STIFLING INNOVATION: EXAMINING THE IMPACTS OF REGULATORY BURDENS ON SMALL BUSINESSES IN HEALTHCARE

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STIFLING INNOVATION: EXAMINING THE IMPACTS OF REGULATORY BURDENS ON SMALL BUSINESSES IN HEALTHCARE

WEDNESDAY, MAY 8, 2024

HOUSE OF REPRESENTATIVES,
COMMITTEE ON SMALL BUSINESS,
Washington, DC.

The Committee met, pursuant to call, at 10:03 a.m. in Room in Room 2360, Rayburn House Office Building, Hon. Roger Williams [chairman of the Committee] presiding.

Present: Representatives Williams, Luetkemeyer, Stauber, Meuser, Van Duyne, Ellzey, Molinaro, Alford, Crane, LaLota, Maloy, Velázquez, McGarvey, Gluesenkamp Perez, Scholten, Thanedar, and Davids.

Chairman WILLIAMS. I would like to welcome everybody. And before we get started, I want to recognize Congressman Stauber from the great State of Minnesota to lead us in the pledge and the prayer.

Mr. STAUBER. Dear Lord, thank you for bringing us together once again in this Small Business Committee. We thank you for the Chairman and the Ranking Member and calling on both sides of the aisle, and we ask that this be a productive meeting.

We thank the witnesses for traveling here. We thank them for their safe trip here and ask that they return safely back to their families.

In your name, we pray. Amen.

Please join me in the pledge.

I pledge allegiance to the Flag of the United States of America, and to the Republic for which it stands, one nation, under God, indivisible, with liberty and justice for all.

Chairman WILLIAMS. Good morning, everyone. And I now call the Committee on Small Business to order.

Without objection, the Chair is authorized to declare a recess of the Committee at any time.

I now recognize myself for my opening statement.

Welcome to today's hearing which will focus on how overregulation in the healthcare industry limits small firms' competitiveness and stifles innovation.

I would like to start off by thanking our witnesses for joining us here today. Thank you for being here. Your attendance is greatly appreciated, and we value your input on these important issues.

Now, we have consistently heard how overregulation is preventing some of our best and brightest entrepreneurs from inno-

vating and trying new things. We have heard this from many different industries, and today we will examine how these efforts can still stifle innovation in the healthcare industry.

The FDA is responsible for protecting the public's health by ensuring the safety and efficiency of the new pharmaceutical drugs, as well as medical devices. There is a lot of responsibility that goes along with this authority. For obvious reasons, we want to make sure that the medicines people are taking are going to be fulfilling their intended purpose. However, there is always a tradeoff in these decisions. And if the FDA shies away from any and all risk, it will significantly limit innovation and make it harder for small businesses to make an impact in the healthcare industry. The balance between risk and innovation is a fine line, and we must be able to maintain that.

This Committee knows that small businesses are on the tip of the spear across the economy when it comes to innovation in any industry. The healthcare industry is not an exception. Developing a new drug can take more than a decade and bring with it an extremely high price tag. Unfortunately, on top of the cost of development, small businesses have to spend significant time navigating the FDA's bureaucratic process. This presents a significant barrier to entry for main street. So navigating red tape does not only hamper what drugs come to market but also how doctors are treating patients.

In a hearing we held earlier this year, we heard from a doctor who was serving as a witness say they only spend 12 percent of their day focusing on direct patient care. The other 88 percent is spent complying with government and private insurance requirements. Doctors want to treat, cure, and innovate, not spend time on bureaucratic red tape.

This issue is very personal to me. My wife is currently undergoing treatment for glioblastoma. Throughout this process, I have heard countless stories from Americans looking for cures and learned about many of the challenges in bringing new treatments to the marketplace.

As a small business owner myself, I know the potential that lies within Main Street America today, and we just need to make sure they can operate in an environment that allows them to flourish.

I am looking forward to today's discussion, and I hope the hearing shines a light on the burdensome red tape currently restricting our small firms and providers.

I ask unanimous consent to submit three op-eds from the SBE Council for the record. And, without objection, I will order that.

I would like to, once again, thank our witnesses again for being here with us today, and I am very much looking forward to our conversation.

With that, I would like to yield to our distinguished Ranking Member from New York, Ms. Velázquez.

Ms. VELÁZQUEZ. Thank you, Mr. Chairman.

I would like to thank all of the witnesses for being here today.

We all know that small businesses are the lifeblood of the economy, but that is especially the case in healthcare, where small companies are at the forefront of technological innovation and are de-

veloping many groundbreaking new products. And their influence is growing each year.

According to one study, the proportion of new drugs discovered by small startups more than doubled between 2009 and 2018, and by some accounts, now makes up as much as 80 percent of the market. Yet despite their outside role in advancing innovation, small firms encounter significant barriers.

The process of bringing a new drug or device to market can be costly and time-consuming because of the need to prove the safety and efficacy of the product. The FDA is responsible for protecting us all from potentially harmful substances or defective devices. For most, new drugs manufacturers often must conduct animal testing before obtaining approval to conduct clinical trials on humans, and those clinical trials can take years and cost millions of dollars.

Some will say this process stifles innovation, but ensuring medical products prove their safety and clinical effectiveness prior to going on the market is necessary for public trust, individual safety, and advancing genuine innovation.

With that said, it is important we recognize FDA's attempts to balance scientific scrutiny with regulatory flexibility. Congress and the FDA have long sought to expedite this process, particularly for rare disease treatments. Expedited programs of the FDA help bring potentially life-saving treatments to the market much quicker, bringing hope to many patients and their families.

Unfortunately, that does not come without potential for abuse. Occasionally, drug companies use expedited programs to circumvent placebo-control studies, then drag their feet on conducting the required follow-up studies. As a result, companies are able to garner millions of dollars in sales without proving clinical effectiveness. While I have full faith in the FDA's decision-making process, there must be safeguards in place to prevent drug companies from taking advantage of regulatory flexibility.

Our regulatory system in healthcare is essential for keeping patients safe and delivering genuine innovation to the market, but that cannot be done without strict scientific standards.

I hope today we can discuss ways to support small businesses and their role in saving lives with new innovation, while also recognizing the need to prove those products are safe and effective for human use and consumption.

I would like to thank all the witnesses again for being here, and I yield back.

Chairman WILLIAMS. The lady yields back.

And I will now introduce our witnesses.

Our first witness here with us today is Dr. Brian Miller. Dr. Miller is an assistant professor of medicine at the Johns Hopkins University School of Medicine located in Baltimore, Maryland. In addition to being an assistant professor of medicine, Dr. Miller is a nonresident fellow with the American Enterprise Institute where he focuses on a variety of issues, such as Medicare payments, Food and Drug Administration, and healthcare competition.

Dr. Miller earned a medical degree from the Feinberg School of Medicine at Northwestern University, a master's of business administration from the Kenan-Flagler Business School at the University of North Carolina at Chapel Hill, a master of public health

from Johns Hopkins Bloomberg School of Public Health, and two bachelor of science degrees in biochemistry and chemistry from the University of Washington.

I want to thank you for joining us here today, and we look forward to the conversation ahead.

I now recognize my colleague, Representative LaLota, from the great State of New York, to briefly introduce his constituent who is appearing before us today.

Mr. LALOTA. Thank you, Chairman.

It is my privilege to introduce our next witness, Dr. David Eagle, M.D. Dr. Eagle is a board certified hematologist oncologist, and has practiced medicine for over 20 years. He is currently the Chair of legislative affairs and patient advocacy at the New York Cancer & Blood Specialists located on Long Island in Patchogue, New York, just outside of my district.

Dr. Eagle has published multiple oncology, health policy, and cost-of-care articles. He has previously served as an editorial board member for the journal Oncology. He also has appeared on The Oncology Show on Sirius XM's Doctor Radio channel, and provided briefings on oncology health policy for congressional staff right here on Capitol Hill.

Dr. Eagle is a past president of the Community Oncology Alliance and a member of the American Society of Clinical Oncology. He graduated from the University of Virginia School of Medicine, and then completed his internal medicine residency and fellowship at the University of Florida.

Thank you, Doctor, for joining us today. We look forward to the conversation ahead.

Chairman WILLIAMS. The gentleman yields back.

And now our next witness here with us today is William Newell. Mr. Newell is the chief executive officer of Sutro Biopharma, Inc., located in South San Francisco, California. Mr. Newell has served as CEO of the Sutro Biopharma for over 15 years, developing cancer therapeutics for areas of unmet needs. Previously, he served as the president of Aerovance, Inc., a biotechnology company focused on respiratory diseases.

Mr. Newell currently serves on the boards of directors of the Biotechnology Innovation Organization's Health Section, Emerging Company Section, and is a member of the executive committee. Mr. Newell received an AB in government from Dartmouth College and a JD from the University of Michigan Law School.

Thank you for joining us today, and we look forward to the conversation ahead.

And I now recognize the Ranking Member from New York, Ms. Velázquez, to briefly introduce our last witness appearing before us today.

Ms. VELÁZQUEZ. Thank you, Mr. Chairman.

Our final witness is Dr. Diana Zuckerman, the President of the National Center for Health Research, a nonprofit public health think tank that conducts and analyzes research on health policy issues. She is trained as a post-doctoral fellow in epidemiology and public health at Yale Medical School.

Dr. Zuckerman worked in the House and Senate and then as a senior White House advisor. She was also a fellow at the Univer-

sity of Pennsylvania Center for Bioethics and the first nonphysician elected to the Women in Medicine International Hall of Fame.

Welcome, Dr. Zuckerman, and thank you for being here this morning.

Chairman WILLIAMS. Okay. The lady yields back.

And before recognizing the witnesses, I would like to remind them that their oral testimony is restricted to 5 minutes in length, and we stick with that. If you see the light turn red in front of you, it means your 5 minutes have concluded, and you should wrap up your testimony. Now, if you keep going, you are going to hear this. Okay? And that will—we will want to wrap it up, as I say.

I now recognize Dr. Miller for his 5-minute opening remarks.

STATEMENTS OF DR. BRIAN J. MILLER, M.D., M.B.A., M.P.H., ASSISTANT PROFESSOR OF MEDICINE, JOHNS HOPKINS UNIVERSITY SCHOOL OF MEDICINE; DR. DAVID ANTHONY EAGLE, M.D., MEDICAL ONCOLOGIST & CHAIR OF LEGISLATIVE AFFAIRS AND PATIENT ADVOCACY, COMMUNITY ONCOLOGY ALLIANCE; MR. WILLIAM J. NEWELL, J.D., CHIEF EXECUTIVE OFFICER, SUTRO BIOPHARMA, INC.; AND DR. DIANA ZUCKERMAN, PRESIDENT, NATIONAL CENTER FOR HEALTH RESEARCH

STATEMENT OF DR. BRIAN J. MILLER

Dr. MILLER. Thank you, Chairman Williams, and Ranking Member Velázquez, and distinguished Members of the Committee.

I am a practicing physician at Hopkins, an internist, a nonresident fellow at the American Enterprise Institute. I run a large policy research group, and I actually worked at the FDA. I was a reviewer in the Center for Drug Evaluation in the Office of New Drugs. I also serve on the Medicare Payment Advisory Commission, and I previously served 4 years on CMS' Medicare Evidence Development & Coverage Advisory Committee.

Today I am here in my personal capacity. My views are my own and not those of Hopkins, the American Enterprise Institute, or MedPAC. Disclosures aside, I want to start and talk about innovation.

So my grandmother was this tiny, little, part-Norwegian lady who would bake for us every year at Thanksgiving. And she loved to garden. And I remember as a kid, for a couple years she couldn't bake pie for us—and I love food. It is a problem. I love carbs. I remember her hands being red and swollen.

Then I remember one year she baked a pie again, and I remember that year because that was the year the first TNF alpha inhibitor was introduced, and her rheumatoid arthritis was able to be treated. So she could bake again, garden, and go about her life.

So innovation, for me, when I think about it in my head, the first thing I remember is eating apple pie as a child. So innovation is not really just a number; it is your life.

A bipartisan Congress has worked for 30 years on pharmaceutical product, innovation, and promoting safe medical products. I saw the direct impact of these tools when I worked at the FDA, tools like accelerated approval, which is an objective science-based mechanism for early market entry for drugs meeting an unmet

need based upon a surrogate end point. There has been a lot of critique of these sorts of tools recently.

We should remember where they came from. In the eighties we had the AIDS crisis, and activists from ACT UP actually occupied the FDA for a day in 1988 and made the news. They didn't have access to treatments, the FDA was a barrier, and products were available in other countries. And this started a 30-year cycle of FDA reform.

If we actually read the studies from the critics of these pathways, it says that many of these studies get done. They sometimes get done slowly. When I look at this and say, if these trials are done late, yes, maybe the company should—there are other things that the company can or should do, but it also tells me that we need to change how we do clinical trials.

And, for me, as I said, it is not just my grandmother, it is personal. My father died of glioblastoma 2 weeks before I moved to Chicago and started medical school, and he was 58. For me, innovation is about people and their lives and their functional status.

I think about clinical trials—we are stuck in the seventies. The FDA has promoted concepts as real-world evidence, patient-report outcomes, but we have to make it real. Making it real could mean for something like COPD, you do a 6-minute walk test at home, you assess spirometry for lung function at home. You do for Parkinson's, say, a video interview to assess their ability to move.

All these outcomes are patient-focused, and they are and can be done in the setting of the patient's home. We are not necessarily forcing the patient to drive to a study center. They also decrease the cost of trials, which allows for small companies to develop innovative products, and they also increase the diversity of the patients who participate in clinical trials.

How do we do this? Well, you need the FDA reviewer to be the counselor to the company, because the reviewer is sitting on top of an entire therapeutic area at the FDA. The problem is that there is too much management, too many administrative layers at the FDA. If you are a reviewer, you have your—you are doing the work, then you have a team leader, you have a deputy division director, a division director, then you might have an office director, and then you might have a super office director, all of whom are highly trained technical physicians and scientists reviewing the work that you already did.

So I think that we need to collapse some of those layers of administration, put those people back on the front line of reviewing medical products for efficacy and safety so that they can be a better counselor to industry, and particularly small business and entrepreneurs.

Other things that we can and should do include returning the staff to the office so that if you are a small company, you are not just facing a sea of black on Zoom.

In conclusion, I think there are a lot of things that we can do that are pragmatic and that are bipartisan to help lower barriers to entry for small companies and entrepreneurs to safely—to create safe and effective, innovative products to treat disease and help everyday Americans.

And I thank you.

Chairman WILLIAMS. Thank you.

And I now recognize Dr. Eagle for his 5-minute opening remarks.

STATEMENT OF DR. DAVID ANTHONY EAGLE

Dr. EAGLE. Chairman Williams, Ranking Member Velázquez, and Members of the House Committee on Small Business, thank you for the opportunity to participate as a witness in this critically important hearing on regulatory and related burdens in small businesses in healthcare.

I am a practicing medical oncologist with New York Cancer & Blood Specialists, and serve as Chair of legislative affairs and patient advocacy. I am a board member and past president of the Community Oncology Alliance, a nonprofit group advocating for independent nonhospital-owned community cancer care.

My career began in a small practice, three-physician independent community oncology practice, in Mooresville, North Carolina. I have been privileged to care for patients in an era of immense scientific progress in overcoming a disease as it threatens to take our closest loved ones from us.

My physician partners and I work closely with our nurses, nurse practitioners, medical assistants, and ancillary staff, caring for patients battling cancer and blood disorders. We were a family.

We live with our patients on the front lines of an increasingly broken medical system. We often acted as their last line of defense, not only for their medical illnesses, but also for the confusing and overwhelming medical system in which they and we, as their providers, increasingly were fighting.

Barriers to care, both large and small, sometimes incidental, other times intentional, popped up all over the place and were almost always far too much to bear for patients facing the battle for their lives.

Over time, our small practice, operating as a small business, encountered significant pressures from large, well-funded hospital oligopolies in the Charlotte area and beyond in North Carolina. In 2017, one large health system in our region gave us an ultimatum: Be acquired by us or we will hire physicians to compete against you.

One of the prime motivators for this aggressive move by the health systems was the federal 340B Drug Pricing Program. The acquisition of our practice would generate substantial immediate profits for the health system, allowing it to further expand. Furthermore, we were faced with declining payments from Medicare and commercial insurers.

In 2018, my two partners and I had little choice but to join the large hospital systems of employees. Our small independent practice that had served the community for over 19 years was gone.

Hospital clinics operate under stifling bureaucracies, and as a result, almost immediately I was unable to see the same number of patients that I was able to see daily in my own independent practice. As devastating, the hospital later switched over its billing system and was able to charge significantly more for the same services. Patients came to the same building, were treated by me, their same physician, and received the same drugs but at a higher cost to them. Patients who I treated and followed for years simply left.

Due to consolidation of hospitals into large health systems in North Carolina, I joined my current practice in New York. Unlike the large health systems, we are the only major cancer provider in our region that accepts all insurance plans, while also being the only major cancer treatment provider that does not receive State funding or other subsidies. We have open clinics in underserved communities, are the lowest cost cancer providers in all of our markets, and were recently named the number one physician practice in New York by Castle Connolly, a rating system based on physician peer reviews.

Cancer research, drug development, and care delivery are in a renaissance. I am privileged to practice in an era of breakthrough scientific progress. However, patients often do not get the treatment that they need or in the best manner possible.

We face a fundamental choice in this country. Who is in charge of patient medical care? Is it physicians and patients making decisions on treatments together in the exam room? Or is it insurance companies, massive, consolidated health systems, and government regulators like CMS, controlling personal healthcare decisions from afar? Over my career, the shift has absolutely been toward the latter.

In my written testimony, I touch upon some of the other significant forces pressuring medical practices, including utilization management by consolidated insurers and pharmacy benefit managers, misguided CMS regulations, and Medicare payment cuts that are forcing independent physicians out of business.

These factors, combined with the mounting pressures from mega health systems, are resulting in cancer patients losing access to care, not getting the best treatment, and paying more for their healthcare.

I look forward to your questions.

Chairman WILLIAMS. I now recognize Mr. Newell for his 5-minute opening remarks.

STATEMENT OF MR. WILLIAM J. NEWELL

Mr. NEWELL. Chairman Williams, Ranking Member Velázquez, thank you for the opportunity to appear before this Committee to discuss the drug development ecosystem and the challenges small biotechnology companies face.

My name is Bill Newell, and I am the CEO of Sutro Biopharma, Inc. I have been at Sutro since January 2009, and working in small company biotech since 1998. I also serve on the board of the Biotechnology Innovation Organization and Chair BIO's capital formation work group.

Sutro Biopharma focuses on research and development and manufacturing for next-generation cancer medicines; primarily, antibody drug conjugates commonly referred to as ADCs. Our company is 21 years old, and we are still a pre-commercial company, meaning we have yet to secure FDA approval for a medicine we have developed.

I was employee number 19, and today we have over 300 employees and a market cap of approximately $300 million. Like many U.S.-based biotechs, Sutro has seen substantial domestic job creation with about 40 percent of our workforce in or supporting our

U.S.-based manufacturing facility. This is the world's only manufacturing facility utilizing cell-free protein synthesis technology at scale.

I am pleased to say that we recently began two pivotal phase 3 trials for our most advanced medicine. This is an ADC for patients with late-stage ovarian cancer and for patients with an ultra-rare pediatric leukemia, but readouts are not expected for a few more years. We have multiple additional potential cancer medicines and research in early clinical development.

In many ways, Sutro's corporate journey is a microcosm of the small biotech financing experience. We have raised approximately $190 million in venture capital. We went public in 2018, benefiting from the JOBS Act of 2012. So far, we have raised approximately $535 million in public market offerings.

In addition, collaborations with larger biopharma companies have been essential to advancing our research and development. We have received approximately $720 million in funding and reimbursements for R&D collaborations and our licensing product candidates. This funding comes primarily from large and mid-sized biopharma companies.

In addition, at various points in time we have borrowed from venture lenders. I am proud to say that we are debt-free as of earlier this year.

All told, Sutro has raised almost $1.6 billion in the company's history, and we are still several years away from our first approved medicine. That eyebrow-raising figure is, unfortunately, very typical of the small biotech experience in bringing a product to market.

Also, like many biotechs, we have had our share of failures along the way. This is not unusual for our industry. Only approximately 12 percent of products reaching clinical development stage are ever approved, and just half of products reaching phase 3, pivotal trial stage, ever get FDA approval.

In my remaining time, I want to emphasize the need to reauthorize the pediatric priority review voucher program. The current program expires September 30 of this year, and Sutro and our industry partners strongly support the Creating Hope Reauthorization Act of 2024. This bipartisan bill provides critical incentives to promote research and development for drugs to treat rare diseases impacting children across the country.

As I mentioned, Sutro is pursuing a phase 3 trial for an ultra-rare pediatric leukemia which affects approximately 50 infants and toddlers a year. The sad reality is that the economics of pursuing our medicine in this indication would clearly dictate that we should not do so. The key economic rationale for us to pursue this ultra-rare indication is the value to Sutro of a pediatric priority review voucher. It is cases like this one that make it imperative that this program be reauthorized.

Lastly, I want to thank the Members of this Committee who either co-sponsored or voted for the American Innovation and R&D Competitiveness Act of 2023, H.R. 2673, which was overwhelmingly approved on a bipartisan basis by the House earlier this year.

However, because H.R. 2673 has not yet been enacted into law, we paid $15 million in federal taxes this year as a result of the

switch to a 5-year amortization schedule, even though we have never sold a single vial of medicine. This tax liability means two phase 1 clinical trials for our cancer medicines no longer have funding.

I have included additional policies in my written testimony that highlight other actions Congress can take to support small biotech companies. As the innovation driver of the biopharmaceutical ecosystem and industry that keeps America at the cutting-edge, it is important to create a policy landscape that supports companies like Sutro.

I am honored to be here discussing both the challenges associated with running a small biotech company and the true honor it is to dedicate each and every day to the service of patients and their loved ones.

Thank you for allowing me to testify today. I look forward to answering your questions.

Chairman WILLIAMS. Thank you very much.

And I now recognize Dr. Zuckerman for her 5-minute opening remarks.

STATEMENT OF DR. DIANA ZUCKERMAN

Ms. ZUCKERMAN. Thank you.

Chairman Williams, Ranking Member Velázquez, and distinguished Members of the Committee, I am Dr. Diana Zuckerman, president of the National Center for Health Research, a public health think tank here in Washington.

I appreciate the opportunity to be here today to share the views of the Center, which does not accept funding from any companies with a financial interest in our work. Our goal is to use research information to inform and improve policies and programs for the healthcare of everyone in this country.

As a scientist and a policy expert and a cancer survivor, I look for common ground. I respect the important work of all the other panel members and this important Committee. And I think we can all agree that we want small businesses to succeed and to provide the best possible products.

In medicine, let's agree that we want innovation that is defined as better products and better treatments that have meaningful benefits for patients: Living longer, spending less time in the hospital, and having a better quality of life.

The FDA makes it very clear that it does not expect or require absolute certainty when it approves a drug or medical device, and I will give two examples of their flexibility.

A few weeks ago, a company named Amylyx reported that its one and only drug, Relyvrio, did not work. FDA had granted approval to the drug to treat ALS, also known as Lou Gehrig's disease, even though the company ignored FDA's advice to complete their clinical trial before they asked for approval.

FDA scientists and advisory committee members warned that the evidence was weak and that the drug might not work at all, but FDA approved it anyway because ALS is a terrible disease, and the agency wanted to be flexible, and patients hoped that the product would work.

The drug cost $158,000 per patient per year, even though it was a combination of two much less expensive ingredients, one of which is a dietary supplement sold on Amazon for a few dollars.

The company promised to continue studying the drug, comparing it to placebo, and eventually they reported that patients taking the drug were no better off than patients on placebo. Meanwhile, last year, the company had $380 million in revenues, and the two young men who co-founded the company paid themselves $7.4 million each just last year.

When Relyvrio was taken off the market, the stock dropped 80 percent, and 70 percent of the staff were left—let go.

Another example is Sarepta, a company that submitted its only product for FDA approval based on data for only 12 patients and no placebo group. I have never seen any study like that get approval. But despite that lack of evidence, Exondys 51 for Duchenne muscular dystrophy was approved. And they granted accelerated approval in 2016 because, as a small business, Sarepta did not have the capital to continue to do their clinical trials unless they were able to sell their product and make some money.

The company promised a larger study would be completed in 2020, but here we are, 2024, and it still hasn't happened, and we are still waiting for any credible evidence from the company to show that it works after 8 years.

The price of the drug has increased from about $400,000 per patient per year to over a million dollars per year per patient, and families have gone broke even paying the co pays, and Medicaid has, as a result of that, footed most of the bill.

So these examples are two that show that FDA is sometimes very helpful to small businesses, sometimes ignoring the agency's own requirements to give companies a chance to succeed. And there is research showing that many large companies also benefit from this flexibility. And in my written testimony, I have provided examples of the research evidence and the lower user fees that FDA charges for small businesses and how Congress has passed user fee legislation that helps to level the playing field for small companies.

The bottom line is that we all want medical products to be safe and effective. Small businesses will have the resources to meet the FDA's evidence standards for many types of medical products, but not all.

What's the alternative? If the FDA reduces the burden on companies by not requiring them to provide clear evidence that a new product is safe and effective, that increases the burden on patients, families, and physicians, because we must make life-saving and life-changing decisions without the facts that we need to make the decisions that are best for us and our patients.

Thank you very much.

Chairman WILLIAMS. We will now move to the Member questions. Under the 5-minute rule, I recognize myself for 5 minutes.

Healthcare is something that is extremely personal to every American. We need to make sure that patients can get the care they need by the providers they want at a price they can afford. Unfortunately, I have heard stories, as we have heard today, from people that are not able to abide by these three principles for many

different reasons, one of which is a complicated, expensive process to get new drugs on the market.

I understand these challenges firsthand, after my family searched for the best treatment options for my wife, Patty, after she was diagnosed with glioblastoma in December of 2022. As we learned about the various possibilities and tried to select the best path forward, we were shocked to hear that certain options were only available abroad because they had not been approved in the U.S. yet.

I understand the need to ensure we have safe medicines and treatments coming to the market, but it made me think what hurdles might be in place that are slowing this down and what the effect on patient outcomes may be.

So, Dr. Miller, you worked at the FDA and are currently practicing medicine at Johns Hopkins University and probably have some great insights on the variety of challenges that cause cases like my wife's to be a reality for so many Americans.

So my question is, can you elaborate on some reasonable changes that can be made to streamline the approval process so we can bring more treatments to the marketplace without sacrificing patient safety?

Dr. MILLER. Thank you. And I am very sorry to hear about your wife.

Chairman WILLIAMS. She is doing good.

Dr. MILLER. That is good to hear.

I think the clinical trial reform is the thing that PDUFA has promised for 30 years and, frankly, hasn't delivered on. Getting the reviewer to actually guide small companies and entrepreneurs who are scrappy and want to do things differently is the first step that I would do. And doing that would mean collapsing layers of management and making sure that the primary task of the FDA is focusing on review of products to make sure that they are safe and effective, because the FDA—CDER, for example, has 5,800 employees. Most of them are not focused primarily on that task.

Chairman WILLIAMS. Now, with the passage of the Democrat-led Inflation Reduction Act, the University of Chicago estimates that its provisions will result in 135 fewer drugs being brought to the market. Along with these provisions, President Biden called on to expand these price controls to include 500 drugs, as opposed to the IRA's 50.

The American Dream is built on risk and reward. If there is not going to be a profit incentive to bring new drugs to the marketplace, fewer businesses will dedicate the necessary research and development dollars to make them a reality.

So, Mr. Newell, as a small pharmaceutical company, how do the IRA's price controls impact your business and deter innovation?

Mr. NEWELL. Thank you, Chairman Williams.

As you would have heard from my testimony, we have raised a substantial amount of capital through the years to bring our medicines forward and to build our business. We work in the biologics field, and I have other colleagues who work on small molecule medicines. In particular, those are things that are pills that are given to—for patients.

Particularly in the IRA, we see that there are substantial disincentives for investors to invest in companies that make small molecule medicines because of the different terms allowed for the introduction of the price negotiations that are mandated by the IRA. For biologics companies like Sutro, we have a 13-year window, but a small molecule manufacturer innovator has a 9-year window. That is a disincentive to investors.

Given the tremendous amount of money that it takes to bring a drug forward to prove its safety and efficacy, we should be not disincentivizing people who make small molecule medicines for patients.

Chairman WILLIAMS. All right. Thank you.

And, Dr. Eagle, in your testimony, you emphasize that healthcare consolidation is on the rise and small practices like yours are struggling to keep up.

So in the brief time that we have left, can you please describe to the Committee how consolidation harms patient care, and beyond just patient care, what are the downsides of the consolidation in the healthcare system?

Dr. EAGLE. I think a large consolidated system is, first, they are more expensive. You know, they tend to be hospital systems that charge facility fees. You know, they can provide excellent care, but I think it oftentimes is less convenient for patients.

I think small physician offices, you know, get to know the community better, you know, have better processes, and provide lower cost care for patients and more affordable and more convenient format.

Chairman WILLIAMS. Thank you. I yield back.

And I now recognize the Ranking Member for 5 minutes of questions.

Ms. VELÁZQUEZ. Thank you, Mr. Chairman.

Dr. Zuckerman, the FDA has strict standards for approving new drugs and devices to prove their effectiveness. Is there any evidence that you are aware of that FDA's high standards have driven innovation and investment to other countries?

Ms. ZUCKERMAN. Thank you for that question. I haven't seen that evidence. You have to remember that the standards are very similar in other countries, and there aren't very many drugs or devices that are available in other countries but not here. And we pay a lot more for them. So there is a lot of incentive to—to get FDA approval.

It used to be that FDA was a lot slower than other countries. That is absolutely true. But it is not true anymore, and it is partly because Congress passed the user fee legislation, which really has sped up the approval to the point that it is very, very similar in other countries.

Ms. VELÁZQUEZ. Thank you.

Two years ago, Congress passed the Inflation Reduction Act, which allowed Medicare to cap the price of insulin and negotiate prices for certain drugs. Has this had a measurable effect on research and development spending by pharmaceutical companies?

Ms. ZUCKERMAN. R&D is very important, and it is related to how much money the companies have. But R&D is really only a small percentage compared to stock prices and things like that. So

there really isn't any evidence that, although this could have an effect on which drugs companies invest in but not—it shouldn't have a chilling effect on innovative drugs.

Ms. VELAZQUEZ. Thank you.

Dr. Eagle, you mentioned that utilization management presented barriers to care for doctors and their patients. Do you think the increased use of these practices is a function of the market power of insurance companies?

Dr. EAGLE. Thank you for the question, Congresswoman Velázquez.

Absolutely. You know, we have seen tremendous consolidation among the pharmacy benefit managers, among the insurers. One of the tools they use is utilization management that comes in different forms. One is prior authorization, where, you know, the—after the physician and the patient have decided what they would like to do, we seek approval from the insurance company. Often it is declined, and the next step is a step called clinical peer review, where we have to get on the phone with the insurance company physician.

You know, in cancer medicine, we do very careful decision-making. You know, we look at the literature, we present with colleagues, we discuss cases at tumor boards. It is very frustrating when you then have to turn around and speak with an insurance company to a physician who may not be an oncologist. Fortunately, New York recently passed a law that requires people at least to be in the same specialty as the case they are reviewing. That is a fairly low standard.

My first question is always: Have you read the notes? And then we generally start the conversation from there. It is very common for, you know, PET scans, therapies to be denied.

Another part of utilization management is fail-first therapy, where the insurance company will demand that we use particular therapies before others. I just had a patient yesterday who started treatment for breast cancer where the nausea medicine that I wanted to use was not approved by the insurance company. They required that we substitute an alternative nausea medication. That can be a problem.

Ms. VELAZQUEZ. Thank you.

Dr. Zuckerman, we hear that the FDA often requires absolute certainty when making decisions about approving new products. Can you discuss the standard required by the FDA and the impacts on innovation?

Ms. ZUCKERMAN. Sure. Thank you for that question.

I think the examples I gave show that it is not true that they do require or expect absolute certainty, and, in fact, in science you never expect absolute certainty. But you do want a reasonable assurance that something is likely to work for at least some patients. And that is really what FDA has been doing, as well as making exceptions when a drug is urgently needed for a very serious disease.

Ms. VELAZQUEZ. Thank you.

Mr. Newell, you recently signed a letter of standing with the FDA against judicial interference in the drug approval process. Can you expand on how a decision like this creates uncertainty for your industry?

Mr. NEWELL. As I have explained earlier—thank you for the question. As I have explained earlier, capital is extremely important to allow us to do the work that we do developing new medicines. Capital requires certainty. And if the regulatory authority of the FDA is undermined, we do not have certainty and, therefore, capital will not be available.

Ms. VELAZQUEZ. Thank you. I yield back, Mr. Chairman.

Chairman WILLIAMS. The lady yields back.

And before we go any further, I just want to—you are going to see Members going in and out. It is not because they are leaving or they are mad or anything. We have got other hearings to go back and forth to, so you will see that happen, okay?

I now recognize Representative Luetkemeyer from the great State of Missouri for 5 minutes.

Mr. LUETKEMEYER. Thank you, Mr. Chairman.

A few—not too long ago, a year or two ago, we passed a bill that says the lengthy permit process across the board is really stymieing a lot of businesses, including the approval of drugs.

Ms. Maloy—there we go.

And so we had—the bill said you are going to set it two years.

Have you seen this kick in yet at all? Have you seen this process be shortened at all as a result of this bill, Mr. Miller—Dr. Miller?

Dr. MILLER. You mean the FDA review process?

Mr. LUETKEMEYER. Yeah. Yeah.

Dr. MILLER. Not to my knowledge.

Mr. LUETKEMEYER. Because the process should have been shortened to 2 years.

And, Mr. Newell, your process is taking years and years and years.

Mr. NEWELL. The process has not been shortened.

Mr. LUETKEMEYER. Process has not been shortened. So our bill hasn't taken effect yet. We need to start talking about that, don't we?

You know, Dr. Eagle, you worked in the cancer realm, and in my district, we have the largest nuclear reactor in the country at our—on the university campus at the University of Missouri. About 95 percent of all radiated drugs that are used in cancer actually come from that—actually used around the world come from that nuclear reactor. It is actually at the end of its life span, so we are trying to build a new one. But have you used those drugs? How important is it to you to be able to continue to allow that to happen? Because they do a lot of research there as well. I don't know if you have been utilizing their services or not.

Dr. EAGLE. Thank you for the question. Those are vitally important drugs. You know, we have a new prostate cancer drug, you know, that uses a targeted antibody plus a radiation therapy to treat—to treat prostate cancer. We use other types of treatments called neuroendocrine tumors where that is a vital part of the therapy. It helps people extend lives with minimal toxicity. There will be more of those therapies in the future. It is vital that patients have access to those treatments.

Mr. LUETKEMEYER. They do lots of research there. It is phenomenal. They have these little molecules that go circulating

around the bloodstream looking for cancer cells to go to strike. It is phenomenal stuff. So thank you for that.

Dr. Eagle, you are involved in a practice. And the concern that I have here deals with this consolidation, just as you alluded to with your practice. So some people are going to this concierge care, where you pay a doctor to take care of you, whatever, for a certain price per year or whatever, and—the insurance policy for you to take care—have you seen this already, and what do you think about it? Is it something that is——

Dr. EAGLE. I have seen that primarily in primary care. I have not seen that as much in specialty medicine. In cancer medicine, we try to make every patient a concierge care patient. You know, we allow same-day walk-in visits, patients don't have to have an appointment, same-day infusions.

You know, I think the only place I have really seen that take hold myself is in the primary care sector, but not in other fields.

Mr. LUETKEMEYER. It goes back to the premise of this Committee from the standpoint of the regulatory burden. I mean, it is so difficult for a small practice to continue to do this.

Do the concierge care physicians, are they burdened by the same amount of rules and regulations? Have they found a way around this? What——

Dr. EAGLE. In that case, I think the primary change is the economics. You know, they just operate under a different economic system. You know, the private physicians that faced a decline in the fee schedule for 20 years now, and particularly when you adjust for inflation, payments to physicians is automatically brought down by 26 percent for private physicians.

So I think the concierge model of care is just an effort by certain primary care physicians to solve that problem and spend more time with their patients and not have to be their care so volume-driven.

Mr. LUETKEMEYER. Mr. Newell, you are in the business of developing drugs. I saw the other day where artificial intelligence actually developed a drug for a disease. They put in there the parameters to do something and this, you know, artificial intelligence came up with a drug.

I mean, is this the wave of the future? Am I correct in that?

Mr. NEWELL. We do use artificial intelligence or machine learning to help us identify better targets for our medicines and, perhaps in certain cases, the structure of the medicine that we are going to be making.

Mr. LUETKEMEYER. Will that make it cheaper or does that make it more expensive?

Mr. NEWELL. It is to be determined. I personally do not believe that a medicine will be fully developed using artificial intelligence. There are too many variables in the human body for us to take into consideration at this point in time.

Cancer in particular is a very heterogenous disease, and so what works in one patient may not work in another.

Mr. LUETKEMEYER. Okay.

Mr. NEWELL. And so I think at the end of the day, we need to really be cautious about AI, but it can be helpful in development.

Mr. LUETKEMEYER. Very good. One more question for Dr. Miller.

You know, the administration is working on descheduling cannabis from a schedule I down to schedule III, I believe it is. And part of the problem is the USDA—or FDA has never done the research to be able to say that this is justified. Can you comment on that?

Dr. MILLER. I think that there is still a lot of work to be done to determine what should be done with cannabis. There are several prescription drug-approved formulations of THC. The FDA doesn't have an adequate framework to regulate the other forms of cannabis to ensure that is safe and effective for the average consumer.

Mr. LUETKEMEYER. Thank you very much. My time has expired.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative McGarvey from the great State of Kentucky for 5 minutes.

Mr. MCGARVEY. Thank you, Mr. Chairman.

[Inaudible] I think about my grandmother who passed away with dementia, I think about my dad who lives with Parkinson's, I think about our dear friends Katie and Ellie battling breast cancer.

And so many of us have friends and family who are living and fighting terrible diseases. And when you are in that spot, you want the same thing. You want them to get better. You want hope.

And so when I think about this hearing today, I want to make sure that we are framing this in the right way, because not only do you want hope, but you want that hope that they are going to get better.

And I want to tell a little story about a woman named Frances Oldham Kelsey. You worked at the FDA; you might have heard of her before. This was back in the fifties and sixties. Of course, something again that many people faced, morning sickness.

There was a cancer drug out there, thalidomide, that was being used in other countries that was helping women with morning sickness. Forty-six other countries approved it. There was all sorts of pressure to approve it here in the United States. One woman at the FDA said no. Imagine the pressure on that one person saying no to get rid of morning sickness.

And then it comes out that thalidomide caused incredible birth defects. Tens of thousands of babies around the world were born without limbs, were born with brain defects. The drug was taken off. It was never used. And we did not have—the term was—it was so common, they called them thalidomide babies. It wasn't—it didn't happen here because we had one person who was willing to say, is it safe?

So while we want all of our friends, neighbors, and loved ones to get better, we want them to have that hope, we have to have it be safe. And I will admit, there are obviously regulations that are burdensome and can get in the way. But regulation itself is not a four-letter word.

Regulations have been used to save the lives of everything from the minors in my State of Kentucky to save the lives of people who actually need these treatments and when they come to market.

And so when we are talking about this today, I want to talk about some of the other things that are actually keeping these drugs from getting them in the hands of the people who want and

need them, because that is what we all want. We want people to get and be healthy, and we want it to happen safely.

So, Dr. Zuckerman, I want to ask you a question. What is a bigger barrier to getting new drugs and cheaper drugs to market? Is it FDA regulations or is it Big Pharma practices like pay for delay, patent manipulation, and side deals with generic manufacturers that are keeping most people from getting the drugs they want and need?

Ms. ZUCKERMAN. Thank you for that question. And I just want to say, I met Dr. Kelsey, and she was just a wonderful woman. She lived nearby in Bethesda.

There are a lot of—some products are very complicated, and they do take a long time to develop and they take a long time to study. But patent manipulation is a huge problem, because you have—you have products like Humira, which have a couple of hundred patents—a couple of hundred patents, and they keep adding patents to keep it on the market and to prevent any kind of competition. So it costs enormous amounts of money to patients, and it makes drugs very, very expensive that otherwise would have generic alternatives that would be much less expensive. So I think that is a great example.

Mr. MCGARVEY. Thank you very much. I appreciate that.

And, again, this is something—we want to encourage innovation, we want to continue developing life-saving drugs to get people better. And so, you know, hopefully, that is what we can continue to happen and look at all the reasons why it might not be happening.

Mr. Chairman, I yield back.

Chairman WILLIAMS. The gentleman yields back.

And I now recognize Representative Stauber from the great State of Minnesota for 5 minutes.

Mr. STAUBER. Thank you, Mr. Chair. Thanks to all the witnesses here.

Mr. Miller, in your comments, you said there is too many administrators in the FDA. You have been part of the FDA. How do we—from our standpoint, how do we legislatively reduce the bureaucracy? Like, give me—like, is it a congressional action, or is it you, as experts, placing your own to remove the burdensome redundant administrators?

Dr. MILLER. I would say it is oversight and then also the user fee acts.

Mr. STAUBER. And what?

Mr. MILLER. The user fee act. So when PDUFA comes up for reauthorization, that is a great opportunity to write language to encourage the FDA to have reviewers as opposed to managers.

Mr. STAUBER. Mr. Eagle, you had—you talked about your experience as a small provider, you and two other doctors, and then you were almost forced to be bought up by the large—a larger company. In one of your statements you said, who is in charge of patient care? That is a huge problem, we know, in Medicare.

I think the vast majority of Americans would say that the patient and doctor are in charge. And when the patient and the doctor agree that a 5-night stay in the hospital is needed, rather than Medicare saying, no, you should only spent two nights there, that is—there is a huge problem going on in our country where our phy-

sicians are undercut by, in this instance, Medicare, telling you that your patient didn't need 5 days and we are only paying for two. And we know you are going to make the best decision for your patient, not a bureaucrat at a three-letter agency checking off the boxes.

That—I am hearing that universally around the country. We must fix that. The patient and the doctor must make those decisions. Thanks for bringing that up. And that is a big lift.

Mr. Newell, you have—you have an interesting story on the research and development of the product. I can tell you right now, I know a physician within this country that is in the final stages of his new drug, and I know he used it on a COVID patient the last—that was the last stretch, this is it, and it saved the patient's life.

I can't imagine—and he has talked to me about the FDA and all the process and the bureaucracy. I can't imagine how many COVID patients it would have saved had it been allowed—FDA allowed it earlier. We won't know. But in talking to this physician and others, it is very concerning.

We talk about safety. I mean, we want it to be safe. But I think we have to meet a sweet spot here where the research and development, there is timing on this, there is certainty. We have to have, not administrators at the FDA, but people that will look at these drugs and use their professionalism and experience and yea or nay.

One of the things that frustrates me is, you know, as a small business owner—you as a small business owner, just the rules and regulations that they force upon you. It is unnecessary. I want you and the nurses to have patient care, not pushing paperwork. Thirty-five percent, probably, of your time is pushing paperwork. Would you agree, Doctor?

Dr. EAGLE. Absolutely. And to give you an example of some of the things that we—that affects how patients are cared for, there is a rule in Medicare that they consider it unreasonable for us to see a patient and give them an injection or an infusion and then see them for that same related problem the same day. The rule is—it is termed modifier 25 because that is an exception that insurers and CMS use to except.

So, for instance, I might have a patient who is getting a shot for anemia and they need to also have the anemia managed. They need to follow the doses, they need to make sure it is safe. I can no longer see that patient on the same day they get their shot. So the patients—you know, oftentimes their children have to take off work, their caregivers have to take off extra time from work to bring them for the visit with me and then bring them on a separate day to receive the injection, when both could easily be done and it would be much better for them both to be done at the same day.

That was not a big issue 3 to 4 years ago. But over the last 2 years, we have had to reeducate our entire staff, reorganize our entire office around that rule, and it is absolutely no benefit to the patient.

Mr. STAUBER. My time is up. I want to thank you all for your expert testimony, and thanks for what you are doing to your patients. You are making their lives better.

And I yield back.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative Scholten from the great State of Michigan for 5 minutes.

Ms. SCHOLTEN. Thank you so much, Mr. Chair.

Thank you to our witnesses today.

This is such an incredibly important hearing. And I think, if we have learned anything from the questions today and the testimony, it is that this is about balance. We need federal regulations to keep individuals safe. But I can tell you firsthand from a place like west Michigan where I come from, there are overly burdensome surprise regulations that are hampering innovation. And I am grateful that we are having the hearing today to talk about it. Let's not throw the baby out with the bath water. But let's make sure that we have effective regulations that are doing what they need to do but are not getting in the way of critical lifesaving drugs and medical devices.

Healthcare is growing the economy and saving lives every single day in west Michigan, from the production of the COVID vaccine to innovative cancer research to medical devices that are being created right in my home, my backyard.

From 2018 to 2021, our State saw a 41.8-percent increase in the establishment of research, testing, and medical laboratories. That is about 6 points higher than the national average. Go blue. I am committed to ensuring that that we ease the burden for our small businesses in this critical market while also keeping consumers safe.

This question is for Dr. Zuckerman. As you have discussed in your testimony, the FDA has made concerted efforts to assist small businesses navigating the regulatory process. Can you speak to the FDA's ability to ensure new establishments are aware of the services that they offer, such as the Center for Drug Evaluation and Research and SBIA programs. And are there ways that this outreach can be improved?

Ms. ZUCKERMAN. I am sure there are ways to improve it. I do think that most companies would be aware that, because of the User Fee Act, the FDA is under an obligation to have their scientists meet regularly with companies when the companies request it. The companies do have to request it. But that really helps level the playing field because, otherwise, you know, companies that don't have access to expensive consultants would not have that kind of advice that they need of, what do I need to do to get this across the finish line so to speak?

But I also just want to mention that device regulations are much, much different than drugs.

Ms. SCHOLTEN. Of course.

Ms. ZUCKERMAN. And something like 98 percent of devices get on the market without even having clinical trials. And you'd be surprised what that includes. And I will just say a loved one is trying to decide how to get treatment for a brain tumor. And there are several products on the market that provide radiation that have never been tested in clinical trials except after they went on the market, and those clinical trials are tiny and not very well conducted because they weren't required by the FDA. So that is the balance.

Ms. SCHOLTEN. Yeah, absolutely.

The regulatory process can be lengthy and expensive for small businesses, we recognize this, who are willing to bring their product to market. This question is for you, Dr. Miller, in your testimony, you discussed different ways to improve clinical trial outcomes with greater efficiency. You mentioned the FDA's draft guidance regarding decentralized clinical trials. Are there important mechanisms or other tools that can be strengthened within the FDA to assist in the adoption of these practices to support innovative small businesses in communities like mine, keeping in mind some of the comments that Dr. Zuckerman just made as well.

Dr. MILLER. I think it is operationalizing those guidances, that has always been the trouble with the FDA. When you are a reviewer, you have NDAs. You have INDs. You have all these meetings and everything comes to you. And then you have six layers of bureaucracy above you telling you to work harder. And the six layers of bureaucracy also can do your job because they had the exact same training. So I think it is deburdening the reviewers and making more of them by transforming that bureaucracy into more frontline staff who can interface with companies to help them operationalize those guidances and principles in their development programs. So the tools are there. It is primarily a human capital management challenge.

Ms. SCHOLTEN. Thank you. I yield back.

Chairman WILLIAMS. The lady yields back.

I now recognize Representative Meuser from the great State of Pennsylvania for 5 minutes.

Mr. MEUSER. Thank you, Mr. Chairman.

Thank you very much to our witnesses. We very much appreciate you being here. A very interesting subject, very important subject. I am very glad we are discussing it and has definitely been informative already.

Dr. Eagle, I would like to ask you, the H.R. 2474, the Strengthening Medicare for Patients and Providers Act. I hear all the time from physicians that the reimbursements levels are a challenge, prior authorization requirements and everything else that takes place. Do you think this act will improve things and make it a little bit more consistent as far as your reimbursement levels?

Dr. EAGLE. Thank you for the question, Congressman. I believe that is the act that provides the inflation adjustment, so for physician fee scheduling, and so we fully support that.

Mr. MEUSER. Okay.

Dr. EAGLE. If you look at private physician reimbursements, it has really been flat or declining over the past 20 years and while inflation has increased by 26 percent. So there has been an effective decline for physician payment over the last 20 years. And that explains much of why private physicians are going away. The hospitals and nursing homes have those inflation adjustments. And I think we would fully support that legislation to level the playing field and give the physicians the resources that they need.

Mr. MEUSER. Great.

I come from the home medical equipment industry, formally DME, powered chairs and mobility equipment and such as that. I honestly have never been a fan of CMS or the FDA, more so as I

have gotten into Congress I guess. It seems that even prior authorizations are intended for slowing down payments, controlling costs rather than actually just dealing with it and talking. And I don't mean any particular administration. I mean all the way back to W. Bush and even H. Bush and on through. I just didn't feel that there was the right level of understanding of what the results were, of what their work was, as well as their understanding of what was the most cost-effective ways of handling things. And they would tend to beat up on the industries that pushed back the least, if you will.

So do you feel—Dr. Miller, this is actually for you, do you feel that CMS is listening these days to pharmaceutical concerns and physicians' concerns?

Dr. MILLER. No. And I think that is a longstanding historical problem. One of the things that I think has happened, though, is we have had increasing centralization of payment and decision-making. The IRA created centralized administrative pricing for a small group of drugs. There is a push to expand that over time. When we look at physician services, 8,000 physician services are written and priced by CMS on an annual basis. And that over the past 60 years has destroyed clinical innovation and service delivery. In fact, the industry has had flat or negative labor productivity growth, and so I am really concerned that the IRA is going to drive that for drugs.

Mr. MEUSER. I can't help but—why don't we have people like you in CMS, you know? I am serious. Or even you, Dr. Eagle?

I would like to ask Dr. Newell, Mr. Newell, on your R&D tax credit situation where we are phasing—not us, the R&D tax credit of course is being phased out this year. It is about 80 percent less of the deduction than it was last year, very significant. How does that—and we passed a bill by the way to prohibit, to not allow it to sunset. So it is languishing in Senate right now. So hopefully that gets resolved. But this of course was part of the Tax Cuts and Jobs Act of 2016. Can you tell us how that is affecting your business?

Mr. NEWELL. As I explained in my testimony, thank you, Congressman, for your question. It is very expensive to develop new medicines, particularly cancer medicines. And I take every dollar that we are able to receive from investors or from our partners and reinvest them in the people, the technology and the research and development effort that we do. And here we are 21 years, and we are still not yet at a first approval of a medicine. So that tells you how much investment we have been putting into this effort since 2003.

This year, for the first time, because of a collaboration arrangement or arrangements that we made with other companies, we paid $15 million in taxes to the federal government for the first time ever. That was a remarkable amount of money that did not go to cancer research and development. That would have been the equivalent of two trials, two phase 1 clinical trials to study new medicines in patients. And that is really remarkable to me that, after 80 years, where there was certainty about R&D deductibility now it has gone away, as you have indicated. So it is a problem not only for me but for a lot of other companies as well.

Mr. MEUSER. I yield back, Mr. Chairman.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative Glusenkamp from the great State of Washington for 5 minutes.

Ms. GLUESENKAMP PEREZ. Thank you.

Dr. Eagle, I wanted to focus on some of the structural factors that you mentioned in your testimony that impact cost in patient care. You note that vertical and horizontal consolidation among insurers and PBMs in particular is a particular challenge to providing quality care for your patients. I represent a rural district in southwest Washington State, and I hear from my independent pharmacists all the time that this is literally causing them to close their doors. Can you explain more about how it is also negatively impacting independent medical practitioners?

Dr. EAGLE. Absolutely. Thank you for your question. So, as you mentioned, there has been tremendous vertical integration, particularly among the PBMs. The PBMs are typically part of the insurance company, and they also have their own specialty pharmacies and independent pharmacies. So they have a motivation to use those pharmacies. So they use different tools like restrictive networks and contracting terms that are less favorable to either independent pharmacies or pharmacies like ours that provide medications for patients. So we provide oral cancer medications for patients, but the way we do it is through medically integrated dispensing. So our pharmacy team is located in or clinics. We are connected to the EMR. We know what is happening to the patients. We never supply 90 days of drugs because we know that those expensive medications need to be held or the doses changed. But the PDMs frankly try to do everything they can to get those prescriptions routed to their own specialty pharmacies.

Ms. GLUESENKAMP PEREZ. So how has that impacted, like, an independent physician? Is this kind of going to cause them to close or, I mean, have to join a bigger practice?

Dr. EAGLE. When I was part of a smaller practice in North Carolina, we simply couldn't have a pharmacy, it was just too complicated to do that. As part of my practice in New York, we have six free-standing pharmacy sites that provide tremendous value to patients.

Ms. GLUESENKAMP PEREZ. So what do you advise Congress to do about this?

Dr. EAGLE. Well, I think absolutely with the PBMs there has to be transparency. I think that is the first step. That it is going to take far more than transparency. I think that the fact that they can refer to their own—give referrals to their own in-house pharmacies needs to be looked at. We are also seeing problems now with below water drug reimbursement. So pharmacies are now reimbursing less than the amount of the drug, and nobody can continue to operate that way. I—there are rules already that preclude that, but we need CMS to enforce those rules.

Ms. GLUESENKAMP PEREZ. Okay. Hospital systems too have grown significantly consolidated, as you mentioned in testimony. Can you explain the impact that that level of consolidation has on patients and healthcare costs for the country as a whole? Often we hear that the hospital consolidation is justified because it is more

cost-effective or efficient. But do patients end up bearing the cost of that?

Dr. EAGLE. No. Thank you for that question also. They absolutely do. Hospitals charge more through the facility fees so they have a different set of charges. They cost more than Medicare because they are paid under a different fee schedule than private offices. They are able to negotiate much higher commercial contract rates with private insurers, and so patient copayments can be very high. And just the entire insurance plan costs can be higher because those costs have to be borne by the plans as well also.

Ms. GLUESENKAMP PEREZ. How do we work to help encourage more competition in the system and help independent practices thrive again?

Dr. EAGLE. Yeah. I thank you for that question also. I think payment parity is a nice little—is an excellent place to start. I mean, there is no reason that we should be paying one system more for providing the exact same service as another. I think 340b is another driver as well also. Some hospitals do need 340b, but a lot of hospitals are making tremendous profits off of that program that don't necessarily provide the energy and care that that program, you know, would imply. That is another factor that is driving consolidation in healthcare. In fact, that was one of the motivators, you know, for hospitals to want to acquire oncology practices because the profits from that program are very desirable.

Ms. GLUESENKAMP PEREZ. Thank you very much.

We know that we just saw the last Medicare trustee report a few days ago, which warns that Medicare will face shortfalls in 12 years that will require higher premiums and limits on services or both.

Dr. Zuckerman, how can innovation reduce such shortfalls, or other policy changes we can help ensure Medicare coverage is sustainable for those who count on it?

Ms. ZUCKERMAN. Thank you for that question.

In the ideal world, of course innovation would save money in terms of healthcare, but that is not what has been happening. So many—well, we did a study of cancer drugs and found that many cancer drugs that had been approved actually were never proven to save people's lives, to help people live longer, or to improve the quality of life. So, in fact, the opposite seems to be true that, when Medicare is a little bit more careful or when the FDA is a little bit more careful about what products are approved, that saves money, and that can save a lot of money.

Ms. GLUESENKAMP PEREZ. Thank you very much for your response.

Chairman WILLIAMS. The gentlelady yields back.

I now recognize Representative Van Duyne from the great State of Texas for 5 minutes.

Ms. VAN DUYNE. Thank you very much, Mr. Chairman.

And, Dr. Miller, it is great to see you again.

Last July, the Oversight Subcommittee, which I Chair, held a hearing on the overregulation in healthcare and the impact that it has on small businesses. And I am glad to see that we are holding this hearing with the full committee today, given the level of bipar-

tisan concern regarding how this overregulation is hurting our healthcare system.

In Texas' 24th District, I have hosted a number of roundtables with doctors. And I have always made it a point to ask them how much of their time is spent in front of a computer on a screen as opposed to talking to their patients, as opposed to actually doing the job that they signed up for, that they studied for. And their answers are always astonishing to me; anywhere between 65 and 90 percent of their time is spent checking boxes that CMS has put in front of them. It is not helping their patients. It is not decreasing costs. It is not increasing quality. It is spent on regulatory issues. And it is just shocking to hear that. And this is time that they would obviously rather prefer to spend with their patients, and guarantee you it is time that patients would rather, you know, spend talking to their doctors.

And I will never forget a meeting with a doctor who was forced to sell her own practice because she said she just couldn't afford to do it anymore. And she said now that she is basically not practicing medicine, but she is just filling in boxes, that she just feels like a monkey could do her job because she is only following regulations, not practicing medicine.

And, where regulatory costs reached the point that it is no longer feasible for small private healthcare practices to keep the doors open, it only leads to one thing, which is consolidation. And this is decreasing the quality of care. It eliminates competition, which increases costs. And it limits the possibility of physicians owning their own businesses. And that restricts access to care, and it hurts patients. We can't continue to allow overregulation to shut the doors of small care providers. And I am glad our committee is focused on finding solutions to better provide better and more affordable quality care, patient care.

But, not only are this administration's policies hurting solo and small physician practices, but they are also hampering our ability to be able to provide much-needed new pharmaceuticals and drugs into the market that are produced by our highly regulated U.S. manufacturers. And, at the same time, though, these policies seem to be actually—policies like the Inflation Reduction Act, which Dr. Miller, you were speaking of earlier, but they are actually enriching and empowering some of our most adverse foreign agents.

So, Dr. Miller, I am going to ask you, can you tell us how China is using the Inflation Reduction Act against us when it comes to new drugs?

Dr. MILLER. Thank you for the question.

China views the biopharmaceutical industry as a place where it wants to completely dominate. The IRA sent a massive negative signal to all entrepreneurs, small companies and large companies basically saying that you are going to undergo centralized administrative price regulation. That, plus the inability for the FDA to modernize clinical trials means that the cost of developing drugs and doing trials in the U.S. and manufacturing is very high.

I know people who are building companies, developing new therapies, and they are looking at lower cost sites. And they always ask me about China. They want to manufacture products in China. They want to do clinical trials in China. So that scientific knowl-

edge, that technical knowledge that manufacturing knowledge, the clinical knowledge, in addition to all of the jobs which are highly paid and created a great innovative economic and scientific ecosystem, those ecosystems are leaving for China. And we just passed a law that sent—and told the pharmaceutical industry to do more development in China where it is lower cost. So we essentially functionally supported our greatest adversary.

Ms. VAN DUYNE. Wow. Look, it has been almost a year or two since you testified in this committee about overregulation in healthcare. Do you think things have gotten better, or do you think things have gotten worse?

Dr. MILLER. Worse. I would say that I spend most of my time typing. And I remember one of my professors in medical school said I would be an excellent physician because I was a very fast typist.

Ms. VAN DUYNE. Dr. Eagle, I am going to ask you the same question.

Dr. EAGLE. Things have clearly gotten worse. And thank you for the question. Your point about EMR could not be more on target. That is kind of where the rubber meets the road between a lot of the regulatory requirements and how physicians spend their time, and it is a huge burden for physicians to kind of spend all day checking boxes in the electronic medical record.

A lot of EMRs are really—involve the billing systems, so, you know, the priorities of the EMRs are check-the-box documentation requirements and billing and not true medical decisionmaking that actually benefits patients, you know. I will get 5-page documents from other physicians with a list of diagnosis codes, a list of medications, a list of previous diagnosis, but very little about what they are actually thinking about what the patient needs and what the plan of care should be. And that is a lot of how a lot of these regulatory issues get transferred to physicians is through the EMR. It is a major source of burnout for physicians, and I thank you for making those points.

Ms. VAN DUYNE. Thank you very much.

I yield back.

Chairman WILLIAMS. The lady yields back.

I now recognize Representative Alford from the great State of Missouri for 5 minutes.

Mr. ALFORD. Thank you, Mr. Chair and Ranking Member, for holding this. And thank you of course to our witnesses today.

I represent 772,047 people in a very rural district of Missouri. The Fourth Congressional District. And rural healthcare is kind of at a crisis point I think in America. Facing the continuing healthcare crisis, providers are being forced to shut down or withhold the vital services that they provided to communities they serve.

Rural Americans are uniquely vulnerable to the downstream effects of regulatory burden being forced upon the healthcare industry. That is why I am glad we are having this hearing today, Mr. Chairman.

We have visited numerous hospitals in our district. We have heard firsthand the impact regulations have had on small businesses in our district. When rural providers are forced to shut down, it can leave their patients stranded hours away from the

healthcare that they need and that they deserve. While telehealth is helping meet some of those needs, only 22 percent of our constituents in our district have high-speed internet that is capable of taking part in this technology.

So, Dr. Eagle, I want to start with you. You talked about how difficult it was to maintain your independent practice, that you eventually was forced to join a large hospital system. It is something I hear from my constituents well. They are worried they won't be able to continue their small practices that serve rural Americans. Why is it so difficult to maintain a small practice, especially in rural America?

Dr. EAGLE. I think the two primary drivers—thank you for the question—are economics and regulations, you know. I think that the practice I had in North Carolina was more suburban than rural, but I think the problems are largely similar. I think rural practices suffer from all the same problems that the other practices do, but regulation is harder to manage in those situations. And reimbursement has been declining for the private offices, the same as for everybody else.

I can't speak as an expert on rural medicine in that regard because I never have practiced in a true rural setting. But I think the problems are the same; it is just harder to solve.

Mr. ALFORD. Thank you.

Dr. Miller, another concern I have heard about from my constituents is about CMS' efforts on this great idea of decarbonization. We have got to decarbonize healthcare at a time when rural hospitals are struggling to keep their doors open. CMS—I was being flippant there, if you don't know that—is releasing guidance asking hospitals and healthcare providers to monitor and address their emissions. This is a slap in the face to rural hospitals in my district. In fact, the Golden Valley Memorial Hospital in Clinton, Henry County, told me that to replace a single boiler with a clean alternate that the CMS wants is going to be $3 million bucks, $3 million. Do you think that CMS is fulfilling their statutory duties in attempting to browbeat hospitals into decarbonizing?

Dr. MILLER. Absolutely not. That is completely out of scope for CMS.

Mr. ALFORD. It is kind of crazy, isn't?

Dr. MILLER. Yeah, there is a long history of using the conditions of participation in Medicare to attach a favored regulation. And CMS' primary goal is—should be to assist in running the State Medicaid programs and to run Medicare.

Mr. ALFORD. Should be.

Dr. MILLER. It is not an environmental regulator and should not be.

Mr. ALFORD. Thank you.

Dr. Eagle, back to you. I have often heard from small rural hospitals that are really struggling this year. I visited the Bates County Memorial Hospital, a great crew there, a small facility in Butler, Missouri. And they told me that, in 2022, they lost nearly $2 million providing for Medicare patients. I know we have some bills out there that is going to help with their reimbursements. But what do we do in the meantime? How can these small hospitals and doctors

who want to serve—truly it is almost a ministry to them out in the rural communities—how do they stay alive?

Dr. EAGLE. I know. Thank you for that question. I cannot speak as an expert on hospital administration. But I think that programs like 340b are designed to support, you know, programs, hospitals like those. And I think that, you know, redesigning that program to actually truly meet the hospitals that need the funds would be a step in the right direction.

Mr. ALFORD. Well, thank you. I truly think this is a very important hearing that we are having today. And I thank you for being here.

And, with that, Mr. Chair, I yield back.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative Thanedar from the great State of Michigan for 5 minutes.

Mr. THANEDAR. Thank you, Chairman Williams and Ranking Member, for hosting this important discussion.

And I thank all the witnesses here.

Look now, I came here as an immigrant, got a Ph.D. in chemistry, did my post doc at the University of Michigan, and shortly thereafter started a small innovation research company. So this feels like something that I have done for 25 years. A lot of what we did was develop pharmaceutical formulations, made clinical trial materials for small startup businesses. Many startup businesses, very innovative, you know. A lot of innovation, as you know, happens in small Pharma companies. But often they don't have the resources to do all of the drug development process, do all of the regulatory QAQC testing, all of that. So I had a facility that had 65 scientists at one time, Ph.D. scientists, equipment, formulation, a CGMP facility. So I could provide these kind of services and do innovation. And that helped, because often, you know, some small segments of population that has a rare disease doesn't get noticed by large Pharma because there is just not enough profits in there. And so a lot of small innovative companies are working on that. And what I have noticed is the burden from FDA, the delays getting responses, and overall not having the resources, financial resources to be able to develop these drugs. And my question to you all is that, what can Congress do to facilitate such innovation? What can Congress do to keep our scientific community at the top of innovation, discoveries, and keep our leadership in this area? As a pharmaceutical scientist, I am very interested in your answers here. Anybody.

Dr. MILLER. So I think, moving more towards decentralization of pricing, as opposed to centralization, which is what we did with the IRA. I think another thing, as I said, is clinical trial reform, because the small companies need guidance. If a small the company gets one meeting on Zoom 6 months into their development program and they have lots of questions that they need answered in those first 3 or 6 months as they are setting up trials and making decisions, they need to be able access the brain. They can't pay someone $2,000 an hour who has been doing this in the space for 30 years. They need the FDA reviewer brain who has been in the space for 15, 20 years and sits in that entire area and has seen 15 to 20 years of development programs through INDs, NDAs, sNDAs,

et cetera. So, we need to make sure that the FDA is a partner to the small companies and entrepreneurs. Those SBIA programs are great, but they are not a substitute for having that scientific, clinical, and technical guidance available at the fingertips for those entrepreneurs.

Mr. THANEDAR. Anybody else?

Mr. NEWELL. Thank you, Congressman.

As a CEO of a company that is developing new medicines starting at the 19th employee and now 300 employees, with our CGMP manufacturing facility, I know a lot of what you talk about. The hurdles that we have from a regulatory standpoint are enormous. And that requires a tremendous amount of capital and expertise.

In the development process for our lead program, we started clinical development in 2019. Here we are in 2024, and we are still 2 to 3 years away from having the data set that will be satisfactory for FDA.

One of things that we have had to do is to satisfy a new initiative within FDA called Project Optimus where they are trying to get companies to not identify the most effective dose for the patient, but the lowest, most effective dose for the patient. And so, while we were prepared to start our pivotal trial about a year ago, we had to first study two doses of our drugs in 50 patients, and that will delay our trial about a year. So there are actions that Congress can take to allow and incentivize FDA I think to accelerate development rather than hinder development.

Mr. THANEDAR. Thank you so much. I am out of time, but I would love to work with you, all of you. Thank you.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative Maloy from the great State of Utah for 5 minutes.

Ms. MALOY. Thank you, Mr. Chairman. I have been sitting here listening to your testimony. I had a whole list of questions I wanted to ask, and most of them have already been asked. That is one of advantages of being junior on a committee; you get to learn to think on your feet. But it seems clear that we need to do a better job at trials and approvals. It seems like we are hearing a lot of that.

And, as a Member of Congress, I am nervous about this conversation because having Congress get involved isn't always helpful when we are trying to get things to be more efficient and quicker, but we do have oversight responsibilities with FDA. And I just want to ask, what can we do that would help with the efficiency of approvals and trials without adding more bureaucracy and making it more cumbersome? I want to start with Dr. Miller. I know you have covered this, but I just want to be really clear, how can we help without getting in the way?

Dr. MILLER. So the specific things to ask is, how many staff are in each center, and what are the tasks that they are doing? Another thing is, how long does a company have to wait to get a meeting? How long do they have to wait to get an in-person meeting? How frequently are the staff in the office? I realize some work can be done remotely, but when you are doing intellectual collaborative, frankly, highly creative and interdisciplinary work, you need to be able to walk down the hall. We had a guy in our group whom we

called the grand mentor. He had been there 30 years. We bought him a seersucker jacket. He had basically seen everything. You would just walk down the hall and ask him questions because he had been there forever. That doesn't happen if you are all working remotely at home 30 minutes to hours away from the agency. So I would say making sure also people get back to the office.

Ms. MALOY. Dr. Eagle?

Dr. EAGLE. I can't speak as an authority on clinical trials the way Dr. Miller can. But I will just tell you, in 40 percent of the FDA-approved therapies in oncology—or 40 percent of the FDA-approved therapies are oncology drugs and is how we make progress. It is critical to be able to do these trials in the community, and we do those in our practice. It is a lot of work, though. But the trials are better when they are, you know, in communities because the results are more accurate. You get better diversity of trial participants, and the trial results translate into the real world better than if they are all done at academic centers.

Ms. MALOY. Mr. Newell?

Mr. NEWELL. Thank you for your question.

I would like to build on what Dr. Miller said. When we go through the regulatory process with the FDA, we have a number of meetings. Some of them are on what are called CMC, chemistry manufacturing and control subjects, basically how we make our medicine. Some of them are on the design of the trial. Some of them are on the statistical plans that underpin the design of the trials. Even though we are in California, we would welcome the opportunity for in-person meetings. I don't think we have had an in-person meeting in the 6 years that we have been in clinical development at Sutro Biopharma. We do get Zoom meetings. They usually occur at the very end of the time period at which they have to grant the meeting. And not everyone is on camera during the Zoom meetings themselves. So there is a huge barrier I think to the proper exchange of information and getting to the right answers for the company and for the regulatory system and being in-person and or being on camera would be a great step forward for us in terms of that communication efficiency.

Ms. MALOY. Thank you.

Dr. Eagle, I want to come back to you. Sorry, I am going to skip Dr. Zuckerman for a minute. My colleague from Washington covered a lot of this, but I also hear a lot of frustration from independent pharmacists and independent healthcare providers about, you know, the vertical integration that is making their lives harder and making it harder for them to compete. And, when you were answering questions about that earlier, you said we need more transparency in PBMs. Do you want to just quickly tell us what that would look like?

Dr. EAGLE. Well, I think, if you look at the economics of PBMs, there is—it is a little misunderstood about how—where the money goes. They are truly the only entity in the entire process, and when they contract, it really sees all contracts and really knows how the money flows. So I think that would be a first step.

So, if you look at where drugs rebates go, whether those savings really get passed on to the patients in the plans, that is just not entirely clear. It is stated that that happens, but we just don't

know that. So I think transparency would be a first start, but I think the legislation after that would need to follow as well.

Ms. MALOY. Okay. Mr. Newell, last question for you, and I am almost out of time so really quick answer. But how important—it is troubling to me that you are 21 years old and still not commercial. How important is the timing of reimbursements for small businesses that develop breakthrough technologies from Medicare?

Mr. NEWELL. It is vital. You know, when we do this work, we want to get a medicine to a patient, and we need to ensure that the patient has no barrier to getting that. So reimbursement is vital to the whole ecosystem.

Ms. MALOY. Thank you.

I yield back.

Chairman WILLIAMS. The gentlelady yields back.

I now recognize Representative Molinaro from the great State of New York for 5 minutes.

Mr. MOLINARO. Thank you, Mr. Chairman. Although, every time you say "the great State of New York," I always have chuckled that not only, obviously, are small businesses overburdened by federal regulations—I talked a great deal about that, and we are going to get into that in a little bit—but it is even worse in the State of New York. Sadly the federal/state regulatory regime has not only used overregulation as an enforcement mechanism, but sadly, absent both Congress and even the State legislature's involvement, enforcement and regulation is used as policymaking. And that is a frightening recipe and, quite frankly, has led to so much consolidation and smaller businesses, smaller practices, and smaller providers leaving the community.

For rural communities like the ones I represent in upstate New York, without question, small independent providers serve as a critical lifeline for patients in connecting them with high-quality and affordable medical services. We acknowledge this. From skyrocketing regulatory compliance cost to labor shortages, it is clear our small providers, especially those in rural communities, are struggling to stay afloat. It is one of the reasons I sponsored the Healthcare Workforce Innovation Act to ensure qualified medical, behavioral, and oral health professionals can practice in medically underserved and rural communities. We, in upstate New York, sadly live in medical deserts.

As healthcare consolidation has accelerated over the past two decades, the number of independent practices continues to shrink. You have all acknowledged this. And, according to the American Independent Medical Practice Association, from 2019 to 2022, the share of physicians working hospital health systems or other large corporations grew from 62 to 74 percent. And I worry that that has accelerated even more so in communities throughout New York.

Dr. Eagle, I want to return to some of your testimony where you elaborate a bit on how CMS regulation enacted during COVID-19—which, by the way, I lived on the front line of public health as a county executive during COVID-19. When we didn't know what we didn't know, we made decisions that made sense. And then we knew a lot and started to make even more decisions that made no sense. One such regulation barred the independent medical practices from delivering to patients medications via mail or similar de-

livery means, which of course made no sense during COVID. It makes even less sense now. I joined 53 of my colleagues in issuing a letter urging HHS and CMS to reconsider this regulation to help ensure patients' and their medical providers' needs are accounted for in the regulator process.

Dr. Eagle, if you could, just share a little bit more about your experience as a small provider—modest provider, not so small but small provider, navigating the burdens of the regulatory environment and the impact this particular order had on patient care.

Dr. EAGLE. Thank you so much for the question.

As I mentioned previously, what we do in our practice is medically integrated dispensing so it is different when patients get their oral drugs from us than when they do from a PBM, because the PBMs, they don't do the same patient support, financial support. There is a loss of—they don't know the critical record.

The new CMS regulation is a massive burden, and it was issued through an FAQ, which is not the typical rulemaking process. So that in itself was problematic. But we can no longer mail these drugs to patients. Only the PBMs can mail drugs to patients. So sick cancer patients now have to physically come into the office to pick up their medications. It is not even allowed that the patient's family members can come to the office and pick up the medications. The patients themselves who are battling cancer have to make these trips. In rural areas, in New York, that can be a huge burden, but just getting around the Long Island area and New York City can be a challenge as well also. So that is prime issue that really needs scrutiny. And it would be very easy for CMS to reverse this requirement, but we are very disappointed that they seem to be dug in on this.

Mr. MOLINARO. As are many of us. And if we think the healthcare system—and, again, I try to localize it a bit, the healthcare system in New York is a bit on life support as it is; The mental healthcare system is relatively nonexistent. And access to medications in a more fluid and accessible way for those dealing with mental illnesses is critically important.

I just want to very quickly reference, in my district, there are several small domestic pharmaceutical companies and manufacturers, including Alvogen, that experience extreme challenges working with federal bureaucracy. Lacking certainty in matters involving the government is a challenge for any business of any size. It is one of the reasons this committee exists, but it is especially challenging for small companies that lack financial resources to stay afloat during periods of time while they wait on government to act.

For any of you, if you would, in my 20 seconds, how does that uncertainty in the bureaucratic red tape impact the ability in particular to small biotech companies to meet providers' needs?

Mr. NEWELL. Congressman, maybe I will answer that quickly.

Mr. MOLINARO. Please.

Mr. NEWELL. In order to raise capital, we need to chart a path for the medicines that we are developing. And we need to persuade investors that we know that that path is achievable. When there is uncertainty from a regulatory standpoint, investors sense that uncertainty, and they are less likely to invest. And, without capital, we are not able to bring new medicines to the market.

Mr. MOLINARO. Thank you. Thank you, Mr. Chairman.

Chairman WILLIAMS. The gentleman yields back.

I now recognize Representative LaLota from the great State of New York for 5 minutes.

Mr. LALOTA. The great State of New York, Mr. Chairman.

Chairman WILLIAMS. That is what I said.

Mr. LALOTA. Yes, sir. It is easy to agree with you, Mr. Chairman.

Dr. Eagle, it is always nice to welcome a fellow New Yorker, a fellow Long Islander, to Washington. You have more than 20 years' experience in healthcare, with specialized training in diagnosing and treating cancer. Additionally, you are the past president of the Community Oncology Alliance and are acutely aware of how overregulation affects small practices.

The matter of cancer, we have heard it from several of our Members today, is a personal one to us and our constituents. Me in particular, in March 2006, I lost my father after a yearlong battle to lymphoma. He died in Mather Hospital in Port Jefferson. He was a big, strapping, strong Italian man. And the disease ate him away in many different ways. My three daughters born after never got to meet him.

And, before I ask you a few questions, I want to commend you for your lifetime of dedication to work in this field to try to bring comfort to many families, to try to treat and end this disease. It is commendable that you have spent your professional life on this endeavor.

Specifically and switching gears a little bit, one thing that stood out to me in your written testimony is that your practice treats all patients, including Medicaid. Are you telling me or are you saying that other hospital systems in the New York area do not treat patients who use Medicaid?

Dr. EAGLE. Thank you for the question.

To the best of my knowledge, that is true, you know. We participate in all insurance plans in New York. That is a rarity in New York, frankly. We participate in all the Medicaid plans. I don't believe the nonprofit hospitals do participate in all the Medicaid plans. So, you know, we really try to take care of the community. I think, when a lot of people look how to build healthcare and who provide subsidies to, they look to nonprofit hospitals. But I think, in New York, it is notable that our practice, which is 80-plus million oncologists, we accept all the insurance plans and work very hard to stay in all networks.

Mr. LALOTA. What do you think is the impact of others not accepting Medicaid?

Dr. EAGLE. Well, you can't help a patient unless you see them. The only way you can help a cancer patient is being in their community and give them access to your clinic and their care.

Mr. LALOTA. Great, thanks. And can you describe how oncology care has changed for patients, doctors, and staff after your practice transitioned from your small private practice to your large regional health system?

Dr. EAGLE. When we were a three-physician practice, we started every Monday at 8 o'clock in the morning with a 30-minute meeting with the entire staff, talking about clinic operations, how

to do little things right. When you are in small private practice, the only thing you can do is win on quality and service. We had no other structural advantages. So it was just critically important to make sure that all the staff was involved with every part of the clinic and making it operate as efficient as it possibly could and welcoming to the patients as it possibly could. Unfortunately, with the transition to the hospital, we lost a quarter of our employees before the changes even happened. They just did not want to work for a hospital system. I think today maybe even less than one-third are left. So much of the efficiencies, so much of the personalized care, so much of the things that we personally enjoyed about taking care of cancer patients just became harder to do.

Mr. LALOTA. Yeah. We want to take care of patients. We want to take care of constituents. Can you describe to the committee the difference between how a hospital does this or how a private physician office like yours approaches this? And, specifically, is there an unlevel playing field between the two?

Dr. EAGLE. Structurally there is a tremendously unlevel playing field. Particularly in oncology, there is the 340b program. So hospital oncology programs and nonprofit hospitals can drop substantial profits from that program. They derive higher commercial contract reimbursement. But they also get paid facility fees under the Medicare program and just higher payments generally across the board for Medicare for the same services.

Mr. LALOTA. And the dynamic that seems to favor the large hospitals, is that hurting patient care, or are the patients disadvantaged with the unlevel playing field?

Dr. EAGLE. You know, when I transitioned into the hospital, I was able to see about one-third fewer patients. So I think patients begin to wait longer for appointments. They are paying higher costs both commercially and through facility fees on Medicare. So I think it has tremendous patient impacts.

Mr. LALOTA. Great. Thank you so much. Appreciate all of your feedback today. This is one of the more functional committees in Congress. The Republicans and Democrats seem to agree on a decent amount of things. We have produced a fair amount of legislation subsequent to testimony from folks like you. So I want to say thanks so much for being here.

Mr. Chairman, I yield back.

Chairman WILLIAMS. The gentleman yields back.

And I would like to thank our witnesses today for their testimony, for you appearing. It has been a good hearing, and one—I think we love to have people come up and go before our committee because, most of the time, we are pretty bipartisan. We see things as they should be and try to get something done.

Without objection, Members have 5 legislative days to submit additional materials and written questions for the witnesses to the Chair, which will be forwarded to the witnesses. I ask the witnesses to please respond promptly if that happens. And I have no further business.

Without objection, the committee is adjourned.

[Whereupon, at 11:48 a.m., the committee was adjourned.]

APPENDIX

Testimony of Brian J. Miller, M.D., M.B.A., M.P.H.

Assistant Professor of Medicine and Business (Courtesy)
The Johns Hopkins University School of Medicine
The Johns Hopkins Carey Business School

Nonresident Fellow
American Enterprise Institute

Before the

U.S. House of Representatives Committee on Small Business

On

"Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare."

May 8, 2024

Chairman Williams, Ranking Member Velázquez, and distinguished members of the U.S. House of Representatives Committee on Small Business:

My name is Brian Miller, and I practice hospital medicine at the Johns Hopkins Hospital. As an academic health policy analyst, I serve as an Assistant Professor of Medicine and Business (Courtesy) at the Johns Hopkins University School of Medicine and as a Nonresident Fellow at the American Enterprise Institute. My research focuses on how we can build a more competitive and vibrant health sector to make healthcare more flexible and personalized for patients. This perspective is based upon my prior regulatory experience at four federal regulatory agencies, including the U.S. Food & Drug Administration where I worked in policy and as a reviewer in the Center for Drug Evaluation and Research's Office of New Drugs. Through my current role as a faculty member, I regularly engage with regulators, policymakers, and businesses in search of solutions to help create a better healthcare system for all. Today I am here in my personal capacity, and the views expressed are my own and do not necessarily reflect those of the Johns Hopkins University, the American Enterprise Institute, or the Medicare Payment Advisory Commission.

In my testimony today, I will focus on:

1. Why Pharmaceutical Product Innovation Matters to Patients and Physicians
2. Historical Actions to Address FDA Barriers to Innovation
3. FDA Reform to Promote Pharmaceutical Product Innovation for Small Companies

1. Why Pharmaceutical Product Innovation Matters to Patients and Physicians

As one of the world's wealthiest countries, we spend over $4.5 trillion dollars[1] on healthcare services and related medical products to care for over 330 million Americans. While half of this is spent on physician services and hospitals, the latter a sector with flat or declining labor productivity growth,[2] life sciences innovation has a been a bright spot in the health sector's otherwise dark history with prescription drug spending representing an estimated 9% of national health expenditures.[3] With industry developing over 1,200 new drugs since 1950[4] and over 20,000 prescription drugs approved for marketing (including generics) and over 400 licensed biologic products,[5] patients and their physicians have options for a variety of diseases. Price competition from generics has resulted in increased affordability, with the U.S. Food & Drug Administration's (FDA) own economists demonstrating that entry of even a sixth generic competitor resulting in further price decrements.[6]

Yet, much work remains to be done. An estimated 10 million children suffer from rare diseases–or those affecting less than 200,000 children–and only 5% have treatments.[7] Chronic diseases such as insulin-dependent diabetes affect over 5 million Americans,[8] with the inconvenience and pain of checking one's blood sugar and injecting insulin multiples times per day for the rest of one's life. Complications range from limb amputations[9] and vision loss[10] resulting in significant functional impairment. Still other disease families such as heart failure, a disease managed primarily via

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[1] NHE Fact Sheet. Centers for Medicare & Medicaid Services. https://www.cms.gov/data-research/statistics-trends-and-reports/national-health-expenditure-data/nhe-fact-sheet.
[2] Productivity. U.S. Bureau of Labor Statistics. https://www.bls.gov/productivity/highlights/hospitals-labor-productivity.htm
[3] NHE Fact Sheet. Centers for Medicare & Medicaid Services. https://www.cms.gov/data-research/statistics-trends-and-reports/national-health-expenditure-data/nhe-fact-sheet.
[4] Munos, B. Lessons from 60 years of pharmaceutical innovation. *Nat Rev Drug Discov* **8**, 959–968 (2009). https://doi.org/10.1038/nrd2961
[5] FDA at a Glance. U.S. Food & Drug Administration. October 2019. https://www.fda.gov/media/131874/download
[6] Conrad R, Lutter R. Generic Competition and Drug Prices: New Evidence Linking Greater Generic Competition and Lower Generic Drug Prices. U.S. Food & Drug Administration. December 2019. https://www.fda.gov/media/133509/download?attachment
[7] Hwang TJ, Bourgeois FT, Franklin JM, Kesselheim AS. Impact Of The Priority Review Voucher Program On Drug Development For Rare Pediatric Diseases. *Health Affairs*. 2019/02/01 2019;38(2):313-319. doi:10.1377/hlthaff.2018.05330
[8] https://www.cdc.gov/diabetes/data/statistics-report/index.html
[9] Vogel TR, Petroski GF, Kruse RL. Impact of amputation level and comorbidities on functional status of nursing home residents after lower extremity amputation. *J Vasc Surg*. 2014;59(5):1323-30.e1. doi:10.1016/j.jvs.2013.11.076
[10] Brown MM, Brown GC, Sharma S, Landy J, Bakal J. Quality of life with visual acuity loss from diabetic retinopathy and age-related macular degeneration. *Arch Ophthalmol*. 2002;120(4):481-484. doi:10.1001/archopht.120.4.481

small molecule drugs, affects over 6 million Americans nearly half of whom have difficulty with basic activities such as climbing stairs.[11]

Cancer remains a longstanding policy and political focus as it affects all of us: in 2023, an estimated 1,958,310 Americans were newly diagnosed with cancer and an estimated 609,820 Americans died of cancer the same year.[12] Cancer as a disease family can be viewed through the lens of its organ, cellular, or even molecular origins. Some cancers such as melanoma are responsible for a large number of diagnoses and are frequently caught earlier, thus representing a lesser share of cancer deaths.[13] Others such as Merkel Cell Carcinoma are extremely rare,[14] discovered later, and result in significant mortality.[15]

Impacts are significant for society and individuals. The economic impact of diabetes is estimated at $412 billion,[16] while cancer deaths resulted in lost earnings of $94.4 billion[17]–a number from over a decade ago. The direct costs for those undergoing treatment are real, with a study of 1,037 patients undergoing treatment for acute myeloid leukemia having twice and five times the rate of short and long-term disability claims filed.[18] While statistical data provide an overarching claim, one cannot forget the individual cost: each patient is someone's spouse, children, friend, or co-worker. A new lease on life or restored functional status can transform someone's life–the release of etanercept (Enbrel) in 1998 marked a new chapter in my elderly grandmother's life as her Rheumatoid Arthritis came under control and she was able to bake her famous crescent roles again at Thanksgiving and work in her garden tending her roses. For some Americans, innovation is not just a new lease on life but better living through pharmaceuticals.

2. Historical Actions to Address FDA Barriers to Innovation

Barriers to pharmaceutical product innovation are very real, with the average drug taking 10-15 years to travel from bench to bedside and the average cost of development estimated at $2.6 billion,[19] innovators especially entrepreneurs and small companies face high barriers to innovation. With drugs traversing preclinical research, phase 1 (safety), phase 2 (efficacy and further evaluate safety), and phase 3 (efficacy and adverse events), each research stage acts as a scientific and clinical development gate, with 70% passing phase 1, 33% passing phase 2, 25-30% passing phase 3,[20] and fewer than 8% of experimental therapeutics making it through all three phases of development.

Recognizing the time, expense, and expected scientific and clinical failures in pharmaceutical product development, policymakers have worked through the prescription drug user fee acts in conjunction with agency actions to undertake key initial reforms at FDA to safely promote access to pharmaceutical product innovation, in addition to lowering barriers for small companies and entrepreneurs:

1. Priority Review: drugs that would offer significant improvements in safety or effectiveness–if so designated–are reviewed in 6 months instead of the standard 10 months.[21] A designation created under the original 1992

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[11] Dunlay SM, Manemann SM, Chamberlain AM, et al. Activities of Daily Living and Outcomes in Heart Failure. *Circulation: Heart Failure*. 2015;8(2):261-267. doi:doi:10.1161/CIRCHEARTFAILURE.114.001542

[12] Siegel RL, Miller KD, Wagle NS, Jemal A. Cancer statistics, 2023. *CA Cancer J Clin*. 2023;73(1):17-48. doi:10.3322/caac.21763

[13] Cancer Stat Facts: Melanoma of Skin. National Cancer Institute. https://seer.cancer.gov/statfacts/html/melan.html

[14] Paulson KG, Park SY, Vandeven NA, et al. Merkel cell carcinoma: Current US incidence and projected increases based on changing demographics. *J Am Acad Dermatol*. 2018;78(3):457-463.e2. doi:10.1016/j.jaad.2017.10.028

[15] Fitzgerald TL, Dennis S, Kachare SD, Vohra NA, Wong JH, Zervos EE. Dramatic Increase in the Incidence and Mortality from Merkel Cell Carcinoma in the United States. *Am Surg*. 2015;81(8):802-806. doi:10.1177/000313481508100819

[16] Parker ED, Lin J, Mahoney T, et al. Economic Costs of Diabetes in the U.S. in 2022. *Diabetes Care*. 2024;47(1):26-43. doi:10.2337/dci23-0085

[17] Islami F, Miller KD, Siegel RL, et al. National and State Estimates of Lost Earnings From Cancer Deaths in the United States. *JAMA Oncol*. 2019;5(9):e191460. doi:10.1001/jamaoncol.2019.1460

[18] Pandya BJ, Young C, Packnett ER, et al. Work absenteeism, disability, and lost wages among patients with acute myeloid leukemia and their caregivers: a cohort study using US administrative claims and productivity data. *Expert Rev Pharmacoecon Outcomes Res*. 2024;24(4):521-532. doi:10.1080/14737167.2024.2311305

[19] *Research and development*. PhRMA Org. https://phrma.org/policy-issues/Research-and-Development-Policy-Framework

[20] Step 3: Clinical Research. U.S. Food & Drug Administration. https://www.fda.gov/patients/drug-development-process/step-3-clinical-research

[21] Priority Review. U.S. Food & Drug Administration. https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review

Prescription Drug User Fee Act (PDUFA), priority review has been further expanded upon by a voucher program to incentivize entrepreneurs to develop products for areas such as rare pediatric disease,[22] a program set to expire this September.

2. Fast Track: drugs that are being development to treat a condition with no therapeutic options or that offer an advantage over current treatment qualify for this designation and product sponsors receive more frequent meetings with FDA, more frequent written communication with FDA, eligibility for rolling review, and eligibility for accelerated approval and priority review as salient.[23,24] A designation created as part of the 1997 FDA Modernization Act (FDAMA); of the 3,392 designations filed from 1998-2023, 2238 or 66% were approved.[25]

3. Breakthrough Therapy: Fast Track plus intensive guidance and organization commitment from senior managers.[26] A designation created as part of the 2012 FDA Safety and Innovation Act (FDASIA).[27]

4. Accelerated Approval: Permits earlier approval based upon a surrogate endpoint (e.g. laboratory, radiological measurement) for drugs that treat serious conditions and fulfill an unmet need. The sponsor is required to conduct confirmatory studies once the product is marketed to prove the clinical benefit, converting the accelerated into a traditional approval. Accelerated approval was created as part of PDUFA, in response to the HIV/AIDS epidemic of the 1980s.[28]

While some of these regulatory pathways have been the subject of academic controversy, research examining the accelerated approval pathway by its greatest critiques demonstrates that confirmatory trials are typically completed, albeit at times with a delay,[29,30] further emphasizing the need to increase the access to clinical trials (clinical trial recruitment is a frequent barrier). Still other research has critiqued[31] the FDA's use of surrogate endpoints/biomarkers,[32] failing to recognize that science-based regulatory policy involves tradeoffs between perfect and imperfect information for making regulatory decisions in a real-world setting. Erring on the side of restricting access results in death and debility for many, while permitting access with appropriate and robust oversight safely expands treatment options.

3. FDA Reform to Promote Pharmaceutical Product Innovation for Small Companies

Regulatory barriers are magnified for entrepreneurs and small companies who with limited financial capital, limited patent lives, and increasing payment policy uncertainty face unnecessary regulatory challenges at the FDA. Recent

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[22] Mease, C., Miller, K.L., Fermaglich, L.J. et al. Analysis of the first ten years of FDA's rare pediatric disease priority review voucher program: designations, diseases, and drug development. *Orphanet J Rare Dis* 19, 86 (2024). https://doi.org/10.1186/s13023-024-03097-x

[23] Fast Track. U.S. Food & Drug Administration. https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track

[24] Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics. U.S. Food & Drug Administration. May 2014. https://www.fda.gov/media/86377/download?attachment

[25] CDER Fast Track Designation Requests Received Fiscal Year 1998 – Fiscal Year 2023. U.S. Food & Drug Administration. https://www.fda.gov/media/97830/download

[26] Break Through Therapy. U.S. Food & Drug Administration. https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy

[27] Kepplinger EE. FDA's Expedited Approval Mechanisms for New Drug Products. *Biotechnol Law Rep*. 2015;34(1):15-37. doi:10.1089/blr.2015.9999

[28] Stengel K, Zalewski Z, West M, Gustafson K, Nell A. Understanding the History and Use of the Accelerated Approval Pathway. Avalere. January 4, 2022. https://avalere.com/insights/understanding-the-history-and-use-of-the-accelerated-approval-pathway

[29] Deshmukh AD, Kesselheim AS, Rome BN. Timing of Confirmatory Trials for Drugs Granted Accelerated Approval Based on Surrogate Measures From 2012 to 2021. *JAMA Health Forum*. 2023;4(3):e230217. doi:10.1001/jamahealthforum.2023.0217

[30] Delays in Confirmatory Trials for Drug Applications Granted FDA's Accelerated Approval Raise Concerns. U.S. Department of Health & Human Services. September 2022. https://oig.hhs.gov/oei/reports/OEI-01-21-00401.pdf

[31] Wallach JD, Yoon S, Doernberg H, et al. Associations Between Surrogate Markers and Clinical Outcomes for Nononcologic Chronic Disease Treatments. *JAMA*. Published online April 22, 2024. doi:10.1001/jama.2024.4175

[32] Table of Surrogate Endpoints That Were the Basis of Drug Approval or Licensure. U.S. Food & Drug Administration. https://www.fda.gov/drugs/development-resources/table-surrogate-endpoints-were-basis-drug-approval-or-licensure

FDA actions such as the recent the 528 page laboratory-developed test rule[33] have favored large companies that can bear the cost of regulation, bypassing small companies and entrepreneurs while favoring the precautionary principle instead of managing the complex interplay between continuum of risk and innovation. Instead, policymakers and regulators should promote dynamic competitive markets by doing the hard work of balancing risk and innovation. In this context, two primary policy levers present an opportunity to expand access to innovation, diversifying trials and moving them into the community setting: clinical trial reform and administrative simplification.

Clinical trial reform

While much of the recent focus about diversity in clinical trials has focused on equity,[34,35] practical science suggests that expanding access to clinical trials will improve the efficiency of evidence generation and clinical meaning of pharmaceutical product development. Ensuring that a broader range of Americans can access and are included in clinical trials will advance science, improve clinical practice, and expand access to novel therapeutics. Three policy levers within the FDA can expand access to clinical trials: 1) real world evidence to drive patient-reported outcomes, 2) flexibility in outcomes assessment, and 3) promoting positive creativity in trial design.

Patient reported outcomes using real-world evidence can lower barriers to and the costs of participating in and executing clinical trials. While the FDA has long had a framework for real world evidence[36] and guidance dating back to 2009 on patient-reported outcomes,[37] functionally what this means is focusing on outcomes meaningful to the end user. While lab tests and intermediate biomarkers are useful proxies and provide important interim and statistical insight into the efficacy of therapeutics, their collection may be burdensome to the patient involving transit to a study center, sample collection, and a delay in processing. High level patient-focused outcomes such as hospitalization matter, albeit in many conditions occur infrequently and thus require large study populations in order to detect statistically and clinically meaningful differences, massively raising costs thus favoring large companies and pushing product developers towards biomarkers.[38]

A shift towards patient reported outcomes does not have to be burdensome to patients or innovators. For example, in chronic obstructive pulmonary disease, breathlessness and loss of functional status are key indicators, with 46% of patients reporting moderate to severe shortness of breath[39] while functional capacity as measured by six-minute walk distance and muscle strength declined with time.[40] Other conditions such as Parkinson's disease result in impairments in strength and coordination, that can potentially be measured through simple functional tests.[41] While historically functional assessments occur in a clinical setting undertaken by a physician, many could be undertaken in the home setting, either by the patient themselves assisted by a family, or remotely with automated or live guidance from a trained layperson or skilled medical professional such as a study nurse. Examining pharmaceutical product development through this lens would both reduce the cost of development for small companies, lower barriers to trial participation for poor and minority beneficiaries, and improve the clinical meaningfulness of outcomes of clinical trials.

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[33] "Medical devices, Laboratory Developed Tests." May 6, 2024. https://www.federalregister.gov/documents/2024/05/06/2024-08935/medical-devices-laboratory-developed-tests

[34] Improving Representation in Clinical Trials and Research: Building Research Equity for Women and Underrepresented Groups. National Academies of Sciences, Engineering, and Medicine. 2022. https://nap.nationalacademies.org/catalog/26479/improving-representation-in-clinical-trials-and-research-building-research-equity

[35] Schwartz Aaron L, Alsan M, Morris Alanna A, Halpern Scott D. Why Diverse Clinical Trial Participation Matters. *New England Journal of Medicine*. 2023/04/05 2023;388(14):1252-1254. doi:10.1056/NEJMp2215609

[36] Framework for FDA's Real-World Evidence Program. U.S. Food & Drug Administration. December 2018. https://www.fda.gov/media/120060/download?attachment

[37] Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. U.S. Food & Drug Administration. December 2009. https://www.fda.gov/media/77832/download

[38] Hinder M, Yi BA, Langenickel TH. Developing Drugs for Heart Failure With Reduced Ejection Fraction: What Have We Learned From Clinical Trials?. *Clin Pharmacol Ther*. 2018;103(5):802-814. doi:10.1002/cpt.1010

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[40] Kapella MC, Larson JL, Covey MK, Alex CG. Functional performance in chronic obstructive pulmonary disease declines with time. *Med Sci Sports Exerc*. 2011;43(2):218-224. doi:10.1249/MSS.0b013e3181eb6024

[41] Clael S, Brandão E, Caland L, et al. Association of Strength and Physical Functions in People with Parkinson's Disease. *Neurosci J*. 2018;2018:8507018. Published 2018 Dec 12. doi:10.1155/2018/8507018

Real world evidence and patient reported outcomes must be partnered with flexibility in assessment of outcomes. Clinical trials as executed today require patients to travel to study sites for assessment, interview, and exam by clinical staff throughout the duration of a clinical study. This incurs direct (e.g. transit cost) and indirect cost (e.g. lost wages, childcare) for study participants, limiting access to those without financial means or adequate family and social support. While the FDA has issued draft guidance for decentralized clinical trials,[42] the FDA and small companies together must make this policy a reality. For movement disorders, conducting exams at home with subsequent review of recorded standardized exams by a fellowship-trained movement disorders neurologist at a subsequent date would reduce the cost of trial execution for small companies, increase access to a broader patient population, and support a focus on meaningful outcomes. This type of thinking is rare in product development, as it requires close engagement and supportive partnership for creative small companies by FDA review divisions, with the FDA's 2017 review of valbenazine representing one such example.[43]

Finally, positive creativity in trial design is a must. Over a hundred years of clinical progress has demonstrated that we have a variety of ways in which researchers can answer the question of a drug's safety and effectiveness. While the FDA's standard of two adequate and well-controlled studies was historically seen as a high barrier to entry,[44] positive creativity in trial design such as enrichment,[45] adaptive designs,[46] master protocols,[47] and the transition of clinical trials into community settings[48] as part of routine clinical practice can expand access, increase the diversity of trials, and lower costs thus lowering the barriers to small companies and entrepreneurship in pharmaceutical product innovation. Other steps to use historical trial designs that are less frequently deployed such as a repurposing a study population after a washout period and crossover trials can allow product developers to "do more with less." While small companies can legally take these steps, a management top-heavy FDA with overburdened and inaccessible review staff drives companies to take well-trodden, risk averse paths in clinical development programs, raising costs and limiting patient populations studies.

Administrative simplification to both support and transform the role of the FDA reviewer

In order to realize the long held goal of clinical trial reform, the FDA needs to provide more customized counseling to entrepreneurial small pharmaceutical product developers. In order to do so, the FDA will need to refocus the efforts of staff. Consider the Center for Drug Evaluation and Research (CDER), the primary drug review center at FDA with 5,482 employees in 2022[49] that grew to 5,785 employees in Q1 2024.[50] Out of 12 offices reporting to the CDER Center Director, 2 are directly responsible for drug review: the Office of New Drugs (responsible for reviewing novel, branded products) and the Office of Generic Drugs.[51] As one can see below, some of the offices below could potentially be combined (e.g. Office of Executive Programs, the Office of Management, and the Office of Strategy Programs), eliminating management overhead and freeing up highly trained and technically-skilled staff to be deployed within the Office of New Drugs on other review and product development oversight tasks. Administrative simplification

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[42] Decentralized Clinical Trials for Drugs, Biological Products, and Devices: Guidance for Industry, Investigators, and Other Stakeholders. U.S. Food & Drug Administration. May 2023. https://www.fda.gov/media/167696/download

[43] Davis Michael C, Miller Brian J, Kalsi Jasmeet K, Birkner T, Mathis Mitchell V. Efficient Trial Design — FDA Approval of Valbenazine for Tardive Dyskinesia. *New England Journal of Medicine*. 376(26):2503-2506. doi:10.1056/NEJMp1704898

[44] Temple R. FDA's Clinical Investigator Course: Design of Clinical Trials. U.S. Food & Drug Administration. November 12, 2013. https://www.fda.gov/media/159878/download

[45] Temple R. Complexities in drug trials: enrichment, biomarkers and surrogates. Interview with Robert Temple. *Biomark Med*. 2008;2(2):109-112. doi:10.2217/17520363.2.2.109

[46] Temple R. Enrichment Strategies for Clinical Trials. U.S. Food & Drug Administration. March 25, 2013. https://www.fda.gov/files/drugs/published/Enrichment-Strategies-for-Clinical-Trials-%28PDF---933KB%29.pdf

[47] Woodcock J, LaVange Lisa M. Master Protocols to Study Multiple Therapies, Multiple Diseases, or Both. *New England Journal of Medicine*. 377(1):62-70. doi:10.1056/NEJMra1510062

[48] Woodcock J, Araojo R, Thompson T, Puckrein Gary A. Integrating Research into Community Practice — Toward Increased Diversity in Clinical Trials. *New England Journal of Medicine*. 2021/10/06 2021;385(15):1351-1353. doi:10.1056/NEJMp2107331

[49] Center for Drug Evaluation and Research & Center for Biologics Evaluation and Research Net Hiring Data. U.S. Food & Drug Administration. https://www.fda.gov/industry/prescription-drug-user-fee-amendments/center-drug-evaluation-and-research-center-biologics-evaluation-and-research-net-hiring-data

[50] Center for Drug Evaluation and Research & Center for Biologics Evaluation and Research Net Hiring Data (FY 2023-2027). U.S. Food & Drug Administration. https://www.fda.gov/industry/fda-user-fee-programs/center-drug-evaluation-and-research-center-biologics-evaluation-and-research-net-hiring-data-fy-2023

[51] Department of Health and Human Services, Food and Drug Administration, Center for Drug Evaluation and Research [Chart]. U.S. Food & Drug Administration. https://www.fda.gov/media/131211/download

would also reduce the number of direct reports, empowering the Center director to advocate and be a stronger lead for organizational change.

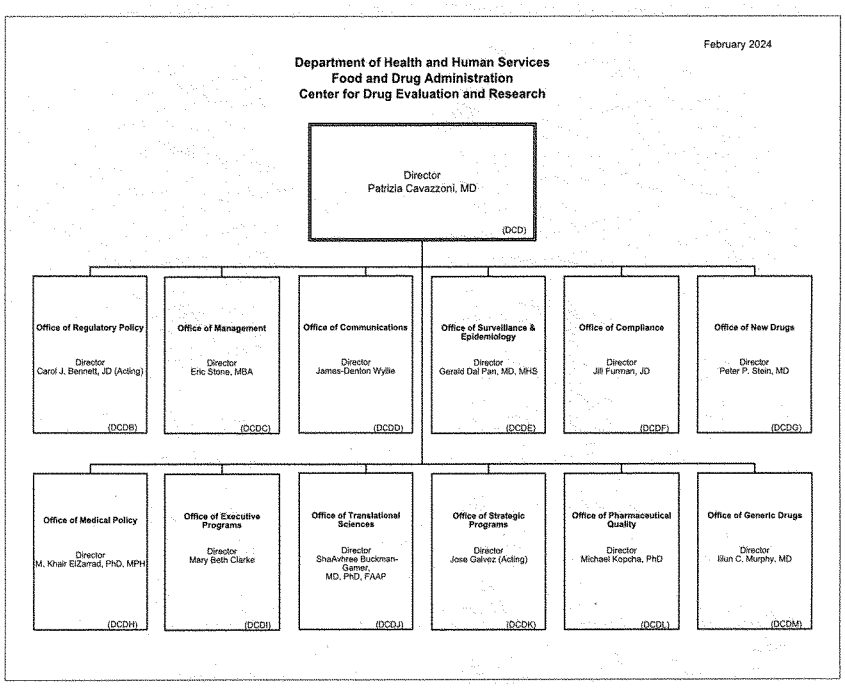


Figure 1: CDER Organizational Chart

Focusing on the Office of New Drugs (OND), administrative layers[52] should also be collapsed in order to reduce administrative overhead and free up staff for front-line product review activities. As a super office, OND has 9 large therapeutic area offices reporting in,[53] each of which are comprised of multiple therapeutic divisions. For example, OND has within it the Office of Rare Diseases, Pediatrics, Urology, and Reproductive Medicine (OPURM). Within OPRUM, one of multiple divisions is the Division of Pediatrics and Maternal Health (DPMH), which has a Division Director, Deputy Director, and then Team Leaders for each of the review teams comprised of medical officers (who are directly responsible for the review) and other technical leads.

[52] "Reorganization of the office of new drugs with corresponding changes to the office of translational sciences and the office of pharmaceutical quality." September 26, 2019. https://www.outsourcedpharma.com/doc/reorganization-office-new-drugs-corresponding-changes-translational-sciences-pharmaceutical-quality-0001

[53] Office of New Drugs. U.S. Food & Drug Administration. https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/office-new-drugs

As a consequence, OND, OPURM, and DPMH each have highly paid physician and scientific leaders who are primarily involved in management activities, as opposed to scientific review of novel pharmaceutical products. In addition to simplifying offices reporting to CDER, flattening the organizational structure within the Office of New Drugs would free up highly trained medical and scientific staff for primary review work. This would rebalance the workload for front-line clinical reviewers who are responsible for new drug application (NDA) reviews with statutory timelines and investigation new drug (IND) reviews where the sponsor can initiate the trial within 30 days,[54] all in addition to regular internal and external meetings.

The FDA can also increase efficiency of reviewers by using artificial intelligence (AI) to assist with basic analyses. Doing so would allow reviewers to operate at a higher level focusing on the intellectual framework and review strategy as opposed to pure computational work, empowering reviewers to better partner with small businesses to promote efficient and effective development programs. Promoting customized and efficient review would transform CDER and broadly make it more like its counterpart, the Center for Biologics Evaluation and Research (CBER), which regularly engages with small companies working in boutique product and rare disease areas.

Increasing the number of reviewers interfacing with industry by repurposing administrative and managerial staff will decrease the average workload of the clinical review while allowing the FDA to meet its statutorily-required review tasks. What does this mean operationally? For a small company that needs guidance on how to creatively design and execute trials, FDA reviewers will finally have the bandwidth to apply their knowledge and guide product developers to more efficiently and effectively assess safety and efficacy, deploying their "top of industry" view as partners in the regulatory review and product development process, improving safety and efficacy all for the long-term benefit of patients.

This redeployment will also make possible the potential transformation of the role of the FDA reviewer. Reviewers are typically highly trained physicians who depart clinical work and transition into a pure regulatory setting. While there are enormous benefits to seeing a wide range of development programs across a therapeutic area, clinical knowledge and pragmatism fades with time. By increasing the number of review staff without increasing the total FDA CDER head count, there is potential for the reviewer role to be transformed from a pure analytical desk job to that of a hybrid practitioner-reviewer–a role exemplified by some current and former FDA reviewers despite current barriers. By continuing to experience the realities and challenges of clinical practice including the problems that patient face, reviewers will better understand the limitations of the data they see, prioritize clinically meaningful outcomes, more fully grasp the need to decrease data collection burdens, and enthusiastically embrace patient-centered outcomes.

Overall, administrative simplification by eliminating managerial layers, providing clarity in lines of command, and expanding the reviewer pool without increasing headcount will empower CDER to serve as a counselor and regulator to small companies developing products, all to the benefit of patients.

4. Conclusion

Healthcare is one of the most complex and regulated industries in the U.S. Yet, stacked administrative regulation raises costs, reduces access, and favors large companies. As the nation's consummate product regulator, the FDA is responsible for ensuring that approved drugs are safe and effective. The current regulatory regime often presents significant barriers to small companies and entrepreneurs, while simultaneously restricting access to clinical trials and limiting diversity and medical progress. While 30 years of Congressional efforts have served to add appropriate flexibility to the FDA's product regulatory schema while preserving and even improving safety, executing on clinical trial reform for the first time in decades coupled with administrative simplification would reduce burdens on small businesses. This would result in expanded access to innovation by diversifying and reducing the cost of clinical trials while promoting their movement into the clinical setting.

[54] Investigational New Drug (IND) Application. U.S. Food & Drug Administration. https://www.fda.gov/drugs/types-applications/investigational-new-drug-ind-application#Introduction

Written Testimony on Hearing:

Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare

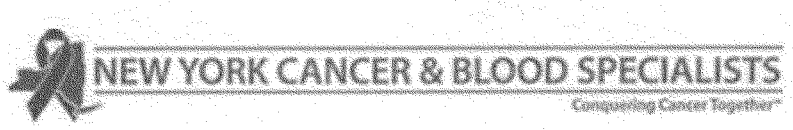
May 8, 2024

United States House of Representatives

Committee on Small Business

David Eagle, M.D.

Practicing Oncologist & Chair of Legislative Affairs and Patient Advocacy



Submitted May 4, 2024

Chairman Williams, Ranking Member Velazquez, and members of the House Committee on Small Business thank you for the opportunity to submit this written testimony and participate as a witness for this critically important hearing on regulatory and related burdens on small businesses in healthcare.

I am a practicing medical oncologist for New York Cancer & Blood Specialists (NYCBS) and serve as chair of legislative affairs and patient advocacy. Our medical practice has 54 locations across the greater Metropolitan New York area providing medical, radiation, and surgical treatment for patients with cancer and blood disorders, and diagnostic imaging services. I am also a Board member of the Community Oncology Alliance (COA), a non-profit group advocating for independent (non-hospital owned) community cancer care and am a Past President.

Although NYCBS may not now fit the traditional definition of a "small business," we started as a very small business with seven oncologists and two locations. Our story is one of survival, and even now, we have to fight daily with the mega health systems in New York, which make us a small business compared to their growing dominance. Furthermore, we are faced with the growing power of the consolidated middlemen of insurers and pharmacy benefit managers (PBMs), state regulations, and the Centers for Medicare & Medicaid Services (CMS), which is driving independent physicians to retire or become hospital employees. In this testimony, I will briefly touch upon all these forces threatening the very existence of small businesses in healthcare, but first, let me tell you about my own story as a physician starting out in a very small medical practice.

My career began in a small, three-physician independent community oncology practice in Mooresville, North Carolina, where I practiced until over two years ago. I have been privileged to care for patients in an era of immense scientific progress in overcoming a disease that threatens to take our closest loved ones from us. My physician partners and I worked closely with our nurses, nurse practitioners, medical assistants, and ancillary staff caring for patients battling cancer and blood disorders. We were a family. We lived with our patients on the front lines of an increasingly broken medical system. We often acted as their last line of defense not only for their medical illness but also for the confusing and overwhelming medical system in which they and we, as their providers, increasingly were fighting. Barriers to care, both large and small, sometimes incidental, other times intentional, popped up all over the place and almost always were far too much to bear for patients facing a battle for their lives. Over the years, these barriers have gotten far worse and more numerous.

Over time, our small practice, operating as a small business, encountered significant pressures from large, well-funded hospital oligopolies in the Charlotte area and beyond in North Carolina. These rapidly consolidating large health systems were increasingly employing internal medicine and related physicians who referred patients to us. As such, these health systems effectively controlled our patient base, with the power to direct patients away from our practice.

In 2017, one large health system in our region gave us an ultimatum – be acquired by us or we will hire physicians to compete against you. One of the prime motivators for this aggressive move by the health system was the federal 340B Drug Discount Program, which provides hospitals with very large discounts on drugs, often exceeding 50 percent. And without any mandate that the hospital pass those discounts on to patients in need, the 340B Program has become a huge profit center for so-called "nonprofit" health systems. As such, the acquisition of our practice would generate substantial immediate profits for the health system, allowing it to further expand.

My partners and I had already witnessed other similar small, independent oncology practices in our immediate area, and North Carolina in general, that lost their battle with these health systems. Unfortunately, large health systems have all the power to shut off patient referrals to a practice, referrals that we depended on to stay in business. Furthermore, we were faced with declining Medicare payment, as well as from consolidating commercial insurers. Those payment issues contributed to a general hostile healthcare environment that stacked the deck against us small healthcare practices like ours.

In 2018, my two partners and I had little choice but to join the large hospital system as employees. Our small, independent practice that had served the community for over 19 years was gone. Hospital clinics operate under stifling bureaucracies and, as a result, almost immediately I was unable to see the same number of patients I was able to see daily in my own independent practice. As devastating, the hospital switched over to its billing system and was able to charge significantly more for the same services – for example, chemotherapy administration – meaning my patients were paying more for the same treatment that had been receiving in my practice. They came to the same building, were treated by me, their same physician, and received the same drugs but paid more. Patients who I had treated and followed for years simply left.

After three years, I left the health system. I simply could not practice in an environment where hospital bureaucracy ruled, impacting my ability to provide the best treatment for my patients but who were paying more for that treatment – much more than when our small practice operated independently of the hospital.

Due to the consolidation of hospitals into large health systems in North Carolina, I joined NYCBS in New York, even though it meant commuting from my still home in Charlotte, North Carolina. As I related, my current practice of almost 300 predominantly oncologists serving all of New York City and Long Island is independent. Unlike the large health systems, we are the only major cancer treatment provider in our region that accepts all insurance plans while also being the only major cancer treatment provider that does not receive state funding or other subsidies. We have opened clinics in underserved communities, are the lowest-cost cancer provider in all of our markets, and were recently named the number one physician practice in New York by Castle Connolly, a rating system based on physician peer reviews.

As I stated previously, every day, our practice is fighting to survive and thrive, especially as federal regulations have created an unduly burdensome environment threatening small provider-based businesses in healthcare. This is an unfolding crisis as the costs of healthcare, especially medical treatment, are escalating out of control, and the increasing demand for physicians is outstripping a decreasing supply. Do you know that during COVID alone roughly145,000 burned-out practitioners, half of which were physicians, walked away from medicine?[1]

Small independent medical practices are vanishing, especially as hospitals in North Carolina and across the country are combining, acquiring physician practices in the process, to become mega health systems. When you couple an almost total lack of regulation from allowing these mega systems to develop[2] with misguided regulation throwing up more barriers to small, independent practices, "small business" in healthcare is going the way of the dinosaurs. You have to grow

[1] https://www.phillymag.com/be-well-philly/2024/04/22/doctors-appointment-scheduling/
[2] https://www.northcarolinahealthnews.org/2024/04/22/the-rise-of-mega-hospitals/

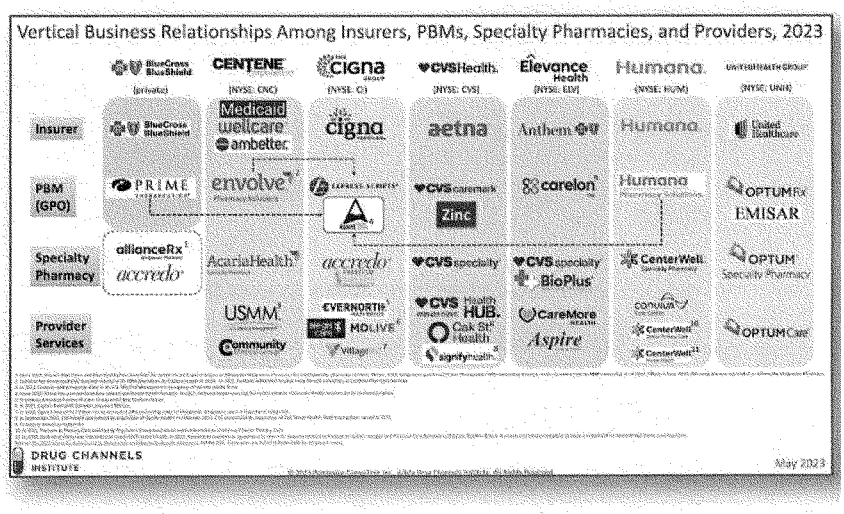
bigger, be more innovative, and fight for misguided regulations to be stripped away to survive. Make no mistake, who suffers in all of this are Americans who are paying more for drugs and medical services, and increasingly having a harder time finding a physician. This is a sobering reality.

Cancer research, drug development, and care delivery are in a renaissance. I am privileged to practice in this era of breakthrough scientific progress. However, patients often do not get the treatment that they need or in the best manner possible. We face a fundamental choice in this country – who is in charge of patient medical care? Is it the physicians and patients making the best treatment decisions together in the exam room? Or is it insurance companies, massive, consolidated health systems, and government regulators like CMS controlling personal healthcare decisions from afar? Over my career, the shift absolutely has been towards the latter.

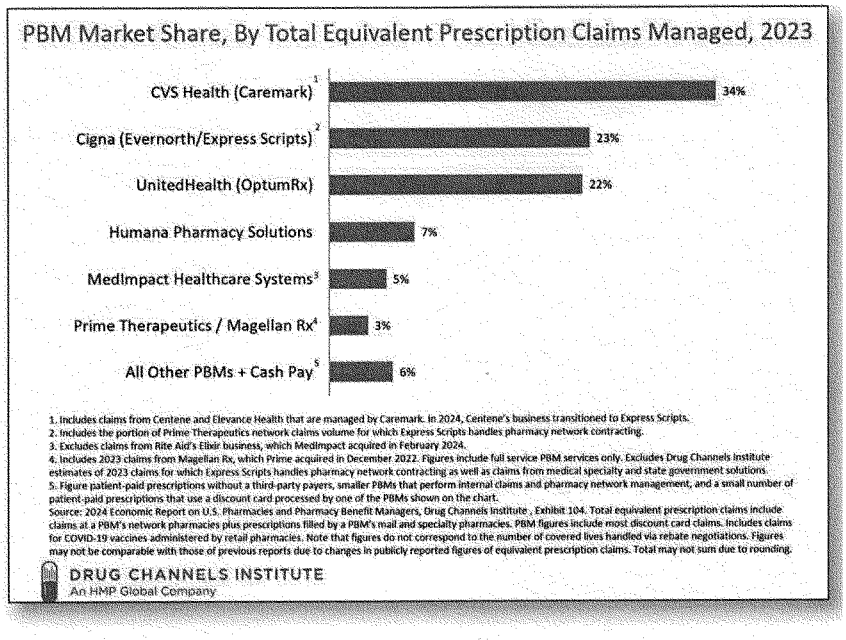
I applaud the House Committee on Small Business for exploring barriers to innovation and the impacts of regulation on small businesses in healthcare. Not to get in the weeds too much, let me touch on some of the misguided regulations that get in my way every day as a practicing oncologist from providing the highest quality, most affordable cancer care to my patients. Hopefully, this will give you a better understanding of what physicians practicing in small healthcare businesses face daily.

Utilization Management by Insurers and PBMs

Let me first explain that there has been tremendous consolidation among insurers, among PBMs, and among the combination of insurers and PBMs. This chart shows the horizontal and vertical integration of insurers, PBMs, and affiliated healthcare delivery entities.



To give further understanding of this consolidation, the largest PBMs control 80 percent of the prescription drug market and the top 6 control 94 percent of all prescription drugs.



What this means is that insurers and their PBMs are increasingly dictating the treatment that my patients receive, as well as how and where they are to receive it. Dictating treatment is done through a number of methods referred to as "utilization management" and include:

- **Prior Authorization** where the insurer/PBM demands that treatment be authorized prior to my administering. Not only do staff and I have to argue with insurance staff, who are often not oncologists, but this is most often unduly time-consuming. There are even increasing reports of how insurers are using AI in prior authorizations.[3]
- **Fail-First Step Therapy** where the insurer/PBM requires the patient to fail first on a sub-optimal therapy before I can administer the best treatment for the patient.
- **Formulary Control** where certain drugs are excluded from the insurer's formulary of their approved medications.

What is important to understand with utilization management is that the insurer and their PBMs are motivated to use the most profitable drugs for them, not the most effective medications for my patients. That is because due to misguided regulations that provide safe harbor protection allowing insurers/PBMs to extract rebates from pharmaceutical manufacturers, these corporate entities are legally allowed to bypass any anti-kickback laws.

Our practice dispenses oral cancer medications to patients through *medically integrated dispensing*. This means that our pharmacy team is closely connected to our team of physicians both through the electronic medical records and the physical locations of our offices. It is common for oral

[3] https://www.propublica.org/article/cigna-pxdx-medical-health-insurance-rejection-claims

cancer therapies to cause side effects necessitating treatment interruption or dose changes. This information is available in real time to our integrated pharmacies. Our integrated pharmacy care reduces waste, improves compliance, and improves outcomes. However, PBMs now often dictate that patients receive their drugs via their mail order pharmacies. This not only takes treatment choices and monitoring out of my hands but also increases costs.

With the consolidation of insurers and PBMs alone, the deck is stacked against the medical practice that is a small business. You are forced to consolidate your practice or simply give up the fight.

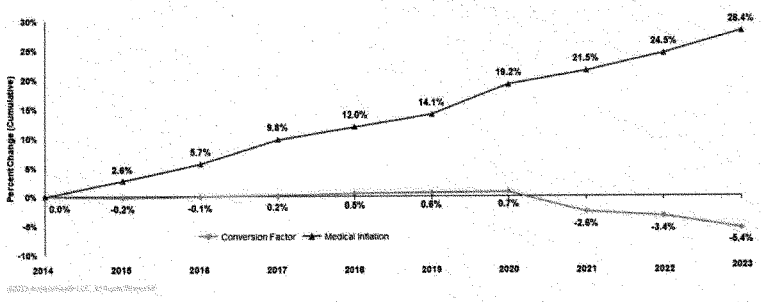
CMS & Medicare Regulations

Hospital Pressures and the 340B Program

I previously touched on pressures from large consolidating health systems, which have been allowed to grow due to a lack of regulation and oversight. Let me just touch on the difference in payment structures and the 340B Program.

There have been more studies and analyses of how hospitals charge more than independent physician-run medical practices, including charging what is called a hospital "facility fee." Simply put, patients pay more in hospitals. That is in large part because Medicare, which is typically the single largest payer in cancer treatment, pays hospitals more for identical procedures than independent community oncology practices like ours. Additionally, Medicare annually provides inflation adjustments to hospitals but does not to physician-run practices. In fact, as this graph shows, as the cumulative inflation rate has increased over the past 10 years, Medicare payments to physicians have been flat and recently declining.

Figure 1. Cumulative Change in Conversion Factor and Medical Inflation Since 2014, by Year



Source: US Bureau of Labor Statistics, "Consumer Price Index Historical Tables for US City Average, 2023"; US Bureau of Labor Statistics, "BLS Data Viewer, 2023"; American Medical Association, "History of Medicare Conversion Factors, 2023."

It is increasingly impossible for independent practices, especially those operating as small businesses, to survive given how CMS implements Medicare payments.

Additionally, CMS has fueled hospital consolidation and acquisition of medical practices, especially in oncology, by grossly over-paying hospitals for deeply discounted 340B drugs. Hospitals with 340B drug discounts not only put independent practices at a disadvantage but are also a prime motivating factor to acquire those practices.

Barriers to Patients Getting Their Drugs Delivered & the Stark Law

What is further harming independent medical practices, especially those like ours treating sick cancer patients, is a relatively new CMS regulation that popped up during the COVID public health emergency (PHE). CMS has ruled that when the PHE expired, it is a Stark Law violation for practices like ours to deliver an oral cancer drug to a patient or even to have a patient's family member or caregiver pick up the drug at our practice for the patient. This presents serious treatment obstacles for patients who are too sick, without regular transportation, and simply unable to appear in person for their oral drugs. The Stark Law was put in place close to 35 years ago to make it a crime for physicians to refer "designated health services" payable by Medicare (and Medicaid) to entities that the physician (or family member) has a financial interest in, with certain exceptions. The current CMS regulation is simply wrong because delivering a drug to a patient, or allowing a patient representative to pick it up, involves no referral to anything I have a financial interest in, yet it puts a real barrier to my patients getting their drugs.

I add that the Stark Law in this day and age is not only archaic but places physician-run practices at a serious disadvantage to hospitals, which can refer to themselves in any manner, regardless of whether it is a clinical or financial detriment to the patient.

Barriers to Patient Efficient Care

Certain CMS regulations compel practices to operate in highly inefficient manners, often lacking discernable logic for physicians and patients alike. For example, a Medicare regulation – referred to as modifier-25 because of the Medicare coding involved – deems it unreasonable for a physician to administer both a cancer drug infusion (such as chemotherapy) or injection and also conduct a professional visit with the patient on the same day. Consider the scenario of a patient with anemia stemming from chronic kidney disease, whose condition typically responds favorably to erythroid stimulating agents administered via subcutaneous injection every two to three weeks. Concurrently, physicians must monitor this therapy, ensuring proper response, adjusting dosage as necessary, and observing for adverse effects such as exacerbation of high blood pressure. While it would be advantageous for patients to receive both the physician visit and the injection on the same day, Medicare does not reimburse for both services concurrently.

As a result of this non-sensical CMS regulation, patient caregivers often need to take additional time off work to accommodate separate appointments for their elderly parents, compounding the burden on families. This issue extends to other treatment areas, including those aimed at enhancing the immune system to combat cancer. The outcome is that sick cancer patients are compelled to make multiple unnecessary visits to their physician's office, adding strain and inconvenience to an already challenging situation.

Previously, Medicare and commercial insurers, following CMS' lead, would accept a "modifier-25" adjustment to billing codes, allowing for treatments and medical evaluations on

the same day. However, recent CMS regulations have become significantly more stringent. As a result, our office had to educate physicians, nursing staff, and clerical personnel to adjust appointments to circumvent this conflict, leading to understandable confusion among staff and patients alike. This situation epitomizes the deleterious impact of misguided regulations, needlessly complicating treatment for cancer patients.

Conclusion

In all of this, physicians are increasingly burdened by onerous paperwork, reporting, and computer data reporting. This takes time away from what we are not only trained to do but take an oath to do – put our patients first in providing them with the highest quality medical treatment. Rather than having regulations and laws to help us do that, it is the other way around. Small healthcare "businesses "are going extinct because the regulatory environment is stacked against them. I could go on and on with additional examples but will be happy to answer any questions, or elaborate on what testimony I have provided, from the committee.

I appreciate the opportunity to testify today.

David Eagle, MD
Practicing Oncologist & Chair of Legislative Affairs and Patient Advocacy
New York Cancer & Blood Specialists

Bill Newell
CEO Sutro Biopharma
House Small Business Committee
Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare
May 8, 2024

Chairman Williams, Ranking Member Velázquez, thank you for the opportunity to appear before this Committee to discuss the drug development ecosystem and the challenges small biotechnology companies face. My name is Bill Newell and I am the CEO of Sutro Biopharma, Inc. I have been at Sutro since January 2009 and working in small company biotech since 1998. I also serve on the Board of the Biotechnology Innovation Organization and chair BIO's Capital Formation Work Group.

The Sutro Story

Sutro Biopharma focuses on research & development and manufacturing for next generation cancer medicines, primarily antibody-drug conjugates (ADCs). Our company is 21 years old, founded in 2003 with patent-protected technology licensed from Stanford University. I was employee number 19 and today we have over 300 employees and a market cap of approximately $300 million. Like many U.S.-based biotechs, Sutro has seen substantial domestic job creation, with about 40% of our work force in or supporting our US-based cGMP manufacturing facility. We built and operate the world's only manufacturing facility utilizing cell-free protein synthesis technology at scale and producing clinical trial materials for Sutro and our partners. We have begun two pivotal Phase 3 trials for our most advanced therapeutic candidate luveltamab tazevibulin – for patients with late-stage ovarian cancer and patients with an ultra-rare pediatric acute myeloid leukemia (AML) – with read outs not expected for a few more years. We have multiple additional potential medicines in research and early clinical development.

In many ways, Sutro's corporate journey is a microcosm of the small biotech experience. We were initially financed by private investors, including venture capitalists and big pharma/biotech venture investors. We raised Series A through E venture rounds totaling approximately $190 million. We went public in 2018, benefiting from the JOBS Act of 2012 that made it easier for small companies to go public. So far, we have raised approximately $535 million in public market offerings. In addition, collaborations with larger industry players have been essential to our growth. We have received approximately $720 million in funding and reimbursements for R&D collaborations and/or licensing of product candidates from large and midsized biopharma companies. In addition, at various points in time, we have borrowed from venture lenders. I am proud to say that we are debt-free as of earlier this year. All told, Sutro has raised almost $1.6 billion in the company's history, and we are still several years from the possibility of a commercial product. That eyebrow-raising figure and our over 20-year company journey is, unfortunately, very typical of the small biotech experience in bringing a product to market.

Also, like many biotechs, we have had our share of failures along the way. Three potential medicines have made it to the clinic, but development was halted by us or our partners as they did not meet criteria for continued advancement. This is not unusual in our industry; only approximately 12% of products reaching clinical development stage are ever approved and just half of products reaching Phase 3 (pivotal trial stage) ever get FDA approval.

Given these high costs and low success rates, small biotech companies and their investors are particularly sensitive to the U.S. policy environment in which we operate. Ensuring a robust domestic biotechnology industry is rightfully recognized as a critical national security issue. In addition, it is also an economic juggernaut, with high growth potential and high wages across the country. Thus, it is critical that we

implement and support policies that encourage our development and reexamine policies that deter investment and delay treatments. Accordingly, I'd like to devote the rest of my testimony to outlining some of the critical policy factors impacting the biotech ecosystem and what policies should be considered for our industry to survive.

Access to Capital

It is a truism that capital is the lifeblood of small biotechs. In our continuing quest for sufficient investment to fund our mission, federal policies that encourage investment and capital formation are essential. Congress should:

- Restore the R&D deduction is extremely important for companies like mine that are being hit with a multi-million dollar tax liability as a result of the switch to 5-year amortization, even though we have no product on the market. Our tax liability reduces our funds that were, and should be, going to research and development to bring new medicines to patients.
- Right-size SEC reporting requirements for small public companies would save small companies millions in reporting costs that provide information our investors don't want or need.
- Reauthorize the SBIR/STTR government grant programs would help the very early-stage companies and provides a critical lifeline that should be reauthorized and expanded.
- Allow for pre-revenue companies with less than 500 employees to monetize their Net Operating Losses (NOLs) today to provide a much-needed cash infusion by forgoing this existing tax benefit in the future.

In the ongoing search for investment, policies like these can make the difference between a promising company succeeding in bringing a new medicine to patients or running out of runway and ending research and development on potential new medicines.

Importance of Strong Protections for Intellectual Property

Very few sectors of the nation's economy are as dependent on predictable, enforceable patent rights as the biotechnology industry. Robust patents that cannot be easily circumvented or invalidated, and that can be predictably enforced against infringers, enable biotechnology companies to secure the enormous financial resources needed to advance biotechnology products to the marketplace. Further, they allow biotechs to engage in the partnering and technology transfer that is necessary to translate basic scientific discoveries into real-world solutions that treat disease, address climate and other environmental challenges, and produce abundant, healthy food for the world. These financing pathways include venture financing, IPOs, follow-on offerings, and licensing partnerships, and are all predicated on the existence of stable and enforceable intellectual property rights. Anyone who has ever watched Shark Tank knows that without a dependable patent system, capital for the cures of the future will not be available.

These financing pathways have been critical to the success of our companies like Sutro. Without strong and reliable patents, we would not have been able to secure the investments or partnerships over the years as we seek to prove the safety and efficacy of our leading therapeutic drugs. If patents can be invalidated under overly broad criteria, if the ability to enforce them becomes limited, or if limits on patent eligibility call into question the ability to obtain patent protection for innovative cures, third parties would be less likely to invest in or license the technology and major sources of R&D funding would move elsewhere. The result – patients waiting for the next new cure or treatment will have to wait longer or may not ever get it at all. Because investment-intensive businesses can tolerate only so much risk, even moderate additional uncertainty can cause business decisions to tip against developing a high-risk, but potentially highly beneficial, therapeutic medicine.

Unfortunately, changes to our patent laws through legislation, agency actions, and court decisions, have severely weakened our patent system. Although the U.S. patent system was once considered the gold standard for the rest of the world, in the latest global survey conducted by the U.S. Chamber of Commerce, our patent system was rated behind Singapore, Japan, and South Korea.[1]

There are several reasons why the US patent system is no longer the international gold standard. As the Chamber of Commerce report notes, "the patenting environment in the United States continues to be held back by uncertainty over what constitutes patent-eligible subject matter and patent nullity proceedings through the inter parties review, which occurs before the specialized Patent Trial and Appeals Board within the U.S. Patent and Trademark Office (PTO). Since the Supreme Court decisions in the Bilski, Myriad, Mayo, and Alice cases, there has been a high and sustained level of uncertainty about which inventions are patentable in the United States."[2] These continuing threats merit the attention of Congress.

It has become clear that the PTO's Inter Partes Review (IPR) system of administrative patent challenges in the Patent Trial and Appeal Board (PTAB) is having a game-changing effect on the reliability of patents as a basis of investment in the biotechnology industry. Patents that are involved in district court litigation are now routinely subjected to concurrent administrative litigation in the PTO, where they are being invalidated at rates so high that the basic procedural fairness of these proceedings is increasingly being questioned. This creates a great risk of duplicative proceedings and inconsistent outcomes, as alleged infringers seek to gain advantages or leverage over patent owners that would not exist under district court litigation alone. For example, the way claims are interpreted, and other procedural protections are less favorable to patent owners in the PTO administrative setting.

In addition, third parties with no commercial interest in the patent or field to which the patent pertains have figured out that they can extort settlements or otherwise gain financially from bringing, or even threatening to bring, patent challenges against critical patents owned or licensed by biotech companies. Biotech companies can be particularly vulnerable to such extortion because – in contrast to most high-tech companies – biotech companies often rely on just a handful of highly valuable patents to protect their products and massive investment therein.

Such abuses of the PTO administrative review system are attractive and growing because, as is quite clear to those following the evidence to date, the rules governing these proceedings are unfairly stacked against patent owners in many ways. In particular, the PTO uses a claim construction standard that is much broader than that used in district court and has limited the ability of patent owners to file narrowing amendments to preserve their patent claims.

Bipartisan legislation to address the most glaring problems with the PTAB system has been introduced in both Chambers: the "Promoting and Respecting Economically Vital American Innovation Leadership Act" or the "PREVAIL Act" introduced by Senators Chris Coons (D-DE) and Thom Tills (R-NC) and Representatives Ken Buck (R-CO) and Deborah Ross (D-NC).[3]

Continued uncertainty remains with respect to patent eligible material under section 101 of the Patent Act.[4] It is important that our patent system keeps pace with advancements in science. Our foreign competitors in Europe and China now extend patent protection to significant innovations that are not

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[1] US Chamber of Commerce, Global Innovation Policy Center, International IP Indelx, 12th ed. at 45 (2024).
[2] Id. at 46.
[3] S. 2220/H.R. 4370.
[4] 35 USC §101.

patent eligible in this country. That places us at an unacceptable competitive disadvantage in emerging technologies.[5] It is legislation like the PREVAIL Act, and others such as S. 2140, the Patent Eligibility Restoration Act of 2023, introduced by Senators Tills and Coons, that would bring predictability to our patent system and place U.S. innovators such as Sutro on an even footing with our competitors around the world.

I am also concerned about calls to expropriate patents. The Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights[6] would reverse more than 40 years of successful federal policy under administrations of both parties by using the Bayh-Dole Act as a price control mechanism. The threat of government seizure of patents would place a cloud over every patent developed with federal funding. It would ensure that federally funded research remained on the shelf rather than attracting startups prepared to make the substantial investments to further invent and develop life changing therapeutics.

Modernize the FDA to Ensure a Predictable Regulatory Environment

Industry routinely invests in innovative tools and approaches to develop better medicines that meet the needs of patients, such as decentralized clinical trials, model-informed drug development, drug development tools, complex innovative trial design, patient-focused drug development, and leveraging real-world data. In general, there remains a degree of hesitation among drug developers regarding the adoption of such innovative approaches for clinical trials which is largely attributed to a lack of clarity, consistency and dedicated Agency resources. While the FDA encourages industry to explore new methodologies, such as new study designs and approaches, industry still finds these methodologies risky to implement due to continued uncertainty regarding the regulatory outcome. For the industry to effectively design and conduct innovative clinical trials, the FDA's timely, substantive, and interactive scientific input is needed to help reduce regulatory uncertainty.

Preserve and Protect the Accelerated Approval Program

The FDA's traditional approval pathway measures how drugs improve patients' symptoms, functioning, or survival. But it can take years to verify this "clinical benefit." For patients whose disease can't wait that long, the Accelerated Approval Program evaluates drugs based on "surrogate endpoints" or "intermediate clinical endpoints" likely to predict clinical benefit. These endpoints might include X-rays, reduction in tumor size, or blood tests. Policy discussions about the evaluation of the Accelerated Approval Program cannot be complete without assessing its impact on its most important target outcome: patient survival. A recent analysis shows[7] that that from 2006-2022, more than 900,000 patients with cancer gained approximately 263,000 life years as a result of earlier access to medicines through accelerated approval. For this reason, the Accelerated Approval Program needs to be protected from detractors who question its benefit.

Unfortunately, in the past few years the FDA has tightened, to the point of constricting, the utilization of the Accelerated Approval Program. For example, small companies with new cancer medicines previously

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[5] Adam Mossoff, Kevin Madigan, Turning Gold to Lead: How Patent Eligibility Doctrine Is Undermining U.S. Leadership in Innovation, 24 Geo. Mason L.Rev. 939 (2017).
[6] 88 FR 85593 (Dec. 8, 2023).

[7] *From <https://jnccn.org/view/journals/jnccn/aop/article-10.6004-jnccn.2024.7010/article-10.6004-jnccn.2024.7010.xml?sp_sn=linkedin&spclid=128E9C41-D359-4165-BC81-BFBC1C18BB60&ArticleBodyColorStyles=abstract%20%2F%20extract>*

could seek to take advantage of the Accelerated Approval Program by first pursuing a single arm pivotal trial in one hundred or so late-stage cancer patients using surrogate end points and then further demonstrating patient benefit with a much larger confirmatory trial to demonstrate prolonged disease control and ultimately a survival benefit. Achieving a successful outcome and accelerated approval, the small company would raise additional funding and start a much larger (hundreds of patients) confirmatory trial. The data underpinning the accelerated approval provides the confidence to the company and its investors to make the substantial additional investment in the confirmatory trial.

Recently, the FDA has required that companies have completed or substantially completed enrollment in their confirmatory trial by the time the FDA takes action on a request for accelerated approval. Sutro has recently been held to this newly changed regulatory standard. This forces companies like Sutro to commit additional resources (hundred of millions of dollars) much earlier than would have been previously required. This additional accelerated expense can prove extremely challenging for small companies and threatens the promise of rapidly bringing new life-extending medicines to patients who desperately need them.

User Fees in PDUFA VII

The biotechnology industry supports the FDA's ability to pursue its mission through user fees. Indeed, industry now pays approximately 75% of FDA's drug review program costs. Of great concern, in 2023, FDA increased user fees by 25%, meaning that a New Drug Application or Biologics License Application is $4 million. To continue to develop innovative new medicines for the American people, the biotechnology industry needs user fees that are predictable, stable and affordable.

Reauthorize the Pediatric Priority Review Voucher Program

The Pediatric Priority Review Voucher (PPRV) program provides critical incentives to promote R&D for drugs to treat rare diseases impacting children across the country. Rare diseases, by definition, impact a small percentage of the patient population. The costs of drug development paired with the risk involved of bringing a successful drug to market can often discourage investment in the rare disease space. This is especially true for rare diseases unique to children.[8] The current program expires Sept. 30, 2024, and Sutro and our industry partners strongly support the *Creating Hope Reauthorization Act of 2024*. This bipartisan bill would reauthorize the Pediatric Rare Disease Priority Voucher Program. As I testified earlier today, Sutro is pursuing a pivotal trial for an ultra-rare pediatric acute myeloid leukemia (AML). This type of AML affects globally approximately 50 infants and toddlers a year. While our medicine shows encouraging efficacy in this very aggressive form of AML, as well as good tolerability, and while the pivotal study is for a small number of these children, the economics of pursuing this indication would clearly dictate that we not do so. The key economic rationale for us to pursue this ultra-rare indication for regulatory approval is the value to Sutro of a Pediatric Priority Review Voucher. It is cases like this one that make it imperative that this program be reauthorized.

Fix the Disincentives in the Inflation Reduction Act

The Inflation Reduction Act (IRA) authorizes the Secretary of Health and Human Services to "negotiate" the price Medicare pays for certain medicines. With stiff penalties for drug companies that don't comply, there is little room for negotiation and so, it is simply a price control. The IRA's impact drives companies to make difficult choices on therapy class, population size, which indications to pursue first and whether to invest in new indications. Companies and investors now factor in more limited economics in their decision making, not the science. I believe that fewer medicines will be developed as a result.

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[8] https://bio.news/federal-policy/reauthorize-pprv-pediatric-rare-disease-priority-review-voucher-vaccine-safety-systems/

Continue Investment in Rare Disease Drug Development and Pass the ORPHAN Cures Act
As discussed, developing new drugs is an incredibly risky and capital-intensive endeavor-- only 12% of drugs entering clinical trials ultimately receive FDA approval.[9] [10] [11] Now consider rare diseases-- which in some cases afflict just a few hundred people.[12] [13] Such a small patient population makes it extraordinarily difficult for biotech companies to justify the massive R&D costs required to develop a new treatment.

Congress sought to help alleviate the lack of investment in rare diseases in 1983 when it passed the Orphan Drug Act. The law gives tax credits to companies who develop novel rare disease treatments, also known as "orphan drugs."[14] The legislation has been a resounding success-- FDA-approved treatments for rare conditions have increased over 2,000% since its passage.[15] In fact, Sutro has received orphan drug designation, as well as rare pediatric disease designation for our treatment of this ultra-rare AML.

But instead of building on the successes of the Orphan Drug Act, the Inflation Reduction Act (IRA) punishes companies trying to find novel treatments for rare diseases. The law permits the federal government to impose severe price caps on prescription drugs covered under Medicare. The IRA exempted orphan drugs from the price controls if they treat a *single* rare disease.[16] But medicines that treat *multiple* rare diseases don't qualify for the exemption.

This limited exception from negotiation is a significant problem. Drug makers routinely investigate whether a drug already approved to treat one rare condition could possibly treat another.[17] Historically, this "follow-on" research has provided transformational cures to patient groups who don't have access to effective treatments. The IRA is already forcing some drug companies to freeze efforts to find additional applications for existing rare disease drugs.[18] In short, the IRA's negative treatment of orphan products is a direct contradiction of the positive, and life-changing, work done by Congress in passing the Orphan Drug Act itself many years ago.

Ensure Critical Research for Small Molecule Drugs
Certain areas of research will feel the devastating impact of the IRA even more than others because IRA price controls apply differently to different kinds of medicine. "Small molecule" (oral) drugs can be subject to price controls just nine years after earning FDA approval. By contrast, biologics – typically infused or injected medicines – are not subject to price controls for 13 years.

Most pharmaceuticals on the market today, including at least 89 anti-tumor drugs for treating cancer, are small-molecule drugs. But the new IRA rules disincentivize research into this critical area of medicine. Consider that much research on oncology medicines happens after they earn FDA approval and patient

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[9] https://www.m2gen.com/company-news/industry-insights/how-long-do-new-cancer-drug-therapies-take-to-go-to-market#:~:text=On%20average%2C%20it%20takes%2010,the%20Study%20of%20Drug%20Development

[10] https://www.bio.org/sites/default/files/legacy/bioorg/docs/Clinical%20Development%20Success%20Rates%202006-2015%20-%20BIO,%20Biomedtracker,%20Amplion%202016.pdf p. 3

[11] https://www.cbo.gov/publication/57126#:~:text=Only%20about%2012%20percent%20of,for%20introduction%20by%20the%20FDA.

[12] https://rarediseases.org/wp-content/uploads/2019/01/RDD-FAQ-2019.pdf p. 1

[13] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4543882/

[14] https://oig.hhs.gov/oei/reports/oei-09-00-00380.pdf p. 1

[15] https://pkdcure.org/saving-the-orphan-drug-tax-credit/ (MATH: 650-30/30 = 20.667 x 100 = 2067%)

[16] https://www.kff.org/medicare/issue-brief/explaining-the-prescription-drug-provisions-in-the-inflation-reduction-act/

[17] https://www.cahc.net/newsroom/2023/3/1/how-the-inflation-reduction-act-is-impacting-rare-disease-patients#:~:text=In%20the%20rare,in%20later%20years.

[18] https://www.bloomberg.com/news/articles/2022-10-27/alnylam-halts-work-on-eye-drug--citing-new-us-law-over-pricing?sref=C4viNJ4s

benefit in one indication has been established. That's when scientists can perform additional tests to discover new uses for a drug, sometimes over the course of a decade or more. It's not uncommon for a medicine developed to treat one cancer to prove highly effective at treating other forms of the disease. But the threat of near-term price controls makes companies much less likely to invest in additional post-approval research.

Given the choice between a nine-year and a 13-year window until price controls kick in, many will choose to prioritize research and development efforts on biologics. An industry colleague who leads a small private biotech company working on small molecule medicines for the most important undrugged targets across human disease told me that he is feeling the discriminatory effects of this "pill penalty". He shared with me that venture investors, who previously funded companies like his, have pulled back from funding such early stage small molecule discovery platforms citing the IRA. But by disincentivizing work on small-molecule drugs, the IRA is robbing patients of life-changing new treatments. Cancer isn't the only research area that will suffer. For example, for neurological diseases like Alzheimer's or the disease that took my mother's life Progressive Supranuclear Palsy, small-molecule medicines may offer some of our best prospects for breakthroughs.

There seems to be a misguided notion that small molecules are cheaper to develop and less risky. This is not the case – the calculus for funding their development is essentially the similar as that for biologics and they merit the same treatment. Extremely effective and cutting-edge medicines exist in both classes – and both avenues of development are complex, expensive, and fraught with failure. One is not "better" than the other. And of course, such distinctions mean little to patients, who just want the best medicine available. Congress should protect innovation and patient access to needed medicines by revising the new rules for Medicare "negotiation" and apply the same 13-year window to both small-molecule drugs and biologics.

Congress should pass two bipartisan pieces of legislation that would mitigate these market distortions.

- The Ensuring Pathways to Innovative Cures (EPIC) Act (H.R. 7174), sponsored by Reps. Greg Murphy, M.D. (R-NC) and Don Davis (D-NC) would fix the small molecule "pill penalty" to ensure continued R&D investments into small molecule medicines.
- The Maintaining Investments in New Innovation (MINI) Act (H.R. 5547/S.476), sponsored by Reps. Wiley Nickel (D-NC) and John Joyce, M.D. (R-PA), and Senators Bob Menendez (D-NJ) and Marsha Blackburn (R-TN), would extend the negotiation of genetically targeted therapies (small complex molecules) to 13 years.

IRA Implementation

Beyond the problems inherent in the IRA statute itself is CMS's implementation of the IRA. Congress should increase its oversight of the Centers for Medicare & Medicaid Services (CMS) as the Agency moves forward in implementing the IRA's price negotiation program. Unfortunately, a critical policy that CMS finalized was its decision that, in determining which drugs are eligible for negotiation, it would not treat drugs approved under unique New Drug Applications (NDAs) or Biologics License Applications (BLAs) as distinct drugs but, rather, would combine NDAs and BLAs with the same active moiety/active ingredient together for negotiation purposes. CMS must reverse this policy as it is bad for innovation, bad for patients, and not supported by the statute. CMS's approach leaves no incentive for therapeutic advancement in additional indications and will have significant, negative impacts on treatments for patients for decades.

CMS must also clarify how its review of the evidence will inform its setting of the maximum fair price ("MFP") for a drug selected for negotiation. CMS's approach remains unclear and presents untenable levels of uncertainty. Essentially, CMS has said it will use the net price of the "therapeutic alternatives" of drugs selected for negotiation as a starting point and then adjust this starting point based on its review of the clinical evidence. In addition, CMS has said it may make further adjustments based on other data manufacturers are required to submit, such as "recoupment" of research and development costs. But CMS has not provided a framework for how it will review all this evidence. Nor has the agency indicated how certain evidence or factors will be weighed. This lack of clarity and uncertainty is of great concern. CMS should clarify its standards for evidence review and be transparent and accountable about what evidence drove its decisions in setting the MFP and why. Further, CMS's review of the evidence should focus on factors that are critical for patients, specifically factors related to clinical benefit and unmet medical need and de-emphasize manufacturer specific data elements such as cost of production and research and development costs.

Other CMS Policies Impacting Small Biotech Companies

Last year, under the guise of "technical changes", CMS proposed to upend more than 30 years of historical precedent under the Medicaid Drug Rebate Program (MDRP). CMS's proposed changes present significant access threats to a vulnerable group of patients and are without any solid legal grounding.

Of particular concern is the proposed rule's new definition of "best price." Current law defines this as the lowest or "best" price available to any entity in the drug supply chain, be it a wholesaler, insurer, nonprofit, or government entity. The proposed rule would fundamentally change how this best price is determined-- and in a way that makes it vastly more difficult for small- and medium-sized firms like ours to serve Medicaid patients. Specifically, the proposed rule mandates that companies aggregate or "stack" any discounts or rebates provided to various entities who encounter the drug unit in the drug supply chain to calculate the best price. This task is not only daunting but is impossible to implement.

In addition to operational impediments, the rule's overall cost to our companies would be significant and could make ongoing participation untenable. It could thus dramatically reduce the number of drugs available to vulnerable patients and seniors. In so doing, it further could create perverse incentives, decreasing the potential that companies would offer rebates beyond the statutory minimum Medicaid Drug Rebate for fear of not being able to track such discounts and report them accurately under the new rule. This could lead to further market consolidation and higher ultimate costs for entities like providers and hospitals.

By increasing both the costs and risks involved in serving underprivileged patients through the Medicaid Rebate Program, the rule would discourage investment in medicines from which these vulnerable populations are most likely to benefit. This decreased investment will affect not only small and mid-size companies but also companies that are seeking to bring their first product to market, as the increased liabilities and uncertainties introduced by these changes will make ongoing investment in treatments for the Medicaid population untenable. The result would be less innovation, fewer new cures, and worse health outcomes for disadvantaged groups.

The policies I laid out are of the greatest impact to emerging biotech companies like Sutro, but there are more to consider as the Federal Government seeks to promote and protect the biotechnology industry's complex ecosystem. Thank you for inviting me to testify today. I look forward to your questions.

Testimony of Dr. Diana Zuckerman, President of the National Center for Health Research

House of Representatives Small Business Committee Hearing

Stifling Innovation:

Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare

May 8, 2024

Chairman Williams, Ranking Member Velazquez, and distinguished members of the Committee on Small Business:

I am Dr. Diana Zuckerman and I am president of the National Center for Health Research, a public health think tank in Washington, D.C. I was trained as a post-doctoral fellow in epidemiology and public health at Yale Medical School, was on the faculty of Vassar and Yale, and a research director at Harvard before coming to Washington as a Congressional Science Fellow. I then spent a decade working as a Congressional investigator on FDA issues at what is now the House Committee on Oversight and Accountability, was in charge of health legislation at the Senate Veterans Affairs Committee, and also served in the White House Office of Science and Technology Policy. While in my current position for the last 25 years, I've served on the Medicare Evidence Development and Coverage Advisory Committee (MEDCAC); been a fellow at the University of Pennsylvania Center for Biomedical Ethics; chaired the Women's Health Promotion Council for the State of Maryland; and trained physicians, patient advocates, and journalists to understand medical research findings as well as FDA and CMS policies.

Thank you for the opportunity to be here today to share my expertise and the views of the National Center for Health Research. Our research center does not accept funding from companies or other entities with a financial interest in our work.

As a scientist, a policy expert, and a cancer survivor, I look for common ground. I respect the very important work of the other panel members and this Committee, and I think we can all agree that we want small businesses to succeed and to provide the best possible products. In medicine and health care, let's agree that we want innovation that is defined as better products and better treatments that have meaningful benefits to patients – living longer, spending less time in hospitals, feeling healthier, and having a better quality of life. It isn't enough for products to be new, they should be better for at least some patients.

The Costs of FDA Flexibility in Drug Approval of a Small Business

The FDA makes it very clear that it does not expect or require absolute certainty when it approves a drug or medical device. Here are examples of evidence that the FDA has considered adequate for approval for two very expensive drugs created by start-up businesses. After that, I will briefly explain FDA regulatory policies and how they differ for drugs and devices.

This March, a company named Amylyx reported that its one and only drug – Relyvrio-- did not work. FDA had granted approval to Relyvrio to treat ALS, also known as Lou Gehrig's disease less than 2 years earlier, despite the company ignoring the FDA's advice to complete their clinical trial before requesting approval, and despite the warnings by FDA scientists and FDA Advisory Committee members that the evidence was not persuasive and therefore it might not work. FDA approved it anyway because ALS is a terrible disease and the agency wanted to be flexible since the drug might possibly work. The drug cost $158,000 per patient per year despite its much less expensive ingredients (one is a dietary supplement sold on Amazon for a few dollars). Since the drug was intended to slow deterioration rather than improve health, it was not immediately obvious to patients that the drug was not working – that required comparing the drug to placebo. The company had promised to continue its research comparing the drug to placebo and found that the patients taking the drug were no better than the placebo group. Meanwhile, the company had $380 million in revenues last year and the two young men who had started the company paid themselves $7.4 million each. When Relyvrio was taken off the market, the stock immediately dropped 80%, and 70% of the staff were let go.

Another example is the small business Sarepta, which also had only one product when it submitted an application for approval for Exondys51 for Duchenne Muscular Dystrophy. The company had only 12 boys in their study and started giving the drug to the placebo group because they were so sure it was working. Despite the extremely small study, unclear evidence, and no placebo group, the FDA granted accelerated approval in 2016 because Duchenne muscular dystrophy is a deadly disease and as a small business Sarepta did not have the capital to continue the research unless they could start selling the drug.[1] The company promised a larger study would be completed in 2020. It is now 2024, the larger study has not yet been submitted, and the price of the drug has increased from about $400,000 per patient per year to over $1 million per patient year. Families went broke even paying the co-pays, so Medicaid has footed most of the bill. And we still don't know if it works.

Are FDA Requirements Too Stringent?

There are many large companies that have also benefited from FDA "flexibility" and therefore had drugs or devices approved that were not proven to be safe and effective and were later proven to not be safe or not be effective. For example, our research on 18 unproven cancer drugs that had been approved based on short-term preliminary data such as tumor shrinkage, found that 4-8 years later there was still no evidence for 17 of the drugs that patients lived longer or had a better quality of life.[2] A study of hundreds of new drugs that FDA approved but required to do post-market studies to confirm that they were safe and effective found that more than two-thirds of those required studies were late, especially those for treatments for children for various treatments.[3] These and similar studies indicate that the problems with confirming that new drugs are safe and effective are not unique to small companies but these problems can't be solved by lowering regulatory standards.

The examples of Amylyx and Sarepta are important for this Committee because they show that the FDA is sometimes very helpful to small businesses, ignoring their own written policies. I will briefly describe what those written policies are.

For drugs, the FDA usually requires at least one clinical trial that shows that the drug has benefits that outweigh the risks compared to placebo. FDA does not require the new product to be better than older products that are already on the market, even if those older products are much less expensive. And for numerous products, FDA doesn't even require that the new product has meaningful health benefits, and instead only asks that there is evidence that the new drug probably will have health benefits. In other words, FDA doesn't require absolute certainty or even a high level of certainty, but rather a subjective judgment that there is a reasonable probability of benefit.

For medical devices, the standards are even less rigorous: the FDA standard is a "reasonable assurance of safety" and a "reasonable assurance of effectiveness." Approximately 98% of new devices are cleared for market without any clinical trials and without any clear evidence of safety or effectiveness, as long as the FDA considers the new device to be "substantially equivalent" to a device legally on the market. And the agency's definition of substantial equivalence does not require the new device – even an implanted life-saving device – to be made of the same materials, be the same shape, or have the same mechanism of action. For example, the robotic surgery systems that are widely used today were allowed on the market as substantially equivalent to traditional scalpels and other surgical tools, even though the robotic systems are in other ways very different from those tools.

What about the other 2% of medical devices – the highest risk devices that require clinical trials? High risk devices that can either save a life or cause a death -- such as an artificial heart -- are only required to submit one clinical trial (rather than 2 that are traditionally required for drugs) and these studies are very rarely randomized double-blind clinical trials, even though that is considered the gold standard for testing the benefits of medical products. And yet, a recent study of a national random sample of physicians found that most believed that FDA approval decisions should be based on 2 randomized double blind clinical trials and many did not realize that the FDA often did not require that evidence.[4]

FDA Has Reduced Regulatory Burdens on Small Businesses

Given the implications for the health of all of us when we are patients, and for the health of our friends and loved ones, I am comfortable with the FDA being careful not to unduly burden small businesses, but I want to be able to trust that the medical products – drugs and devices – made by small businesses are just as safe and effective as those made by the largest companies. If they aren't, any benefits to a small business will be short-lived, as they were with Relyvrio.

There are some specific FDA policies that help reduce regulatory burdens on small businesses. User fee legislation is negotiated by FDA and industry every 5 years and then passed by

Congress. The negotiations are behind closed doors but reportedly focus on the fees that companies must pay the FDA when they submit applications to the FDA average timeline for FDA reviews. In addition, user fee legislation has an important benefit for small businesses – it requires the FDA to regularly meet with industry staff throughout the application process to answer the applicants' questions and help ensure that the applicant has the information necessary for a successful application. Such meetings are required regardless of the size of the company, and that helps to level the playing field for smaller companies and start-ups that otherwise would not have access to that level of specific advice about their application materials.

User fees also provide lower fees and waivers for small businesses. Device companies must pay a registration fee of $7,653, but the FDA waives all user fees for small businesses with revenues below $30 million when they submit their first medical device application. Equally important, the user fee for most device applications submitted by businesses with revenues below $100 million only cost $5,800 this year, since that is the price that medical device companies negotiated with the FDA and Congress passed in MDUFA, the medical device user fee legislation. Here are FDA's device user fees for different types of applications in FY 2024 taken directly from www.FDA.gov:

Application Type	**Standard Fee**	**Small Business Fee†**
510(k)‡	$21,760	$5,440
513(g)	$6,528	$3,264
PMA, PDP, PMR, BLA	$483,560	$120,890
De Novo Classification Request	$145,068	$36,267
Panel-track Supplement	$386,848	$96,712
180-Day Supplement	$72,534	$18,134
Real-Time Supplement	$33,849	$8,462
BLA Efficacy Supplement	$483,560	$120,890
30-Day Notice	$7,737	$3,869
Annual Fee for Periodic Reporting on a Class III device (PMAs,PDPs, and PMRs)	$16,925	$4,231

Drug user fees are much higher because applications are much more complex and require more staff resources. However, FDA allows waivers for small businesses, defined as "an entity that

has fewer than 500 employees, including employees of affiliates, and that does not have a drug product that has been approved under a human drug application and introduced or delivered for introduction into interstate commerce." Similarly, FDA may grant a fee waiver for applications meeting small business applications for biologics.

Conclusions

Good evidence for medical products requires resources. Small businesses will have the resources to meet the FDA's evidence standards for many types of medical products, but if we want these products to be safe and effective enough to help patients, some small businesses will not be able to raise the capital to provide the kind of evidence that patients and health professionals need to make informed decisions. That is the reason why small businesses often partner with larger companies on novel medical products. However, when the FDA reduces the burden on companies to provide clear evidence that a product is safe and effective, that increases the burden on patients and physicians to make life-changing and life-saving medical decisions without the facts they need to make the decisions that are best for them.

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Hearing: "Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare"

Questions for the Record from Representative Nydia Velázquez, Ranking Member

Dr. Diana Zuckerman, President, National Center for Health Research • Dr. Zuckerman, in answering the question from Ms. Scholten, Dr. Miller said the issue that FDA reviewers have is the overwhelming workload they need to complete that leaves them unable to operationalize new guidance that can streamline the clinical trial process for small firms. He further recommends that FDA have more front-line staff to help operationalize the guidance. Should Congress provide the FDA with additional resources to take the burden off of the reviewers?

I agree that the FDA needs more front-line staff to review applications and to help operationalize guidance. However, too often FDA ignores its own excellent guidance to industry about what the agency's expectations are for the types of evidence required for approval. Indeed, FDA clearly states that the agency guidance documents are merely recommendations and are not enforced. Does it make sense for the agency to spend so much time developing guidance, asking for public comment, revising their guidance documents in response to public comments, and then ignoring the guidance by approving medical products that don't meet the standards set out in the guidance? While many small businesses applaud FDA "flexibility" in its approach to evidence, that flexibility has the potential to result in biased decisions, especially favoring companies that have stronger relationships with FDA decision-making staff, which could negatively impact small businesses. In addition, I challenge Mr. Miller's claim that the FDA has too many managers; FDA regulates products comprising 20 cents of every dollar spent by U.S. consumers, and that requires managers as well as reviewers. What is the evidence that the managers are not performing essential functions? If there is such evidence, I would encourage Congress to address that issue.

- Dr. Zuckerman, the FDA recently issued guidance on decentralizing clinical trials. In your view is this a positive development for speeding up clinical trials and advancing innovation?

Decentralizing clinical trials is an ideal bipartisan issue because it facilitates clinical trial participation by patients who otherwise would not have access to free treatments. Those include patients who live in rural areas, those who live far from the major medical centers that often recruit patients for clinical trials, and patients who lack the resources or transportation to travel to the sites where most clinical trials take place. Ideally, decentralization would enable patients who are more representative of the patient population to participate – more diverse in terms of geography, rural/urban areas, age, sex, medical status, race, ethnicity, and income. This is a win-win situation because it expands the availability of potentially effective new treatments as well as "standard of care" treatment while also providing essential information about who is most likely to benefit. It doesn't just expand access to experimental treatments because most studies of new experimental treatments compare the new treatment to a "control" group that will receive the currently available approved treatments and standard of care for free. When decentralization

enables patients to be examined and tested remotely throughout most of the clinical trial, that would enable people who are too ill to travel, live too far away, lack affordable transportation, or are responsible for caring for children or other family members to participate. However, it would require that the patients have access to adequate technology and testing needed for remote participation in clinical trials, so that the physicians and researchers are able to carefully monitor the patients' health and collect accurate data at all stages of the study. This could be accomplished by arranging for patients who lack such technology to have broadband access and to be examined by physicians and other healthcare professionals at local doctors' offices, urgent care clinics, and community hospitals. Researchers could also arrange for patients lacking broadband access to have occasional access to broadband technology at nearby schools and other public buildings.

- Dr. Zuckerman, what improvements could be made to the FDA process that help speed innovative products to market without compromising scientific evidence?

The pendulum for FDA approval has swung too far, going from the excessively high standards required 30+ years ago to standards that are too subjective and opaque. Under the current approval process, some companies are unable to bring their products to market because of research shortcomings, while others are given FDA approval based more on wishful thinking and lobbying than on scientific evidence. Since user fees fund most FDA staff at the Center for Drug Evaluation and Research (CDER) primarily to speed up pre-market reviews, there is almost no funding available for FDA staff to monitor the safety and effectiveness of new drugs after they have been approved. As a result, it is too often left to independent researchers at major medical centers to conduct essential research that reveals whether some of the most expensive treatments for cancer and rare diseases actually work and have benefits that outweigh the risks. For example, academic researchers at Brigham and Women's Hospital and Harvard Medical School recently reported that most cancer drugs that went through the accelerated approval pathway did not demonstrate benefit in overall survival or quality of life within 5 years of accelerated approval.[1] Our Center's previous research on cancer drugs approved through various FDA pathways found similarly alarming results.[2]

Although the Center for Devices and Radiologic Health (CDHR) is less dependent on user fees, they too have very limited resources to monitor the safety or effectiveness of medical implants and other devices that are already on the market. That is especially dangerous, because our research shows that the rush to get new medical devices on the market quickly has resulted in most medical devices being cleared for market without any evidence of safety or effectiveness.[3] In addition, research indicates that medical devices that have been allowed on the market as "substantially equivalent" to devices that have been recalled are more likely to also be recalled due to deaths or irreparable harm, but the FDA has ignored years of recommendations to require more evidence of safety and effectiveness, especially for implanted devices.[3,4,5,6]

- Dr. Zuckerman, the FDA recently proposed new regulations on laboratory developed tests. Some claim that this will shift more market power to larger companies who can bear the costs of research evidence and user fees. Can you explain why this regulation is necessary, and some of the pitfalls of allowing the industry to go unregulated?

I agree with the Food and Drug Administration's (FDA) conclusion that it has the authority to regulate all in vitro diagnostic tests, including laboratory-developed tests (LDTs), under the Federal Food, Drug, and Cosmetic Act (FD&C Act). The final FDA rule on LDTs is essential, because many patients are experiencing significant harm due to inaccurate LDTs. While undue regulation is by definition not appropriate, all patients must be able to trust the accuracy of diagnostic tests. The new FDA rule, similar to the wording in the VALID Act that Congress considered, prioritizes regulations to ensure the accuracy of higher-risk diagnostic testing.

According to the Centers for Disease Control and Prevention, 70% of today's medical decisions rely on laboratory test results.[7] Inaccurate LDTs can lead to false-positive test results where patients falsely believe they have a serious medical condition, and false-negative results may cause a patient's life-threatening condition to be missed. For example, one investigation of prenatal genetic tests that were intended to screen for rare diseases found that positive results were wrong 80%-93% of the time.[8] Further, an FDA report included specific examples of LDTs that could harm large numbers of patients because they falsely diagnose Lyme disease and ovarian cancer for people who do not have it, falsely telling people with HPV or a type of high risk breast cancer that they do not have those conditions, or are just generally inaccurate in diagnosing fibromyalgia or the risk of heart disease.[9] Research also shows that LDTs have erroneously provided inaccurate test results that lead to harmful decisions for cancer patients regarding chemotherapy treatments. For example, one study found that the number of false positives for a recently marketed LDT intended to diagnose cancer, known as the Galleri blood test, was higher than the number of true positives.[10] Unfortunately, this is not a rare occurrence. In fact, at the May 22, 2024, House Energy and Commerce Committee Hearing, Dr. Jeff Shuren, Director of the FDA's Center for Devices and Radiological Health, testified that a cancer patient's ability to get the right treatment is determined more by the laboratory that conducts the diagnostic testing rather than the tumor biology.

Failure to adequately regulate LDTs also has had a negative impact on the economy, resulting in wasted spending on unnecessary or harmful medical treatments, lawsuits, lost wages, and other costs. These examples are just a few examples illustrating the financial benefits of appropriate regulation of lab developed tests.

FDA plans to use the same risk-based enforcement approach for diagnostic tests made by labs that the agency currently uses for diagnostic tests made by companies. When FDA's rule begins to phase in effective July 5, 2024, it will not target or unduly burden small businesses. Instead, the rule simply classifies LDTs as medical devices and regulates them the same way as diagnostic tests made by companies; in spite of their potential for saving lives or harming them, medical devices are held to a much less rigorous standard than prescription drugs.

Despite numerous efforts for several years, Congress has not passed legislation that would establish a regulatory framework for lab developed tests and the resources needed to implement it. Due to the urgent need for patients to be able to rely on the accuracy of all diagnostic tests, patients are grateful that the FDA has decided to move forward rather than waiting for Congress. However, FDA must have the adequate resources needed to ensure that LDTs are appropriately reviewed and regulated, and to educate industry, including small businesses, about the new requirements mandated by this rule. Additionally, resources are essential for FDA to hire

additional staff so that the scientific reviews are conducted in a timely manner by well-trained scientific staff at the FDA, rather than relying on third party entities that are paid by the companies and therefore have an inherent conflict of interest.

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U.S. House of Representatives
Committee on Small Business

"Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare"

May 8, 2024

Statement for the Record
American Academy of Dermatology Association

Chairman Williams and Ranking Member Velazquez, on behalf of the American Academy of Dermatology Association (Academy) and its more than 17,000 U.S. members, thank you for the opportunity to submit a statement for the record for the Committee's hearing entitled *"Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare"*

The Academy is committed to excellence in the medical and surgical treatment of skin disease; advocating for high standards in clinical practice, education, and research in dermatology and dermatopathology; and driving continuous improvement in patient care and outcomes while reducing the burden of skin disease. As of 2023, 57% of dermatologists reported working in a dermatologist-owned practice, and 17% work as solo practitioners. Burdensome regulatory policies in the health care space can be damaging to small physician practices.

The Academy applauds the Committee for its efforts to examine policies that not only detrimentally impact small physician practices, but limit patients' ability to receive innovative and timely treatments. In dermatology, drugs and other therapies are frequently delayed or denied due to unnecessary prior authorization and step therapy policies. These

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overburdensome policies require tedious amounts of time to complete, often requiring multiple staff to oversee, taking time away from the practices' ability to focus on patient care.

As you explore ways to reduce regulatory burdens on small practices, one critical aspect that needs immediate attention is the instability of the Medicare physician payment system and the need for reform. The AADA firmly believes that Congress must take action to advance Medicare physician payment reform by:

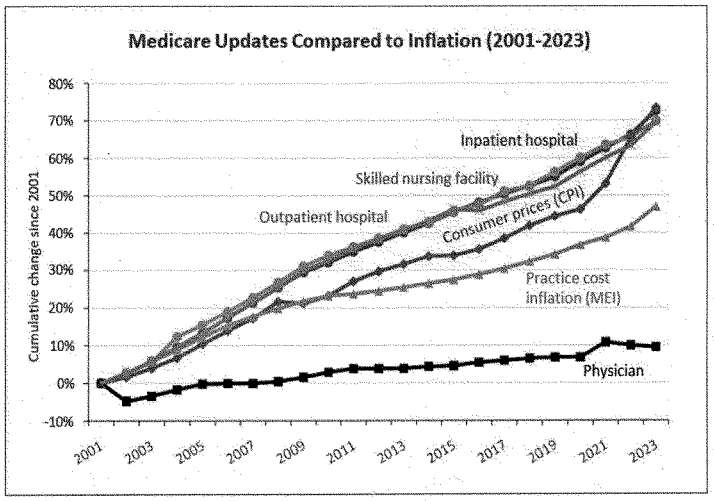
- Establishing a positive annual inflation adjustment.
- Increasing the budget neutrality threshold, supporting a lookback period to rectify errors associated with utilization assumptions, and allowing specific services to be excluded from budget neutrality requirements.
- Reforming the Quality Payment Program (QPP) to increase physician input and improve patient care without overly burdensome documentation and compliance activity.

In addition to these reforms, it's important to emphasize that Americans should have access to affordable, high-quality dermatologic care with the freedom to choose their own physicians and health insurance that best meets their needs. The Medicare program must ensure beneficiaries have adequate access to networks of specialists and subspecialists, including board-certified dermatologists. This goal can only be possible when health care policy is driven by the welfare of patients over short-sighted and siloed budgetary policies that increase overall health care spending and further erode the stability and predictability of the Medicare system.

Inflation and the Siloed Medicare Program Structure

The failure of the Medicare Physician Fee Schedule (MPFS) to keep up with inflation is the greatest threat to maintaining seniors' timely access to care in physician offices. Hospitals and other healthcare facilities receive Medicare payment updates, but physicians receiving payments under the MPFS are excluded from this type of adjustment. In fact, CMS finalized a 3.4% cut in the Calendar Year (CY) 2024 MPFS final rule. While the AADA appreciates the partial relief Congress provided to the MPFS in the Consolidated Appropriations Act, 2024, physician payments still ultimately received a cut from 2023.

Since 2001, the cost of operating a medical practice has increased 47%. During this time, Medicare hospital and nursing facility updates resulted in a roughly 70% increase in payments to these entities, significantly outpacing physician reimbursement. *Adjusted for inflation in practice costs, Medicare physician reimbursement declined 30% from 2001 to 2024.* This out-of-balance payment structure disproportionately threatens the viability of medical practices, especially smaller, independent, physician-owned practices, as well as those serving low-income or historically marginalized patients. This issue is further exacerbated by rising costs and inflation, leading to increased consolidation and hospital ownership of physician practices, resulting in higher expenses and reduced competition.



Sources: Federal Register, Medicare Trustees' Reports, Bureau of Labor Statistics, Congressional Budget Office

Congress and CMS need to re-examine the siloed approach to reimbursement tied to the Medicare program. According to the 2020 and 2021 Medicare Trustees' report, MPFS spending per enrollee was $2,107 in 2011 and $2,389 in 2021, growing at an average annual rate of 1.3%. However, in contrast, Medicare spending per enrollee in Part A fee-for-service (FFS) was $5,178 in 2011 and $5,576 in 2021 – a 7.7% increase and more than double the cost per patient treated under the MPFS.

In considering the failure of the MPFS to keep up with the rising costs of delivering medical care, it is important to remember that physicians rely on reimbursement to cover a multitude of practice expenses. These expenses include staff salaries, benefits, federal and state regulatory compliance costs, and expenses associated with insurance mandates, such as step therapy and prior authorization. Moreover, technology requirements associated with compliance of the QPP are costly and contribute to the financial strain placed on physician offices.

Physician practices are often small businesses that contribute to the economy of their communities. Other industries can adjust their products' pricing to reflect rising costs and increased staff salaries. However, physicians do not have the ability to do this. In fact, in the face of crippling inflation the MPFS serves to destabilize practices with year-after-year cuts. Such a structure is unsustainable, and we must not expect physicians delivering essential medical care to Medicare beneficiaries and their communities to endure it. Many physicians have already had to close their doors, leave their communities, retire early, or leave the practice of medicine. The below chart demonstrates the staggering numbers of physicians

leaving the workforce, and this trend will continue as nearly 45% of physicians are older than age 55. The loss of experienced physicians is detrimental to patient outcomes and the young physicians who rely on them as a learning resource.[1]

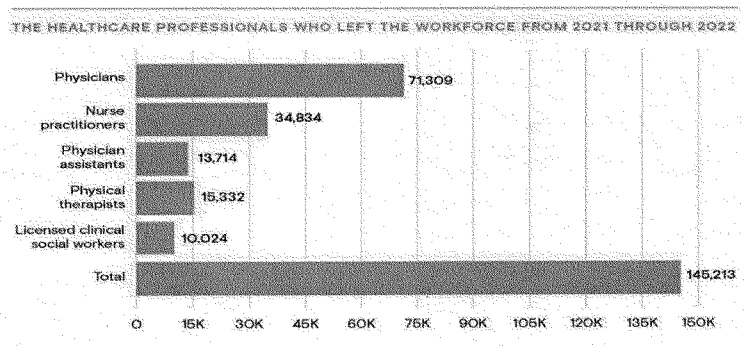


Fig. 1 Analysis of data from Definitive Healthcare's Atlas All-Payor Claims and PhysicianView products. Data sourced from a stable panel of billing organizations from Q1 2021 through Q1 2023. Physicians deemed as dropped out practiced in 2021 and ceased activity by Q4 of 2022. Some providers may still be practicing, but not filing claims. Data accessed September 2023.

The inability to provide inflationary pay raises to practice employees is contributing to the current healthcare workforce crisis in which we are seeing increasing burnout rates and a mass exodus of our clinical, administrative, and clerical staff into other industries. With reduced staff comes a diminished capacity to provide quality care and maintain patient access. Reduced staffing leads to barriers in communicating and coordinating care, such as scheduling appointments and discussing lab reports, which can impact patient satisfaction and outcomes.

The threat of future additional cuts to Medicare physician reimbursement jeopardizes physicians' ability to keep the doors open and care for patients in our communities. Fewer physicians in our communities means longer wait times for patients to receive care. When those patients do receive care, their only option may be non-physician providers of care with less training, or more expensive care in sub optimal settings including emergency departments and hospital-based practices. This is real, not theoretical, and is already occurring in our communities. Medicare patients will suffer in the end with delayed and second-rate care at a higher cost.

Physicians need positive, inflation-based reimbursement updates to maintain financial stability and ensure patients have continued access to care. Inflationary updates tied to the Medicare Economic Index (MEI) need to be based on current data. In fact, the Medicare Payment Advisory Commission (MedPAC) recommended that Congress tie physician payment updates to the Medicare Economic Index (MEI) or

[1] https://www.definitivehc.com/sites/default/files/resources/pdfs/Addressing-the-healthcare-staffing-shortage-2023.pdf

practice cost inflation rates for 2025.[2] Specifically, MedPAC recommended that Congress update the 2024 Medicare base payment rate for physician and other health professional services by the amount specified in current law plus 50% of the projected increase in the MEI. Based on CMS's MEI projections at the time of the publication of the March 2024 MedPAC Report to Congress, the recommended update for 2025 would be equivalent to 1.3% above current law.

The AADA appreciates MedPAC's acknowledgment that the current Medicare physician payment system has not kept up with the cost of practicing medicine. While we value this recognition, Congress should adopt a 2025 Medicare payment update that fully acknowledges the inflationary growth of health care costs. This step is crucial for ensuring financial stability in the Medicare physician payment system and maintaining continued access to high-quality patient care.

The AADA urges Congress to pass H.R. 2474, the Strengthening Medicare for Patients and Providers Act, which would provide an inflationary update to the conversion factor under the Medicare physician fee schedule based on the Medicare economic index.

Budget Neutrality

Downward pressure on Medicare reimbursement is due to budget neutrality requirements, and has thus resulted in a decline of 30% in reimbursement since 2001. The Medicare statute requires that changes made to fee schedule payments be implemented in a budget-neutral manner.

Furthermore, by law, CMS must also create utilization assumptions for newly introduced services. When an overestimation occurs, it remains uncorrectable, leading to irreversible reductions in the funding allocated to the Medicare physician payment pool. For example, in 2013, transitional care management services were added to the MPFS. While CMS estimated 5.6 million new claims, actual utilization was under 300,000 for the first year and less than a million claims after three years. This overestimation led to a $5.2 billion reduction in Medicare physician payments from 2013 to 2021. This example highlights the unintended consequences of the current budget policies within the flawed system. We firmly believe that CMS should have the authority to rectify utilization assumption errors that impact budget neutrality.

In the absence of eliminating budget neutrality policy, we encourage Congress to pass H.R. 6371, the Provider Reimbursement Stability Act, to revise the budget neutrality policies to: (a) prevent erroneous utilization estimates from leading to inappropriate cuts; (b) clarify the types of services subject to budget neutrality adjustments; and (c) update the projected expenditure threshold triggering the budget neutrality adjustment, which has remained unchanged since 1992.

Reform Quality Payment Program

Value-Based Models

Current value-based programs are burdensome, have not demonstrated improved care, and are not clinically relevant to the physician or the patient, and we have serious concerns with the viability and effectiveness of the Merit-based Incentive Payment System (MIPS) program. Numerous studies have

[2] https://www.medpac.gov/document/march-2024-report-to-the-congress-medicare-payment-policy/

highlighted persistent challenges associated with MIPS, including practices serving high-risk patients and those that are small or in rural areas. A study titled "Evaluation of the Merit-Based Incentive Payment System and Surgeons Caring for Patients at High Social Risk," examined whether MIPS disproportionately penalized surgeons who care for patients at high social risk. This study found a connection between caring for high social risk patients, lower MIPS scores, and a higher likelihood of facing negative payment adjustments.[3]

Additionally, the Government Accountability Office (GAO) was tasked with reviewing several aspects concerning small and rural practices in relation to Medicare payment incentive programs, including MIPS. The GAO's findings indicated that physician practices with 15 or fewer providers, whether located in rural or non-rural areas, had a higher likelihood of receiving negative payment adjustments in Medicare incentive programs compared to larger practices.[4]

These studies highlight flaws in traditional MIPS, particularly in terms of potential disparities in care and the financial burdens placed on physicians when caring for high-risk patient populations and physicians in small practices. **The AADA recommends that Congress establish incentives, funding, and flexibility for physician offices with targeting small and solo practices.**

MIPS Value Pathways

Since the passage of the Medicare Access and CHIP Reauthorization Act of 2015 (MACRA), CMS routinely introduces new changes to MIPS, requiring physicians to adjust continuously. Physicians are increasingly frustrated by the frequent modifications to the Quality Payment Program (QPP), including the associated administrative burdens of adhering to new program requirements and the lack of incentive payments to adequately compensate for participation efforts. While the AADA acknowledges CMS' attempt to address some of these concerns by introducing MIPS Value Pathways (MVPs) aimed at creating more meaningful groups of measures and activities to offer a more comprehensive assessment of quality of care, this new reporting option is falling short of achieving the Agency's goal.

The AADA has significant concerns with the Agency's approach to constructing MVPs, as it is using excessively broad measure sets that lack alignment and provide no added benefit in terms of enhancing patient care or helping patients determine the value of the clinician managing their care. CMS' approach fails to account for the realities of clinical practice and adds yet another layer of complexity to an already confusing program. Take for example, CMS' candidate MVP for Dermatological Care. Despite nearly two years of discussions and meetings between CMS and the AADA, CMS continues to express interest in the use of a single MVP for dermatology. This decision ignores the critical problem of a one-size-fits-all approach, as it cannot effectively compare costs and quality of care. We have shared with CMS that each subspecialty within dermatology provides unique services to distinct patient populations with varying practice patterns. This diversity in the practice of dermatology makes a one-size-fits-all model ineffective

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[3] Byrd JN, Chung KC. Evaluation of the Merit-Based Incentive Payment System and Surgeons Caring for Patients at High Social Risk. JAMA Surg. 2021;156(11):1018-1024. doi:10.1001/jamasurg.2021.3746.

[4] Medicare Small and Rural Practices' Experiences in Previous Programs and Expected Performance in the Merit-Based Incentive Payment System Report to Congressional Requesters United States Government Accountability Office.; 2018. https://www.gao.gov/assets/gao-18-428.pdf.

for comparing the cost and quality of care. For instance, dermatologists who treat psoriasis, which is currently considered in the candidate MVP's quality measures may not treat melanoma, which is currently the only measure related to cost available in the candidate MVP. Regardless of how CMS ultimately scores MVP participants, if CMS finalizes an MVP that includes a cost measure for a cancer-related disease and quality measures for an inflammatory skin disease, patients and clinicians will question its purpose and the extent to which it fails to drive value-based care.

Due to these numerous concerns, the AADA calls on Congress to urge CMS pause on moving forward with the MVPs. The AADA welcomes the opportunity to continue working with CMS and the Congress to identify opportunities to improve quality, patient outcomes, and efficiencies.

Burden on Physician Practices

Furthermore, the QPP must keep a keen focus on preventing physician and staff burnout based on the Department of Health and Human Services (HHS)[5] own priorities. This includes providing relief from systems-level factors that contribute to healthcare worker burnout by instituting measures that:

- Implement systems changes that reduce administrative paperwork overall.
- Facilitate coordination at the systems level without adding administrative burden to healthcare practices and healthcare workers.
- Provide funds to purchase human-centered technology that facilitates providing value-based care; and
- Ensure engagement in value-based care does not lead to additional workload, overhead, and work hours for specialists.

Conclusion

On behalf of the AADA and its member dermatologists, thank you for holding this hearing, allowing the opportunity for stakeholders to submit a statement for the record, and for your commitment to ensuring physicians can continue to serve their Medicare patients. The AADA looks forward to working with you and asks that you continue to consider including physician stakeholders' opinions in your ongoing hearings. Should you have any questions, please contact Jennifer Mangone at jmangone@aad.org.

[5] https://www.hhs.gov/sites/default/files/health-worker-wellbeing-advisory.pdf

AAFP
AMERICAN ACADEMY OF FAMILY PHYSICIANS

May 8, 2024

The Honorable Roger Williams
Chairman
Committee on Small Business
U.S. House of Representatives
2361 Rayburn House Office Building
Washington, DC 20515

The Honorable Nydia Velázquez
Ranking Member
Committee on Small Business
U.S. House of Representatives
2069 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Williams and Ranking Member Velázquez:

On behalf of the American Academy of Family Physicians (AAFP), representing more than 130,000 family physicians and medical students across the country, I write to thank you both for your bipartisan leadership in addressing issues impacting family physicians and their patients through today's hearing entitled "Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare."

Administrative functions and regulatory compliance overburden family physicians at the point of care and after patient care hours, making it one of the principal factors fueling health care consolidation and forcing many primary care practices to either sell or close their doors altogether. These functions include activities such as electronic health record (EHR) documentation, submitting claims to get paid, reporting on quality and performance measures, and navigating prior authorization and step therapy requirements. Studies have estimated that primary care physicians spend nearly 50% of their time on cumbersome administrative tasks.[i] Many practices have to hire dedicated staff to submit claims or prior authorization requests. Administrative burden is piling up, while physician payment is failing to keep pace with inflation. These trends are taking physicians' time away from providing quality care to patients and putting financial strain on primary care practices.

Increasingly, family physicians report that independent practice is simply unsustainable. Data confirms that physician employment is growing and physician practice acquisitions have accelerated in recent years, including by health systems, payers, and corporate entities such as private equity. A 2017 study found that from 2010 to 2016, the share of primary care physicians working in organizations owned by a hospital or health care system increased by a dramatic 57% — while the shares in independent solo practice or organizations owned by a medical group decreased.[ii]

The volume of administrative tasks imposed on physicians is exacerbating physician burnout and is the most immediate threat to the future of small primary care practices and their ability to deliver high-quality, timely patient care. It is with this in mind that the AAFP offers the following feedback and recommendations to Congress to help preserve the viability of independent

practice and relieve family physicians from the never-ending avalanche of administrative and regulatory burden.

Getting Paid for Primary Care is Unnecessarily Complex

A 2009 study found that physician practices collectively spend about $30 billion a year alone on administrative costs related to billing and coding.[iii] One can assume that, when adjusted for inflation today, that number is significantly higher. To get paid, physicians must submit unique codes for each and every service they provide – documenting both what they did and why they did it. This is incompatible with the continuous, comprehensive nature of primary care which spans everything from basic preventive services to more complex services involving chronic care management, integrated behavioral health, and care coordination.

Every billing code has its own accompanying rules (some associated with the code set(s) and others created by Medicare and other payers) that govern when they may be reported either independently or in conjunction with other codes. This is true in almost any fee-for-service payment system, whether traditional Medicare, Medicare Advantage, or commercial insurance. Some research has concluded that creating additional billing codes for distinct activities in the MPFS may not be an effective strategy for supporting primary care, due to the burden associated with billing each one.[iv]

The retrospective, volume-based nature of FFS also fails to account for the costs of longitudinally managing patients' overall health. It does not provide practices with the time and flexibility to invest in the care management staff and population health tools that enable practices to efficiently and effectively meet patients' individual evolving health needs.

For these reasons, **the AAFP has long advocated to accelerate the transition to value-based care using alternative payment models (APMs) that provide prospective, population-based payments to support the provision of comprehensive, longitudinal primary care.** We strongly believe well-designed APMs provide primary care a path out of the under-valued and overly burdensome FFS payment system that exists today, and in turn will better enable the Medicare program to meet the needs of its growing and aging beneficiary population in new and innovative ways. Unfortunately, a dearth of primary care APMs and the inadequacy of FFS payment rates that often underlie APMs are undermining the transition to value-based care. Because most APMs are designed based on FFS payment rates, modernizing FFS payment for primary care is one essential strategy to support physicians' transition into value-based care.

Therefore, the Academy continues to urge Congress to consider legislative solutions, including reforms to the Medicare Access and CHIP Reauthorization Act (MACRA), that would address unsustainable FFS payment rates for physicians and alleviate some of the associated administrative burden for practices, while promoting patients' access to continuous, comprehensive primary care. This includes proposals such as providing an annual inflationary update for Medicare physician payment to give small practices a fighting chance at keeping their doors open and reforming existing budget neutrality requirements that hinder CMS' ability to appropriately pay for all the services a beneficiary needs.

Finally, federal policymakers should provide more opportunities for primary care practices to participate in APMs that provide upfront or advanced payments and other supports to enable the investments required to be successful in value-based payment. While value-based payment does not eliminate the administrative burden associated with coding and billing *entirely*, **prospective, population-based payments provide practices with the resources and flexibility needed to handle administrative functions more efficiently while delivering and investing in high-quality, patient-centered care**.

Quality and Performance Measurement

Quality and performance measurement has proliferated in the past 25 years, leading to significant burdens on physicians. This is especially true for primary care physicians, who are disproportionately accountable for a growing number of disease-specific process measures that fail to capture the true nature and value of comprehensive, patient-centered primary care.

While quality measurement is essential to moving toward a value-based health care system, our current approach fails to measure what matters to patients and clinicians or drive meaningful quality improvement. The eagerness to measure has burdened family physicians with the onerous task of capturing structured electronic data to feed an excessive number of measures, taken time away from patients, and led to loss of joy in practice. Quality measurement has become a high-burden, high-cost administrative exercise, focused on financial concerns with little benefit to patient care, population health, and cost reduction.

We must standardize quality and performance measures with a single universal set – across payers and programs – that meets the highest standards of validity and reliability and is derived from data extracted from multiple data sources. The measures should focus on outcomes that matter most to patients and that have the greatest overall impact on better health of the population, better health care, and lower costs. Right now, it is a logistical nightmare to try and meet all of the different quality measures across plans. On average, family medicine practices contract with about ten different payers. Keeping track of and successfully reporting different measures for each of these payers creates confusion and additional reporting burden and can actually undermine meaningful practice improvements. Aligning measures across payers will also help to identify disparities in care quality (and, in some cases, utilization and access) across different payers, states, and lines of service. Greater alignment will also drive improvements in data collection automation, which will reduce reporting burden on family physicians and other clinicians.

Importantly, measures must reflect things which a physician can control instead of penalizing them for the things they can't. For example, there is a code available for physicians to bill to indicate that they offered the patient a vaccine but they refused to take it. However, the measures only reflect that the patient chose not to get a recommended vaccine - the fact that the physician offered it has no impact. Performance measurement should focus on improving outcomes that matter most to patients and have the greatest impact on improving the health of the population, creating a better experience of care, and lowering the per capita cost of care, while also returning joy to the practice of caregiving for physicians and other clinicians.

Medicare's Quality Payment Program

The Quality Payment Program (QPP), implemented as part of the passage of MACRA in 2015, have been a significant source of burden for practices, particularly small practices. MACRA was intended to serve as an on-ramp to value-based payment by giving physicians experience with being measured on their performance and quality. While the AAFP supported the intent of MACRA, it has not led to quality improvement and has also not achieved its original goal to streamline Medicare's existing quality programs and simplify reporting requirements.

There is broad consensus that the QPP has increased administrative burden and complexity as its requirements change year after year. While all programs should be flexible and make improvements, the QPP has primarily changed the requirements without making improvements or reducing burden. For example, one qualitative study found that the average per-physician cost to participate in QPP's Merit-based Incentive Payment System (MIPS) was $12,811, and physicians and staff together spent 201.7 hours annually per physician on MIPS activities.[v] The costs were higher for small and medium primary care practices ($18,466 and $13,631, respectively). Importantly, this study *only* analyzed the time and financial costs for participating in MIPS. **Previous studies have found that practices spend an average of 785.2 hours $40,069 per physician per year on quality reporting requirements.**

Since there is a dearth of APMs and the MIPS requirements do not closely align with any existing APM, MIPS is primarily a reporting program with arbitrary requirements that do not meaningfully contribute to improved patient outcomes. **The significant burden associated with these programs forces practices to direct their time and resources on complying with reporting requirements rather than building the skills and infrastructure that would allow them to succeed in value-based payment.**

In addition, MIPS must be budget neutral – meaning the total value of annual positive adjustments are equal to the total value of negative adjustments. This has led to many practices who met their performance requirements getting a negative adjustment, and for those that receive a positive one, it is very modest. Therefore, MIPS adds administrative burden without leading to a meaningful increase in payment. The program particularly disadvantages small and rural practices, who consistently have lower than average MIPS scores. As the performance threshold increases, it will become more difficult for small and rural practices to avoid a negative payment adjustment, which can be up to 9% to their Medicare Part B services.

The inflexibility of the MACRA statute has created significant barriers to implementation of reforms aimed at moving physicians from payment on volume to value. Health care markets, value-based care models, and other factors can change quickly and additional flexibility is needed to ensure programs keep pace with these changes without awaiting congressional intervention. **For all these reasons, the AAFP continues to Congress advance MIPS and QPP reforms to alleviate the administrative costs of reporting to the program, ensure it drives meaningful quality improvement, and assist physician practices in building the necessary competencies to transition into APMs.** Specific recommendations include:

- **Granting CMS the authority to provide credit across multiple performance categories**. MIPS uses four siloed performance categories – all with different measures

and reporting requirements. Despite multiple calls for consolidation and cross-category credit, CMS argues that they do not have the statutory authority to alter the program in that regard. One significant step toward reducing burden would be to give CMS the flexibility to provide cross-category credit. For example, a physician who reports a quality measure related to depression screening should automatically receive credit for the corresponding improvement activity.

- **Allowing practices to attest to using certified electronic health record technology (CEHRT) in place of reporting on Promoting Interoperability measures**. The AAFP has advocated for practices to be able to attest to their use of CEHRT rather than requiring multiple burdensome measures, but CMS does not have the authority to offer such an option. Years of policy changes to the legacy Meaningful Use program and now the promoting interoperability category have failed to move the needle on health information exchange. It is beyond time to move away from such burdensome requirements – doing so would be an important step toward reducing the burden of the MIPS program.
- **Providing CMS with the authority to modify the qualifying participant threshold through rulemaking to ensure advanced APM participation is attainable.** Existing thresholds set in federal statute are creating barriers for physician practices seeking to move into more advanced models. Providing CMS with the authority to modify the thresholds will help ensure the QPP is facilitating the transition to APMs instead of preventing it.
- **Providing technical assistance, shared learning collaboratives, and data infrastructure to support all primary care practices to transition to APMs.** Primary care's information needs are particularly complex which requires technical capabilities and a reliance on others to fill information gaps, including payers and other provider organizations. Often, IT departments may be non-existent or staffed by non-IT personnel, posing challenges when implementing new or updated hardware or software, connecting to regional health information exchanges (HIEs), and setting up registries. Additionally, building and understanding reports from an EHR is time-consuming, burdensome, and can be costly if there is a need for custom reports. Safety nets also face additional reporting burden on top of payer reports due to other reporting requirements based on their funding streams (grants, Uniform Data System, etc.).
- **Funding technical assistance programs to support overall adoption of APMs by all practices in all settings**. MACRA provided funding to support small practices with direct assistance through tools and resources to help them navigate the complex MIPS reporting requirements. In response, CMS created the QPP Small, Underserved, and Rural Support (QPP SURS) program which provided small practices in rural and health professional shortage areas with technical assistance at no cost to them. Unfortunately funding for the QPP SURS expired in February 2022 and has not been renewed.

Prior Authorization

Prior authorization (PA) is the process by which physicians must obtain advanced approval from a health plan before delivering a procedure, device, supply, or medication for insurance to cover that service's cost. Health plans – including Medicare Advantage (MA) and Medicaid managed care organizations (MCOs) – that use utilization management processes, such as prior

authorization, frequently describe them as a cost-control mechanism. However, repeated evidence has shown that many MCOs use prior authorization inappropriately, causing care delays and worsening patient outcomes and satisfaction. A 2022 report from the Department of Health and Human Services (HHS) Office of Inspector General (OIG) confirmed that MA plans sometimes deny prior authorization and payment requests that meet Medicare coverage rules by using clinical criteria not in Medicare coverage rules and requesting unnecessary documentation, as well as making errors.[vi]

In addition to enrollees in MA plans, enrollees in other health plans needing care for their own chronic illness,[vii] their children's chronic illness,[viii] and rare diseases[ix] have experienced barriers to care from prior authorization requirements. In 2022, California-based L.A. Care, which administers Medicaid and other types of coverage, failed to address a backlog of more than 9,000 prior authorization requests and more than 67,000 complaints or appeals.[x] Meanwhile, an OIG report published in July 2023 found that Medicaid MCOs denied one out of every eight prior authorization requests in 2019. Approximately 2.7 million Medicaid beneficiaries were enrolled in MCOs with prior authorization denial rates greater than 25%.[xi] However, minimal data collection on and oversight of these practices is being done by state Medicaid agencies. This is largely because current federal rules do not require states to collect and monitor data needed to assess access to care, monitor the clinical appropriateness of denials, or require that states publicly report information on plan denials and appeals outcomes.

In an American Medical Association (AMA) survey of physicians, 94% reported that prior authorization delays access to care, while 80% reported that it led to patients abandoning their treatment and 33% reported that it had led to a serious adverse event for their patient.[xii] Additionally, 86% of surveyed physicians reported that prior authorization sometimes, always, or often leads to higher overall utilization of health care resources, such as additional office visits, emergency department visits, or hospitalizations.

In March, the Medicaid and CHIP Payment and Access Commission (MACPAC) convened to discuss denials and appeals within Medicaid managed care. In their research, they noted the lack of federal requirements for collecting key data as described above. They also identified some of the challenges and barriers impeding the ability for individuals to pursue appeals in Medicaid; for example, MCOs are required to mail denial notices, but beneficiaries do not always receive these denial notices in time to pursue an appeal within the allotted time frames. In light of these findings, MACPAC put forward seven recommendations to improve the appeals and denials process for individuals enrolled in Medicaid:

- States should be required to establish an independent, external medical review process that can be accessed at the beneficiary's choice;
- CMS should issue guidance to improve the clarity and content of denial notices and clarify how Medicaid funding may be used to support external entities, such as ombudsperson services;
- MCOs should be required to provide beneficiaries with the option to receive electronic denial notices in addition to mailed notices;
- CMS should extend the timeline for beneficiaries to request continuation of benefits and issue guidance to improve beneficiary awareness of their rights to continue receiving services while an appeal is pending;

- CMS should require states collect and report data on denials, use of continuation of benefits, and appeals outcomes, and use the data to improve delivery of care to patients;
- States should be required to conduct routine clinical appropriateness audits of managed care denials and ensure access to medically necessary care; and
- CMS should publicly post all state Managed Care Program Annual Reports and require states to include denials and appeals data on their quality rating system websites to ensure beneficiaries can access this information when selecting a plan.

The AAFP strongly urges Congress to act upon these MACPAC recommendations to improve the denials and appeals processes for Medicaid beneficiaries and ensure patients have timely access to medically necessary care as recommended by their physician.

Additionally, we also applauded CMS for finalizing a regulation earlier this year that will streamline prior authorization processes, implement electronic prior authorization, and improve transparency across all of its payers, including Medicare Advantage and Medicaid managed care, as well as address inappropriate coverage denials. However, we continue to advocate for the passage of legislation to enshrine these necessary reforms into statute. Specifically, **the Academy continues to push for reintroduction and passage of the Improving Seniors' Timely Access to Care Act**, which passed the House last Congress and would codify many of the regulatory provisions by requiring implementation of an electric prior authorization program in MA and streamlining and standardizing of PA processes.

Additionally, **Congress should advance other legislation to reign in prior authorization across all Medicare plans**. Specifically, the AAFP supports the Getting Over Lengthy Delays in Care as Required by Doctors (GOLD CARD) Act (H.R.4968), which exempts qualifying physicians from prior authorization requirements under Medicare Advantage plans. Physicians would qualify if at least 90% of their prior PA requests were approved in the preceding twelve months. This approval, referred to as a "gold card," would remain in effect for a year. Further, we have endorsed the Reducing Medically Unnecessary Delays in Care Act (H.R. 5213), which would require all PAs be made by a licensed physician who is board certified in the specialty relevant to the item or service requested. It would also require plans to create policies based on medical necessity and written clinical criteria.

Step Therapy and Prescription Drug Formularies

Step therapy is another utilization management protocol used by insurers, which requires patients to try one or more insurer-preferred medications prior to the medication their physician prescribed. This practice can take weeks or months and can result in patients not being able to access the treatments they need in a timely manner. Physicians can request exceptions to step therapy requirements, but insurers may not respond promptly to such requests, resulting in a further delay of treatment.

Health plans often use step therapy protocols as a tool to reduce prescription drug spending; however, studies suggest that any savings may be offset by increased care costs resulting from additional outpatient visits, hospitalizations, and more.[xiii,xiv,xv,xvi] Most concerning is that health

plans' step therapy protocols are frequently more stringent than recommended treatment guidelines or inconsistent with recommended treatment pathways, and as a result they create a barrier for patients to receive timely, medically-indicated treatment.[xvii] Research has also found that step therapy requirements prevent patients from adhering to effective medication regimens, which can lead to worse health outcomes.[xviii]

Family physicians see patients lose control of their previously well-managed diabetes and hypertension as a result of these tactics, in addition to requiring more office visits and in some cases emergency department visits and hospital stays. Therefore, **the AAFP urges Congress to pass the Safe Step Act (S. 652 / H.R. 2630), which would require employer-sponsored health plans to provide a clear and transparent exception process for any step therapy protocol.**

Additionally, when medication coverage changes, physicians are often only told that the medication is not covered – they are not given any additional information, such as a list of alternatives that *are* covered. This means they can spend a great deal of time going back-and-forth with the pharmacy trying to figure out what alternative medicine is covered by a patient's plan. Physicians often find themselves prescribing a medication that is not covered, or not preferred by the patient's insurance company, which can lead to the patient not taking the prescribed medication. Therefore, **Congress should pass the Real-Time Benefit Tool Implementation Act (H.R. 7512), which requires prescription drug plan sponsors to implement at least one electronic real-time benefit tool to allow physicians to see drug costs before prescribing.**

Further, the AAFP has and continues to strongly urge that the recently finalized regulation from CMS on electronic prior authorization be expanded to Medicare Part D plans and prescription drug coverage across other impacted payers.

Health Information Technology and Interoperability

Federal regulations – particularly those pertaining to health information technology (HIT) – can be confusing for practices because, while the Office of the National Coordinator for HIT (ONC) is generally considered the administration lead on HIT, regulations are issued by multiple agencies and often use differing definitions for the same terms. Therefore, in addition to the burdens each individual regulation brings, the volume of regulations and the pace at which they are updated is overwhelming to small and independent practices and causes change fatigue for staff and physicians.

Physicians are dependent on their CEHRT to provide them the functionality and interoperability needed to support the requirements that are placed on them by health IT regulations. When regulations do not sequence requirements appropriately or do not allow enough time for technical development and deployment, the regulations can cause significant burden on practices and physicians. We also recommend Congress work with ONC to advance real-world testing through various authorities, including ensuring new standards perform successfully in real-world testing before mandating their adoption.

In addition, the lack of standardization across EHR platforms burdens physicians and inhibits effective information sharing and care coordination across the patient's care team. The AAFP has long supported efforts to advance interoperability and data sharing standards, including ONC's development of information blocking regulations. Information blocking is when an individual or entity impedes the delivery or utilization of an EHR, making interoperability impossible. Below are some examples of this lack of interoperability shared by family physicians:

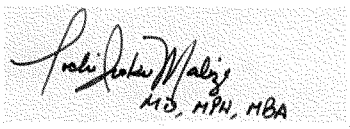
- One family physician shared that they use a different EHR than their local hospital system, and the emergency department (ED) and inpatient services cannot see the physician practice's updated medication list despite both organizations being connected through Epic's Care Everywhere. When patients are discharged from the hospital, they are routinely discharged on a medication list that has no reflection of their home medications because the medication list in the hospital system was wrong in the ED, stayed wrong upon admission, was never corrected during the hospitalization, and therefore, was of course still wrong upon discharge.
- Another physician stated that consulting subspecialists in their two main systems assume that "everyone" can see their notes and no longer send chart notes in response to referrals. The practice's referral coordinator spends time every day trying to track down consult notes from subspecialists who think their notes are visible throughout the system due to their "connected" systems. When notes do come in as an electronic "Record of Care," they are not tied back to the referral order to close the loop automatically. Instead, they must be manually labelled as a consult note and attached to the order that generated the initial referral by a staff person or the physician.

However, while the Academy supported the development of information blocking regulations, we continue to urge federal policymakers to provide support in implementing these requirements, particularly for small practices. Despite ONC's longstanding efforts to reach and educate the health care community about information blocking, significant knowledge gaps still exist regarding the implementation and enforcement of these regulations. Several independent, small, rural, and solo medical practices are still unaware or underinformed about information blocking requirements.

The AAFP has urged the Department of Health and Human Services, ONC, CMS, and other agencies to develop an intra-agency communications plan and educational outreach program specifically designed to reach physicians in underserved communities and small practices. Family physicians want to follow regulations and appropriately share information with their patients and other members of their patients' care team, and significantly more education is needed for practices to be able to achieve those goals.

Thank you for your leadership to address one of the most pressing challenges facing family physicians, especially small practices. We look forward to working with you to advance policies that will alleviate administrative burden and ensure physicians can spend more time on what truly matters: their patients. Should you have any questions, please contact Natalie Williams, Senior Manager of Legislative Affairs, at nwilliams@aafp.org or Anna Waldman, Associate of Legislative Affairs, at awaldman@aafp.org.

Sincerely,



Tochi Iroku-Malize, MD, MPH, MBA, FAAFP
American Academy of Family Physicians, Board Chair
[remove once approved]

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Associated Builders and Contractors

May 8, 2024

The Honorable Roger Williams
Chair
House Committee on Small Business
U.S. House of Representatives
Washington, DC 20515

The Honorable Nydia Valázquez
Ranking Member
House Committee on Small Business
U.S. House of Representatives
Washington, DC 20515

Dear Chairman Williams, Ranking Member Valázquez and Members of the House Committee on Small Business:

On behalf of Associated Builders and Contractors, a national construction industry trade association with 68 chapters representing more than 23,000 members, I appreciate the opportunity to comment on today's hearing, "Stifling Innovation: Examining the Impacts of Regulatory Burdens on Small Businesses in Healthcare." The majority of ABC's general contractor and subcontractor members qualify as small businesses as defined by the Small Business Administration and the construction industry consistently has one of the highest concentrations of small business participation.

Providing quality health care benefits is a top priority for ABC and its member companies, and ABC advocates for policies that would ensure employer-sponsored coverage is strengthened and remains a viable, affordable option for millions of hardworking Americans and their families. As a member of the Partnership for Employer-Sponsored Coverage, ABC encourages Congress to consider principles and priorities that are important for ensuring employment-based health coverage thrives.

Relevant to this hearing, ABC supports compliance relief for employers, specifically by streamlining the reporting requirements brought on by the Affordable Care Act. Internal Revenue Service employer information reporting requirements generate undue compliance burdens and costs for employers while creating a more difficult process for employees. The ACA fails to consider that most small businesses do not have the time or resources to understand these reporting obligations and therefore risk being noncompliant.

Similarly, altering the definitions of an applicable large employer and flexibility in the definition of a full-time employee under the employer mandate would enable employees to pick up extra hours, provide consistent federal definitions across different laws and enable businesses to hire more employees and grow their operations.

ABC appreciates the committee's efforts to cut back on regulation to ensure that employers of all sizes can offer sponsored coverage to their employees, and we look forward to our continued work toward improving the playing field for America's small businesses.

Sincerely,

Kristen Swearingen
Vice President, Legislative & Political Affairs

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