

ACQUIRED RARE DISEASE DRUG THERAPY EXCEPTION PROCESS

Policy Number: ADMINISTRATIVE 192.10 T2

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Related Policies

- [Clinical Trials](#)
- [Experimental/Investigational Treatment](#)
- [Off-Label/Unproven Specialty Drug Treatment](#)

INSTRUCTIONS FOR USE

The services described in Oxford policies are subject to the terms, conditions and limitations of the member's contract or certificate. Unless otherwise stated, Oxford policies do not apply to Medicare Advantage members. Oxford reserves the right, in its sole discretion, to modify policies as necessary without prior written notice unless otherwise required by Oxford's administrative procedures or applicable state law. The term Oxford includes Oxford Health Plans, LLC and all of its subsidiaries as appropriate for these policies.

Certain policies may not be applicable to Self-Funded members and certain insured products. Refer to the member specific benefit plan document or Certificate of Coverage 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 specific benefit plan document or Certificate of Coverage, the member specific benefit plan document or Certificate of Coverage will govern.

CONDITIONS OF COVERAGE

Applicable Lines of Business/Products	This policy applies to Oxford New York Commercial plan membership.
Benefit Type	General benefits package
Referral Required (Does not apply to non-gatekeeper products)	No
Authorization Required (Precertification always required for inpatient admission)	Yes ¹
Precertification with Medical Director Review Required	Yes ¹
Applicable Site(s) of Service (If site of service is not listed, Medical Director review is required)	Inpatient, Outpatient, Office
Special Considerations	¹ Precertification with review by a Medical Director review or their designee is required. This policy may need to be reviewed in conjunction with Oxford's policy on Experimental/Investigational Treatment .

BENEFIT CONSIDERATIONS

Before using this policy, please check the member specific benefit plan document and any federal or state mandates, if applicable.

Essential Health Benefits for Individual and Small Group

For plan years beginning on or after January 1, 2014, the Affordable Care Act of 2010 (ACA) requires fully insured non-grandfathered individual and small group plans (inside and outside of Exchanges) to provide coverage for ten categories of Essential Health Benefits ("EHBs"). Large group plans (both self-funded and fully insured), and small group ASO plans, are not subject to the requirement to offer coverage for EHBs. However, if such plans choose to provide coverage for benefits which are deemed EHBs, the ACA requires all dollar limits on those benefits to be removed on all Grandfathered and Non-Grandfathered plans. The determination of which benefits constitute EHBs is made on a state by state basis. As such, when using this policy, it is important to refer to the member specific benefit plan document to determine benefit coverage.

DEFINITIONS

Acquired Rare Disease: The disease is developed after birth and occurs infrequently and in a small number of patients. The disease will be considered rare if there are not enough patients with the disease to have a standard of care or justify a clinical trial or provide enough data to have a peer review published study at the time of the request.

Center of Excellence: Academic medical centers that routinely conduct nationally sponsored, multi-centered clinical trials in the category of disease the member has, and have received designation as an institution with special standing in the treatment of the disease (for example, a Comprehensive Cancer Center for the treatment of cancer).

DESCRIPTION OF SERVICES

Coverage for experimental and investigational treatments and procedures is specifically excluded under the member's Certificate with Oxford. In addition to the clinical situations for which Oxford will approve benefits for Experimental and Investigational Therapy (see Oxford's [Experimental/Investigational Treatment](#) policy) and Clinical Trials (see Oxford's [Clinical Trials](#) policy), Oxford will also consider requests for off-label usage of an FDA approved drug where the member has an Acquired Rare Disease and the drug therapy request is clinically reasonable.

Off-label drug therapies are those medical therapies that use a FDA approved drug for a non-indicated use (an unproven indication for that drug). (See Oxford's [Off-Label/Unproven Specialty Drug Treatment](#) policy.)

Unproven therapies/drugs are treatments or procedures that lack significant medical documentation and/or evidence to support their medical effectiveness for any indication.

POLICY AND RATIONALE

Oxford recognizes that for certain types of rare diseases there may be no standard of care treatment or peer reviewed documents in scientific and medical literature which establish that an experimental and/or investigational treatment or procedure may be more beneficial than the standard treatments available to treat a member's Acquired Rare Disease.

Oxford has determined that it will create a limited exception to the exclusion of experimental and investigational treatments and approve benefits for in-network drug therapies 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 specialist/consultant/peer reviewer with expertise in the area of practice appropriate to treat the member's Acquired Rare Disease.

Unproven Therapies

Under no circumstances will this policy extend coverage to Unproven Therapies.

Unproven therapies are treatments or procedures that lack significant medical documentation and/or evidence to support their medical effectiveness. Oxford does not provide coverage for any treatment modality that has not been proven medically effective (other than as discussed below) or is not generally recognized as effective or appropriate for the particular diagnosis or treatment of the member's particular condition or disease.

TREATMENT AND APPLICATION GUIDELINES

After receiving a request for experimental treatment of an Acquired Rare Disease, an Oxford Medical Director will review the relevant clinical and patient information. As part of that review, the Medical Director, in his/her discretion, will determine whether the disease is an Acquired Rare Disease and whether the proposed drug therapy is clinically reasonable.

For purposes of this policy, clinically reasonable means:

- The drug is FDA approved and is not contraindicated for the proposed use.

- There is evidence of early success with the drug therapy and at least a small number of patients with the same Acquired Rare Disease have responded to treatment but there is not enough information to have a peer review published study at this time.
- The evidence showing early success is from a Center of Excellence which treats members with the same Acquired Rare Disease.
- The benefit likely exceeds the risk to the member in receiving the drug therapy.
- The treatment results will be available for use by the medical community by establishment of a patient registry to evaluate the effectiveness of the drug therapy for patients with this Acquired Rare Disease.
- The member has not failed a previous course or trial of the drug therapy.
- The member does not have any other comorbidity which would preclude the proposed drug therapy.
- The member has signed an informed consent.

The Medical Director will consult with the specialist who has received early success with use of the proposed treatment if possible and/or an outside consultant. The specialist/consultant must have credentials in the specific discipline of medicine that treats the member's Acquired Rare Disease. That specialist or consultant will be asked to certify that the basis of the medical documents submitted that:

- The member has an Acquired Rare Disease.
- There have not been, and are not likely to be in the period of time during which the member must be treated, either clinical trials or articles published in the peer reviewed medical literature showing that the proposed treatment is likely to benefit patients who have the specific rare disease.
- The requested drug therapy protocol is clinically reasonable to treat the member's Acquired Rare Disease, with stated rationales that support that conclusion.
- Based on the consultant's opinion, the benefits of the treatment are likely to outweigh the risks of treatment.
- The specialist/consultant has treated patients with this condition.

Precertification will be required for each course of drug therapy. If the member does not respond to the initial prescribed course of drug therapy, Oxford will not continue to approve the therapy and the therapy will be denied as an Unproven Therapy.

Documentation

The following documentation must be submitted to Oxford demonstrating the criteria below have been satisfied. Without all such documentation, Oxford will deny any such request.

Necessary Information

The following supporting documentation must be provided by the member and/or the member's provider for consideration of the drug therapy:

- Certification from the member's attending physician* which includes:
 - A statement that the member has an Acquired Rare Disease.
 - A statement of the evidence relied upon to recommend the proposed drug therapy and a statement of why any 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.
 - A copy of any available medical and scientific evidence, upon which the attending physician based his recommendation for the proposed 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 written description of the proposed treatment (or protocol if available), which must include:
 - Specific goals
 - A rationale and background for the plan
 - Criteria for patient selection
 - Specific directions for administering the therapy
 - Specific directions for the monitoring of patients
 - A definition of quantitative measures for determining treatment or intervention response
 - 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 effectiveness of the proposed drug therapy for treatment of the member's condition and the contact information for the specialist who can discuss the evidence of early success of the drug therapy with an Oxford Medical Director.
- Depending upon the nature of the proposed drug therapy and/or the member's disease, the specialist/consultant or Oxford may require additional documentation to review the requested therapy.

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 drug therapy.

PAYMENT GUIDELINES

This policy creates a limited exception to the total exclusion of benefits for Experimental/Investigational treatments. Generally, payment of benefits under this policy is limited to participating providers within Oxford's network contracted specifically to provide these services.

Oxford will only consider requests for Acquired Rare Disease treatment at an out-of-network Center of Excellence if the requested drug therapy meets the criteria in this policy and no network physician will treat the member using the requested drug protocol. Even if Oxford approves drug therapy at an out-of-network Center of Excellence, Oxford reserves the right to move the member to an in-network provider for future treatments if an Oxford provider becomes available.

In-network and out-of-network benefits are subject to all applicable cost sharing requirements. Out-of-network services will be paid based upon the out-of-network or UCR fee schedule applicable to the member's plan and subject to the member's out-of-network cost-share.

REFERENCES

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

POLICY HISTORY/REVISION INFORMATION

Date	Action/Description
02/01/2018	<ul style="list-style-type: none">Added reference link to policy titled <i>Off-Label/Unproven Specialty Drug Treatment</i>Updated supporting information to reflect the most current description of servicesArchived previous policy version ADMINISTRATIVE 192.9 T2