Journal of Neonatal Biology
ISSN: 2167-0897
OPEN ACCESS Freely available online

Editorial

Editorial on Neonatal Gene Therapy

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INTRODUCTION

Application of gene therapy has several advantages if performed in new-borns to treat genetic and infectious diseases. Because of the minimal adverse effect of the underlying disease on cells of the new-born, the relatively small size of infants and the large amount of future growth, gene therapy may be more successful in new-borns than in older children or adults. The presence of umbilical cord blood of new-borns provides unique and susceptible target for the genetic modification of hematopoietic stem cells. At first trial of gene therapy of new-borns, we insert a normal adenosine deaminase gene three neonates umbilical cord blood cells with a congenital immune deficiency.

For researching human models, transgene delivery and expression during the foetal or neonatal phase is a valuable tool. It can also be used therapeutically alongside adult gene therapyto prevent or treat monogenetic disorders in the future. Long- term phenotypic correction after fetal or neonatal applicationhas been identified in these promising studies, which have benefited from recent advances in vector technology and optimization of administration routes to suitable disease models

Many genetic defects have such severe effects that a viable embryo or fetus is unlikely to develop. However, a small numberof mutations have such a slight effect during pregnancy, when the fetus is still on the maternal life support system that the catastrophic effects do not occur until after birth. Intracranial hemorrhage or bleeding underneath the scalp, at the site ofvenipuncture, at the umbilical stump, or after circumcision is normal in hemophilic neonates. While uncontrolled bleeding into the joints of newborns is rare, uncontrolled hemarthrosis causes significant and permanent local harm as the child starts to crawl. As a result, a compelling argument has been made for early prophylactic replacement of clotting factors in those that are most seriously affected.

Maternal influence

Since neonatal antibody development is delayed, transplacental transport of maternal immunoglobulins to the developing fetus is crucial in protecting the newborn from infection. Fc receptors, which are thought to help transport, are mainly expressed on fetal endothelial cells during the third trimester.

The efficacy of early gene transfer has been demonstrated in animal models in recent studies. However, in comparison to adults, the processes underlying these successes, as well as those impeding even better results, are also poorly understood. Many factors can influence the best age for intervention, including the nature and seriousness of the disorder, the ease with which it can be identified, the safety and effectiveness of gene therapy, the intervention's practicality, and, finally, the legal, social, and financial consequences of such an approach.

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Received: March 05, 2021, **Accepted:** March 14, 2021, **Published:** March 22, 2021

Citation: Raajitha B (2021) Editorial on Neonatal Gene Therapy. J Neonatal Biol. Vol: 10, Iss: 3