House Bill 345
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Interested Party
Ohio House Health Committee
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Chairman Huffman, Vice Chairwoman Gavarone, Ranking Member Antonio and members of the House Health Committee, my name is Kristina Moorhead and I am here on behalf of the Pharmaceutical Research and Manufacturers of America (PhRMA), to briefly comment on House Bill 345, legislation that would create the Solemn Covenant of the States to award cash prizes for curing certain diseases. However, I plan to spend most of my time today addressing an important issue that has been brought in testimony before this committee; namely that the pharmaceutical industry is not conducting valuable research to find cures for diseases that impact your constituents and many Ohioans.

Let me state that PhRMA has taken no position on House Bill 345. As the bill is currently written, our member companies have indicated it would be most unlikely that they would choose to apply for a "cash prize" as described in the legislation. However, PhRMA companies remain committed to their research mission by trying to find cures and valuable treatments to complex diseases. House Bill 345 is a bold initiative, and if the General Assembly finds that the mechanism created in this bill serves as a needed stimulus for additional research, then PhRMA is not opposed to that effort.

The main reason I am here today is to discuss the incredible research that is being done by innovative biopharmaceutical companies to find cures and treatments for diseases and chronic conditions. Across the United States right now, there are men and women whose life's work in biopharmaceutical research has resulted in more than 7,000 potential cures and treatments in development today that have the potential to save and improve patient lives.

The process for developing new medicines is a challenging one that on average takes at least ten years for a potential medicine to make it from initial discovery to the marketplace for patient use. While ten years feels like a long time, consider that for nearly 2,000 years our understanding of disease and the human body was so limited that the primary treatment and cure for illness and disease was bloodletting. This practice was discarded only in the late 1800s when the first medicines were introduced, including aspirin in 1899.

The progress in understanding disease and the human body in the past one hundred years has moved at lightning speed compared to any other time in history, and the result has been that the average life expectancy in the United States has increased from 47 to 78 years of age largely due to new medicines that treat and cure disease.[1]

[1] Centers for Disease Control and Prevention

While you are probably aware of the groundbreaking discoveries of antibiotics in the 1940s and the polio vaccine in the 1950s, you may not be as familiar with lesser known research that is resulting in some of today's most innovative cures and treatments for disease.

It was the dedication of researchers beginning in the early 1990s to understand the human genome and the relationship between genes and disease that almost 30 years later is resulting in some of the most innovative, interesting, and successful cures and treatments.

MIT Technology Review wrote in December 2016 that the year had been gene therapy's most promising to-date.[2] One year later, they wrote that 2017 proved to be even bigger.[3] Two pioneering treatments that use a patient's own immune cells to fight rare types of cancer called Chimeric Antigen Receptor or CAR-T therapies were approved last year. In a clinical trial of children and young adults with acute lymphoblastic leukemia receiving one of these CAR-T therapies, 83% had their cancer go into remission within three months.

This past December, a gene therapy cure for an inherited form of vision loss and blindness in children and adults was approved by the FDA.[4] And on the horizon, initial research in gene therapy for sickle cell disease and hemophilia B is showing promising results that could cure these diseases in the coming years.[5,6]

New discoveries that provide cures to patients are not limited to gene therapies. The vaccination for the human papilloma virus was approved in 2006, a treatment that prevents cervical, head and neck cancers caused by the virus. With 70% of cervical cancer cases caused by the human papilloma virus, lower HPV infection rates due to the HPV vaccination are one step ahead of a cure by providing preventative treatment of HPV-related cancers in both men and women. And the vaccine has the potential eradicate HPV-related diseases with higher vaccination rates.

A new hepatitis C drug was approved for use in 2013 that resulted in a 95% cure rate for patients with the disease. With estimates of between 2.7 and 3.9 million people in the United States with a chronic hepatitis C infection, we now have a tool that both cures the disease and has the potential to eradicate hepatitis C if enough patients can be cured and transmission of the disease is halted.

While cures are the "holy grail" of biopharmaceutical research, many developments in treatments allow patients to live longer and better-quality lives. One unique perspective of an Ohio cancer patient facing Stage IV lung cancer without a cure belongs to Matt Hiznay, a young researcher at the Cleveland Clinic's Molecular Medicine Ph.D Program who is also researching cure for his cancer. Some of you may remember Matt as the featured speaker at PhRMA's 2017 Legislative Day and his personal story of genetic testing that revealed a cancer-causing gene mutation, and how he received a life-saving, brand-new gene therapy that had been available for

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[2] https://www.technologyreview.com/s/603206/everything-you-need-to-know-about-gene-therapys-most-promising-year/

[3] https://www.technologyreview.com/s/609643/2017-was-the-year-of-gene-therapy-breakthroughs/

[4] Biallelc RPE65 mutation-associated retinal dystrophy

[5] http://www.nejm.org/doi/full/10.1056/NEJMoa1609677

[6] https://www.technologyreview.com/s/601651/gene-therapy-is-curing-hemophilia/

less than a month. In a span of two months, Matt experienced a complete response to the cancer treatment and moved from the intensive care unit back to his normal life. Unfortunately, Matt has since experienced a cancer recurrence and his cancer cells have become resistant to the initial therapy that cured him. But even in a time span of only a few years, new research has resulted in a clinical trial for patients whose cancer becomes resistant to the initial gene therapy, which Matt is participating in while continuing his studies.

The work of pharmaceutical researchers has resulted in cures and treatments that have extended and improved the lives of patients, but failure is also a part of their story. Sometimes months and even years of research can lead to a "dead end" that does not produce the results and goals first hoped for and expected at the beginning of the journey to find a new cure. In fact, the likelihood that a drug entering a clinical trial will eventually be approved for patients is less than 12%. In spite of that, the amazing researchers at pharmaceutical companies spend every day working toward finding cures or meaningful therapies to improve the quality of life for you, your family, your friends, your neighbors and your fellow Ohioans.

But we understand that for patients with a serious disease, and their caregivers, it can be a heartbreaking reality to live in a world without a cure or treatment. We are seeing exciting cures and treatments for diseases where we have identified mutations and malfunctions in single genes. However, for diseases that involve multiple genes and environmental factors, such as Alzheimer's disease, diabetes and heart failure, finding that holygrail cure remains challenging as researchers work to understand the complex factors that cause disease.

But our researchers are not giving up on finding cures and treatments. Impatience to find the next treatment and cure in our industry is a virtue that drives us to work harder, smarter and faster to create hope for patients. We appreciate that same impatience virtue you also have as advocates for Ohio patients in pressing to find new cures and treatments. We are committed to continue working together pursuing this mutual goal, and look forward to the future where we can marvel at the work of researchers that results in new cures and treatments in coming years.