

Drugs ~Infectious diseases~

Enhancer Compounds of Gene Transfer by controlling the Autophagy mechanism

Principal Investigator

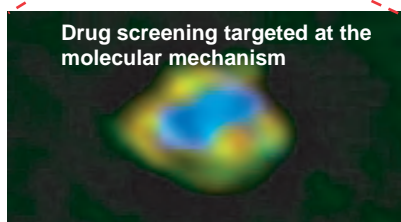
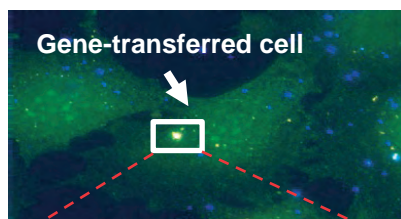
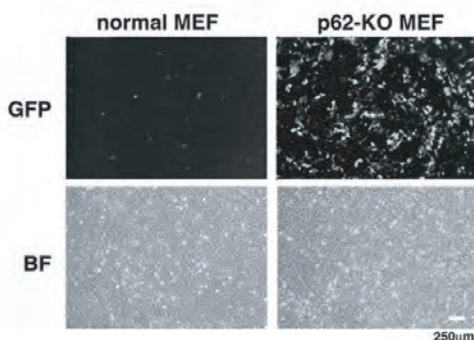
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Project Outline

Gene Transfer Technology has proved to be a pivotal technology in the areas of the treatment of intractable diseases and regenerative medicine through genome editing and iPSC production. In order to enhance gene transfer efficiency, it is necessary to set up the protocol for each cell type such as transfection reagents. Especially, in the case of using hard-to-get samples such as patient-derived cells, it tends to be difficult to consider the conditions for high-efficiency transfection. We have tried to identify compounds that are effective in promoting gene transfer efficiency for a wide range of cell types. As a result, we have found that a combination of several compounds produces a significant stimulatory effect on gene transfer efficiency.

Loss of autophagy-related factors that act as the foreign-pathogen elimination leads to a striking enhancement of gene transfer efficiency. Therefore, it is suggested that the difference in gene transfer efficiency corresponds to the difference of activity of the degradation pathway of nucleic acid after its incorporation into cells



Foreign genes invading the cytoplasm are promptly decomposed by autophagy. This complex contains p62.

Approaches for the compound screening

Effective screening of compounds is implemented utilizing non-biased(A) and target-selected(B) screening systems.

