Pursuing ‘organic gene therapy’ for sickle cell anemia and beta thalassemia

Scientists in Australia have solved a 50-year-old mystery that could lead to the development of new gene therapies for blood disorders such as sickle cell anemia and ?-thalassemia. The team, led by University of New South Wales (UNSW) professor Merlin Crossley, Ph.D., has identified the gene-control mechanism that allows some individuals with these blood disorders to keep producing a fetal form of human hemoglobin, which naturally compensates for the lack of adult hemoglobin and so reduces disease severity. The researchers also used CRISPR/Cas9 gene editing to introduce these naturally beneficial mutations into cultured blood cells and boost production of fetal hemoglobin directly.

“Our new approach can be seen as a forerunner to ‘organic gene therapy’ for a range of common inherited blood disorders including ?-thalassemia and sickle cell anemia,” says Dr. Crossley, who is UNSW deputy vice-chancellor academic. “It is organic because no new DNA is introduced into the cells; rather we engineer in naturally occurring, benign mutations that are known to be beneficial to people with these conditions. It should prove to be a safe and effective therapy, although more research would be needed to scale the processes up into effective treatments.”

The team reports its findings in a paper published [April 2] in Nature Genetics, which is entitled “Natural Regulatory Mutations Elevate the Fetal Globin Gene via Disruption of BCL11A and ZBTB7A Binding.”

Read full, original post: CRISPR Use Could Lead to “Organic” Gene Therapies for Blood Disorders