VIEWPOINT

Priorities for a New FDA

Martin A. Makary, MD, MPH; Vinay Prasad, MD, MPH

Why does it take more than 10 years for a new drug to come to market? Why are childhood chronic diseases so prevalent? And how can regulators adapt to meet the challenges facing clinicians today? These questions are at the forefront for the US Food and Drug Administration (FDA).

The US leads the world in sophisticated cell and gene therapies and other innovative treatments, but in terms of the health of the population, our medical system has been a 50-year failure. Forty percent of US children now have a chronic medical condition[1] and 1 in 6 has a neurodevelopmental disorder.[2] Life expectancy has plateaued or fallen and is not commensurate with health care spending.

Over recent decades, obesity, diabetes, colon cancer, depression, and autoimmune diseases have increased in young people, and early-onset Alzheimer disease has increased by at least 300%.[3] Historical and comparative global data do not support a genetic etiology for this rise, nor a deficit in willpower. Fresh new ideas are needed to address root causes and develop innovative approaches.

At the FDA, we will examine the role of ultraprocessed foods, food additives, and environmental toxins, the introduction of which has paralleled the epidemic of chronic diseases. We will transition from a purely reactionary health care system to one that is proactive, intellectually curious about underlying causes, and financially aligned to promote health—not just treat sickness. At the same time, the FDA must have the courage to create new pathways for therapeutic and device developers to respond to the current forest fire that is the worsening health of the US population. We will rapidly usher to market new products with transformational potential.

Here are our priorities for a new FDA. Each incorporates the 2 guiding principles of our philosophy—gold-standard science and common sense.

Accelerating Cures

During the COVID-19 pandemic, review processes that took a year were performed in weeks. We believe this is clear demonstration that rapid or instant reviews are possible. At the FDA, we aim to pilot programs to deliver sponsors a decision in weeks. Our pilot will leverage expertise across FDA divisions and departments. For efficiency, the pathway will ask sponsors to submit much of the arduous paperwork to the FDA in advance of their final application, enabling regulators to examine the manufacturing plans, draft labels, and packaging before a clinical trial is complete. The time from when pivotal trial results are known to when decisions are made must be shortened. This has implications for public welfare and will improve the risk-reward calculation of drug development.

In addition, we must reevaluate legacy processes at the agency that slow down decisions and do not increase safety. For example, monographs (required recipes for products) have stifled innovation in areas such as infant formula. As a result, there have been no changes to the infant formula recipe since 1998, with the exception of adding selenium. Europe and China are surpassing the US in some sectors. Reducing excessive, redundant, or uninformative animal testing is another area of interest.

To reinvigorate innovation, we must become a user-friendly FDA that partners with industry rather than takes a receive-only posture. Such a close relationship can provide guidance and predictability to developers and investors. In the case of small companies, the FDA can provide the regulatory navigation that companies may lack internally. At the same time, the FDA will guard against a cozy relationship that has characterized the agency in the past and led to allegations of industry capture. The scientific evaluation of products will always remain strictly independent.

For this reason, the FDA recently removed industry members of all FDA advisory committees where statutorily permitted. And at the recent Vaccines and Related Biologic Products Advisory Meeting, section 502 waivers (which waive voluntary disclosures) were not granted. The FDA will take conflict of interest seriously. We will never forget one of the worst self-inflicted wounds of US health care—the FDA's illegal approval of oxycontin for chronic pain based on a 14-day study, the immediate hiring of the former FDA regulator by Purdue Pharma, and a subsequent epidemic that killed approximately 1 million people in the US.[4]

Unleashing AI

The advent of generative artificial intelligence (AI) holds several promises to modernize the FDA and radically increase efficiency in the review process. From day one, we have made it a top priority, and on May 8, 2025, the agency implemented the first AI-assisted scientific review pilot using the latest generative AI technology. We believe AI can make a first-pass review of documentation received by the FDA as part of an application that often exceeds 500 000 pages and aid in generating standardized tables.

The FDA will also modernize how it reviews AI-based technologies. A diversity of use cases and rapid changes in technology make the legacy review mechanism appear byzantine. Rethinking our approach to AI, balancing safety and accuracy while fueling innovation, is a leading FDA priority.

Finally, we have developed a road map to reduce animal testing using AI-based computational modeling to predict toxicity-leveraging chip technology. These new technologies not only have the promise of better predicting toxicities in humans, but they can also speed up drug development and reduce animal waste and cruelty—another top priority.

Healthier Food for Children

The oversized role of the food industry has corrupted our basic conception of the food pyramid and ignored our growing understanding

of food processing and ingredients. There has been little research on arguably the most important potential root causes of chronic disease in the US: our increasingly chemically manipulated diet. The FDA will restore focus on the "F" in FDA.

We have begun conducting a full inventory of concerning ingredients in the US that are not allowed in other developed countries. We plan to apply gold-standard science and common sense to the problem. Already, we have taken action to remove all 9 petroleum-based food dyes from the US food supply and approve novel food dyes that use natural ingredients, begun work on defining ultraprocessed foods, and recently conducted an expert panel on talc, which is ingested by millions of Americans every day. Ironically, talc has been removed from baby powder used on the skin because of its carcinogenicity, but adults ingest it regularly because it occurs in common medications,[5] and children ingest it because it occurs in candy. Like so many additives, its addition is strictly for convenience—to prevent pills from getting stuck in machines or wrappers from sticking to candy. The aforementioned petroleum dyes produced bright colors meant to attract attention from children[6]; for all additives, the benefit-to-harm balance must be reevaluated.

Harnessing Big Data

The mass availability of health data and cloud computing has enabled 2 new opportunities: (1) researching root causes of chronic diseases and (2) postapproval monitoring of new products. In the past, randomized clinical trials were the sole method used to determine if a product was safe and effective. But not all randomized trials are informative—some have poor control groups, unrepresentative populations, or inadequate statistical plans.[7]

Advances in causal inference in nonrandomized data, including the use of target trials, which attempt to balance confounding and time zero, have potential to yield actionable causal conclusions,[8] in many cases at lower cost.[9] Moreover, postapproval monitoring in Big Data will allow the FDA and researchers to see safety signals in real time and evaluate effectiveness in the real world. This is particularly important for products addressing rare diseases, where premarket randomized trials may not be feasible. Leveraging Big Data may also enable a broad move from a requirement for 2 pivotal clinical trials used by FDA regulators for many products in the past down to 1. Finally, robust clinical data can provide better inferences about adverse events than those derived from existing clunky adverse reporting databases that rely on self-reported data. Current systems both wrongly condemn and wrongly exonerate medical products.

Financial Toxicity

The high price of drugs in the US relative to other Organisation for Economic Co-operation and Development nations represents a great American rip-off. Financial toxicity harms patients. No one took an oath to treat a patient and then ruin their life financially.

Although the FDA, per statutory law, will not consider price in benefit-risk calculations, the FDA will use its power to address costs. These include expediting generic medications and massively streamlining the burden to develop biosimilar compounds.

The FDA is committed to supporting the national priority of most favored-nation pricing of pharmaceuticals, as appropriate. The US is the largest purchaser of pharmaceutical products and should have price equity with other wealthy nations. Most large pharmaceutical companies spend more on marketing, including direct-to-consumer advertisements that can be misleading, than they spend on research and development.

In summary, the FDA will be focused on delivering faster cures and meaningful treatments for patients, especially those with neglected and rare diseases, healthier food for children, and common-sense approaches to rebuild the public trust. The FDA and our great medical profession should unite to consider fresh new approaches to the evolving health topics facing the US today.

ARTICLE INFORMATION

Author Affiliations: US Food and Drug Administration, Silver Spring, Maryland.

Corresponding Author: Martin A. Makary, MD, MPH, Office of the Commissioner, US Food and Drug Administration, Bldg 1, Ste 200, Silver Spring, MD (CommissionersOffice@fda.hhs.gov).

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REFERENCES

1. Centers for Disease Control and Prevention. Managing chronic health conditions. July 9, 2024. Accessed June 5, 2025. https://www.cdc.gov/school-health-conditions/chronic-conditions/?CDC_AAref_Val=https://www.cdc.gov/healthyschools/chronicconditions.htm#cdc_generic_section_2-resources

2. Zablotsky B, Black LI, Maenner MJ, et al. Prevalence and trends of developmental disabilities among children in the United States: 2009-2017. *Pediatrics*. 2019;144(4):e20190811. doi:10.1542/peds.2019-0811

3. Blue Cross Blue Shield. Early-onset dementia and Alzheimer's diagnoses spiked 373 percent for Generation X and Millennials. February 27, 2020. Accessed June 5, 2025. https://www.bcbs.com/about-us/association-news/early-onset-dementia-and-alzheimers-diagnoses-spiked-373-percent-generation-x-and-millennials

4. Hornblower S. How the FDA helped ignite, and then worsened, the opioid crisis. Bloomberg. April 25, 2025. Accessed June 5, 2025. https://www.bloomberg.com/news/features/2025-04-25/the-fda-s-untold-role-in-igniting-the-opioid-crisis

5. Sharma A, Tidmarsh G. Reviewing the safety of our foods and drugs: an urgent need for a comprehensive reevaluation by FDA of talc in the American food and drug supply. *J Acad Public Health*. Published online April 10, 2025. doi:10.70542/rcj-japh-art-16avrw

6. Spence C. On the psychological impact of food colour. *Flavour (Lond)*. 2015;4:21. doi:10.1186/s13411-015-0031-3

7. Prasad V, Berger VW. Hard-wired bias: how even double-blind, randomized controlled trials can be skewed from the start. *Mayo Clin Proc*. 2015;90(9):1171-1175. doi:10.1016/j.mayocp.2015.05.006

8. Wang SV, Schneeweiss S, Franklin JM, et al; RCT-DUPLICATE Initiative. Emulation of randomized clinical trials with nonrandomized database analyses: results of 32 clinical trials. *JAMA*. 2023;329(16):1376-1385. doi:10.1001/jama.2023.4221

9. Barosa M, Prasad V. The cost of emulated target trials: is real-world data cheaper than randomized studies? *Eur J Clin Invest*. 2025;e70035. doi:10.1111/eci.70035