



Christian Mueller is an Associate Professor in the Department of Pediatrics and in the Horae Gene Therapy Center at the University of Massachusetts Medical School.

Dr Mueller holds a PhD in Genetics from the University of Florida. He subsequently completed his postdoctoral work as a Parker B Francis Fellow while working on his Master in Clinical Investigation at the University of Massachusetts Medical School. In 2011 he opened the Mueller Lab for Gene Therapy in the Horae Gene Therapy Center. The main focus of his lab is recombinant adeno-associated virus (rAAV)-mediated gene therapy for rare genetic diseases such as alpha-one antitrypsin deficiency (AATD), amyotrophic lateral sclerosis (ALS), and Huntington's disease (HD). Dr Mueller is also investigating adaptive immune responses to AAV capsid and is currently collaborating on various AAV clinical trials to characterize these immune responses in patients. One of these collaborations has resulted in the appreciation that human patients treated with intra-muscular injections of rAAV1 can elicit capsid specific T-regulatory immune responses to the capsid. Dr Mueller also pioneered the use of rAAV to deliver artificial miRNAs to silence genes with toxic gain-of-function products. Some of this research culminated with the development of a dual function rAAV vector that is able to augment normal alpha-one antitrypsin (AAT) while simultaneously silencing mutant AAT, as well as rAAV vectors for silencing SOD1, C9ORF72 and HTT as therapies for ALS and HD. Dr Mueller is currently translating the dual-function vector for AATD as well as those for ALS into the clinic.