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The FDA Wants to Interfere in the Practice of Medicine

A little-noticed provision of the omnibus spending bill could give the agency power to ban off-label use of approved therapies.

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Secreted within the 2023 omnibus appropriations bill—4,155 pages, spending $1.7 trillion—is a 19-line section that could change the way medicine is practiced.

Physicians routinely prescribe drugs and employ medical devices that are approved and labeled by the Food and Drug Administration for a particular use. Yet sometimes physicians discern other beneficial uses for these technologies, which they prescribe for their patients without specific official sanction. The new legislation amends the Food, Drug and Cosmetic Act, or FDCA, to give the FDA the authority to ban some of these off-label uses of otherwise approved products. This unwarranted intrusion into the physician-patient relationship threatens to undermine medical innovation and patient care.

The new provision was enacted at the FDA's urging in response to a decision by the U.S. Circuit Court of Appeals for the District of Columbia. The case, *Judge Rotenberg Education Center v. FDA*, involved a 2020 final rule in which the FDA banned the use of an electrical stimulation device, only in the treatment of self-injurious behaviors such as head banging and self-biting. The agency didn't ban other uses of these devices, such as treating addiction.

The court held that the FDA had the power to ban a medical device altogether under Section 360f of the FDCA if it poses "an unreasonable and substantial risk of illness or injury." But barring a practitioner from prescribing or using an otherwise approved device for a specific off-label indication would violate another FDCA section, which bars the FDA from regulating the "practice of medicine."

The omnibus bill amends Section 360f to allow a finding that a device can pose an unreasonable risk for "one or more intended uses" and ban those uses while leaving it

approved for other uses. Since the new provision lets the FDA skirt the ban on interfering with the practice of medicine by banning devices for particular uses, the agency will likely claim this as a precedent allowing it to ban off-label uses of drugs as well.

This is a problem for many reasons. The statute gives the FDA the power, without any public input, to prevent patients' access to off-label therapies even though their physicians and their patients have found the treatments to be beneficial or even essential. That was the situation in the *Rotenberg* case, in which the center and the families of patients had to sue the FDA because the banned devices were often the only effective treatment to keep patients from harming themselves.

Yet 1 in 5 prescriptions written are for an off-label use. In some fields off-label use is the rule, not the exception. In oncology, the standard treatment for specific types or stages of cancer often includes the off-label use of one or more drugs. And off-label uses are routine in pediatrics, where scientific, ethical and logistical concerns preclude conducting large trials for approval in children.

Allowing the FDA to ban certain off-label uses will impair clinical progress. Off-label use enables physicians to assess their patients' unique circumstances and use their own evolving scientific knowledge in deciding to try approved products for new indications. If the treatment proves useful, formal studies are performed and published. If enough evidence accumulates, the treatment becomes the standard of care, even if the manufacturer didn't submit the product for a separate, lengthy and costly FDA review.

Examples abound. Erythromycin, a common antibiotic labeled for use in infectious diseases, is widely used off label to increase stomach motility and tolerance of oral feeding. Clinical use followed by randomized controlled trials established the off-label use of tricyclic antidepressants such as nortriptyline and desipramine as first-line treatments of neuropathic pain. Other antidepressants, such as amitriptyline and trazodone, are prescribed off label as sleep aids. Rituximab, a lymphoma drug, is used off label to treat a benign disorder, immune thrombocytopenia.

This process works in reverse, too. When evidence accumulates that off-label uses aren't effective, practitioners cease prescribing the drugs for the relevant indications. Ivermectin and hydroxychloroquine, which were advanced and then abandoned as treatments for Covid, are recent examples.

Substituting regulators' wisdom for the cost-benefit judgment of physicians and their patients will discourage attempts to use approved products in new and beneficial ways and deprive patients of valuable treatments. Congress should reconsider this ill-advised legislation.

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The Food and Drug Administration headquarters in White Oak, Md., Aug. 29, 2020.
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