

Human stem cells to model stroke and to test new treatments

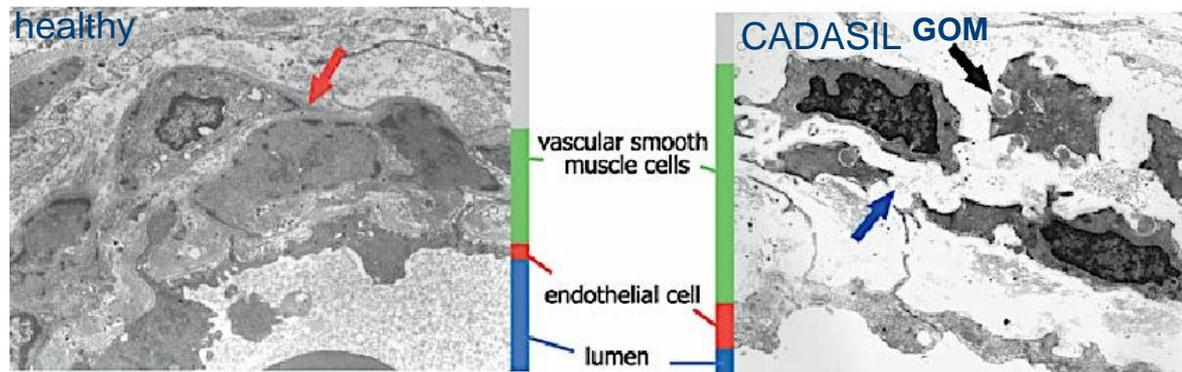
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CURRENT CADASIL MODEL

CADASIL is one of the most common genetic causes of stroke and dementia.

This disease is caused by mutations in the gene NOTCH3, which is found in the muscle layer of blood vessels in the brain (vascular smooth muscle cells).

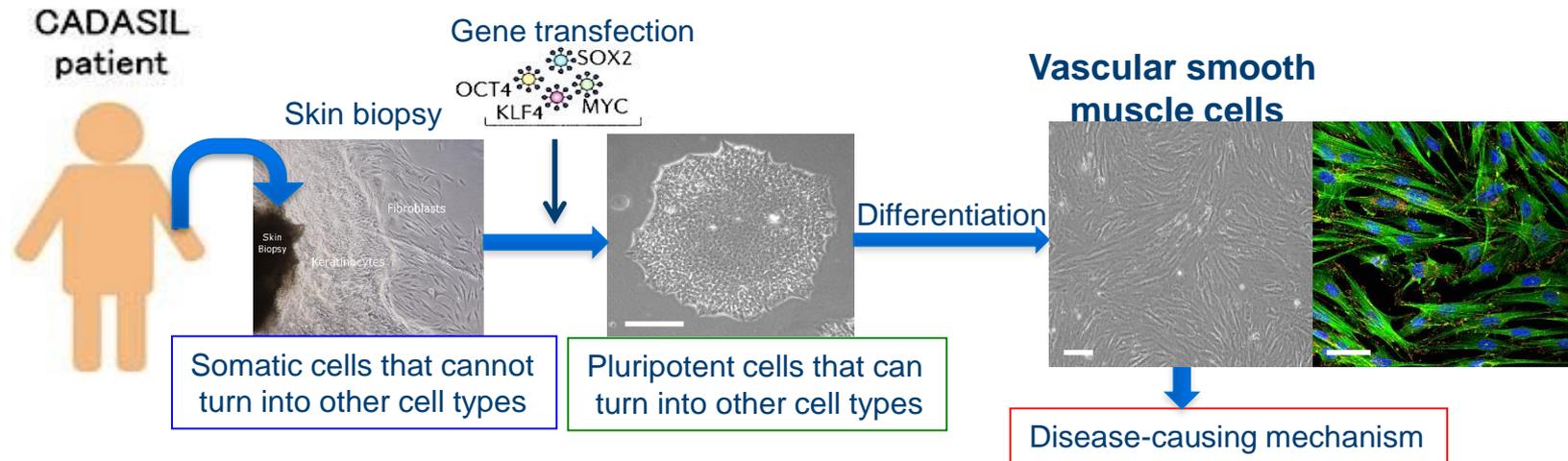


There are animal models expressing CADASIL mutations which reproduce the cerebrovascular dysfunction. However the results are variable due to the species differences between mouse and man.

Currently there is no treatment for CADASIL because we don't know how mutations in NOTCH3 cause disease.

NEW HUMAN MODEL FOR CADASIL

We can generate Human stem cells from a piece of skin donated by patients with CADASIL.



From these stem cells, smooth muscle cells can be generated in a tissue culture dish in the lab and these cells will have the same abnormalities as the patient's own blood vessel cells.

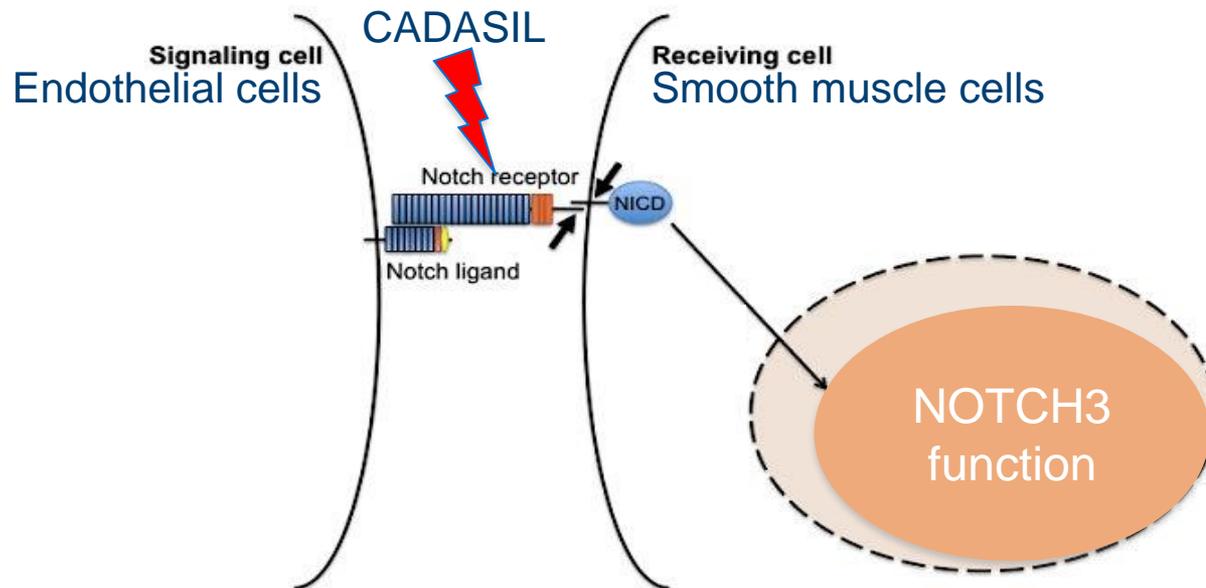
This process creates a 'disease-in-a-dish', which is a new human platform to study CADASIL, which can be used to test new treatments.

NOTCH3 MUTATIONS IN CADASIL

Our human stem cell based model can be used to study one specific cell type affected by CADASIL mutations.

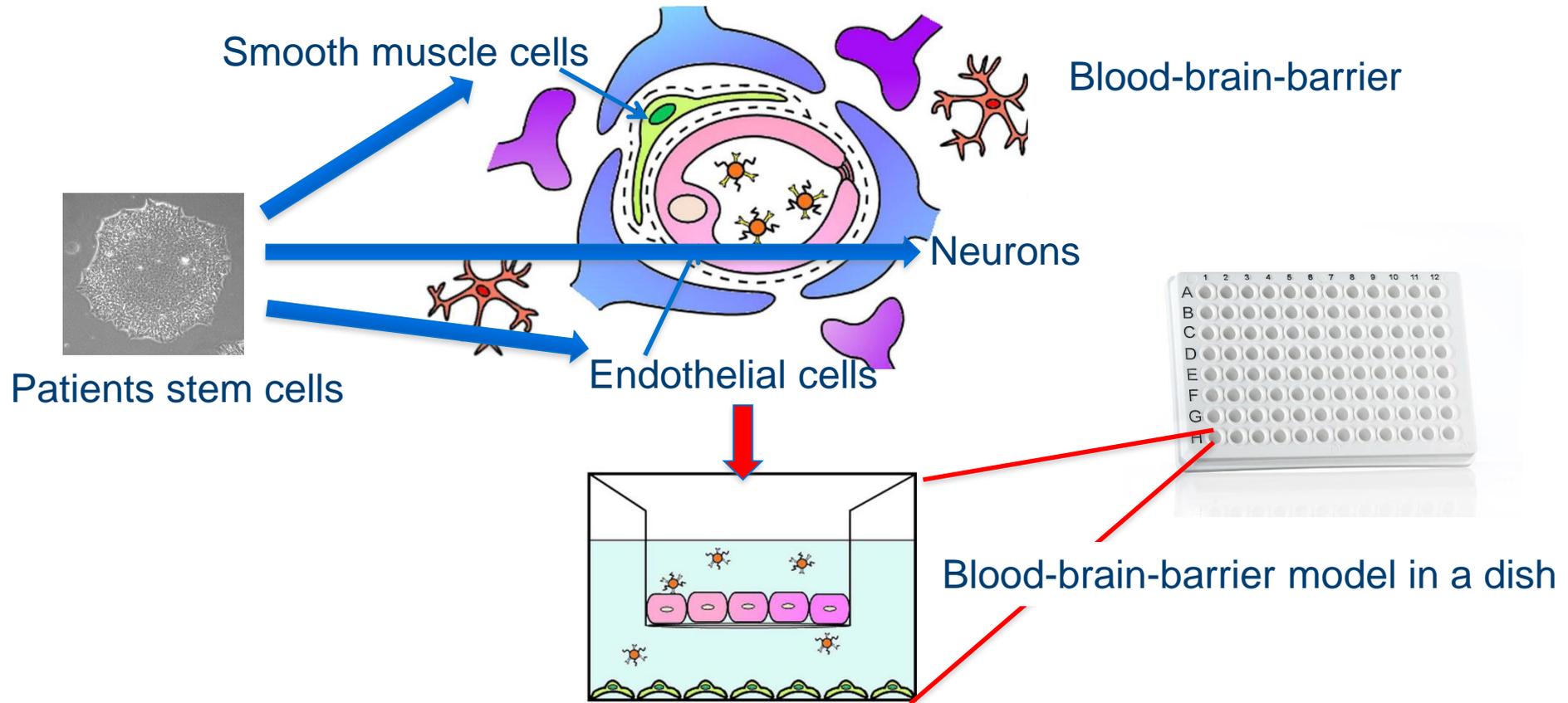
Vascular smooth muscle cells are important for NOTCH3 function.

Mutations in NOTCH3 prevent the normal signaling and cause accumulation of NOTCH3 on the surface of the cells.

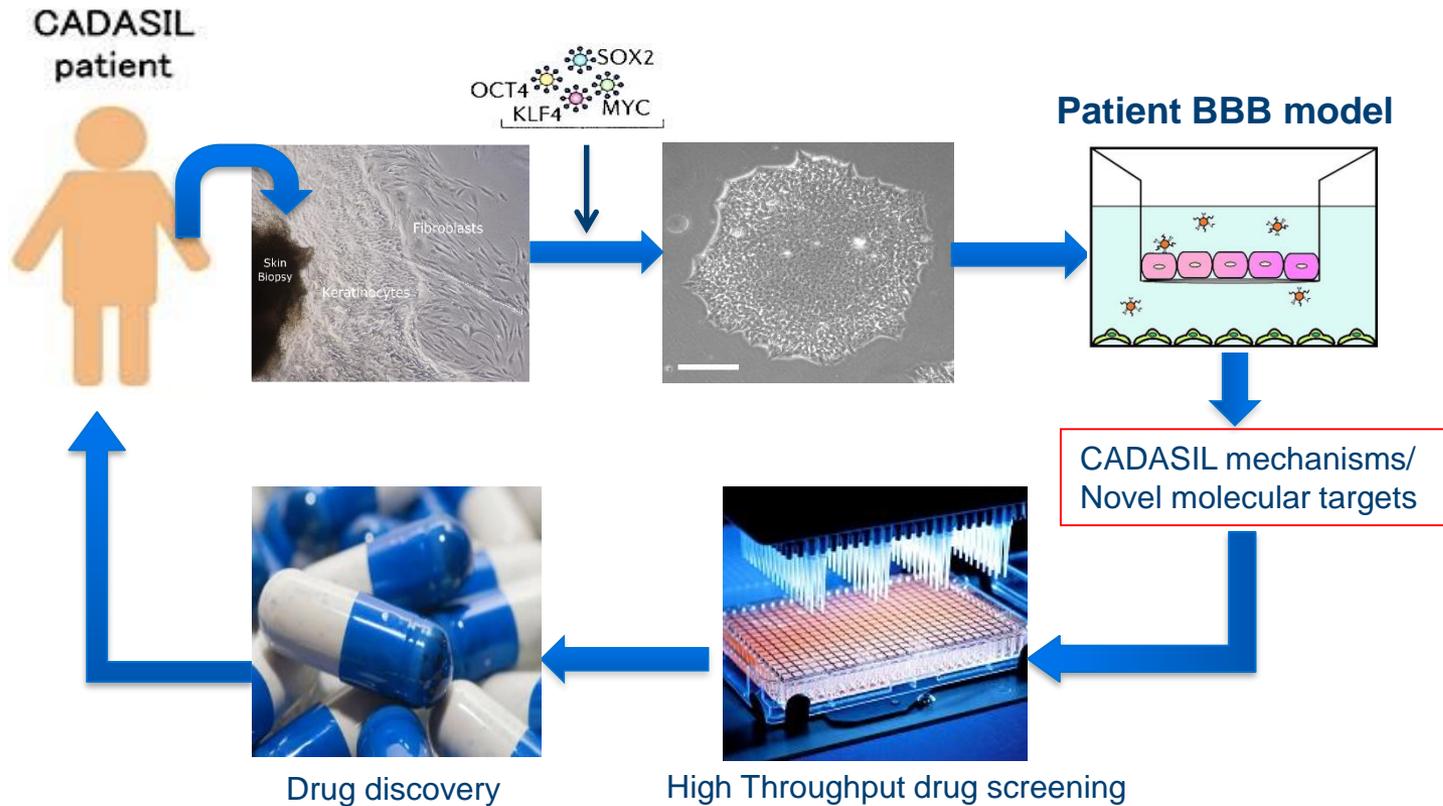


NEW HUMAN MODEL OF BBB FOR CADASIL

Human stem cells will be used to create a multi-cells system to model the blood-brain-barrier (BBB) of CADASIL patients

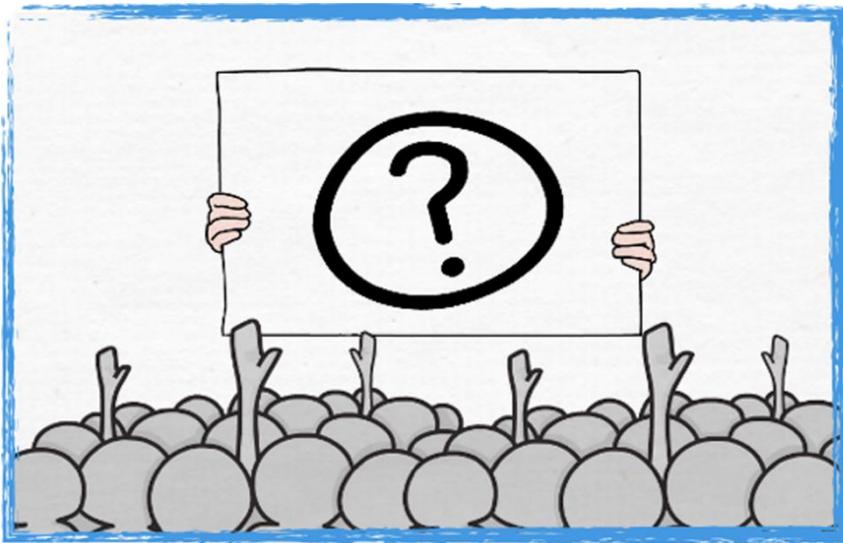


NEW CADASIL MODEL FOR DRUG DISCOVERY



This human model will allow us to better understand the ways that mutations in NOTCH3 cause disease and open the way for new treatments for CADASIL.

Thanks



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