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CRISPR-Based iPSC Drug Discovery Platform: Targeting GNAS-Driven Disorders for Precision Medicine

Tech ID: 34577 / UC Case 2023-012-0

TECHNOLOGY DESCRIPTION

UCSF innovators have developed a cutting-edge drug discovery platform using genetically modified induced pluripotent stem cells (iPSCs) with activating mutations in the GNAS gene to screen for agents that selectively inhibit hyperactive stimulatory guanine nucleotide-binding protein alpha subunit (Gsa) activity. Addressing the critical need for effective therapeutic screening tools for GNAS-related disorders such as fibrous dysplasia, McCune-Albright syndrome, cancer, and benign tumors, this innovation utilizes CRISPR-based genome editing to simulate human disease states for precise and high-throughput screening. The platform enables measurement of adenylate cyclase activity, cAMP production, GTPase activity, and binding interactions, overcoming traditional limitations by providing unique structural insights for selective inhibitor design. Currently in early development, the technology has shown promising results with validated candidate compounds and has the potential to accelerate therapeutic discovery for CNS disorders and other diseases linked to GNAS hyperactivation. This breakthrough offers biotech companies, technology developers, and venture capitalists a transformative opportunity to advance precision medicine and drug development.

RELATED MATERIALS

- ▶ [AI is a viable alternative to high throughput screening: a 318-target study, PMID: PMC10987645](#)
- [PMID: 38565852](#) - 04/02/2024

PATENT STATUS

Patent Pending

CONTACT

Catherine Smith
Catherine.Smith2@ucsf.edu
tel: [510-646-0631](tel:510-646-0631).



OTHER INFORMATION

KEYWORDS

CRISPR-based drug
discovery, GNAS

hyperactivation screening
platform, cAMP production
assay technology, iPSC
technology for GNAS
disorders, High-throughput
screening for GNAS
inhibitors, Stem cell-based
drug discovery solutions

CATEGORIZED AS

- ▶ **Medical**
- ▶ Disease: Central Nervous System
- ▶ New Chemical Entities, Drug Leads
- ▶ Research Tools
- ▶ Screening

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ADDRESS

UCSF
Innovation Ventures
600 16th St, Genentech Hall, S-272,
San Francisco,CA 94158

CONTACT

Tel:
innovation@ucsf.edu
https://innovation.ucsf.edu
Fax:

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