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PR Guidelines for Completing an Experimental/Investigational External Review
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Medical treatments and medications are constantly changing, and novel therapies keep both physicians and their patients keenly interested in health issues. Very few newer treatments, however, will become "the standard of care." New innovations in medicine require rigorous testing and clinical trials to substantiate their value, and allow entrance onto the "standard practice" stage.

Most health plans do not approve treatments still considered experimental or investigational (E/I), or they are approved only when very limited clinical conditions exist – such as documented failure with the current standard of care therapy.

What is a definition of E/I medication? The following is the FDA definition of an E/I drug:

> *"Investigational drugs, sometimes referred to as experimental drugs, are still being studied to determine if they are safe and effective, and how best to use them for their intended purpose. They are different from approved drugs, which began as investigational drugs and have undergone the extensive testing in animals and humans to show that they are safe and effective for treating that illness."* [1]

The foundation of a health plan's criteria for coverage usually incorporates FDA approval of medications, treatments, services, and even testing for various medical conditions. When coverage for a medication, treatment, service, or test is denied, a patient usually has a right to appeal through their health plan's appeal process. Often this happens when the treating physician orders or prescribes a treatment that is believed to be in the best interest of the patient, even if the treatment is "off-label" or not explicitly FDA approved.

These appeals often end up going to an independent review organization (IRO), such as CIMRO, to provide an external review of the treatment in question. Once the issue is given to an IRO for external review, the peer reviewer has an opportunity to respond to the questions at hand, employing their personal lines of logic and professionalism to reach a decision.

Each case requires an in-depth review of the health plan provisions, evidenced-based findings that demonstrate the effectiveness of the treatment under review, and the unique case-specific issue and history of the member in question. Certain treatments may be considered appropriate in some cases and not indicated in others, depending on all factors under review.[2]

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[1] U.S. Food and Drug Administration. Understanding unapproved use of approved drugs "off label." 2018.

[2] National Association of Independent Review Organizations (NAIRO) www.nairo.org

What are the tools that may be utilized most effectively by CIMRO IRO peer reviewers?

- FDA approval
- Peer-reviewed medical literature and clinical trials showing evidence-based standards and evidence of treatment benefit
- Current medical community validation of the treatment modality, even if it is “off-label” or not FDA Approved, per se
- Personal clinical experience
- Medical appropriateness for each unique case specific issue, such as therapeutic failure with standard treatment combined with evidence-based data, shows the proposed E/I treatment is more likely to be beneficial without placing the patient at undue risk than any available standand health care service treatment.

In summary, CIMRO reviewers have a high degree of flexibility to create logical and persuasive decisions in IRO cases, which can be eye-opening and gratifying at the same time. A quality review involves detailed examination of the health plan contract and whether clinical criteria were met for approval of a novel therapy. Our reviewers are in a unique position to be able to evaluate E/I treatments at the cutting edge of medicine, knowing some of these treatments will, indeed, become the standard of care in the future.

Please visit https://www.nairo.org/assets/docs/NAIRO-Issue-Brief-Providing-Leading-Assessments-of-Experimental-and-Investigational-EI-Treatments.pdf for additional information on E/I reviews.