

For brokers and producers only

Date: May 16, 2019

Market: **All (Excludes Medicare Supplement)**

Zolgensma – Pending FDA Approval

Zolgensma, a new gene therapy which treats spinal muscular atrophy (SMA) Type 1, is currently being reviewed for approval by the Food and Drug Administration (FDA). Zolgensma is a one-time IV infusion with a speculated cost of \$4 million per treatment. The FDA is expected to make a decision by May 17, 2019.

If approved, Zolgensma will be covered as a CareFirst medical benefit and subject to prior authorization. Due to the time sensitivity of the drug's administration, all prior authorizations will be reviewed within 48 hours. Sites of care are expected to vary between inpatient and outpatient setting. Zolgensma will not be subject to site of care management due to the member's expected young age at the time of administration.

CareFirst is actively exploring value and outcomes-based contracting opportunities for Zolgensma.

About SMA

SMA is a rare, genetic neuromuscular disease characterized by a deficiency in the survival motor neuron protein. This protein deficiency causes irreversible, progressive degeneration of motor neurons, preventing patients from reaching motor milestones or retaining motor functions.

SMA is estimated to effect 1 in 10,000 live births or 500 new cases per year. The most severe cases affect infants and young children.

There are four subtypes of SMA, which are related to the age of onset and number of motor milestones reached. Type 1 SMA represents approximately 60% of cases. It's estimated that 125-250 patients are living with SMA Type 1 in the U.S. The age of onset for patients with Type 1 SMA is between birth and 6 months old. The highest achievement motor milestone for SMA Type 1 is sitting up with support only. The natural age of death for these patients is less than 2 years old.

Currently, Spinraza (nusinersin) is the only FDA approved treatment option for SMA Type 1. Similar to Zolgensma's management plan, Spinraza is also covered under the medical benefit and subject to prior authorization. Spinraza costs \$750,000 for the first year of treatment (\$125,000/injection) and \$375,000 per year thereafter. Its treatment plan includes induction and maintenance phases comprised of multiple intrathecal infusions over the course of 1-2 years.

For more information

If you have any questions, please contact your broker sales representative.