

Stem cell mining: urine cells to biobanking

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Key message

- A safe and accessible source of somatic cell generating induced pluripotent stem cells (iPSCs) in pediatric neurogenic disorders
- A noninvasive and simple method for isolating urine cells, which can effectively reprogram into pluripotent stem cells using episomal vectors
- Establishing a urine-derived iPSC bank as a reliable and scalable resource for disease modeling, therapeutic testing, and personalized medicine in pediatric neurogenic disorders.

Introduction

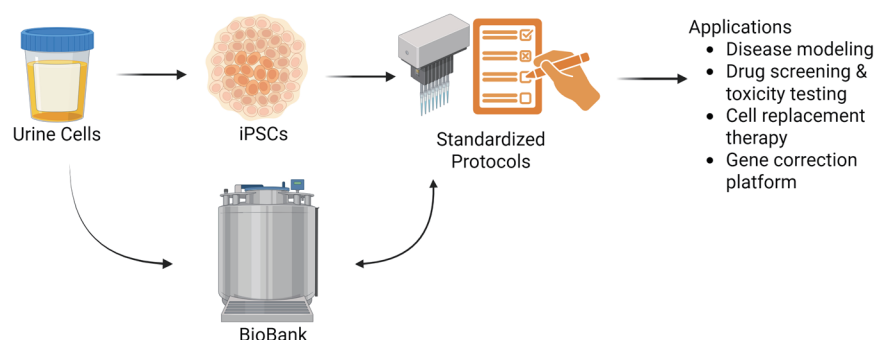
Precision medicine strategies utilizing induced pluripotent stem cells (iPSCs) contribute to the understanding of disease mechanisms and development of cell-based therapies. However, the clinical use of iPSCs has been limited because of difficulties in obtaining donor cells, inconsistent reprogramming resources, and standard protocols. In this issue of Clinical and Experimental Pediatrics, Thai et al.¹⁾ have described the construction of an iPSC bank using urine-derived cells from pediatric patients with neurogenetic disorders. This study provides

evidence that noninvasive, reliable, and safe approaches enable increased access to iPSC resources for pediatric patients.

iPSCs are derived primarily from fibroblasts and blood mononuclear cells. However, they are difficult to use in pediatric patients with neurogenic disorders. In contrast, urine-derived cells can be collected non-invasively multiple times without the need for specialized techniques. Previous research has highlighted this advantage using optimized methods in order to efficiently produce iPSCs and induce neural stem cells from urine cells.²⁾ Dionne et al.³⁾ successfully reprogrammed samples from Fragile X syndrome patients with FXS using Sendai viral vectors. Additionally, urine-derived iPSCs from pediatric patients with brain tumors were transformed into mesenchymal stromal cells with therapeutic potential.⁴⁾ These findings suggest that urine cells are a potential starting point for clinically relevant studies on pediatric stem cells.

Key features and clinical implications

Thai and colleagues have established a disease-specific iPSC bank for early onset neurogenic disorders. The authors have used episomal plasmid vectors to reprogram



Graphical abstract. Pipeline of urine cell-derived induced pluripotent stem cells (iPSCs) for neurogenetic disease therapy. Figure created in <https://BioRender.com>.

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urine cells from 12 patients and confirmed pluripotency using markers and trilineage differentiation tests. Genomic integrity analysis revealed that some lines developed chromosomal abnormalities, emphasizing the need for quality control during long-term culture. This study provides a standardized protocol that eliminates the need for feeder cells, viral vectors, and undefined sera, thereby supporting efforts to improve clinical safety.

Obtaining patient-derived tissues from rare neurogenic disorders has long been a challenge on translational research. An iPSC banking approach using urine cells addresses this limitation by providing a noninvasive, readily accessible platform for disease modeling, testing therapeutic agents, and exploring regenerative medicine.

Challenges

However, several challenges must be addressed for translational applications. First, standardized protocols for reprogramming and culture must be consistent across laboratories. Second, genetic monitoring is required because cells are unstable over time. Third, a cost-effective, efficient, and large-scale banking system is required. Finally, safety levels must be maintained for long-term clinical applications.

Conclusion

Thai et al.¹⁾ faced the challenge of producing high-quality iPSCs in pediatric stem cell research and establishing a disease-specific biobank, particularly for rare neurogenic disorders, thereby providing a foundation for broadening precision medicine. Efforts to integrate standardized protocols, quality control, and the collaborative sharing of resources are essential for the full translational potential of this research.

Footnotes

Conflicts of interest: No potential conflict of interest relevant to this article was reported.

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