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## September Newsletter

### CIRM September Newsletter

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### Treatment for Spinal Cord Injury To Start Clinical Trial Funded by California's Stem Cell Agency



Asterias Biotherapeutics Inc. has been given approval by the FDA to begin a clinical trial of its stem cell therapy in patients with spinal cord injury, which is being funded by CIRM.

The Phase 1/2a clinical trial is designed to test the safety of this approach and to see if it has any benefit for patients. The trial will use increasing doses of Asterias' AST-OPC1 cells. These are a form of cell called oligodendrocyte progenitor, which are capable of becoming several different kinds of cells some of which play a supporting role and help protect nerve cells in the central nervous system, the area damaged in spinal cord injury.

- [Read our press release](#)
- [Read our blog about this milestone](#)
- [Read more about Asterias Biotherapeutics](#)

### Type 1 Diabetes Treatment Funded by California Stem Cell Agency Cleared To Begin Clinical Trial



ViaCyte Inc., a regenerative medicine company headquartered in San Diego, has been given approval to begin a clinical trial for a promising treatment for type 1 diabetes, a program that is funded by CIRM.

The Food and Drug Administration gave ViaCyte the go-ahead after reviewing their application for an Investigational New Drug application ("IND"). The phase 1/2 clinical trial will see if their VC-01™ product candidate is safe and shows preliminary evidence of being beneficial to patients.

- [Read our press release](#)

### **World's Largest Pharmaceutical Company Signs Agreement with ViaCyte for a Stem Cell Treatment for Type 1 Diabetes**



And in more ViaCyte news, the company has announced a major agreement with Janssen Pharmaceuticals, Inc., a Johnson & Johnson company, and its affiliated investment fund, the Johnson & Johnson Development Corporation (JJDC).

On August 19th, ViaCyte got approval from the FDA to start a clinical trial of its treatment for type 1 diabetes. CIRM has invested almost \$40 million to help develop the treatment over the past six years. Under this new agreement Janssen and JJDC will provide ViaCyte with \$20 million with a future right to consider a longer-term transaction related to the VC-01™ combination product that ViaCyte is developing for type 1 diabetes.

- [Read our press release](#)
- [Read more about VC-01™ on Viacyte's website](#)

### **Dr. David Higgins appointed as Patient Advocate for Parkinson's disease to Stem Cell Agency Board**



His personal experience as someone living with Parkinson's disease is one of the reasons why Dr. Higgins has been named as the newest member of the Independent Citizens Oversight Committee (ICOC), the governing Board of the state's Stem Cell Agency.

The appointment was made by State Controller John Chiang who praised Dr. Higgins for the wide range of attributes he brings to the role:

"Diagnosed with Parkinson's in 2011, his experience with the disease and its impact on a personal level brings a distinct perspective to the ICOC. Dr. Higgins also brings a broad range of experience from the biotechnology field. As a trained molecular biologist, his involvement in drug development and business operations places him in a unique position, understanding both science and process."

- [Read our press release about Higgins' appointment](#)

### **Debut of CIRM's New Video Series: "Stem Cells in your Face"**

To a stem cell scientist, the phrase "disease in a dish" describes a powerful way to study human disease in the lab. But to a non-scientist it sounds like a scene from some disgusting sci-fi horror cooking show.

To clear up this confusion, we created a new video: Disease in a Dish: That's a Mouthful. The first in our new video series, "Stem Cells in your Face," this video takes a lighthearted approach to how scientists use stem cells to study ALS, also known as Lou Gehrig's disease.

Click below to watch the video in full on YouTube:



### **Creativity Program Students Reach New Heights with Stem Cell-Themed Rendition of "Let it Go"**

This summer we sponsored high school interns in stem cell labs throughout California as part of our annual Creativity Program. As part of the program, we asked those students to share their experiences through blog posts, photos and videos.

This year, the students outdid themselves with their creative submissions. We were especially thrilled with the outstanding group video from CIRM Interns at City of Hope in Los Angeles, with their own special version of the popular song, "Let it Go" from the movie *Frozen*. And now, the song has made a splash nationwide—with coverage from ABC 7 Bay Area and even NBC New York!

Click below to view the video on YouTube—and check out our other top student submissions at The Stem Cellar.



### The Latest Publications on CIRM-funded Research

CIRM grantees are making steady progress on understanding the fundamentals of stem cell biology and moving stem cell-based therapies toward clinical trials. Here are examples of CIRM-funded research that was published in July along with press releases from the grantees' home institutions and entries from the Stem Cellar, CIRM's official blog:

#### August 7th - *Neuron*

- The Stem Cellar: (UCSD) Grafted Stem Cells Snake through Spinal Cord, CIRM-Funded Study Finds

#### August 14th - *Nature*

- The Stem Cellar: (UCSD) Creaky Cell Machinery Affects the Aging Immune System, CIRM-Funded Study Finds

### Upcoming CIRM Events

**September 10th:** The CIRM governing Board will hold its next meeting at the Claremont Hotel in Berkeley, CA.

The agenda and details will be posted to the **meetings page** 10 days before the event.

**September 11th:** Free Webinar, "Use of Induced Pluripotent Stem Cells as Screening Tools and Therapeutics, Presentations from FDA and Leading Experts."

Registration and details available here. Advanced registration required.

**October 7th-8th:** CIRM Grantees present at Stem Cell Meeting on the Mesa Investor & Partnering Forum.

CIRM is proud to have 6 of its Grantees presenting at the Investor and Partnering Forum at the Stem Cell Meeting on the Mesa (SCMOM) sponsored by the Alliance for Regenerative Medicine.

The 4th Annual Partnering Forum at SCMOM includes companies who are conducting 100+ clinical trials with an additional 25+ products already on the market! With 10+ hours of presentations over the course of two days, this meeting provides attendees a unique opportunity to forge collaborations with regenerative medicine's top industry leaders.

For more information about SCMOM please visit: <http://stemcellmeetingonthemesa.com/partnering-forum/>

## CIRM Funding Opportunities

### Current Applications

**RFA 14-03: CIRM Strategic Partnership IV Awards** - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. **This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.**

**RFA 14-02: CIRM Preclinical Development I Awards** - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. **This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.**

**PA 14-01: CIRM Accelerated Development Pathway** - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. **This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.**

**RFA 13-04: External Innovation Pilot Program** - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. **This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.**

**PA 08-06: CIRM Conference Grants** - Applications for conference grants will be accepted, reviewed and approved periodically

Get more info about CIRM RFAs

Sign up to receive CIRM funding announcements

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