HEARING TESTIMONY

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ON BEHALF OF THE

BIOTECHNOLOGY INDUSTRY ORGANIZATION

BEFORE THE HOUSE OF REPRESENTATIVES COMMITTEE ON SMALL BUSINESS

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Good morning Chairman Graves, Ranking Member Velázquez, Members of the Committee, ladies and gentleman. I am President and Chief Executive Officer of MacroGenics Inc and Chairman of the Board of Applied Genetics Technology Corporation (AGTC). I am appearing before this Committee on behalf of the Biotechnology Industry Organization (BIO). BIO represents more than 1,200 companies, academic institutions, state biotechnology centers and related organizations in all 50 states.

I am a scientist, physician, and entrepreneur and have worked at both the NIH and in the biotechnology industry for the past twenty-seven 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, West Nile virus infections, and many types of cancer. I have seen the importance and impact of the SBIR program in the biotechnology industry, not only on fostering the growth of fledgling companies during some of the most challenging times in their business cycles, but in enhancing

the advancement of important products to the marketplace. Sadly, from my perspective, current rules, as a result of a 2003 Office of Hearings and Appeals ruling, have inhibited and interfered with the growth and survival of small private biotechnology companies and the development of promising technologies and products due to the inability of venture-backed companies to participate in the SBIR program. Let me provide an example of each with two quite different outcomes for programs developing vital treatments for children.

In the early 1990's, MedImmune was a small biotechnology company in Gaithersburg, MD, founded in 1988, funded by venture capitalists, which became a publicly-traded company on NASDQ in 1991. One of the lead programs in the company at the time was a monoclonal antibody to prevent a viral infection called respiratory syncytial virus (RSV) in neonates. The research and development of this program was funded by SBIR Phase 1 and 2 grants. This funding was critical in supporting the company and the research program. Today, this product called Synagis, the first and only FDA approved monoclonal antibody product to prevent an infectious disease, has been used in over 600,000 children and is still MedImmune's most significant product. MedImmune was acquired by AstraZeneca in 2007, one of the largest acquisitions of a biotechnology company by a pharmaceutical company. MedImmune now employs thousands of highly skilled professionals. If current SBIR rules prevailed at that time when MedImmune's scientists first applied for an SBIR grant, MedImmune would have been ineligible to receive those SBIR funds and it would have significantly, impacted the development of that program and the company.

Contrast that outcome with AGTC. Today, AGTC is a small private biotechnology company in Alachua, Florida, developing cutting-edge product candidates to treat and cure different genetic diseases using adeno-associated viral (AAV) vectors produced from their proprietary manufacturing process. The company, by all parameters, is small. They have seven employees rent space in a university lab, have no product revenues, and have large capital requirements to advance their programs through early stages of pre-clinical and clinical development. They have raised $45M from venture capitalists to date and because of their capital structure are ineligible to receive SBIR funds. All of the venture capital funds are being used to support two early clinical stage programs at the company and there is no additional capital available to support other promising avenues of research. AGTC received several SBIR grants from 2001-2003 for three different projects to advance treatments for rare diseases and expand their technology platform and the results from this research were valuable in advancing the company's mission.

These were projects that were either too early in their development cycle or targeted to too small of a patient population to be of interest to financial investors. In 2003, the company applied for a Phase I/II SBIR grant that was initially approved for award with a very good score and excellent reviews, but the application had to be withdrawn due to circumstances of VC ownership. This grant would have advanced a treatment for Pompe's disease, a fatal genetic disorder that in many cases results in death of infants by one year of age. No investors were willing to fund this early stage work on Pompe's and no further work has been done on this program in the past eight years.

Currently, the company is working on one of the most promising programs to treat blindness in children caused by genetic disorders. The first eye disorder being addressed is Leber's congenital amaurosis (LCA), a rare retinal disease affecting a few thousand patients in the U.S. An initial clinical trial has resulted in the restoration of partial sight in the first legally-blind patients with the inherited defective gene when they were treated with the AAV vector containing the normal form of the gene. This ground-breaking work using the company's AAV vector product candidate, as well as studies conducted by other investigators, was published in *Human Gene Therapy* and the *New England Journal of Medicine (2009)*. AGTC is starting additional clinical trials to test this promising therapy in patients with LCA with its current funds. However, the company desires to generate and test other gene replacement candidates for three other genetic eye diseases, particularly those with larger number of affected individuals, but cannot do so because resources are unavailable and they are unable to receive SBIR funds for the high risk, but likely rewarding approach to treating these debilitating eye disorders. In fact, AGTC applied for an SBIR grant in 2010 to develop a treatment for one of these genetic eye diseases called achromatopsia in anticipation of congressional resolution of matters of SBIR funding related to VC ownership. The grant was scored and awarded, but AGTC is unable to accept the funds due to the prevailing rules.

As developers of the next-generation of treatments for diseases that would have been considered unapproachable just a decade ago, it is incumbent on our system to find ways to support these risky, but transformational therapies that could improve the lives of children and adults suffering from genetic disorders, infectious diseases, cancer, and autoimmune diseases, among others. We want to take advantage of the ground-breaking scientific discoveries in basic research that has been achieved in the last decade at the NIH, in academic centers, and in industry and translate them into tangible treatments as rapidly as possible to improve the lives for patients. This has

personal and economic benefits to the individuals affected, the organizations and companies working on these initiatives, and our society in general.

The SBIR program is an important component in the foundation and growth of new biotechnology-based companies and we ask that this funding vehicle be available to companies after they raise venture capital, so that we can continue to develop these life-changing products. This policy is supported by the 2009 National Research Council's 2009 report "Venture Funding and the NIH SBIR Program." This study found that "…restricting access to SBIR funding for firms that benefit from venture investments would thus appear to disproportionately affect some of the most commercially promising small innovative firms…" and that the current SBA eligibility rules have "…the potential to diminish the positive impact of the nation's investments in research and development in the biomedical area." The report recommended that the SBA ruling be repealed or modified so that majority-venture funded companies with significant commercial potential can compete for SBIR funding.

The ability of the SBIR program to provide critical funding for projects with the most potential to benefit the public, will remain hampered, unless SBIR reauthorization updates the program to address the current realities facing small, innovative American companies. Impacts of the economic downturn are still being felt by the industry. The amount of venture capital dollars decreased by 27% between 2009 and 2010 (BioWorld Today; January, 2011) and finding funding for promising early-stage projects is as difficult as it has ever been. This is an industry that provides high-paying jobs to millions of individuals. This is a 21st century industry whose potential both as an economic driver and in delivering solutions to our nation's most critical public health needs has not yet been maximized. SBIR could play a critical role in helping achieve those goals.

OPPORTUNITY TO STRENGTHEN/RESTORE SBIR PROGRAM

Increase Science-Based Competition

Allowing small, U.S. biotechnology companies that are majority owned by venture capital companies to once again compete for SBIR awards based on scientific merit will ensure the most competitive pool of applicants 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 that 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. 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 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, simply 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 nonsensical, 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 supports an SBIR reauthorization legislation that creates a more rational and effective affiliation process regarding determinations about an SBIR applicant's investors' portfolio companies supported by its investor. This is common-sense and would provide clarity and peace of mind for small business entrepreneurs looking to participate in the SBIR 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. This is an industry full of potential to create high-paying jobs and to provide solutions to our nation's most

critical public health needs. BIO believes that the modernizations to the SBIR program being considered by the committee will help to accomplish this important objective.