HEARING TESTIMONY
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ON BEHALF OF
THE BIOTECHNOLOGY INDUSTRY ORGANIZATION (BIO)

BEFORE THE HOUSE OF REPRESENTATIVES COMMITTEE ON SMALL BUSINESS

"SBIR: AMERICA'S NATIONAL TECHNOLOGY DEVELOPMENT INCUBATOR."

JANUARY 29, 2008

Chairwoman Velázquez , Ranking Member Chabot, and Members of the Committee:

Thank you for providing the opportunity to testify before you today regarding the reauthorization of the Small Business Innovation Research Program (SBIR).

My name is Doug Doerfler and I have been President and Chief Executive Officer of Maxcyte, Inc. in Gaithersburg, MD since 1999. Currently, I serve on the Biotechnology Industry Organization's (BIO's) Board of Directors, the Executive Committee of the Emerging Company Section Board of Governors and am co-chair of the Capital Formation Committee.

I have led the development of global biotechnology companies and products for more than 25 years. MaxCyte currently has approximately 20 employees who are developing novel therapeutics using cells that have been modified by our process to treat serious diseases. We have one product in Phase I/II clinical human testing for the treatment of patients with Leukemia, a product in Phase IIa human clinical trials for the treatment of Pulmonary Arterial Hypertension and additional products in pre-clinical development for the treatment of cardiovascular disease, cancers and infectious disease. These programs are partnered with commercial partners and major Universities, including Baylor, the University of Pennsylvania, Duke University and Stanford University. MaxCyte was the proud recipient of Phase I SBIR grants in 2003.

Today I am testifying on behalf of BIO, an organization representing more than 1,000 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.

SBIR'S CRITICAL ROLE IN COMMERCIALIZATION OF BIOTECHNOLOGY INNOVATIONS

Biotechnology Company Profile and Path to Product Development

Before discussing the critical role of the Small Business Investment Research (SBIR) program in the commercialization of biotechnology innovations, I would first like to provide a description of a typical biotechnology company and the capital required for research and development. BIO has over 600 emerging companies in its membership. In a recent survey conducted by BIO, 80 % of respondents had fewer than 50 employees.

Promising biotechnology research by these companies 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 it takes between 8 and 12 years to bring a biotechnology therapy to market and costs between $800 million and $1.2 billion.[1] In the absence of product revenue biotechnology companies are almost entirely reliant on capital markets or other sources of financing to fund research and development. This is particularly challenging at the earliest, highest-risk stages of research and development. The majority of biotechnology companies are without any product revenue for a decade or more. As a result, significant capital requirements to advance a new therapy to the market necessitate fundraising through a combination of angel investors, venture capital firms and occasionally other investors. The role and importance of venture capital fundraising cannot be understated. In 2006 alone, venture capital investment in the life sciences and medical devices industry totaled $7.2 billion in 2006, up from $2.8 billion in 1998.

Biotechnology companies are generally a collection of research projects with one lead product and an average of 5 other therapies or candidates in early stage/pre-clinical research.[2] Typically, a biotechnology company will begin fundraising for its lead product in development. Companies generally raise between $5 million and $15 million in their first round of venture financing, an amount that usually results in multiple venture capital companies owning more than 50 percent of the company. This is especially the case with very young companies whose valuation may reflect their high-risk, early stage nature. However, it is typically the case that no single venture capital company will own more than 15 to 20 percent of the equity.

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

[2] BIO sponsored, third-party administered, survey of 144 BIO emerging companies' Chief Executive Officers and Chief Financial Officers, March-April 2007

Despite the extensive fundraising a biotechnology company undertakes for the lead product, these funds are not interchangeable, that is they are tied to very specific milestones to support the lead 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 other sources of financing, like Small Business Investment Research (SBIR) grants, have been instrumental in advancing research and development in biotechnology.

Mission of SBIR: Bringing Innovation to the Public

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 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 program. When the program approached reauthorization in the early 90's a report by the National Research Council discussed continued concerns that *"U.S. technological performance is challenged less in the creation of new technologies than in their commercialization and adoption."*.[3] Currently, these grants set aside 2.5% of certain departments and agencies extramural research budgets for innovative research grants with an aim towards commercialization.

Historical Success of SBIR Program

For twenty years small, domestic biotechnology companies competed for SBIR grants. In addition to providing critical funding, these grants were a powerful signal to the private sector that 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. These grants have helped make the U.S. the world's leader in biotechnology by providing critical early-stage funding for innovative research.

[3] (National Research Council, *The Government Role in Civilian Technology*: *Building a New Alliance*, Washington, D.C.: National Academy Press, 1992, pp. 29).

IMPACT OF RECENT CHANGES TO SBIR PROGRAM

Unintended Consequences of the SBA's Domestic Company Proxy

On April 7, 2003, the Small Business Administration (SBA) Office of Hearings and Appeals (OHA) 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 base upon SBA regulations, not underlying statute, 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 U.S. citizens.

SBA has stated the ownership rule is meant to be a proxy for determining that a company is domestic.[4] However, the use of capital structure as proxy for determining domesticity and the subsequent OHA ruling has had the unintentional consequence of excluding a sizeable portion of the biotechnology industry that would otherwise be eligible to participate in the program. These are companies that have participated in the SBIR program for 20 years prior to this ruling and were a fundamental part of the aforementioned success of the SBIR program. These are companies solely based in the United States and are majority funded through a combination of U.S. based venture capital companies and citizens. The result is that many emerging biotechnology companies are ineligible to compete for SBIR grants.[5] Perhaps more importantly, this ruling has the potential of negatively impacting the competitive pool of SBIR applicants and the program's ability to award projects with the highest scientific merit and commercialization potential.

My own company, MaxCyte was in the fundraising process in 2003, when we submitted a proposal to NIH to do basic research in our technology and expand its capability so one day it may be used for biodefense or pandemic influenza vaccine development. Venture funds were not interested in this project as it was too early and risky but were clearly motivated by our team's ability to obtain attractive scores for our program through the NIH study section process. We received $95,000 in funding for our Phase I and subsequently closed on a $20.0 million venture round. We were able to satisfy the rigorous milestones of our project including breakthrough science to prove general concept-although we are currently eligible for follow on SBIR funding our eligibility may change with another needed financing.

There are numerous examples of promising discoveries that have been shelved or delayed as a result of the recent interpretation of ownership. I will mention just a couple of examples.

1. Intronn Inc. (Gaithersburg, MD) won SBIR grant for Phase I and II study to advance research in treatment for Cystic Fibosis. They were awarded a second

[4] (54 Fed. Reg. 5264 (Dec. 21, 1989) Interim Final Rule on defining a business concern for the purposes of the SBIR program.)

[5] BIO sponsored, third-party administered, survey of 144 BIO emerging companies' Chief Executive Officers and Chief Financial Officers, March-April 2007

Phase II grant in 2003 but the award was rescinded due to the new rule on venture capital investment. The project was shelved.

2. Paratek Pharmaceuticals (Boston, MA) won a Phase I SBIR grant in 2001 to research antibiotic therapies for things such as malaria and anthrax. In 2003, due to changes in SBIR rules, Paratek was forced to turn down a Phase II grant and their antibiotic therapy research program was shut down.

3. Xcyte Therapies (Seattle, WA) received a Phase I SBIR grant in 2002 to develop new treatments for cancerous tumors in the kidney and prostate. In early 2004 Xcyte Therapies received a Phase II SBIR grant to help fund clinical testing but was unable to use the funds as they were deemed ineligible.

These are ironic outcomes considering that venture capital is a necessary part of the ability to achieve SBIR's mission of supporting commercialization. It is unfortunate that venture capital invested with the goal of bringing new therapies to the market has, in many instances, caused SBIR funding to be pulled and research projects to shelved. This is exactly the opposite of what Congress had in mind when they created SBIR.

OPPORTUNITY TO STRENGTHEN/RESTORE SBIR PROGRAM

I appreciate the opportunity to discuss changes to the SBIR program that I believe would strengthen the program and make it more effective in the years to come. My recommendations can be grouped under four general goals for SBIR Reauthorization. First, increasing competition for SBIR grants and, as such, improving science and fostering innovation and commercialization by small companies. Second, clarifying SBIR eligibility rules to make them easier to understand and increasing transparency regarding the program's operation. Third, maintaining agency flexibility so as to make certain the SBIR program continues to serve the needs of individual agencies. And fourth, making certain that the SBIR guidelines appropriately safeguard taxpayer funds.

I will touch briefly on each of these important goals.

Increase Competition and Foster Innovation and Commercialization

SBA's 2003 ruling that excludes majority venture-backed companies inhibits the SBIR program from receiving the most competitive pool of applicants possible and stifles the ability of SBIR to carry out its mission to fund projects that will improve public health and have the most commercial potential. It is vital to the American public to ensure they realize the benefits not just of products with commercial potential but the benefits of projects funded based on scientific merit and deemed to be of value to promoting our citizens public health.

The current SBA interpretation would deem eligible a public company with 300 employees, as well as, a private company with 400 employees, $200 million in venture

capital from multiple venture capital firms that equal 49% of equity with additional angel investment dollars. However, a private company with 20 employees, $50,000 in annual revenue and $8 million in venture capital by multiple venture capital funds equaling 56% of equity – even though no one venture capital firm has more than 30% of total equity – is ineligible. Among BIO emerging companies, a significant amount are ineligible, the majority of which would apply to SBIR if able. These companies are working on breakthroughs for the treatment of diseases such as Alzheimer's, lupus, and leukemia.

The National Institutes of Health (NIH) have documented disturbing trends since the 2003 ruling. Applications for SBIR grants at NIH have declined by 11.9 percent in 2005 and by 14.6 percent in 2006.[6] Additionally, the number of new small businesses participating in the program has decreased to the lowest proportion in a decade.[7] The Director of the National Institutes of Health, Dr. Elias Zerhourni, 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.

Some critics have recommended that biotech companies look for other grants to fund their research. However, this is easier said than done. For instance, only 0.4% of non-SBIR/STTR grants at NIH went to biotech companies. SBIR supports small business concerns to conduct high-risk, early-stage, innovative research that has a focus toward commercialization of a product or service. Unlike other NIH grant mechanisms, SBIR grants are not hypothesis-driven research. Hypothesis driven research is scientific research solely for the purpose of advancing knowledge in the subject area and is not concerned with commercialization. SBIR is the only program that bridges the two.

BIO respectfully requests the Committee recognize the necessary and complex involvement of venture capital in small biotechnology companies. As stated previously, small biotechnology companies have high and intense capital needs (up to $1 billion) and an unusually long development time of 5-12 years. The vast majority of biotechnology companies raise between $5 million and $15 million in their first round of venture financing for their lead product(s), an amount that usually results in the venture capital firms collectively owning more than 50% of the company. However, the investment group usually consists of several firms, none of which owns more than 15-20% of the company. SBIR plays a critical role in aiding small biotechnology companies in their early stage research to navigate through the "valley of death" where the concept is too high-risk for private market support.

BIO respectfully asks the Committee to reinstate the eligibility of small biotechnology firms into the SBIR program. This will ensure the most competitive pool of applicants

[6] The National Institutes of Health

[7] Testimony from Jo Anne Goodnight, SBIR/STTR Program Coordinator for NIH to the House Subcommittee on Technology and Innovation, Committee on Science and Technology: *The SBIR and STTR Programs at the National Institutes of Health – How are Programs Managed Today;* June, 26, 2007).

and that grants awarded will be based on projects that show the most promise in bringing breakthrough therapies to the public.

Clarify SBIR eligibility rules to make the application process more straightforward and user-friendly

It is equally important the reauthorization clarify SBA affiliation regulations. Under current SBA regulations, when determining the size of a business, the SBA considers the number of direct employees at the business as well as affiliated businesses' employees. Businesses are affiliates of each other if the SBA determines that another business has either affirmative or negative control. Current regulations state that a venture capital company that holds a minority share in another business can be considered an affiliate of that business. If the SBA determines a venture capital company is affiliated with the business, not only are the employees of the venture capital company included in the size determination but so are the employees of all other businesses in which the venture capital firm is invested.

As a result of these affiliation rules, a small company with 50 employees could be deemed to be affiliated with hundreds of other employees of companies with which the small company has no relationship whatsoever, just because the companies share a common investor. It is important to note that this can be the case where the VC investor owns a minority stake in the small business applying for SBIR.

Not only are these affiliation rules non-sensical, the manner in which they are applied is often a mystery to the small business applying for the SBIR grant. As a result, a small company may certify in good faith that it is eligible for an SBIR grant, only to later find out that the SBA has affiliated it with a large number of employees at other unrelated companies, thus making the small business ineligible. BIO recommends the reauthorization bill provide language to clarify that investment by a venture capital operating company does not make that company an affiliate of another company for the purposes of determining size. This is a common-sense measure that will provide clarity and peace of mind for small business entrepreneurs looking to participate in the SBIR program.

Maintain Agency Flexibility

BIO also supports maintaining agency flexibility in the SBIR program. One of the great strengths of the SBIR program is that Congress provided the affected departments and agencies with flexibility in establishing the program. Maintaining flexibility in the program is also supported by a National Research Council 2007 report which states, *"…flexibility is a positive attribute in that it permits each agency to adapt its SBIR program to the agency's particular mission, scale and working culture."* [8]

[8] National Research Council, *An Assessment of the Small Business Innovation Research Program at the National Science Foundation*: Washington, D.C.: National Academy Press, 2007. pp 21 (www.nap.edu/catalog/11929.html)

The reality is that various government agencies may structure their SBIR program in different ways to meet differing agency needs. This is a good thing, so long as the original goals of the SBIR program are preserved. Certain agencies, for example, may need the flexibility to award larger grants, if the project they are funding is in an area where research is typically more expensive. This is sometimes the case for biotechnology companies researching therapies that are especially novel or cutting-edge. For this reason, BIO does not believe that a hard cap should be applied to the SBIR grant amounts. Agencies should be the best judge of how to use their SBIR funds to advance science and commercialize new innovations.

Additionally, any caps on SBIR grants, if imposed, should apply to particular SBIR phases and should not apply to the entire amount that the agency spends on a particular project. The NIH, for example, has chosen to implement a commercialization assistance program for those companies who may need extra funding before they can attract private dollars. A hard dollar cap in the SBIR program could threaten such a program and this would be, in BIO's opinion, very unfortunate.

Appropriately safeguard taxpayer dollars

As with any government program, Congress has the obligation to ensure that taxpayer funds are being used in an efficient and effective manner. The SBIR program is not a basic research program, it is about developing new products for the benefit of society. There have been concerns expressed over the number of grants an individual company may receive from the SBIR program. While BIO supports some agency flexibility in these decisions, we would support reasonable limitations, such as capping the number of awards per company to 5 -10 awards per year/per company

No company should make SBIR grants the basis of its business model. SBIR exists to fill the funding void for companies that are raising private capital to do their research and development. SBIR plays the very important role of funding early-stage research, research that might not otherwise be funded or whose development would otherwise be significantly delayed. Any company that receives excessively large numbers of SBIR grants year after year, without commercializing technology, is probably not the type of company into which the federal government should be investing taxpayer resources. BIO believes it is appropriate to include safeguards in the SBIR reauthorization bill to ensure that firms are applying for SBIR grants as a supplement to the private capital they have raised and are not trying to "game" the program.

CLOSING REMARKS

Congress can continue to support the United States biotechnology community by allowing the government to partner with small biotechnology companies that have promising science but need additional resources at key stages of development not readily available in the private capital markets. SBIR should be an aggressively competitive program that fulfills federal research and development goals of bringing breakthrough public health discoveries to the public.

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