



HEARING TESTIMONY

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**ON BEHALF OF THE
BIOTECHNOLOGY INDUSTRY ORGANIZATION**

**BEFORE THE HOUSE OF REPRESENTATIVES COMMITTEE ON APPROPRIATIONS
SUBCOMMITTEE ON LABOR, HEALTH AND HUMAN SERVICES,
EDUCATION, AND RELATED AGENCIES**

***BUDGET HEARING – DEPARTMENT OF HEALTH AND HUMAN SERVICES – NIH
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Good morning Chairman Rehberg, Ranking Member DeLauro, Members of the Committee, ladies, and gentlemen. I am Scott Koenig, President and Chief Executive Officer of MacroGenics, Inc. and Chairman of the Board of Applied Genetic Technologies Corp (AGTC). I am appearing before this Committee on behalf of the Biotechnology Industry Organization (BIO). BIO represents more than 1,100 companies, academic institutions, state biotechnology centers, and related organizations in all 50 states.

I am a scientist, physician, and entrepreneur and have worked both at the National Institutes of Health (NIH) and in the biotechnology industry for the past twenty-eight years. During my career I have held positions including Senior Vice President of Research at MedImmune, Inc., co-founder and CEO of MacroGenics, Inc., and Board member of AGTC. During this time I have been involved in the development of multiple biological products, such as a therapy to prevent a fatal respiratory viral illness in premature infants, a vaccine to prevent cervical cancer, and a number of other promising biological therapeutics still in development such as treatments for juvenile diabetes and many types of cancer.

It is my privilege to testify before the Subcommittee today to discuss the importance of funding NIH and specifically to talk about the newly-formed National Center for Advancing Translational Science (NCATS). I will discuss the opportunities and potential benefits of NCATS working with the scientific community to find

solutions to critical scientific barriers and developing novel scientific approaches, methodologies, and tools to improve efficiencies in how we, as an industry, research and develop breakthrough treatments and therapies.

Importance of Investing in Biomedical Research

The importance of supporting biomedical research and innovation and the development of new treatments and therapies in the United States cannot be overstated, especially in a time where we are driving towards building a 21st century economy while simultaneously facing increased competition around the globe to sustain our world leadership in biomedical innovation. Life science R&D provides high-wage jobs at both public research institutions and in the biotech companies that typically locate near centers of academic research. The indirect effects of increased research funding on the regional economy are significant. For example, sponsored biomedical research directly generates jobs in the host institutions, and indirect and induced job creation in the region amounts to additional job growth. In fact, the nation's 1.42 million bioscience jobs support an additional 6.6 million jobs in the United States, resulting in a total employment impact of over 8 million jobs.¹

It is imperative that we as a country continue to invest in scientific discovery and innovation. Federally-supported biomedical research builds the foundation of scientific and clinical knowledge that is widely communicated and used to improve the development of diagnostics, treatments, and cures. The federal government funds biomedical research in the United States primarily through the NIH. Decreasing investment in NIH-supported research will significantly inhibit our ability to make new scientific discoveries that could advance clinical and translational knowledge in how we diagnose and treat diseases. NIH-supported research advances the early stages of development of new biomedical products. This provides the foundation from which these scientific findings can then be transferred to the industry to research and develop these early-stage discoveries into the next generation of treatments and cures. This collaborative ecosystem also serves to create numerous direct jobs within the companies themselves as well as the indirect job creation with the numerous laboratories and suppliers contracted by the companies.

The NIH is the nation's premier biomedical research agency and there is no private sector alternative for much of the basic research that NIH supports. Ensuring that NIH is well-funded is necessary to sustain the public-private collaboration that is transforming biomedical discoveries into innovative treatments for patients. It is critical that in an environment of budgetary constraint we do not lose the next generation of discoveries that offer solutions to one of nation's leading cost drivers – patients suffering from chronic and debilitating diseases. For example, Medicare is expected to spend over \$100 billion in 2012 caring for individuals suffering from

¹ Battelle/BIO State Bioscience Initiatives 2010. Battelle Technology Partnership Practice, May 2010.

Alzheimer's disease.² Delaying the onset of Alzheimer's by just five years would save \$50 billion per year.³ By 2030, almost one out of every five Americans – some 72 million people – will be 65 years or older. And as almost 75 cents of every health care dollar spent is for taking care of individuals suffering from a chronic disease, it could not be clearer that we have a national imperative to find new solutions in how we treat patients and diseases. This can only be achieved with continued federal investment in biomedical research and policies that incentivize innovation by the biopharma industry. However, after nearly a decade of budgets below biomedical inflation, NIH's inflation-adjusted funding is close to 20 percent lower today than in FY 2003. Decreasing investments in biomedical research will have long-term impacts, as making scientific discoveries and developing those discoveries into treatments and therapies that will improve the lives of patients is a long and difficult process. It is a process that requires sustained commitment and investment.

We are facing unprecedented competition from around the globe to be the leader in biomedical research. In 2008, China pledged to invest \$12 billion in drug development,⁴ and in 2011, the Chinese government named biotech one of seven industries that will receive \$1.7 trillion in government funding over the next five years.⁵ The European Union's Innovative Medicines Initiative is pumping \$2.65 billion into Europe's biopharma industry⁶ and India's Bioconnect initiative has funded over 200 new biopharma projects.⁷ While America has developed more cures and breakthrough medicines than any other country and is home to over 2,500 biotech companies, this is not a position that will be sustained without continued investment and policies focused on supporting and incentivizing the next generation of biomedical discoveries, treatments, and cures. Only by continuing to invest in the biomedical research and development ecosystem will we improve public health, find new solutions in how we treat patients suffering from costly and debilitating diseases, maintain global leadership, and be in a position to increase jobs in this growing industry here in the United States.

NCATS: Opportunity to Engage Industry and other Stakeholders in Finding Solutions to Critical Scientific Barriers

BIO has been actively engaged in conversations with NIH since the concept of creating a new institute focused on translational research was first presented by NIH's Scientific Management Review Board in December of

² Alzheimer's Association, March 2012 Fact Sheet. http://www.alz.org/documents_custom/2012_Facts_Figures_Fact_Sheet.pdf

³ Journal of the American Geriatrics Society, 2002, 50:1-7. via Research!America, "Facts about Alzheimer's Disease." <http://www.researchamerica.org/uploads/factsheet4alzheimers.pdf>

⁴ Daverman, Richard. "China Launches 'Mega Program' to Fund Drug Development." ChinaBio Today. 9 November 2008. <http://www.chinabiotoday.com/articles/20081109>

⁵ Buckley, Chris. "China to invest US\$1.7 trillion over 5 years in 'strategic sectors': US official." The China Post. 23 November 2011. <http://www.chinapost.com.tw/business/asia-china/2011/11/23/323724/China-to.htm>

⁶ Hodgson, John. "€2 billion IMI launched with European pharma." *Nature Biotechnology* 26, 717-718 (2008).

⁷ Dandekar, Vikas. "India Draws Lessons From China To Help Foster Biotech Industry." PharmAsia News. 7 February 2012.

2010. Director Collins, senior staff at NIH, and, more recently, NCATS Acting Director Dr. Insel and Acting Deputy Director Dr. Hudson have been very generous with their time and have made a concerted and consistent effort to meet with BIO and listen to BIO's concerns and recommendations.

The stated mission of NCATS is "to catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions." NIH's press release in December 2011 stated that "NCATS will serve as the nation's hub for catalyzing innovations in translational science. Working closely with partners in the regulatory, academic, nonprofit, and private sectors, NCATS will strive to identify and overcome hurdles that slow the development of effective treatments and cures."⁸ Currently, the new NCATS has seven programs: the Molecular Libraries Program; Therapeutics for Rare and Neglected Diseases (TNRD); Bridging Interventional Development Gaps; the Office of Rare Diseases Research; Clinical and Translational Science Awards; Food and Drug Administration (FDA)-NIH Regulatory Science; and the Cures Acceleration Network (CAN). Additionally, NCATS has announced three new initiatives: a NIH- Defense Advanced Research Projects Agency (DARPA)-FDA collaboration to develop a tissue chip for drug screening; drug rescuing and repurposing; and target validation. It will be important for NCATS to establish a very focused set of priorities for each of these programs that will individually and collectively serve to improve clinical research and development processes.

BIO is supportive of the overarching goal of NCATS and agrees with report language included in the FY 2012 appropriations and statements made by NIH that research initiatives undertaken by NCATS should not be duplicative of the research and development done by industry. The focus should be on tools and methodologies that will serve to help the biopharma industry as a whole to more efficiently and effectively conduct research and development. Today, it requires an average of 10 to 15 years and \$800 million to over \$1 billion to develop a new drug, and not only is that cost increasing, it is increasing at an alarming rate.^{9,10,11,12} In part, this increase in cost can be attributed to the increased complexity of regulatory requirements. For example, between 1999

⁸ "NIH establishes National Center for Advancing Translational Sciences." NIH Press Release, 23 December 2011. <http://www.nih.gov/news/health/dec2011/od-23.htm>

⁹ "Returns to R&D on New Drug Introductions in the 1980s." *Journal of Health Economics* 13, no. 4 (1994): 383-406

¹⁰ H.G. Grabowski, J. Vernon, and J.A. DiMasi, "Returns on Research and Development for 1990s New Drug Introductions," *Pharmacoeconomics* 20, supp. 3 (2002): 11-29

¹¹ J. Dimasi and H Grabowski J "The Cost of Biopharmaceutical R&D: is Biotech Different?" *Managerial and Decision Economics* no 28 (2007): 469-79

¹² Munos, Bernard. "Lessons from 60 years of pharmaceutical innovation." *Nature Reviews Drug Discovery* 8, 959-968 (December 2009).

and 2005 the average length of clinical trials grew by 70%.¹³ The primary metric for determining the ultimate success of NCATS is whether the initiatives undertaken yield significant reductions in time and expenses in the development of new therapeutics, while expanding the terrain of promising novel targets and pathways, and thus improve the delivery by drug developers of the next generation of medicines to patients.

In order for NCATS to achieve its goal to enhance the development of innovative medicines, it must develop substantive partnerships and collaborations with industry, regulators, principal investigators, life science investors, and patient organizations. It is crucial that NCATS' research priorities are developed with input from those that are in the trenches and thus most knowledgeable about where scientific barriers and addressable inefficiencies exist in the development process. Among the inaugural initiatives highlighted by NCATS is one to identify methods and tools that would enable drug developers to better predict toxicology in humans earlier in the drug development process. Such an initiative should be universally endorsed. To uncover and quantify safety risks at a preclinical or early clinical testing stage for any new molecule in a predictable manner, using laboratory tools in the absence of significant patient exposure or large animal requirements, is a winning proposition for patients, health providers, the government, and the drug development industry. The NCATS collaboration with DARPA and FDA to develop a new chip technology that would allow researchers to rapidly screen for new and safe classes of compounds more efficiently and predictably than current methods is one such approach that could vastly improve the drug development process across the board. Likewise, aligned in these efforts are approaches which would identify and validate drug targets more efficiently. A resounding success in these latter two proposals would help maintain a robust pipeline of potential breakthrough treatments and cures. Certainly, the input from industrial collaborators on these and other research priorities as they are implemented and mature would enhance the chance of achieving a salutary outcome.

Another area where collaboration between NCATS and industry is vital is the proposal to repurpose and rescue drugs. Industrial partners will be required to develop, manufacture, and market these drugs and therefore, we encourage NCATS to identify partners in industry early, so they may be included in addressing issues related to intellectual property, quality assurance, and design and conduct of clinical trials for safety and efficacy, especially those conducted at any significant scale. NCATS should not seek to create a new internal infrastructure to manufacture compounds. Rather NCATS should engage partners that can help ensure the quality and scalability of manufactured clinical materials as well as the design of clinical programs that meet both industry and FDA standards. Certainly, in the rare cases where NCATS advances a product into Phase 2

¹³ Tufts Center for the Study of Drug Development. 2008. "Growing Protocol Design Complexity Stresses Investigators, Volunteers." Impact Report. 10.1.

studies without these notions in mind, it may impede the registration of new product candidates in a timely and cost-efficient manner.

It is critical that efforts undertaken by NCATS are not duplicative of the biopharma sector. This would not only be inefficient, but impractical as testing and developing a single novel treatment and therapy is extremely resource-, time-, and cost-intensive. We would encourage NCATS to focus primarily on pre-clinical and early clinical Phase 0 and 1 studies with new molecular compounds, with the exception of work being done by TRND where efforts on rare diseases may require additional clinical studies in order to attract industry and investment partners. NCATS should also not be duplicative of translational research being done by NIH grantees (universities, medical research institutions, *etc.*) or other NIH institutes. However, having NCATS coordinate and leverage research initiatives being conducted outside of NCATS that would help achieve its mission of advancing translational science would be beneficial. Additionally, NCATS should engage with other public-private partnerships such as the Biomarkers Consortium and Reagan-Udall Foundation that are focused on advancing translational and regulatory science to again ensure efforts are not being duplicated as well as to look for synergies in research opportunities. NCATS could provide a valuable service in acting as point of contact and convener for public-private partnerships, industry, and other stakeholders to reach out to NIH with potential research collaborations and assist in establishing a dialogue between public, non-profit, and private sectors about what the key scientific barriers are in developing treatments for specific diseases with the goal of developing comprehensive strategies leveraging all stakeholders' expertise and resources to find solutions.

At present the vast majority of the NCATS budget is dedicated to the Clinical Translational Science Awards (CTSAs). BIO has a continued interest in learning how the CTSAs will be utilized to advance the NCATS mission. We believe that the CTSAs should be utilized aggressively to conduct early clinical research and develop novel tools and approaches that would improve efficiencies in clinical trial research and development. We encourage the engagement of CTSAs in clinical investigations that help to validate biomarkers, identify the impact of specific genes or epigenetic factors that would predict clinical efficacy or safety signals within specific classes of molecules, or establish principles for conducting clinical studies with innovative designs, including those demonstrating synergies among classes of drugs that lead to better therapeutic options for patients.

NCATS has a real opportunity to take a leadership role in improving the science of drug development that will enable industry to more efficiently develop the next generation of medicines. However, the success of these discoveries will only be realized if they are adopted and advanced by industry. For those NCATS endeavors

that have impact on regulatory decisions and approvals, such as implementation of new methodologies and tools that affect current practices in toxicology, or the utilization of surrogate biomarkers for objective clinical endpoints, or novel designs in the conduct of clinical trials that accelerate drug evaluations, they must be accepted by the FDA. Thus, it is imperative that in addition to collaborating with industry, academia, investors, and patients, NCATS must work closely with FDA in fostering the development and adoption of these new tools and practices, including those who are participate in the FDA-NIH Joint Leadership Council.

Lastly, I would like to briefly discuss the Cures Acceleration Network (CAN) program. BIO has long supported the funding and implementation of CAN and believes the authorization that this law provides to NIH – matching grant and other transactions authorities – will be very beneficial to NIH. Additionally, it provides a unique funding opportunity for industry and other stakeholders working on innovative drugs to treat diseases of critical import to public health or on drugs that otherwise might not be able to obtain sufficient private sector funding for development (*e.g.*, ultra rare diseases). Beyond providing critical funding, this program creates a collaborative space among the grantee, NIH, and FDA not only to develop high need cures, but also to develop those drugs using modern clinical research development tools and approaches. BIO is interested in continuing to work with NIH as this newly established program evolves.

Conclusion

In conclusion, BIO believes there is a real opportunity, if it is implemented correctly, for NCATS to collaborate with industry, academia, and patient organizations to systematically identify key scientific areas of research such as predictive toxicology and tools and methodologies to improve clinical trial efficiency, and to accelerate target identification and validation, that would serve to enhance the drug development process as a whole. These collaborations must be systematic and transparent with a clear understanding and opportunity on how industry can engage with NIH on this initiative. Input should be sought from industry, public-private partnerships, and others involved in advancing translational/regulatory science, and they should be consulted when establishing research priorities. We encourage NIH and NCATS leadership to continue working with advocacy, trade, patient, and academic organizations to ensure a truly collaborative environment and to maximize coordination among the various programs under NCATS to ensure that each is leveraged in an efficient manner to advance the NCATS mission.

Thank you for the opportunity to testify before you today. We look forward to continuing to work with Congress and the NIH as this new Institute takes shape with the hope that NCATS is a productive and collaborative entity whose research outcomes improve the process of developing drugs. This will spur private

sector investment back into biomedical drug development and, most importantly, improve our ability to deliver treatments and cures to patients.