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Guidelines for CIMRO Physician Reviewers 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 the 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 which is felt 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 Physician Reviewer then has an opportunity to respond to the questions at hand, employing their personal lines of logic and professionalism to reach a decision.

What are the tools which may be utilized most effectively by CIMRO IRO Physician Reviewers?

- FDA Approval
- Peer-reviewed medical literature and clinical trials offering evidence-based standards/evidence of treatment benefit
- Current medical community validation of the treatment modality, even if "off-label" or not FDA approved per se
- Personal clinical experience
- Medical appropriateness under the circumstances, such as therapeutic failure with standard treatment combined with a documented evidence-based logic the proposed E/I treatment is likely to benefit the patient without undue risk

In summary, the free-rein ability of CIMRO's Physician Reviewers to create a logical and persuasive decision in IRO cases is in many ways eye-opening and gratifying at the same time. Our reviewers are in a unique position to be able to evaluate experimental and investigational treatments at the cutting edge of medicine, knowing some of these treatments will indeed become the "standard of care" in the future. A quality review involves detailed examination of the health plan contract and whether clinical criteria were met for approval of a novel therapy. The tools utilized for a decision may include prior FDA approval, incorporation of evidence-based standards demonstrating treatment benefit, personal experience, and a detailed description of the medical appropriateness under the clinical circumstances presented.

Please visit http://nairo.org/site/1920nair/NAIRO_Issue_Brief_EI.pdf for additional information re: experimental and investigational.

References

1. U.S. Food and Drug Administration. Understanding Investigational Drugs and Off Label Use of Approved Drugs. www.fda.gov/ForPatients/Other/OffLabel/ucm20041767.htm. Accessed October 2014.