



LUSPATERCEPT - A TREATMENT FOR THALASSEMIA?

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Dear Editor,

Among a plethora of widespread diseases in Pakistan, β -thalassemia is the commonest genetic disorder. According to report published by Bloomberg stated that, about 6,000 children in Pakistan are diagnosed with this genetic disorder every year, with 6-9% carrier rate.¹ Consanguinity is considered to be the primary cause for higher prevalence in Pakistan. We have iron chelation and regular blood transfusions as the primary treatment alternatives for β -thalassemia patients, However, bone marrow transplantation is considered to be an only and effective cure.²

For red blood cells maturation, Sotatercept and Luspatercept are certain Activin receptor fusion compounds which vanquishes the TGF- β pathway for negative regulation. The conjoining of Activin receptor ligands suppresses the abnormal Smad 2/3 signaling and avert cell-cycle arrest and apoptosis in erythroblasts, hence, encouraging late-stage differentiation. Scientists have reported that, Luspatercept bestows a new horizon to counter this disease which involves the differentiation of RBC's, thus, multiplying the number of crude erythroid precursors in the blood. It is worth mentioning that, in any way it cannot be considered a substitute of RBC's while treating acute anemic patients.³

The use of Luspatercept culminated the increased hemoglobin levels in Phase one study which involved normal post-menopausal-women and decreased the burden of transfusion by 22% in 13 weeks of Phase two study. United States Food and Drug Administration (FDA) has approved this drug for use among

adult patients who have β -thalassemia and regularly needs RBC's transfusions in 2019. The efficacy of Luspatercept was determined in a trial named 'BELIEVE' which was conducted at 66 different multicenter covering around 16 countries. In these trials 224 people were administered with Luspatercept and 112 participants were given placebo. It was disclosed that the Luspatercept group had a higher significance of reduced transfusion burden. While describing the major effects of this drug, the scientists recorded arthralgia, headache, hyperuricemia and hypertension, however, it was also reported that patients who underwent splenectomy noticed 3.6% thrombo-embolic events.⁴

Specifically mentioning the situation of Pakistan, the over-burden of massive transfusions negatively impacts individuals and drastically utilize the limited health care facilities resources. We are quite hopeful that with the use of this drug, we can counter the β -thalassemia cases in Pakistan.⁵ There is a positive hope that it could prove as stakeholder in making patients more independent in their lives. Big-wigs Pharmaceuticals and government of Pakistan must work in a joint-venture to open its doors for clinical trials in Pakistan as we did in COVID-19 vaccines, so that we can evaluate the efficacy and feasibility of Luspatercept for our people.⁶

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CONFLICT OF INTEREST

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