HEMOGLOBINOPATHIES AND CRISPR-Cas9: FIRST RESULT IN ADULTS

Hemoglobinopathies lend themselves to genetic manipulations because hematopoietic cells can be withdrawn, treated and reintroduced. And there is also a shortcut. Instead of manipulating the specific mutation of the patient, one could reactivate the fetal hemoglobin. There are cases in which the pathology is attenuated, even completely, because of the casual presence of a mutation that causes the persistence of fetal hemoglobin (https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6292363/). This 2018 publication was about the strategy of reactivating fetal hemoglobin using CRISPR-Cas9 (https://www.sciencedirect.com/science/article/pii/S0168952518301562?via%3Dihub). Now there are results obtained by CRISPR Therapeutics (Zurich https://investors.vrtx.com/news-releases/news-release-details/crispr-therapeutics-and-vertex-announce-positive-safety-and) and there is a short paragraph in Science titled "CRISPR's first clinical success?" (https://science.sciencemag.org/content/366/6468/930).