



President's Council of Advisors on Science and Technology
Eisenhower Executive Office Building
Washington, DC 20502

FOR IMMEDIATE RELEASE
September 25, 2012

Contacts: Rick Weiss, 202 456-6037 rweiss@ostp.eop.gov
Phil Larson, 202 456-6043 plarson@ostp.eop.gov

President's Council of Advisors on Science and Technology Releases Report on Innovation in Drug Discovery and Development

The United States should set a goal of doubling the output of innovative new medicines that meet critical public health needs over the next 10 to 15 years, while continuing to increase drug safety, a presidentially appointed council of experts advised in a report released today. The council recommends a number of actions involving industry, academia, and the Federal Government.

While basic biomedical sciences have seen stunning progress in past decades, challenges remain in translating those scientific advances into practical solutions, according to the report—*Propelling Innovation in Drug Discovery, Development, and Evaluation*—produced by the President's Council of Advisors on Science and Technology ([PCAST](#)). The report assesses the reasons for that long-term trend.

To support innovation and accelerate the development of new therapies, the report makes a number of detailed recommendations aimed at bolstering the discovery and development of new therapeutic compounds; optimizing processes used by the Food and Drug Administration (FDA) to evaluate the safety and efficacy of candidate drugs; enhancing long-term monitoring of approved medicines; and enhancing public understanding about the benefits and risks of medicines.

“With improved collaboration among all the participants in the drug development ecosystem and optimization of drug-evaluation pathways, American researchers and companies should be able to accelerate the development of safe and effective drugs while also strengthening the U.S. economy,” said Eric Lander, who co-chairs PCAST.

The report notes that heart disease and stroke remain leading causes of mortality, many common cancers are still incurable unless they are caught in the earliest stages, and the vast majority of rare diseases lack effective therapies altogether. Infectious diseases, including those caused by antibiotic-resistant bacteria and viruses with pandemic potential, pose a constant threat of large-scale mortality. And treatments for psychiatric diseases, which impose a tremendous burden on society, are frustratingly limited in their efficacy, as are treatments for neurodegenerative diseases such as Alzheimer's.

All three major components of the drug development ecosystem—basic biomedical research in universities and research institutes, clinical research in hospitals, and drug discovery and development in the biopharmaceutical industry—are facing growing challenges as the time,

complexity, and cost of developing drugs have gone up, the report states. The rate of new-drug applications submitted by industry to the FDA, as well as new drug approvals, has remained relatively constant for 20 years. In an encouraging sign, however, the FDA approved 35 new medicines in the past year—among the highest totals in the past decade.

The report concludes there are two critical needs related to drug discovery and development that must be addressed to advance innovation:

- (1) Scientists need better methodologies and tools for translating basic biological insights into validated therapeutic targets and leads—a gap in the drug discovery and development pipeline that academic scientists often view as “too applied” and pharmaceutical companies often eschew as “too basic” to justify private investment.
- (2) Pharmaceutical developers and regulators need to incorporate new efficiencies into clinical trials of candidate medicines—complex and costly human studies that today constitute fully 40 percent of the biopharmaceutical industry’s R&D budget.

To achieve some of the report’s broader goals, PCAST recommends the creation of a public-private “Partnership to Accelerate Therapeutics,” involving representatives from the biopharmaceutical industry; the academic biomedical research and ethics community; physician societies and pharmacists; patient-focused research foundations and advocacy groups; healthcare providers and insurers; and the Federal Government. The Partnership would help identify and plan collaborative actions to speed drug development while balancing competing stakeholder interests and minimizing duplication of efforts.

In addition, the report concludes that the return on investment in certain disease domains may be too low to justify their pursuit by companies, even though the potential benefits for public health in these domains may be large. It recommends that the Department of Health and Human Services commission a study to assess potential mechanisms to encourage companies to tackle important medical challenges that may be financially unattractive.

“There is a tremendous need for new antibiotics, for example, but the potential market share for such medicines is typically small and their duration of use is typically short,” said PCAST member Christine Cassel, a physician who—with Lander and PCAST members Ed Penhoet and Rick Levin—oversaw a group of 30 outside experts who helped inform PCAST in its work.

The new report can be viewed at www.WhiteHouse.gov/ostp/pcast.

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