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ENZYME REPLACEMENT THERAPY WITH VELAGLUCERASE ALFA IMPROVES KEY CLINICAL PARAMETERS IN A PEDIATRIC SUBGROUP WITH TYPE 1 GAUCHER DISEASE

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Background and aims: A randomized, double-blind, Phase III trial of velaglucerase alfa in type 1 Gaucher disease provides an opportunity to examine outcomes in pediatric patients.

Methods: Of 25 patients in the trial, 7 pediatric patients (28%) were enrolled and randomized to receive velaglucerase alfa at 60 U/kg (n=4) or 45 U/kg (n=3) as a 1-hour infusion, every other week for 12 months.

Results: At 12 months, the mean hemoglobin concentration increased in both groups (60 U/kg: 16.3% increase, 1.74 g/dL, 95% CI: 0.72, 2.78; 45 U/kg: 24.7% increase, 2.77 g/dL, 95% CI: -0.99, 6.53). Mean platelet counts also increased in both groups (60 U/kg: 26.4% increase, $+49.9 \times 10^9/L$, 95% CI: -32.1, +131.9; 45 U/kg: 38.9% increase, $+60.3 \times 10^9/L$, 95% CI: -103.1, +223.7). Mean spleen volume, normalized by body weight, decreased in both groups (60 U/kg: 64.6% decrease, -2.1 cm³, 95% CI: -5.3, +1.1; 45 U/kg: 47.6% decrease, -0.7 cm³, 95% CI: -2.6, +1.2) as did mean liver volume (60 U/kg: 18.3%, -0.7 cm³, 95% CI: -1.4, 0.0; 45 U/kg: 10.6% decrease, -0.3 cm³, 95% CI: -1.7, +1.1). No children developed antibodies to velaglucerase alfa, experienced a severe or a serious treatment-emergent adverse event, or discontinued due to an adverse event.

Conclusions: In these pediatric patients with type 1 Gaucher disease, velaglucerase alfa was generally well tolerated at doses of 60 U/kg and 45 U/kg, and provided clinically meaningful improvements in disease parameters.

Velaglucerase alfa is approved in the U.S.

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CLINICAL STUDY OF ACCIDENTAL ACUTE POISONING IN CHILDREN UNDER 3 YEARS OF AGE

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Background: Acute poisoning in children, especially in infants and toddlers is caused by accident. Young children are especially vulnerable categories as they are exposed to many potential toxins in age when they are curious toward environment and have need for great physical activity.

Parents have the greatest responsibility for the prevention of acute poisoning in children. They should pay attention to issues that are known as "poisoning prevention 4R": "to recognize," "to remove," "to be ready," to "respond."

Methods: It is a prospective study for 3 years period (2006-2009) that includes every case of acute poisoning at our hospital.

Results: There were 98 children, 68 of them were younger than 3 years of age - even 70.88%. At this age, 94% had access to toxic substances at their home. In 38% of cases, parents had currently seen the act of poisoning but it was late to stop as they were busy with other actions than taking care of their children.

The most frequent cause of acute poisoning (64%) are drugs - mostly sedatives and antihypertensive drugs. In few cases it was due to replacement or overdose of drug given by parents.

In 15% acute poisoning was caused by ingestion of industrial products for household and 11% due to pesticides.

Conclusion: Problem can be prevented if more attention is paid to educate parents how to recognize toxic substance, safely store it at home and be ready to give first aid to their child if poisoning happens.