalk.com/recombinant-protein-therapeutics