Docket #: S23-285

Rapid generation of human forebrain, midbrain, and hindbrain cells from human pluripotent stem cells

Creating human brain progenitors and neurons from human pluripotent stem cells (hPSCs) offers vast possibilities to study, model and treat neurological and neurodegenerative diseases, which are among the most intractable diseases that afflict our society. Despite successes in generating certain types of brain progenitors and neurons from hPSCs, generating a wide range of these cells for various brain regions has remained difficult. In particular, it has remained incredibly challenging to generate hindbrain cells. The hindbrain is a key region that constitutes the majority of the brainstem and is a life-sustaining region that controls breathing, eating, wakefulness, and sleep.

The Loh Lab at Stanford has developed methods to rapidly create forebrain, midbrain, and hindbrain progenitors from hPSCs (within several days), and additionally, multiple types of forebrain and hindbrain neurons (within two weeks). This is accomplished by treating hPSCs with different combinations of extracellular signals in defined, serum-free medium. While these hPSCs can differentiated into multiple brain regions, the inventors have accomplished the first human hindbrain motor neurons differentiation from hPSCs. Hindbrain motor neurons are destroyed by neuron degenerative diseases like spinal muscular atrophy. Therefore, the invention's newfound ability to create human hindbrain motor neurons and other types of neurons provides new opportunities for basic research, disease modeling, and regenerative therapies to replace missing or malfunctioning neurons in vivo.

Stage of Development:
Research - In Vitro

Applications

- Cell therapy
- Neurodegenerative diseases
- Cellular techniques

Advantages

- First and only known invention of a hindbrain cell therapy candidate
- Enables study of hindbrain brain cells, such as neurons, which have never been accessible for this scale of research before
- Method can be used to differentiate other neuron cells for all brain regions

Patents

- Published Application: WO2025049991

Innovators

- Kyle Loh
- Carolyn Dundes
- Rayyan Jokhai
- Hadia Ahsan
- Rachel Salomon-Shulman

Licensing Contact

Inyoung Lee

Licensing Manager, Life Sciences

Email