

**(Annamarie Dillon) OK, we are on? Everybody can hear me? Great, first I would like to thank the organizers for the opportunity to come here today to provide you with short updates on our QR-421a program that I'll walk you through in a moment.**

Just to let you know, that as a publicly traded company this presentation will include some forward-looking statements. So, as it was mentioned, ProQR is a Dutch biotech and we are based in Leiden and our approach and therapeutic strategy within the company is to really try to have a patient centric approach. We are focusing on rare genetic disorders, where there is a med need and currently no treatments available.

It is also important that we understand the cause of the disease for which we hope to develop RNA therapies that we will be able to deliver locally to the target organ, so in this case it would be the eye. I included this slide just to provide a very high-level overview of drug developments, it is a very busy slide, but it's just to highlight the lot of the different steps that have to be taken. From a drug, taking a drug candidate to pre-clinical testing and all the different steps through clinical research.

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The studies that will have to be performed to better understand the drug candidate and to make sure that it is safe and that it works and to be able to submit the dossiers to the regular authorities for the approval. And ultimately that physicians will be able to prescribe it for their patients.

This is just an overview slide of our ophthalmology pipeline. So, we have several programs currently in development at different stages. Our most advanced program - we heard yesterday, for those that were there - is for Leber's congenital amaurosis type 10, and for the compound called QR-110. It is exciting for us that we'll expect our first clinical results of that later this year. The next program moving towards the clinic is our program for RP exon 13 mutations. We have some other work ongoing more in the discovery phase.

So, just a brief introduction to QR-421a, it is a bit of a mouth full, I grant you that. So, we are trying to develop a RNA therapy, it is working on the RNA and not the DNA, to treat the eye symptoms, so the retinitis pigmentosa. I am not sure what went wrong there. The therapy will target mutations in exon 13 of the USH2A gene, so it is a targeted approach. And there is many different mutations within exon 13 and there is a public available data base listing those out. Building on the work - I think the sound is gone? It is back again? Building on the work from Aron van Wijk and his team in Nijmegen we are moving this program forward. This shows a cartoon, here on the left is a healthy photoreceptor, where there is no mutations for,

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RNA is translated in usherin protein, which is important for the maintenance of the photoreceptor.

In the middle cartoon we have a situation where there is a mutation present in exon 13 whereby the RNA is broken down. This absence of the usherin protein leads to retinal degeneration of the photoreceptors. Can people hear me, because I don't hear? Ok. This third picture is just an illustration to try to show what our approach and treatment strategy is to remove exon 13 from the pre-RNA, so that the messenger RNA, that will be produced will be slightly shortened, but we expect to be functional. So in a way to maintain the photoreceptors.

So, with all of the work that has been done to date, we are fast approaching moving this program into clinical trials. The primary objectives of this clinical trial, which we are going to be calling Stellar, will be looking primarily at the safety, tolerability and effectivity. We will be looking to see if it will have some effect of QR-421a in adults with RP and in mutation in exon 13.

So I can only echo what Mariya mentioned earlier about the importance of understanding and knowing what your mutation is because as more clinical trials, not only this clinical trial, but future clinical trials we see more and more clinical trials focusing in on certain mutations. We have on top of doing all of the different safety tests and making sure that this is safe we will also see if it'll have some impacts if we can check that an efficiency with regards to vision. I can't share a nice diagram of the clinical

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trial design today, we are still interacting with the regulatory authorities so what we can say now at this moment in time, that we hope to finalize this very soon.

It is good to mention, that we envision that QR-421a will be administered through an injection into the eye, called an intravitreal injection. We will also be looking at different dose levels within the patients, and for this first study we will only include adult patients with mutations in exon 13. We hope, as mentioned earlier in the meeting, to move this program before the end of the year, that we hope to start it and start recruiting participants into this study and we hope to be able to share more information next year. That we will have some data, clinical data, that we will be able to share. And the study as foreseen now will be conducted in North America and Europe.

It is also very heartwarming to mention that we have been working and that we have the support of the Foundation Fighting Blindness, they are supporting us financially with this program, and we are partnering with them moving this program forward. And also it is good to mention that we have been interacting with representatives of the Usher community these past months. Before we even talked about writing a protocol we had an advisory board earlier this year, with representatives both from the international Usher community, but also from several different countries in North America and across Europe.

This was extremely helpful for us, as we started thinking about the design of the study. And any time we have

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questions, representatives that we reach out to are very gracious with their advice and support to us, so I wanted to say a big thank you for that. If you would like to stay updated on where we are at, and how the program is moving forward, I would encourage you to just periodically check our website as we will keep that updated with the latest information. And once the clinical trial is on [clinicaltrials.gov](https://clinicaltrials.gov), you will be able to see a full list of participating sites as well. With that, I think I am on time.

(applause)