

Novel Method for Long-term *In Vitro* Propagation of Highly Pure Nephron Progenitor Cells

INVENTION: Investigators at Salk have developed a novel 3D culture system that facilitates the stable longterm *in vitro* propagation of both mouse and human nephron progenitor cells (NPCs) isolated from embryonic kidney tissue. Standard methods currently used to generate NPCs involve derivation directly from pluripotent stem cells (PSCs). Although obtaining NPCs using this method has been effective, functional competency *in vivo* has not been demonstrated. In addition, other issues have limited the utility of this method including: high cost, purity issues, long differentiation times and safety concerns. This new method overcomes these limitations and allows for unlimited expansion of highly pure NPCs, which display full nephrogenic potential. Salk scientists were able to use the cells to generate functional mini-kidneys and upon transplantation, restored kidney function in a murine kidney injury model. This new method holds great promise for advancing the study of kidney development and disease, and the development of new drug and cellular therapies.

APPLICATIONS:

- Kidney organogenesis
- Drug screening/Toxicity evaluation
- Disease modeling
- Cell therapy
- Elucidation of mechanisms of kidney development and disease

ADVANTAGES:

- Unlimited expansion potential in culture
- High Purity
- Genome stability in long-term culture
- Derivative cells can restore kidney function upon transplantation into kidney injury models
- Amenable to gene editing

STAGE OF DEVELOPMENT: Validated in murine models of kidney injury

BACKGROUND: Nephron progenitor cells (NPCs) are a transient, self-renewing population of stem cells found in the developing kidney. They give rise to all nephrons, the basic structural and functional unit of the kidney. The nephron is essential for regulating the concentration of water and electrolytes in the blood. The adult mammalian kidney lacks an identifiable population of NPCs, thereby rendering it incapable of regeneration after injury. Destruction of nephrons due to trauma, disease and aging eventually leads to end-stage renal disease (ESRD), which is treated with either dialysis or kidney transplantation. ESRD is associated with significant morbidity and mortality, and its prevalence is on the rise. Alternative strategies for the study of kidney development and disease, and development of novel therapies are greatly needed to both prevent and treat kidney-related diseases.

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