



SCIENCE FOR THE BENEFIT OF HUMANITY

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ePiggyBac: A Novel Tool For Reprogramming Human Cells

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Technology Summary

Human embryonic stem cells (hESCs) hold immense potential for regenerative medicine, drug screens and basic research for human diseases. The ability to fully tap into the therapeutic potential of hESCs is limited by several factors, including difficulties in generating transgenic hESC lines, lack of reliable reporter systems for gain or loss of function. The present gene targeting techniques for hESCs are often limited to small cargo size and one expression cassette per insertion. These existing systems result in the stable integration of transfer vectors with hESCs, resulting in exogenous inserts in the genome and irreversible genome modifications, which in turn may lead to higher predisposition towards tumorigenesis or uncontrolled cell behavior.

To overcome these limitations, our scientists have developed an ePiggyBac system, by engineering PiggyBac transposons. This system is capable of effectively delivering multigene inserts in hESCs in a reversible manner. In addition, it is also able to carry insulators, inducible expression cassettes, and short hairpin RNAs for gain- and loss- of-function approaches. Hence, the ePiggyBac is a powerful research tool for the stem cell field, and can be used to develop clinical-grade pluripotent cell lines.

Advantage

- Can deliver inserts up to 18Kb
- Show transgenesis efficiency of ~ 90%, which can be reversed.

Area of Application

- Research tool for basic research, drug discovery and development

Stage of Development

- *In vivo* data available from mouse and human cells.

Lead Inventor

- Ali H Brivanlou

Patent Information

- U.S. Patent 8,592,211 (Issued on November 26, 2013)

References

- Lacoste, *et al.* (2009), *Cell Stem Cell*: 5, 332-342.