Executive Summary

**Purpose:** Ensure appropriate utilization and control of Nulibry™ (fosdenopterin)

**Why Issue Selected:** On February 26, 2021, Nulibry™ became the first FDA-approved agent to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A, a rare condition with a median age of survival of 4 years that is estimated to occur in 1 in 100,000 - 200,000 newborns worldwide. Three types of MoCD have been classified: Type A, Type B, and Type C. Type A, the most common type, is an autosomal recessive condition caused by a mutation in the molybdenum cofactor synthesis 1 (MOCS1) gene which leads to deficient synthesis of cyclic pyranopterin monophosphate (cPMP). The resultant downstream effect of the DNA sequence change includes a buildup of the neurotoxic metabolite S-sulfocysteine (SSC), which leads to rapid and irreversible neurological damage. Patients with the classical severe form of MoCD often develop the characteristic signs/symptoms of encephalopathy, intractable seizures, feeding difficulties, and developmental delays within the first few days of life.

Prior to Nulibry, no therapeutic alternatives existed beyond supportive therapies, such as those for the management of seizures. Nulibry, administered as a once daily intravenous infusion, functions as a substrate replacement therapy by providing an exogenous source of cPMP. Clinical trials demonstrated an increased 3-year survival rate of 84% vs 55% in patients treated with Nulibry compared to those from a natural history study. The most common side effects were catheter-related complications, pyrexia, and viral infections.

Due to the high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of Nulibry.
### Setting & Population
- Drug class for review: Nulibry™ (fosdenopterin)
- Age range: All appropriate MO HealthNet participants

### Approval Criteria

#### Initial Therapy:
- Confirmed or suspected molybdenum cofactor deficiency (MoCD) Type A **AND**
- Prescribed by or in consultation with a neonatologist or other specialist in the treated disease state

#### Continuation of Therapy:
- Initial approval is for 3 months, renewal of prior authorization may be given following documentation of the following:
  - Genetic testing to confirm biallelic pathogenic variant of MOCS1 gene **AND**
  - Claim does not exceed 0.9 mg/kg per day

### Denial Criteria
- Therapy will be denied if all approval criteria are not met

### Required Documentation

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<thead>
<tr>
<th>Laboratory Results:</th>
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<tbody>
<tr>
<td>Progress Notes:</td>
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<td>MedWatch Form:</td>
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<td>Other:</td>
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### Disposition of Edit

- Denial: Exception code “0682” (Clinical Edit)
- Rule Type: CE

### Default Approval Period

- 1 year

### References