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Leveraging CRISPR-Cas9 for neurodegenerative disease research

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The development of CRISPR-Cas9 gene editing and induced pluripotent stem cell (iPSC) technology have revolutionized the investigation of neurodegenerative disease. Our research leverages these advancements to investigate human microglia biology and develop novel therapeutic strategies. As the innate immune cell of the brain, microglia are highly implicated across several neurodegenerative disorders like Alzheimer's Disease (AD). In our lab, we have utilized CRISPR-editing to investigate the impact of AD-variants like the *PLCG2 P522R* mutation on iPSC-derived microglia in chimeric animal models. Second, we have deployed genetic-correction of pathogenic mutations to examine the therapeutic potential for microglia transplantation for the treatment of primary microgliopathies like CSF1R-related leukoencephalopathy. Lastly, we recently harnessed CRISPR-editing to engineer iPSC-microglia as "living drugs" capable of delivering disease-modifying payloads from within the CNS. Collectively, these studies highlight the transformative power of CRISPR-Cas9 gene editing as both a fundamental research tool and a platform for creating next-generation treatments for neurodegenerative disease.
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