Testimony of Rachel King, CEO

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To the United States Senate Committee on Small Business and Entrepreneurship

Hearing on "Protecting Innovation and Entrepreneurship in Patent Reform"

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Summary of Testimony:

Chairman Vitter, ranking member Cardin, and members of the Committee, thank you for inviting me here today to testify on the subject of protecting innovation and entrepreneurship in patent reform. I am Rachel King, CEO of GlycoMimetics, a small publicly traded biotechnology company located in Maryland that utilizes novel and proprietary technology to develop treatments for diseases with high unmet needs such as sickle cell disease and blood cancer. I have spent most of my career working within the biotech industry, as an executive at two start-up companies and as a venture capital investor supporting the growth of a portfolio of such companies.

Based on this experience, I can attest that very few sectors of the nation's economy are as dependent on predictable, enforceable patent rights as is the biotechnology industry. Robust patents that cannot be easily circumvented or invalidated, and that can be predictably enforced against infringers, enable biotech companies to secure the enormous financial resources and tolerate the high levels of risk needed to advance biotech products to the marketplace. Because such risks and costs cannot usually be borne by any one entity alone, especially the small companies that make up the vast majority of biotech companies, biotech development depends heavily on licensing, partnering, and access to capital. And it is strong and reliable patents that enable us to engage in the partnering and technology transfer that is necessary to turn basic scientific discoveries into real-world solutions for disease, pollution, and hunger.

My own company's story is a perfect case study in how the biotech ecosystem works. It took GlycoMimetics seven years and $60 million from several rounds of private financings just to complete our initial study of sickle cell patients; and another three years and a major partnership worth hundreds of millions of dollars with a large pharmaceutical company to advance our compound through a positive Phase II clinical trial. Based on this success, we were able to go public in January 2014, raising another $64 million to further support our research and development and to advance our second program to try to treat certain blood cancers. If all goes well, a product may finally be available for patients in a few more years. That would be 15 years after the founding of our company, with tens of millions of dollars invested, and tremendous risks along the way.

I can say with absolute certainty that our ability as a small company to secure all of this financing and partnerships over more than a decade was possible only because of the strength of our patent portfolio. If patents can be invalidated under overly broad criteria, or if the ability to enforce them becomes limited due to excessive lawsuit filing requirements or undue delays or complications in obtaining discovery and moving a case through the courts,

third parties would be less likely to invest in or license the technology – and major sources of R&D funding would dry up for small companies while a cloud of uncertainty hangs over their patent portfolio. The result – patients waiting for the next new cure or treatment will have to wait longer, or may never get it at all. With all due respect, I would urge Congress to keep such considerations in mind as it attempts to address abuses of the patent litigation system by so-called "patent trolls."

Congress also should focus not just on abuses *by* patent owners, but also those perpetrated *against* patent owners. In particular, Congress should reassess the new administrative patent challenge system known as Inter Partes Review (IPR), which is having a game-changing effect on the reliability of patents as a basis of biotech investment. Patents in IPR are being invalidated at rates so high – roughly 80% -- that the basic procedural fairness of these proceedings is increasingly being questioned. Based on this emerging data, hedge funds and other third parties with no commercial interest in the patents have figured out that they can extort settlements or otherwise gain financially from bringing, or even threatening to bring, patent challenges against critical patents of biotech companies – including by "shorting the stock" of such companies and then filing IPRs to drive down the stock prices and profit therefrom. Biotech companies can be particularly vulnerable to such abuses because they are small companies that often rely on just a handful of highly valuable patents to protect their products and massive investment therein.

In this regard, I want to express my support for the recently-introduced STRONG Patents Act sponsored by Senators Coons, Durbin, and Hirono, which would ensure that IPR proceedings are no longer unfairly stacked against patent owners.

Let me close with some sobering facts. One out of five Americans can expect to develop Alzheimer's disease during retirement, and the risk of developing cancer is even greater. While much has been said about abuses in the patent system that drive up certain business costs, we must keep in mind that that same patent system encourages risk-taking and long-term investment in potential solutions for the biggest problems facing the generations to come: disease, hunger, and pollution. It is critical that the future path of our patent system continues to preserve the incentives for small business innovation that have made the United States the global leader in medical, agricultural, and environmental biotechnology.

Thank you again for the opportunity to present my views on this important matter and I am happy to answer any questions the Committee may have.

Introduction

By way of personal introduction, I am Rachel King, CEO of GlycoMimetics, Inc., a small Maryland-based company. GlycoMimetics is a publicly traded, clinical-stage biotechnology company that utilizes novel and proprietary glycobiology technology to develop treatments for diseases, especially those with high unmet needs. Since the company's inception in 2003, GlycoMimetics has developed a robust, diversified product pipeline. The company's mission is to continue to advance its pipeline, providing hope for patients with sickle cell, cancer, and other serious diseases.

In order to advance this mission, the company secured an initial round of private venture financing, known as Series A, in an amount of $4.5 million. Over the next six years, we needed to raise an additional $53 million in Series B and C financings to keep the company afloat as we identified a lead compound and initiated a Phase 1 clinical trial in late 2008. After completing this pilot study of our lead compound in sickle cell patients in 2010, we were able to secure a major $340 partnership with a large pharmaceutical company to advance our clinical R&D program through Phase II, reporting positive top line data in mid-

2013. Based on these results, we were able to complete an initial public offering for our company in January 2014, raising an additional $64 million to support our R&D pipeline. Our ability to secure all of this financing over more than a decade was possible only because of the strength of our patent portfolio.

Prior to joining GlycoMimetics, I was an Executive in Residence at New Enterprise Associates, one of the nation's leading venture capital firms. Prior to that, I spent 10 years with Genetic Therapy, Inc., through the company's early stage, initial public offering, and eventual sale to Novartis. After the sale, I was named CEO and ran the company as a wholly owned subsidiary. I received my B.A degree from Dartmouth College and my MBA from Harvard Business School.

I also currently serve as the Chair of the Board of Directors for the Biotechnology Industry Organization, the biotech industry's leading national trade association, as well as Chair of the Maryland Life Sciences Advisory Board appointed by Governor Martin O'Malley. However, my testimony today represents my own views, based on my experiences as an investor in and CEO of small biotech companies, and not necessarily the views of the organizations which I chair.

Background on the Role of Patents in the Biotech Business Model

Very few sectors of the nation's economy are as dependent on predictable, enforceable patent rights as is the biotechnology industry. Robust patents that cannot be easily circumvented or invalidated, and that can be predictably enforced against infringers, enable biotechnology companies to secure the enormous financial resources needed to advance biotechnology products to the marketplace, and to engage in the partnering and technology transfer that is necessary to translate basic scientific discoveries into real-world solutions for disease, pollution, and hunger.

Research and development within the biotechnology industry comes at a very high cost, and every idea that is funded comes with a much greater risk of failure than success. Investment thus is predicated on an expected return in the form of patent-protected products or services that ultimately reach the market. The typical biotech company does not have a product on the market yet, nor a steady source of revenue, and spends tens of millions of dollars on R&D annually. The biotechnology industry, as a whole, is responsible for well more than 20 billion dollars of annual research investment, and provides employment to millions of individuals nationwide. Virtually all of this investment is through private sector funding.[1] Developing a single therapy requires an average investment ranging from $1.2 billion to over $2 billion, and the clinical testing period alone consumes more than eight years on average.[2]

Such investments are not only expensive; they are risky. For every successful biopharmaceutical product, thousands of candidates are designed, screened, and rejected after significant investments have been made. The chances that a biopharmaceutical medicine will advance from the laboratory bench to the hospital bedside are approximately

[1] Moving Research from the Bench to the Bedside: Hearings Before the Subcomm. on Health of the House Comm. on Energy and Commerce, 108th Cong., 1st Sess. 47 (2003) (testimony of Phylliss Gardner, M.D) (http://archives.energycommerce.house.gov/reparchives/108/Hearings/07102003hearing990/Gardner1579.htm) ("The biotechnology industry is the most research and development-intensive and capital-focused industry in the world," noting that 98 percent of research and development investment comes from the private sector).

[2] Joseph A. Di Masi and Henry G. Grabowski, The Cost of Biopharmaceutical R & D: Is Biotech Different? Manage. Decis. Econ. 28: 469-479 (2007) (hereafter: "Di Masi and Grabowski").

one in 5,000.[3] Only a small minority of candidate drugs even advance to human clinical trials, and most of those will never ultimately reach the market. For example, at the time human clinical testing begins, the odds that a biopharmaceutical compound will eventually receive FDA approval are less than one-third.[4]

Because such risks and costs cannot usually be borne by any one entity alone, biotech drug development depends heavily on licensing, partnering, and access to capital. Patents allow biotech inventions of great societal value to be passed or shared among parties best suited to unlock their potential at any given stage of development and commercialization – each contributing its part, each sharing the risk of failure, each increasing the odds that a product eventually reaches patients. Such sharing of risks, costs, and talents has been critical to the success so far of my own company, GlycoMimetics. Without strong and reliable patents, we would not have been able to secure the investment or partnerships that have kept our doors open for so many years as we seek to prove the safety and efficacy of our leading therapeutic candidates.

If patents can be invalidated under overly broad criteria, or if the ability to enforce them becomes limited due to an exceedingly high bar to filing a lawsuit or excessive delays or complications in prosecuting a case through the courts, third parties would be less likely to invest in or license the technology, and major sources of R&D funding would move elsewhere. The result – patients waiting for the next new cure or treatment will have to wait longer, or may not ever get it at all.

For these reasons, currently-pending patent litigation reform legislation is highly relevant to the biotech business model. A small or mid-sized biotech company that today decides to begin development of, for example, an Alzheimer's treatment must look a decade or more into the future. Long-term financial commitments will be required; several hundreds of millions of dollars will need to be raised; and development partnerships will need to be secured in a situation where the cost of capital is high and the odds of ultimate success are small. Because investment-intensive businesses can tolerate only so much risk, even moderate additional uncertainty can cause business decisions to tip against developing a high-risk, but potentially highly-beneficial, product.

This is not an academic consideration. Every biotech executive has stories to tell about promising experimental compounds that had very favorable medicinal properties, but were never developed because their patent protection was too uncertain. The injection of additional systemic uncertainty by, for example, making the enforceability of patents against infringers more uncertain can negatively affect which new cures and treatments may become available a decade from now.

The average American today can realistically hope to live into her or his eighth decade. At retirement, one out of five Americans can expect to develop Alzheimer's disease during her or his remaining years. The risk of developing cancer is even greater. While much has been said about inefficiencies in the patent system that drive up business costs in some sectors today, we must keep in mind that that same patent system encourages risk-taking and long-term investment in potential solutions for the biggest problems facing our world and the generations to come: disease, hunger, and pollution. Great care must be taken to ensure that we do not forget the patent system's longer-term benefits to society. It is critical that the future path of our patent system is one that preserves and maintains the

[3] Secretary of Health and Human Services Tommy G. Thompson, Remarks at the Milken Institute's Global Conference (Apr. 26, 2004), available at www.hhs.gov/news/speech/2004/040426.html

[4] Di Masi and Grabowski, at 472-3.

incentives for innovation that have made the United States the global leader in medical, agricultural, and environmental biotechnology.

Views on Patent System Reforms

As a CEO of a small business, I am sensitive to the concerns that have been raised by some small business owners about the negative impact on their businesses from the meritless assertion of overly broad and questionable patents by so-called "patent trolls." Small businesses have fewer human and financial resources to deal with such legal maneuverings, and they distract management from its focus on advancing the company's R&D or operations. There are real costs to such abuses, and Congress should consider how best to protect small businesses from them. In particular, there are several legislative proposals designed to curtail the sending of indiscriminate, bad faith patent demand letters, enhance transparency around patent ownership and enforcement, and protect innocent consumers or end users from infringement suits based on their purchase and use of technology or products manufactured by others.

But Congress also must recognize that small businesses often must defend their inventions and their companies against very real threats posed by larger corporate infringers. And when they are forced to do so, it is critical that the litigation system operate in a cost-efficient, timely, balanced, and fairly predictable manner. Otherwise, investors and partners will simply dry up while a cloud of uncertainty hangs over the small company's patent portfolio.

In this regard, it has become clear that the PTO's Inter Partes Review (IPR) system of administrative patent challenges is having a game-changing effect on the reliability of patents as a basis of investment in the biotechnology industry. Patents that are involved in district court litigation are now routinely subjected to concurrent administrative litigation in the PTO, where they are being invalidated at rates so high that the basic procedural fairness of these proceedings is increasingly being questioned. This creates a great risk of duplicative proceedings and inconsistent outcomes, as alleged infringers seek to gain advantages or leverage over patent owners that would not exist under district court litigation alone. For example, the way claims are interpreted and other procedural protections are less favorable to patent owners in the PTO administrative setting.

In addition, third parties with no commercial interest in the patent or field to which the patent pertains have figured out that they can extort settlements or otherwise gain financially from bringing, or even threatening to bring, patent challenges against critical patents owned or licensed by biotech companies. Biotech companies can be particularly vulnerable to such extortion because – in contrast to most high-tech companies – biotech companies often rely on just a handful of highly valuable patents to protect their products and massive investment therein. This already is being seen by several biotech companies, who have been approached by third parties threatening to file IPRs unless the company makes a substantial financial payment to them. And a hedge fund manager recently made news by announcing his plans to "short" the stocks of more than a dozen biotech companies and then file IPRs against their most valuable product patents in an attempt to drive down their stock prices. The first such IPR petition, filed by this hedge fund in February against Acorda Therapeutics (a mid-size biotech company which brought to market an innovative treatment for multiple sclerosis) caused the value of the company to drop by over $150 million in one afternoon. A second IPR has now been filed against this same company, and other hedge funds are starting to get into the IPR business as well.

Such abuses of the PTO administrative review system are attractive and growing because, as is quite clear to those following the evidence to date, the rules governing these

proceedings are unfairly stacked against patent owners in many ways. In particular, the PTO uses a claim construction standard that is much broader than that used in district court, and has limited the ability of patent owners to file narrowing amendments to preserve their patent claims. This is why another hedge fund recently filed an IPR against a biotech company named Allergan, even though the patents at issue were upheld in district court litigation and on appeal. The hedge fund specifically notes in its PTO filing that it believes these same patent claims would fall under the PTO's broader claim analysis – a result that, to me, would be incredibly unfair after four years of court litigation on the same issues.

I don't believe that Congress intended for the IPR system to be used in this abusive manner. To this end, a number of productive proposals to reform the IPR system have been circulated that deserve this Committee's consideration. Specifically, the STRONG Patents Act, as recently introduced by Senators Coons, Durbin, and Hirono, would address such IPR abuses by, among other things, harmonizing the PTO's standards with those used in district court and thus minimizing incentives to "game" the two different systems; allowing greater patent amendment rights; and preventing the improper use of the proceedings by those with no legitimate interest.

Congress also should avoid making changes to the general patent litigation system that would raise the cost of or delay patent enforcement, as doing so would particularly impact small businesses most negatively. For example, efforts to vastly increase the amount of detailed information that must be included in every complaint for patent infringement, or proposals to delay discovery against accused infringers, would make it more difficult for small businesses to protect their inventions in a timely and cost-effective manner.

I also am concerned about several proposals that would grant the authority for a court to join third parties with a financial interest in the plaintiff or patent at issue – such as investors, licensors, or commercial partners – to the litigation as unwilling co-plaintiffs to pay the other side's costs, unless they renounce all interest in the patents at issue. The net result of such joinder provisions would be to create many additional encumbrances, especially for small businesses, that would make partnering, collaborations, and the enforcement of patents needlessly more expensive and more complicated. Business partners, patent owners, financing companies, and others who engage only in arm's length business with the patentee should not be subjected to potential liability or forced to renounce their rights just to avoid being dragged into litigation between two other parties.

While there have been efforts to limit the applicability of some of the above litigation changes to cases not involving real commercial competitors, the language is often imprecise and fails to recognize that not all patent litigation in biotechnology would fall into any such exceptions. In fact, the vast majority of American biotechnology companies are far from having a product on the market, yet depend critically on the enforceability of their patents to attract funding, to enter into development partnerships, and to advance their technology. A solution must be found for such businesses as well, businesses that are actively trying to develop, and seeking investment to further develop, patent-protected inventions.

Conclusion

I want to thank the Committee for the opportunity to testify today and explain a view of the patent system from the perspective of a small, innovative, investment-intensive biotech business. I urge the Members of this Committee and the full Senate to ensure that adopted reforms are truly targeted at abusive practices – both by patent owners and against patent owners – and do not have negative, unintended consequences for the vast majority of legitimate patent owners or licensees who simply are seeking to protect and enforce their patents in good faith.