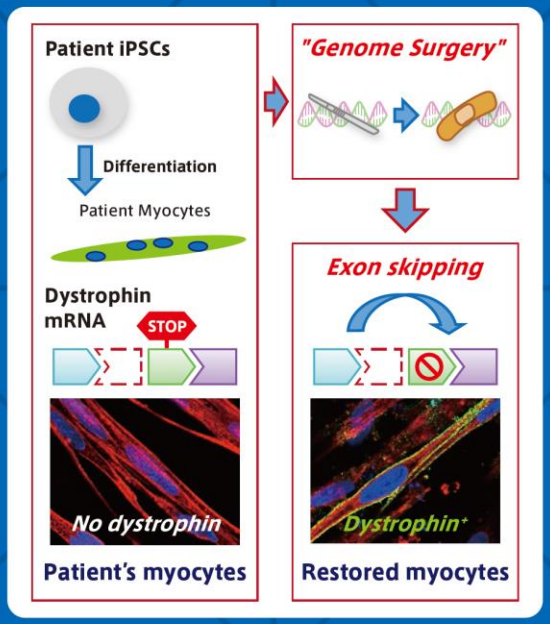
<Concept/Strategy>



〈In Vivo Genome Editing project : Therapeutic genome editing for congenital muscular dystrophy〉

Akitsu Hotta

Dr. Hotta's team aims to correct the causal genetic mutations involved in severe muscular dystrophy using state-of-the-art genome editing and delivery technologies. The team aims to develop technology that will enable them to create new gene therapies while, at the same time, confirming repair efficiency and safety using patient-derived iPS cells.

<Concept>

- When patient-derived iPS cells, which harbor a genetic mutation in the dystrophin gene, are differentiated into skeletal muscle cells, dystrophin protein expression is absent.
- By using genome editing technology to skip exons that carry a genetic mutation, it is possible to rescue the expression of dystrophin protein that retains some degree of functionality.

<Progress>

- Restoration of dystrophin protein expression by genome editing system in muscular dystrophy mice harboring "humanized dystrophin gene".

<Progress>