**Sample Format: Letter of Medical Necessity**

[Insert onto physician letterhead]

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| --- | --- |
| [Medical Director][Insurance Company][Address][City, State, ZIP] | **RE: Member Name** [Insert Member Name]**Member Number** [Insert Member Number]**Group Number** [Insert Group Number] |

**REQUEST:** Authorization for treatment with CTEXLITM (chenodiol) tablets

**DOSE AND FREQUENCY:** [Insert Dose & Frequency]

**REQUEST TYPE:** EXPEDITED/PRIORITY REVIEW

Dear [Insert Name of Medical Director]:

I am writing to support my request for an **expedited** **authorization** for my patient mentioned above to receive CTEXLITM (chenodiol) tablets. CTEXLI is indicated for the treatment of cerebrotendinous xanthomatosis (CTX) in adults.

This patient has previously been approved for Chenodal**®** (chenodiol) treatment in CTX under an FDA medical necessity allowance. I am requesting a switch from Chenodal due to the recent approval granted by the FDA for CTEXLI tablets for the treatment of CTX in adults. This update requires that patients diagnosed with CTX and currently taking Chenodal tablets be transitioned to CTEXLI (NDC 79378-0310-90). CTEXLI contains the same active ingredient as Chenodal. There are no differences in the dosage, strength or the drug product. No changes to this patient’s dosage or strength are required.

Cerebrotendinous xanthomatosis is an ultra-rare, progressive, genetic lipid storage disorder. CTX can present in early childhood with a range of clinical manifestations. Four hallmark clinical symptoms are chronic diarrhea, early onset cataracts, tendon xanthomas, and progressive neurological deterioration. The clinical presentation of CTX patients can vary widely which often results in delayed diagnosis. These patients are often diagnosed and managed by a variety of specialist such as geneticists, pediatricians, and ophthalmologists.

Early identification and treatment of CTX is essential in order to delay or prevent neurological deterioration. Diagnostic criteria generally includes a combination of symptoms and biochemical markers or genetic testing. Left untreated, CTX patients can develop worsening ataxia, seizures, and dementia. This could eventually progress to severe disability or premature death.

CTEXLI is administered orally three times daily. CTEXLI was studied in the phase 3 RESTORE trial and showed meaningful reductions in urine bile alcohols and cholestanol. Both biomarkers are linked to CTX disease progression. The study included double-blinded CTEXLI withdrawal periods, resulting in rapid and significant increases to these key biomarkers. During the withdrawal periods, the majority of patients required blinded rescue therapy with CTEXLI.

Due to the rapid increase of toxic biomarkers, any delay in patient transition to CTEXLI may irreversibly worsen the patient’s CTX, potentially including neurological deterioration.

This letter serves to document my patient’s diagnosis, medical history and to summarize my treatment rationale.

**Summary of Patient’s Diagnosis and History**

[Patient Name] is [Age] years old and was initially diagnosed with [Diagnosis] [ICD-10-CM] on [Date]. This diagnosis was confirmed by [insert details of patient’s genetic testing and/or clinical symptoms]. [Patient Name] has been in my care since [Date].

[Insert a summary of the patient’s clinical history, current symptoms and condition, and relevant lab/test results]. Highlight the factors leading you to recommend use of CTEXLI. Include any relevant previous treatments of CTX, such as Chenodal, with patient’s response to those interventions.

Note: Exercise your medical judgment and discretion when providing a diagnosis and characterization of the patient’s medical condition].

**Rationale for Treatment**

[Include your clinical rationale, patient’s likely prognosis without continuing treatment with CTEXLI and your credentials in treating CTX].

Considering the patient’s history, condition, current treatment for CTX, and the full Prescribing Information supporting uses of CTEXLI, I believe treatment with CTEXLI at this time is medically necessary and should be a covered treatment for my patient. [Include support for treatment rationale: You may consider including documents that provide additional clinical information to support the recommendation for CTEXLI for this patient, such as the full Prescribing Information or peer-reviewed journal articles].

Given the urgent nature of this request, please provide an expedited priority review and authorization. Contact my office at [insert phone number] if I can provide you with any additional information.

Sincerely,

[Insert Physician Name and Participating Provider Number]

Enclosures: [include full Prescribing Information and the additional support noted above].

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