Title: Strategy for Retinal Cell Therapy

Abstract:

Developing retinal cell therapy with iPSC-derived retinal pigment epithelium (iPSC-RPE) cells has provided critical insights into robust manufacturing and patient-centric specification strategies. Our initial approach involved autologous iPSC-RPE transplantation, focusing on achieving high reproducibility across diverse patient samples and cell line generation processes. This led to the establishment of a robust and flexible manufacturing method, producing equivalent cell quality regardless of donor variability.

As we moved toward standardization, we evaluated different delivery formats. We learned that preclinical models were insufficient to determine optimal formulation. Clinical research involving actual patients was essential to identify the most effective and safe transplant formulation. This highlights a key regulatory consideration: cell-based products often differ in structure and function in vivo compared to their final released form. Specifications must reflect these clinical realities rather than rely solely on in vitro parameters.

Moreover, inefficient or overly rigid specification criteria can lead to unnecessary costs and delays. Regulators and developers must collaborate to design flexible, yet meaningful specifications grounded in their outcomes in patients and post-transplant behavior. Additionally, the rise of AI presents transformative opportunities in automating quality and process control, reducing human error, cost, and time across manufacturing stages.

This presentation will illustrate practical lessons from iPSC-RPE development and propose a forward-looking patient-oriented framework in regenerative medicine.