Potential Use of MicroRNA Technology in Thalassemia Therapy

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Abstract	Thalassemia encompasses a group of inherited hemoglobin disorders characterized by reduced or absent production of the alpha- or [3-globin chains, leading to anemia and other complications. Current management relies on lifelong blood transfusions and iron chelation, which is burdensome for patients. This review summarizes the emerging therapeutic potential of modulating microRNAs (miRNAs) to treat thalassemia. MiRNAs are small non-coding RNAs that regulate gene expression through sequence-specific binding to messenger RNAs (mRNAs). While they commonly repress gene expression by binding to the 3' untranslated regions (UTRs) of target mRNAs, miRNAs can also interact with 5'UTRs and gene promoters to activate gene expression. Many miRNAs are now recognized as critical regulators of erythropoiesis and are abnormally expressed in [3-thalassemia. Therapeutically restoring levels of deficient miRNAs or inhibiting overexpression through miRNA mimics or inhibitors (antagomir), respectively, has shown preclinical efficacy in ameliorating thalassemic phenotypes. The miR-144/451 cluster is especially compelling for targeted upregulation to reactivate fetal hemoglobin synthesis. Advances in delivery systems are addressing previous challenges in stability and targeting of miRNA-based drugs. While still early, gene therapy studies suggest combinatorial approaches with miRNA modulation may provide synergistic benefits. Several key considerations remain including enhancing delivery, minimizing off-target effects, and demonstrating long-term safety and efficacy. While no miRNA therapies have yet progressed to clinical testing for thalassemia specifically, important lessons are being learned through clinical trials for other diseases and conditions, such as cancer, cardiovascular diseases, and viral. If limitations can be overcome through multi-dis ciplinary collaboration, miRNAs hold great promise to expand and transform treatment options for thalassemia in the future by precisely targeting pathogenic molecular networks. Ongoing innovations, such as advancements in miRNA delivery systems, improved targeting mechanisms, and enhanced understanding of miRNA biology, continue to drive progress in this emerging field towards realizing the clinical potential of miRNA-based medicines for thalassemia patients.
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