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Gene Editing and Stem Cell Engineering for Inherited Genetic Disorders

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Inherited genetic disorders affect millions of individuals worldwide and are often associated with lifelong disability and limited treatment options. The convergence of stem cell biology and gene-editing technologies has opened new possibilities for correcting disease-causing mutations at their source. This presentation focuses on the application of CRISPR-Cas9, base editing, and prime editing in patient-derived induced pluripotent stem cells (iPSCs). By correcting pathogenic genetic variants ex vivo, researchers can generate genetically repaired cells that retain the patient's biological characteristics while minimizing immune rejection following transplantation.

Recent advances have demonstrated encouraging progress in treating disorders such as sickle cell disease, β-thalassemia, Duchenne muscular dystrophy, cystic fibrosis, and inherited retinal diseases. Gene-corrected stem cells have shown improved differentiation potential, restoration of normal cellular function, and reduced disease phenotypes in preclinical studies. Early-phase clinical trials are beginning to validate the safety and therapeutic feasibility of these approaches. The presentation also examines current challenges, including off-target editing, genomic stability, ethical considerations, long-term monitoring, and regulatory oversight. Emerging technologies such as high-fidelity CRISPR systems, epigenome editing, and AI-assisted guide RNA design are expected to further improve the precision and safety of stem cell engineering. The integration of gene editing with regenerative medicine represents a transformative strategy for developing durable, personalized treatments that address the root causes of genetic diseases.

Biography

James Patel is a Senior Lecturer in Molecular Genetics at University College London. His research focuses on genome editing, induced pluripotent stem cells, and translational therapies for inherited diseases. He has contributed to several international clinical research collaborations and has published more than 85 peer-reviewed articles in the fields of stem cell biology and gene therapy.