

Dr. Danielle Carnival
Deputy Assistant to the President for the Cancer Moonshot
The White House
1600 Pennsylvania Ave NW
Washington, DC 20500

October 17, 2023

Dear Dr. Carnival:

As leaders of research and development (R&D) efforts at America's biopharmaceutical companies, we share the goal of the Cancer Moonshot to make real progress toward ending cancer as we know it.

We, too, are in the business of moonshots.

Every day, tens of thousands of scientists at America's biopharmaceutical companies go to work with the hope that they will create the next breakthrough to change someone's life. We are devoted to taking calculated risks, to shooting for the stars, to pursuing the possibilities that are just out of reach – because sometimes we succeed. The progress we have made in the last decade alone in understanding human biology has opened an array of possibilities for not just treatments, but cures.

That is why we are deeply concerned that recent policies will inevitably result in fewer innovative treatments and cures for many diseases, including cancer.

Over the last several decades, America has been the engine of biopharmaceutical progress because of our unique innovation ecosystem – one that combines support for basic disease research in government and academia with strong intellectual property rights and private sector-led development and delivery of new medicines. Our companies have more than doubled our annual investment in the search for new treatments and cures over the last decade, including nearly \$101 billion in 2022 alone.

Today, there are more than 1,300 therapeutics and vaccines in development for cancer, all of which are in clinical trials or awaiting review by the U.S. Food and Drug Administration (FDA). In 2022 alone, 12 new therapies for a range of different cancers were approved by the FDA's Center for Drug Evaluation and Research. Since 1991, the overall cancer mortality rate has declined 33%, averting 3.8 million cancer deaths over this period. But our work is far from done.

Unfortunately, some of the administration's recent policies are putting that work at risk by creating obstacles to patient-centered drug development:

Reducing investment and development of cancer medicines. Congress passed the Inflation Reduction Act (IRA), which gives the Department of Health and Human Services unchecked authority to set the price of certain medicines in Medicare. Elsewhere in the world, government price setting discourages investment in innovation and ultimately limits access to new cures and treatments. We are concerned that the same will happen here in the U.S. and cancer patients will be especially hard-hit.

The IRA will have an especially pronounced negative impact on R&D of small molecule drugs – those that typically come in pill or tablet form. Small molecule drugs have a unique ability to target specific processes inside cells that allow tumors to grow and spread and may be more convenient for patients and improve adherence. But the IRA allows selection of small molecule drugs for price setting just seven years after they’ve been approved by the FDA, much earlier than other types of medicines. The president’s budget proposes cutting that time further to just five years. The consequences of this “pill penalty” are predictable – some companies will shift focus away from researching and developing small molecule drugs targeting cancer, as well as other complex diseases like mental health and neurological disorders.

Constraining post-approval R&D into new indications and other advances. Even after the FDA approves a medicine, we conduct more research to identify additional uses for earlier stage cancers or different types of cancers, disease settings or patient populations. This additional research is critical to fully realizing the patient benefit of approved medicines. Post-approval R&D takes years, and nearly half the time, these additional indications for medicines are approved seven or more years after the first approval. The IRA empowers the federal government price setting to occur so soon after initial FDA approval, threatening the continued investment and R&D necessary to evaluate the full therapeutic value of the medicine, cutting off hope for patients facing this devastating disease.

Limiting the availability of medicines approved by the FDA through the accelerated approval pathway. In 1992, Congress put in place the accelerated approval pathway at the urging of patients to help address the HIV/AIDS crisis. Accelerated approval has sped the development of new medicines for patients with serious and life-threatening conditions where limited alternative or viable treatments exist. Importantly, drugs with accelerated approval are subject to the same statutory standards for safety and effectiveness as traditional approval. In 2022, Congress provided FDA with additional authority to help ensure completion of required follow-up studies.

Now, despite congressional intent, the administration is overlooking the program’s success in driving longer life expectancy and better health outcomes by proposing policies that would limit Medicare payment for medicines granted accelerated approval. Nearly 70% of

medicines with accelerated approval have been in oncology, so patients with cancer will be disproportionately harmed. By limiting access to these treatments, the administration is sending a signal that scientists should abandon accelerated approval, which could mean lifesaving medicines could take longer to reach patients, with potentially devastating consequences.

Undermining intellectual property (IP) protections and public-private sector collaborations.

For biopharmaceutical companies, IP protections are the foundation upon which scientific innovation is built. The certainty of knowing an invention will be protected is what enables us to make the long-term, risky investments necessary to create new medicines. Similarly, the Bayh Dole Act provides the certainty for the private sector to partner with the federal government to translate research into useful commercial products across a multitude of sectors, including our own. The administration's reconsideration of march-in authority under the Bayh-Dole Act and its support for eroding IP protections by agreeing at the World Trade Organization to waive commitments to protect IP that enabled the development of COVID-19 vaccines creates uncertainty that could further chill R&D and inhibit our ability to address a broad range of diseases, including cancer.

To achieve the goals of the Cancer Moonshot, the administration should support policies that make the work by research scientists possible to deliver for patients and our society. We stand ready to partner with you to find solutions that leverage the best of American innovation to extend and improve the lives of those with cancer and other diseases. Together, we can make great strides without further damaging patient access to medicines or America's unique and productive innovation ecosystem.

Respectfully,

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