ORAL ARGUMENT NOT YET SCHEDULED
No. 25-5133

UNITED STATES COURT OF APPEALS
FOR THE DISTRICT OF COLUMBIA CIRCUIT

VERTEX PHARMACEUTICALS INC.,
Plaintiff-Appellant,

v.

U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES, et al.,
Defendants-Appellees.

On Appeal from the United States District Court
for the District of Columbia
No. 1:24-cv-02046-JEB (Honorable James E. Boasberg)

BRIEF OF *AMICI CURIAE* PHARMACEUTICAL RESEARCH AND MANUFACTURERS OF AMERICA & BIOTECHNOLOGY INNOVATION ORGANIZATION

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CERTIFICATE AS TO PARTIES, RULINGS, AND RELATED CASES

Pursuant to D.C. Circuit Rule 28(a)(1), amici certify:

A. Parties and Amici. Except for *amici curiae*, including Biotechnology Innovation Organization and Pharmaceutical Research and Manufacturers of America, all parties and amici appearing before the district court and in this Court are listed in the Brief for Appellant.

B. Ruling Under Review. References to the ruling at issue appear in the Brief for Appellant.

C. Related Cases. *Amici* is unaware of any related cases pending before this Court or any other court.

/s/ *Kwaku A. Akowuah*
Kwaku A. Akowuah

RULE 26.1 DISCLOSURE STATEMENT

Pursuant to Rule 26.1 of the Federal Rules of Appellate Procedure and Circuit Rule 26.1, *amici* respectfully submit the following Corporate Disclosure Statements:

Amicus curiae Pharmaceutical Research and Manufacturers of America (PhRMA) is a trade association with its headquarters in the District of Columbia. PhRMA has no parent corporation, and no publicly held company owns 10 percent or more of its stock. PhRMA's member companies are listed on its website at https://phrma.org/en/About.

Amicus curiae Biotechnology Innovation Organization (BIO) is a trade association headquartered in the District of Columbia. BIO has no parent corporation, and no publicly held company owns 10 percent or more of its stock. BIO's member companies are listed on its website at https://www.bio.org/member/bio-member-directory.

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GLOSSARY

BIO	Biotechnology Innovation Organization
CAR	Chimeric Antigen Receptor
PhRMA	Pharmaceutical Research and Manufacturers of America

INTEREST OF *AMICI CURIAE*[1]

This brief is submitted on behalf of *amici* BIO and PhRMA.

BIO is the premier biotechnology advocacy organization representing biotech companies, industry leaders, and state biotech associations in the United States and more than 35 countries around the globe. BIO members range from biotech start-ups to some of the world's largest biopharmaceutical companies—all united by the same goal: to develop medical and scientific breakthroughs that prevent and fight disease, restore health, and improve patients' lives. Consistent with this mission, BIO's members pioneered the field of cell and gene therapies and continue to drive the field's advancement. BIO also organizes the BIO International Convention and a series of annual conferences that drive partnerships, investment, and progress within the sector.

PhRMA represents the country's leading innovative biopharmaceutical research companies, which are focused on developing

[1] All parties have consented to the filing of this *amicus* brief. No counsel for a party authored this brief in whole or in part, no party or counsel for a party contributed money that was intended to fund preparing or submitting this brief, and no person other than *amici* or its counsel contributed money that was intended to fund the preparation or submission of this brief.

innovative medicines that transform lives and create a healthier world. Together, PhRMA's members are fighting for solutions to ensure patients can access and afford medicines that prevent, treat and cure disease. PhRMA member companies have invested more than $850 billion in the search for new treatments and cures over the last decade, supporting nearly five million jobs in the United States.

Many of *amici*'s members create patient assistance programs to broaden patient access to life-changing medical treatments, including in settings where the government's own policy design creates barriers to access. The government's overbroad interpretation of the Anti-Kickback Statute, endorsed by the district court, threatens these essential programs, and with them patient access to innovative and life-changing medical treatments. *Amici* therefore have a strong interest in the proper interpretation of the Anti-Kickback Statute.

SUMMARY OF ARGUMENT

This case presents an important question about the scope of the Anti-Kickback Statute that is certain to recur as cell and gene therapies and other complex breakthroughs become a more prominent part of our arsenal for combatting serious disease.

Cell and gene therapies hold tremendous promise for treating, and even curing, previously incurable diseases, such as blood disorders and various cancers. And they reflect significant breakthroughs in innovation based on years of research and development. For certain of these diseases, cell and gene therapies are the only treatment option, where there previously were none.

A patient with a normally functioning immune system cannot be successfully treated with some cell and gene therapies, because the patient's immune system will reject the therapy. The patient's immune function must therefore be suppressed before treatment. And under the current standard of care, immune suppression is most commonly achieved through chemotherapy.

Chemotherapy, of course, is arduous and carries health risks all its own, including the risk of damage to various organ systems. Among the risks of chemotherapy is compromising or perhaps even destroying the patient's ability to have children. The precise degree of risk depends on the specific chemotherapy regimen involved, but the risk can be high or even almost certain.

In the decision below, the district court held that the Anti-Kickback Statute precludes a pharmaceutical company from providing financial assistance to a patient to afford fertility preservation services (services such as egg, embryo, or sperm freezing for patients) for which the patient would otherwise pay out-of-pocket, thus mitigating one of the known and expected—and devastating—side effects associated with the conditioning regimen necessary to the company's groundbreaking gene therapy. Specifically, the company's drug, CASGEVY, is a potentially curative treatment for two serious congenital blood disorders—sickle cell disease and transfusion-dependent beta-thalassemia—that collectively afflict tens of thousands of Americans. The district court agreed with the government that the patient assistance program, which provides financial support for fertility preservation services for which the patient would otherwise likely pay out-of-pocket, unlawfully "induces" patients suffering from sickle cell disease and transfusion-dependent beta-thalassemia to seek treatment with CASGEVY. The court also relied in part on the theory that such assistance could be "conceivably considered a 'reward'" for taking CASGEVY.

That ruling was mistaken. There are no "rewards" involved when a patient with a serious genetic disease must undergo fertility-compromising chemotherapy to realize the benefits of a potentially curative treatment prescribed by their doctor. Assistance programs like the one at issue here simply seek to provide compassionate patient support to mitigate a devastating side effect of the treatment (infertility), much as physicians seek to mitigate other risks of the chemotherapy course (such as lung or liver damage). No patient would choose to suffer sickle cell disease or transfusion-dependent beta-thalassemia in order to access fertility preservation services. Nor would any patient choose to go through such an intensive treatment regimen for the purpose of fertility preservation. Patients choose to seek treatment for relief of their debilitating disorders, not to obtain mitigating assistance for a harm caused by the treatment itself. The program thus cannot be considered an "inducement" or a "reward" for taking CASGEVY, and offering the program should not be criminal.

The district court's contrary conclusion raises serious constitutional concerns. Its reading of the statute renders commonplace and compassionate conduct potentially criminal—punishing charitable

assistance to those who need help countering an expected side effect of medical treatment. The breadth of this reading invites arbitrary enforcement by agencies and prosecutors, threatening the due process rights of those subject to the law. Rather than adopt an "inclusive" interpretation of the Anti-Kickback Statute, as the district court did, this Court should adopt a narrowing construction that criminalizes only corrupt conduct and that leaves room for conduct that improves patient outcomes. *E.g.*, *McDonnell v. United States*, 579 U.S. 550, 576 (2016).

The district court's decision is also detrimental from a policy standpoint. It threatens to have a chilling effect on innovative research with the potential to cure or end symptoms for devastating diseases. The future health of the patients who need these treatments depends in part on broadening patient access to life-changing medical treatments like the one at issue here. The district court's decision should be reversed.

ARGUMENT

I. Cell And Gene Therapies Are Innovative, Life-Changing Treatments, But The Therapies Themselves Are Complex To Administer And Carry Serious Side Effects.

Cell and gene therapies are a rapidly growing class of treatments that represent a paradigm shift in treating and potentially curing a wide range of conditions, from sickle cell disease to cancer to lupus. Cell

therapy involves the transfer of live cells into a patient to cure or ameliorate a disease.[2] Similarly, gene therapy replaces or alters a patient's disease-causing genes to treat a genetic disorder.[3]

Nearly fifty cell and gene therapies have been approved by the Food and Drug Administration for marketing in the United States.[4] These include chimeric antigen receptor (CAR) T-cell therapies that modify the genes in a person's white blood cells to help the body fight cancer.[5] And they include gene therapies for rare inherited conditions from sickle cell disease to spinal muscular atrophy. There are more than 400 cell and gene therapies in various stages of clinical development in the United

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[2] *See* Am. Soc'y of Gene & Cell Therapy, *Gene and Cell Therapy FAQs*, https://www.asgct.org/education/more-resources/gene-and-cell-therapy-faqs (last visited Aug. 27, 2025).

[3] *See* Nat'l Heart, Lung, & Blood Inst., *What are Genetic Therapies?*, https://www.nhlbi.nih.gov/health/genetic-therapies (last updated Mar. 24, 2022).

[4] FDA, *Approved Cellular & Gene Therapy Products*, https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products (last updated Aug. 15, 2025).

[5] *See* Am. Cancer Soc'y, *CAR T-cell Therapy and Its Side Effects*, https://www.cancer.org/cancer/managing-cancer/treatment-types/immunotherapy/car-t-cell.html (last revised July 7, 2025).

States,[6] including therapies with the potential to cure inherited disorders and autoimmune diseases, or treat infections like HIV.[7]

Treatment with a cell or gene therapy can be life-changing for patients. Many of the diseases these therapies target are life-threatening and historically difficult to treat—like sickle cell disease. The new cell and gene therapies are often one-time treatment processes that "can correct underlying causes of a disease, address symptoms, and halt disease progression."[8]

There are significant barriers to patients' accessing these treatments, however. Administering a cell or gene therapy is often a months-long process from preparation to administration and post-administration monitoring. Although there are different types of cell and gene therapies, many such therapies start with preparatory treatments

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[6] This figure reflects PhRMA analysis of public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. Data current as of May 30, 2025.

[7] Am. Soc'y of Gene & Cell Therapy, *Gene, Cell, + RNA Therapy Landscape Report* (2024), www.asgct.org/global/documents/asgct-citeline-q1-2024-report.aspx .

[8] *See* Ctr. for Medicare & Medicaid Servs., *Cell and Gene Therapy (CGT) Access Model*, https://www.cms.gov/cell-and-gene-therapy-cgt-access-model (last updated Aug. 19, 2025).

(including extraction of a patient's own cells) and a conditioning regimen that decreases activity in the patient's immune system to prevent a response that rejects the infusion of new cells or genetic material. These pretreatments and conditioning regimens are essential to the success of the cell or gene therapy. But they can be arduous, and often involve drugs—such as chemotherapy—that carry serious side effects, including infertility.[9]

For example, CAR T-cell therapy is an oncology treatment that currently involves multiple steps, often including a weeks-long hospital stay. To start, one or more outpatient procedures are performed to collect cells from the patient's blood. These procedures, which separate the patient's white blood cells and then return the remaining blood to the body, take several hours and have associated risks. Then, the patient must wait several weeks for the product manufacturing process, which typically includes modification and multiplication of cells in a lab, followed by testing to ensure product specifications are met. A few days before the modified cells are infused back into the patient, the patient is

[9] *See* Am. Soc'y of Gene & Cell Therapy, *Cell Therapy Basics*, https://patienteducation.asgct.org/gene-therapy-101/cell-therapy-basics (last updated Dec. 18, 2023).

given a course of chemotherapy to lower the body's immune defenses and allows the CAR T cells a chance to destroy the cancer without the patient's body attacking the T cells.[10] After the chemotherapy, the patient receives the CAR T cells as an infusion. For several weeks after the infusion, the patient must be checked regularly at the hospital—a level of monitoring that sometimes requires the patient to stay at or near the hospital for an extended period.[11]

Vertex's therapy for sickle cell disease and transfusion-dependent thalassemia provides another illustration of the difficulties of administering cell and gene therapies. A patient's treatment process for CASGEVY begins by preparing for stem cell collection. This may involve red blood cell transfusions to ensure that the patient has enough working blood cells for a successful collection. Stem cells are collected at authorized treatment centers with specialized training in administering

[10] *Cell Therapy Basics*, *supra*.

[11] Because these therapies are only given in specialized medical centers, some patients and caregivers must travel significant distances, sometimes requiring multi-night stays, many times during their treatment journey. Post-therapy, some patients and their caregivers may need to stay near the treating medical center to monitor for, and potentially treat, life threatening side-effects, again requiring prolonged lodging.

CASGEVY, and patients typically require two cycles of collection, separated by a minimum of fourteen days. After the patient's blood stem cells are collected, they are shipped to a lab where the cells are edited and the patient's individual treatment dose is manufactured—a process that takes up to six months.[12]

Once the treatment is ready, the patient must return to the treatment center to undergo chemotherapy conditioning and receive their infusion. The chemotherapy, which takes several days, removes the patient's existing blood stem cells from the bone marrow to prepare the marrow for the edited stem cells. High doses of the chemotherapy drug busulfan are required to accomplish this.

Busulfan carries known serious side effects—including increased risk of infections, pulmonary fibrosis that can cause difficulty breathing, and liver or kidney damage.[13] And like many chemotherapy medications, busulfan, especially in high doses, may cause permanent infertility,

[12] *See* CASGEVY, *The CASGEVY Treatment Journey*, https://www.casgevy.com/sickle-cell-disease/treatment-journey (last visited Aug. 27, 2025).

[13] *See* NIH, *Busulfan*, Nat'l Libr. Of Med., https://pubchem.ncbi.nlm.nih.gov/compound/Busulfan (last visited Aug. 27, 2025).

particularly in female patients.[14] After the course of chemotherapy, the patient receives their CASGEVY as a one-time infusion into the blood. Each patient remains at the treatment center to be closely monitored, for as long as four to six weeks.[15]

Although difficult to administer, CASGEVY is incredibly effective. Unlike earlier non-cell therapy treatments for both sickle cell disease and transfusion-dependent beta-thalassemia, CASGEVY does more than just treat symptoms—it targets the diseases' causes. JA 134, 152. In clinical trials, it eliminated severe, painful, vaso-occlusive crises in 94% of patients with sickle cell disease and ended red blood cell transfusion dependence for 91.4% of transfusion-dependent beta-thalassemia patients. JA 132, 585.

II. Vertex's Program Does Not "Induce" Patients To Purchase CASGEVY, Even If "Induce" Takes Its Ordinary Meaning.

For the reasons Vertex outlines in its brief, the Anti-Kickback Statute is best read to use the term "induce" in its specialized, criminal-

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[14] *See* Am. Cancer Soc'y, *How Cancer and Cancer Treatment Can Affect Fertility in Women*, https://www.cancer.org/cancer/managing-cancer/side-effects/fertility/how-cancer-treatments-affect-fertility-women.html (last visited Aug. 27, 2025) (listing busulfan as one of the chemotherapy medications most likely to cause infertility).

[15] *See The CASGEVY Treatment Journey*, *supra*.

law sense. Br. at 26–39. Under that reading, "induce" captures only corrupt conduct akin to soliciting or facilitating illegal activity. *See United States v. Hansen*, 599 U.S. 762, 776 (2023). And Vertex's program does not qualify. As Vertex persuasively explains, it does not operate its program with the corrupt intent to encourage any patient or provider to unlawfully purchase or prescribe CASGEVY. Br. at 48–51. That straightforward logic is sufficient to determine that the Vertex program does not violate the statute.

The Court may also reach the same conclusion by an alternate path. That is, even if this Court agrees with the government and the district court that the Anti-Kickback Statute uses the word "induce" in its ordinary-language sense, it should conclude that Vertex's program does not violate that law and should reverse the decision below. As ordinarily used, the word "induce" does not cover the mitigation of an expected harm associated with an already-desired activity. And that is what Vertex's program does: For patients already prescribed CASGEVY by their doctors, that program provides an opportunity to address a devastating side effect caused by the chemotherapy that is medically necessary prior to administration of CASGEVY. The district court therefore erred in

concluding that Vertex's program is unlawful, even if the word "induce" is read as the government prefers. *See* Op. 36–37.

At the government's urging, the district court held that the word "induce" carries its ordinary meaning, rather than its specialized, criminal-law meaning. As the district court explained, in common parlance, "induce" means to "'entice or persuade another person to take a certain course of action.'" Op. 15 (quoting *Induce*, Black's Law Dictionary (11th ed. 2019)). Other definitions confirm that, to qualify as inducement, the enticement or persuasion should "move" the other person, *Induce*, Merriam-Webster Dictionary ("to move by persuasion or influence"), or "prevail on" that person. *Id.* (also defining "induce" to mean "bring on or about, to affect, cause, to influence to an act or course of conduct, lead by persuasion or reasoning, incite by motives, prevail on" (quoting *Induce*, Black's Law Dictionary (4th ed. 1968)). Highlighting this feature of inducement, a dictionary published contemporaneously with the passage of the Anti-Kickback Statute supplies a telling example: "I was induced to come against my will." *Longman Dictionary of Contemporary English* 570 (1978). These definitions, including those

relied on by the district court, show that inducement requires more than counteracting a harm associated with a desired course of action.

Vertex's program thus does not "induce" patients to pursue CASGEVY. CASGEVY is potentially curative treatment for sickle cell disease or transfusion-dependent thalassemia, serious and painful conditions that, until recently, had no cure. Vertex's program is available only to patients suffering from those disorders *after* they have been prescribed CASGEVY. JA 166–67. At the patient's option, the program mitigates a potential harm, infertility, that is almost certain to arise as a result of the chemotherapy administered to prepare for a successful infusion of CASGEVY. The program thus aims to preserve the status quo ante by maintaining the patient's ability to have a family, despite the debilitating effects of chemotherapy. The program therefore addresses a potential harm caused by the conditioning regimen that is a necessary precursor to the treatment but offers no benefit to patients independent of the treatment that could "entice" or "persuade" them to take CASGEVY "against [their] will."

Reaching the opposite conclusion, the district court reasoned that Vertex's program qualified as "induce[ment]" because the program was

adopted "to motivate" patients and providers to choose CASGEVY. Op. 36. That reasoning misses the mark, in part because it is insufficiently attuned to the real-world choices that patients and their families face when deciding whether to proceed with this treatment. Patients suffering from the blood disorders treated by CASGEVY experience excruciating pain and shortened life span (in the case of sickle cell disease) and severe anemia and a lifetime of blood transfusions (in the case of transfusion-dependent thalassemia). Patients are motivated to go through the array of financial and medical hardships associated with treatment to potentially cure their underlying diseases. They are not motivated to seek treatment to receive reimbursement for fertility preservation services—services that become necessary only as a result of the treatment. Vertex's program does not motivate a patient whose disorder can be treated by CASGEVY to take CASGEVY. Instead, the program is merely a compassionate option for patients who may otherwise face the medical hardship of sacrificing their fertility to cure their life-altering disease.

Returning to the statute's terms, no one would say that a physician "induces" a patient to undergo a CASGEVY treatment by taking steps to mitigate the risk that the busulfan dose will cause liver or lung damage.

Likewise, it is unreasonable and unnatural to conclude that treatment to mitigate the risk of a different side effect—infertility—is an "inducement" that might "motivate" a patient to act against what would otherwise be her will and embark on a difficult treatment process. And for much the same reason, the district court erred in suggesting that financial support for fertility services could be characterized as a "'reward' or 'recompense' to patients or providers." Op. 36. No one would undergo an arduous course of chemotherapy that risks harm to their fertility, and risks other serious complications, for the "reward" of fertility preservation services.

III. THE GOVERNMENT'S SWEEPING INTERPRETATION OF THE ANTI-KICKBACK STATUTE CRIMINALIZES COMMONPLACE CONDUCT AND ENCOURAGES ARBITRARY ENFORCEMENT.

"[E]xpansive interpretation[s]" of criminal statutes that penalize "commonplace" conduct "raise significant constitutional concerns." *McDonnell*, 579 U.S. at 574–75. Those constitutional concerns compel a narrowed reading of the Anti-Kickback Statute here—even if the overbroad reasoning shared by the government and the district court were plausible.

The Due Process Clause "requires that a penal statute define the criminal offense . . . in a manner that does not encourage arbitrary and discriminatory enforcement." *Kolender v. Lawson*, 461 U.S. 352, 357 (1983). Absent "minimal guidelines to govern law enforcement, . . . a criminal statute may permit 'a standardless sweep [that] allows policemen, prosecutors, and juries to pursue their personal predilections." *Id.* at 358 (internal quotation marks omitted). But a regime where everyone is guilty and the government can pick and choose whom to prosecute is antithetical to the rule of law. Recognizing this, the Supreme Court has "declined to rely on 'the Government's discretion' to protect against overzealous prosecutions," instructing instead that a criminal statute "'that can linguistically be interpreted to be either a meat axe or a scalpel should reasonably be taken to be the latter.'" *McDonnell,* 579 U.S. at 576 (quoting *United States v. Sun-Diamond Growers*, 526 U.S. 398, 408, 412 (1999)).

In line with its own instruction, the Supreme Court has repeatedly given statutes narrowing constructions to avoid criminalizing commonplace conduct. In *McDonnell*, for example, the Supreme Court rejected a "sweep[ing]" reading of federal bribery law that would "subject"

public officials "to prosecution, without fair notice, for the most prosaic interactions" with constituents. *Id.* "'Invoking so shapeless a provision to condemn someone to prison' for up to 15 years raises the serious concern that the provision 'does not comport with the Constitution's guarantee of due process,'" the Court explained. *Id.* (quoting *Johnson v. United States*, 576 U.S. 591, 602 (2015)). And in *United States v. Kozminski*, 487 U.S. 931, 948 (1988), the Court limited the reach of statutes prohibiting "involuntary servitude" to cases "involving the compulsion of services by the use or threatened use of physical or legal coercion." A broader interpretation advanced by the government "appear[ed] to criminalize a broad range of day-to-day activity" and "would delegate to prosecutors and juries the inherently legislative task of determining what type of coercive activities are so morally reprehensible that they should be punished as crimes," exposing "individuals to the risk of arbitrary or discriminatory prosecution and conviction." *Id.* at 949.

The interpretation of the Anti-Kickback Statute advanced by the government and adopted by the district court raises the same sorts of due process concerns as the statutes interpreted narrowly in cases like *McDonnell* and *Kozminski*. As discussed, the district court reasoned that

the Anti-Kickback Statute prohibits any compensation given with even a partial "motive" to "influence" patients or providers to make a particular choice about medical care, Op. 36—even if that compensation provides patients with no benefit independent from the treatment itself. The district court itself described this as an "inclusive definition." That interpretation would criminalize "commonplace" and "innocuous" interactions involving providers or patients. *Snyder v. United States*, 144 S. Ct. 1947, 1952 (2024) (rejecting government's reading of bribery statute that would have swept up "commonplace" and "innocuous" gratuities). The logic of the district court's decision would criminalize charities, fertility preservation clinics, friends, or family members who chose to pay for a CASGEVY patient's fertility preservation services, just as it criminalizes Vertex's assistance to patients for that purpose. And "nothing but the Government's discretion prevents" these plainly innocuous "examples from being prosecuted." *Sun-Diamond Growers of Cal.*, 526 U.S. at 408.

The Department of Health and Human Services' Office of Inspector General's ability to issue written advisory opinions about what the Anti-Kickback Statute prohibits does not fix the problem. *See* 42 U.S.C.

§ 1320a-7d(b)(2). "Under the 'standardless sweep' of the Government's reading," *McDonnell*, 579 U.S. at 576, any financial arrangement involving federal healthcare programs presumptively violates the Anti-Kickback Statute. Under the Inspector General's interpretation, the advisory opinion process becomes a subjective exercise, in which the only real question that remains is whether the Inspector General will decline to "impos[e] . . . a sanction" for a particular violation. 42 U.S.C. § 1320a-7d(b)(2)(E). In that regard, any relief an advisory opinion establishes is situation-specific. And according to the Inspector General's Office, nothing it says in answering that enforcement-discretion question is judicially reviewable. *See Pharm. Coal. for Patient Access v. United States*, No. 3:22-CV-714 (RCY), 2024 WL 187707, at *15 (E.D. Va. Jan. 17, 2024), *aff'd*, 126 F.4th 947 (4th Cir. 2025). Accordingly, the Inspector General's advisory opinion process heightens, rather than assuages, concerns about arbitrary enforcement.

The harsh nature of the statutory penalties further exacerbates the constitutional concerns with the government's overbroad reading. An individual convicted for violating the Anti-Kickback Statute faces ten years in prison. 42 U.S.C. § 1320a7b(b). Convicted entities can be

excluded from participating in government healthcare programs and barred from contracting with the government, 2 C.F.R. §§ 180.800, 376.10—"likely a death knell for any company," *United States v. Facteau*, No. 15-CR-10076-ADB, 2020 WL 5517573, at *1 (D. Mass. Sept. 14, 2020). Even if courts or juries would ultimately find some financial arrangements by pharmaceutical companies to be lawful, the risks of substantial prison time or severe financial consequences will chill healthcare companies from adopting those arrangements, preventing patients from getting the treatments they need. *See infra* Part IV.

This Court should avoid that result by rejecting the government and the district court's expansive interpretation of the Anti-Kickback Statute, in favor of a narrower construction that leaves room for "acceptable and beneficial conduct." *Ruan v. United States*, 597 U.S. 450, 459 (2022) (adopting narrower construction of the Controlled Substances Act to "diminish the risk of 'overdeterrence'").

IV. Patient Assistance Programs Designed To Improve Access To Innovative Cell And Gene Therapies Are Essential And Should Be Embraced Rather Than Criminalized.

Criminalizing assistance programs designed to offset serious treatment side effects will ultimately harm patients and the public

health. Rulings like the district court's decision here could chill innovation and investment in cell and gene therapies and hinder patient access to these life-changing treatments.

As discussed, cell and gene therapies are a promising and rapidly expanding realm of new treatments. The Food and Drug Administration has already approved nearly 50 products that cure or treat multiple forms of cancer, genetic disorders, and more. And researchers and pharmaceutical companies have hundreds of cell and gene therapies in the development pipeline in the United States.

To give just one example, multiple clinical trials are underway to study how CAR T-cell therapy could revolutionize treatment for autoimmune diseases, such as lupus—a disease that affects about 1.5 million Americans.[16] The symptoms of lupus and other autoimmune disorders are currently managed with immunosuppressive medications that can have serious side effects that must be tolerated over decades. But recent studies show that CAR T-cell therapy could eliminate or

[16] *See* U. Chi. Med., *Clinical trials to study new use for CAR T-cell therapy: Treating autoimmune diseases* (Jan. 21, 2025), https://www.uchicagomedicine.org/forefront/immunotherapy-articles/car-t-cell-therapy-treating-autoimmune-diseases.

reduce disease biomarkers and symptoms of autoimmunity, with just a single infusion.[17]

Administration of these newly developed cell and gene therapies, like administration of the existing therapies described above, will demand multi-step preparatory treatments that carry serious risks and may require long hospital stays. These represent real barriers to patients' access to current and future cell and gene therapies that could cure or halt the symptoms of devastating diseases.

Patient assistance programs that counteract side effects and mitigate other difficulties associated with the conditioning for these treatments will be essential for patient access. The Center for Medicare and Medicaid Services knows this. Last year, that agency announced a pilot program to increase access to cell and gene therapies among Medicaid beneficiaries. The initial focus of the pilot is on access to gene therapy treatments for people living with sickle cell disease. Tellingly, pharmaceutical manufacturers participating in the government's own

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[17] Liam Connolly, *A breakthrough for lupus treatment? Study explores CAR T-cell therapy for autoimmune disease*, U. Cal. Davis Health (May 13, 2024), https://health.ucdavis.edu/news/headlines/a-breakthrough-for-lupus-treatment-study-explores-car-t-cell-therapy-for-autoimmune-disease/2024/05.

pilot program are required to cover certain fertility preservation services, because lack of access to those services "presents a significant access barrier to individuals" considering this therapy. JA 599.

Another example is manufacturer travel assistance to patients and caregivers, which makes it possible for patients to endure the lengthy hospital stays sometimes associated with treatment. *See, e.g.*, Off. of Inspector Gen., *Advisory Op. No. 24-13* at 3 (December 31, 2024); Off. of Inspector Gen., *Advisory Op. No. 25-05* (June 22, 2025); Off. of Inspector Gen., *Advisory Opinion No. 25-06* (July 2, 2025). Last year, for example, a manufacturer offering a T-cell immunotherapy product to treat tumors proposed to the Inspector General to provide travel assistance, lodging, and a stipend for a patient and a caregiver to travel to a treatment center for a month to receive pre-treatment chemotherapy, the infusion, and post-treatment monitoring. *See Advisory Op. No. 24-13*. The Inspector General determined that the arrangement implicated the Anti-Kickback Statute, because it could "induce" patients to purchase the manufacturer's product. *Id.* at 6. Unlike with Vertex's program, however, the Inspector General ultimately exercised its discretion to issue a favorable advisory opinion, concluding that the travel assistance

program presented a "sufficiently low" "risk of fraud and abuse," thereby eliminating the risk of a sanction for the arrangement. *Id.*

The stakes for patients are too high to rely on the Inspector General's goodwill to ensure access to life-changing cell and gene therapies. Both types of assistance programs should be permitted—not as a matter of the Inspector General's grace—but because they are not criminal acts under the Anti-Kickback Statute. That law is meant to ensure that patients receive appropriate treatment, free from the influence of bribery and other improper incentives, not to push them to forego treatment by forcing them to endure otherwise treatable side-effects. Manufacturer assistance to address side effects of the conditioning treatments, including but not limited to infertility, is a compassionate and charitable act designed to help patients avoid the terrible dilemma of having to choose between foregoing the future possibility of building a family and seeking a cure for one's devastating disease.

The district court's ruling tells manufacturers that these support programs may be criminal, chilling those efforts to help patients access life-changing therapies. This Court should send the opposite message:

Patient access programs such as these do not violate the law, which does not force patients to choose between a life-saving treatment and a family. Programs like the compassionate patient assistance here are lawful and essential.

CONCLUSION

The district court's judgment should be reversed.

Respectfully submitted,

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CERTIFICATE OF COMPLIANCE

This brief complies with the typeface requirements of Fed. R. App. P. 32(a)(5) and type-style requirements of Fed. R. App. P. 32(a)(6) because it has been prepared in a proportionally spaced typeface using Microsoft Word in 14-point Century Schoolbook font.

This brief complies with the word-count limitation of Fed. R. App. P. 29(a)(5) and any applicable scheduling order. This brief contains 4,886 words, not counting the parts excluded by Fed. R. App. P. 32(f) and Circuit Rule 32(e)(1).

/s/ *Kwaku A. Akowuah*
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CERTIFICATE OF SERVICE

I hereby certify that on August 28, 2025, I will cause the foregoing document to be electronically filed through this Court's CM/ECF system. Participants in the case who are registered CM/ECF users will be served by the CM/ECF system.

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