Hearing Testimony
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On behalf of
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Subcommittee on Investigations and Oversight

SBIR: Advancing Medical Breakthroughs

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Chairman Altmire, Ranking Member Graves, and Members of the Subcommittee:

Thank you for the opportunity to provide testimony before you regarding the nation's Small Business Innovation Research (SBIR) Program and its impact on advancing medical breakthroughs.

I am Dr. Melvin Billingsley, president and Chief Executive Officer (CEO) of the Life Sciences Greenhouse of Central Pennsylvania (LSGPA). I am the founding CEO of LSGPA, and, along with my fellow CEOs John Manzetti of the Pittsburgh Life Sciences Greenhouse and Barbara Schilberg of BioAdvance, have worked diligently to support and invest in emerging life science companies in Pennsylvania. I have considerable experience with the National Institutes of Health (NIH) grant system, and have been grant recipient from NIH. I have served as a reviewer for the United States Department of Defense (DoD), for NIH, and for NIH SBIR awards; thus, I am familiar with the importance of the SBIR program as a key catalyst for commercialization of innovative, life-saving technologies.

Pennsylvania's Life Sciences Greenhouse (LSG) program was created through a one-time set aside of $100 million of Pennsylvania's share of the Tobacco Master Settlement Agreement. The LSGs were designed as a flexible mechanism for the commercialization of life science business opportunities by accelerating technology transfer, enhancing collaboration, and attracting new business. There are three regional greenhouses in Pennsylvania: the Pittsburgh Life Sciences Greenhouse; BioAdvance: the Biotechnology Greenhouse of Southeastern Pennsylvania; and the Life Sciences Greenhouse of Central Pennsylvania. Each provides services based on the needs of our respective regions; however, we each provide direct early-stage investment for emerging companies. I have provided with this testimony a fact sheet on each greenhouse.

I am testifying today at the request of Pennsylvania Bio, the statewide life science association representing the interests of the Commonwealth's research, biotechnology, medical device, diagnostic and pharmaceutical industry. The Association represents more than 300 organizations across the commonwealth. Pennsylvania is a major recipient of funding from the NIH, ranking fifth overall in the past year with more than $1.4 billion in funding. In addition, in

2005, Pennsylvania companies received significant SBIR funding from the NIH; there were 45 Phase I projects totaling $6.9 million, and 31 Phase II projects totaling $15.3 million.

Needs of Emerging Companies in Bringing Therapies to Patients

Before we engage in a discussion about the value of the federal SBIR program, it is important to review the needs of emerging companies. I recognize that you may have heard this in other forums, but to bring a new therapy to patients, which is the goal of every company that is engaged in medial research, it takes an enormous amount of patience, time and capital. The Tufts Center for the Study of Drug Development estimates between 8 to 12 years and between $800 million to $1.2 billion to bring a product through clinical trials to FDA approval, if a company is fortunate.

Emerging companies are going through this process without any product revenue and rely solely on other means to fund the company and the research. The intellectual property of emerging life science companies is often the only basis for future value. Consequently, many of these companies will need to access the capital markets at some point in order to advance their products.

Companies first need early-stage risk capital, which is often the toughest to find in the market. The Life Sciences Greenhouses can address this gap. As a result, the three Pennsylvania Greenhouses collectively have seen a huge demand for this funding. The greenhouses have committed $35 million to 149 projects in Pennsylvania, but the need for investment far exceeds the funds available. As of June 2007, the greenhouses have received 814 applications requesting a total of $314.6 million. Our early-stage funding helps companies reach that next step in funding and product development:

- The Greenhouses' portfolio companies have attracted more than $500 million in additional funding beyond the greenhouse investment.
- Each dollar invested by the greenhouses has leveraged currently at least $10 dollars from additional sources, and this number continues to increase. This is a greater than 10:1 leverage, which is exceptional.
- The greenhouses have helped to create/retain 2,363 jobs in the commonwealth, nearly 700 of which were created through the greenhouses' investment portfolios.
- SBIR and other Federal funding remains a critical funding mechanism for emerging companies. From 2003-2007, LSG-supported projects have attracted more than $78 million in Federal funding (2007 Annual Report).

After seed funding from the LSGs and other programs such as SBIRs, most companies need funding that is often best met via professional venture capital (VC) investment. In Pennsylvania, VC investment in the life science industry has been on the rise, reaching a high of $476 million in 2006. According to Pennsylvania Bio's 2007 report on the life sciences industry in the Commonwealth, the life sciences accounted for 60 percent of all VC funding in Pennsylvania in 2006.

Outstanding, life-saving research is happening in our young companies in Pennsylvania, and we need to advance this research to the commercial market, where it can impact the health of our citizens.

Role of SBIR Funding

The SBIR program was enacted in 1982, and for nearly 20 years small, domestic life science companies successfully competed for these grants. SBIR grants, along with other government programs, can play a significant role in the funding continuum for emerging life sciences companies.

For example, in Pennsylvania, Yaupon Therapeutics, a BioAdvance-supported company, has progressed four therapeutic programs using approximately $14 million in funding over the last five years. Yaupon has benefited from the larger Phase II SBIR grants, including a $920,000 Phase II SBIR grant for a tobacco addiction compound in 2005. The company also received a $700,000 Orphan Drug grant in 2006 for a different program. Finally, the NIDA has been funding the development of a therapeutic agent to treat methamphetamine addiction, which is scheduled to begin Phase II trials. This funding assistance has been critical to the company's ability to move these programs concurrently. Now that the programs have progressed into the clinic, the company has been able to attract $15 million in venture capital.

Similarly, Azevan Pharmaceuticals, Inc, supported by LSGPA, has received an $800,000 NIH Phase II SBIR grant to develop novel therapeutics for aggression and anxiety. The company's lead compound has just completed Phase I clinical trials, and several of the pre-clinical studies were supported in part by the National Toxicology Program via the NIMH. This company has also attracted venture investment, which is needed to progress through Phase II clinical studies. Although the SBIR funding is significant, the amount of funds needed to complete clinical trials is a significant hurdle, and one best met via venture capital.

Launched in 2002, the Pittsburgh Life Sciences Greenhouse (PLSG) SBIR Advance Program is the only southwestern Pennsylvania resource dedicated to the specific needs of life sciences entrepreneurs. SBIR Advance is designed to enhance an entrepreneur's existing understanding of the SBIR Phase 1, Phase II, and Fast-Track proposal processes. Since inception, 110 companies have participated in the SBIR Advance Program which is directly responsible for bringing $13 million of non-dilutive SBIR funding into the region. One of those companies, Cohera Medical, Inc. is a PLSG supported medical device company whose patented product, TissuGlu™, is currently in pre-clinical testing and is designed to adhere tissues to prevent fluid accumulation in deep wounds. Cohera has closed a series A financing for $6.79 million and has been awarded two Phase I grants for $309,838 and was just funded a Phase II grant for $1.6 million with total SBIR support nearing $2 million. The SBIR funding has been critical to TissuGlu™'s pre-clinical testing and use to create a variety of products that meet surgeons' needs across many specialties.

Improvements to the program

Even the most successful program can be improved, and since recent administrative rulings by the Small Business Administration have weakened the SBIR program's ability to support life science innovation, we see areas for improvement within the SBIR program. Two specific areas need to be addressed in order to strengthen the program:

- Eligibility for venture-backed companies needs to be restored.
- Larger grant programs need to be fostered to help address "the valley of death" as companies seek venture capital funding.

Restore eligibility for venture-backed companies

New interpretations set in place in 2003 preclude many companies that are more than 51 percent venture backed from competing for SBIR grants. We've seen this impact in Pennsylvania, where companies have had to turn down SBIR grants and in turn terminate promising research. BioRexis Pharmaceutical Corporation is one such company. BioRexis had received VC funding to advance its lead product for Type II diabetes. The company had an additional program it was researching for a botulism anti-toxin. This was a program of great interest to the Department of Defense and in 2004, BioRexis received a $980,000 SBIR grant to explore the development of a long-acting inhibitor of botulism. Because of the company's venture capital investment, it was unable to draw down this grant, and the program was halted.

The BioRexis experience illustrates a particular need for venture backed companies. When venture capitalists invest in a company, it is often to advance the company's lead product and move the company more quickly to an "exit": an IPO, FDA approval, an acquisition or merger. Companies, though, are often looking at other indications for their technology or are advancing a second research project, as was BioRexis. SBIR funding can be enormously important for a second project. There is always the risk that a company's lead product will fail. We have many examples of this in Pennsylvania, most notably two of our successful "Pennsylvania-born" companies, Cephalon and Centocor. Each failed to receive FDA approval on their very first products, but because each company was able to successfully advance another project, both are successful, thriving companies today.

Since 2003, the life science industry has been seeking to redress this interpretation. We greatly appreciate Congressman Altmire's support with HR 3567, the Small Business Investment Expansion Act, and we thank the House for passing this legislation. I encourage the Senate to take up this legislation at its earliest convenience.

Address the "Valley of Death"

Many of you may have heard the term the "valley of death" as it relates to life science company financing. Companies can use the early-stage risk funding and government grant programs to advance companies to the point of human clinical trials, but large amounts of capital are needed to bring a promising product through the development process. Venture capitalists in recent years are trending toward coming in later in the development process. The period between when a company completes preclinical work and the later stage research and development is known as the "valley of death."

Phase I and Phase II funds can often be insufficient to get to the early clinical stages. A larger grant pool, such as a Phase II B program can help bridge this funding and attract venture capital earlier. This was the original intent of the federal SBIR program—early stage support leading to commercialization and higher capitalization. However, the long timelines and regulatory atmosphere for life science products presents a unique challenge for an SBIR-funded company. It is important to recognize that the significant capital risk occurs well beyond the early stage trials and preclinical development.

To this end, the NIH needs to maintain its flexibility in the SBIR program. Different award sizes are needed for different kinds of research support. For some Agencies, award sizes may not need to be as large. For some life science research, awards will need to be much larger. Flexibility is critical to maintaining a successful SBIR program. The amounts should always be commensurate with what the science and technology require. Artificial caps could threaten innovation.

Summary

The changes proposed by the Small Business Committee will greatly enhance the impact of SBIR companies in the life science area. SBIR grants have several positive impacts. First, Phase I capital can be used to develop products to the point of proof of principle, allowing key data to be generated in support of commercialization and technology transfer. This early validation of a technology via the peer-review system affords a level of technical approval and acceptance. Second, the critical SBIR phase II funds allow the leap to more extensive data generation that can warrant early stage investments such as those by the LSGs. This stage still lacks sufficient funding to complete all of the necessary preclinical trials needed prior to initiation of regulated trials, however. Programs such as the competitive Phase II B program provide enhanced levels of funding, matched at least 1:1 with private funds, to carry on preclinical trials needed to receive FDA approval to move the drug or device into the clinical trials area. Third, the SBIR programs need to recognize that venture-backed and other professional equity funds are needed to generate the amounts of funding needed to propel a company into the early stages of clinical trials. This is a high risk, high cost endeavor.

Thus, the research and development supported by the basic investments in NIH and National Science Foundation can be translated towards commercialization via programs such as the SBIR and Small Business Technology Transfer (STTR) grants. However, in order to maximize the impact of innovative technologies on human health, and to recognize the significant risk involved in new product development, we strongly recommend that SBIR programs reflect the intrinsic risks and rewards in the complex and costly system of regulatory approval of new products to treat disease.