

Chapter 1

Section 3.1

Rare Diseases

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1.0 DESCRIPTION

TRICARE defines a rare disease as any disease or condition that affects less than 200,000 persons in the United States.

2.0 POLICY

2.1 Coverage for treatment of rare diseases may be considered on a case-by-case basis. Case-by-case review is not required for drugs, devices, medical treatments, and procedures that have already been established as safe and effective for treatment of rare diseases.

2.1.1 In reviewing the case, any or all of the following sources may be used to determine if the proposed benefit is considered safe and effective.

2.1.2 Trials published in refereed medical literature.

2.1.3 Formal technology assessments.

2.1.4 National medical policy organization positions.

2.1.5 National professional associations.

2.1.6 National expert opinion organizations.

2.2 If case review indicates that the proposed benefit for a rare disease is safe and effective for that disease, benefits may be allowed. If benefits are denied, an appropriate appealing party may request an appeal.

2.3 Off-label use of rituximab may be considered for cost-sharing for the treatment of recurrent nodular CD20 positive lymphocyte predominant Hodgkin's disease. The effective date is January 1, 2003.

2.4 Off-label use of rituximab may be considered for cost-sharing in reducing proteinuria for the treatment of Immunoglobulin A (IgA) nephropathy (proliferative glomerulonephritis). The effective date is May 1, 2007.

2.5 Effective May 13, 2009, Intraperitoneal Hyperthermic Chemotherapy (IPHC) (Current Procedural Terminology (CPT)¹ procedure codes 77600, 77605, and 96445) in conjunction with cytoreductive surgery or peritonectomy for treatment of pseudomyxoma peritonei resulting from appendiceal carcinoma may be covered on a case-by-case basis for adult patients when all of the following criteria are met:

- There is no evidence of distant metastasis.
- There is evidence of low histological aggressiveness of the disease.
- The patient has not undergone preoperative systemic chemotherapy.
- The patient's condition does not preclude major surgery.
- The chemotherapeutic agents used are Mitomycin C, Cisplatin (also known as Cisplatinum), or Fluorouracil.

2.6 External Infusion Pumps (EIPs) for insulin may be considered for cost-sharing when the diagnosis is Cystic Fibrosis-Related Diabetes (CFRD) with fasting hyperglycemia. See [Chapter 8, Section 2.3](#) for policy regarding EIPs. Effective January 21, 2009.

2.7 Post-operative proton beam radiosurgery/radiotherapy (CPT¹ procedure codes 77520, 77522, 77523, and 77525) may be considered for cost-sharing when the diagnosis is sacral chordoma. See [Chapter 5, Section 3.1](#) for policy regarding proton beam radiosurgery/radiotherapy.

2.8 Extracorporeal photopheresis (CPT¹ procedure code 36522) may be considered for cost-sharing when the diagnosis is Bronchiolitis Obliterans Syndrome (BOS) that is refractory to immunosuppressive drug treatment. See [Chapter 4, Section 9.2](#) for policy regarding photopheresis.

3.0 EXCLUSIONS

3.1 Intracranial angioplasty with stenting (CPT¹ procedure code 61635) of the venous sinuses for treatment of pseudotumor cerebri (also known as idiopathic intracranial hypertension and benign intracranial hypertension) is unproven.

3.2 Off-label use of rituximab for pediatric Immunoglobulin A (IgA) is unproven.

3.3 Proton Beam Therapy (PBT)/radiosurgery/radiotherapy for the treatment of thymoma is unproven.

- END -

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