Skysona™ (elivaldogene autotemcel) – FDA drug safety communication

- On November 27, 2024, the FDA announced it is investigating reports of hematologic malignancies associated with the use of Skysona (elivaldogene autotemcel).
- The FDA has received reports of hematologic malignancies, including life-threatening cases of myelodysplastic syndrome and acute myeloid leukemia, following treatment of early, active cerebral adrenoleukodystrophy (CALD) patients with Skysona.
    - Reports were received from clinical trials, with cases diagnosed between 14 to 92 months post-treatment.
- Skysona is an autologous hematopoietic stem cell (HSC)-based gene therapy approved to slow the progression of neurologic dysfunction in boys 4 - 17 years of age with early, active CALD.
- The FDA is investigating the known risk of hematologic malignancies with serious outcomes, including those such as hospitalization, the requirement for allogeneic HSC transplantation, and death, and is evaluating the need for further regulatory action.
- Given the risk of hematologic malignancy, providers should carefully consider alternative therapies, including allogeneic HSC transplant for patients who have a suitable, willing, and available human leukocyte antigen (HLA)-matched donor, prior to deciding to treat a child with Skysona.
- Skysona carries a boxed warning for hematologic malignancy.
    - Patients and clinical trial participants receiving treatment with Skysona should be monitored lifelong for hematologic malignancy.
    - Patients should be closely monitored with complete blood counts at least every 3 months and through assessments for evidence of clonal expansion or predominance at least twice in the first year after Skysona administration and annually thereafter, and bone marrow evaluations should be considered as clinically indicated.