March 7, 2019

The Honorable Alex M. Azar II
Department of Health and Human Services
200 Independence Avenue, S.W.
Washington, DC 20201

Dear Secretary Azar:

The undersigned organizations, representing millions of American taxpayers, patients and consumers, are writing to urge you to embrace transformative healthcare opportunities on the horizon, including cell and gene therapies that represent the next revolution in medicine. Unfortunately, our current healthcare system is not designed to efficiently handle these new transformative treatments, which often involve higher initial costs, but offer significantly greater long-term value for patients and taxpayers.

There are currently nearly 300 cell and gene therapies in development, and Food and Drug Administration (FDA) Commissioner Dr. Scott Gottlieb has stated that "by 2025, we predict that the FDA will be approving 10 to 20 cell and gene therapy products a year based on an assessment of the current pipeline and the clinical success rates of these products." Cell and gene therapies are arguably among the most difficult treatment models ever created and costs associated with developing them reflect these challenges. In fact, the first gene therapy took more than two decades to get from initial study to a licensed product.

The immense amount of R&D that goes into these cell and gene therapies is often reflected in their prices. For example, a gene therapy that cures a form of congenital blindness costs $850,000. And, gene therapy can cost anywhere from $373,000 to $475,000 to treat specific types of cancer. At first glance these costs seem daunting, but when compared to a lifetime of managing a chronic disease or addressing a disease where no treatment exists, the financial benefits are clear.

As dozens of new cell and gene therapies are approved in the coming years, our healthcare system as currently structured is simply not prepared to handle treatments where initial costs are high, and savings are realized over a long period of time. So, it will be crucial for the Department of Health and Human Services (HHS), the Centers for Medicare & Medicaid Services, payers, and the industry to come together to create a framework to ensure patient access to gene and cell therapies as quickly as possible. Encouraging novel pricing and reimbursement agreements among the government, payers, providers, and manufacturers is essential to ensuring patient access to this technology that will save lives and reduce the long-term taxpayer burden.

Additionally, HHS' leadership is needed not just for the cures currently in the pipeline, but further innovation. If pharmaceutical and biotech companies continue to put resources towards potentially lifesaving gene therapies, they must be able to recoup those costs and be incentivized to reinvest in more new therapies for other deadly and debilitating diseases. HHS should take the lead in exploring creative payment models to provide certainty to researchers and ensure that upfront costs don't keep these groundbreaking treatments from patients, so that private sector innovators have the incentives necessary to develop the next generation of cures.

Again, we commend you for your commitment to addressing the high cost of healthcare in our country, and your efforts to expand access to revolutionary medicines. Cell and gene therapies have the potential to save lives and can offset significant direct and indirect costs to our healthcare

system, thus saving taxpayers money in the long run. We look forward to supporting HHS efforts to ensure these therapies are accessible to patients and further reduce long-term costs for all parts of our healthcare system.

Sincerely,

David Williams
President
Taxpayers Protection Alliance

Steve Pociask
President
American Consumer Institute

Brent Wm. Gardner
Chief Government Affairs Officer
Americans for Prosperity

Andrew F. Quinlan
President
Center for Freedom and Prosperity

Jeffrey Mazzella
President
Center for Individual Freedom

Tom Schatz
President
Citizens Against Government Waste

Matthew Kandrach
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Consumer Action for a Strong Economy

Nathan Nascimento
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Freedom Partners Chamber of Commerce

George Landrith
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Carrie L. Lukas
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