

## / IMPROVE ALS DRUG SCREENING

**Project ALS Commitment:** \$1.8M

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**Background:** In 2008, a collaboration led by Project ALS developed the induced pluripotent stem (iPS) cell model, allowing researchers to create an ALS patient's motor neurons in a dish from a simple skin punch. This extraordinary breakthrough, sometimes called *ALS in a Dish* for its ability to effectively model human brain disease outside of the body for the first time, can be used to screen for drugs more efficiently and understand what happens to motor neurons in ALS in an actual person living with the disease.

Now, even as ALS organizations around the world undertake ambitious projects that rely on the development of iPS cell lines from hundreds—even thousands—of people with ALS, considerable doubt remains in the ALS research field about the accuracy of the *ALS in a Dish* model. Most significantly, motor neurons developed using the iPS technique are young—and therefore, even though they may recapitulate the motor neurons of a person currently living with ALS, they don't show symptoms of the disease. Project ALS is working with leading stem cell biologists to refine the iPS model so it more accurately models *ALS in a Dish*—with the goal of more effective, efficient screens for drugs that may slow or stop ALS.

**Summary of Progress:** The ultimate goal of this project is to be able to predict the efficacy of potential ALS drugs more accurately by creating the “gold standard” of ALS drug screening by differentiating ALS patients' cells directly into mature motor neurons that reflect the *phenotype*—observable traits—of the ALS disease process. In tandem, Project ALS researchers have improved current iPS models to advance drug screening now. Notably, they have (1) developed a new technology that enables them to trace a motor neuron over a longer period of time than ever before, so its phenotype can be studied for months instead of days; (2) created a “reporter” line that, for the first time, allows researchers to trace both spinal and cranial motor neurons, and (3) refined a panel of stressors so even as young cells, *ALS in a Dish* shows symptoms of ALS.

**Relevant publications:**

[Development of ALS in a Dish](#)

[Eggan: Improving iPS motor neurons](#)

[Rubin: Discussion of how to create “gold standard” iPS cells](#)

Contact us for additional relevant publications or research updates.