

Reform or regulatory overreach? **The mixed legacy of Trump's FDA reforms**

Recent policy changes by the Trump administration have presented both challenges and opportunities for the biotechnology and pharmaceutical industries, which are facing up to the prospect of a 25%+ hike in tariffs this year – a measure aimed at boosting domestic production.

The tariffs imposed on the drug industry are the latest in a series of actions designed to accelerate innovation and reduce costs. Longer-term reforms also intend to fast-track drug approvals and relax regulatory oversight of digital health tools.

But the policy shifts have been met with both praise and concern, not least the US Food and Drug Administration's (FDA) increasingly flexible approach to drug approvals.

Reforms such as the expanded Accelerated Approval Pathway (AAP) and a push for adaptive clinical trial designs have allowed pharmaceutical companies to bring treatments to market more quickly.

The question now is whether these policies are creating a more efficient, innovation-driven regulatory framework—or if they risk undermining critical safeguards designed to protect public health.

AAP as a priority

The AAP ensured that Aduhelm (aducanumab), a drug used to treat Alzheimer's was approved in 2021 despite question marks over its efficacy and reliance on amyloid plaque biomarkers rather than clear cognitive improvement.

Likewise, new cancer immunotherapies, such as pembrolizumab received accelerated approval for multiple indications based on biomarker-driven data.

Other notable regulatory changes place an emphasis on digital health and innovation, in which concerted efforts have been made to pushed for greater integration of digital health tools and telemedicine within regulatory frameworks.

By promoting a more flexible approach, the FDA have been able to keep pace with rapid technological advancements.

"We [the FDA] have a lot of quantitatively gifted people and people with experience in digital technologies and computing at every level," said ex-FDA Commissioner Robert Califf, MD in a 2024 interview published in JAMA. *"...there are whole new areas that we're going to have to stay abreast of. It's very well understood that it's going to be a challenge. ...we've got good people, and I'm not worried about their basic understanding.*

"...we're doing better than most people imagine, but the field is changing exponentially, not linearly. So it is a worry. And as generative AI comes in and the whole space of consumer interactions with devices coming into play in the nonmedical area,..."

Although less visible, a range of resources were also allocated to the FDA to support these faster review timelines and cultivate a more dynamic regulatory environment.

The increase in funding gave the agency wider scope to expand its workforce and refine its review processes, supporting the accelerated approval of effective therapies.

The impact of shifting policies

By refining approval pathways and embracing real world evidence, the FDA has been able to authorise the market entry of promising new therapies.

Indeed, pharmaceutical firms have been able to make these drugs available much sooner to those most in need, where time is of the essence—such as oncology and rare genetic disorders.

Regarding their investigational gene therapy, isaralgagene civaparvovec (ST-920), **Sandy Macrae**, Chief Executive Officer of Sangamo Therapeutics, stated: *"I strongly believe in the potential for ST-920 to alleviate many manifestations of Fabry disease and am delighted to have a clear regulatory pathway that could bring this treatment to patients significantly sooner than originally anticipated."*

Other drug bosses, such as the CEO of Israel's Teva Pharmaceutical Industries, have demanded more saying: *"In the FDA (Food and Drug Administration), there is no speed."*

"I would like them to do things on a regular time because the more generics we bring, the more savings we bring to the United States healthcare system, so just approve them," Richard Francis told **Reuters**. *"Many of these products are approved in Europe."*

He added that drugs, such as Huntington's treatment Austedo, should be exempt from funding restrictions.

Trial designs and endpoints

But where the updated guidance has been most felt has been its impact on adaptive trial designs and acceptance of novel endpoints that have allowed companies to conduct more efficient clinical studies.

This flexibility has reduced both the cost and duration of clinical trials while improving data quality.

Regulators now have a more nuanced understanding of a drug's safety and efficacy, which in turn supports more informed decision-making.

This streamlining is certainly needed. As drugs enter each clinical trial stage, the spiralling costs would make even the biggest pharma corporations break out in a cold sweat. According to The Gates Foundation, a vaccine for tuberculosis, in which a smaller share of the population is infected during the study period, can cost up to $550m (€508m) for a phase 3 trial.

That is at the extreme end. A paper looking into vaccines for rotavirus reported an estimate of $2,000 to $3,000 per patient for phase 3 trials alone, whilst research published in The Lancet suggests that the 5,500-person trial of the R21 malaria vaccine could have cost around $11m (€10.1m).

The same could be said about novel endpoints such as surrogate markers, which also speed up the approval of new therapies.

The FDA's AAP currently uses surrogate endpoints to approve drugs for serious conditions, possibly cutting the time to market. However, this approach has raised questions about the strength of evidence about the drugs' effectiveness and the need for confirmatory trials post-approval.

Industry reaction

The impact of these reforms is already being felt with drug approvals moving faster and digital health innovations experiencing a wave of regulatory backing.

"By permitting earlier access to novel therapies, the accelerated approval pathway has helped extend, and even save patients' lives," said Eli Lilly and Company of AAP's benefits.

However, some argue that adaptive trial designs and novel endpoints have introduced efficiencies that benefit both patients and drug developers. Others



worry that the reduced reliance on traditional clinical endpoints could lead to gaps in long term safety data.
In a JAMA **study** that looked into the approval of drugs based on limited data, one of the researchers stated that they had found: *"numerous situations in which required confirmatory studies with rigorous designs and outcomes are not pursued or are not completed in a timely fashion."*
Further disruption came last month with the resignation of Dr Peter Marks, the FDA's top vaccine official, over disagreements with US Health Secretary Robert F. Kennedy Jr., a vocal critic of vaccines and Covid.
"The U.S. has long been recognized as the world leader in medical and scientific innovation and regulation, grounded in transparency and scientific rigor," said **John Crowley**, President and CEO of the Biotechnology Innovation Organization (BIO), in a written statement.
"It is imperative that we retain and recruit scientific expertise and strong leadership at our health agencies and that the high standards that are the hallmark of these health institutions are upheld and advanced."
Voicing similar concerns about the recent downsizing at the FDA, John Murphy III, President and CEO of The Association for Accessible Medicines (AAM) said: *"While we support improving FDA efficiency to deliver more affordable generic and biosimilar medicines to patients faster, many of the reported cuts appear to do the opposite."*
The cuts have appeared to gather momentum in recent weeks with senior FDA officials Peter Stein, Hilary Marston, and Jeanne Marrazzo, reported to have resigned, been laid off, and placed on administrative leave, respectively.

Future considerations

With one eye on the future, the trajectory of U.S. pharmaceutical policy under the Trump administration remains in a state of transition with reforms emphasising speed and flexibility.
However, signs that the regulatory pendulum may swing back toward stricter oversight, point towards the FDA's post-market surveillance policies.
With questions raised over the accelerated approval process, regulators may move to introduce more robust real-world evidence demands to ensure that expedited drugs deliver on their early promise.
Pricing reform is also a controversial issue too. While the administration leans towards transparency over price caps, growing pressure may force a shift toward more assertive cost-containment actions.
The future and subsequent success of U.S. pharma regulation hinges on the policies' balance between innovation, affordability, and public safety.
The key question remains: Can the industry sustain its newfound agility without compromising public trust?"

WILL CHU
Editor
24/7 BIOPHARMA

