CNS/Orphan/Pediatrics

COMMERCIALISATION OPPORTUNITY

Treatment for White Matter Impairment in Newborns

Summary

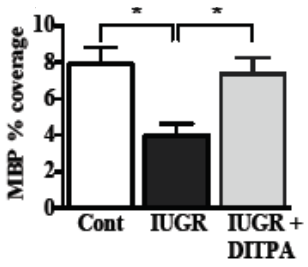
Failure of the fetus to grow during pregnancy is usually associated with poor functioning of the placenta, however no treatment can reverse this. One of the most serious outcomes of poor fetal growth before birth – or Intrauterine Growth Restriction (IUGR) - is the failure of the brain cells that produce myelin (i.e., the white matter) in the brain to develop. This failure often leads to significant brain damage at birth and the life-long legacy of cerebral palsy. Thus IUGR is a significant medical and societal burden with up to 9% of all pregnancies in developed countries complicated by IUGR. In Australia this equates to ~27,000 babies per year.

Brain development in IUGR infants is often suboptimal because the cells that produce myelin – the oligodendrocytes – are retarded in their maturation. The myelination of the axons of nerve cells is essential for efficient transmission of electrical impulses, which in turn is the basis of all motor, sensory, and cognitive functions. Thus, impaired myelination in IUGR infants results in poor neurological outcomes for the child, and as mentioned above, can be severe enough to cause cerebral palsy.

Based on our discovery that thyroid hormone signaling - a key driver of oligodendrocyte maturation - is impaired in the white matter of IUGR neonates, we have developed a treatment to correct this, and to provide for the recovery of myelination in the brains of babies affected by IUGR. We have found that DITPA (diiodothyropropanoic acid), a synthetic thyroid hormone analogue that readily enters the brain, can overcome the decreased thyroid hormone signaling in brains of IUGR rat pups, and can promote maturation of the immature oligodendrocytes, and thus restores myelination.

Key Data

Myelination (MBP staining) in the control, IUGR and IUGR+DITPA rat brain at 7 days age, following daily postnatal i.p. DITPA treatment on days 1-6. n=3-5, *p<0.05.



IP position

A method of treatment patent has been filed with a priority date in May 2016.

Advantages and research strengths

- Accelerated path to market: pre-clinical regulatory studies complete & treatment approved for other pediatric central nervous system indication
- Strong patent position
- Orphan drug indication
- Known Mechanism of Action
- Will be the Best in Class treatment
- Neonatal ICU trial expertise with DITPA treatment

Market

There is considerable unmet need in the field of neonatal white matter brain injury and IUGR - currently, there are no approved treatments to cure, reverse, or improve outcomes for babies affected by IUGR.

DITPA treatment for IUGR will have a substantially accelerated path to market given it is used for a rare childhood genetic metabolic condition and has an excellent safety profile. Specialised and commercially successful products in the growing neonatal/paediatric area include Inomax (nitric oxide) and Curosurf (surfactant).

An additional incentive for partnering is potential eligibility for a highly valuable Rare Pediatric Disease Priority Voucher from the FDA.

Opportunity

Hudson welcomes opportunities for co-investment or collaboration to further develop this project. We have assembled an experienced clinical, scientific and commercial team ideal to steer the clinical development of DITPA treatment for IUGR.

For further information

Rob Merriel, BD Executive
rob.merriel@hudson.org.au
+61 418 186 265

Nadine Brew, BD Coordinator
nadine.brew@hudson.org.au
+61 423 351 757

A/Prof Flora Wong
Monash Health Consultant Neonatologist
Laboratory Head, Neonatal Brain Protection Laboratory
+61 3 9594 5482