

NATIONAL PRESS CLUB HEADLINERS LUNCHEON WITH
SCOTT GOTTLIEB, FORMER FDA COMMISSIONER

SUBJECT: COMMISSIONER GOTTLIEB'S FDA TENURE

MODERATOR: ALISON FITZGERALD KODJAK OF THE NATIONAL PRESS CLUB

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ALISON FITZGERALD KODJAK: Hi, everybody. I just want your attention for a moment. My name is Alison Fitzgerald Kodjak. I'm the president of the National Press Club. We are very pleased to have you all here. We're going to get started in three minutes. And this will be broadcast live, so if you would all take a moment and take out your phones and look at them, and then check if the ringer is on, and then actively turn it off. And then check again; that would be great. And that way we will not have phones ringing in the middle of our program.

I also want to let everyone know there are cards, like this, on your tables. If you want to ask questions of Dr. Gottlieb, write them on the cards and pass them up to the head table, and we will get to as many questions as we can. We had a lot of questions submitted ahead of time, so we may not get to everything.

And otherwise, enjoy the rest of your meal and we'll get started in three minutes.
Thank you.

[sounds gavel] Good afternoon, everybody. And welcome to the National Press Club. My name's Alison Fitzgerald Kodjak. I am a correspondent at NPR News, and I'm the 112th president of the National Press Club.

We have a terrific program today, and we invite you to listen, watch or follow along on Twitter using the #NPCLive.

For our C-SPAN and other broadcast audiences, please be aware that in the room today are members of the general public, along with the working press. So any applause or reaction you hear is not necessarily from working journalists.

I'm going to begin by introducing our head table. So please hold your applause until everybody's been introduced. To my far left is Aaron Cohen. He's the president of Aaron Cohen PR; Alicia Mundy, writer at Washington Monthly Magazine; John Hillkirk, senior editor for Enterprise at Kaiser Health News; Lori Russo, president of Stanton Communications and the NPC Headliners Team co-lead.

Skipping over the podium, we have Ferdous Al-Faruque, a senior reporter at Medtech Insight and a member of the board of governors of the National Press Club; he's actually the vice chair. Skipping over our speaker for a moment, we have Anna Edney. She is a health policy reporter at Bloomberg News; William Pierce, Bill Pierce, the senior director at APCO Worldwide and the NPC Headliners Team member who organized today's event. Next to Bill is Steve Usdin, a Washington editor at BioCentury; Susannah Luthi, Congress reporter at Modern Healthcare. And at the far end, we have Joyce Frieden, Washington editor at MedPage Today.

Thanks everybody for being here. [applause]

I also want to take a moment to acknowledge the additional members of our Headliners Team for organizing today's event. That is Donna Leinwand Leger, who's the co-leader of the team; our Press Club staff liaison Lindsay Underwood; and our executive director Bill McCarren. Thank you everybody for your help.

When Dr. Scott Gottlieb addressed the Press Club in 2017, his introduction began like this: Being a successful commissioner of the US Drug and Food Administration requires a set of skills that are hard to find. It requires both understanding the complexities of science and negotiating with the universe of stakeholders – Congress, the White House, drug manufacturers, patient groups and scientists. And that leaves out the whole food part of the equation.

So it's certainly not a recipe for popularity. But Dr. Gottlieb has been praised an effective advocate for public health. In an administration that committed publicly to rolling back regulation, Dr. Gottlieb pushed for aggressive curbs on e-cigarettes and the vaping industry. The agency followed with a massive enforcement action that saw more than a thousand convenience stores and gas stations fined or warned about selling e-cigarettes to minors.

During his tenure, the FDA approved a record number of drugs, especially generics and orphan drugs. However, what may have separated Dr. Gottlieb from his predecessors most was his Twitter feed. [laughter] He used it to communicate FDA policies, priorities and plans. I followed both of them. Some of those tweets stoked controversy, as well as showcased his trademark wit.

The FDA took public comments on whether producers of some vegan products can call them "milk" or "cheese." As a legal case meandered through the courts, Dr. Gottlieb offered a running Twitter feed on the nature of almond milk. In one tweet he wrote, "An almond doesn't lactate, I will confess." [laughter] And he posted photos of the milk he found in the refrigerators at Fox News and Bloomberg. The debate eventually turned existential when Dr. Gottlieb took to quoting the French writer Albert Camus.

Dr. Gottlieb came to FDA in 2017 after a long career in public health and government service. He's been at FDA before in the early 2000s. And between his tours at FDA, he worked on implementation of the Medicare drug benefit as a senior advisor for the Centers for Medicare and Medicaid Services.

Dr. Gottlieb previously served as clinical assistant professor at the New York University School of Medicine in Manhattan, where he also practiced medicine as a hospitalist physician.

He's a graduate of Wesleyan University with a degree in economics and Mount Sinai School of Medicine where he just shared that he gave the commencement speech 20 years after graduating.

Dr. Gottlieb resigned his FDA post on April 5th, and President Trump greeted the news with sadness in a tweet – "He and his talents will greatly be missed."

Dr. Gottlieb has said that from his new perch at the American Enterprise Institute, he will be weighing in on the issue that everybody is talking about, drug prices.

So we are pleased to give him a warm National Press Club welcome. Dr. Gottlieb. [applause]

DR. SCOTT GOTTLIEB: Thanks a lot. Thanks for the honor of being here again with you. I've learned a couple of new things already today. I was telling Anna Edney, to my right – we were talking about all the press releases that we put out at FDA – I was commenting that my goal was to try to put out 2000 words on a daily basis so that by the time reporters were done reading the 2000 words, it didn't leave a whole lot of time. And she said that she had learned that what she should do is just search for the word "today" and then she'd get right to the news in the whole press release. [laughter]

These are the kinds of things I wish I knew before I took the job. And maybe if I have the opportunity to do something in government service again, I'll be smarter next time and I won't use the word "today"; I'll come up with some other way to introduce the actual news peg halfway through the press release.

I had the privilege to be at FDA during really what I think is a remarkable period of political and scientific opportunity. On the one hand, we had the tailwind of substantial new authorities and resources to advance novel medical innovation. That policy momentum came from a number of things – the recently passed 21st Century Cures Act, the recent

reauthorization of the Prescription Drug User Fee Act, all of which gave the FDA new resources, new authorities to help safely regulate all these new technologies.

And in front of us, we had gene therapies, cell-based regenerative medicine, more targeted therapies, and the introduction of better tools for delivering therapies from digital health apps to artificial intelligence to next generation sequencing. We are living in what I think is really an age of momentous progress and rapid cycles of innovation when it comes to healthcare. We have more ability to use technology today to achieve sizable and really secular advances in the practice of medicine than at any time before.

This stands in stark contrast to any other historical period of medical practice. That history, if you look back, was generally marked by a gradual evolution in advances in the standard of care. It's a history where, over time, small advances in science and technology would consolidate to provide incremental, gradual improvements in medical care over long stretches of time.

By contrast, if you look at what we're living in today, the age we're in now, it's one where the improvements to care and outcomes are no longer gradual. Instead, the advances can be rapid and very dramatic over a very short period of time. It's a time where in one year, we might be treating a chronic disease like sickle cell disease, and a year later able to cure the same disease with a gene therapy.

That's really what I saw at the FDA and what I was seeing in the pipeline.

To advance these opportunities, at FDA we focused on shaping the modern regulatory framework for the efficient and safe regulation of these new technological platforms. Now there's a big focus of what I think define the period of time we were in. And looking back, I hope the policies that we crafted and set in motion will be one of the defining features of that period of time that I had the opportunity to be part of as the agency's leader.

But as with all these scientific models, as we firm up the framework for developing and safely regulating these technologies, we're struggling with the proper framework for reimbursing them. We need to make sure that patients who can benefit from these advances have access to them, regardless of their economic status. It isn't just a question of price. Fundamentally, I think it's a question of access. And while price impacts access, it's not the only factor impacting access.

Sometimes a high price for a transformative innovation is well justified by the risk and costs of development and the value that's being delivered to patients. But even when a price that can be justified by the value that a product offers, access can still be forestalled, especially for patients in Medicaid, especially for patients in skinny health plans and other situations where they might be underinsured. And in an age of curative therapy, access really becomes paramount.

A treatment that can deliver a cure takes on a really special public health value. People's destiny should not depend on whether they can pay for a cure.

The very success in this regard of the pharmaceutical sector in delivering really outright cures for more diseases now and certainly going forward raises that imperative to make sure that everyone has equal access to these opportunities.

This can be especially true when it comes to transformative new technology where the costs are high, and the prerogative to widely and rapidly adopt a new product is strong owing to its transformative medical potential. These kinds of paradigm changes don't fit neatly into the way public insurance schemes are structured and budgeted right now.

As a result, increasingly we see privately insured patients have faster and broader access to potentially lifesaving new technologies, while patients in Medicaid programs or with skinny health plans can be locked out of these same opportunities. And that sets up an intolerable circumstance. I believe the tension over the high price of these technologies is often an expression of angst over this differential access.

It's a rightful angst. People should be upset by these circumstances. But we need to focus our attention on the public health dimension. And that dimension really turns on the question of access. It turns on making sure that when a cure is at hand for a vexing disease, the ability to alter the destiny of a child or a cancer patient shouldn't be rationed.

We're seeing these same struggles play out in Medicare. The current leadership at CMS, who I worked with, is acutely focused on the challenges of paying for disruptive and beneficial new technologies like gene therapy and Car-T. They've advanced a number of thoughtful policies to try and contemplate how Medicare will create the modern framework to provide efficient, equitable access to these advances.

But despite those efforts, challenges persist. It's a result of longstanding, structural features of Medicare that make it hard for the program to embrace coverage of new technology. And Medicare isn't an ordinary payer. The decisions it makes end up driving a lot of the private insurance activity in the private market.

By design, Medicare has features that are often deliberately calculated to slow the introduction of new technology, and smooth out its costs over time. But the delay can deny patients access to breakthrough technology for a long time. Although a new product is covered while it's under a national coverage determination, hospitals can be confused about how it will be covered at all in these situations and stop providing it. This, in fact, was the case for a therapy class known as Car-T.

There are other challenges when it comes to hospital-administered technologies like Car-T that are paid for under existing hospital-based diagnostic related groups, or DRGs. The DRGs, as many of you know, are basically bundled payments for categories of items and services, including drugs. But it can take a long time to develop a new reimbursement level for a DRG in order to accommodate the cost of the introduction of a new technology. And that difficulty of paying for new, hospital-based innovations makes entrepreneurs reluctant to pursue new treatments that must be administered in the hospital setting.

New technology add-on payments are meant as a stopgap way to offset the costs of a beneficial new technology while Medicare modifies its other payment rules. And the leadership of CMS recently has taken thoughtful new steps to more routinely extend these payments to breakthrough devices and increase the rate that's paid in these circumstances.

But these payments are still often insufficient to offset the reimbursement shortfalls. And this can be especially true for drugs. Here, payment is sometimes less than 50% of the cost of a drug itself. And the add-on payments, while helpful, are another policy Band-Aid that don't fully cover the program's systemic wounds.

In the end, there's a bureaucratic roulette when it comes to coverage for new technologies. Which Medicare payment scheme a drug falls into has a lot to do with its commercial success, and, in turn, whether investors will back it in the first place.

That makes no sense. It's exactly backward. Innovation should follow the clinical opportunity, not payment rules. And this is especially true when payment rules are more dependent on site of service than any measure of value to patients or the benefits that patients are going to ultimately derive.

We're seeing situations quite literally where investment and development is being fashioned in a way to create products that can carve around reimbursement obstacles, rather than following the science towards products that will have the best therapeutic profile.

Consider Car-T again for the treatment of cancer. The autologous or the self-derived nature of these treatments contributes to their efficacy and the fact that the cells are derived from the patient who's ultimately going to receive product. But there's a belief that these same attributes also contribute to risks that require it to be infused in hospitals – risks like cytokine release syndrome. Because these drugs are delivered in a hospital, it puts them under the inpatient hospital-based payment scheme.

And here's the rub once again. Being under that hospital-based payment scheme means the hospitals lose money on each infusion. Site of service, rather than clinical considerations, drive the payment. Hospitals have lost a lot of money on each infusion when you talk to them. Now some aren't even able to treat patients with this new technology. And manufacturers have had a hard time getting pricing that they say compensates for the cost of goods, or the value they're offering.

And so, as a consequence, much of the new investment in Car-T is going toward developing what's called allogeneic treatments. These are cells that can be taken off the shelf and aren't derived from the specific patient. It's more akin to the way a traditional biologic is made.

And make no mistake. There's no clear view that the allogeneic cells work as well as the autologous preparations. In fact, there's a view that they probably won't work as well.

And in order to deliver these cells in an allogenic way, you'll probably have to boost them with another therapeutic in order to get the same response.

But instead, the allogeneic cells might be easier to administer in a doctor's office or a hospital outpatient department. And that could, quite literally, game around the reimbursement challenges plaguing Medicare's current inpatient system.

So what's happening is that the investment and development strategies are being shaped in some measure to match the reimbursement paradigm, rather than merely leverage the best scientific and clinical opportunity for patients. The reimbursement framework for a cancer cure should really be the easy part. And the coverage should be engineered to accommodate the best science and not the other way around.

Over time, these challenges have not been lost on policymakers. But the policy Band-Aids, again, that have been crafted to blunt these incongruencies don't really cover the wounds. The inpatient prospective payment system, which essentially bundles the payment for hospital-based services, makes it hard for new technologies to qualify for add-on payments that were meant to offset the cost of these disruptive new innovations.

Medicare faces challenges on the outpatient side too. Under the office-based and hospital outpatient payment schemes, Medicare can be slow to make coverage decisions. It can also pay for a lot of things in ways that don't drive value or evidence creation. And the program chronically overpays for services that deliver negligible value. It's subject to manipulation and at times fraud. And it's slow to spot the ways that its insurance schemes are sometimes gamed by people, or to erect rules to curtail these practices.

Now this isn't to say in any way to knock on the people at CMS who all recognize these challenges. And I worked at the agency for a period of time. They're working hard to address these issues. But it's a function of the rules they're forced to operate under and the tools and resources that they're given to try to address these challenges.

There are some notable examples, if you look back on history, where the labored process by which Medicare adopts new innovation slowed beneficial advances in the practice of medicine:

In the early 2000s, CMS substantially and chronically underpaid for a new class of radiopharmaceutical drugs. The drugs were designed to deliver a radioactive payload directly to cancer cells. Two of these drugs were Zevalin and Bexxar. They're not used anymore. Or they're used in very limited fashions now.

The FDA approved the drugs to treat patients with relapsed or refractory non-Hodgkins lymphoma whose disease no longer responded to chemo or radiation. But CMS set reimbursement rates for Bexxar and Zevalin at about \$10,000 and \$15,000, respectively. And the drugs cost more than \$20,000 per dose. Treatment could also require more than one dose.

And I should say I have no financial interest in either of these drugs I haven't been out of the agency long enough to develop any conflicts of interest yet. So just give me a little time. [laughter]

Hospitals weren't getting paid equal to what the products cost. So they stopped doing this type of therapy in hospital outpatient departments altogether. And if Medicare beneficiaries couldn't get access to these drugs, well, nobody could. Under the federal rules, hospitals that don't offer a drug to Medicare patients are barred from offering it to others, even if other insurers fully cover the treatment costs.

The drugs were really a clinical success for certain patients. I don't want to overstate their success, but they worked for certain patients. In certain patients, they were the only beneficial treatment. But the medications were a commercial failure. And the episode had a broader effect on the market for other radiopharmaceuticals that had to be administered in hospital facilities, demonstrating Medicare's outsized influence. Innovators quite literally got out of the business of making radiopharmaceutical drugs despite the promise of this technological approach to the treatment of cancer.

The Medicare scheme, by design, aims to slow the introduction of new technology platforms and the sort of paradigmatic clinical change that can result from this progress. The program is structured in a way to smooth out the fiscal impact of technological change. Yet the nature of where and how the innovation occurs is offering the opportunity for exactly the opposite right now – sharp and sweeping improvements in how we can approach the care for many diseases, and even the opportunity to cure intractable illnesses.

Sometimes the innovation is so novel it falls entirely outside any existing payment bucket at all, any benefits structure. In these cases, Medicare grapples with a way to pay for a product even in circumstances where it might embrace the technology. And this was the case recently with insulin pumps.

The remedy can be to seek specific authority from Congress to cover a new benefit class. But patients can't wait for Congress to separately act on each new category of technology every time there's a paradigm change in the state of medical care.

We should consider these impacts as we also contemplate now Medicare for All. Now, I know Medicare for All, the whole idea has a certain seductive political appeal for its seeming simplicity. So much so that I think the prospects for single-payer Medicare probably are nearer to happening than I think many fully appreciate right now as a political matter.

The political candidates advancing this proposal could implement it on a fairly slim majority. The two key elements of Medicare for All could be achieved through budget reconciliation – expanding benefits to include younger people, and eliminating private insurance coverage by taxing employer sponsored insurance.

If a Democratic candidate wins the White House in 2020, it's conceivable that the new President could also seize control of the Senate, even on a slim majority. Democrats

already used budget reconciliation to pass much of the Affordable Care Act, and Republicans used it for its repeal.

Expanding covered services in legislation may be harder under reconciliation. So when you look at some of the proposals right now, they talk about hardwiring into legislation some new benefits that would be relevant to a younger age population. But lowering the eligibility age or even implementing[sic] cost-sharing might be possible through reconciliation.

And Congress could ultimately leave it up to CMS to approve coverage for new treatments within the existing benefit categories. The agency already has legal discretion to determine which services are reasonable and necessary for diagnosis or treatment of disease. If Medicare is expanded to cover younger patients, rather than hardwiring specific new benefits into the initial legislation, these decisions could be left to rulemaking.

Now, I'm not advocating this approach. I think it would squelch innovation and I think it would hold back medical progress. There are many reasons to be highly skeptical of expanding Medicare and using it to crowd out private coverage. And the potential impact on innovation and the adoption of transformative new medical technology should not be underestimated.

The private market has shown a much better ability to incorporate beneficial new innovations than government insurance programs like Medicare and Medicaid. It's been much more willing to embrace sweeping advances in medical care.

But consider if there was Medicare for All. With CMS driving the adoption curve for new innovation, we're likely to see even more investment be distorted in ways that are designed to fit advances into rigid and often outdated coverage categories rather than optimize the scientific opportunity set. We're likely to see adoption curves slow further. And we're likely to see the chance for paradigmatic changes in medical practice become more elusive.

We're living in a time right now where there's opportunity from science to alter or cure disease, and these opportunities are firmly at hand. Scientific challenges certainly remain. But our biggest obstacle may be policy.

Our biggest challenges may be our inability to devise coverage schemes that can enable the efficient, and, when appropriate, the rapid adoption of these innovations, allow for a return on capital that maintains investment in these high-risk endeavors, and, most important, enables equal access to a cure regardless of a person's wealth.

The scientific and regulatory challenges may turn out to be the relatively easy part when compared to the challenges for paying for these things, especially if we want to finance these opportunities in a fashion that optimizes access for patients who most need them and doesn't discourage future investment and innovation.

Thank you very much. [applause]

MS KODJAK: Again, a reminder, if you have questions for Dr. Gottlieb, put them on your card and pass them up. I am going to start. When you talk about the challenges that Medicare faces in smoothing out payments and how it's going to deal with therapies like Car-T that are hugely expensive, is there a way to do— like Louisiana just adopted a sort of subscription model of treatment for Hepatitis C which allowed them to make a sort of annual payment of a certain amount and then be able to do the treatment as needed. Is there a way that CMS could look at a program like that?

DR. GOTTLIEB: I think we should certainly try to think of a different structure when it comes to paying for these kinds of technologies. The Medicaid question is an interesting question. And when I travel around— well, I'm starting to travel around. But when you talk to sponsors who are developing things like gene therapies, there's a lot of activity going on and discussion going on with private payers about ways to try to come up with completely different schemes where you can potentially amortize the cost of a gene therapy where there's a lot of upfront cost. You can backend some of the payments after you see the durability of the effect that you're going to get from the gene therapy product.

I think those discussions are highly valuable. I think it's important to see if we can innovate payment models. But those new models aren't going to be necessarily transferable to the Medicaid market. And I think that the sponsors who are developing these potentially curative therapies, if they have limited bandwidth to be having a discussion with anyone in the marketplace, they ought to be focusing on the Medicaid market. Because ultimately in the private market— there'll be situations where you're dealing with such larger numbers of patients, it's going to be hard for the private market to absorb these things. But ultimately in the private market, the employer-sponsored market, these are insurable risks. If you're going to cure a pediatric inherited disease, an insurance pool might have one or two patients in that pool with the disease. And that's an insurable risk, and that child would have cost that insurance pool a lot of money regardless.

I think the real challenge is going to be in Medicaid where a lot of— staying on the subject of pediatric inherited diseases, a lot of these kids end up on Medicare disability and they're dually eligible, so you see a disproportionate number of children in these situations in Medicaid. That's across the board for a lot of these severely debilitating diseases. And again, it's creating the specter that a product could come to market. You've worked out the coverage with Aetna, United. But you haven't worked it out with the states yet and the Medicaid. And you're going to have a situation where you're curing kids in the private market and you're not able to do it in the public market.

And that's the intolerable situation. I don't think that feature is tenable for any period of time. If you're going to come to the market with a cure, you've got to be able to provide equal access regardless of the ability to pay. So that's where it becomes important to think of these things that you can do. Could you tender to states? Could you try to structure it like a service where you're offering it to states. It might require a waiver from the state.

I think people need to think through these challenges of how they make sure there's equal access at the time of market introduction.

I'm trying to spend some time thinking about these Medicaid challenges now. My biggest challenge is that before I came into the government, when I had a really hard Medicaid problem, I would call up this one consultant who was really, really good at Medicaid. Her name was Seema Vermer. [laughter] Now I no longer have access to her so I can't call her. So I've got to find a new guru on these issues.

MS KODJAK: When you're talking, again, about Medicare, and then comparing it to the reimbursements for the private market, there's an implicit criticism that Medicare's not paying enough. Or maybe explicit. But one, Medicare's population is inherently older and sicker than any private market insurance pool. And second, you have people screaming that we're spending way too much federal money on healthcare. So where can you go from there?

DR. GOTTLIEB: It's not just that Medicare isn't paying enough. It's sometimes that Medicare isn't paying at all, or you see these situations, like with the Car-T. If you can deliver in the outpatient setting, the reimbursement rate's diametrically different than if you deliver in the in-patient setting. So quite literally, by changing site of service, you're changing the payment scheme considerably.

And even on the outpatient side, where companies are getting reimbursed where the government's effectively a price-taker, I'm not going to sit and argue that the government's getting a good deal in those situations either because oftentimes they don't have any ability to either make sure that they're paying for the value that's being delivered to the Medicare beneficiaries, or trying to incentivize additional evidence creation that can help guide the future delivery of these products.

So I think on both ends of the spectrum it's not working real well. But it behooves us to try to— the temptation in government always is to try to take a new technology and fit it into an existing regulatory paradigm, rather than trying to create a new regulatory paradigm.

What we did with the new technology on the FDA's side was, when we had Digital Health, we created a new paradigm where we took a firm-based approach to the regulation of Digital Health, recognizing that the existing medical device approval process doesn't lend itself to something that's so highly iterative.

When we had next-generation sequencing, we recognized – or artificial intelligence – we recognized that you would have to adapt those platforms so quickly that, again, we couldn't require companies to come in with pre-market approval every time they changed the variables in a next-generation sequencing platform. So we allowed them to demonstrate their utility and their safety and effectiveness by being judged against a publicly available database. And if you can meet a certain threshold of accuracy against a publicly available database that you would test your device against, that could suffice for regulatory approval.

When it came to gene therapy, we thought we recognized that the complexity, the uncertainty, the risk is in the product features of a drug, not necessarily the clinical portion of the review. So we inverted the review process there.

So we tried to think about how to create brand new regulatory paradigms rather than trying to fit these very novel products into the existing paradigms. I think we need to do the same thing on the reimbursement side. I think we need to figure out completely different structures to try to reimburse Car-T gene therapy, things like that, rather than try to fit them into the existing paradigm because it's not going to work.

MS KODJAK: I'm going to step back from our deep wonkery for a moment and go to some of these questions. Name one proposal that you support to reduce drug prices that you couldn't say when you were commissioner. [laughter]

DR. GOTTLIEB: It's interesting because the sort of common answer is, of things that I was against, I really was against. I really did think reimportation was not effective. I really do not believe the government should be directly negotiating prices. I've long advocated that we should move Medicare Part B into a competitively bid scheme. I was not talking about that a lot when I was in the FDA role because I didn't want to be trying to talk about advanced Medicare policy.

I've spoken about this before I came into the government and since I left in the last month. We devised Medicare Part B's reimbursement scheme, ASP-plus-6%, at a time when – I was there at the time, so this is back in 2003/4 – with the MMA. We believed, on the Medicare Part B side, that Medicare would perpetually be a price-taker when it came to these injectable drugs. We looked at a landscape where there was one EGFR inhibitor, one VEGF inhibitor, one or two TNF inhibitors on the market at the time. And we thought these are going to be monopoly products in perpetuity.

MS KODJAK: Can you say that in English, by the way?

DR. GOTTLIEB: [laughter] All right. Well, we thought Medicare would have to— there wouldn't be competition, there wouldn't be product competition. There'd be one drug in a category. And so, this ASP-plus-6% was devised to say, okay, Medicare is going to have to accept whatever price the manufacturer wants to sell it at. But we devised it in a way that would be hard to take big price increases because doctors were buying the drug, they were only being paid the average sales price on a backward-looking basis. So if you took a big price increase, the doctor would be out of money, they'd be under water. So it was hard for a manufacturer to take a big price increase.

What we're living in right now is a multisource world—

Is that okay?

MS KODJAK: Yeah.

DR. GOTTLIEB: –where there are lots of products in these categories. And not just multiple products in these categories, but there are therapeutically interchangeable products. There's biosimilars at a lower cost. But Medicare isn't competitively bid. You don't have multiple products competing against each other to get on a formulary by offering a lower price, a better deal to the program.

So I think we should be moving Medicare Part B into something like Medicare Part D, where we take advantage of that product competition. So that's something I didn't talk a lot about.

On the reimportation, I'll just say I am concerned about that. And I will remind folks that the reason that we have the closed system in this country, the reason why we passed the Prescription Drug Marketing Act and Drug Quality Security Act was, at least in part, and in some cases more than just a little bit in part, because of counterfeit drug problems that were originating in Florida.

Florida, for folks who remember, who have long memories, had a substantial problem with small wholesalers that were bringing counterfeit drugs into the United States and was becoming a distribution point for counterfeit drugs in the US. And they ultimately recognized that, too, and they were one of the first states to implement a paper pedigree, a paper pedigree that many people at the time felt wasn't sufficient and it ultimately led to federal legislation and a movement towards a national paper pedigree and an electronic pedigree. And Bill probably remembers some of this history, too.

So there's a long history there of challenges that we ought not to lose sight of as we start to consider not just potentially legalizing the importation of drugs, but legalizing it through Florida.

FDA took an enforcement action against a company while I was there – it's all public – that was partially based in Florida, that was purporting to bring drugs in through brick-and-mortar pharmacies in Canada and I think the UK and Australia, if I remember correctly. When FDA put out that statement, the agency said, I said in the statement that we had concerns that the drugs weren't coming from brick-and-mortar pharmacies in Canada.

So there's no real foolproof way to do this. The situations where the agency has allowed for limited reimportation is in the setting of shortages, and it does do that sometimes. They are very, very resource-intensive. Ensuring the end-to-end security of drugs coming in from other countries in those situations ends up being an extremely resource-intensive endeavor for the agency, not something that could be replicated on a wide scale.

MS KODJAK: What do you think about Secretary Azar's international pricing index proposal?

DR. GOTTLIEB: I certainly support what the Secretary's been trying to do to try to address the price and drug access challenge. I don't want to speak for the Secretary, but I

think he recognizes it from its public health dimension. And the challenge being one of not just prices, but how the prices impact access.

I think ultimately, if you move the Part B system— there's multiple ways to address Part B. You could base it off of an index of foreign prices as a way to try to create an international market for prices with the hope that that's going to put pressure on manufacturers to offer these drugs at more of a global price.

But the other option is to move it into a competitively bid scheme. My hope always was that that proposal would ultimately lead to more of a consensus about making that a true market and putting it into a competitively bid scheme. I think that's far— I always used to say to manufacturers, even years ago, ASP-plus-6% is a price-fixing scheme. It's just fixed to the price that they like. But a price that can be fixed at a level that you like can be fixed at a level that you don't like. And I think some of the manufacturers don't like the level that the IPI would fix it at.

But it's an argument for ultimately trying to move that into a competitive framework. And that's something that was hard to do administratively. It would be hard for them to do that through CMMI, through regulation. I think it would take legislation to do in a robust fashion.

But there have been two other proposals in the past: Secretary Leavitt examined moving the drugs associated with durable medical equipment into— that were paid for under Part B into Part D and scored the savings on that. And it was meaningful. And under the Obama administration, they did a separate analysis looking at what it would save to move more of the drugs in. And the savings were substantial, tens of billions of dollars a year in savings if you move the Part B drugs into a Part D-like scheme.

Now, you end up with formularies, so patients can't get access to every single drug. You'll have the same things you have in the private market that many of you probably have in your privately insured schemes. But this is the direction that the private market certainly has taken.

Is that okay?

MS KODJAK: [laughter] I'm not trying to be the language police. I just want to make sure our audience can hear. There's also been a proposal to eliminate the anti-kickback and safe harbor, which basically would change how drug discounts are organized in the private market. There's some sense that this would increase transparency, yet maybe increase the price of drugs or insurance. How do you think that fits in with your vision of changing how drugs are paid for?

DR. GOTTLIEB: I certainly think of the proposals this could potentially be the most disruptive in terms of changing how things are priced in the market. And the whole idea of this list versus net spread and the way that companies rebate isn't benefiting patients. Because ultimately patients who are out-of-pocket or underinsured or in closed formularies

where their drugs aren't insured, they're the ones paying the list price. So patients who can least afford it are paying the full list price, which is basically a fake price to everyone else, and not gain the benefits of the net price.

And so, to have a discounting in the form of backended rebates is not benefiting pants who are out-of-pocket. And you're ultimately using the money that those sick patients are spending to subsidize the premiums for everyone else because the rebates don't go back to the patient in most cases, although some insurers try to do that. They go to bringing down the premiums.

And it's partly a problem with the way the insurance companies compete, because they compete on the basis of having the lowest premiums and not necessarily on the benefit design. People don't look at the benefit design often when they're buying an insurance plan, they look at the out-of-pocket and what their premiums are going to be.

So I think taking away the safe harbor for those rebates under the anti-kickback statutes could be disruptive in forcing the market to have to contemplate more upfront discounts, rather than these backended rebates. I think it's certainly going to push in that direction.

Now, there's debate about whether doing it just in Part D is enough to really push the market. If I had to guess what would happen if that rule gets finalized and goes into effect, I think that there'll be a reduction in the average list price. You might not see complete compression by the way the list versus net spread, but I think you'll get a downward pressure on the list price and more of the market is going to move towards schemes where they don't have these big rebates, these big artificial list prices.

Part D is a big enough market to influence a good portion of the market. I think some of the scorers, the CBO scorers and others were skeptical because they think you have to do it across the whole market in order to have an influence. But remember, when we created MMA, there was no such thing as a specialty tier, really. There was like 5% of the market had a specialty tier. We put it into the Medicare Modernization Act deliberately, in part to try to incentivize the market to move out of what was maligned at the time as sort of me-too drugs and move towards more specialty drugs, more curative therapy single-source drugs. We wanted people to be investing in very highly innovative things. So there was a structure created that preserved the reimbursement for those drugs, the specialty tier.

Once Medicare did that, you saw the private market adopt a very similar structure. So Medicare drove the activity of a formulary design across the private market. I think the same thing can happen here.

MS KODJAK: I have a question here about insulin, and I'm going to sort of expand on it, because one of the things, as a healthcare reporter, we hear is just massive complaints about the cost of insulin, the rising prices for essentially a therapy that's been around for a hundred years. And I know that it's changed a bit. Can you talk a little bit about insulin? This questioner says, Will insulin ever be affordable in the United States?

DR. GOTTLIEB: I mean, it gets back to the last question, too, because when you look at the net pricing in the insulin market, for certain the insulins where the list price has gone up a lot, the net price actually has gone down over time in recent years. Part of the challenge is, the more competitive the market, the more you have big rebating on formularies. And so, the list prices get pushed up even as the net prices come down.

So it's exactly the opposite of what you'd expect. You expect a competitive market, the pricing should have come down. Yet in these competitive categories, the list prices go up because they want to have these big rebates.

I do think that the steps that we took at FDA to move the insulins over to the biologics pathway and open them up to biosimilar competition is going to get more competition on to the market. So historically, four biologics are regulated as drugs – insulin, human growth hormone, interferon and hyaluronidase. So by a quirk of history, there were four biologics that were regulated as drugs under the Food, Drug and Cosmetics Act, under 505– I think it's 505. it's like a month, I was at Disneyworld, I forgot the statute. [laughter]

So we moved those over to be regulated under the Public Health Services Act as biologics which for the first time is going to open them up to biosimilar competition. And I think you're going to see a lot of biosimilars come on to the market as competition to the branded insulins. There's some people who are saying, Well, it'll make it harder to get copies on to the market. It can't be any harder than on copies, right? I mean, you didn't see generic insulins coming on to the market, so it can't get any worse. I think it's going to be transformative. I think you're going to see a lot of competition.

But also remember, in the insulin market, there's this problem with these older drugs that you mentioned where the prices don't seem to be coming down, the list prices at least. But there's also been innovation in this market, and I don't want to lose sight of that. The innovation's been very incremental with these insulins, but the subsequent cycles of insulin all have slightly better profiles. As a physician, what you want to do is you want to have an insulin product that gives patients a basal rate, a sort of level amount of insulin over the course of the day and makes it less likely that they're going to have blood sugar that's too high and less likely that they're going to have blood sugar that's too low. That's the optimal profile.

And each generation of insulin gets slightly better to delivering that. So there has been meaningful innovation here. And these aren't sort of paradigmatic changes in different generations of insulin. But incremental changes, incremental benefits can mean a lot in this market.

MS KODJAK: This sounds like you're going to have classes of drugs for different levels of income, but should there be a cheap insulin that's still available to people who need it? They can still get to work on a car if they have to roll down their window by hand. It's not as good, but– right now, we hear so much about people limiting and skipping doses and not taking their insulin. Where do you stand on that?

DR. GOTTLIEB: The answer is that there are, there are cheap insulins on the market that would do the basic job. They're not as good. And I think sometimes as a physician you have to think about these things because a patient's better off with a product that's not going to be as good as what's available than nothing at all. And you need to be mindful of that.

But ultimately, you don't want people to have to use a product that's not as good or older and not delivering the same benefits because, again, they're underinsured or uninsured. This gets back to what I was saying in the outset of my remarks about access. We need to figure out a way that there's not this differential access in the market. And you have two tiers of society – one that can benefit from new technology and one that you can't. And then you're further disadvantaging people who already face a lot of obstacles in society. Now they're facing obstacles because their health is poor and they're a diabetic who can't afford the good insulins and so they're going to have amputations and heart disease, and it's just going to exacerbate poverty. The inability to access medical technology could really exacerbate poverty if we don't address this.

It's apparent in insulins. I can make this sort of argument in the insulins, but that's going to play out over 20 years. What if you have a cure to sickle cell disease, or you have cure to a pediatric inherited disorder? Then that differential access becomes really stark. You look at some of these genetic diseases and you see high divorce rates among families; families get pushed into poverty; one parent can't work. So this is having a really serious impact on society, a lot of these health burdens. If you can eliminate it, that's wonderful. But what happens when you only eliminate it for the wealthy half of the society? You've just now exacerbated tensions in society. And I think that's untenable and that's what I think we need to solve for, first and foremost.

MS KODJAK: I'm going to go back to biosimilars, which seems like a stark change in subject matter. This questioner said, Despite efforts by government and others, the biosimilars industry seems slow to get off the ground with few new biosimilars getting approval. What can you do to fix that to improve it?

DR. GOTTLIEB: I actually am optimistic about the future of biosimilars. I really am. I think that the expectation that this was going to be off to the races in year one I don't think was ever realistic. This was always going to be a slow evolution, a slow market to take shape. And the biggest impediment, quite frankly, is physician reluctance to prescribe these products. And that's not that unusual either because if you look back at the early days of Hatch-Waxman, there was a lot of physician reluctance around prescribing generic drugs. Doctors weren't sure they were the same as the branded drugs, and it took a while to educate physicians and have doctors getting comfortable with prescribing these products.

So I think that we've seen some successful biosimilar launches now. Companies have figured it out how to do it successfully. We've seen more chronic care biologics coming off patent where you can develop biosimilars to it. I think that's going to be easier to introduce into the market rather than curative therapy. I think there's going to be more lingering

reluctance among doctors when it comes to curative therapy, like for treatment of cancer, where a doctor might have a lot of experience with a cancer treatment and be more reluctant to switch over to a biosimilar.

But this market is evolving and taking shape. I still think the history, the sort of rebating structure has a real impact here. Because if you're a biosimilar and you launch at even a substantial discount, if a health plan puts you on their formulary, they're going to lose the rebates on the incumbent biologic. So unless they can move enough of their physicians over from the incumbent biologic onto the biosimilar, even if the biosimilar's heavily discounted, they might lose money.

If the biologic's \$100 and they're getting a \$40 rebate and a biosimilar comes in at \$70, that sounds really great. But if they put the biosimilar on formulary, now instead of paying 100 for the incumbent, they're paying 140 because they lose the rebate. Sure, the biosimilar's at \$70, but if they can only move 10% patients over to that biosimilar, they've lost a lot of money, and they might lose money for a lot of quarters. Because what they tell you is they can only over like 10 or 15% over a year.

And again, it's because of the physician reluctance. I don't know if Chip's here; if I'm misspeaking, you can tell me I'm wrong.

So that's the commercial impediments there. But I think this is evolving in the right direction. If I was going to be making investments on where I think you can have a more disruptive effect, one is to address the whole rebating structure. And I think what the Secretary is doing could do that. But the other is to focus more resources on physician education, trying to accelerate that adoption curve.

If you look at health plans like Kaiser or Intermountain Health, even Harvard Pilgrim, who have more control over their physicians, they can get adoption more quickly. And if you talk to biosimilar manufacturers— I'll end here. I'm trying to eat up the clock. No, I'm kidding.

If you talk to the biosimilar manufacturers, they say that their target market is that employer market, the big employers, or the health plans that sort of own their own physicians or employ their own physicians. That's like 20% to 50% of the market. And that's what all the biosimilar manufacturers go in and try to target.

MS KODJAK: I'm going to move on from drugs and drug pricing, because we just have gone through a lot of time, to cigarettes, tobacco, vaping. This was a huge initiative of yours at FDA. Do you think that what you started will continue?

DR. GOTTLIEB: I certainly do. Look, there's a long arc to policymaking in Washington. I remember when I was at FDA in 2005, I lit the fuse on generic drug user fees, and it took six years to get generic drug user fees, but we got it.

I think having opened the door to the policies that we did, I think there's an inevitability to the implementation of those policies. And remember, what we announced in

the summer of 2017 was to advance rulemaking to regulate nicotine and combustible cigarettes, to render them minimally non-addictive, to more rapidly migrate adult smokers off of combustible tobacco, recognizing that it was the nicotine that kept people coming back to tobacco and it was products of combustion that caused all the death and disease from tobacco use.

And so, if you could migrate an adult smoker on to other forms of— hopefully off of nicotine altogether, but recognizing there's adults who still want to enjoy satisfying levels of nicotine, if you could migrate adult smokers off of combustible tobacco into other forms of nicotine delivery, you could have a substantial public health benefit. And the idea was by using product regulation to regulate nicotine levels in the combustible tobacco, you could more rapidly accelerate that transition, that curve.

We put out policies to try to make the market for the development of nicotine replacement therapy more efficient, to get more NRT on to the market. That's the optimal form of nicotine replacement. But we also recognize that electronic nicotine delivery systems, like e-cigarettes, could be a beneficial alternative. Certainly not a safe alternative, but a less harmful alternative to smoking cigarettes.

And so, even as we implemented the deeming rule – which took ten years to develop, and I implemented my third month on the job – even as we implemented the deeming rule, all the regulation that came with that – the regulation of manufacturing, the requires for labeling, the inspections of vaping and e-cigarette facilities – we pushed off the application deadlines to give those products more time to come into the agency with applications so that they could successful to come through the regulatory process. We didn't want to sweep the market of e-cigarettes at that time.

We felt that was a coherent balanced policy. I feel that vision's going to go forward. I think there's an inevitability to it. What we couldn't envision at the time – and we didn't know and nobody else knew – was the youth epidemic that was under way. And it was under way, we just didn't know it. We started to get the first reports in early 2018, mostly from schoolteachers, people who had middle school students who we were hearing it from. And it wasn't until we got the data on – the day I got it was August 30, 2018 – that we saw the National Youth Tobacco Survey data on e-cigarette use among kids and saw a 78% increase in teenage use. And that's when we had to shift our policy with respect to how much we were going to be willing to accommodate the vaping products at that time, recognizing that the public health virtue of those products isn't as strong as it was in the summer of 2017, once we were putting it against all that youth use.

But I still feel that these policies will go forward. The other piece of this was to regulate, to ban characterizing flavors in tobacco. We promulgated a guide on combustible tobacco. We promulgated guidance to do that in non-grandfathered cigars. So we put that in place. Ultimately, the agency needs to go through a rulemaking to take all characterizing flavors out of tobacco. But it was Congress's vision to do that under the Tobacco Control Act.

They banned characterizing flavors in cigarettes but for menthol, and they explicitly directed FDA to start a process to evaluate taking menthol out of cigarettes as well. At the time it was politically difficult to put that into legislation, but that was always, in my view, the long-term goal because characterizing flavors in tobacco is a primary vehicle by which kids initiate on combustible tobacco. If you look at kids who initiate on cigarettes – it's come down, but almost half of all kids who initiate on cigarettes, combustible cigarettes initiate on a mentholated cigarette. And why? They do it because the menthol masks the unpleasant features of smoking, makes it easier to initiate on the cigarette.

So if you want to address the youth tobacco problem in this country – and it's a substantial problem; youth smoking rates are starting to go back up – you have to address menthol in cigarettes.

MS KODJAK: We have a couple questions related to alternative products. Philip Morris has an FDA clearance to sell a heat-not-burn tobacco. And somebody else asked about a Swedish technology– how do you say it, Dan?

DAN: Snus.

MS KODJAK: Which apparently delivers nicotine without tobacco.

DR. GOTTLIEB: Do you like Snus?

DAN: No. [laughter]

DR. GOTTLIEB: You don't like the flavor?

DAN: [58:52]

DR. GOTTLIEB: I'm kidding. Just trying to objectify a reporter, I'm sorry. [laughter]

MS KODJAK: We'll get to that later. What do you think about these alternative products that are tobacco delivery? Are they safer? Should they be on the market? Or do you want to get rid of them all?

DR. GOTTLIEB: I don't believe any of them have a claim to be a modified risk tobacco product. And IQOS, as I recall, from the public press reports, wasn't approved. It got a PMTA application, so got approved to come to market, but didn't get approved to make claims around being a modified risk tobacco product.

I still maintain, I still believe in the vision that nicotine delivery exists on a continuum of risk. And the public health goal– there's two public health goals: try to get people off of nicotine, where we can, but ultimately try to move people down that continuum of risk, recognizing that nicotine's a legal substance. Nicotine itself isn't inherently what causes all

the death and disease from tobacco use, it's the combustion. And if you can move people down that continuum of risk, you can do a lot of public health good.

And so, a product that is less harmful than combusting tobacco, but certainly not safe in its own right, there is a place in the regulatory framework for that product. The Tobacco Control Act envisioned that that would be the regulatory framework that FDA would embrace and stand up. And we would help get products on to the market that had modified risk features and can move people down that risk continuum.

So I still fundamentally believe in that. I think the only caveat there is that these modified risk products can't become vehicles by which kids become addicted on nicotine. And we now have ample data showing that kids who become initiated on nicotine through e-cigarettes, some proportion of them are going to become long-term smokers of cigarettes. And all the dramatic gains that we've made historically in this country reducing youth tobacco use rates could be reversed if we're not careful.

MS KODJAK: I'm going to take a moment to apologize to all those people who sent in questions because I have piles of them here and we are running out of time.

DR. GOTTLIEB: I can take them and answer them on Twitter. [laughter]

MS KODJAK: I'll give you these to take home. Scott Gottlieb, MD. So there are a couple questions about marijuana, of course. There's a major push for it on states legalizing marijuana. Do you have concerns about the quality of research into what amounts of THC cause what levels of intoxication.

DR. GOTTLIEB: I have significant concerns and had significant concerns when I was in the role about the great natural experiment we're conducting in this country by making THC widely available and watching marijuana use rates among kids go back up and the impact this has on developing brains. And when you talk to researchers in this field who look at the biological effects of marijuana on young people, a lot of very credible scientists are very concerned about this, including the head of SAMHSA who's shared a lot of concerns publicly.

So I have a lot of concerns about this. I have a lot of concerns about the claims that are being made around marijuana. It seems like somehow marijuana is this free-pass zone where state dispensaries can make any claim they want about it and be supported by the political class. I just don't get it. We hold pharmaceutical products to really high standards for claims in the market for good public health reasons. And then we allow a dispensary to say that marijuana can cure pancreatic cancer.

Where we took enforcement action when I was there, we tried to focus— you can boil the ocean, maybe – probably not – and there's so many claims being made, and so we tried to focus on claims that I would call over-the-line claims. If you were claiming that marijuana could treat cancer or reverse Alzheimer's disease, those were over-the-line claims and we

took enforcement action on the basis of those claims. And I suspect the agency will continue to do that. That's been the historical position of the agency.

But this is so far down the field at this point that I think there's going to be eventually some federal reckoning where the federal government at some point is going to have to contemplate a federal scheme for better or for worse here that's going to put the federal government back into some kind of harmony with the states where there's some kind of federal regulation.

It's unfortunate because I think it's going to further potentiate use of a compound that ultimately— at least in the developing brain, we now have enough evidence to suggest it has some real concerns.

Now we also have sort of superimposed on that the nicotine use, rising nicotine use among kids, watching 20% and rising rates of e-cigarette use among teens. So you have teens interchangeably using their vaping product with THC on weekends and nicotine on weekdays. And that's another natural experiment that I think is fraught with a lot of danger, the effect that that's going to have on a developing brain.

MS KODJAK: We are just about out of time, so I am going to take this moment to present you with what is a growing collection, I think, of National Press Club coffee mugs. We hope you'll come back and get a third at some point.

One final question, which anyone who follows you on Twitter will appreciate. What is on your socks today? [laughter] I'm objectifying him.

DR. GOTTLIEB: They're just hot pink. There's no pattern on them.

MS KODJAK: No pattern? Hot pink socks. You can take a look later.

DR. GOTTLIEB: [laughter]

MS KODJAK: Thank you very much, Dr. Gottlieb, for joining us today. [applause]

[sounds gavel] We are adjourned.

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