Lack of a US Food and Drug Administration indication should not limit access to appropriate treatment

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Editorials in the *Journal of the American Academy of Dermatology* are not usually intended to be used in a prior approval packet. This one is different.

The US Food and Drug Administration (FDA) recognizes the need for off-label use of medications, and states that "health care providers generally may prescribe the drug for an unapproved use when they judge that it is medically appropriate for their patient."[1] Between 17% and 73% of prescriptions for the 10 most common dermatologic diseases are outside their labeled indications.[2]

Access to medications should not be solely determined by FDA indication

Drug labeling informs key indications for initial FDA approval but often fails to keep up with new indications. The American Academy of Dermatology guidelines endorse off-label use of medications for common diseases like atopic dermatitis (AD), psoriasis, and acne. Prednisone is labeled for psoriasis, but not recommended; it is also a first-line off-label systemic therapy for bullous pemphigoid.

Hidradenitis suppurativa and AD are examples of arbitrary FDA indications. First-line therapies for hidradenitis suppurativa are not labeled. Only adalimumab is approved and costs $120,000 annually, but patients are "unlikely to have complete resolution of their symptoms."[3] Dupilumab is labeled for AD and is extremely effective. However, payers routinely insist on more toxic and less effective oral off-label immunosuppressant medications before it is approved.

Of the approximately 3000 dermatologic conditions, many are so rare that they lack unique international classification of disease codes. The *International Classification of Diseases, 10th revision* does not include erosive pustulosis of the scalp or actinic prurigo, but multiple codes exist for attacks by turkeys and macaws. Erosive pustulosis may respond to topical steroids, but if they fail, prior authorization based on FDA indications is futile. Actinic prurigo is devastating, but can be managed

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with thalidomide, which was initially approved to treat erythema nodosum leprosum. After >3 decades, the FDA acknowledges that only a few orphan diseases have approved treatments, and the lack of an FDA indication should not limit access to appropriate treatment for these orphan diseases.[4]

Access to appropriate medications should not be constrained by age

Children lack approved medications and treatment regimens and therefore suffer. Even for acne vulgaris, evidence-based treatment for children <12 years of age is off-label. It is evident that insurers cannot rationally base children's access to medications on FDA indication alone. The indication for clobetasol ointment is "relief of the inflammatory and pruritic manifestations of corticosteroid-responsive dermatoses,"[5] except for patients <12 years of age, for whom is it "not recommended"[5] but occasionally needed.

Access to medication should not be compromised because disease states are poorly defined

Systemic and chronic discoid lupus erythematosus can only be treated with hydroxychloroquine based on labeled indications; all other types have no specific treatment approved by the FDA.

In summary, insurance company denials for medications based on FDA indication are misguided; the FDA fully agrees.[1] Denial of evidence-based therapy by insurers results in treatment delays, poor patient outcomes, and systemic inefficiency as physicians, staff, and patients struggle with prior authorizations and appeals. Therapy must be based on shared decisions, best evidence, and all relevant patient-specific factors. Simply denying coverage because of the lack of FDA initial labeling is fundamentally unacceptable.

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