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Examining Policies that Inhibit Innovation and Patient Access

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The opinions expressed in this testimony are mine and were prepared by me.

Chairman Buchanan, Ranking Member Doggett, and members of the Health Subcommittee of the House Committee on Ways & Means, I appreciate the opportunity to submit this written testimony and to be asked to appear as a witness at this extremely important hearing. I frame this written testimony, opening statement, and answers to questions from the perspective of cancer treatment.

I am the Executive Director of the Community Oncology Alliance (COA), an organization dedicated to advocating for the complex care and access needs of patients with cancer and the community oncology practices that serve them. COA is the only non-profit organization in the United States dedicated solely to independent community oncology practices, which serve the majority of Americans receiving treatment for cancer. Since its grassroots founding 20 years ago, COA's mission has been to ensure that patients with cancer receive quality, affordable, and accessible cancer care in their own communities where they live and work, regardless of their racial, ethnic, demographic, or socioeconomic status.

My wife Susan practiced as a certified oncology nurse for 10 years, administering cancer therapies to patients with solid tumors. We have had family and friends with cancer, living with it and dying from the disease. **I want to make it very clear that my overriding goal is to ensure that every American with cancer, regardless of demographic, financial, or any other status, has access to the highest quality, most affordable cancer care close to home.** I should also add that my wife and I are both Medicare beneficiaries.

I am alarmed at what is happening right now with cancer care and will happen in years to come as a result of misguided and even destructive public policy. As I write this, oncology practices are dealing with a shortage of mainstay generic cancer drugs. Treatments are being delayed and oncologists are having to make decisions as to how to treat their patients with alternative and, typically, lesser therapies. Unfortunately, delays and denials of cancer drugs are something oncologists deal with daily at the hands of pharmacy benefit managers (PBMs). The top PBMs are an oligopoly with a stranglehold on the nation's prescription drug market. They fuel drug costs for Americans and are pushing independent pharmacists out of business, creating pharmacy "deserts" in rural areas. These PBMs have also merged with the top health insurers to throw "prior authorization" roadblocks, "fail first" step therapy, and other so-called "utilization management" tactics at oncologists to dictate how to treat their patients. Then, the PBMs mandate how, when, and where cancer patients will get their potentially life-saving drugs, often via PBM-owned mail order pharmacies.

I am equally alarmed by the rising cost of cancer drugs. Nothing I write or say defends pharmaceutical companies or lets them off the hook. They have primary responsibility for drug costs because they set the launch and subsequent list prices of prescription drugs. However, as I will explain, our country has a bizarre, convoluted health system where the "price" of drugs and the "cost" to patients are two very different and disjointed things. As Dr. Mark Fendrick of the University of Michigan and creator of value-based insurance design has often lectured me, when Americans talk about the high "price" of drugs, they are really referring to the high "cost" to them – namely, what they pay out-of-pocket. I believe that the pharmaceutical industry has to get more

proactive and creative in how companies approach pricing drugs, especially tying prices to drug effectiveness. At the same time, those in Congress and elsewhere who want to eviscerate the industry are basically signing death warrants for those Americans with cancer and other serious diseases. We all need to work together to arrive at meaningful, real solutions.

Our nation has made great strides in cancer treatment, especially with the increasing availability of immuno-oncology drugs (immunotherapies). As I was preparing this testimony, an oncologist called me about a 35-year-old woman who had recurring gastrointestinal, esophageal, and brain cancers since she was 18 years old. Six months ago, she developed a cancer in the small bowel that was metastatic (i.e., spread to other organs). She was put on a treatment regimen including immunotherapy. After four months, she is in complete remission. My wife calls these therapies nothing short of "revolutionary" as she witnessed over her 10 years as an oncology nurse as immunotherapies became available for treating cancer..

The good news in cancer treatment is that we have more precise diagnostic tools and drugs. Mortality from cancer is decreasing and Americans with the disease are living longer.[1] The bad news is the cost of treating cancer is increasing, especially as new, innovative drugs available have much higher prices. Unfortunately, both underlying drug prices and out-of-pocket costs are the result in part due to misguided public policy that has piled bad policy and regulation upon bad policy and regulation. Rather than having a healthy economic market for drugs that fosters competition, which in turn motivates innovation and controls prices, we have a highly regulated market with forced price controls and mandatory discounts that leads to shortages, stifles innovation, and actually increases costs for patients and taxpayers.

Let me now discuss the impact that new public policy and regulation will have on cancer treatment, as well as patient cost and access.

Inflation Reduction Act (IRA)

There are certain provisions of the IRA that are positive in helping lower the out-of-pocket costs of prescription drugs for Medicare beneficiaries like me and my wife. However, the provision empowering the government (Medicare) to "negotiate" drug prices is fraught with numerous perils. It is clear that the reality of how groundbreaking new pharmaceuticals are developed, especially cancer drugs, is simply not understood.

A cancer drug is typically indicated for not just one cancer type (e.g., breast cancer) but for a variety of cancers, even subsets within cancers (metastatic breast cancer, HER2-, etc.). For a variety of reasons, what typically happens is a new cancer drug is introduced with one or maybe two indications. Then, as the drug is approved and used to treat cancer, a pharmaceutical company

[1] "Risk of Dying from Cancer Continues to Drop at an Accelerated Pace", American Cancer Society, January 12, 2022.

will conduct research and development on other indications (e.g., other cancers, expanded indications within a cancer, different drug combinations, etc.) for the drug.

As one example, the Food & Drug Administration (FDA) approved a small-molecule oral targeted therapy, Imbruvica (ibrutinib), for mantle cell lymphoma on November 13, 2013. Subsequent to that, the FDA approved the drug 11 times since 2013 for other indications and drug combinations, with the most recent approval on August 24, 2022, for pediatric patients with chronic graft-versus-host disease. It is the first and only drug in its class to be approved for the treatment of children with this disease.

Imbruvica is likely to be one of the initial Medicare Part D drugs targeted for "negotiation." I call your attention to a current article in Health Affairs[2] examining the complexity of "negotiating" the price of Imbruvica for just one of its indications. Understand that there will only be one "negotiated" price of the drug, yet the drug, as with most other cancer drugs, has varying relative value in different types of cancer. How will this reasonably be accounted for in one "negotiated" price of the drug?

Additionally, with a limit on the years of exclusivity before a drug with no generic or biosimilar competition gets "negotiated," there will likely be two consequences. First, drug manufacturers will increase the launch prices of their drugs knowing that in time, if a drug has no competition, its price will be "negotiated" downward. This means that under the IRA, all patients, not just Medicare beneficiaries benefiting from the lower "negotiated" price, will be paying more for their drugs because, ironically, *launch prices will increase*. Second, and especially disconcerting, is that drug companies will not invest in expanded indication research anytime near when a drug will be the target of government "negotiation." This will most certainly limit research and development for new indications and uses of a drug. For example, in the case of Imbruvica, it is highly unlikely that the pediatric breakthrough use of the drug would have been invested in and investigated. And it's important to note that many indications in pediatric cancers are developed after cancer drugs are first approved for adult cancers.

Please understand, I am not justifying or defending any pricing decisions relative to Imbruvica. I am simply using it as a case study of how a drug's lifecycle is complicated and as a convenient example to consider given the just-released Health Affairs article.

With Medicare Part B drugs subject to drug price "negotiations," it is even more complex than with Part D. These are drugs that have to be administered (infused or otherwise) under direct physician supervision. As such, the "negotiated" price of the targeted Medicare Part B drug will create a second Medicare reimbursement rate – the "negotiated" "maximum fair price" (MFP) rate for providers, in addition to the current reimbursement rate based on "average sales price" (ASP). Not only will this create an operational nightmare of having two reimbursement rates for community oncology practices and other independent physician Part B providers, but it will

[2] "Medicare Price Negotiation: The Example Of Ibrutinib", Health Affairs Forefront, May 2, 2023.

drastically reduce the variable component of Medicare reimbursement above the fixed "negotiated" MFP. According to an analysis by Avalere Health[3] this variable add-on reimbursement would be reduced by 49.5 percent.

As crafted, the IRA puts providers in the middle of the "negotiations" between the government and drug companies. Rather than a rebate provided by the drug company to Medicare for the "negotiated" amount, the IRA creates this second reimbursement rate based on MFP in addition to the ASP-based rate. This is not only an operational challenge to administer but is a drastic payment cut to the "negotiated" drugs. Additionally, the MFP will be included in the calculation of ASP, thus further driving down total reimbursement.

History has clearly documented that repeated and misguided cancer care payment cuts cause independent cancer care providers to close or merge with expensive hospital systems.[4] When independent practices close, medical care almost always shifts to much more expensive hospitals. Furthermore, access to care is threatened as cancer clinics and other specialty facilities simply close, especially in rural areas, due to financial pressures. Ironically, this results in higher out-of-pocket costs for patients and in access issues, especially in rural areas.

As with Part D drugs, given the lifecycle development of indications of Part B drugs over time, it is especially concerning how innovation in new cancer drugs and new indications will be impacted by misguided and poorly implemented federal public policies, such as the IRA. In effect, this is a grand experiment on the nation's cancer care system but without any safeguards or small-scale demonstrations, or pilots to guard against unintended consequences. Think about this as akin to developing a drug. Before any widescale clinical research is conducted, limited trials are conducted to assess the safety and basic effectiveness of the drug. That is not happening here.

I now call the Committee's attention to examples of how new, innovative cancer therapies are producing remarkable results.

A 75-year-old female developed stage IV breast cancer in 2018 that was ER/PR negative and HER2+. She received appropriate indicated treatment for her type of cancer and achieved near complete remission in 2019. She did well until the summer of 2022 when she developed a right-sided neck lesion. Her biopsy confirmed recurrent disease with metastases to bone, lungs, and lymph nodes. She received the same treatment as before, and her follow-up scans in April revealed disease progression. She had a large node (the size of a golf ball) on the right side of her neck. She was depressed and planning to give up. Her oncology team decided to switch her over to an innovative new therapy. After just one dose, she had a significant reduction in the size of the mass in her neck.

[3] "IRA Medicare Part B Negotiation Shifts Financial Risk to Physicians", Avalere Insights & Analysis, November 29, 2022.
[4] "2020 Community Oncology Alliance Practice Impact Report", Community Oncology Alliance, April 24, 2020.

A 40-year-old teacher with metastatic breast cancer is on immunotherapy (in conjunction with chemotherapy) and now has no evidence of disease for more than two years. She continues to teach, which is remarkable because her triple-negative breast cancer has an average survival rate of just one year.

A 91-year-old with melanoma that had spread to his liver and brain underwent surgery, radiation therapy, and an immunotherapy that was granted accelerated approval for exactly his disease scenario less than a year after FDA approval. That patient is former United States President Jimmy Carter.

I can go on and on about the 40-year-old who presented with a near terminal diagnosis at age 31 but, after a year of immunotherapy nine years ago is alive and working. Or the 55-year-old now eight years from an initial presentation of stage IV melanoma with metastatic disease to the liver and brain, who is in remission after two years of immunotherapy. This is the innovation we must protect and foster.

In relation to biosimilars, the IRA does increase Medicare reimbursement for biosimilars. However, it is important to understand that according to a recent research paper in Health Affairs,[5] biosimilar adoption in 340B hospitals is lower and use of more expensive biologic drugs is higher. According to the investigators, *"Our findings suggest that the* [340B] *program inhibited biosimilar uptake, possibly as a result of financial incentives making reference drugs more profitable than biosimilar medications."* This certainly jeopardizes the continued development of a healthy biosimilar market, which has so much promise in bringing down the prices of expensive biologics.

The Accelerating Clinical Evidence Model

Following President Biden's *Executive Order on Lowering Prescription Drug Costs for Americans*[6], the Health and Human Services (HHS) Secretary issued a report[7] directing the CMS Innovation Center (CMMI) to test models relating to the Executive Order. One such model is the *Accelerating Clinical Evidence Model*, which *"would adjust Medicare Part B payment amounts for Accelerated Approval Program (AAP) drugs to give manufacturers an incentive to expedite and complete confirmatory clinical trials"* per the HHS report. However, the report goes on to say that, *"Although drugs with multiple indications make up a large portion of accelerated approvals, CMS Part B fee-for-service drug payments are not tied to specific indications, making a variable, indication-based pricing scheme difficult to implement."*

As background, the AAP is extremely important to producing new, innovative treatments for many types of cancer and to expediting their availability to cancer patients. However, as with the IRA,

[5] "The Role Of Financial Incentives In Biosimilar Uptake In Medicare: Evidence From The 340B Program", Health Affairs, May 2023.
[6] "Executive Order on Lowering Prescription Drug Costs for Americans", White House, October 14, 2022.
[7] "A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans", HHS, February 14, 2023.

there are two problematic themes in this proposed *Accelerating Clinical Evidence Model*. First, HHS acknowledges that drugs with multiple indications, which is the case with most cancer therapies, make up a large percentage of accelerated approvals. What this means is that because Part B drug reimbursement is not tied to specific indications, HHS could lower reimbursement for a drug because clinical trials are lagging *for just one indication*. Second, as with the IRA, this model puts providers in the middle of the government and drug manufacturers. In this case, providers are "hostages" of sort, being used as an "incentive" for drug manufacturers to timely proceed with clinical trials related to accelerated approval drugs. This is yet another pressure point that will simply drive independent oncology practices into the arms of more expensive health systems, typically those making money off of 340B drugs. Additionally, this will likely force drug manufacturers to rethink and limit research into expanding a drug's indications. Coupled with the IRA, this is a chilling prognosis for the impact on new cancer therapies and indications.

I also want to comment on the use of CMMI to essentially change Medicare drug reimbursement without new laws from Congress. The prior two administrations attempted to use CMMI three times to lower Medicare drug reimbursement for the vast majority of the country. Let me state for the record that I was a big supporter of CMMI as a means of bringing innovation to CMS in "testing" models in a contained phase one pilot before rolling the model out in phase two nationally or at least to a larger population after models have demonstrated success without negatively impacting patients. That was certainly the intent of Congress and the letter of the law in crafting CMMI. However, unfortunately, CMMI has become a vehicle for the executive branch to end-run Congress in order to change reimbursement on a large scale without any phase one limited testing. That is not the law and not the intent of Congress in crafting CMMI.

Generic Drug Shortages

The current shortages of key generic mainstay cancer drugs include cisplatin, carboplatin, methotrexate, BCG, and even sterile water. Drug shortages are nothing new as I testified to Congress over 11 years ago.[8] The current shortages can be blamed in part on being exacerbated by COVID-related supply chain issues and historic inflation, but the root cause is the same: financial. Why is there less financial incentive for drug makers to manufacture generic drugs? Simply because the mandatory discounts (340B) and rebates (PBMs), where applicable, make these products a financial loser. This ties right back to the concept of government intervention in driving down prices – essentially, price fixing – that will certainly make drug manufacturers more selective in what drugs and indications they research and develop. And as I write this testimony, I am hearing from community oncology practices across the country that are running out of these essential drugs and will be forced to make very difficult treatment decisions. *This is a true crisis!*

[8] "Testimony on: Drug Shortages Crisis to the United States House of Representatives Committee on Oversight and Government Reform Subcommittee on Health Care, District of Columbia, Census, and the National Archives", Ted Okon, Community Oncology Alliance, November 27, 2011.

Conclusion

The United States has way too much bad public policy that is negatively impacting and distorting the prescription drug market and, unfortunately, is now adding more. If Congress is not careful, it will end up putting even greater pressure on independent physicians, forcing them to continue to merge into ever consolidating expensive large hospitals and health systems, most with 340B drug discounts. The result will be that, regardless of how low drug prices are "negotiated" down, Americans will end up with higher out-of-pocket costs for drugs and their overall care. And access to innovative oncology and other specialty providers will be limited, especially in rural areas and made more severe due to health care workforce shortages.

As I have repeatedly stated, drug companies have a major role to play in the underlying pricing of drugs. They need to be more proactive and creative in how drugs are priced. However, that requires regulations and bad public policies impeding value-based and other creative pricing mechanisms are removed. Additionally, to ignore that there are many forces at work – notably, PBMs and 340B hospitals – fueling drug costs higher for Americans is sticking our collective heads in the sand. Fortunately, it is encouraging to see both the Senate and House coming together in a bipartisan spirit to address PBM issues.

It is extremely important that we increase access to mainstay generic drugs and, most importantly, to innovative new cancer therapies. I just heard from an oncologist as I was preparing this testimony about a 74-year-old woman whose lung cancer was so extensive it eroded into her heart, and she almost died. Now, after two years of immunotherapy, with no harsh chemotherapy, she has no sign of cancer and has lived a normal life for over four years, the last two requiring no treatment. Or the case of a 58-year-old man with such extensive lung cancer that he required emergency abdominal surgery because the cancer had spread and caused his bowel to perforate. After immunotherapy alone, he has been in remission for over two years and would never have survived more than a few weeks or months at best without this treatment. This was previously unheard of in cancer treatment.

It is unsettling that the *Coverage with Evidence Development* (CED) program, which is intended to allow drugs approved by the FDA to reach patients sooner, may be turning the corner and being used to limit payments for drugs approved. This is just more regulatory action by the payment agency (CMS) limiting access to new drugs approved by the safety and effectiveness (FDA) agency.

I will conclude by offering some specific summary recommendations for Congress related to innovation, access, and drug costs.

- Congress needs to ensure that new regulation and law does not hinder drug innovation, especially with cancer and other types of therapies that have long lifecycles of new indication approvals.
- Congress needs to remove providers from the middle of government and drug company "negotiations." Rather than create a new reimbursement (MFP) in addition to the current

rate (ASP), the "negotiated" amount should be rebated from the drug maker to Medicare. Rebate mechanisms are already in place in government programs *and the IRA* to do just that.

- Congress needs to put "guardrails" on CMMI so that it tests smaller pilot models which show clear success before larger demonstrations or rollouts. CMMI should be a testing agency, not a vehicle for the executive branch to bypass Congress.
- Congress needs to ensure that CMS does not overstep the boundaries of its mandate versus that of the FDA in using CED to block Medicare beneficiaries like me and my wife from having access to innovative, potentially lifesaving drugs. Congress needs to legislate guardrails to keep CMMI true to is mission and congressional intent.
- Congress needs to understand the complexities of differences between drug "prices" and "costs" and address the intermediaries who profit off of drugs, resulting in the widening of the gap between drug "list" prices and "net" prices.

COA stands ready to work with Congress on these recommendations and others. We want to provide meaningful input on ensuring that drug costs come down for Americans with cancer and other serious diseases, as well as fostering research and availability of innovative new cancer therapies and incentivizing the manufacturing of essential generic drugs.

I appreciate the opportunity to provide this testimony.

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