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02.04.2026

Tags: USA, Policy, Regulation, FDA, Rare Diseases, CGT

Paul Kim, a leading FDA attorney and policy expert formerly at both the FDA and the US Congress, discusses the current shifts within the American life sciences and regulatory landscape. Kim examines the prevailing sentiment of the industry amidst a period of significant structural upheaval and regulatory whiplash. He offers a critical analysis of the National Priority Voucher (CNPV) pilot program, deconstructing the risks of allowing economic policy to outpace scientific rigor. Kim further addresses the specific bottlenecks currently stifling the cell and gene therapy (CGT) sector, the necessity of permanent legislative solutions for the Rare Pediatric Disease Priority Review Voucher program, and the vital role of congressional oversight in re-establishing the stability and trust essential to the US regulatory engine.

We are now roughly one year into the second Trump Administration, a period marked by significant structural and policy shifts across the healthcare and life sciences sectors. Looking at the landscape today, what is the prevailing sentiment within the industry regarding these changes?

I believe the life science community—innovative companies, investigators, patients, and academic researchers alike—collectively feels we are weathering an unprecedented storm. This is a tumultuous time where the very fabric of biomedical innovation, and the foundational assumptions we have operated on successfully for a generation, have been thrown aside. We see an Administration prepared to take extra-legal actions that defy standing law, disregard congressional

oversight, and, in some cases, ignore the courts.

That sense of capriciousness is antithetical to the stability and predictability we intentionally built into the system over 30 years through acts of Congress and layers of regulatory guidance. Those mechanisms at the FDA have been thrown into question.

When I started working on FDA policy 35 years ago, there was an inherent drug gap: innovative molecular entities were being authorized in Europe and Japan long before the US because the FDA was heavily under-resourced. It's easy to forget how much has changed since then through enactments like PDUFA, consistent bipartisan oversight, and foundational steps like the Orphan Drug Act of 1983. These milestones are now being challenged. Instead of engendering innovation, the current environment is discouraging it.

I often wonder if the FDA's lawyers are even being consulted on some of these recent policy choices. The volatility is perfectly exemplified by the Refuse to File (RTF) issued to Moderna. The alleged basis that the trial was not adequate and well-controlled is a reversal of decades of empirical and legal interpretation. If you polled a thousand experts in our sector, you would find an overwhelming consensus that the trial was, in fact, adequate. The agency's action was arbitrary and extra-legal.

We are seeing a trend of sponsors experiencing what I call regulatory whiplash. There is a deep-seated worry that commitments made during a pre-IND or pre-submission meeting will not hold up two or three years downstream—after tens of millions of dollars have been spent and hundreds of patients have been exposed to experimental therapies.

In my career, from the 90s to today, this level of uncertainty is unprecedented. It's particularly unfortunate because it didn't have to be this way. We didn't see this Wild West environment during the first Trump Administration. While Operation Warp Speed was an incredible achievement, we have now seen the departure of key architects like Peter Marks, who spoke clearly about a disregard for science and the imposition of policy before evidence. We now have a dynamic, led by figures like Dr. Prasad, that is engendering uncertainty not just at CBER, but across the entire agency. [CBER Director Prasad was dismissed from FDA a second and final time in March 2026]

The Administration recently introduced the National Priority Voucher (CNPV) pilot program, which has generated considerable uncertainty and debate. For our audience, could you clarify the core intentions of the CNPV, and more importantly, address the

specific ambiguities or structural issues that are currently causing concern within community?

The CNPV is an administrative creation designed to establish an extraordinarily rapid drug review pathway of just one to two months. To put that in perspective, the current PDUFA baseline for a priority review of a new molecular entity is six months. On the surface, this could be seen as a significant, non-legislative reform to address urgent, unmet medical needs.

However, the way the Administration has constructed the CNPV also involves what I would call a naked bargain—trading FDA efficiency for economic concessions. By making market pricing and Most Favored Nation (MFN) price agreements a criterion for selection, we have blurred the boundary between two separate bodies of law and administrative responsibility, essentially merging the mandates of the FDA and CMS.

If we look at the other selection criteria like responding to public health crises, innovative therapies, or unmet needs, these are all traditional concepts already baked into Section 506 of the Federal Food, Drug, and Cosmetic Act and its well-established expedited pathways like accelerated approval, Fast Track, Breakthrough, and Regenerative Medicine Advanced Therapy (RMAT) designation. These existing pathways are ‘meat and potatoes’ regulation that provide higher levels of engagement, technical assistance, and a collaborative back-and-forth between the agency and sponsors.

The CNPV attempts to do something different with its tumor board-style review, but it doesn’t offer anything new regarding conventional medical needs. Another new—and problematic—CNPV focus is on supply chain resilience and onshoring. While the agency does deal with drug shortages, its fundamental mission is to ensure the safety and effectiveness of products, not to serve as the Department of Commerce. When affordability and onshoring become the stage’s center, they distract from that core mission.

Perhaps most concerning is that the CNPV decisions are made behind a curtain. Unlike the section 506 pathways, where the doors are open to any sponsor that meet the statutory criteria for eligibility, the CNPV is an opaque process. Only a select number of special entities receive these vouchers, and they are being awarded based on political criteria and MFN agreements rather than transparent standards. This black box approach to awarding what are effectively golden tickets is a fundamental problem for the industry; FDA has no business choosing economic winners and losers behind a curtain.

You have previously commented on the inherent risks when policy initiatives lead ahead of scientific rigor in regulation. In your view, does the CNPV represent a case where policy is outstripping scientific frameworks, and what are the long-term implications if this trend continues?

It’s extraordinarily problematic. I want the agency to stay in its original lane and focus on the foundational criteria of unmet medical need and innovative therapies that it has inhabited for generations. Seeing leadership use public platforms to prioritize low drug prices gives me angina, because once that door is open, I don’t know where it ends.

The CNPV worries me not just as a standalone program, but as a wedge. By involving the agency in pharmacoeconomic considerations, we are walking a direct path toward Health Technology Assessments and comparative effectiveness becoming third rails for drug approvals in the U.S. There is a direct line from this policy to a future where administrative actions could suddenly make MFN agreements a criterion for other review pathways. We could foresee a dismal reality where six-month priority reviews are only granted to drugs that satisfy these third-rail economic demands.

When you take Inflation Reduction Act (IRA) drug pricing and MFN agreements and put them into a blender with FDA drug reviews, the wheels come off. It creates an indistinguishable mess for the sector. Venture capitalists and investigators making day zero decisions will no longer just be looking at safety and effectiveness—they will have to account for government-mandated marketability. Formalizing these economic concerns as part of the FDA’s business rather than leaving them to CMS or insurers is extraordinarily concerning.

We are seeing a lot of track being laid down for price controls to take wing over the next few years. The push to codify MFN agreements into statute is equally alarming, especially since the underlying executive orders expressly discuss the possibility of pulling FDA approvals as part of those agreements. This isn’t just a theoretical concern. It’s being enacted as policy on paper.

Ultimately, this erosive process breaches the foundational firewall between career scientists and political appointees. That firewall is essential to the credibility of the FDA’s gold standard. We are shifting toward a paradigm where investment decisions and reimbursement concessions could alter the standards for market entry. If that trend continues, it will fundamentally undermine the stability of the US’ historic biomedical and regulatory leadership.

The FDA Office of Therapeutic Products recently announced increased flexibility regarding CMC requirements for cell and gene therapies. From your perspective, does this represent a meaningful shift in regulatory philosophy, or is there still a need for clarification on what the actual expectations will be in practice?

If you were to ask me if this was groundbreaking or if it illuminated previously misunderstood corners of drug development, I would say no. In many ways, this was more of a repackaging exercise than a true shift in posture.

What is more concerning than the substance of the announcement is the medium. As I have noted, the agency is increasingly abandoning the conventional methods of conveying its thinking—such as formal guidance documents, Federal Register notices, and administrative rulemaking—in favor of webpages and press releases. The trouble with a press release is that you cannot file comments back to the agency. This effectively cuts off the dialogue between the regulator and the licensed community.

Standard guidances include a 60-day comment period, and Notice and Comment rulemaking is, by definition, designed to be a two-way street. By relying on press releases and unilateral webpage updates, the agency is signaling two things: first, that it’s not interested in what the community thinks about these policies, and second, that the message is largely performative.

To be clear, the flexibilities mentioned in the January announcement embody many of the positive strides CBER has made over the years. These tools are already built into existing expedited pathways and have been developed experientially through work with sponsors. So, while the content of the CGT announcement is not bad, it’s certainly not new. By framing existing practices as a new flexible approach without allowing for community input, the agency is choosing performance over the collaborative administrative process that provides the industry with true long-term clarity.

If you had to identify the primary policy bottlenecks currently stifling the progress of cell and gene therapies in the US, which specific areas would you point to?

I believe we have yet to experience the full impact of last year’s cataclysmic upheaval. Following the DOGE layoffs and initial 20 percent budget cut proposals, one out of every five people at the agency is now gone. Despite claims of restaffing, the FDA’s own data shows that the payroll has remained stagnant since the end of last year. These cuts, imposed in the first half of 2026, have

not been mitigated by impactful hiring.

As a result, we are going to see increasing friction: more missed opportunities, more written responses in lieu of face-to-face or hybrid meetings, and a decline in the quality of feedback. The loss of leadership and deep experience in these incredibly novel technological areas will inevitably bear on the agency's day-to-day operations.

While the 2025 tally of New Molecular Entities (NMEs) showed conformity to historic norms—around 35 approvals—I suspect we will see downward pressure on that number this coming year. This is due both to the reduced workforce and the prevailing volatility.

On the very day the agency announced its CMC flexibility for cell and gene therapies, Atara received a Complete Response Letter (CRL). This was a case where the public record suggests the agency reversed its own position on whether a single-arm trial was adequate to demonstrate efficacy. When you look at the collective data points from sponsors like Unicure, Capricor, Replimune, Biohaven, Atara, and Moderna, an unprecedented pattern of volatility emerges. We are seeing the agency backtrack on prior commitments, and that is absolute poison to early-stage innovation.

The US has historically led in rare disease innovation, largely due to the incentives provided by the Orphan Drug Act. However, the Priority Review Voucher (PRV) program was only recently reauthorized on a temporary basis. Given its importance to orphan therapy advancement, how vital is a permanent solution for the PRV program?

It's essential to recognize that the PRV program exists in three flavors. The tropical disease voucher was a bipartisan construct that is permanently authorized. However, the rare pediatric and medical countermeasure vouchers remain trapped in temporary cycles—the latter of which is currently expired. Permanent authorization for all PRVs is long overdue. While Congress initially wanted to observe how the program functioned, the consistent success across reauthorization cycles should have already triggered a permanent fix.

I liken the failure to permanently reauthorize these programs to a light switch: it's either on or off. If you leave a room dark for long enough, people will stop entering it. This uncertainty significantly attenuates the value of the voucher and its meaningful impact on innovation. If Congress has concerns about the program being an industry giveaway, they should use a dimmer switch rather than turning the lights off entirely. You can titrate the policy by capping the resale value of a

voucher or limiting the number of PRVs a single sponsor can receive. There are many ways to modulate the policy without abandoning it.

The current situation reveals two things: a profound level of congressional dysfunction and a failure to listen to patients. Organizations like NORD have produced reports demonstrating how these vouchers are used by small companies to develop first-time therapies for rare diseases where no alternatives exist. The patients themselves have made it clear that even if you stipulate that this is an economic incentive for industry, it's a giveaway worth making in terms of clinical outcomes.

This dysfunction is compounded by a shift in our legal environment. Following the *Loper Bright* decision, the Supreme Court has made it clear that agencies no longer have broad deference in their interpretations of the law. It's black-letter law or nothing. We saw this with the *Catalyst* decision regarding orphan drug exclusivity. For years, the FDA maintained stability in the rare disease space only by effectively disregarding an 11th Circuit court ruling.

In a post-*Loper Bright* world, agencies can no longer ignore the courts to maintain continuity. The Supreme Court has signaled that Congress must enact specific, clear statutes. The fact that it took so long for Congress to answer that call and correct the Catalyst decision and its interpretation of orphan drug exclusivity is not just a matter of partisanship. It's a concerning symptom of a breakdown in how our government is supposed to function.

With nearly 50 percent of novel drug authorizations now carrying an orphan drug designation, the landscape of rare disease has changed dramatically since the 1980s. Do you believe the current definition and criteria for orphan designation need to evolve to better reflect the current state of drug development?

The statistic that half of all NMEs are orphan drugs often creates a degree of cognitive dissonance for those who don't understand the market failures the Orphan Drug Act was designed to meet. While that number is high, I would argue that we should not be looking to recalibrate the Act or change its incentives. In fact, we should be doubling down. When you consider the vast iceberg of 10,000 rare diseases for which there are still no approved therapies, the level of unmet need remains staggering.

We do not need a new definition, instead, we need a focus on the interstitial stuff that the Act alone cannot solve. Experts like David Liu have suggested we have the scientific capability to cure thousands of rare or N-of-1 diseases within a few years. However, achieving that doesn't require

changing the FDA's foundational laws—it requires addressing the translation and manufacturing bottlenecks that currently stifle those efforts.

The Orphan Drug Act provides a solid hub, but we are missing the spokes. There is a critical lack of public-private partnerships and consortia to find validated biomarkers and endpoints for these ultra-rare conditions. Other key challenges—contract manufacturing for small batches, conducting natural history studies, and validating early-stage endpoints—are not strictly FDA concerns. This is the domain of translational research, where agencies like NCATS and ARPA-H should be leading.

The undiscovered country for rare disease innovation lies in federal coordination. Currently, the FDA and these research agencies rarely communicate. The federal government has a vital role to play in promoting collaborations, matchmaking for small-batch manufacturing, and enabling studies to happen sooner. This is where the mindset of supply chain resilience and onshoring should be applied to ensure that the infrastructure exists to bring a discovery through the valley of death to the rare disease patient.

Ultimately, this is a role for Congress. I don't want to see temporary administrative policies that may disappear with the next change in leadership. We need these translational solutions codified into law—creating a permanent foundation, much like the Orphan Drug Act itself, that the industry can reliably build upon for the next generation of cures.

As a final message to the community, what specific actions are required at the federal and FDA levels to re-establish the sense of trust and regulatory stability that the life sciences sector requires to thrive?

It's incumbent upon us as a community to recognize our responsibility in affecting long-term positive change. Over the past year, we have seen significant volatility. To counter this, our first priority must be ensuring that the career staff at the FDA have the air cover and resources necessary to do their jobs consistent with the law. We cannot expect high efficiency and extraordinary rigor if staff members are forced to work multiple roles due to budget cuts. We must enable them to maintain the old normal of a high-functioning, stable regulatory environment.

Reestablishing this stability is a political exercise. What is currently lacking is the rigorous congressional oversight required to hold the Administration accountable for its balls and strikes. As we look toward the reauthorization of PDUFA VIII, my expectations have become very fundamental: I just want the lights to stay on. We must ensure that reviewers can actually perform their work

without disruption. A timely, clean reauthorization of PDUFA is essential.

Furthermore, if programs like the CNPV are going to continue to exist, we must insist that Congress holds the pen. These pathways should be authored statutorily rather than through administrative whim. By codifying such programs, we can ensure they make sense, and crucially, ensure they do not drain resources from other vital reviews.

It’s not all darkness and depression—there are clear paths forward. However, it requires us to work collectively with Congress to move away from capricious administrative actions and back toward the permanent, predictable foundations of law.

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