

Who are ProQR and what do they do?

ProQR Therapeutics is a Dutch company on a mission to create new medicines for rare diseases with very high need such as EB. To treat these diseases, ProQR use RNA therapies that are designed to target the type of mutation that causes a genetic disease. RNA therapies consist of a short piece of synthetic RNA made in a laboratory. This technology enables them to make temporary corrections to “genes” and therefore restore protein expression that is crucial for our bodies to function.

What is exon skipping?

A mutation that causes a disease can sometimes be repaired using the exon skipping method. An RNA therapy can be designed to cause the cell to ‘skip’ the part of the RNA (the exon) that contains the mutation so that the rest of the RNA can still make a protein. The protein is a little shorter than normal, but can still perform its function. This is how QR-313 is designed to work.

What is QR-313?

QR-313 is an experimental drug designed to exclude exon 73 (exon skipping) from the *COL7A1* mRNA (the ‘blueprint’ for making proteins) with the aim to restore functionality of collagen type VII protein. This in turn may potentially lead to the formation of anchoring fibrils that bind the dermal (inner) and epidermal (outer) skin layers together, improved wound healing and skin strength. QR-313 gel will be applied directly onto wounded skin.



ProQR is recruiting the first cohort in the WINGS study of patients 12 years and older with RDEB due to mutation(s) in a specific part of the *COL7A1* gene called exon 73. Enrolment is closing soon.

Check out the WINGS website <https://rdebtrial.com/> for more information about what participation in the study involves.

If you or your child is interested in WINGS please contact one of the participating US sites below:

California

Stanford University School of Medicine, LPCH
Palo Alto, California, United States, 94305
Contact: Kunju Sridhar 650-721-4902 kunju@stanford.edu
Principal Investigator: Peter Marinkovich, MD

Colorado

Children's Hospital Colorado
Aurora, Colorado, United States, 80045
Contact: Kathleen Peoples 720-777-4708 kathleen.peoples@childrenscolorado.org
Principal Investigator: Anna L Bruckner, MD

United States, Ohio

Cincinnati Children's Hospital
Cincinnati, Ohio, United States, 15005
Contact: Bret Augsburger 513-803-9009 Bret.Augsburger@cchmc.org
Principal Investigator: Anne Lucky, MD

More detailed information WINGS can be found on [Clinicaltrials.gov](https://clinicaltrials.gov) [here](#)