PHARMACY POLICY STATEMENT
North Carolina Marketplace

DRUG NAME	Nulibry (fosdenopterin)
BENEFIT TYPE	Medical or Pharmacy
STATUS	Prior Authorization Required

Nulibry, approved by the FDA in 2021, is cyclic pyranopterin monophosphate (cPMP) indicated to reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A. MoCD Type A is an ultra-rare autosomal recessive, inborn error of metabolism that results in accumulation of a neurotoxic metabolite of sulfite (SSC) which causes rapid and progressive neurological damage that usually presents shortly after birth. Mutations in the molybdenum cofactor synthesis 1 gene (MOCS1) lead to deficient synthesis of cPMP. Nulibry provides a synthetic exogenous source of cPMP as substrate replacement therapy.
Nulibry is the first drug to target the underlying etiology of MoCD Type A and reduce the risk of mortality. Prior to Nulibry, treatment had been strictly supportive, such as anticonvulsants for seizures.

Nulibry (fosdenopterin) will be considered for coverage when the following criteria are met:

Molybdenum Cofactor Deficiency (MoCD) Type A

For initial authorization:

1. Medication must be prescribed by or in consultation with a neonatologist, geneticist, metabolic specialist, or pediatric neurologist; AND
2. ONE of the following:
   a) Member has a diagnosis of MoCD Type A confirmed by genetic testing (must show mutation in the MOSC1 gene), OR
   b) Member has a presumptive diagnosis of MoCD Type A with early presenting characteristics such as seizures of unknown origin, exaggerated startle response, axial hypotonia, strongly positive sulfite dipstick, etc AND genetic testing is to be immediately completed.
   NOTE: If genetic testing does not confirm the diagnosis, Nulibry must be discontinued; AND
3. Documentation of baseline S-sulfocysteine (SSC) level; AND
4. ' R F X P H Q W D W L R Q R I P. H P E H U ¶ V Z H L J K W
5. Dosage allowed/Quantity limit:
   Less than 1 year of age: Dosing based on weight per package insert
   Age 1 year or older: 0.9 mg/kg IV once daily

If all the above requirements are met , the medication will be approved for 6 months .