QR-421A STELLAR TRIAL RESULTS

FFB Ush 1b Meeting, 13th Sept 2021

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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 preclinical and clinical trial plans and related timing of trials and results, the design of planned trials for QR-421a and the expected regulatory pathway for this product candidate, including the potential for the Sirius and Celeste trials to serve as the sole registration trials in this indication, research and development, future financial position, future revenues, projected costs, prospects, therapeutic potential of our product candidates, 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.

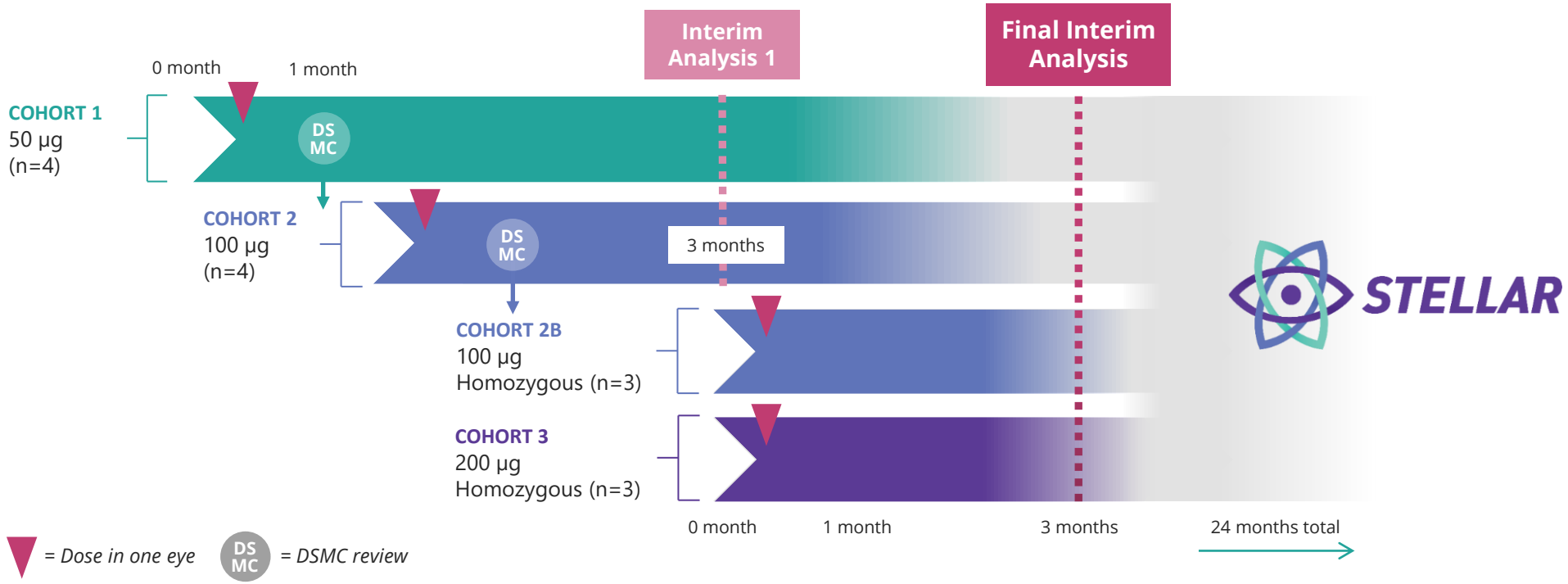
Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this presentation. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our annual report filed on Form 20-F. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by us and our collaborative partners whose operations and activities may be slowed or halted by the COVID-19 pandemic; the likelihood of our clinical programs being executed on timelines provided and reliance on our contract research organizations and predictability of timely enrollment of subjects and patients to advance our clinical trials and maintain their own operations; our reliance on contract manufacturers to supply materials for research and development and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the unpredictability of the duration and results of the regulatory review of applications or clearances that are necessary to initiate and continue to advance and progress our clinical programs; the ability to secure, maintain and realize the intended benefits of collaborations with partners; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in research and development; and general business, operational, financial and accounting risks, and risks related to litigation and disputes with third parties. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law

QR-421a: First-in-class RNA therapy

- QR-421a targets Exon 13 mutations in Ush2a (>16,000 patients)
- QR-421a aims to prevent patients from going blind
- $7.5M co-funding from Foundation Fighting Blindness

QR-421a Phase 1/2 trial in Usher & nsRP

Enrollment completed; 2nd and final Interim Analysis conducted



***Stellar* Phase 1/2 trial**

- Randomized, sham masked, single ascending dose, global multicenter, 24-month study

Key endpoints include:

- **Visual acuity (VA):** Best-Corrected VA
- **Visual field:** Static perimetry, microperimetry, dark-adapted chromatic (DAC) perimetry
- **Optical Coherence Tomography (OCT) Imaging**

Goal: to identify for next trial:

- **Registration endpoint(s)**
- **Dose, dosing regimen**
- **Population**

QR-421a observed to be safe and well tolerated

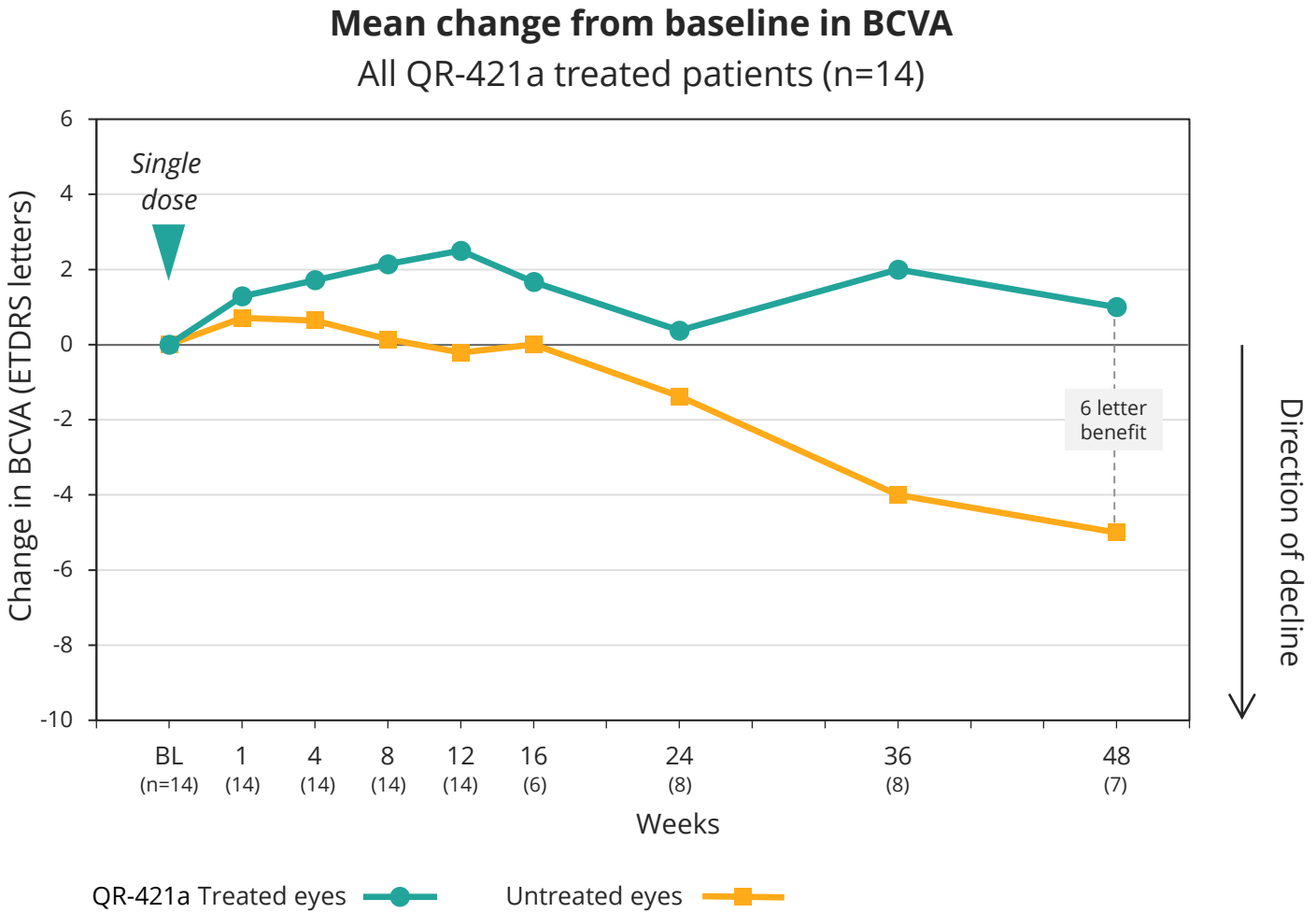
- **QR-421a was observed to be safe and well tolerated in >3,700 subject follow up days**
- **No SAEs, no inflammation**
- Cataracts occur in >30% patients in natural history of disease
  - 1 patient had worsening of pre-existing cataracts in both the treated and untreated eye with cataract extractions in both eyes
  - Deemed not treatment related by Investigator
- Cystoid Macular Edema (CME) known to occur as part of natural history of disease in >30% of the patients
  - No new occurring cases of CME during study
  - 1 patient with CME at baseline progressed during study, classified as mild, managed with standard of care

Advanced population efficacy results

Population with progressed visual acuity loss

BCVA stabilization in treated eye

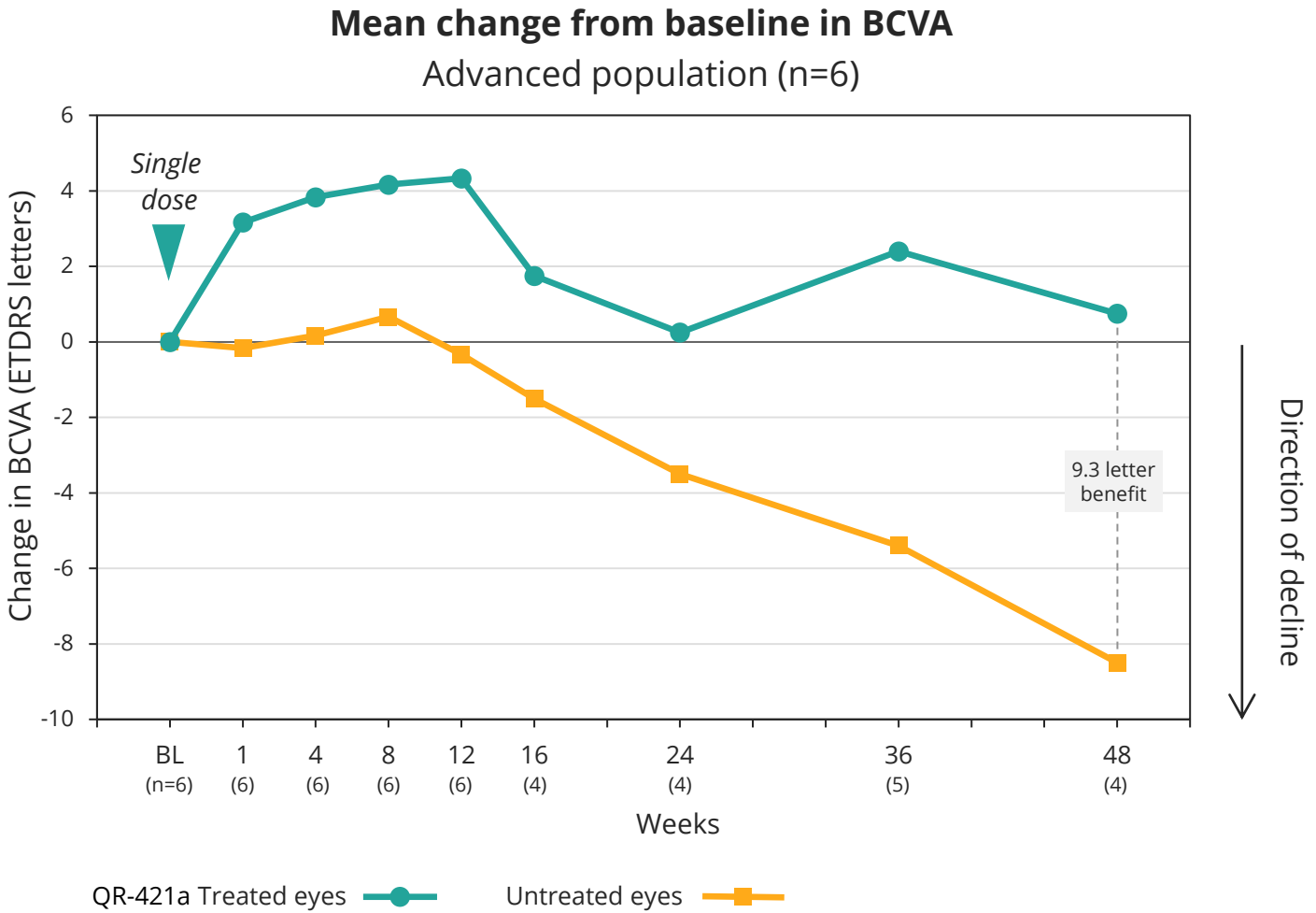
Mean 6 letter benefit at week 48



- Stabilization of vision observed in treated eye vs decline in untreated eye in all patients
- 6 letter benefit at week 48, after single dose
- Sustained effect is consistent with the long half-life of QR-421a

BCVA stabilization driven by advanced population

Mean 9.3 letter benefit at week 48



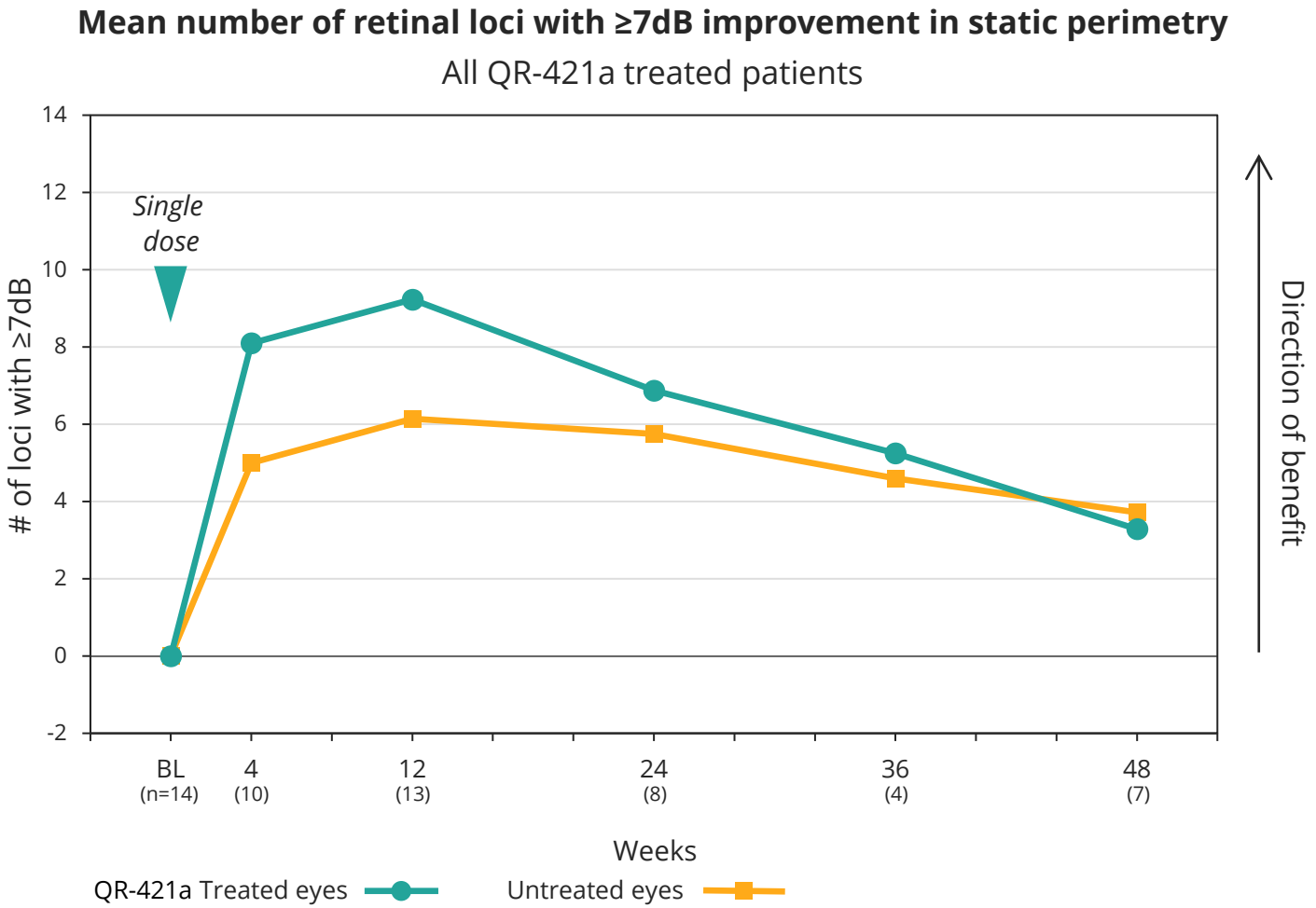
- BCVA response is driven by advanced disease population
- Stabilization of vision in treated eye after single dose
- Deterioration of untreated eye in line with expected natural history of disease
- Mean 9.3 letter benefit at week 48 in the advanced population
- Sustained effect is consistent with the long half-life of QR-421a

Early-moderate population efficacy results

Population with visual field loss, but minimal visual acuity loss

Improvement in treated eyes on static perimetry

Measuring retinal sensitivity in peripheral visual field

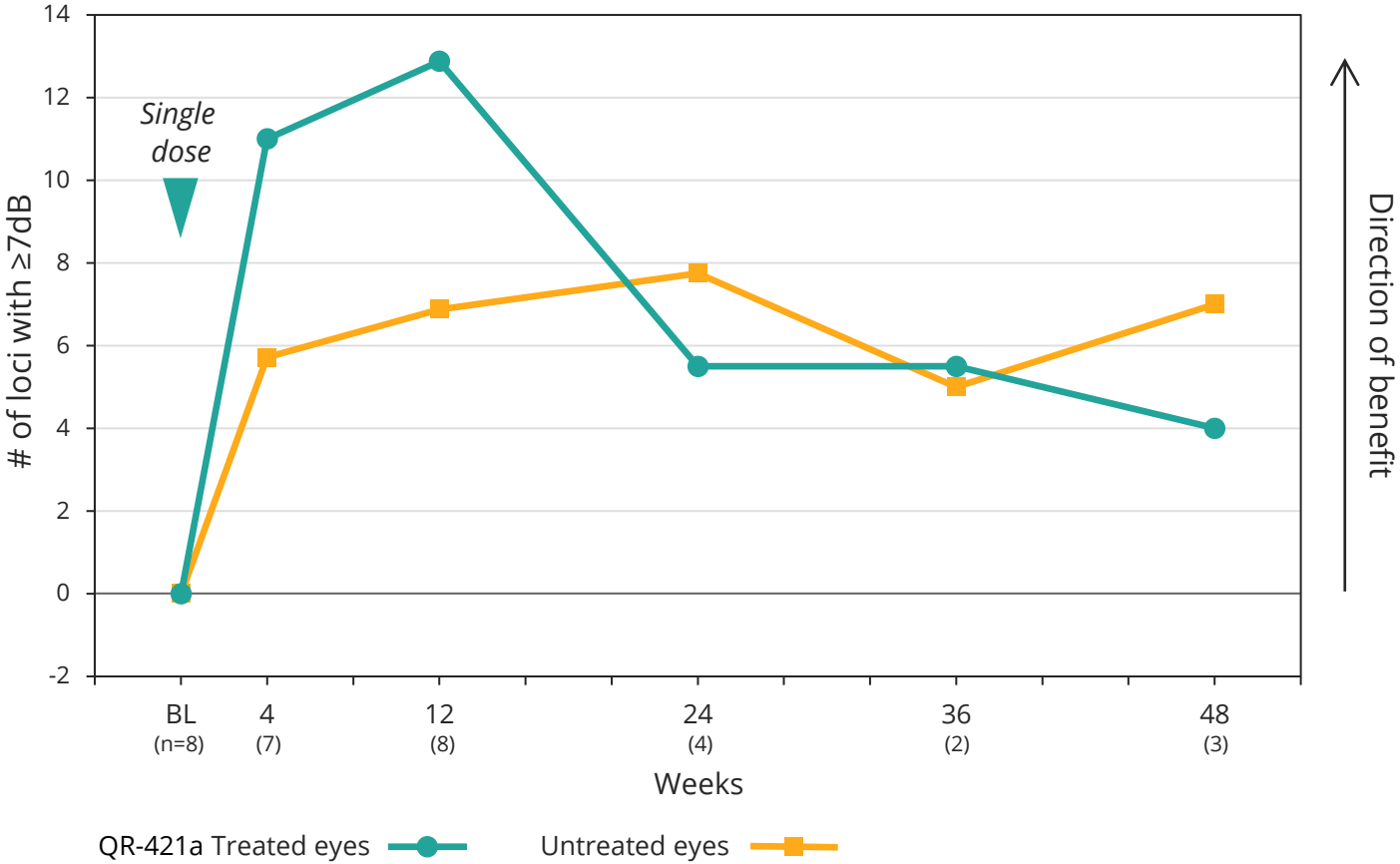


- Benefit observed in treated eyes vs untreated eyes
- Benefit sustained for 9+ months after single dose
- Static perimetry improvement in line with approvable endpoint threshold

Static perimetry improvement driven by early-moderate population

Mean number of retinal loci with ≥7dB improvement in static perimetry

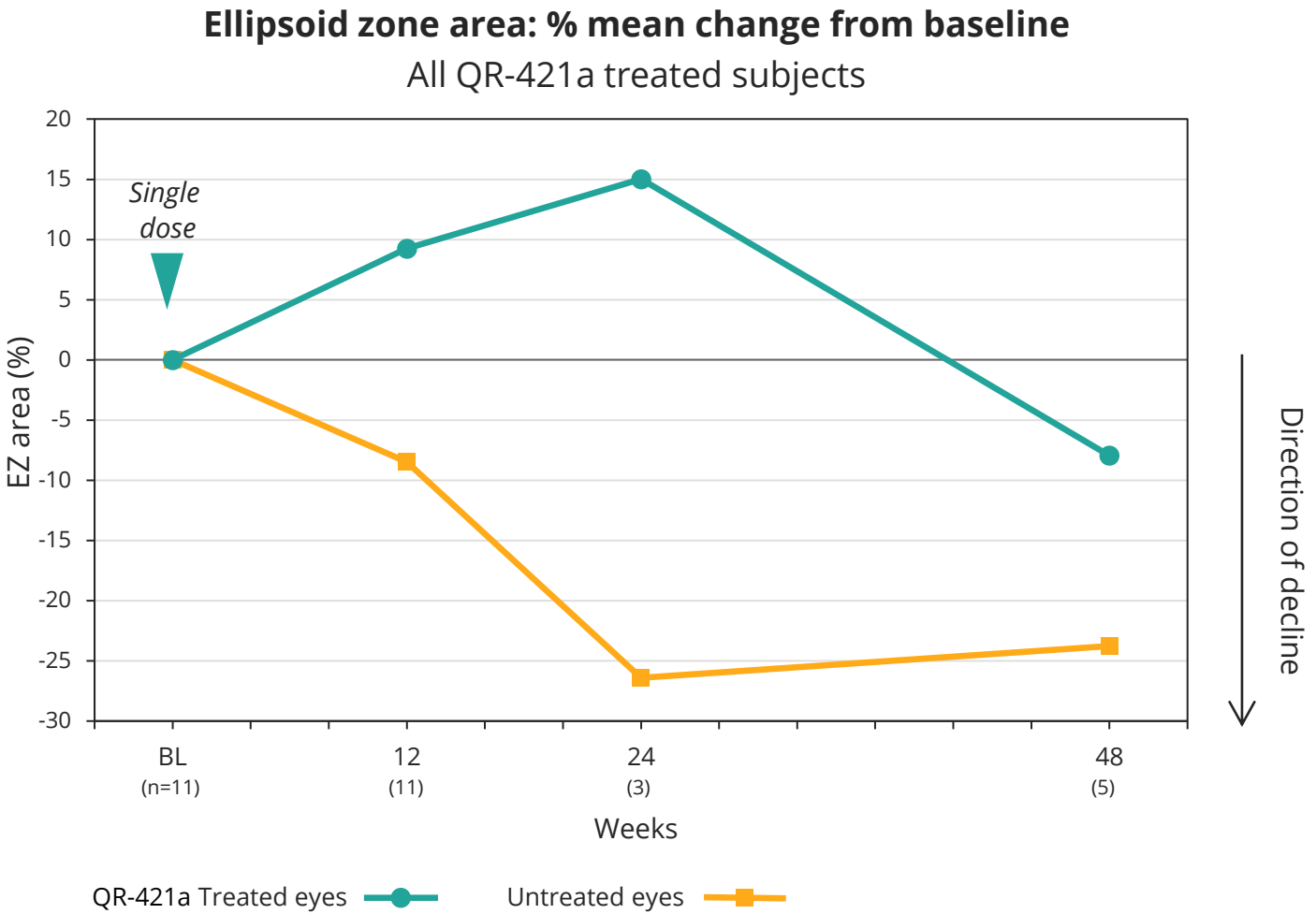
Early-moderate population (n=8)



- Benefit observed in treated eyes vs untreated eyes after single dose
- Magnitude greater in early-moderate population
- Static perimetry improvement in line with approvable endpoint threshold

Stabilization of retinal structure in treated eyes

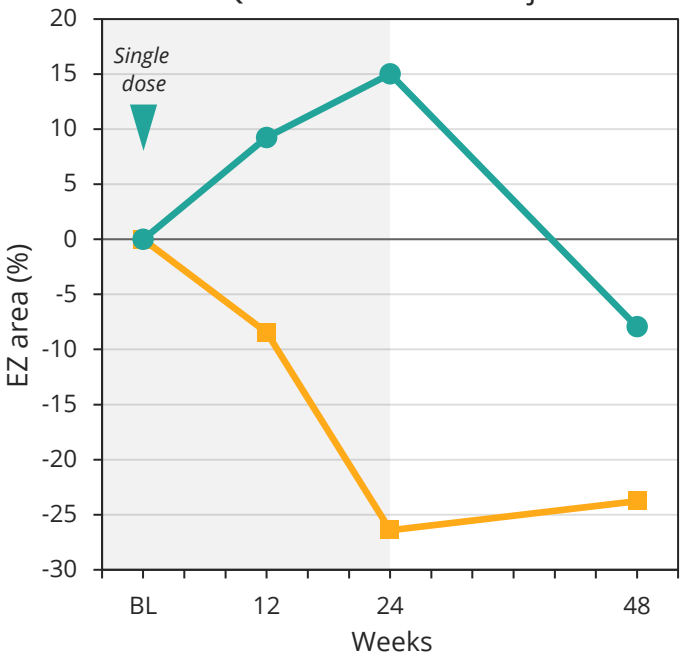
Measured by OCT based Ellipsoid Zone (EZ) in the central macular area



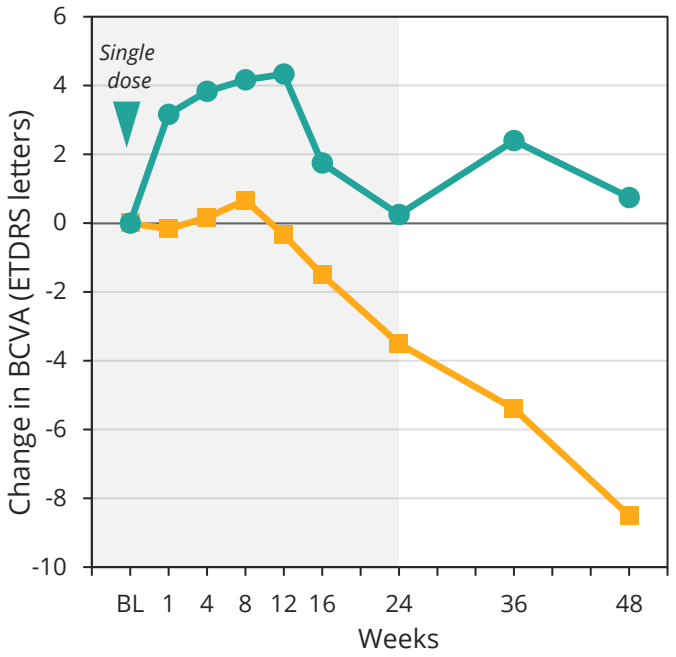
- Stabilization in the treated eyes out to 48 weeks, after single dose
- Deterioration in untreated eyes in line with natural history
- Benefit on OCT provides objective validation of response on BCVA and other endpoints

Dosing interval identified at 6 months

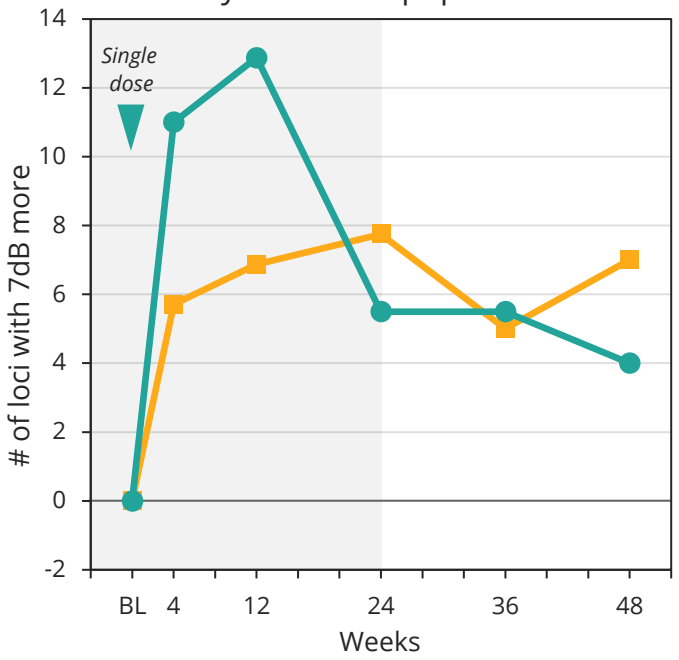
Ellipsoid zone area:
% mean change from baseline
All QR-421a treated subjects



Mean Change from Baseline in BCVA
Advanced Population



Mean Number of retinal loci with ≥7dB improvement in static perimetry
Early-moderate population



QR-421a Treated eyes — Untreated eyes

- Effect sustained for approx. 6 months across endpoints
- Durability in line with half-life and pre-clinical modeling
- **Redosing interval established at 6 Months**

Summary of Phase 1/2 results

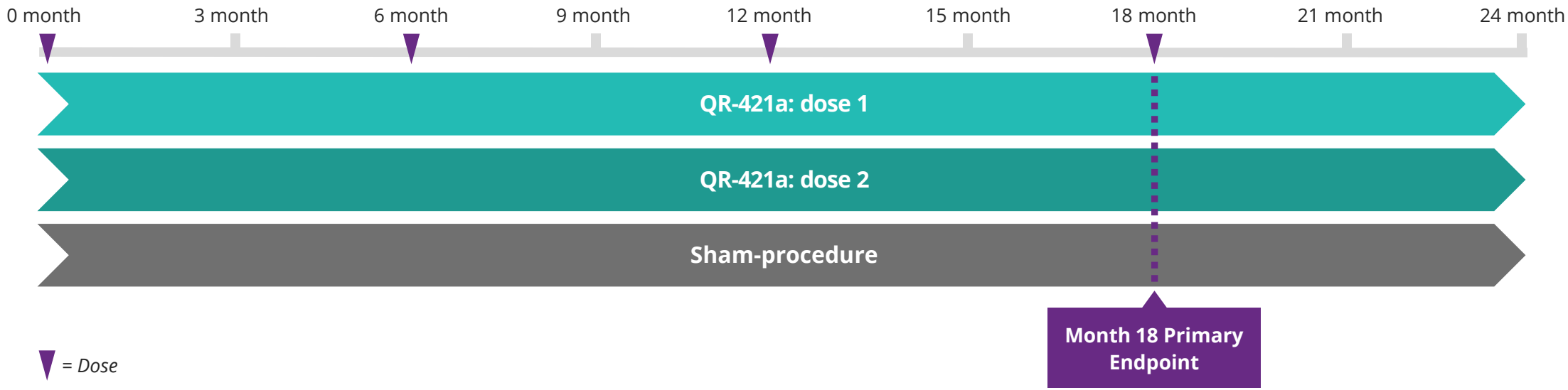
- ✓ **QR-421a was observed to be safe and well tolerated**
- ✓ **Clinical proof of concept established, consistent with baseline disease, after single dose**
  - ✓ Advanced disease: 100% of patients had a BCVA benefit, 0% in sham group
  - ✓ Early-moderate population: Improvement on Static Perimetry
  - ✓ Supported by key secondary endpoints:
    - ✓ Stabilization of EZ area on OCT imaging (objective measurement)
    - ✓ Stabilization of Microperimetry-based retinal sensitivity
  - ✓ Dose range and dose interval established

- **All information acquired in *Stellar* to design Phase 2/3 studies:**
  - *Sirius* clinical study: a Phase 2/3 study in *advanced patients*
  - *Celeste* clinical study: a Phase 2/3 study in *early-moderate patients*

QR-421a planned Phase 2/3 for *Advanced Patients*

Preliminary design, to be agreed with regulators

SIRIUS

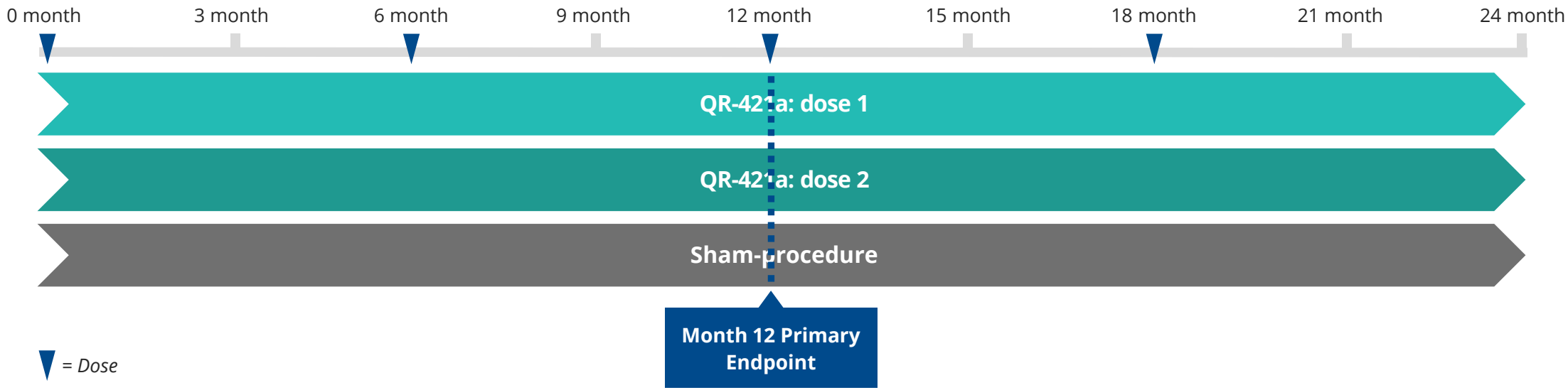


- Double-masked, randomized, sham controlled, 24-month, multiple dose study
- Population:
  - Approx. 100 patients
  - Homozygous and heterozygous, Usher and nsRP
  - Baseline BCVA ≤ 20/40
- Primary endpoint: Visual Acuity
- Key secondary endpoint: OCT, Mobility course, Perimetry
- **Anticipated start of trial: YE 2021**

QR-421a planned Phase 2/3 for *Early-Moderate patients*

Preliminary design, to be agreed with regulators

CELESTE



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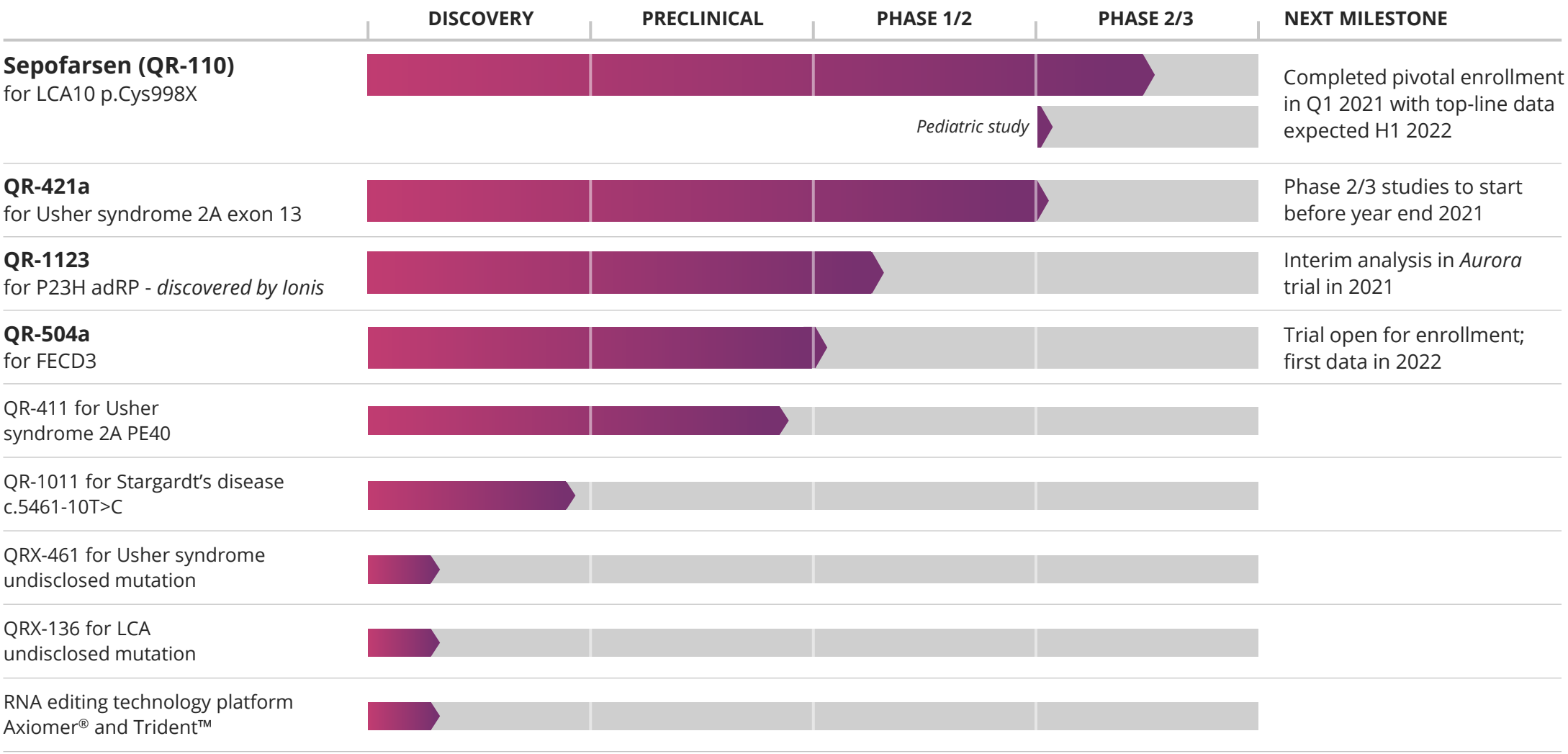
- Population:
  - Approx. 100 patients
  - Homozygous and heterozygous, Usher and nsRP
- Primary endpoint: Static Perimetry
- Key secondary endpoint: Mobility course, BCVA, OCT
- **Anticipated start of trial: YE 2021**

Deep pipeline in ophthalmology

With multiple near-term catalysts



Acknowledgements

- Patients/Caregivers
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THANK YOU