NAIRO
National Association of Independent Review Organizations

Providing Leading Assessments of Experimental and Investigational (E/I) Treatments
Independent Review Organizations deliver evidence-based reviews of E/I appeals

In the field of medicine, innovations occur every day, and every year brings a parade of new treatments, services and medications. Like other industries, some of these innovations become ingrained in daily use, others fade from relevance, and still others remain at the cutting-edge but are not yet part of standard practice. This creates a fine line for health plans and treating providers to ensure patients are receiving care that is both clinically necessary and appropriate.

In health care terms, many health plan contracts will not approve treatments that are still considered experimental or investigational (E/I), or at the very least will only approve certain new or innovative treatments if specific clinical indications and criteria have been met.

New medications are a good example of E/I treatment. The Food and Drug Administration (FDA) defines E/I drugs, which extrapolates to E/I treatment on the whole, as:

"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]

FDA approval is a key component to many health plans' criteria involving allowed treatments and procedures. Additional considerations may include peer-reviewed literature, clinical trials, endorsements by the medical community regarding safety and effectiveness, and medical appropriateness in a particular case.

If the coverage of a treatment or service is denied based on E/I criteria, a patient often has the right to appeal through their health plan's internal and external appeal process. In this event, Independent Review Organizations (IROs) can play a critical role in providing external review of the treatment in question – and providing an evidence-based opinion based on the latest scientific evidence and standards within the medical community.

IROs have access to physicians and clinicians that are affiliated with academic medical centers and research hospitals, and are often on the cutting edge of new treatments,

services and medications. In the sections below, we'll further explain why accredited IROs are well-positioned to provide reviews of E/I services, and which E/I treatments and therapies IROs might make a decision upon.

Examples of Treatments Reviewed for E/I Consideration:

- **Proton beam radiation therapy (PBRT).** Proton therapy is non-invasive form of radiation intended to treat various types of cancer.
- **Genetic testing.** Mapping the human genome was one of the most important scientific advances to occur in the history of medicine, and one of the offshoots – genetic testing, in which clinicians can analyze DNA – gives patients an unprecedented ability to screen for potentially inherited disorders.
- **Combination drug therapies.** Advances in science and medicine also allow physicians to more completely understand the biology of their patients and the way that specific drugs interact with patients on a molecular level. As a result, physicians may recommend using a combination of drugs to treat their patient's underlying disease.
- **Off-label use of approved drugs.** Medications approved by the FDA are intended to treat a designated disease, yet researchers find that some drugs are effective for treating other conditions as well. Off-label drug use describes the situation when medications are used to treat a disease other than the one it was approved for, or when a different drug administration or dosage is used.

Expertise in Action

As the examples above show, many therapies often involve complex and cutting-edge treatment options. When such advanced treatments are requested, disputes regarding the appropriateness and necessity are commonplace between payers and plan participants.

Each case will require an in-depth review of 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.

If a health plan denies coverage for a given service or treatment as E/I, the patient has the right to appeal the denied claim. If or when this situation occurs, Independent Review Organizations step in to assess the claim and determine if the service should or should not be covered. With the expertise of clinical reviewers and specialized knowledge of health care services, accredited IROs are equipped to make evidence-based decisions about these types of appeals.

More specifically, accredited IROs offer:

- **Deep expertise and experience.** IROs have immediate access to physicians and allied health care practitioners who are at the vanguard of medical treatments and services. Accredited IROs provide expertise in experimental and investigational reviews and are up-to-date on the accepted standards of care.
- **Large panels of clinical reviewers.** IROs feature expansive reviewer panels of hundreds to thousands of experts, who can provide access to all recognized specialties and sub-specialties.

- **Affiliations to leading research and knowledge centers.** Many IRO reviewers are affiliated with or have relationships with medical centers of excellence and research hospitals, which links them to the latest knowledge and resources of accepted medical practices and treatments.
- **Advanced, ongoing education and training.** The training and credentialing programs for IROs are designed to ensure the use of reviewers who are knowledgeable of the most current peer-reviewed literature and evidence-based medicine.

When IROs consider appeals of E/I cases, they follow a legal framework, which considers several factors. Specifically, IROs and their clinical reviewers assess two factors:

- **FDA approval.** Clinical reviewers assess whether the recommended service or treatment has been approved by the FDA for the patient's condition.
- **Evidence-based standards and/or medical or scientific evidence.** Also, clinical reviewers will study the evidence to see if "the expected benefits of the recommended or requested health care service or treatment is more likely than not to be beneficial to the covered person than any available standard health care services or treatment and the adverse risks of the recommended or requested health care service or treatment would not be substantially increased over those of available standard health care services or treatments."[2]

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.
2. National Association of Insurance Commissioners. Uniform Health Carrier External Review Model Act. Section I (5).

About NAIRO

NAIRO (The National Association of Independent Review Organizations) was formed by the majority of URAC-accredited IROs. The mission of NAIRO is to promote the quality and integrity of the independent review process at the internal and external levels. Utilizing the expertise of board-certified clinicians throughout the country, NAIRO members embrace an evidence-based approach to independent medical peer review, in order to help resolve coverage disputes between enrollees and their health plans. More information can be found at www.nairo.org.

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