OPTUM®

Utilization Management Policy

Experimental and Investigational Technologies

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Original Effective Date:	3/2001
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Policy Statement

A technology (service, procedure, device, supply, or drug) that is deemed investigational or experimental will be denied based upon the applicable exclusions recorded in the member's benefit coverage document or medical policy; or as not medically necessary in accordance with the criteria established by Optum* by OptumHealth Care Solutions, LLC and OrthoNet, LLC.

Optum and OrthoNet apply the following criteria when determining if a technology is investigational or experimental:

- Does the technology have final approval from the appropriate governmental regulatory bodies (if applicable)?
- Does the scientific evidence permit confident conclusions concerning the effect of the technology on health outcomes?
- Does the technology improve the net health outcome (the balance between benefit and harms)?
- Is the technology at least as beneficial and safe as any established alternatives?
- Is the expected health improvement attainable in the clinical practice setting (i.e., not restricted to investigational settings)?

Purpose

This policy outlines the general criteria and guidelines used in determining the medical necessity of experimental/investigational or potentially experimental/investigational healthcare technologies.

Scope

All in and out of network programs, involving all provider types, where Utilization Review (UR) determinations are rendered. This policy is applicable only when there is no other policy, criteria, or coverage statement available. This policy does not apply to healthcare technologies employed within clinical trials.

Definitions

Experimental or investigational means that the technology is:

- not of proven benefit for the particular diagnosis or treatment of a particular condition; or
- not generally recognized by the healthcare community as reflected in the published peer-reviewed clinical/scientific literature as effective or appropriate for the particular diagnosis or treatment of a particular condition
- There is uncertainty about the risks associated with the proposed use of the technology, including the risk of not receiving established and effective intervention.
- The technology is generally provided or performed in special settings for research purposes or under a controlled environment or clinical protocol.

Peer-review: The peer-review process subjects research studies to the scrutiny of others, who are experts in the same field (peers), and is considered necessary to ensure scientific quality.

Background

Historically, experimental and investigational technologies (i.e., services, procedures, devices, supplies, or drugs) are standard benefit exclusions defined in the member's certificate of coverage or health plan summary. Additionally, benefit coverage documents may exclude specific technologies as experimental or investigational.

Judgments about which technologies are safe and effective, and which remain of uncertain value, must be made by health care plans and their delegates. The criteria used to distinguish experimental/investigational from standard/established technologies relate to:

- The populations and conditions for which use is expected to be helpful (i.e., indications for use)
- The known/unknown risks and ethical considerations (safety)
- The degree of certainty regarding anticipated clinical outcomes (clinical effectiveness)
- The skills, personnel, and site requirements for appropriate application (practitioner expertise, the facilities necessary for proper use)

Standard technologies are characterized by broadly established indications for use; empirical knowledge of the risk of adverse events and contraindications to use; a precise understanding of the type, duration and magnitude of benefits to be gained; and specification of the conditions required for successful use of the technology. In contrast, a technology is considered experimental and investigational when any of the following conditions are met:

- The technology is not of proven benefit for a specific diagnosis or treatment of a particular condition.
- The technology is not generally recognized by the medical community as effective or appropriate for the diagnosis or treatment of a particular condition.
- There is uncertainty about the risks associated with the proposed use of the technology, including the risk of not receiving established and effective intervention.
- The technology is generally provided or performed in special settings for research purposes or under a controlled environment or clinical protocol.

The basis for determining the experimental or investigational status of a technology is explicitly derived from information obtained using transparent and evidence-informed processes. These include descriptions of the methods used to identify evidence, critical appraisal of research evidence, considered judgments and contextual factors. The findings are then translated into terminology that is consistent with the member's benefit documents. The process of structured qualitative and quantitative literature review is described in further detail in Utilization Management (UM) policy 429 (Guidelines for Utilization Management Policy Development & Revision).

Clinical and scientific evidence means the following sources:

A. Peer-reviewed scientific studies published in or accepted for publication by healthcare journals that meet nationally recognized requirements for scientific manuscripts and that submit most of their published articles for review by experts who are not part of the editorial staff.
B. Peer-reviewed literature, biomedical compendia, and other healthcare literature that meet the criteria of the National Institute of Health's National Library of Medicine for indexing in index Medicus, Excerpta Medicus (EMBASE), Medline, or MEDLARS database Health Services Technology Assessment Research (STAR).
C. Healthcare journals recognized by the Secretary of Health and Human Services, under Section 1861(t)(2) of the Social Security Act (42U.S.C. 1395x).
D. Peer-reviewed abstracts accepted for presentation at major healthcare association meetings
E. Findings, studies, or research conducted by or under the auspices of federal government agencies and nationally or internationally recognized research institutes including the:
    - Agency for Healthcare Research and Quality,
    - National Institutes of Health,
    - National Cancer Institute,
    - National Academy of Sciences,
    - Center for Medicare and Medicaid Services, and
    - Any national board recognized by the National Institutes of Health or designated governmental oversight agency for the purpose of evaluating the medical value of health services.

Peer-reviewed literature shall not include publications or supplements to publications sponsored to a significant extent by a product manufacturing company or by an entrepreneur.

References

- Article 49 of the Public Health Law – Utilization Review and External Appeal – New York State, July 1999
- Beebe DB, Rosenfeld AB, Collins N. An approach to decisions about coverage of investigational treatments. HMO Pract. 1997;11(2):65-67.
- Centers for Medicare and Medicaid (CMS) National Coverage Policy and current Centers for Medicare and Medicaid (CMS) Policy Manual(s) http://www.cms.hhs.gov
- Hansen DT, Mootz RD. Formal Processes in Health Care Technology Assessment: A Primer For The Chiropractic Profession. Topics in Clinical Chiropractic 1996;3(1)
- Priester R, Vawter DE, Gervais KG. Investigational treatments: Process, payment, and priorities. JAMA. 1997; 278(17):1403-1404.
- Reiser SJ. Criteria for standard versus experimental therapy. Health Aff (Millwood) 1994;13(3):127-136.
- Steinberg EP, Tunis S, Shapiro D. Insurance coverage for experimental technologies. Health Aff (Millwood) 1995;14(4):143-158.
- Wiesel SW. How Third-party Payers Evaluate New Treatments. The BackLetter, 2004; 19(8):96

Policy History/Revision Information

Date	Action/Description
3/07/2001	Original effective date
9/20/2002	Annual review and approval completed
11/11/2003	Annual review and approval completed
10/18/2004	Annual review and approval completed
2/14/2006	Annual review and approval completed
4/10/2008	Annual review and approval completed
1/15/2009	Policy reformatted
4/30/2009	Annual review and approval completed
4/08/2010	Annual review and approval completed
10/26/2010	Policy rebranded to "OptumHealth Care Solutions, Inc. (OptumHealth)"
4/07/2011	Annual review and approval completed
4/19/2012	Annual review and approval completed
4/18/2013	Annual review and approval completed
4/17/2014	Annual review and approval completed; Policy rebranded "Optum* by OptumHealth Care Solutions, Inc."
4/16/2015	Annual review and approval completed
4/21/2016	Annual review and approval completed
4/20/2017	Annual review and approval completed; Legal entity name changed from "OptumHealth Care Solutions, Inc." to "OptumHealth Care Solutions, LLC."
4/26/2018	Annual review and approval completed; no significant changes made to the document
4/25/2019	Annual review and approval completed; no significant changes made to the document
4/23/2020	Annual review and approval completed; no significant changes made to the document
4/22/2021	Annual review and approval completed; no significant changes made to the document
5/03/2022	Annual review and approval completed; Incorporated OrthoNet content; Revised Policy Statement, Purpose, Scope, Definitions, Background, and References. Added Appendix – OrthoNet Guidelines
6/29/2022	Updated legal entity name "OptumHealth Care Solutions, LLC." to *Optum™ Physical Health ("Optum") includes OptumHealth Care Solutions, LLC; ACN Group IPA of New York, Inc.; ACN Group IPA of California, Inc. d/b/a OptumHealth Physical Health of California; Managed Physical Network, Inc.; and OrthoNet Holdings, Inc. which includes OrthoNet New York IPA, Inc., OrthoNet West, Inc., OrthoNet, LLC, OrthoNet of the South, Inc.
4/27/23	Annual review and approval completed; no significant changes made to the document. Updated contact email from policy.inquiry@optumhealth.com to phpolicy_inquiry@optum.com.

Contact Information

Please forward any commentary or feedback on Optum utilization management policies to: phpolicy_inquiry@optum.com with the word "Policy" in the subject line.

The services described in Optum* by OptumHealth Care Solutions, LLC policies are subject to the terms, conditions and limitations of the Member's contract or certificate. Optum reserves the right, in its sole discretion, to modify policies as necessary without prior written notice unless otherwise required by Optum's administrative procedures.

Certain internal policies may not be applicable to self-funded members and certain insured products. Refer to the member's Summary Plan Description (SPD) or Certificate of Coverage (COC) to determine whether coverage is provided or if there are any exclusions or benefit limitations applicable to any of these policies. If there is a difference between any policy and the member's SPD or COC, the member's SPD or COC will govern.

Appendix

OrthoNet Guidelines

Coverage Criteria:

The proposed experimental/investigational treatment must meet **all** the criteria outlined below before approval is granted.

***Note:** For New York Plans the member's condition and/or disease is not required to be life threatening or disabling.

1. The member must have a life threatening or disabling condition or disease.* For purposes of this policy, a disabling condition means that the member is unable to engage in any substantial gainful activities by reason of any medically determinable physical or mental impairment which can be expected to result in death or which has lasted or can be expected to last for a continuous period of time (not less than 12 months). In the case of a child under the age of 18, "disabling condition" means the child suffers from a medically determinable physical or mental impairment of comparable severity.

2. The member's medical record, in conjunction with at least two (2) published, peer-reviewed documents from the available scientific and medical evidence and any other pertinent information supplied, must establish that the proposed experimental or investigational treatment is likely to be more beneficial than any standard treatment(s) for the member's life-threatening or disabling condition or disease.*

3. The opinion of the attending physician recommending that the treatment or procedure is likely to be more beneficial to the member than any covered standard health service or procedure must be based upon at least two (2) peer-reviewed documents from the available medical and scientific evidence.

4. In the absence of any evidence of a standard treatment with which to compare the experimental treatment, the attending physician's opinion must be based upon at least two (2) peer-reviewed medical and scientific publications which show a definite positive effect of the proposed treatment on health outcomes for the disease or condition from which the patient suffers or the diagnosis for which it is being prescribed. In other words, the literature must show that well-designed investigations with measurable results (that can be duplicated) support that the treatment would be scientifically effective and that the beneficial effects of the treatment outweigh the harmful effects of the treatment.

5. Documentation:
Necessary Information: The following supporting documentation must be provided by the member and/or the member's provider for consideration of the clinical trial:

A. Certification from the member's attending physician, which includes:

1) a statement that the member has a life-threatening or disabling condition or disease for which (a) standard health service or procedures have been ineffective or would be medically inappropriate; or (b) there does not exist a more beneficial standard health service or procedure covered by the health care plan;
2) a statement of the evidence relied upon to recommend the proposed treatment or procedure and a statement of why the standard therapy available would not be beneficial, would be ineffective, or would be inappropriate, including an assessment of the risks and benefits of the proposed treatment;
3) citation to two (2) documents from the available medical and scientific evidence, upon which the attending physician based his/her recommendation for the proposed treatment and an explanation why, in his/her opinion, these documents establish that the treatment or procedure is likely to be more beneficial to the member than any covered standard health service or procedure, would provide a positive effect on the member's condition or illness, and that the benefits outweigh the harmful effects of the treatment.
    i. The attending physician must be a board-certified or board-eligible physician qualified to practice in the area appropriate to treat the member's condition.
    ii. A copy of the two (2) documents from the available medical and scientific evidence, upon which the attending physician based his or her recommendation for the proposed treatment.

B. A written description of the proposed treatment (or protocol if available), which must include:
1.) specific goals
2.) a rationale and background for the plan
3.) criteria for patient selection
4.) specific directions for administering the therapy or intervention
5.) specific directions for monitoring patients
6.) a definition of quantitative measures for determining treatment or intervention response
7.) methods for documenting and treating adverse reactions to the treatment or intervention.

C. A copy of the member's informed consent form

D. A copy of the member's medical and treatment records, including results of tests or studies and showing the member's current condition and any treatment received for the condition

E. The available clinical or pre-clinical data that indicate the treatment's effectiveness for treatment, prevention, or palliation of the member's condition.

Additional Information:

A. The outside consultant or the health plan, depending upon the nature of the proposed treatment and/or the member's condition or disease, may require additional documentation to review the requested treatment.

B. OrthoNet, on behalf of the health plan, will also accept and consider any additional pertinent clinical documentation, peer-review publications, and/or relevant data concerning the protocol that the member and/or the member's physician would like to provide in support of the request for the experimental treatment.

CT Members
OrthoNet, on behalf of the health plan, will also provide coverage under this policy for:

(1) off-label use of cancer drugs for another type of cancer, unless specifically contraindicated by the Food and Drug Administration.
(2) procedures, treatments, or usage of any drug if the procedure, treatment, or usage has successfully completed a Phase III clinical trial of the FDA for (a) the illness or condition being treated or (b) the diagnosis for which it is being prescribed.

New York Plans Only
When an anti-cancer drug is being used to treat a type of cancer for which it is not specifically FDA approved, NY insurance law requires coverage if the drug/cancer combination is recognized in any of the following pharmaceutical reference compendia:

1. The American Hospital Formulary Service-Drug Information (AHFS-DI)
2. NCCN Drugs and Biologics Compendium
3. Thomson Micromedex DrugDex
4. Elsevier Gold Standard's Clinical Pharmacology
5. Other authoritative compendia, as identified by the Federal Secretary of Health and Human Services or the Centers for Medicare & Medicaid Services (CMS), recommended by review article or editorial comment in a major peer-reviewed professional journal.

Experimental/Investigational Procedures:

1. Request is submitted for pre-certification of services.
2. Contract Assistant (CA) logs demographics.
3. CA sends information to the OrthoCare Manager (OCM), who, if needed, requests any additional information for review and benefit coverage determination.
4. Case is then reviewed by OrthoNet Medical Director for appropriateness.
5. OrthoNet will adhere to specific payor requirements related to the handling of Experimental/Investigational Treatment(s).

Some procedures are considered Experimental/Investigational at this time. The following is a sample list; it is not all-inclusive. This list is a living document that will be updated to either remove or add items from the list, based solely on the availability of peer-reviewed publications accepted widely as precise and accurate.

Examples of experimental and investigational:

1. Collagen Meniscus Implant (CenterPulse Orthopedics, Inc/ Regen Biologics, Inc.)

2. Botulinum Toxin for Pain Management (to relieve chronic back, neck, and headache pain other than those caused by migraines, diagnosed properly, using widely accepted diagnostic clinical criteria)

3. Autologus Cartilage Transplantation for lesions of the patella and ankle

4. RACZ procedure for epidural lysis of adhesions (with or without the use of spinal endoscope)

5. When proposed to be used in the treatment of chronic pain (as a late or early resort option), any electrical stimulation of any nervous system pathway component (with the exception of direct stimulation of dorsal columns of spinal cord from the epidural space)

6. (a) Any biomedical treatment (including spine surgery) proposed for the alleviation or palliation of acute or chronic cancer or non-cancer-related pain if the treatment is NOT going to be directed at treating the source of pain. The source of pain can be from any of the specific anatomic elements of the Pain Pathway (involved in acute nociception) or the astrocytes/microglia. A structure/anatomic element (natural or man-made) cannot be expected to be a pain source when it does not have an intrinsic nociceptor and or a nerve supply connecting it to the acute nociceptive ascending pain pathway. (b) Use of human origin (autologous or allogenic) or synthetic blood component or stem cells (obtained or grown from human adult cells or embryonic cells or placenta) injected, ingested, applied, or implanted directly or delivered via a device in the diagnosis or treatment of pain (acute onset or chronic). The only exceptions are when the technology is used in the treatment of a complication from a procedure-related, post-dural puncture headache, or when the technology is used within the context of a health plan or a government agency approved and monitored clinical trial conducted within United States jurisdiction.

7. **Note:** Coverage for experimental/investigational treatments and procedures is generally specifically excluded under the member's certificate. The health plan may cover in-network experimental/investigational treatment when the criteria outlined in this policy are met through the recommendation of OrthoNet (under Behavioral or Mental Health Benefits category, but not medical).