

NCATS: The National Center for Advancing Translational Sciences

Accelerating the translation of discoveries to new therapies for patients

The current drug development paradigm is not working – it takes an average of **13 years and \$1 billion** to develop a new drug. Despite our strong national commitment to medical research, and to the National Institutes of Health (NIH) in particular, the advances achieved in basic science are not being translated into new therapies, better prevention strategies, and cures fast enough.

Creation of the National Center for Advancing Translational Sciences (NCATS) would **speed the translation of basic discoveries to real world applications and ensure that we are focused on the end goal of our investment in research – treatments and cures.**

NIH Director Francis Collins has described his [vision for NCATS](#) to the scientific community.

***But what does NCATS mean to patients?
To patients, saving time means saving lives.***

NCATS would accomplish this by creating tools and making these widely available, thereby fostering efficiencies. This means the process of translational research will be improved and patients will benefit.

This vision provides an opportunity for the patient and disease communities to work together on addressing a problem that cuts across diseases.

And as a component of the NIH, **NCATS would be able to leverage its extensive network of relationships and collaborations with academia, patient and disease organizations, and industry.**

For example, **only 1 in 10,000 potential therapeutic compounds makes it through the drug development pipeline to the marketplace.** However, in recent years, researchers have been learning that a compound that is a failure for one condition may turn out to be a godsend for people suffering from another disease.

NCATS would support creative ways to rescue drugs that have been abandoned by industry before FDA approval.

To date, there has been no effort to encourage this in a broad and systematic way. While most compounds prove to be safe in human clinical trials, many fall by the wayside relatively late in the development process because they do not effectively treat the condition for which they were intended. When that happens, biotechnology and pharmaceutical companies typically shelve the compound and all related data.

The result is tens of thousands of compounds sitting 'abandoned' in industry freezers. NCATS wants to change that. **Given its status as a neutral third party, NCATS may be able to serve as an honest broker to match compounds abandoned by industry before approval with potential new applications.**

One example of the power of drug rescue is azidothymidine (AZT), a compound that was abandoned in the 1960s after failing to show efficacy against cancer. More than two decades later, a collaborative effort between NIH and the private sector allowed NIH researchers to rescue AZT after they discovered that it was effective against an entirely different health threat: the human immunodeficiency virus, which causes AIDS. AZT went on to become the first drug approved to treat HIV/AIDS, providing much-needed hope for people suffering from what was then a swiftly fatal disease.

All told, **it took 25 months from the points when researchers learned AZT was active against HIV in the lab and FDA approval**—one of the shortest drug development timelines in recent history.

Another way to speed the drug development process and to reduce costs is to find new uses for drugs already approved by the FDA for a different condition. Many pharmaceutical companies have already started exploring repurposing FDA-approved drugs. However, there have been only limited exchanges of information with other companies and with academic researchers working to find treatments for other diseases.

NCATS would be a logical entity to facilitate the repurposing of FDA-approved drugs.

We have already seen how this can work. **One noteworthy example of repurposing is thalidomide.** Originally developed in the 1950s to treat morning sickness, it was pulled off the market because it caused severe birth defects. However, researchers later found that the drug could improve pain and skin inflammation in people suffering from leprosy. More recently, research teams supported by NIH found that thalidomide could inhibit the growth of certain cancers, and, in 2006, the FDA approved the drug to treat multiple myeloma.

NCATS would also serve to strengthen the translational efforts underway at many NIH Institutes. Many Institutes at NIH support both basic and translational research portfolios in an effort to move discoveries through the pipeline more quickly.

- Mid-sized and smaller Institutes that may not possess significant expertise in drug development would benefit greatly from having access to a central hub at NIH where such expertise exists.
- Another specific way that NCATS would assist the Institutes in enhancing their translational research efforts is in the design of projects; one Institute Director indicated that in a project they had just designed, **if NCATS had existed as a resource approximately two years would have been saved** because the expertise would have been in place to advise them to be more efficient.

NCATS would have accelerated much-needed safety studies on stroke therapies

National Institute of Neurological Disorders and Stroke (NINDS) had solid evidence from hypertension rats that they can reduce to almost nothing the number of strokes rats have as they age. It has taken NINDS seven years to get to the point where they have toxicology studies that allow them to move into safety studies in humans. **If NCATS had been in existence, the process of moving this forward into people would have been accelerated.** Toxicity is the most common reason for drug development failure, and NCATS plans to tackle this problem by developing better ways to predict the safety of compounds before they are tested in humans. Creative ways of doing preclinical toxicology testing that test compounds directly on human cells as opposed to relying on animals is needed. NCATS efforts in this area will build upon what has been learned by Tox21, a recently formed partnership involving NIH, the Environmental Protection Agency, and the Food and Drug Administration. This consortium has already begun testing new cell-based approaches for predicting the safety of various chemical compounds, many of which are potential drugs.