Government-funded journal seen by some as waste of grant money

This summer, California's $3 billion stem cell agency is scheduled to launch a state-subsidized open-access research journal. With a $600,000 taxpayer-backed starting fund, the publication is intended as a forum for translational scientists and regulators geared toward moving stem cell–based therapies to the clinic. But with more than a dozen stem cell–focused journals already crowding library shelves and a limited agency budget, many critics wonder whether this is the best use of government research dollars.

"They need to demonstrate a need, and I don't think they have done that," says John Simpson, stem cell project director of Consumer Watchdog, an advocacy group based in Santa Monica, California.

Agency staff first approved funding for the online scientific publication last June, citing the lack of a suitable journal in which to publish translational successes and failures in the stem cell field. "Some of this work gets published, but it's in variable places," says Alan Trounson, president of the California Institute for Regenerative Medicine (CIRM), based in San Francisco, "and very little if any of the negative work that doesn't result in success moving forward is published."

A government-funded research journal is not entirely an unprecedented move. In the US, the National Institute of Environmental Health Sciences publishes *Environmental Health Perspectives*, a monthly open-access journal with the second-highest impact factor among environmental sciences publications, and the Centers for Disease Control and Prevention puts out the *Morbidity and Mortality Weekly Report*. Other countries have similar arrangements. In Australia, for instance, the Commonwealth Scientific and Industrial Research Organisation publishes close to 30 titles, spanning reproductive biology to wildlife research, all based on a subscription model.

Yet CIRM's proposed journal, tentatively slated to launch by the end of the summer, will be different. Instead of acting as the publisher, the stem cell agency plans to provide only start-up support. The company or society selected to run the journal is expected to become self-sustaining within three years. According to CIRM spokesperson Don Gibbons, the agency chose the journal's publisher on 24 January, but, as *Nature Medicine* went to press, had yet to make the deal public owing to ongoing contractual negotiations. Contenders included two private companies—Elsevier, which publishes *Cell Stem Cell* and *Stem Cell Research*, and AlphaMed Press, which publishes *Stem Cells*—and the nonprofit International Society for Stem Cell Research.

Arnold Kriegstein, director of the Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research at the University of California–San Francisco, applauds the move. He says that what sets this new stem cell journal apart is that it provides researchers an outlet to publish what doesn't work, saving researchers time and saving taxpayers money. "What I find most novel is the idea that there would be negative results published here," he says. "I think that's the big attraction and the big element that seems to be missing for what's out there currently."

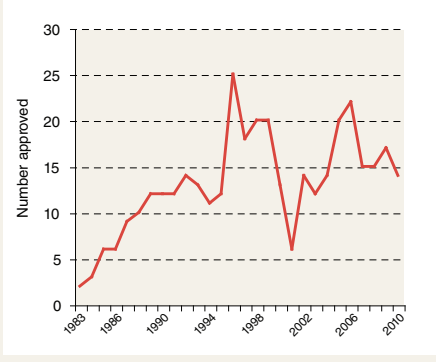
But Martin Frank, executive director of the American Physiological Society, which publishes 14 journals, argues that government research dollars are better spent at the bench. "We are not flush with money today," he says.

Michelle Pflumm

Despite surge in orphan drug designations, approvals still lag

In 1983, US lawmakers passed the Orphan Drug Act to encourage pharmaceutical companies to pursue treatments for largely ignored diseases affecting small populations. And for the next 15 years or so, the number of rare diseases given orphan drug status hovered between about 40 and 80 per year. But over the last decade, that number began steadily increasing, and last year the US Food and Drug Administration (FDA) granted a record 192 designations.

"There's been a substantial upsurge of interest in orphan drugs and rare disorders," says James Cloyd, director of the Center for Orphan Drug Research at the University of Minnesota–Twin Cities College of Pharmacy. According to Cloyd, reasons for this jump include a push from federal regulators and patient advocacy groups to target rare diseases, new genomic technologies that allows researchers to subtype diseases into rare niches, and the death of the blockbuster model of drug development



Developing orphan drugs "makes a lot of business sense if you don't have a blockbuster," says Syamala Ariyanchira, an independent Malaysia-based pharmaceutical consultant who estimates that the orphan drug market will exceed $80 billion this year.

Yet despite all of the new designations from the FDA—which give drug developers extended market exclusivity, tax breaks on clinical trials and a waiver of some application fees—the regulatory agency approved only 14 new orphan drugs in 2010, a number on par with the average across the past two decades.

Part of the explanation is that orphan designation is relatively simple, requiring only that a company show scientific rationale that its experimental drug will treat a specific rare disease, whereas drug approval still requires lengthy clinical trials. Thus, Ian Phillips, director of the Center for Rare Disease Therapies at the Keck Graduate Institute in Claremont, California, predicts the uptick in current designations will translate into new marketed therapies in about six to nine years.

Although the increase in designations is a welcome advance, there are still fewer than 400 approved orphan drugs and around 7,000 rare diseases still without any available treatments, notes Sharon Terry, president and chief executive of Genetic Alliance, a Washington, DC–based advocacy group. "I'm not sure that's a success story," she says.

Monica Heger