Original Article

Usage of U7 small nuclear ribonucleic acid in gene therapy of hemoglobin D Punjab disorder: Rationale?

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BACKGROUND: Hemoglobin (Hb) D Punjab disorder is a congenital hemoglobinopathy described in India. It is a disorder due to defect in beta-globin gene.
MATERIALS AND METHODS: Here, the author assesses the possibility of U7.623 gene therapy for Hb D Punjab disorder. A standard bioinformatic analysis to study the effect of co-expression between nucleic acid sequence for human Hb D Punjab beta-globin chain and U7.623 was performed.
RESULT: It can be seen that fully recovery of Hb function and biological process can be derived via gene ontology study.
CONCLUSION: Here, there is a rationale to use U7 small nuclear ribonucleic acid as a possible tool for gene therapy in Hb D Punjab disorder.

Key words: Hemoglobin D Punjab, U7 small nuclear ribonucleic acid, gene therapy

Introduction

Of several hemoglobinopathies identified in India, hemoglobin (Hb) D Punjab disorder is a disorder firstly described in India.[1,2] This is a hematological disorder due to defect in beta-globin gene (beta 121 Glu → Gln). Since it is a genetic disorder, therefore, there is no specific treatment at the root cause in the previous day. However, due to the recent medical technology in the present, the treatment based on gene therapy becomes feasible.

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Of several gene therapy techniques, the repair of defective splicing by small nuclear ribonucleic acids (snRNAs) is a new modality for treatment of hemoglobinopathy. The principle of using snRNA is aiming at replacement of the natural antisense sequence with that targeted to the desired RNA with the expectation for permanent expression of RNA antisense to the specific defective thalassemic splice sites in beta-globin RNA.[3] Here, the author assesses the possibility of U7.623 gene therapy for Hb D Punjab disorder. A standard bioinformatic analysis to study the effect of co-expression between nucleic acid sequence for human Hb D Punjab beta-globin chain and U7.623 was performed.

Materials and Methods

This work is performed as a bioinformatics study. The protocol in this work is the same as standard referenced work published by Wiwanitkit.[4] Briefly, the database PubMed was used for data mining of the nucleic acid sequence for human beta-globin chain following by in silico assignment of mutation corresponding to Hb D Punjab. The in silico combination between nucleic acid sequence for human Hb D Punjab beta-globin chain and U7.623 was done and tested for the expression by ontology prediction method.[5]

Results

According to this work, the co-expression between nucleic acid sequence for human Hb D Punjab beta-globin chain and U7.623 was assessed and the

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complete resolution of molecular function (oxygen transporter activity) and biological process (oxygen transport) could be derived.

Discussion

The problem of hemoglobinopathy is a big problem in hematology. Several kinds of hemoglobinopathies are reported around the world. In this work, the author focused the interest on Hb D Punjab, a beta hemoglobinopathy. Similar to other kinds of hemoglobinopathy, the poor oxygen transport in the defective red blood cell in Hb D Punjab can be seen. Due to the fact that this is a congenital disorder with underlying gene defect, the concept of gene therapy can be the new effective therapeutic tool.

Here, the author tested for the U7 snRNA regimen. This specific regimen is designed as antisense for the beta-globin mutation in intron 2. This regimen is effective for gene repair in beta thalassemia.[6] This regimen was also tested for the feasibility in other beta hemoglobinopathies and favorable outcomes were reported.[4,7]

Here, the author used the already accepted published technique in the previous works for extrapolation in the scenario of Hb D Punjab. Of interest, it can be seen that fully recovery of Hb function and biological process can be derived via gene ontology study. Here, there is a rationale to use U7 snRNA as a possible tool for gene therapy in Hb D Punjab disorder.

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