

Chapter 1

Section 3.1

Rare Diseases

Issue Date: May 18, 1994

Authority: [32 CFR 199.2\(b\)](#) and [32 CFR 199.4\(g\)\(15\)](#)

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 Off-label use of rituximab (HCPCS J9310) may be considered for cost-sharing for the treatment of neuromyelitis optica. The effective date is March 26, 2010.

2.6 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.

2.7 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.

2.8 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.

2.9 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.

2.10 Off-label use of Selective Internal Radiation Therapy (SIRT) with yttrium-90 microspheres (resin or glass) may be considered for cost-sharing for the treatment of unresectable liver metastases from neuroendocrine tumors. The effective date is May 1, 2008. See [Chapter 5, Section 3.2](#) for policy regarding brachytherapy/radiation therapy.

2.11 Radiofrequency Ablation (RFA), when performed using an U.S. Food and Drug Administration (FDA) approved electrosurgical cutting and coagulation device, may be considered for cost-sharing for the treatment of liver metastases from gastric cancer. The effective date is June 1, 2010.

2.12 Effective September 1, 2012, the NovoTTF-100A system (HCPCS A4555 and E0766) may be cost-shared for treatment of adult patients (22 years of age or older) with recurrent glioblastoma after surgical and radiation options have been exhausted.

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2.13 Effective February 4, 2011, Radiesse® Voice laryngoplasty injections may be cost-shared for the treatment of type 1 laryngeal cleft (also described as supraglottic interarytenoid defects that extend no further than the true vocal folds).

2.14 Effective November 27, 1995, Orthotopic Liver Transplantation (OLT) may be cost-shared for the treatment of Crigler-Najjar Syndrome Type I. OLT may be performed both prior to the onset of neurological symptoms or after the onset of neurological symptoms.

2.15 Effective June 5, 2013, off-label use of intravenous immune globulin for the treatment of Hashimoto's Encephalopathy, may be considered in exceptional circumstances where there is progressive neurological decline despite appropriate steroid therapy or where steroid therapy is contraindicated.

2.16 Effective January 4, 2013, allogeneic hematopoietic cell transplant (CPT² procedure code 38240) for the treatment of primary plasma cell leukemia.

2.17 Off-label use of Photodynamic Therapy (CPT² procedure code 67221) with Visudyne (HCPCS J3396) may be considered for cost-sharing for the treatment of retinal astrocytic hamartoma in Tuberous Sclerosis. The effective date is February 1, 2008.

2.18 Effective June 25, 2014, intracranial angioplasty with stenting (CPT² procedure code 61635) of the venous sinuses may be considered for cost-sharing for the treatment of pseudotumor cerebri (also known as idiopathic intracranial hypertension and benign intracranial hypertension).

2.19 Effective February 1, 2012, OLT (CPT² procedure code 47135) for the treatment of Acute Intermittent Porphyria.

3.0 EXCLUSIONS

3.1 The off-label use of rituximab for the treatment of pediatric linear Immunoglobulin A (IgA) dermatosis is unproven.

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

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