MassHealth Zolgensma (onasemnogene abeparvovec-xioi) Monitoring Program

Zolgensma (onasemnogene abeparvovec-xioi) requires prior authorization (PA) and will be managed by the Zolgensma Monitoring Program.

Zolgensma (onasemnogene abeparvovec-xioi)

Zolgensma (onasemnogene abeparvovec-xioi) is a prescription gene therapy used to treat spinal muscular atrophy (SMA) in children less than two years of age. Zolgensma is designed to deliver a functional copy of the human survival motor neuron (SMN) gene, replacing the non-functional or missing SMN1 gene. After a single intravenous infusion, Zolgensma results in the expression of the SMN protein.

Zolgensma Monitoring Program

Zolgensma will require PA. This therapy is administered by a one-time infusion and the durability of response is currently unknown. As such, MassHealth Drug Utilization Review (DUR) will be reaching out to prescribers yearly to monitor for ongoing clinical effectiveness and to confirm sustained response.

Following PA approval, MassHealth DUR will outreach to the prescriber to inform of the Zolgensma Monitoring Program and confirm administration date.

Approximately one year following infusion, the prescriber will need to submit documentation of the member's response to treatment. Information collected will include survival, need for respiratory assistance, including permanent invasive ventilation, neuromuscular functional assessments and use of other medications for SMA.

DUR will be outreaching at ongoing, yearly intervals in order to conduct long-term monitoring for Zolgensma. At these intervals, prescribers will inform DUR of the member's response.

1. Zolgensma [package insert]. Bannockburn, IL (NJ): AveXis, Inc.; 2019 May.