

Viewpoint: Redefining ‘medically necessary’ — Debate unfolds over how to cover ‘off label’ treatments for rare diseases

The term “medically necessary” is the yardstick by which insurance companies, including Medicare and Medicaid, decide if they will pay for a particular treatment. For the millions of Americans living with rare diseases, most of which do not have FDA-approved treatments, identifying a treatment as medically necessary can be a lifeline — or the end of the line.

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A drug’s label includes copious information about its chemical composition, dosing instructions, and what conditions it has been approved to treat. When a clinician prescribes a treatment for an off-label use — one not listed on the label — an insurer will make a coverage decision based on special medical references known as compendia.... However, there are no rare-disease compendia to track off-label treatment protocols.

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Marc [Yale] lives with [mucous membrane pemphigoid](#), a rare, life-threatening autoimmune disease that causes blistering lesions throughout the body. He fought with his health insurer for two years to get the right treatment — a drug that is the standard of care for his very rare condition. He was told that the treatment his doctors prescribed was off-label, experimental, and not on any compendia, and his insurer insisted that he use other medications instead. While Marc fought with his insurer, he lost his eyesight, had to stop working, and was hospitalized.

[**This is an excerpt. Read the original post here**](#)