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Introduction

Alpha synuclein (aSyn) plays an important role in PD patients and mutations/multiplications in the gene leading to PD. Commonly used rodent models over express wildtype and mutant forms of aSyn and have been helpful in understanding molecular mechanisms and the role of aSyn in PD pathogenesis. However, the lack of comparable phenotypes makes it challenging to reproduce PD in animal models. Therefore, it is important to have preclinical tools that best suits the scientific questions we want to answer to further our understanding of aSyn biology to development of resources for PD research and drug development communities that endeavors to provide researchers with easy access to rigorously validated preclinical tools for their studies. Here, we present novel viral vectors from the MJFF preclinical tools for their studies. Here, we present novel viral vectors from the MJFF preclinical tools for their studies.



1. Validation and Characterization of Human AAV-aSyn as a Model of Nigrostriatal Degeneration AAV5-Null vector @ 100% AAV5-aSyn @ 100% Vector Optimization Svn1 CB/ C/S1 PGK Naïve Injected side Naïve Injected side AAV5-aSyn @ 100% C/S1 PGK Syn1 (2.3E+12) Syn21 shipped to users; ddPCR-matched at 2.4E12. 2. Functional Validation of AAV1/2 Human A53T aSyn and AAV1/2 Empty Vectors D Forepaw Use Asymmetry AAV1/2-CMV/CBA-AAV1/2-CMV/CBA-Dopamine Cylinder Test **Null/Empty-**WPRE-BGH-polyA Human-A53T-alpha-synuclin-



(From Skull to Bregma)



Plasmid maps for AAV1/2 A53T aSyn (2A) and AAV1/2 Empty Vector Control (2B). CMV/CBA promoter consisting of chicken Beta-actin promoter hybridized with the CMV immediate early enhance sequence and is highly efficient in mouse tissue The Woodchuck post-transcriptional types. regulatory element (WPRE) and the presence of a bovine growth hormone (BGH) polyadenylation sequence ensures high transcription following transduction. (2C) Injection paradigm for wild type Sprague rats; sac'd 6 weeks post injection.

The Michael J. Fox Foundation's Development and Distribution of Novel Alpha-Synuclein Viral Vectors to Study Parkinson's disease.



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