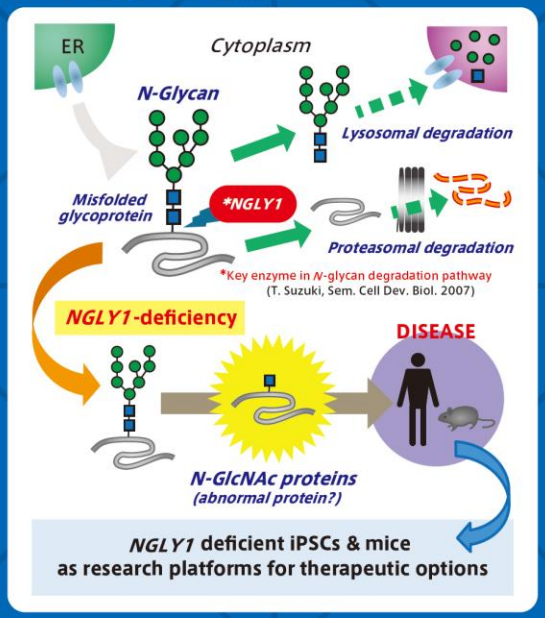
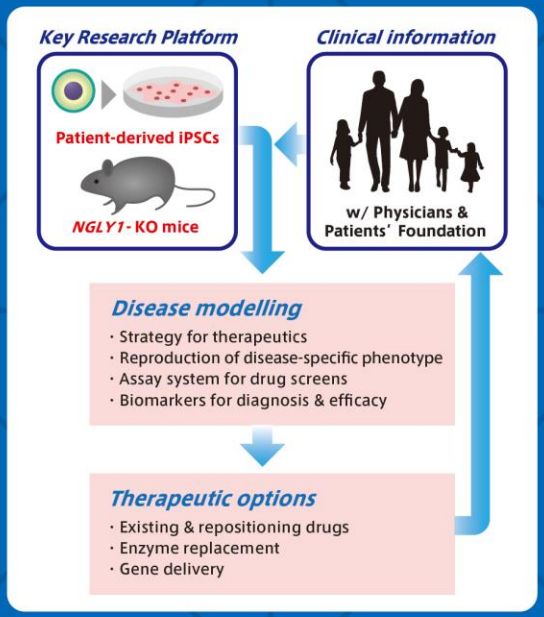
Tadashi Suzuki

〈NGLY1 Deficiency Project : Development of therapeutic agents for rare hereditary diseases using iPS cells〉

Dr. Suzuki's team is focusing on a deficiency in the *NGLY1* gene that encodes for the de-*N*-glycosylating enzyme *N*-glycanase. They will develop innovative therapeutics for *NGLY1* deficiency, a rare inherited disease that presently does not have any therapeutic options, through a combination of basic research findings, iPSC technology and a drug discovery platform.

<Concept/Strategy>





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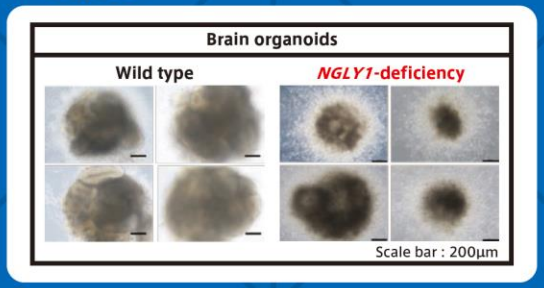

- Recent data suggested abnormalities in brain organoid developed from patient-derived iPSCs
  - Many large neural tissues that have greatly expanded in wild type brain organoids but not in *NGLY1*-deficiency organoids (day 20).
  - *NGLY1*-deficiency organoid which has failed to produce neuroepithelial buds, instead displaying extended cell processes consistent with direct neural differentiation.