Hearing Testimony
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On Behalf Of
The Biotechnology Industry Organization (BIO)

Before the House Committee on Science and Technology's
Subcommittee on Technology and Innovation

"Small Business Innovation Research Reauthorization on the 25th Program Anniversary"

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Chairman Wu, Ranking Member Gingrey, and Members of Science and Technology Subcommittee on Technology and Innovation:

I appreciate the opportunity to testify before the Subcommittee today regarding the Small Business Innovation Research (SBIR) grant program. For more than twenty years the SBIR program has served as a platform by which innovative, small companies can compete to participate in federal research and development.

My name is Dr. Gary McGarrity, I am the Executive Vice President of Scientific and Clinical Affairs at VIRxSYS. VIRxSYS is a private biotech company whose mission is to develop gene therapies using its proprietary lentiviral vector delivery system. We have completed Phase I safety testing and are now in Phase II clinical trials testing the first application of our gene therapy technology against HIV. I have16 years experience with biotech companies and an additional 14 years of in-depth scientific experience. Prior to joining VIRxSYS, I was the CEO of Intronn, Inc., which developed products to fight cystic fibrosis.

I am testifying today on behalf of the Biotechnology Industry Organization (BIO), an organization representing more than 1,100 biotechnology companies, academic

institutions, state biotechnology centers and related organizations in 50 U.S. states and 31 other nations. BIO members are involved in the research and development of health care, agricultural, industrial, and environmental biotechnology products. The overwhelming majority of BIO member companies are small, early stage research and development oriented companies pursuing innovations that have the potential to improve human health, expand our food supply, and provide new sources of energy.

Biotechnology Companies' Aggressive Capital Needs:

The largest obstacle to delivering on the scientific promise of biotechnology is accessing sufficient capital to fund research and development. BIO has over 600 emerging companies in its membership that have fewer than 350 employees and do not yet have a product on the market. In the absence of product revenue, biotechnology companies are almost entirely reliant on the capital markets or other sources of non-dilutive financing to fund research and development. This is particularly challenging at the earliest, highest-risk stages of research and development.

Promising biotechnology research has a long, arduous road from preclinical research, through Phase I, safety, Phase II, efficacy, and Phase III broader population clinical trials, and ultimately to FDA approval of a therapy. It is estimated that it takes 97.7 months, or 8 years to bring a biotechnology therapy to market and costs between $800 million and $1.2 billion[1]. For the majority of biotechnology companies that are without any product revenue, the significant capital requirements necessitate fundraising through a combination of angel investors and venture capital firms. The role and importance of private equity fundraising in the biotechnology industry cannot be understated.

Typically, a biotechnology company will begin by fundraising for its lead product in development. The lead product is the one that is furthest along in clinical development, in the case of VIRxSYS our lead product is VRX496, which as I previously stated, is in Phase II clinical trials. To get to this point we undertook five rounds of private fundraising.

Biotechnology companies are generally a collection of research projects that range from early to very-early stage development. In addition to the lead therapy biotechnology companies have, on average, five other therapies or candidates in development, which are often at the very earliest stage of pre-clinical research. These candidates may be an outgrowth of research on the lead product or a result of utilizing a particular technology to address a different disease with a completely different set of intellectual property.

Despite the extensive fundraising that a biotechnology company undertakes for the lead product, these funds are not interchangeable, that is they are often tied to very specific milestones to support the lead the product's development. As such, in order to develop secondary or tertiary candidates/therapies a company has to find secondary sources of fundraising capital. At the very earliest stages of development this is particularly

[1] Tufts Center for the Study of Drug Development.
http://csdd.tufts.edu/NewsEvents/NewsArticle.asp?newsid=69

challenging, and it is often times in this capacity that the SBIR grants were instrumental in advancing research and development in biotechnology for over twenty years.

Critical Role of the SBIR Program:

Congress created the SBIR grant program in order to utilize the capabilities of small, innovative, domestic companies to fulfill federal research and development needs. In the early 1980's there was growing concern that the United States federal research and development spending was not improving the health and well being of the citizenry through the development and commercialization of new products and therapies. Furthermore, it was recognized that some early stage, promising scientific research failed to be funded through the markets because it was viewed as too high risk. This failure of the markets is often referred to as the "valley of death." In biotechnology, the "valley of death" delays potential therapies for HIV, cancer, and infectious diseases from reaching patients, who often lack other comparable alternatives.

For these reasons, in 1983 Congress authorized the SBIR grant program. These grants set aside 2.5% of certain departments' and agencies' extramural research budgets for innovative research grants with an aim towards commercialization. One of the great strengths of the SBIR program is that Congress provided the affected departments and agencies with flexibility in establishing the program. As a result, the SBIR program both assists the Department of Defense in its procurement needs and furthers the National Institutes of Health's (NIH's) mission of advancing science and improving health.

In order to participate in the program, Congress provided discretion to the Small Business Administration (SBA) to determine the definition of a qualifying small business concern (SBC). However, the Congress did make clear that the program should be open only to domestic, small companies. In order to be awarded an SBIR grant, an applicant's research is thoroughly examined through peer reviewed research groups that are comprised of experts in the particular field. It should be made clear that the SBIR program was never intended to prop up small businesses through corporate welfare, but instead its mission is to fund competitive and innovative research in small, domestic companies with the goal of commercializing a product.

There are two SBIR grant phases. Phase I grants are for proof of concept or technical merit. These grants are typically no greater than $100,000 although the granting agency does have some flexibility to fund awards that exceed this amount. Companies that successfully complete a Phase I grant can apply for a Phase II grant. A Phase II application is evaluated again on the science and technical merit and feasibility as well as the commercialization potential, as evidenced by private sector, non-SBIR funding commitments. Phase II awards are typically no greater than $750,000, but again, agencies have some flexibility to fund awards at a higher amount. This flexibility should be maintained because it allows expert peer review groups to adequately fund awards where merited by the science.

Unintended Consequences of the SBA's Domestic Company Proxy

For twenty years small, domestic biotechnology companies competed for SBIR grants. In addition to providing non-equity diluting funding, these grants were a powerful signal to the private sector that a company's research was compelling and possessed scientific and technical merit. In biotechnology, the SBIR program has played a role in advancing the science and research of companies that have ultimately brought a product to market. For example, there are 163 companies and affiliates involved in the development of the 252 FDA approved biologics, 32% of those companies and affiliates have received at least one SBIR/STTR award.

However, today most biotechnology companies are excluded from participating in the SBIR program as a result of a SBA Office of Hearings and Appeals (OHA) ruling. On April 7, 2003, the SBA arbitrarily ruled that a biotechnology firm, Cognetix, did not meet the SBIR size standard because it had venture capital investment in excess of 50%. This ruling is based upon SBA regulations, not underlying statue, by which a small business concern (SBC) for the SBIR program is defined as having fewer than 500 employees, including affiliates, and is at least 51% owned by US citizens.

SBA has stated that the ownership rule is meant to be a proxy for determining that a company is domestic[2]. However, the use of capital structure as proxy for determining domesticity and the subsequent OHA ruling has the unintentional consequence of excluding a sizeable portion of the biotechnology industry that would be otherwise eligible to participate in the program. These are companies that are solely based in the United States and are majority funded through a combination of US based venture capital companies and citizens.

VIRxSYS is a unique biotechnology company because the five rounds of fundraising that the company has undergone have been financed through more than 600 private individuals. VIRxSYS is eligible for applying for an SBIR grant. However, I have led both an SBIR-eligible and a non-eligible biotechnology company.

Intronn, Inc., where I was formerly CEO, successfully applied for a Phase I SBIR grant in the area of cystic fibrosis. After meeting the objectives of the Phase I grant, Intronn, Inc. applied for and was granted a Phase II grant. This funding continued to advance the research in cystic fibrosis and as a result Intronn, Inc.'s work was published on the cover of the *Nature Biotechnology* journal. In the summer of 2003, Intronn, Inc. successfully applied for a second Phase II SBIR grant to determine if the candidate was appropriate for Phase I clinical trials.

However, Intronn, Inc. never was able to use this award because several months later NIH requested information on the capital structure of the company. As a result of the previous success with SBIR awards, the company had attracted venture capital investment, which made us no longer eligible, despite the fact that we were clearly a

[2] 54 Fed. Reg. 52634 (Dec. 21, 1989) Interim Final Rule on defining a business concern for the purposes of the SBIR program

small, domestic company at the time of the award. The award was rescinded; we closed down this promising research into Cystic Fibrosis, which was also funded by the Cystic Fibrosis Foundation, and laid off employees. Based upon the reports of other small biotechnology companies, Intronn, Inc.'s experience of having to abandon promising science is, by no means, an isolated incidence.

Arguably, excluding companies from the SBIR program solely on the basis of their capital structure could benefit still eligible companies like VIRxSYS. Yet it does so by making the program less competitive. As evidence of the impact of the new rules on biotech and medical device companies, applications for SBIR grants at the NIH declined by 11.9 percent in 2005 and by 14.6 percent in 2006[3]. As the Director of the National Institutes of Health (NIH), Dr. Elias Zerhouni, wrote in a letter to SBA Administrator Barreto dated June 28, 2005: "*NIH believes that the current rule undermines the statutory purposes of the SBIR program . . . It undermines NIH's ability to award SBIR funds to those applicants whom we believe are most likely to improve human health*." (emphasis added) I would like to submit this letter for the record.

A recent survey of small biotech companies found that 50% are ineligible for the SBIR program because of their capital structure. Additionally, 85% of the companies surveyed said that if the rules were changed to allow them to apply for these grants they would do so[4]. These companies are researching and developing therapies for diabetes, Alzheimer's, lupus and leukemia, among others diseases.

I am willing to compete with small, domestic, majority-backed venture capital companies for SBIR grants based on the scientific and technical merit of VIRxSYS research. That's the American way. I respectfully request that should the Subcommittee reauthorize the SBIR program, that it allow domestic, small companies to compete for SBIR grants regardless of its capital structure. SBIR should be a competitive program that fulfills federal research and development needs while addressing a failure in the market system. It is not meant to repeatedly be a source of corporate welfare but instead should fund highly qualified research.

Again, thank you for providing me with the opportunity to testify today before the Subcommittee.

[3] The National Institutes of Health

[4] Survey of 144 BIO emerging companies' Chief Executive Officers and Chief Financial Officers, March-April, 2007