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Antisense oligonucleotide therapeutic approach for PCDH19-related encephalopathy

Stanford researchers have developed a genetic strategy using antisense oligonucleotides (ASO) to reduce the levels of PCDH19 in human forebrain neurons, as a therapeutic approach for PCDH19-related encephalopathy.

PCDH19-related encephalopathy is a severe genetic disorder characterized by epileptic seizures and autism spectrum disorders (ASD). This condition presents a unique inheritance pattern: males who lack PCDH19 are unaffected, while heterozygous females experience severe symptoms due to random X-chromosome inactivation, leading to disrupted cell-cell interactions in the forebrain.

To address this, the researchers at Stanford designed a series of ASOs to modulate the splicing and reduce the levels of PCDH19. By applying these ASOs directly to dissociated cells from human cortical organoids, they identified one ASO that effectively reduced PCDH19 expression.

Stage of Development
Proof of concept: in organoid models

Applications

- Epilepsies in children

Advantages

- Targeted therapy

Patents

- Published Application: WO2024206668

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