Antisense Oligonucleotide QR-313

National Cancer Institute

Source

National Cancer Institute. Antisense Oligonucleotide QR-313. NCI Thesaurus. Code C162452.

A twenty-one nucleotide-containing antisense oligonucleotide (AON) with potential use in the treatment of recessive dystrophic epidermolysis bullosa (RDEB) due to mutations in exon 73 of the COL7A1 gene. Upon topical administration, QR-313 hybridizes to a specific sequence in COL7A1 pre-mRNA, resulting in exclusion of exon 73 from mRNA and translation of a functional type VII collagen protein. This may restore functionality of integument anchoring fibrils, prevent blistering, and improve wound healing in patients with DEB. Type VII collagen is a major component of anchoring fibrils, attachment structures that mediate dermal-epidermal adherence in human skin. DEB is an inherited mechano-bullous disorder caused by mutations in the COL7A1 gene, which lead to perturbations in anchoring fibrils.