# NCATS launches drug repurposing program

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Francis Collins, NIH director, told big pharma to "open up their freezers."

A newly launched initiative from the US National Institutes of Health (NIH) National Center for Advancing Translational Sciences (NCATS) brings academic scientists and eight of the world's largest pharma companies together to find new uses for abandoned compounds. The program—Discovering New Therapeutic Uses for Existing Molecules—announced in May, will provide opportunities for academic researchers to access some of big pharma's compounds that were shelved during development. First to join were Pfizer, AstraZeneca and Eli Lilly. Five other companies—Abbott, Bristol-Myers Squibb, GlaxoSmithKline, Janssen Pharmaceuticals and Sanofi—followed, increasing the number of compounds available for study to 58. The hope is that by allowing a much wider range of minds to ponder over these promising but abandoned compounds, novel uses for the drugs can be found. The deal gives researchers outside the NIH access to the compounds together with valuable information, and established templates for potentially complex agreements. Although there is widespread agreement that the project marks an important step forward in the relations between industry and academia, there are also doubts about its value.

For decades, academic researchers have bemoaned the fact that many pharmaceutical companies are secretive about compounds in development, particularly those that are not even being pursued because of business or other considerations not related to safety. These compounds, many argue, represent a great starting point from which to search for drugs for various conditions. "The compounds being made available have already been subjected to years of research and lots of money, now they are being made available to the entire brain trust of NIH," says Kathy Hudson, NIH deputy director for science, outreach and policy.

"There may be knowledge out there we don't know about," says Steve Felstead, vice president of clinical research at Pfizer in New York. "We don't tend to cover every possible therapeutic indication when we study compounds; perhaps the wider community can facilitate some new clinical or preclinical studies that will give us vital new information."

The first requests for applications went out mid-June as the list of molecules available for repurposing was disclosed (http://www.ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/directory.html). Those brief applications will undergo 'garden variety' NIH peer review, after which some researchers will be invited to submit a longer application. Using the project's templates, designed to streamline the legal and administrative process for partnering across multiple organizations, the researchers will at the same time start forging an agreement with the compound's owner, thereby saving time and duplication of effort. This eases the administrative burden on pharmaceutical companies considerably, as they do not have to negotiate separate agreements with various academic research centers. NIH aims to make $20 million worth over grants within a year, hoping other institutes will at the same time help fund projects in their fields. Pre-applications responding to this first call are due by August 14.

The project is modeled on the UK's Medical Research Council (MRC) and London-based AstraZeneca mechanisms-of-disease grant program launched in December last year. The MRC project grants academics access to 22 AstraZeneca compounds, and information about them is posted on the MRC website.

Indeed, the NIH group worked closely with MRC and AstraZeneca in developing its new program, although there are some key differences, according to Hudson. The MRC project involves just AstraZeneca and a wider range of chemical compounds, she says, the NCATS arrangement involves several pharmaceutical companies. "Some of [the

## IN brief

### Canada approves stem cell product

On May 17, Health Canada approved its first stem cell therapy, Prochymal, produced by Columbia, Maryland–based Osiris Therapeutics. Only a few weeks later, Medsafe, the medical regulatory agency in New Zealand, approved it, too. In both countries, the drug, a preparation of bone marrow–derived mesenchymal stem cells, will be used to treat graft-versus-host disease (GVHD), in children under 18. The approval is surprising given that in 2009, Prochymal missed two phase 3 trial endpoints in severe refractory GVHD despite startling phase 2 results, prompting the company to abandon the indication in the United States (*Nat. Biotechnol.* **27**, 966–967, 2009). The Canadian go-ahead was based on a subset of data, in which Osiris claimed a clinically meaningful response in some 64% of children. GVHD, a life-threatening immune system reaction, affects more than 40% of children who receive bone marrow transplants. But Health Canada itself says the efficacy data are not conclusive and, as a condition of its approval, Osiris must carry out an international trial within five years. Edwin Horwitz, a pediatric oncologist at the Children's Hospital of Philadelphia, found the approval of a subset "mind boggling," especially because Osiris has not published its data. "The ultimate success depends on doctors' willingness to use it," he says. "Data would lend confidence." Willem Fibbe, who also researches the clinical use of mesenchymal stem cells, says it is not clear whether the study was blinded, whether the *post hoc* analysis resulted in a biased interpretation of data, or whether the study was sufficiently powered. "Altogether, I have some pretty serious methodological concerns related to design and interpretation of the data," says Fibbe. Lee Buckler, managing director of the Bellingham, Washington–based consulting firm, Cell Therapy Group, admitted that publishing the data would be useful, but he says the Canadian government's willingness to look—where the US Food & Drug Administration does not—at retrospective data, is valid. "They have a different risk-benefit analysis of the drug," says Buckler. *David Cyranoski*

## IN their words

**"There's not really much doubt: if you want to reach the upper echelons of wealth, creating a social networking site is a better bet than inventing a drug."** Journalist Matthew Herper, following Facebook's floatation, on how researchers who invent lifesaving drugs don't become billionaires the way IT entrepreneurs have. (*Forbes*, 18 May 2012)