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**Discovery of therapeutics for Huntington's Disease**

**Grant Award Details**

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Discovery of therapeutics for Huntington's Disease

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-10182

**Project Objective:** Screening and discovery of small molecule therapeutics with in vivo proof of concept as development candidates for the treatment of Huntington's Disease.

**Investigator:**

<b>Name:</b>	Ali Brivanlou
<b>Institution:</b>	Rumi Scientific CA
<b>Type:</b>	PI

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**Disease Focus:** Huntington's Disease, Neurological Disorders

**Human Stem Cell Use:** Embryonic Stem Cell

**Award Value:** \$1,399,800

**Status:** Active

**Grant Application Details**

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**Application Title:** Discovery of therapeutics for Huntington's Disease

**Public Abstract:****Research Objective**

The objective of the proposed research is to perform 3 independent hESC-based screens to identify drug candidates for Huntington's Disease.

**Impact**

There are currently no effective treatments for HD. Combination of human isogenic HD-mutants, novel tools and technology will provide therapeutic solutions for this neurodegenerative orphan disease.

**Major Proposed Activities**

- Screening of 2,000 natural compounds for hits that can rescue the HD germ layer phenotypic signature.
- Screening of 2,000 natural compounds for hits that can rescue the HD early neuronal phenotypic signature.
- Screening of 2,000 natural compounds for hits that can rescue the HD 'giant multinucleated neurons' phenotypic signature.
- In vitro estimation of the potency and toxicity of the top 10 candidate compounds.
- In vivo pharmacokinetics studies of the top 5 candidate compounds.
- In vivo validation of candidate compounds in an HD mouse model.

**Statement of Benefit to California:**

There are two main benefits for California: First, we will introduce a technology, which does not yet exist outside of my laboratory. This complements the mission of CIRM. Because our platform has a wider application than just modeling HD phenotypes, we anticipate the creation of new industries using these methods. Secondly, an estimated 40,000 Californians struggle with this incurable disease. Any improvement to their conditions will be of tremendous value for them, and their loved ones.

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**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/discovery-therapeutics-huntington%E2%80%99s-disease>