UTSouthwestern
Medical Center

TITLE: Novel Compound Supports Hematopoietic Stem Cells and Red Blood Cells
INVENTORS: Chengcheng Zhang
TECHNOLOGY: Biologicals
UTSD: 2766

SUMMARY: Hematopoietic stem cell (HSCs) transplantation is used to treat patients with leukemia, lymphoma, some solid cancers, autoimmune diseases, and genetic defects such as sickle cell anemia and thalassemia. An ability to expand HSCs in culture would further clinical applications in many aspects. For example, in both autologous transplant and allogeneic transplant, high doses of HSCs are needed to achieve rapid and sustained engraftment that are critical for the patients' survival and recovery; this is especially true when cord blood HSCs are used. Furthermore, for gene therapy, ex vivo expansion of HSCs could be used in selective expansion of transduced cells in which the desired genes are introduced at the appropriate genomic location.

The water-soluble toad skin extracts have been reported to be safe to treat patients of aplastic anemia and cancers. They have been shown to stimulate the regeneration of multiple hematopoietic lineages in human and mice. We have identified an active component in the extract that can promote regeneration of hematopoietic stem cells (HSCs) and red blood cell proliferation. This discovery has been validated by a number of assays including culture of HSCs followed by bone marrow transplantation analysis and hematopoietic recovery assay in a mouse model of bone marrow failure. This compound and its derivatives, currently under synthesis, should have direct clinical applications on patients with bone marrow failure, anemia of various causes, and chemotherapy/ radiotherapy/HSC transplantation recipients.

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Please reference UT Southwestern Case Number: **2766**