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(來函請敍明此檔案號碼)
(IN REPLY PLEASE QUOTE THIS FILE REF.)

Dear Healthcare Professionals,

Zolgensma: fatal cases of acute liver failure

Your attention is drawn to the European Medicines Agency's (EMA) announcement that the Pharmacovigilance Risk Assessment Committee (PRAC) has discussed a direct healthcare professional communications (DHPC) containing important information for Zolgensma (onasemnogene abeparvovec).

Fatal cases of acute liver failure were recently reported in patients treated with Zolgensma (onasemnogene abeparvovec), a gene therapy medicine for the treatment of spinal muscular atrophy (SMA), a serious rare condition of the nerves that causes muscle wasting and weakness.

PRAC's DHPC informs healthcare professionals of the fatal cases of liver failure and the updated recommendations for monitoring liver function, assessing suspected liver injury after infusion and further advice regarding tapering the corticosteroid treatment.

PRAC advises that healthcare professionals should promptly assess patients with worsening liver function tests and/or signs or symptoms of acute liver illness. If patients do not respond adequately to treatment with corticosteroids, treating physicians should consult a paediatric gastroenterologist or hepatologist and consider adjustment of the corticosteroid regimen.

PRAC's DHPC for Zolgensma will be forwarded to EMA's committee for advanced therapies (CAT) and to EMA's human medicines committee (CHMP). Following the CHMP decision, the DHPC will be disseminated to healthcare professionals by the marketing authorisation holders, according to an agreed communication plan, and published on the EMA's 'Direct healthcare professional communications' page and in national registers in European Union (EU) Member States.

Please refer to the following website in EMA for details:
https://www.ema.europa.eu/en/news/meeting-highlights-pharmacovigilance-risk-assessment-committee-prac-9-12-january-2023

In Hong Kong, there is one registered pharmaceutical product containing onasemnogene abeparvovec, namely Zolgensma Solution For Infusion 2 X 10^13 Vector Genomes/ml (HK-67654). The product is registered by Novartis Pharmaceuticals (HK) Limited. It is a prescription-only medicine. So far, the Department of Health (DH) has not received any case of adverse drug reaction related to onasemnogene abeparvovec. Related news on the risk of acute liver failure associated with the use of onasemnogene abeparvovec was previously issued by Health Canada, and was posted on the Drug Office website on 13 September 2022. The current package insert of the above local onasemnogene abeparvovec-containing product include safety information on the risk of acute liver failure. In light of the above EMA's announcement, the matter will be discussed by the Registration Committee of the Pharmacy and Poisons Board.

Please note that this letter serves as a means for the DH to communicate important new safety information about registered pharmaceutical products with healthcare professionals in Hong Kong and is not intended to serve as guidelines or to replace professional clinical judgement. Healthcare professionals are advised to balance the risk of possible adverse effects against the benefit of treatment.

Please report any adverse events caused by drugs to the Adverse Drug Reaction Unit of the DH (tel. no.: 2319 2920, fax: 2319 6319 or email: adr@dh.gov.hk). For details, please refer to the website at Drug Office under "ADR Reporting": http://www.drugoffice.gov.hk/adr.html. You may wish to visit the Drug Office's website for subscription and browsing of "Drug News" which is a monthly digest of drug safety news and information issued by Drug Office.

Yours faithfully,

(Terence MAN)
for Assistant Director (Drug)