

## Chapter 1

## Section 3.1

### Rare Diseases

Issue Date: May 18, 1994

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

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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)<sup>1</sup> 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.

### **3.0 EXCLUSION**

Intracranial angioplasty with stenting (CPT<sup>1</sup> procedure code 61635) of the venous sinuses for treatment of pseudotumor cerebri (also known as idiopathic intracranial hypertension and benign intracranial hypertension) is unproven.

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