Physician Administered Drugs

ITVISMA® (onasemnogene abeparvovec-brve)

C9309 1 unit = 1 Therapeutic Dose

Indications

ITVISMA is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in SMN1 gene.

PA CRITERIA:

Patient demographics

A. Age of patient is within the age range as recommended by the FDA label;
AND
B. There are no contraindications or documented intolerance to corticosteroid therapy

Diagnostic criteria

A. Genetic testing confirms SMA with the presence of **ONE** of the following:

1. Homozygous deletions of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13)

OR

2. Homozygous mutation in the SMN1 SMN1 gene (e.g., biallelic mutations of exon 7)

OR

3. Compound heterozygous mutation in the SMN1 gene [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)]

AND
B. Genetic testing confirming 4 or fewer copies of SMN2 gene

Previous treatment

A. Patient has not received Zolgensma

AND

B. If the patient is on nusinersen (Spinraza) or risdiplam (Evrysdi), it will be discontinued prior to administration of Itvisma.

Current status

Patient must not have advanced SMA, including but not limited to any of the following:

A. Complete paralysis of limbs

OR

B. Invasive ventilator support (tracheostomy)

OR

C. Respiratory assistance for more than 16 or more hours per day (including non-invasive respiratory support) continuously for 14 or more days in the absence of acute reversible illness (excluding perioperative ventilation)

Diagnostic/Treatment teams

Diagnosed and prescribed by a medical geneticist, a pediatric neuromuscular specialist, or a neurologist with experience in the diagnosis and management of SMA

Concomitant therapy

Patient will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and following receipt of Itvisma within accordance of the FDA approved Itvisma labeling

Baseline information

A. Documentation of baseline laboratory tests demonstrating Anti-AAV9 titers $\leq$ 1:50 as determined by ELISA binding immunoassay, (if Anti-AAV titers > 1:50, retesting may be performed provided age requirement at time of dosing is still met)

AND

B. Documentation of baseline liver assessments (e.g., alanine aminotransferase, total bilirubin, gamma-glutamyl transferase or glutamate dehydrogenase), platelet counts, and troponin-I less than the upper limit of normal

Other commitments

A. Physician attests that liver assessments (e.g., alanine aminotransferase, total bilirubin, gamma-glutamyl transferase or glutamate dehydrogenase), platelet counts, and troponin-I ; as recommended in the package insert or in compliance with current standards will be performed to assess safety

AND

B. Itvisma will be dosed in accordance with the FDA approved Itvisma labeling

AND

C. Itvisma will be administered intrathecally in accordance with the FDA approved Itvisma labeling

NOTES:

- Single, one time injection per lifetime
- Dose to be administered does not exceed one kit of Itvisma