

## **MEDIA RELEASE**

**5 November, 2009**

### **Monash Children's pioneers world-first life saving treatment for babies**

A team of doctors at Southern Health's Monash Children's have led the way in developing a world first drug designed to cure babies diagnosed with a rare and fatal inborn metabolic disorder.

An infant born in Victoria and diagnosed with molybdenum cofactor deficiency type A was last year granted special permission to be treated with the experimental drug CPMP precursor Z.

Baby Z was diagnosed with a rare and fatal disease that doctors now believe can be cured with this world first compound.

This inborn metabolic disorder usually results in neuro-degeneration, progressive brain damage and death in early infancy in most cases.

Up until now, no treatment has been available.

This discovery has led to a specialist team from Southern Health preparing to embark on a world-wide journey to ensure children world-wide have access to this special first time cure.

Monash Children's Neonatal Paediatrician Dr Alex Veldman and a specialist team of doctors and scientists will this weekend fly to the United States for discussions with the US FDA to develop this treatment for world-wide usage. They will also visit Europe to discuss a trial of the treatment there.

"This is a first life saving treatment for this fatal disease with global implications," Dr Veldman said.

"The team at Southern Health managed to get this therapy from bench to bedside in about two weeks, a process which normally takes several years."

Baby Z was born in May 2008. At 60 hours the baby started having seizures and was transferred to Monash Children's for specialist care.

Metabolic screening revealed low serum and urine Uric Acid and high urine S-Sulphocysteine levels in baby Z. This finding was consistent with Molybdenum co-factor deficiency.



## Timeline for saving Baby Z:

May 26th: The paper published by Prof. G. Schwarz reported possible treatment of MoCD with cPMP in an animal model is discovered by biochemist Dr Rob Gianello.

May 27th: After a first reading of some of the relevant literature, a contact between Monash Children's and Prof. G. Schwarz in Cologne is made.

May 28th to May 30th: Further detailed reading of the literature and first draft of a treatment plan for compassionate use of cPMP as rescue treatment in a patient with MoCD is written by A. Veldman, G. Schwarz and Dr. R. Gianello.

May 28th: cPMP, donated by Prof. G. Schwarz, arrives in Australia. Dr. R. Gianello and his team starts to work on Purity Analysis and Endotoxin Assays. The TGA is informed.

May 30th: An preliminary application to the for an urgent Meeting of the Southern Health BioEthics Panel in the following week is made.

May 31st and June 1st: The Treatment for compassionate use of cPMP as Rescue treatment in a patient with MoCD is finalized.

June 4<sup>th</sup>: Approval of the Treatment Plan by the Southern Health BioEthics Panel. Southern Health legal team prepare a 150 page submission seeking approval from the Family Court for experimental rescue therapy.

June 6th: The case of Baby Z. is heard by the Family Court of Australia. Approval of the Treatment Plan by the Family Court of Australia. Preparation of the cPMP infusion is organized by the Southern Health Pharmacy team and Dr. Robert Gianello from Monash University.

Baby Z is the first patient with MoCD to receive experimental replacement therapy cPMP under maximal intensive care monitoring.

Within a few days after starting the patient on cPMP, the baby's level of alertness improved significantly and the twitching and startle reactions decreased.

The head circumference, which remained static during the first 6 weeks of life, started to increase after cPMP substitution was commenced.

An EEG performed 3 weeks after start of cPMP substitution showed a 90% reduction of seizure activity if compared to the EEG prior to treatment.

Baby Z was discharged home at about 4 weeks into its treatment course.

Latest developments:

Extensive toxicology studies of cPMP in animal models have commenced, work on alternative administration routes are underway.

Initial meetings with the US FDA to discuss the process of registration of cPMP are scheduled for



Nov. 10th, 2009.

Teleconference with the European EMEA to discuss the process of registration of cPMP is also scheduled for Nov. 10th, 2009.

A protocol for a international study enrolling 10 patients was approved has been approved by Southern Health.

A four week-old German boy, Baby P, with MoCD has now commenced treatment with cPMP.

Like Baby Z, Baby P has improved rapidly and is not experiencing any side effects of the medication.

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