USHER SYNDROME TYPE 2

Development program

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Project Lead
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Forward looking statements

This presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including but not limited to, statements regarding our strategy, future operations, future pre-clinical and clinical trial plans and related timing of trials and results, research and development, future financial position, future revenues, projected costs, prospects, therapeutic potential of our products, plans and objectives of management, are forward-looking statements. The words "aim," "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements represent our management's beliefs and assumptions only as of the date of this presentation. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we assume no obligation to update any forward-looking statements except as required by applicable law. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those that may be described in greater detail in the annual report filed on Form 20-F for the year ended December 31, 2016 that we have filed with the U.S. Securities and Exchange Commission (the "SEC") and any subsequent filings we have made with the SEC. We have included important factors in the cautionary statements included in that annual report, particularly in the Risk Factors section, and subsequent filings with the SEC that we believe could cause actual results or events to differ materially from the forward-looking statements that we make.

ProQR mission and strategy

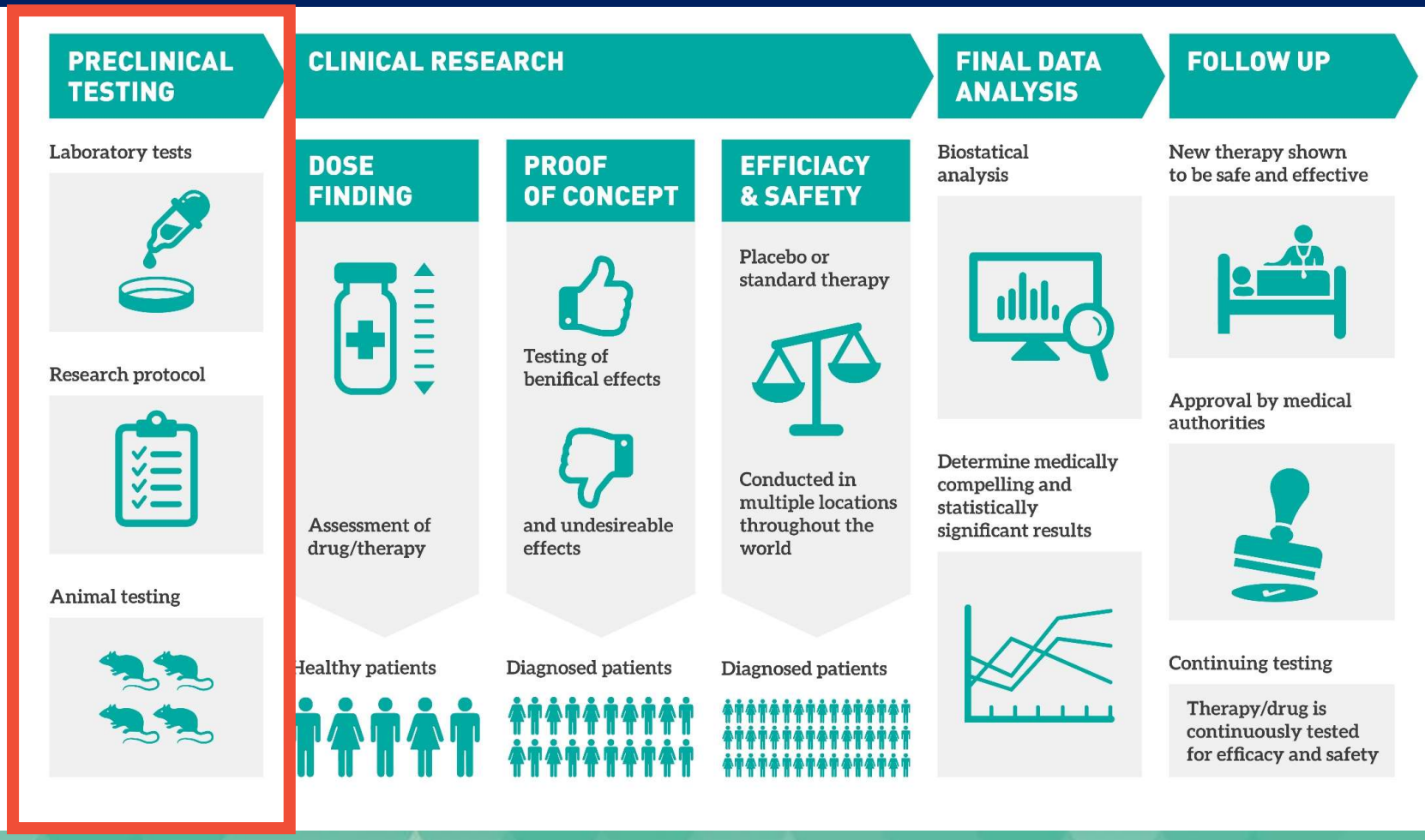
Treat Genetic Diseases

- Most are rare diseases
- Less than 10% of genetic diseases have a treatment
- Create treatments for severe diseases where we can have a big impact

Novel RNA Therapies

- Designed specifically for a genetic mutation (personalized medicine)
- Treat the underlying cause of the disease

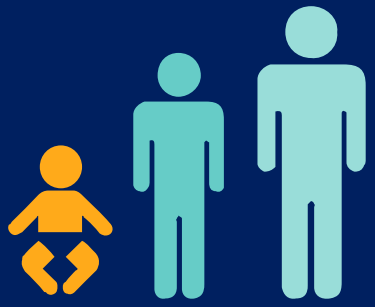
Introduction to Drug Development



QRX-421 for RP in Usher syndrome type 2 (USH2)

RNA therapy

Eye symptoms (RP)



Inherited (genetic) disease

QRX-421 targets mutations in exon 13 of the USH2A gene

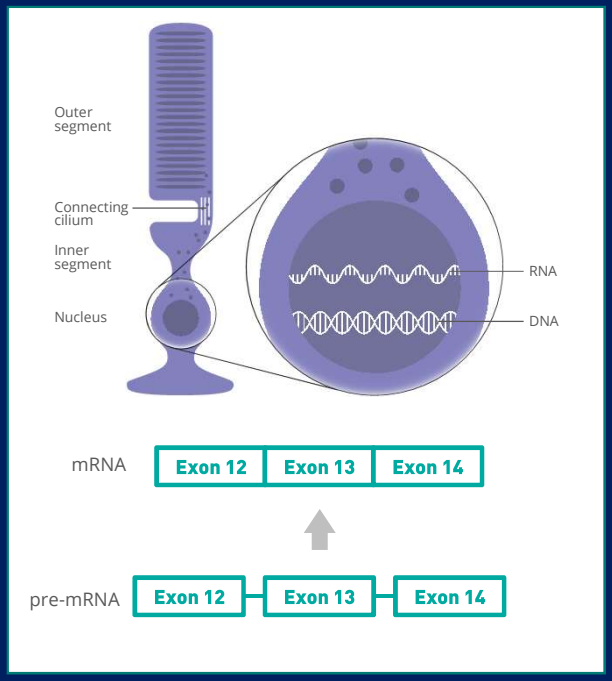
Different mutations cause Usher syndrome type 2

- Most common mutations in exon 13 of the USH2A gene are c.2299delG and c.2276G>T
- A list of known exon 13 mutations can be found at www.lovd.nl/USH2A

QRX-421 for RP in USH2

USH2A exon 13 skipping

Photoreceptor



In the absence of a mutation, RNA is translated in usherin protein. This protein is important for maintenance of photoreceptors.

QRX-421 for RP in USH2

USH2A exon 13 skipping

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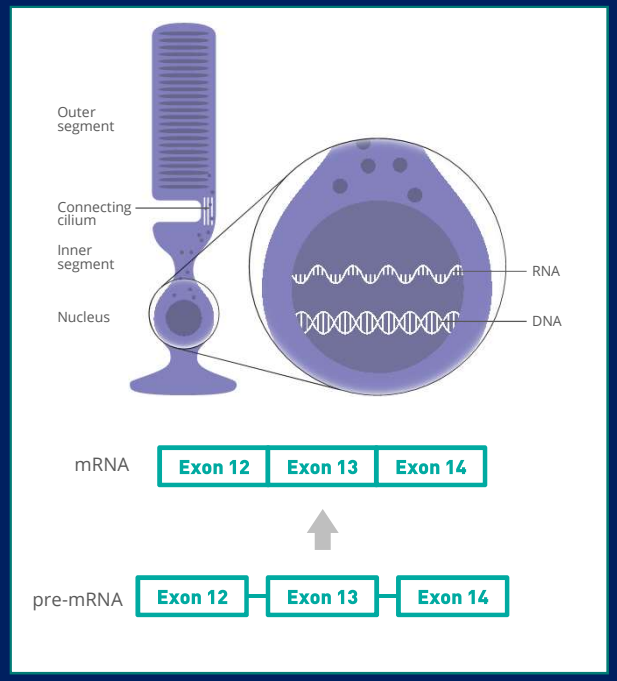

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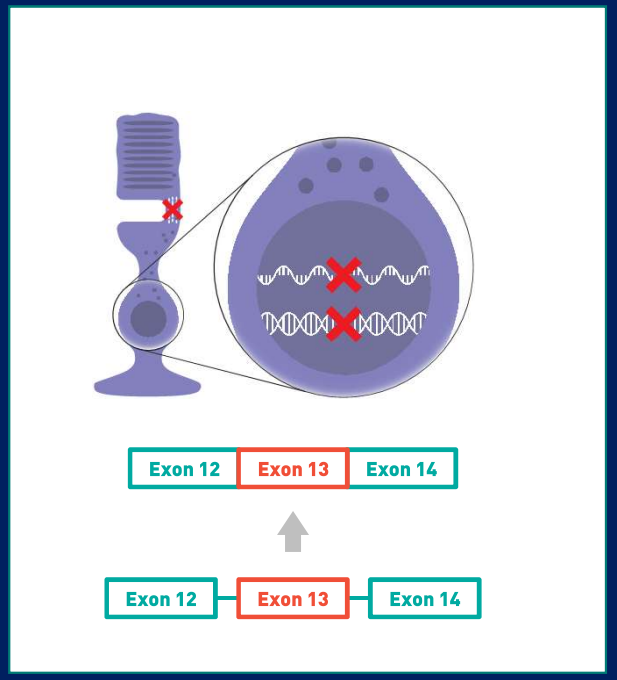
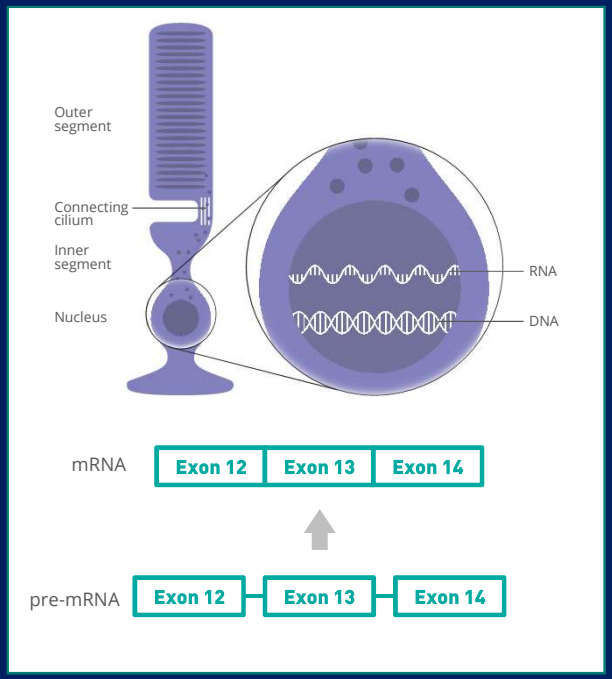


When a mutation is present in exon 13, RNA is broken down, which leads to absence of usherin protein and degeneration of photoreceptors.

QRX-421 for RP in USH2

USH2A exon 13 skipping

Photoreceptor



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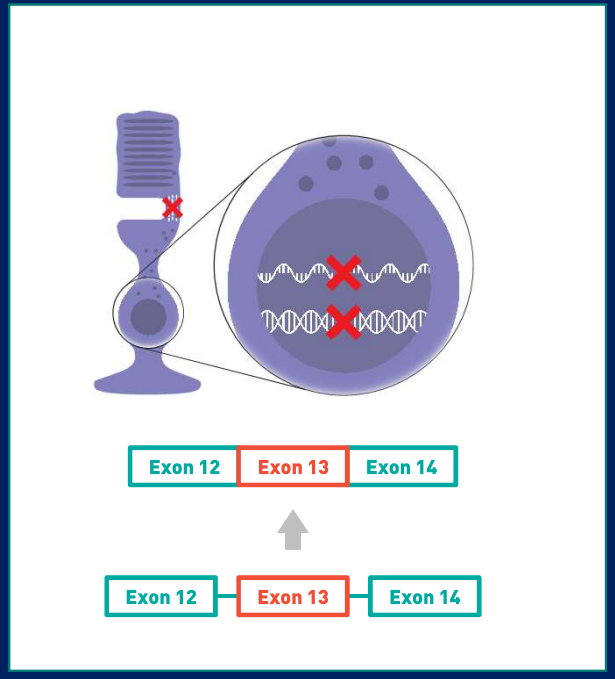

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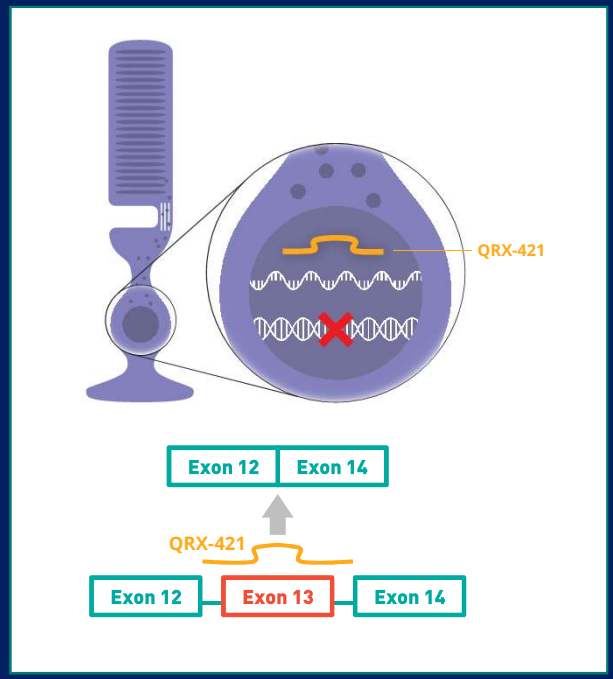
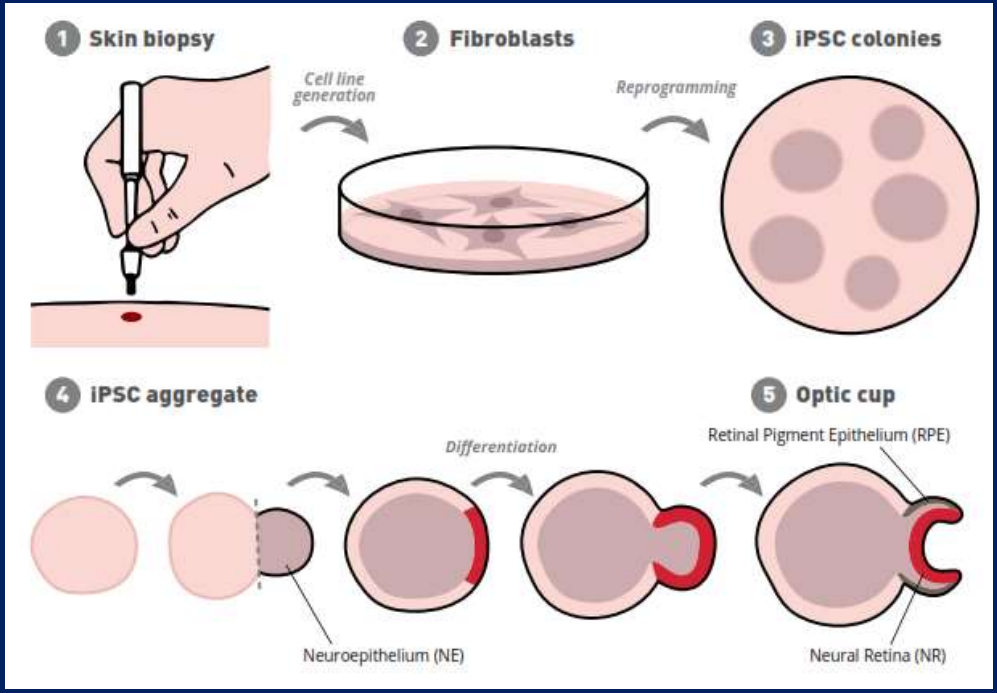


Our treatment strategy is to remove exon 13 from the RNA and restore the usherin protein and maintenance of the photoreceptors.

Eye cup model contains retinal structure

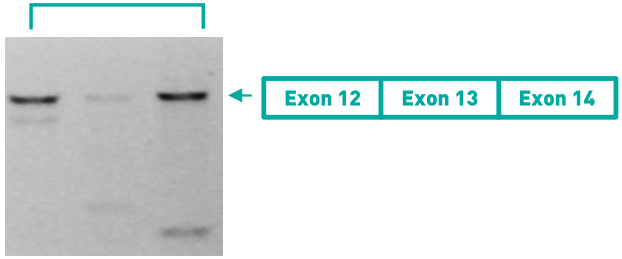


- A real 3D model of human **patient** retina
- Can be grown from any **patient**
- Can show the effect of the mutation in human cells
- Can test human therapeutic compound
- Has been used in successful regulatory submissions in US & EU

QRX-421 mediated exon 13 skip in eye cups

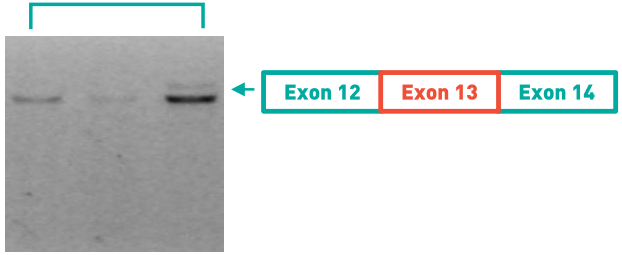
Control

No treatment



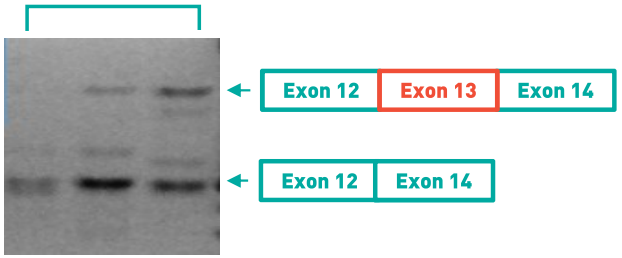
Usher patient

No treatment



Usher patient

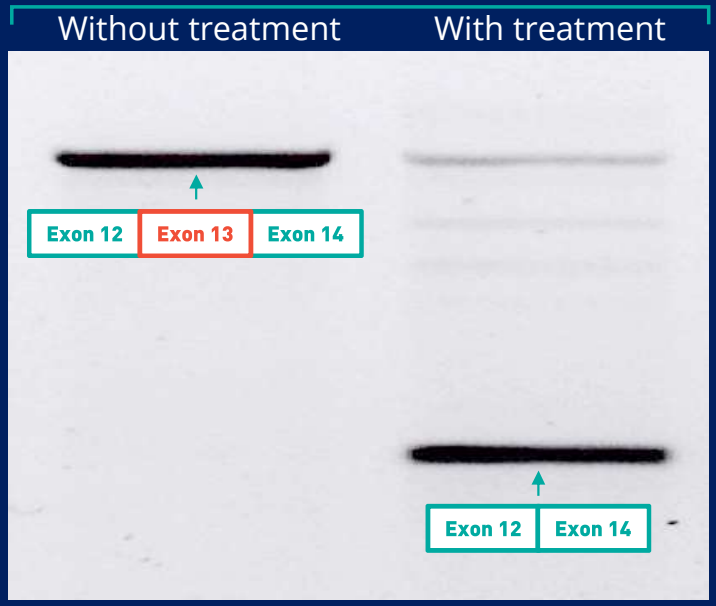
QRX-421



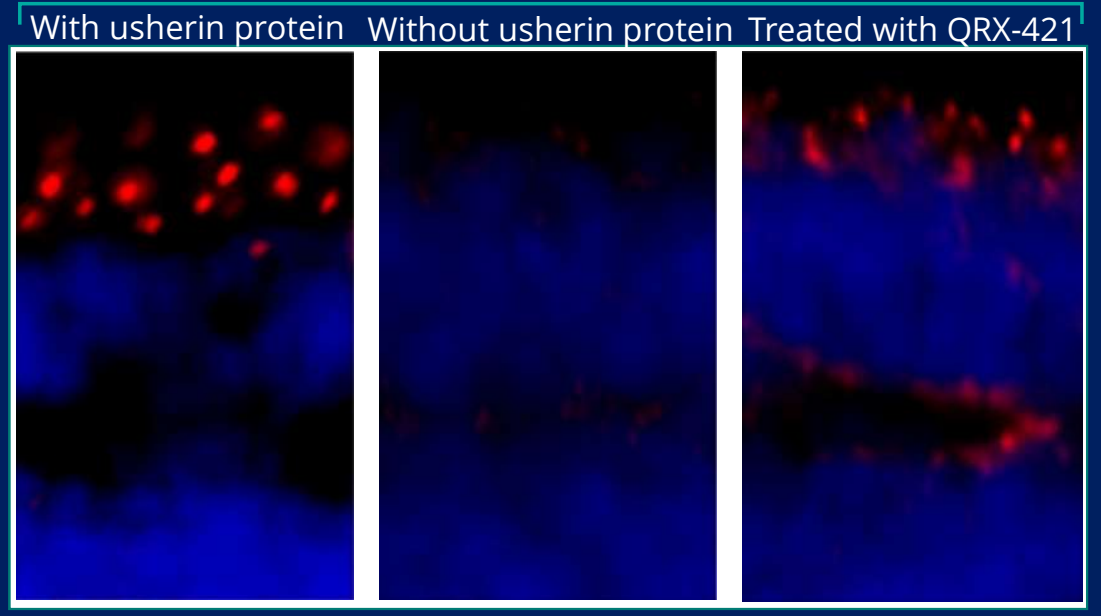
Erwin van Wijk, Radboudumc, Nijmegen, the Netherlands

Restoration of usherin protein expression in zebrafish retina

Exon 13 skip in RNA in zebrafish retina



Usherin protein (in red) in zebrafish retina



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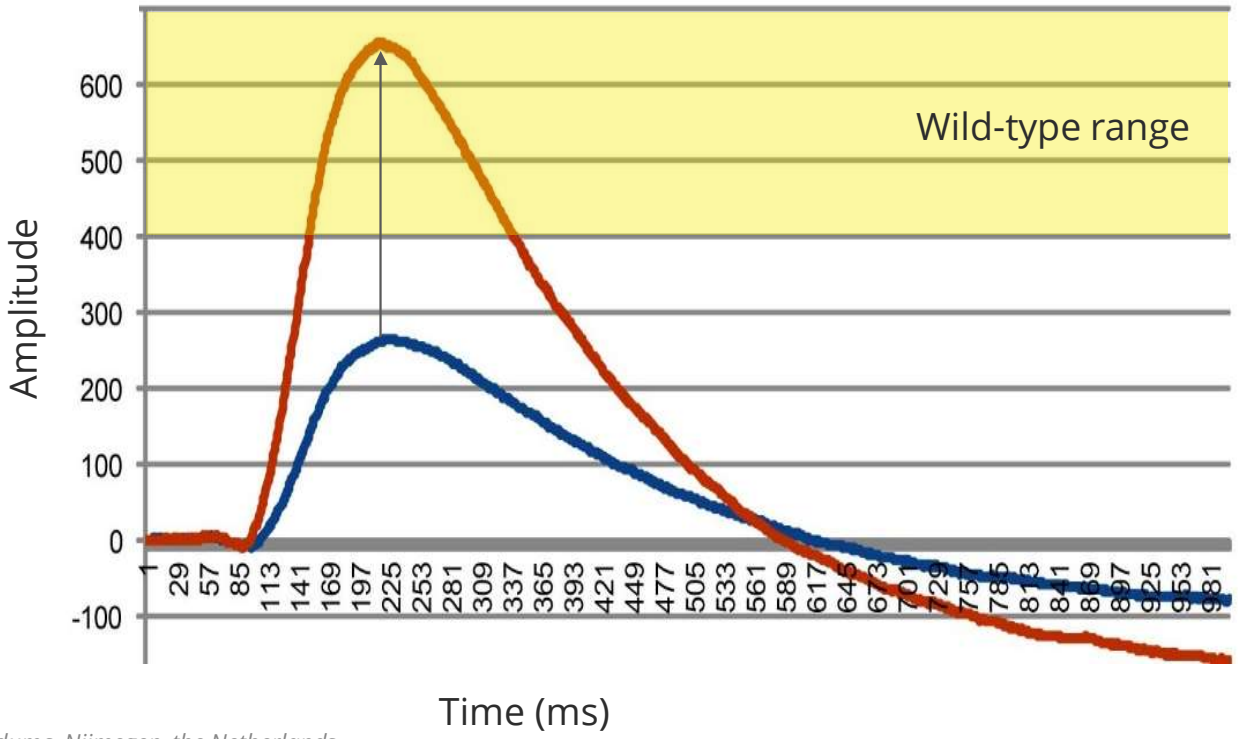
Restoration of ERG amplitude in USH2 zebrafish

Treated exon 13 mutant zebrafish
Exon 13 mutant zebrafish without treatment



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Overview: QRX-421 for RP in USH2

mRNA profile restoration

Local delivery to the eye

mRNA profile restoration in eye-cups

Restoration ush2a protein levels

Functional restoration in Fish model

Clinical candidate selected

Ready to go into Development

Thank you!

- **The Usher Community** who have been so supportive of our efforts
- **Erwin van Wijk and colleagues at Radboudumc** for their collaboration to pursue this very rare indication
- **The regulators** who are willing to help us address the challenges of ultra-rare disease drug development
- For more information and to stay updated on our progress please visit ProQR's website www.proqr.com

ProQR®

IT'S IN OUR RNA