EXPERIMENTAL/INVESTIGATIONAL TREATMENT

Policy Number: EXPERIMENTAL 003.12 T2

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Conditions of Coverage

Applicable Lines of Business/Products: This policy applies to Oxford Commercial plan membership for CT and NY plans.

Benefit Type: General benefits package

Referral Required: No

Authorization Required: Yes¹

Precertification with Medical Director Review Required: Yes¹²

Applicable Site(s) of Service: Inpatient, Outpatient, Office

Special Considerations: ¹This medical policy must be reviewed in conjunction with the policy titled Clinical Trials.

²Precertification with review by a Medical Director review or their designee is required.

Coverage Rationale

Experimental and/or Investigational Treatment

Oxford recognizes that peer reviewed documents in scientific and medical literature may establish that an experimental and/or investigational treatment or procedure may be better than the standard treatments available to treat a member's life threatening or disabling condition and/or disease. Oxford has determined that it will create a limited exception to the exclusion of experimental and investigational treatments and provide coverage for in-network experimental and investigational procedures that meet the criteria set forth in this policy. Such coverage is subject to the member's other benefits and exclusions. Oxford's determination of whether the criteria have been met will be based upon the opinion of an independent consultant/peer reviewer with expertise in the area of practice appropriate to treat the member's condition or disease.

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

Unproven Therapies

Under no circumstances will this policy extend coverage to unproven therapies. Unproven therapies are treatments or procedures that lack significant medical documentation to support their medical effectiveness. Oxford does not provide coverage for any treatment modality that has not been proven medically effective or is not generally recognized as effective or appropriate for the particular diagnosis or treatment of the member's particular condition.
The proposed experimental/investigational treatment must meet all of the criteria outlined below before approval is granted. In addition, documentation must be submitted to Oxford demonstrating that the criteria below have been satisfied. Without all such documentation, Oxford will deny any such request.

**Coverage Criteria**

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

- 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 not less than twelve months. In the case of a child under the age of eighteen, disabling condition means that the child suffers from a medically determinable physical or mental impairment of comparable severity.
- 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.*
- 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.
- 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.

**Documentation: Necessary Information**

The following supporting documentation must be provided by the member and/or the member’s provider for consideration:

- Certification from the Member’s attending physician which includes:
  - 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
  - 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
  - Citation to two documents from the available medical and scientific evidence, upon which the attending physician based his recommendation for the proposed treatment and an explanation why, in the opinion of the physician, 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 or would provide a positive effect on the member’s condition or illness and that the benefits outweigh the harmful effects the treatment
    - The attending physician must be a board certified or board eligible physician qualified to practice in the area of practice appropriate to treat the member’s condition
    - A copy of the two documents from the available medical and scientific evidence, upon which the attending physician based his recommendation for the proposed treatment
- A written description of the proposed treatment (or protocol if available), which must include:
  - Specific goals
  - A rational and background for the plan
  - Criteria for patient selection
  - Specific directions for administering the therapy or intervention
  - Specific directions for the monitoring of patients
  - A definition of quantitative measures for determining treatment or intervention response; and
  - Methods for documenting and treating adverse reactions to the treatment or intervention
- A copy of the member’s informed consent form
- A copy of the member’s medical and treatment records, including results of tests or studies, showing the member’s current condition and any treatment the member has received for the condition
- 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**

- The outside consultant or Oxford, 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.
• Oxford 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.

**Connecticut Plans Only**

For members having a Connecticut Plans, Oxford will also provide coverage, under this policy, for:

- Off-label use of cancer drugs for another type of cancer, unless specifically contraindicated by the Food and Drug Administration; and
- 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.
- A patient is eligible to receive treatment with an investigational drug, biological product or device if the patient has:
  - Considered all other treatment options currently approved by the federal FDA;
  - Been unable to participate in a clinical trial for the terminal illness that is not more than 100 miles from the patient's home address, or not been accepted to a clinical trial not more than 1 week after completion of the clinical trial application process;
  - Received a recommendation from his or her treating physician for an investigational drug, biological product or device;
  - Given written, informed consent for the use of the investigational drug, biological product or device or, if the patient is a minor or lacks the mental capacity to provide informed consent, a parent of the minor or a legal guardian of the minor or adult patient has given such written, informed consent on the patient's behalf; and
  - Obtained written documentation from his or her treating physician stating that the patient meets the requirements of this subsection.

"Investigational drug, biological product or device" means a drug, biological product or biological device that has successfully completed a phase one clinical trial of the federal Food and Drug Administration (FDA) but has not yet been approved for general use by the FDA and remains under investigation in a clinical trial approved by the FDA.

**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:

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

**BACKGROUND**

Coverage for experimental and investigational treatments and procedures is specifically excluded under the member's Certificate with Oxford. Oxford will cover in-network experimental/investigational treatment (not meeting the requirements of a clinical trial, as defined in the policy titled Clinical Trials) when the criteria outlined in this policy is met. In general, experimental and investigational treatments and procedures are those medical treatments and procedures that have not successfully completed a Phase III trial, have not been approved by the FDA and are not generally recognized as the accepted standard treatment for the disease or condition from which the patient suffers. Experimental and investigational treatments include off label therapies. Off-label therapies are those medical therapies that use a FDA approved drug or procedure for a non-indicated use. Refer to the policy titled Off-Label/Unproven Specialty Drug Treatment.

**BENEFIT CONSIDERATIONS**

Coverage of experimental treatment is subject to the Member’s benefits and exclusions under the member's Certificate with Oxford. This policy does not expand coverage to other items specifically excluded from coverage and items not listed as a covered benefit in the member's Certificate with Oxford.

**For New Jersey Small Plans**: Refer to the policy titled Experimental/Investigational Treatment for NJ Plans.

**For New York Plans**: The member's condition and/or disease are not required to be life threatening or disabling.
REFERENCES

Consensus Document of the New Jersey Working Group to Improve Outcomes in Cancer Patients: Signed by Norman C. Payson, MD and 8 other NJ health plan CEOs. This voluntary agreement was announced by the Governor of the State of New Jersey on December 16, 1999.

CT Managed Care Accountability Act, Public Act No. 99-284 sec. 15.

CT Public Act No. 16-214

NY Insurance Law §§ 4900, 4910, 4914; NY Social Services Law § 208.

POLICY HISTORY/REVISION INFORMATION

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<th>Action/Description</th>
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<tr>
<td>04/01/2019</td>
<td>• Reorganized policy template; simplified and relocated Instructions for Use and Benefit Considerations section</td>
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| 02/01/2019 | • Updated list of related policies; added reference link to the policy titled Acquired Rare Disease Drug Therapy Exception Process  
• Updated conditions of coverage to clarify this policy applies to Connecticut (CT) and New York (NY) Commercial plan membership  
• Revised coverage rationale for Connecticut plans only; added language to indicate:  
  o A patient is eligible to receive treatment with an investigational drug, biological product or device if the patient has:  
    ▪ Considered all other treatment options currently approved by the federal FDA;  
    ▪ Been unable to participate in a clinical trial for the terminal illness that is not more than 100 miles from the patient's home address, or not been accepted to a clinical trial not more than 1 week after completion of the clinical trial application process;  
    ▪ Received a recommendation from his or her treating physician for an investigational drug, biological product or device;  
    ▪ Given written, informed consent for the use of the investigational drug, biological product or device or, if the patient is a minor or lacks the mental capacity to provide informed consent, a parent of the minor or a legal guardian of the minor or adult patient has given such written, informed consent on the patient's behalf; and  
    ▪ Obtained written documentation from his or her treating physician stating that the patient meets the requirements of this subsection  
  o "Investigational drug, biological product or device" means a drug, biological product or biological device that has successfully completed a phase one clinical trial of the federal Food and Drug Administration (FDA) but has not yet been approved for general use by the FDA and remains under investigation in a clinical trial approved by the FDA  
• Updated supporting information to reflect the most current references  
• Archived previous policy version EXPERIMENTAL 003.11 T2 |

INSTRUCTIONS FOR USE

This policy provides assistance in interpreting UnitedHealthcare Oxford standard benefit plans. When deciding coverage, the member specific benefit plan document must be referenced as the terms of the member specific benefit plan may differ from the standard plan. In the event of a conflict, the member specific benefit plan document governs. Before using this policy, please check the member specific benefit plan document and any applicable federal or state mandates. UnitedHealthcare Oxford reserves the right to modify its policies as necessary. This policy is provided for informational purposes. It does not constitute medical advice.

The term Oxford includes Oxford Health Plans, LLC and all of its subsidiaries as appropriate for these policies. Unless otherwise stated, Oxford policies do not apply to Medicare Advantage members.

UnitedHealthcare may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. UnitedHealthcare Oxford policies are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice.