Testimony of

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Hearing on "THE FUTURE OF MEDICINE: LEGISLATION TO ENCOURAGE INNOVATION AND IMPROVE OVERSIGHT"

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Good morning, Chairwoman Eshoo, Ranking Member Guthrie, and the Members of the Subcommittee.

My name is Lucy Vereshchagina, Vice President, Science and Regulatory Advocacy at the Pharmaceutical Research and Manufacturers of America or PhRMA. PhRMA represents the country's leading innovative biopharmaceutical research companies, which are devoted to researching and developing medicines that enable patients to live longer, healthier, and more productive lives. Over the past 20 years, PhRMA member companies have invested more than $1 trillion in the search for new treatments and cures, including an estimated $91.1 billion in 2020 alone.

I am pleased to appear before you today to provide PhRMA's perspective on the future of medicine and the critical role that biopharmaceutical innovation plays in delivering life-saving medicines for patients.

PhRMA's key priority remains timely reauthorization of the Prescription Drug User Fee Act (PDUFA) and the Biosimilar User Fee Act (BsUFA) prior to the expiration of these programs later this year. As PhRMA stated at the Committee's February 3rd hearing on user fees, both programs are critical for ensuring patients have timely access to lifesaving medicines. PhRMA and its member companies strongly support the PDUFA VII and BsUFA III agreements as negotiated and are committed to working closely with Congress, FDA and all stakeholders to ensure the continued success of these programs.

Both the PDUFA VII and BsUFA III agreements were carefully considered by the biopharmaceutical industry and negotiated with FDA to ensure that the Agency is equipped with the necessary resources to help us deliver new treatments and cures to meet patients' unmet medical needs, and that the Agency is able to keep pace with ever-evolving science and have the necessary resources to ensure a timely review of innovative drugs and biologics, as well as biosimilar and interchangeable biosimilar products. Both agreements were thoughtfully negotiated with the FDA in a transparent manner, with patient organizations and others engaging with FDA through dedicated stakeholder discussions and public meetings.

PhRMA welcomes the opportunity to discuss the various policy proposals under consideration by the Committee. However, we would be concerned with any policy proposals and legislative riders that would undermine the negotiated user fee agreements and threaten passage of PDUFA VII and BsUFA III. PhRMA therefore urges Congress to reauthorize PDUFA VII and BsUFA III in a timely manner to prevent any disruptions to these critical programs and FDA operations. Delay would threaten timely access for patients to new drugs, biologic or biosimilars and create uncertainty for FDA staff. We need to ensure that the United States retains its global leadership in biopharmaceutical innovation.

I would like to highlight a few policy areas under consideration at the hearing today. First, as the Committee is considering legislative changes to the accelerated approval pathway, it is important to note this pathway has provided timely access to treatments for HIV/AIDS, cancers, and rare

diseases, leading to better health outcomes for millions of patients. It is important to stress up front that these products are approved under the same rigorous standards of safety and efficacy as traditional approvals. Furthermore, the PDUFA VII agreement contains efforts aimed at modernizing the U.S. regulatory and drug development paradigm and improving efficiencies in drug review. For example, FDA will update their review processes to provide timelier discussions and ensure agreement with sponsors on postmarketing requirements (PMRs), including for drugs and biologics approved under the accelerated approval pathway.

The longstanding accelerated approval pathway has helped expedite the availability of more than 200 treatments for patients with serious or life-threatening diseases and where there is significant unmet medical need. PhRMA member companies are committed to providing patients with safe, effective and high-quality innovative therapies, and the accelerated approval pathway helps further that goal. It is a critical tool for patients and regulators, and the biopharmaceutical industry continues to support the pathway in its current form.

Second, preserving incentives for rare disease drug development, including those under the Orphan Drug Act, is critical for continued research and development investments and providing hope to millions of Americans with rare diseases who still do not have access to FDA-approved treatments for their ailments. Rare pediatric cancers in particular are a very challenging area of research and development presenting unique scientific, ethical, and logistical considerations. The last user fee reauthorization, the FDA Reauthorization Act of 2017 (FDARA), included new requirements for pediatric studies of certain molecularly-targeted oncology drugs. It also required GAO to study and report to Congress on the effectiveness of these new requirements on the development of drugs and biologics for pediatric cancer indications. As the original pediatric provisions went into effect less than 2 years ago (in August 2020), additional time is needed to fully realize the full impact on pediatric oncology drug development. PhRMA believes it would be premature to make any changes or impose additional requirements while FDA and industry continue to implement these provisions and before the GAO assessment report is completed in August 2023.

Third, PDUFA VII will advance new and powerful tools – including novel clinical trial designs, digital health technologies, and real-world evidence – that will harness scientific advances, expedite drug development, and ultimately increase patient access to new therapies, including those with rare and pediatric diseases. The broader use of digital technologies during the pandemic has also allowed the continuation of clinical trials that otherwise may have been suspended. Further, remote or decentralized clinical trials facilitated by the use of digital health technologies can enable more diverse patient populations to participate.

Improving clinical trial diversity is a critical component in broader efforts to address deeply rooted disparities across the U.S. health care system. PhRMA and our member companies are enhancing diversity in clinical trials through a number of meaningful steps. In addition to long-standing company efforts to improve diversity in clinical trials, our members voluntarily came together to renew their focus and commitment to increasing health equity through the launch of the first-ever industry-wide principles on clinical trial diversity, held a stakeholder workshop and issued a report identifying and addressing the systemic challenges that deter Black and Brown communities from participating in clinical trials.

Making a meaningful change in clinical trial diversity requires all stakeholders including industry, patient and community organizations, medical providers, policy makers, and regulators, to work together to address the existing challenges. PhRMA shares the goal of enhancing diversity in clinical trials, but policies that would create additional mandates for sponsors would have serious unintended consequences of reenforcing rather than overcoming known barriers to participation for patients including; unfeasibly large and long studies, delayed access to medicines, or disincentives for industry to invest in highly risky therapeutic areas. Instead, stakeholders must come together to address these barriers through efforts including advancing a community-based infrastructure focused on clinical trial diversity in underserved communities.

In conclusion, PhRMA urges Congress to reauthorize PDUFA and BsUFA in a timely manner to protect against any disruptions to these critical programs. We look forward to continuing to work with the Committee, Members of Congress, and other stakeholders on these important issues. Thank you for the opportunity to provide this testimony. I would be happy to address any questions.