

CONCEPT CLEARANCE RECORD
FY 2017 RESEARCH INITIATIVE – NCATS

TITLE: NIH-Industry Program: Discovering New Therapeutic Uses for Existing Molecules

OBJECTIVE(S): To support the exploration of new therapeutic uses for investigational drugs or biologics (assets) from across a broad range of human diseases. This innovative program enables investigators to propose new therapeutic uses for assets from pharmaceutical company partners. The overarching goal is to enable an efficient drug repurposing partnership model that is adopted by the biomedical research community.

DESCRIPTION: NCATS' Discovering New Therapeutic Uses for Existing Molecules (New Therapeutic Uses) program was designed to enable efficient three-way drug repurposing partnerships among academia, government and pharmaceutical partners. Strategies used to accomplish this include posting limited confidential information about investigational assets (new molecular entities and biologics) offered by pharmaceutical companies to a public website. This facilitates crowdsourcing of ideas for new therapeutic uses from academic medical centers or companies in the form of grant applications to NCATS. The program has shortened the time needed to establish collaborations between pharmaceutical companies and academic institutions to 3 - 4 months (from the typical 9 - 12 months or more), by publicly posting template collaborative research agreements and template confidential disclosure agreements that are executed between the applicant institution and the pharmaceutical company that provides the asset for testing a new therapeutic use. Some participating companies have indicated they now use template agreements as a starting point for negotiations with academic medical centers, even when the NIH is not involved, as a result of their experience using template agreements in the New Therapeutic Uses program. NCATS is issuing a new round of funding opportunity announcements for New Therapeutic Uses.

IMPORTANCE: This program offers a unique opportunity for researchers to test new indications for well-developed assets from pharmaceutical companies. Assets have undergone significant pre-clinical and safety testing in humans and are ready for additional testing in patient populations. Bringing together the best assets from pharmaceutical companies with the best new ideas from academic researchers could produce new treatments much more quickly than starting from scratch.

HISTORY: NIH launched the New Therapeutic Uses collaborative program in May 2012. By pairing researchers with a selection of pharmaceutical industry assets, this innovative program aims to test ideas for new therapeutic uses, ultimately identifying promising new treatments for patients.

In June 2013, as part of a pilot initiative, NIH awarded \$12.7 million to fund two- and three-year cooperative agreements for nine projects in areas that include Alzheimer's disease, alcoholism and lymphangioliomyomatosis, a type of progressive lung disease.

In May 2014, NCATS issued four funding opportunities and provided information about partially developed therapeutic assets. For the first time since the launch of the program, the participating pharmaceutical companies made assets available that were suitable for exploring pediatric indications.

In July 2015, NCATS awarded nearly \$3 million to support four academic research groups to test a selection of pharmaceutical industry assets for new therapeutic uses.

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