Changing What We Ask Of The FDA

We should let the FDA focus on determining whether a drug is safe enough to use, and let patients, physicians, and insurers decide whether it is worth paying for.

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Only about 5% of the roughly 10,000 known rare diseases have an FDA-approved treatment. This means that if you are one of the nearly 30 million Americans living with a rare condition, the odds that a medicine for your condition will ever clear the FDA's desk are about one in twenty.[1]

Last February, one of those long shots almost paid off—and then it didn't. Atara Biotherapeutics and Pierre Fabre Pharmaceuticals had developed a cell therapy called Ebvallo (tabelecleucel) for a rare cancer that strikes patients after a stem cell or organ transplant. It affects roughly 500 people per year in the United States, and is usually fatal within weeks or months. According to a report from STAT News, the FDA's own internal reviewers had recommended the drug for approval. But then, in what a former agency staffer called a complete reversal, the agency rejected it anyway citing "deficient clinical data."[2] (The drug is approved and commercialized in Europe.)

A year prior, the opposite failure unfolded. Sarepta's Elevidys, which is a gene therapy for Duchenne muscular dystrophy, had already secured approval in 2023. However, in July 2025, an FDA official moved to suspend it due to multiple patient fatalities. The clinical benefit was thin, and on one efficacy measure (a walking test), treated patients only gained a fraction of a second compared to placebo. The price tag for this therapy was $3.2 million per infusion, and a large share of expected revenue would be coming from Medicaid. What followed was not scientific pressure but an intense pressure campaign from the company and patient advocates, which reportedly led the reviewing official to temporarily leave the FDA.[3]

FDA decisions don't merely certify that a drug is safe and sufficiently effective to use. Practically speaking, they also function as the on ramp to reimbursement, since Medicare, Medicaid, and private insurers all depend on FDA status when deciding what to cover. Approval by the FDA not only opens a treatment option, but also a taxpayer-funded pipeline that is expensive and politically risky to close again.

Cost is not supposed to enter into FDA approval decisions, but it is hard to set it completely aside. What verdict should a drug approval process deliver when a drug is safe and highly effective, but extremely expensive? How about when a drug is safe, marginally effective, and extremely expensive? It's easy to see how some approval decisions can start to become political instead of remaining a purely scientific judgement call.

Perhaps we could improve on the status quo by changing what an approval or a rejection means. Imagine a new system where for drugs that are sold directly to private patients and insurers, the FDA's role is only to confirm basic safety. The matter of efficacy would become a question for patients, physicians, and insurers to navigate themselves. Doctors and patients would rely on informed consent and tort liability, while insurers decide whether to pay. Once a drug meets the basic safety benchmark, the choice to use and pay for it privately should sit between willing parties, not a government reviewer.

Continuing the thought experiment, imagine for decisions bearing the inclusion of Medicare or Medicaid, the bar swings the other way. Both safety and efficacy will be reviewed, along with cost as a factor, since public programs spend taxpayer money in a non-competitive market rather than two people freely agreeing to a trade.

For precedent, we might look at how off-label prescribing already separates clinical judgement from coverage decisions. It treats the freedom to use an "unapproved" treatment separate from who must pay for it. This logic could be extended into the private market as a rule, rather than a narrow carve out. A workable general idea could be: safety floor for everyone; full efficacy and cost review if the public is going to be asked to cover the bill.

The status quo requires proof of efficacy before any private sale and use of a drug. That assumes that government regulators are better positioned than informed patients, their physicians, and insurers in evaluating those tradeoffs. When taxpayer funds are at stake, that assumption becomes easier to justify since someone must decide how public money gets spent. But if the costs and risk fall completely on private parties our approach could be more permissive.

For the roughly 500 patients a year facing the disease Atara and Pierre Fabre were trying to treat, and for the families watching a $3.2 million therapy become political football, the fix isn't asking FDA reviewers to make braver calls under intense pressure. It's giving them a narrower and more honest job: decide if it's safe, and let the market decide if it's worth buying. That respects private interests. We can save the harder judgment about efficacy just for those situations where, due to the existence of public payers, there is more of a public interest involved.

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Declaration of Conflicting Interests

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