Zolgensma acute liver failure update

Aug 11, 2022

Novartis Gene Therapies is committed to patient safety and the ongoing monitoring of adverse events (AEs) as it relates to the use of Zolgensma® (onasemnogene abeparvovec-xioi), a one-time gene therapy for spinal muscular atrophy (SMA). Acute liver failure is a known side effect and is highlighted in the Zolgensma product's labeling including the Box Warning in the U.S. Prescribing Information. Following two recent patient fatalities, and in alignment with health authorities, we will be updating the labeling to specify that fatal acute liver failure has been reported. While this is clinically important safety information, it is not a new safety signal and we firmly believe in the overall favorable risk/benefit profile of Zolgensma, which to date has been used to treat more than 2,300 patients worldwide across clinical trials, managed access programs, and in the commercial setting.

We have notified health authorities in all markets where Zolgensma is used and are communicating to relevant healthcare professionals as an additional step in markets where this action is supported by health authorities.
