U.S. HOUSE OF REPRESENTATIVES
COMMITTEE ON SCIENCE, SPACE, AND TECHNOLOGY
SUBCOMMITTEE ON RESEARCH AND TECHNOLOGY

HEARING CHARTER

Policies to Spur Innovative Medical Breakthrough from Laboratories to Patients

Thursday, July 17, 2014
9:00 a.m. - 11:00 a.m.
2318 Rayburn House Office Building

Purpose

On Thursday, July 17, 2014, the Subcommittee on Research and Technology will hold a hearing to explore public and private sector efforts in basic, applied, translational, and clinical scientific research for medical breakthroughs discovered through interdisciplinary biomedical R&D combined with chemistry, physics, mathematics, computing, and engineering. The hearing will explore what public policies may spur more innovation and investment for medical breakthroughs.

Witnesses

Dr. Craig Venter, Founder, Chairman, and Chief Executive Officer, J. Craig Venter Institute, Synthetic Genomics, Inc., and Human Longevity, Inc.

Dr. Marc Tessier-Lavigne, President and Carson Family Professor, Laboratory of Brain Development and Repair, The Rockefeller University

Dr. Jay Keasling, Hubbard Howe Jr. Distinguished Professor of Biochemical Engineering, University of California, Berkeley; Professor, Department of Chemical & Biomolecular Engineering, University of California, Berkeley; Professor Department of Bioengineering, University of California, Berkeley; Director, Synthetic Biology Engineering Research Center

Dr. Harold Varmus, Director, National Cancer Institute at the National Institutes of Health

Hearing Overview

Understanding and treating disease and injury remains one of the most challenging problems facing the scientific community. Progress in the biological sciences has enjoyed a long symbiotic relationship with the physical and mathematical sciences; for example, physicists such as Lord Rayleigh, Hermann Helmholtz and Erwin Schrödinger made fundamental contributions to our understanding of physiology and medicine.[1]

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[1] From Photons To Perception: A Physicist Looks At The Brain, Dr. William Bialek. Available From http://online.itp.ucsb.edu/plecture/bialek/

Biomedical research has been crucial to advancing our understanding of cures to complex human diseases and injury. Publicly-funded biomedical research is estimated at approximately $50 billion annually (with the NIH budget $30.1 billion in FY2014), while privately-funded biomedical research in the U.S. is estimated at over $70 billion annually.[2]

Medical sciences research is becoming an increasingly interdisciplinary field, with important contributions from fields as diverse as the chemistry, physical sciences, applied mathematics, computer science, and engineering,[3] fields which have enjoyed public funding support from the National Science Foundation (NSF). Each of these disciplines has furthered our understanding of biological mechanisms, thereby allowing researchers to move towards an integrated picture of the human body. In FY 2014, NSF's budget for the Biological Science Directorate is over $720 million, with several other Directorates also funding biological-related research.

One aspect of biomedical research for medical breakthroughs involves the creation of new drugs and vaccines. In the past 40 years, 153 FDA-approved drugs, vaccines, and new starts for existing drugs were discovered from research done at public sector research institutions (PSRIs). Moreover, roughly 10-20% of all drugs involved in new-drug applications approved between 1990-2007 were the result of research performed by PSRIs. Of the 252 new drugs approved by the FDA between 1998 and 2007, 31% of the 118 drugs were considered "scientifically novel" and a result of research originally performed at universities.[4] Over the FY2002-FY2012 time period, $917.7 million in royalties were generated from licenses on NIH-owned patents[5]. To leverage these Federal investments, legislation such as the Stevenson-Wydler Act and Bayh-Dole Act have facilitated the commercialization of government-funded R&D, through policies that encourage collaboration between government, universities, and industry.

Modern advances in the physical, mathematical, engineering and computer sciences have also converged to create new research fields that advance biomedical science. One such example is the field of *synthetic biology*, where living systems are seen as building blocks that can be retrieved from their natural context, reshaped, standardized for a given specification and then re-purposed for a specific task such as making a drug.[6]

Synthetic biology represents a new approach to biological engineering, a key enabling technology with the potential to fundamentally change the approach, tools and techniques of modern biological research and innovation, to the benefit of society. Because of its

[2] http://www.nejm.org/doi/full/10.1056/NEJMp1311068

[3] http://www.cancer.gov/aboutnci/director/speeches/impact-of-physics-1999

[4] http://www.nejm.org/doi/pdf/10.1056/NEJMsa1008268
http://news.heartland.org/sites/all/modules/custom/heartland_migration/files/pdfs/28819.pdf

[5] Federal R&D, Drug Discovery, and Pricing: Insights from the NIH-University-Industry Relationship, CRS Report, November 30, 2012

[6] "Synthetic Biology", Discover Magazine, October 2013, Vol 34, No 8, pp 48-54.

interdisciplinary nature, this field has the potential to enhance many industrial sectors beyond medicine, including agriculture and environmental remediation.[7]

Economic Impact

The economic implications for scientific research that can cure or alleviate the effects of human diseases or injuries is significant. For example, the monetary cost of dementia, including Alzheimer's disease, in the United States ranges from $157 billion to $215 billion annually. The House Research and Technology Subcommittee held a hearing in July 2013 to discuss interdisciplinary brain science research.

Incentives for innovation in the industrial community clearly have contributed to substantial research and development by the biopharmaceutical and biotechnology sectors. In 2012, research and development spending by the biopharmaceutical industry in the United States totaled around $63.1 billion.[8] Domestic R&D spending for members of the Pharmaceutical Research and Manufacturers of America (PhRMA) was an estimated $37.5 billion, with 22.7% of domestic sales reinvested in research and development.[9] U.S. biotechnology companies spent $19.3 billion on R&D, and produced $63.7 billion in revenue in 2012.[10] For an industry that practically did not exist 30 years ago, biotechnology in the United States has provided new products and processes for the international marketplace, including more than 300 biotech drugs and vaccines.[11]

Issues for Consideration

The science community has a number of metrics aimed at measuring scientific quality, including the number of publications, citations, Nobel Prizes, society-specific honors, and others. Economic output metrics, on the other hand, have proven more difficult to gather and characterize and have been challenging for policymakers seeking to develop research and development (R&D) policy decisions. Given the desire for more evidence-based budgeting of R&D, recent analysis has shown that there were meaningful differences in the rate and quality of patenting across NIH from 2003 to 2012.[12] Patents are positively correlated with higher levels of regional employment, start-up companies, and greater economic impact, and may be a useful metric for policymakers when assessing effectiveness of public investment.

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[7] "Next Steps for European synthetic biology: a strategic vision from ERASynBio" Report, April 2014. Available From http://www.bbsrc.com/web/FILES/Publications/1404-era-synbio-strategic-vision.pdf

[8] Bureau of Economic Analysis, U.S. Department of Commerce, National Income and Product Accounts Tables, Table 5.6.5. Private Fixed Investment in Intellectual Property Products by Type. Available From http://www.bea.gov/iTable/iTable.cfm?ReqID=9&step=1#reqid=9&step=3&isuri=1&903=331

[9] 2014 Profile: Biopharmaceutical Research Industry, PhRMA, "Key Facts," and p. 74, http://www.phrma.org/sites/default/files/pdf/2014_PhRMA_PROFILE.pdf

[10] Beyond Border: Matters of Evidence, Biotechnology Industry Report 2013, Ernst and Young. Available From http://www.ey.com/Publication/vwLUAssets/Beyond_borders/$File/Beyond_borders.pdf

[11] "The Biopharmaceutical Industry: Creating Research, Progress and Hope". Available From http://www.phrma.org/about/biopharmaceutical_sector

[12] Kalutkiewicz M, Ehman R, "Patents as Proxies: NIH hubs of innovation" Nature Biotechnology 32, 536–537 (2014). Available From http://www.nature.com/nbt/journal/v32/n6/abs/nbt.2917.html

Rothwell, J. et al. Patenting Prosperity: Invention and Economic Performance in the United State and its Metropolitan Areas (Brookings Institution; February 2013). Available From http://www.brookings.edu/research/reports/2013/02/patenting-prosperity-rothwell

There are also significant concerns that R&D budgets—especially for the pharmaceutical industry—are being cut in private sector companies in order to return profits to their shareholders.[13] R&D investments often represent the long-term seed corn for future breakthroughs, while profit-taking is short-term gain for the shareholders. Thus, leadership within companies must carefully balance short-term and long-term interests of the company and their shareholders.

Many pharmaceutical companies have developed alternative means of acquiring new technologies, including an increasing number of alliances between large businesses, small companies, government laboratories, research hospitals, and universities. [14] Such partnerships can augment funding sources from private and public sectors, increase technology transfer, stimulate additional innovation, lead to new products and processes, and expand markets. On the other hand, such collaborations have some downsides such as unfair advantages and conflicts of interest, among others.[15]

[13] "Do Drug Companies Make Drugs, or Money" June 2, 2014 New York Times. Available From http://dealbook.nytimes.com/2014/06/02/do-drug-companies-make-drugs-or-money/?_php=true&_type=blogs&_r=0

[14] Patricia M. Danzon, Sean Nicholson, Nuno Sousa Pereira, Productivity in Pharmaceutical-Biotechnology R&D: The Role of Experience and Alliances, National Bureau of Economic Research, Working Paper 9615, April 2003, 5. Available From http://www.nber.org/papers. See also, Nadine Roijakkers and John Hagedoorn, "Inter-firm R&D Partnering in Pharmaceutical Biotechnology since 1975: Trends, Patterns, and Networks," Research Policy, April 2006, 444.

[15] Federal R&D, Drug Discovery, and Pricing: Insights from the NIH-University-Industry Relationship, Wendy H. Schacht. Available From http://www.crs.gov/pages/Reports.aspx?PRODCODE=RL32324&Source=search