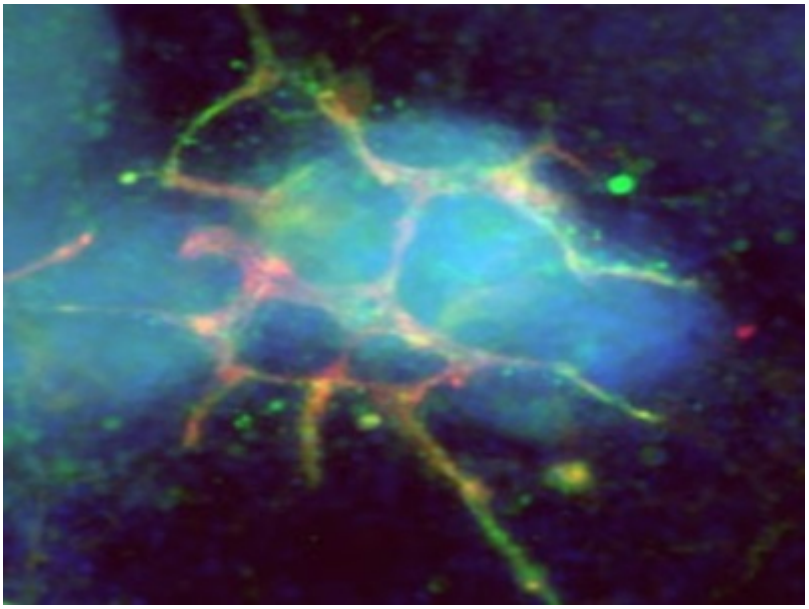
Targeting von Willebrand Factor to Model Disease in Human Pluripotent Stem Cells

The disclosed technology offers a CRISPR-Cas9 generated, isogenic hPSC-EC in-vitro model for studying disease dynamics and therapeutic candidates for VWF-deficient diseases such as Von Willebrand disease (VWD).



What is the Problem?

Von Willebrand disease (VWD) is a blood disorder in which patients have diminished expression or function of von Willebrand factor (VWF). VWF is a blood-clotting protein and low expression can lead to reduced blood clotting ability in patients. Methods of studying VWD and its potential therapeutics are currently limited to VWF knockout animal models and VWF-deficient human donor EC models. These methods are limited by the high degree of variability between donor cell populations, and the low tolerance of passage that the cells have in culture.

What is the Solution?

The solution is the disclosed technology that offers a CRISPR-Cas9 generated, isogenic hPSC-EC in-vitro model for studying disease dynamics and therapeutic candidates for VWF-deficient diseases, such as VWD. This innovation provides an isogenic VWF hPSC-EC cell line proven to be an accurate model for assessing the efficacy of new therapeutics in VWF-deficient diseases. The

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Category

Research Tools
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Selection of Available Technologies

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model enables therapeutic efficacy studies through highly sensitive induction of VWF expression, increasing the clotting ability in patients with VWD.

What is the Competitive Advantage?

The competitive advantage of this technology lies in its ability to improve research methods for studying VWD and potential therapeutics. With a population prevalence of clinically relevant reduced VWF levels estimated to be approximately 1% in the United States, and high costs associated with treating severe VWD, this innovation has the potential to contribute to the development of more effective and cost-efficient treatments.The technology can be applied to a wide range of applications, including studying pathogenicity of VWF mutations, developing gene-edited hPSC-EC therapies, and investigating delayed-release formulations to prolong increased VWF levels in patients with VWD.