

The efficacy and safety of octreotide treatment for diazoxide-unresponsive congenital hyperinsulinism in China

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OBJECTIVES

Octreotide is an off-label medicine for CHI but widely used nowadays. However, the efficacy and adverse effects have been reported varied in centers. To evaluate the efficacy and safety of the subcutaneous octreotide injection for diazoxide-unresponsive CHI in China.

METHODS

Diazoxide-unresponsive CHI children treated with subcutaneous octreotide injection at an adjusted dosage of up to 50µg/kg/day were involved in the study. Octreotide is ineffective when blood glucose (BG) <2.8mmol/L or is completely effective if BG > 3.3mmol/L over 48 hours by every 2 hours check after wean-off iv dextrose. BG between the above means partial effectiveness.

RESULTS

Twenty-five Chinese (15 males) children were enrolled in the study. The mean age of last visit was (1.6±0.9) (maximum 3.3) years. The octreotide was completely effective, partly effective in 12, 9 patients respectively and ineffective in 4 (16%). The effectiveness (included complete and partial effectiveness) of octreotide was not different between gene-positive and -negative group. Transient elevation of liver enzymes occurred in 20% patients, asymptomatic gallbladder pathology occurred in 1 patient. The growth charts of this cohort patients were in normal range (mean height SDS was 0.3±1.5 at the last follow-up).

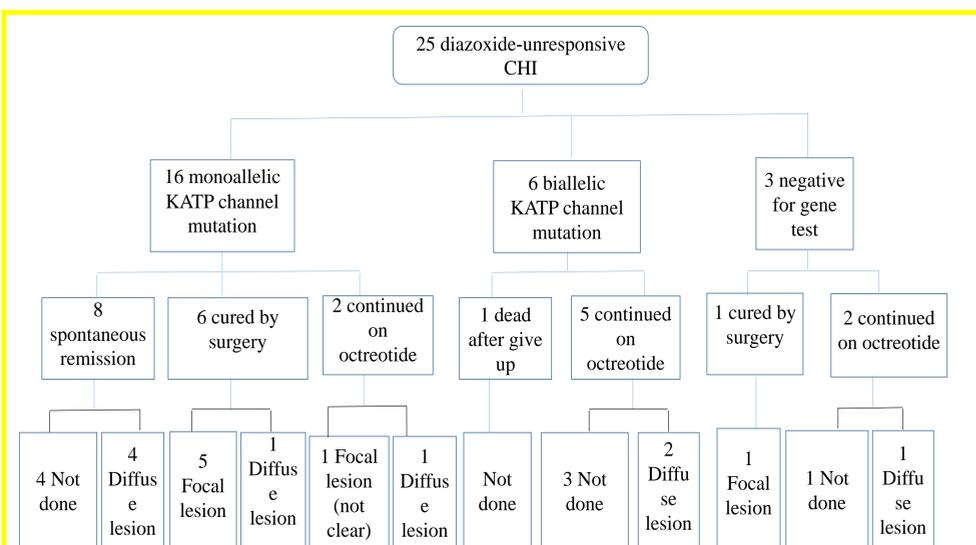


Figure 1 The gene mutation analysis, histopathology, and prognosis of 25 diazoxide-unresponsive CHI treated with subcutaneous octreotide injection
“Not done” means 18F-DOPA PET scans were not performed in these patients

CONCLUSIONS

The octreotide was well tolerated, effective therapy for diazoxide unresponsive CHI cases. It could be a choice for diazoxide-unresponsive patients.

References

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