Lysosomal Storage Diseases & Vitamin K substances

The Pharmacy Practice Forum held on 21 July 2010 was almost cancelled due to an approaching typhoon. In wind and rain I still went to KWH Pharmacy, expecting to see less attendees. On the contrary, I was warmed as the conference room was crowded with over 20 pharmacists and interns from different hospitals.

Gaucher Disease, Pompe Disease, Mucopolysaccharidoses (MPS) disorders, what are they? Miss Suzanne Leung briefly described each of the above Lysosomal Storage Diseases (LSD). LSDs are rare metabolic diseases in which the patients were born with deficiency of a particular enzyme or protein. Accumulation of unwanted substances in the body causes various clinical manifestations and eventually death. To date, there is no known cure for LSDs. Supportive care is usually given while patients wait for bone marrow transplantation. Recently, enzyme replacement therapy (ERT) was introduced into HA Drug Formulary. It does not treat the underlying disease, but it replaces the lacking enzyme.

Suzanne illustrated a case of Galsulfase use in a MPS VI patient. The major concerns surrounding ERTs are extremely expensive treatment cost, marginal benefits demonstrated by limited number of small trials and the lack of long term data on survival benefit and drug safety. The ultimate question being is it cost-effective to provide life-long enzyme replacement? However, is it unethical if we do not provide an available treatment option which may control symptoms or improve patients' quality of life? This is an open-ended question.

In the second part of the forum, Mr. Ken Lee reminded pharmacists to stop and think whenever they come across any prescription ordering "vitamin K". Vitamin K_1 (phytomenadione) effectively reverses hypoprothrombinaemia and haemorrhage caused by oral anticoagulant therapy or poisoning with warfarin (secondary vitamin K deficiency); while less hydrophobic synthetic vitamin K_4 (acetomenaphthone) is a replacement for primary vitamin K deficiency as it can be directly absorbed from the intestine in the absence of bile salts.

With the help of coagulation cascade, vitamin K cycle, chemical structures and the evidence from literatures, Ken led the floor to discuss an unsettle puzzle: Why vitamin K_4 is not effective to oppose oral anticoagulant effect? If the long phytyl side chain of vitamin K_1 is essential, can vitamin K_4 be also effective after body's enzymatic alkylation? However, in bleeding emergency, we really need a quick and reliable treatment. I was enlightened by opinions from pharmacists and interns. Some of us suggested renaming vitamin K to vitamin K_4 in computerized physician order entry system of HA to increase the awareness of different vitamin K compounds.

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