Switching Patients with Congenital Hyperinsulinism from Standard Octreotide to Long-Acting Release Octreotide Preserves Blood Glucose Control and Improves Quality of Life of Their Caregivers

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BACKGROUND: Congenital hyperinsulinism (CHI; MIM #256450) is the most common cause of persistent hypoglycemia in children, due to unregulated secretion of insulin from pancreatic ß-cells. Recessive inactivating mutations in KATP channel subunits, encoded by ABCC8 and KCNJ11 genes, are the most common causes of CHI. In diazoxide-unresponsive patients, daily injectable octreotide therapy, combined with diet may be effective, permitting to avoid a near-total pancreatectomy.
Treatment with long-acting release (LAR) octreotide has been reported to be successful in children, but needs more research to prove efficacy in younger group of patients.

OBJECTIVE: Aim of the study was to evaluate blood glucose control and quality of life (QoL) in two young patients affected CHI who were switched from multiple daily subcutaneous (s.c.) octreotide injections to a single monthly intramuscolar (i.m.) injection of LAR octreotide.

METHOD: Standard octreotide injections were stopped after the third injection of LAR octreotide. Blood glucose levels were monitored with continuous glucose monitoring (CGM) before and after therapy switching. Auxological and biochemical (glycosylated haemoglobin, full blood count, liver function test, IGF-1) parameters were monitored at each admission. Parents self-reported Paediatric Quality of life questionnarie (PedsQL) was completed 6 months before and after therapy switching.

RESULTS: CGM revealed no significant differences in the blood glucose levels before and after therapy switching. Both the patients presented at least one hypoglycemia < 55 mg/dL. Glycosylated haemoglobin (HbA1c) levels were in the normal range before and after the switching (*table 1*). We didn't report side effects. Clinical and biochemical parameters remained in the normal range during the study. PedsQL questionnarie, completed by patient's mother and father, revealed a clear improvement in psychosocial health and physical health summary score report with agreement between the parents (*table 2*).

		s.c. Octreotide	LAR Octreotide	p-value
Patient 1	Mean Capillary Glycemia (mmol/l)	4,88 ± 0,84	4,88 ± 0,72	0,97
	Mean Glycemia (CGM) (mmol/l)	4,97 ± 1,04	5,12 ± 0,91	1,31
	Hypoglycemia < 3 mmol/l	2	2	
	Hypoglycemia < 2,5 mmol/l	0	0	
	HbA1c %	5,1	5,2	
Patient 2	Mean Capillary Glycemia (mmol/l)	4,95 ± 1,69	5,17 ± 1,25	0,12
	Mean Glycemia (CGM) (mmol/l)	5,72 ± 1,63	5,59 ± 1,06	**0,01**
	Hypoglycemia < 3 mmol/l	3	1	
	Hypoglycemia < 2,5 mmol/l	2	1	
	HbA1c %	5,3	5,4	

	s.c. Octreotide		LAR Octreotide	
Patient 1	**Father**	**Mother**	**Father**	**Mother**
Psychosocial health summary score	73,07	34,61	73,07	65,38
Physical health summary score	34,37	59,37	81,25	71,87
Total	**58,33**	**44,04**	**76,19**	**67,85**
Patient 2	**Father**	**Mother**	**Father**	**Mother**
Psychosocial health summary score	72,25	73,07	82,69	84,61
Psychosocial health summary score	81,84	88,15	84,37	81,25
Total	**78,38**	**79,44**	**83,33**	**83,33**

Table 2. The PedsQL Parent Report for Toddlers module consists of 20 items and gives score for physical, emotional, social and school functioning. It also gives a total scale score, physical health summary score and psychosocial health summary score. After switching from s.c. Octreotide to LAR Octreotide both the parents reported a significant improvement in qol.

CONCLUSIONS: We report two patients affected by CHI who were switched to long acting release (LAR) octreotide therapy with a significant improvement of quality of life, stable blood glucose control and no side effects.

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Fat metabolism and Obesity

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