NIH program strives to turn more lab discoveries into real-world treatments

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Seeing his or her discovery translated into a new drug, vaccine, diagnostic test, or medical device that improves human health is something that gives a biomedical scientist tremendous professional and personal satisfaction. But with almost 70,000 biomedical scientists\(^1\) in this country, why is it rare to meet one who has had that experience?

Translating fundamental scientific knowledge into actual treatments for diseases is exceedingly challenging. Research often reveals the molecular and systemic changes that cause or contribute to a disease. Although that can lead to new ideas about how to prevent or treat that condition, only a tiny fraction of these ideas ever make it to being tested in humans. Of the few that do, the necessary clinical trials can take hundreds of millions of dollars and many years to complete, and in that process most will fail to show sufficient safety and effectiveness. It’s also next to impossible to predict which avenues of research will ultimately lead to medical breakthroughs.

Take, for example, the new class of drugs known as immune checkpoint inhibitors\(^2\). Basic research about the immune system in the 1970s led to pioneering studies in animals in the late 1990s and early 2000s
showing how the immune system can be unleashed to attack tumors. It took another decade of development by biotech and pharmaceutical companies before the clinical benefits of this strategy were proven, but the upshot is a new treatment that looks set to transform cancer care\(^3\) and help millions of patients.

Successfully commercializing academic innovations benefits society as a whole via the creation of new products and jobs\(^4\). So how can we help that happen?

True to its motto of “Turning Discovery Into Health”\(^5\), the National Institutes of Health aims to answer that question. The NIH distributes more than $22 billion\(^6\) in federal funding each year to academic institutions, mostly for knowledge-driven basic research. Now it wants to convert more of the research it supports into tangible products. Authorized by Congress to create a pilot proof-of-concept program, the NIH established the Research Evaluation And Commercialization Hubs\(^7\) (REACH) network in 2015 to lower the barriers that commonly slow the translation of academic research.

As directors of the first three REACH hubs, we are committed to making this concept work.

One key barrier is that most academic researchers know little about the process of bringing a new technology to market. Scientific discovery is only the start of a long and complicated journey that involves issues beyond the comfort zones of most university scientists. Another barrier is that academic scientists usually lack access to the funds and expertise needed to test the feasibility of their ideas for products.

By providing funding, training, and access to a nationwide network of know-how (including a unique mix of federal and industry expertise), the REACH program establishes the infrastructure to identify and foster the most promising biomedical technologies. Unlike traditional NIH research projects, REACH projects involve upfront consideration of issues relevant to commercialization, as well as continuous mentoring and industry-style project management with defined go/no-go milestones. REACH and its sister program, the NIH Centers for Accelerated Innovations\(^8\), have been given the task of developing best practice guidelines for evaluating and commercializing research that can be used across the country and tailored to institutions of various sizes.

The REACH program is expected to yield a crop of validated or “de-risked” medical advances that have been heavily vetted by experts and are ready for the next step — licensing to industry or a new startup company.

Beyond that, it aspires to fundamentally change the culture of research institutions by training more academic scientists who can recognize innovation, evaluate it, advance it, and effectively communicate the value proposition to investors and the public alike.

REACH began with a relatively modest investment of $9 million from the NIH that is matched by university resources. The three REACH hubs, located at our institutions (University of Louisville, Long Island Bioscience Hub, and University of Minnesota), began funding promising product development
projects just over a year ago. From the 400 or so that have been evaluated so far, the REACH pipeline now contains more than 60 experimental treatments and tests that target a wide range of health issues.

New drug candidates include small molecules aimed at cancers like pancreatic and colorectal driven by the RAS family of genes⁹, modified viruses that attack brain tumors known as glioblastomas¹⁰, nanoparticles for treating Alzheimer’s disease, and biologic therapies for ulcerative colitis. Other promising treatments under development include encapsulated insulin-producing islet cells for treating diabetes and acellular tissue grafts to minimize complications in patients undergoing dialysis for end-stage kidney disease. There are also novel technologies for assessing diseases, such as an imaging approach to aid in the surveillance of prostate cancer and a laboratory assay to classify antibiotic-resistant superbugs.

It’s too early to tell which of these will eventually reach the market, but there are already signs that REACH is affecting researchers who are taking part in the program. Anecdotal observations — a neuroscientist forming a startup to develop a novel local anesthetic and a chemical engineer teaming up with a cardiologist to pitch their idea at a “Shark Tank”-style forum — suggest that REACH can help transform the way academics think about their research.

The time for REACH-like programs is ripe for many reasons. Recent technological advances¹¹ — next-generation DNA sequencing, genome-editing, cell-based therapies, nanotechnology, 3-D printing, virtual reality, robotics, and artificial intelligence, to name a few — offer unprecedented opportunities for developing new medical products. We must put these capabilities to practical use to tackle the epidemic of chronic diseases our society now faces. The possibilities are already emerging: gene therapy to correct genetic diseases, wearable sensors to monitor well-being, and sensitive blood tests to detect cancer long before it becomes clinically apparent.

REACH can also help fill a significant gap in biomedical research education by educating participants about non-academic research careers and teaching them the management and communication skills that are valuable in many occupations.

Discoveries made in academia have led to a multitude of life-changing and lifesaving products¹². Clearly, not all ideas for products can — or should — be developed. But thousands of potentially transformative ones languish or fail to fulfill their potential. By helping us get smarter about research translation, programs like REACH can improve the success rate.

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Links
2. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4659410/
3. http://science.sciencemag.org/content/342/6165/1432.full
15. https://mn-reach.umn.edu/

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