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)(ii)

Revision: C-2, May 17, 2017

1.0 DESCRIPTION

TRICARE defines a rare disease as any disease or condition that has a prevalence of less than 200,000 persons in the United States (U.S.).

2.0 POLICY

2.1 Coverage for treatment of rare diseases may be considered on a case-by-case basis. In reviewing the case, the contractor is authorized to approve coverage when it is determined that the proposed treatment for the rare disease is medically necessary, including that the treatment is safe and effective.

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

2.1.1.1 Trials published in refereed medical literature.

2.1.1.2 Formal technology assessments.

2.1.1.3 National medical policy organization positions.

2.1.1.4 National professional associations.

2.1.1.5 National expert opinion organizations.

2.2 In those situations where the contractor finds the proposed treatment is not considered safe and effective, the contractor shall forward the case to the Medical Benefits & Reimbursement Section (MB&RS) to permit the Director, Defense Health Agency (DHA), or designee, to complete an individual case review and make a determination in accordance with 32 CFR 199.4(g)(15)(ii).

2.3 TRICARE Encounter Data (TED) Record Special Processing Code "**RD** - Rare Diseases" shall be coded on all TED records where the contractor has approved treatment for a rare disease. Assignment of Special Processing Code **RD** will allow the DHA to identify procedures approved by contractors

under the Rare Diseases policy and will allow bypass of TED edit 2-160-01R when the procedure code is on the No Government Pay Procedure Code List.

2.4 The contractor shall provide a monthly report as described in the Contract Data Requirements List (CDRL). The report should not include the rare disease treatments previously approved for coverage and listed herein.

2.5 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.

2.6 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.

2.7 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.

2.8 Effective May 13, 2009, Intraperitoneal Hyperthermic Chemotherapy (IPHC) (Current Procedural Terminology (CPT) 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.

2.9 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.

2.10 Post-operative proton beam radiosurgery/radiotherapy (CPT 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.

2.11 Extracorporeal photopheresis (CPT 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.

2.12 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.

2.13 Effective April 15, 2016, Collagen Cross-linking for the treatment of corneal ectasia due to the rare disease Keratoconus is safe and effective and may be considered for cost-sharing.

2.14 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.

2.15 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.

2.16 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).

2.17 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.

2.18 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 neurologic decline despite appropriate steroid therapy or where steroid therapy is contraindicated.

2.19 Effective April 30, 2009, Intrapulmonary Percussive Ventilation (IPV) may be considered for cost-sharing when the diagnosis is Cystic Fibrosis (CF). See Chapter 8, Section 16.1 for policy regarding IPVs.

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

2.21 Off-label use of Photodynamic Therapy (CPT 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.

2.22 Effective June 25, 2014, intracranial angioplasty with stenting (CPT 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).

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

2.24 Effective December 1, 2014, Photodynamic Therapy for the treatment of Central Serous Chorioretinopathy.

2.25 Effective March 31, 2005, off-label use of rituximab injections may be considered for cost-sharing for the treatment of Stiff Person Syndrome.

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.

3.3 Proton Beam Radiation Therapy (PBRT) for the treatment of juvenile nasal angiofibroma is unproven.

3.4 TRICARE Overseas Program (TOP) beneficiaries are not subject to the requirements of this policy.

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