



Hearing Testimony
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On Behalf Of
The Biotechnology Industry Organization

Before the Small Business Committee
United States Senate

“Strengthening Participation of Small Businesses in Federal
Contracting and Innovation Research Programs”

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Chairwoman Snowe, Ranking Member Kerry and the Members of the Small Business Committee:

Thank you for providing the opportunity to testify before you today on strengthening the participation of small businesses in the federal innovation research programs.

My name is Thomas Bigger. I am the President and Chief Executive Officer of Paratek Pharmaceuticals. Paratek is a privately-held, private investment company-backed biopharmaceutical company located in Boston, Massachusetts. We are considered an early-stage company having only one program in the clinical stage, while the majority of our programs are in pre-clinical development.

Paratek is engaged in the discovery and commercialization of new therapeutics that treat life threatening infectious and other serious diseases. Paratek was founded in 1996 by Dr. Stuart B. Levy, Professor at Tufts University School of Medicine, and by Dr. Walter Gilbert, a Nobel Prize winning Professor Emeritus at Harvard University.

Paratek’s primary mission is to develop novel antibiotic and anti-infective agents that overcome the critical worldwide problem of bacterial resistance through the application of our two proprietary technology platforms. Paratek utilizes these platforms, Tet and MAR, to develop multiple products to combat, cure, and prevent infections, such as serious and resistant bacterial infections, and other serious diseases, such as pseudomonal infections, malaria and anthrax, as well as inflammatory diseases, such as asthma, arthritis, multiple sclerosis, and stroke.

Today, I am here to testify 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 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.

As a representative of one of the most innovative high growth sectors of our nation's economy -- one in which the United States maintains a global leadership position -- my testimony will focus on the urgent need for reforms in the current eligibility rules of the Small Business Innovation Research (SBIR) program. Senator Bond has introduced legislation, S.1263, the Save America's Biotechnology Innovation Research Act (SABIR Act), which would make the necessary reforms to the SBIR program. These reforms are essential in providing the most innovative early stage biotechnology companies with the opportunity to compete for, as they did for over two decades, and participate again in the SBIR program. Without reform, we could seriously jeopardize America's innovation leadership and competitiveness in the global biotech market place.

Changes in SBIR Eligibility Rules – Cost to U.S. Biotech Innovation:

Early-stage biotech companies rely on risk capital and, increasingly, federal grant sources to fund research and development activities. Small biotech companies often rely on SBIR Phase I and II grants to fund research and/or development in areas that most private investors or venture capitalists won't fund because they consider these areas to be either too early-stage to fund, too risky from a market opportunity standpoint, or simply lacking in sufficient commercial returns.

To qualify for the SBIR grant, a small business applicant must meet certain eligibility requirements. The size and ownership requirements — or “size standard” — limit eligibility to those companies that are: 1) 51% owned and controlled by one or more *individuals* who are U.S. citizens or permanent residents and 2) have no more than 500 employees, including any affiliates.

For the first 21 years of the program, the Small Business Administration (SBA) interpreted individuals to include individual entities or investment groups, as long as they were majority-owned by Americans. However, on January 10, 2001, the SBA Office of Hearings and Appeals ruled in *CBR Laboratories, Inc.* that the definition of “individuals” no longer included venture capital firms or other investment groups, including funds established by patient groups to support research. Instead they chose to follow a very strict, unorthodox interpretation not followed by other federal agencies that the legal term “individuals” referred only to actual individual human beings, not individual investor groups. This new interpretation of “individuals” resulted in the denial of an SBIR grant in 2003 to Cognetix, a Utah biotech company, because the company was backed by private investment firms in excess of 50% in the aggregate. Many biotech companies have since been denied the opportunity to compete for the SBIR grants and as a result, their work on life-saving and life-enhancing technology is being indefinitely postponed.

Paratek has experienced first hand the detrimental effects of no longer being able to compete for and participate in the SBIR program. In 2003, specifically due to the changes in the SBIR eligibility rules, we had to turn down a Phase II grant and shut down a key antibiotic therapy research program, where ultimately, we had to lay off 10 employees. This program was originally started with Phase I SBIR grant funding in 2001, and the NIH had urged us to accept the grant despite the eligibility changes in order to continue this valuable research. Because we strongly believed in our technology, we pursued other sources of funding, such as from

philanthropic foundations. However, we were unable to find alternative funding until 2 years later, ultimately delaying patient access to the novel therapies that much longer.

The majority of BIO's members who are private companies have also faced similar fate due to the arbitrary changes in the SBIR eligibility rules in 2001 and 2003. For instance, a privately-held, venture-backed, biopharmaceutical company located in St. Louis, Missouri, raised their first or Series A round of financing in 2001. Because of the reinterpretation in the rules, they were unable to participate in the SBIR program and as a result, had to cancel the development of their bio-defense vaccine program. This company's technology, with additional development, could have delivered massive quantities of vaccines against anthrax, cholera, and other diseases as part of America's biodefense and pandemic preparedness efforts.

While there are very few other government grant programs, such as NIH's Cooperative Agreement Program (U-01), for which companies like Paratek could submit applications, these programs are not designed for early stage, innovative research. In the majority of the cases, funding requests for certain diseases are only available through the SBIR program. Even if one could pursue a U-01 grant, the U-01 program typically requires the submission of a substantial body of data and the demonstration that the technology is far more advanced than the proof of concept stage – meaning that significant funding would already have been required for the programs to get it to the point of being competitive for these particular grants. Moreover, truly small biotech companies like Paratek are often squeezed as we may be competing with large international pharmaceutical companies to secure these other types of grants. The SBIR program fills the funding gap that allows small companies like Paratek to continue to innovate and move early stage research forward to the point where we can compete for U-01 and other grants or sources of funding.

BIO member company concerns regarding the impact of current eligibility limitations on biotech innovation are shared by Dr. Zerhouni from the National Institutes of Health (NIH). Dr. Zerhouni, in his letter dated June 15, 2005, strongly urged the SBA to revise its SBIR rules to remedy current rules that “unduly restrict the ability of NIH to fund small companies that receive venture capital investment. [Since] as a result [of the changes in rules], NIH must turn away many deserving applicants and the goals of the SBIR program are being undermined.” NIH raised their concerns again in June 16, 2006, in a letter to the GAO regarding their study on the SBIR program. In its letter, NIH reiterated its belief that “the impact of current eligibility rules presents a significant roadblock in our technology development pipeline and ultimately in the speed in which important products to improve health are brought to market.”

Reality Check: Role of Private Investors and Venture Capital in Biotech Companies

Some have raised the question that biotech companies that are majority owned by private investors or venture capital companies are somehow no longer small businesses. Nothing could be further from the truth. Paratek, with 66 employees, is a small business regardless of whether we get funding from a bank, from venture capitalists or from individuals. What separates biotechnology companies from less capital-intensive industries is the sheer amount of money, the length of time necessary for development, and the required Food and Drug Administration approvals to bring a product to market. The development of a new biotech drug or therapy requires years of research and testing, and hundreds of millions of dollars and often takes a decade or more. As such, private investment is not an option, it is a necessity.

It is also critical to make the distinction that early stage investment firms and venture capitalists invest in biotechnology companies and programs because they hope to realize a return on their

investments, and not because they want to run or control the biotech company. In fact, most venture capital companies are very small organizations, usually operated by 4 to 6 managers. These managers invest in a wide range of companies so as to diversify their risk. Their job is managing money, risk and return; not running a business, and certainly not running a business to obtain SBIR grants. Moreover, just because a company is able to attract private funding does not mean that private investment firms will fund every program. As stated earlier, private investors are often unwilling to fund early stage projects or those with limited potential for commercial return no matter how compelling the therapeutic need.

To further reduce risk, most private investment firms invest as members of a group with other firms. As such, private investment firms typically acquire only minority interests in, and do not control their portfolio companies. In the biotech industry, this tends to be between a 5 to 20 percent interest in any one company. Private investment firms do not have the time, staff, or the desire to engage in the day-to-day operations of their portfolio companies.

As an example, at Paratek, the management and individual investors own approximately 45% of the interest in the company. We have 15 different private investment groups who own at most 5% each. Only one of these firms out of the fifteen has a board seat, and we can safely say that Paratek's management controls the day to day management and direction of the science and not the investors.

Current SBA Regulation Lacks Congressional Mandate

Some have also argued that the recent changes in the SBIR eligibility rules are consistent with the goals of the SBIR program. The Congressional record indicates otherwise. The SBIR statute and its legislative record demonstrate that Congress intended to encourage venture financing of SBIR awardees. In fact, the SBIR statute lists a company's ability to attract private investment and to commercialize its product as a factor to be favorably considered by the contracting agencies in awarding SBIR grants. Indeed, some would argue that the companies that are able to attract private investment are likely to be the ones that have the talent, experience and infrastructure to be best able to identify and make the most of promising, innovative technologies.

The record shows that one of Congress' most important goals was to assist small business concerns as a way to lead the technological advancements in the United States. This point is well demonstrated in the House Small Business Committee report, which states that the legislation was "aimed at stimulating innovation in general and at stimulating the technologically and innovatively oriented small business sector." H. Rept. 97-349, 97th Congr., 1st Sess. 1981 at page 17. Moreover, Congress deliberately sought to promote those businesses that had attracted private sector backing and showed no inclination whatsoever to exclude venture-backed small firms from the program.

In fact, the SBIR program was originally intended for agencies to give preference to companies that have received Phase I grants and have attracted private sector funding to pursue commercialization of their products. The Senate Small Business Committee explained that "[t]his special consideration serves as a built-in incentive for participants in the program to seek ways to build upon the federal research, thus fulfilling one of the bill's primary objectives." S. Rept. 97-194, 97th Cong., 1st Sess., 1981 at page 2. Thus, the SBIR program was specifically meant to "facilitate" the ability of participating firms to attract venture capital, not to prohibit it, as SBA's current regulatory interpretation does by requiring 51% ownership by "natural persons". Thus, the SBA's interpretation of the eligibility rules not only greatly hinders the best

innovators from competing but also seems to defy Congress' intent behind the SBIR program itself.

Paratek embodies Congress' original intent with respect to the SBIR program. We employ highly educated and skilled scientists and other associates. Our novel scientific agents have been validated in Phase I grants and we were urgently in need of the SBIR grant for further validation in a Phase II grant. Although our science showed great promise, in 2003, many private investors were reluctant to fund the programs until we could demonstrate additional proof of concept. As Congress intended, SBIR grants are essential in supporting this early stage research, which further validates and often proves persuasive for additional investment by private investors.

Moreover, companies like Paratek would like to make contributions to the treatment of disease in areas where there are significant unmet needs but where the commercial value is too low to justify private investment at the early stages of research. We believe that we have the technology that can be put to use in areas such as the treatment of malaria, filariasis, spinal muscular atrophy – a severe disease that affects a small population, and other orphan and niche diseases. However, without SBIR funding to advance our research in these areas to the point where we can get other funding, such as from foundations or even U-01 government grants, it is unlikely that we will be able to pursue potentially compelling treatments. As a result, much of this innovative technology will sit on the shelf.

Urgently Need SBIR Eligibility Reform – Support S.1263

The time is now for this Committee to consider and support SBIR eligibility reform. Senator Bond has introduced S.1263, the SABIR Act in 2005. Many of the members of this Committee are co-sponsors. This legislation provides small but majority venture backed companies the opportunity to compete for the SBIR grants. This very important legislation will reverse the misguided SBA interpretation regarding the SBIR rules and return its eligibility standards to where they had been for 21 years prior to 2003. We urge all the Members of this Committee to support S.1263 and to include S.1263 as part of the SBA Reauthorization Act this year. SBIR reform is critical now in order that U.S. biotechnology companies can continue to innovate and remain competitive in the global market place.