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PROGRESS REPORT

Project / Program Title		Investigation of the Renal Bone Axis in Patients with Thalassaemia on Deferasirox
Name		Dr Phillip Wong
Award Received		2017 The Robert-Maple Brown Research Establishment Award in Haematology
Report Date		20 June 2017
Chief Investigator / Supervisor		Dr Phillip Wong/ Associate Professor Frances Milat
Administering Institution		Hudson Institute of Medical Research
Funding Period	Start Date:	1 January 2017
	Finish Date:	1 January 2018

PROJECT SUMMARY

Patients with the genetic blood disorder, thalassaemia major, are at increased risk of excessive loss of calcium through the urine. This project set out to investigate whether urine calcium loss can be improved by one of 2 treatments. Firstly, through the use of a diuretic, and secondly by switching the iron binding drug, deferasirox, to another iron binding drug, deferoxamine.

This is important as excessive loss of calcium in the urine can lead to kidney stones and also worsening bone loss.

PROJECT AIMS / OBJECTIVES

The plan is to perform an open-labelled pilot study on adults with laboratory confirmed hypercalciuria and transfusion-dependent haemoglobinopathies, examining the effect of hydrochlorothiazide 25mg daily on renal and bone endpoints.

SIGNIFICANCE AND OUTCOMES

We have enrolled 96 adult subjects with transfusion-dependent thalassaemia through the Medical Therapy Unit at Monash Health.

Hypercalciuria was present in all patients. 68 patients declined to receive hydrochlorothiazide or switch to deferoxamine and these patients comprised the control group. There were 17 subjects who received hydrochlorothiazide whilst on deferasirox and 11 subjects who switched from deferasirox to desferal.

Our study, so far has demonstrated that hypercalciuria is partly reversible upon cessation of deferasirox and switching to deferoxamine. Furthermore, in patients who continue with deferasirox, the addition of a thiazide diuretic also results in improvements to hypercalciuira.

At this stage, we do not have adequate patients who have undergone DXA to analyse whether improvements to BMD has occurred. Hopefully, within the next 12 months we will have at least 96 patients who have had a DXA study at the 12 month mark. Although, we predict that at least 2 to 3 years of longitudinal DXA data is required to demonstrate both a clinically and statistically significant change in BMD.

PUBLICATIONS / PRESENTATIONS

Invited Symposium at the Australian New Zealand Bone Mineral Society , 2016 - "Thalassaemia Bone Disease"