5种戈谢病治疗药物的安全性风险信号研究Δ

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摘　要　目的　评价5种戈谢病治疗药物（伊米苷酶、维拉苷酶α、他立苷酶α、依利格鲁司特、麦格司他）的不良事件（ADE）特征。**方法**　收集2004年1月—2025年9月美国FDA不良事件报告系统（FAERS）数据库上报的上述5种药物的ADE报告。采用报告比值比法、比例报告比值法进行数据挖掘。利用《国际医学用语词典》（26.0版）药物ADE术语集中的系统器官分类（SOC）和首选术语（PT）进行分类统计。**结果**　分别检索到上述5种药物的ADE报告4 329、1 740、500、1 329、998份，生成4 612、2 605、776、1 157、2 185个ADE信号；涉及26个SOC，5种药物分别累及25、26、16、19、19个SOC，以各类损伤、中毒及操作并发症，感染及侵染类疾病为主；5种药物排名第1的PT依次为骨梗死、锌缺乏、未执行药物监测步骤、鱼鳞病、神经传导检查异常。在新的可疑的PT方面，伊米苷酶、维拉苷酶α为体重增加、自发流产，他立苷酶α、依利格鲁司特均为体重增加，麦格司他为痴呆、情感淡漠等神经精神类信号。在严重ADE方面，伊米苷酶、他立苷酶α主要为其他严重医学事件，住院时间延长以维拉苷酶α、依利格鲁司特为主，麦格司他为死亡。**结论**　临床用药时，除关注常见ADE外，对伊米苷酶需警惕骨梗死、体重增加与自发流产等ADE，强化骨骼、代谢及妊娠相关监测；对维拉苷酶α需警惕锌缺乏、骨梗死、体重增加与自发流产，重点监测营养状态及妊娠风险；对他立苷酶α需警惕未执行药物监测、体重增加，规范用药流程并监测体重变化；对依利格鲁司特需警惕鱼鳞病、体重增加，重点关注皮肤状况；对麦格司他需警惕骨梗死、神经传导检查异常、痴呆，加强神经系统、感染及重症预后监测——以保障患者用药的安全性。

关键词　戈谢病；伊米苷酶；维拉苷酶α；他立苷酶α；依利格鲁司特；麦格司他；不良事件；药物警戒

Research on safety risk signals of five drugs for Gaucher disease

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ABSTRACT　OBJECTIVE To evaluate the characteristics of adverse drug event (ADE) associated with five therapeutic agents for Gaucher disease (imiglucerase, velaglucerase alfa, taliglucerase alfa, eliglustat, and miglustat). **METHODS** ADE reports related to the above five drugs were retrieved from the FDA Adverse Event Reporting System (FAERS) database from January 2004 to September 2025. The reporting odds ratio (ROR) and proportional reporting ratio (PRR) methods were applied for data mining. system organ class (SOC) and preferred term (PT) from the *Medical Dictionary for Regulatory Activities* (MedDRA, version 26.0) drug ADE terminology were used for classification and statistical analysis of ADE. **RESULTS** A total of 4 329, 1 740, 500, 1 329 and 998 ADE reports were retrieved for the aforementioned five drugs, respectively, generating 4 612, 2 605, 776, 1 157 and 2 185 ADE signals. These signals involved 26 SOC, affecting 25, 26, 16, 19, and 19 SOC, respectively. The predominant SOCs were various injuries, poisoning and procedural complications, as well as infections and infestations. The top one PTs of each drug were as follows: bone infarction, zinc deficiency, failure to perform drug monitoring steps, ichthyosis, abnormal nerve conduction examination. New suspected PTs included weight gain and spontaneous abortion for imiglucerase and velaglucerase alfa, weight gain for taliglucerase alfa and eliglustat, and neuropsychiatric manifestations such as dementia and apathy for miglustat. In terms of severe ADEs, other serious medical events were predominant for imiglucerase and taliglucerase alfa; prolonged hospital stays were the main severe ADE for velaglucerase alfa and eliglustat; and death was the leading severe ADE for miglustat. **CONCLUSIONS** In clinical medication, apart from common ADE, for imiglucerase, clinicians should be alert to bone infarction, weight gain and spontaneous abortion, and strengthen monitoring related to bones, metabolism and pregnancy. For velaglucerase alfa, attention should be paid to zinc deficiency, bone infarction, weight gain and spontaneous abortion, with focused monitoring of nutritional status and pregnancy risks. For taliglucerase alfa, failure to perform drug monitoring steps and weight gain require vigilance, and medication procedures should be standardized along with regular weight monitoring. For eliglustat, clinicians need to watch out for ichthyosis and

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