**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 CHOLBAM® (cholic acid) capsules

**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 CHOLBAM® (cholic acid) capsules. CHOLBAM® is an FDA approved medication for the treatment of bile acid synthesis disorders (BASDs) due to single enzyme defects (SEDs) including Smith-Lemli-Opitz Syndrome (SLOS). A non-exhaustive list of BASDs due to SEDs is included below. CHOLBAM® is also FDA approved for adjunctive treatment of peroxisomal disorders (PDs), including Zellweger spectrum disorder (ZSD) in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat-soluble vitamin absorption.

|  |
| --- |
| **Single Enzyme Defect** |
| 3β-HSD |
| AKR1D1 |
| CTX |
| AMACR |
| CYP7A1\* |
| Smith-Lemli-Opitz\*† |

Table 4 from CHOLBAM® prescribing information

\*Indicates no evaluable patients in the pivotal trials for CHOLBAM® approval

†CHOLBAM® along with a cholesterol supplement increased plasma cholesterol in a pilot study of 12 SLOS patients

SLOS, a BASD due to a SED, stems from mutations in 3β-hydroxysterol-Δ7-reductase (DHCR7) which plays a crucial role in the biosynthesis of cholesterol. It is an ultra-rare disease affecting 1 in 20,000 to 1 in 70,000 people. Unlike other BASDs due to SEDs, **SLOS is a disease caused by impaired biosynthesis of cholesterol and is characterized by cholesterol deficiency**. This results in impaired endogenous bile acid production, which negatively impacts absorption of dietary cholesterol worsening cholesterol deficiency. Absorption of fats and fat-soluble vitamins are also decreased.

In addition to decreased cholesterol production, deficient activity of DHCR7 has been demonstrated to increase cholesterol precursors 7-dehydrocholesterol (7-DHC) and 8-dehydrocholesterol (8-DHC). Both precursors are toxic and can interfere with fetal development and growth. An increase in toxic oxysterols has also been noted, which can potentially contribute to progressive retinal and neurological pathologies. Patients diagnosed with SLOS require **urgent treatment** as they are at risk of failure to thrive and neurological delays. **Treatment with CHOLBAM® may improve cholesterol levels and decrease toxic cholesterol precursors.**

Patients with SLOS are often diagnosed and managed by Metabolic Geneticists, Pediatricians, and Primary Care Physicians with other specialists supporting as needed. These specialties can include Ophthalmology, Neurology, Gastroenterology, and Cardiology. Patients with SLOS do not typically report liver abnormalities and therefore, hepatology is not a common treating specialty.

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 or diagnostic assay]. [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 (i.e. Plasma Cholesterol, Cholesterol Precursors, Height, Weight, Liver Panel). Highlight the factors leading you to recommend use of CHOLBAM®.

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 treatment with CHOLBAM® and your credentials in treating SLOS]

In my specialty as a [Insert specialty] or in consultation with a metabolic specialist that cares for patients with SLOS, I have determined this patient is appropriate for CHOLBAM® treatment. Considering the patient’s history, condition, and the full Prescribing Information supporting uses of CHOLBAM®, treatment is medically necessary and should be a covered for my patient. [Include support for treatment rationale: You may consider including documents that provide additional clinical information to support the recommendation for CHOLBAM® for this patient, such as the full Prescribing Information, peer-reviewed journal articles, or clinical guidelines.]

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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