Research Work of the Applicant

My PhD student Vigneshwaran's work focuses on developing a novel CRISPR/Cas9 mediated gene manipulation strategy for the gene therapy of β-hemoglobinopathies. By screening the β-globin locus, he identified a 11kb region named PRR-βE1 which on excision alters the hemoglobin switching and activates the therapeutically beneficial fetal hemoglobin. Moreover, the identified target found to be superior compared to well characterised gene editing targets that are currently being studied in clinical trials. Furthermore, PRR- βE1 editing effectively ameliorated β-thalassemia and sickle cell disease phenotype with near complete absence of mutated β-globin. In addition, the hematopoietic stem and progenitor cells gene edited for PRR-βE1 engrafted and repopulated *in vivo* in immunocompromised mouse model with intact genome integrity. Considering the safety and efficacy of this novel gene editing approach, the PRR-βE1 mediated gene therapy strategy can bring a superior therapeutic outcome for the treatment of β-hemoglobinopathies.

Signature of the nominator

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