Skyline Month 3 Interim Data Analysis

May 2022

Disclaimers

This presentation contains forward-looking statements that reflect AGTC's plans, estimates and beliefs, including statements regarding the potential of the company's gene therapy platform and the strength of interim results from the Skyline Trial in XLRP, the potential of AGTC-501 as a treatment for XLRP, the ability to use the interim Skyline results as a predictor of the success of the final Skyline and Vista clinical trial results and whether these results will support future regulatory filings for AGTC-501 . Forward-looking statements include information concerning our possible or assumed future results of operations, including results and timing of our clinical trials and planned clinical trials, business strategies and operations, financing plans, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities, the effects of competition and the impact of the COVID-19 pandemic, including the impact on AGTC's ability to enroll patients. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipates," "believes," "could," "seeks," "estimates," "expects," "hopes," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" or similar expressions and the negatives of those terms. Actual results could differ materially from those discussed in the forward-looking statements, due to a number of important factors. Risks and uncertainties that may cause actual results to differ materially include, among others: gene therapy is still novel with only a few approved treatments so far; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate or receive reasonable reimbursement; uncertainty inherent in clinical trials, including interim data, and the regulatory review process; risks and uncertainties associated with drug development and commercialization; the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations and financial condition; factors that could cause actual results to differ materially from those described in the forward-looking statements are set forth under the heading "Risk Factors" in our most recent annual report on Form 10-K filed with the Securities and Exchange Commission, or SEC, as supplemented by subsequently filed quarterly reports on Form 10-Q. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our management's plans, estimates, assumptions and beliefs only as of the date of this presentation. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

Agenda

- Introduction — Sue Washer, CEO
- XLRP Skyline Trial 3-Month Interim Data — Susan Schneider, CMO
- Q&A
  - Sue Washer, Susan Schneider, Jon Lieber, CFO and Dr. Robert Sisk, MD, FACS, FASRS, Director of Pediatric Vitreoretinal Surgery and Director of Ophthalmic Genetics – Cincinnati Children's Hospital and the Cincinnati Eye Institute and an investigator in the trial
- Closing Remarks — Sue Washer

Key Takeaways for Skyline Phase 2 Trial

- **Primary Endpoint:** Robust improvement in visual sensitivity
  - Dose group A responders: 1 of 4*, 25% and **Dose group B responders: 5 of 8, 62.5%**
    - Responders defined as patients with a 7 dB or greater improvement in at least 5 loci measured by MAIA microperimetry
  - Vista trial powered to be statistically significant for a 50% response rate
  - Trial remains masked to patients and sites
- Maze data demonstrates positive trends
  - There are trends to improvement both in levels passed, increased speed, and decreased errors
  - Good baseline Best Corrected Visual Acuity (BCVA) and lack of bilateral treatment may have been factors reducing more robust response
- Improvement trends in BCVA
  - Due to improved baseline for these patients, BCVA trends were less pronounced than in Phase 1/2
- Generally well tolerated
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If Vista (Phase 2/3) data is consistent with Skyline and Phase 1/2 data, the Company intends to prepare a BLA filing.

* There were 5 patients in this group, but one patient was not evaluable.

X-Linked Retinitis Pigmentosa (XLRP)

OVERVIEW

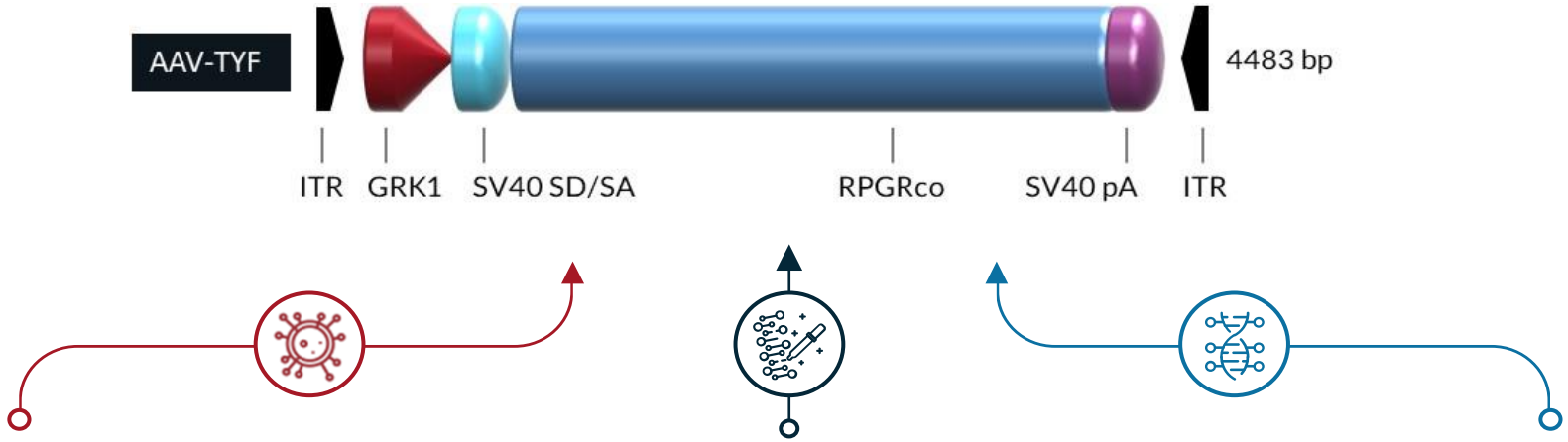
- Missing protein results in degeneration of rods and cones
- ~20,000 patients in US and EU
- No current treatments

IMPACT

- Early night blindness, progressive constriction of visual fields
- Legally blind by a median age of 45

"Blindness is devastating – it robs you of so many things. Number one is your freedom. It robs you of your independence."

Putting the Pieces Together: AGTC Robust XLRP Product Design



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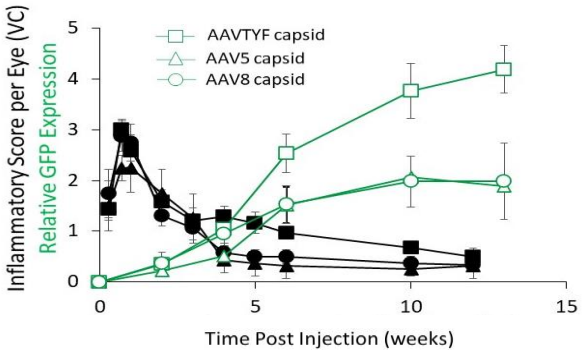
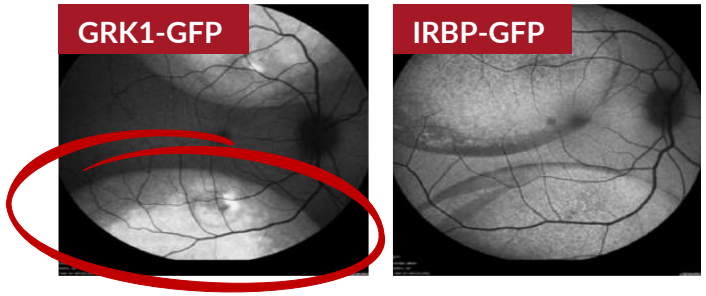


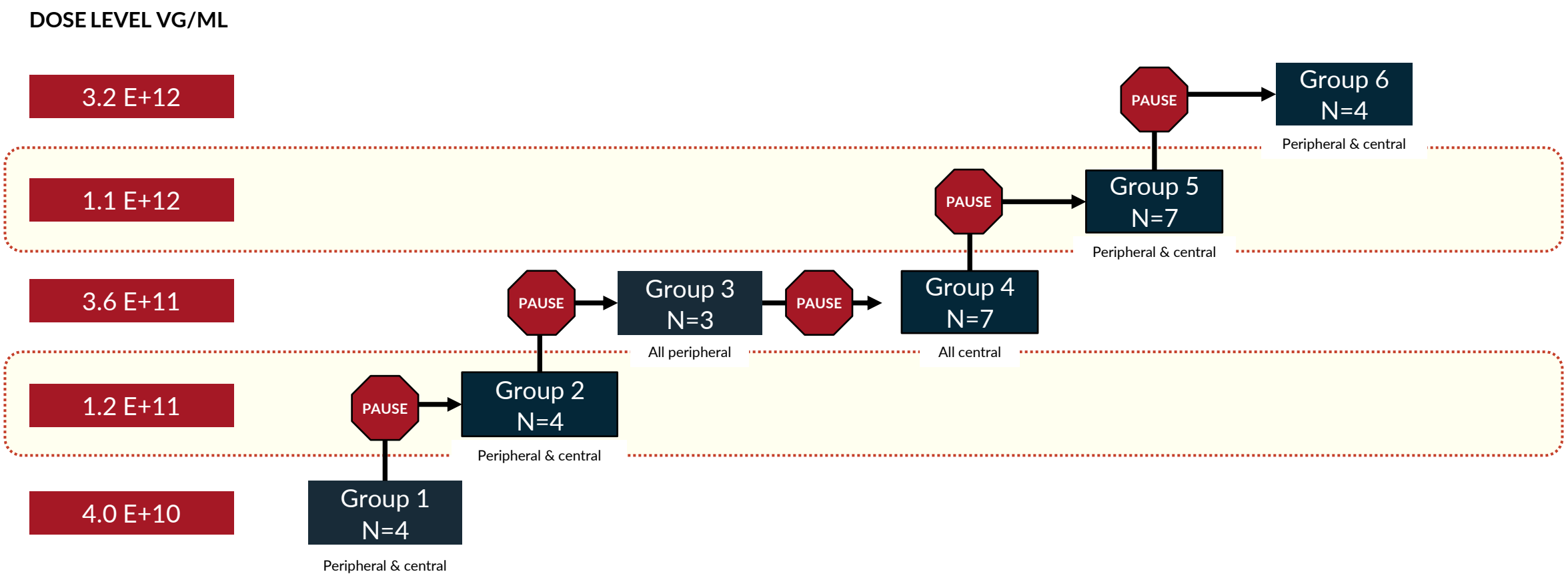
PROMOTER



CODON-OPTIMIZED GENE

Model	In-Life	Procedure	Result
 XLPRA2 dog 9E+10 vg/eye	6-8 weeks	**DNA & RNA** PCR/RT-PCR Sanger Sequencing	✓ Sequence confirmed ✓ Stable RPGR, no mutations
 Rd9 mouse 4E+9 vg/eye	6 weeks	**RNA & Protein** RT-PCR Sanger Sequencing SDS-PAGE/WB	✓ Sequence confirmed ✓ Stable RPGR, no mutations ✓ RPGRco protein correct size ✓ RPGRco glutamylation analysis

XLRP Phase 1/2 Trial Overview: Dose Escalation



PAUSE DSMC Review

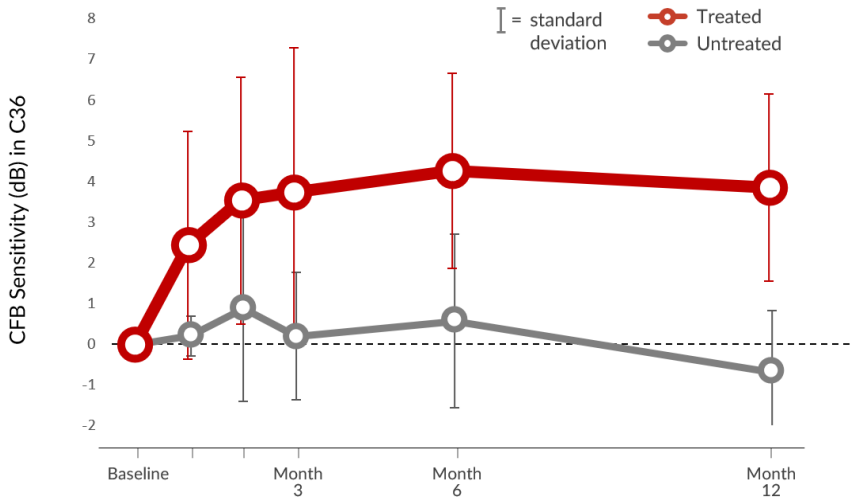
XLRP Phase 1/2 Key Takeaways

Reported positive data from the ongoing Phase 1/2 trial showing:

- Improvement in visual sensitivity through Month 12; 50% for high dose groups
- We believe BCVA improvements are supportive through Month 12
- Correlation between visual sensitivity and retinal structure as measured by OCT through Month 18
- Generally well tolerated through Month 12

Microperimetry – Six Responders at Month 12

All Responders, N=6



Increased mean sensitivity relative to baseline across the central 36 loci

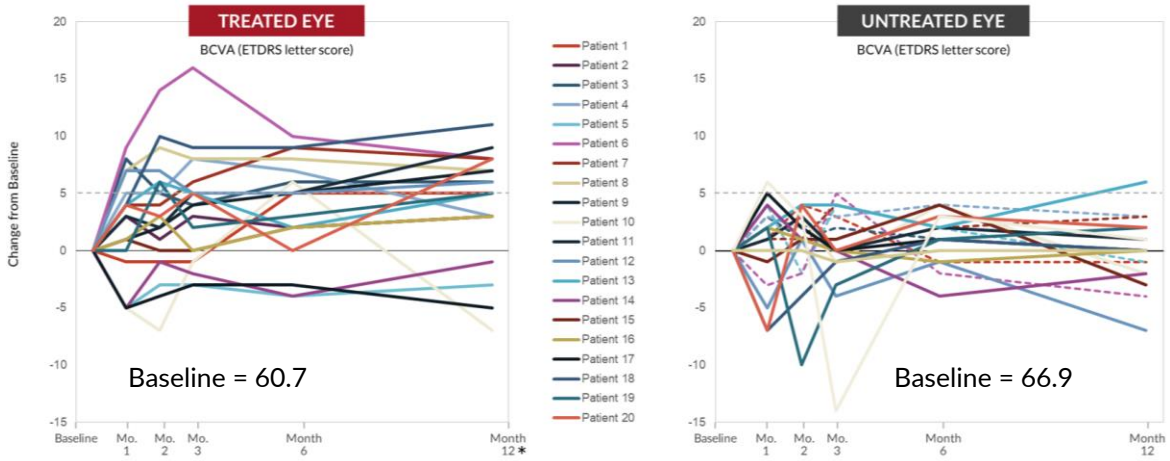
Responder identified as patient with ≥ 7 dB improvement in sensitivity at ≥ 5 loci in central 36 loci of perimetry grid at Month 12

All responders were responders by month 3 and stayed responders

BCVA – Individual Centrally Treated Patient Data at Month 12

All Groups, N=20

Supportive evidence of statistically significant improved visual acuity across all centrally dosed groups



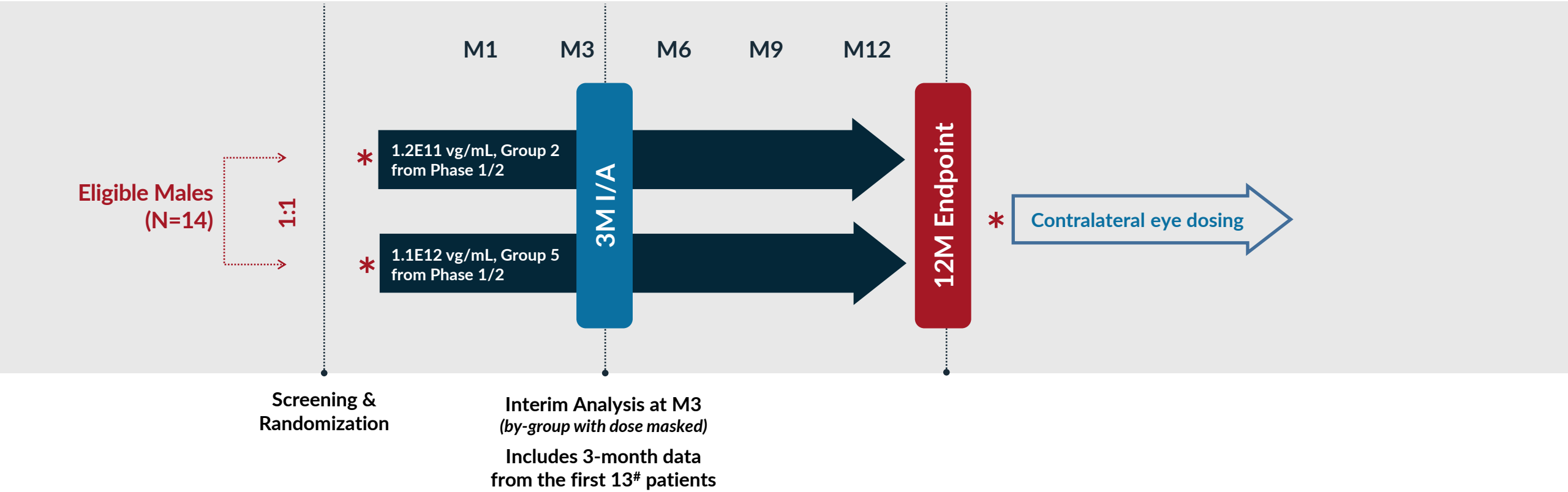
* P = 0.0004 at Month 12 for proportion of patients with ≥5 letter improvement in treated eyes versus fellow untreated eyes (Fischer's exact test)

Efficacy Data

Robust interim data with 62.5% responders for visual sensitivity in Dose Group B at month 3

Skyline Clinical Trial

First chance to verify positive outcomes seen in the Phase 1/2 in a masked fashion and assess performance on the mobility maze

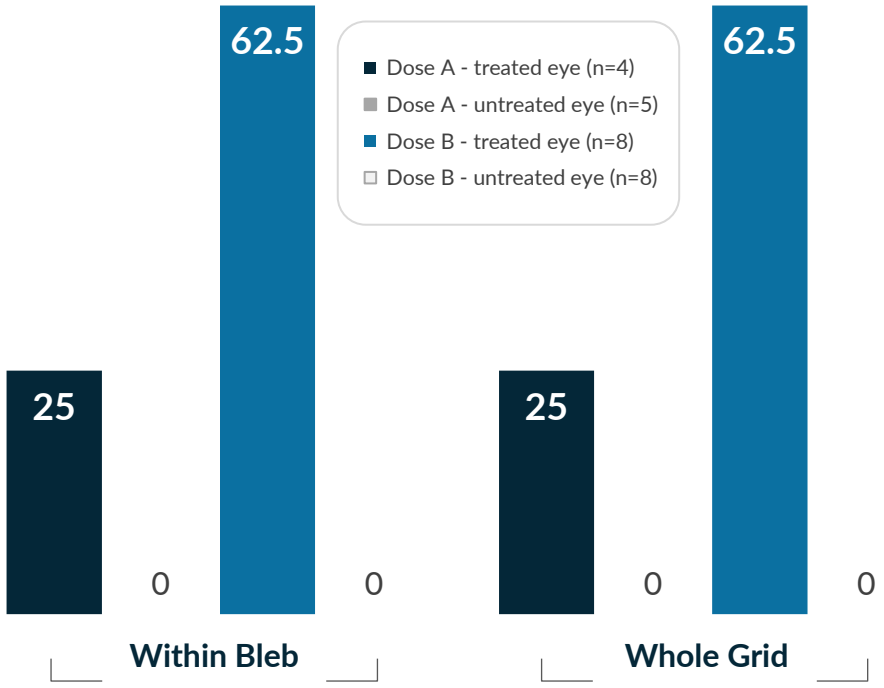


* Sub-retinal injection of AGTC-501 at up to 0.3 mL/eye in the study eye. # Patient 14 did not reach Month 3 prior to data analysis.

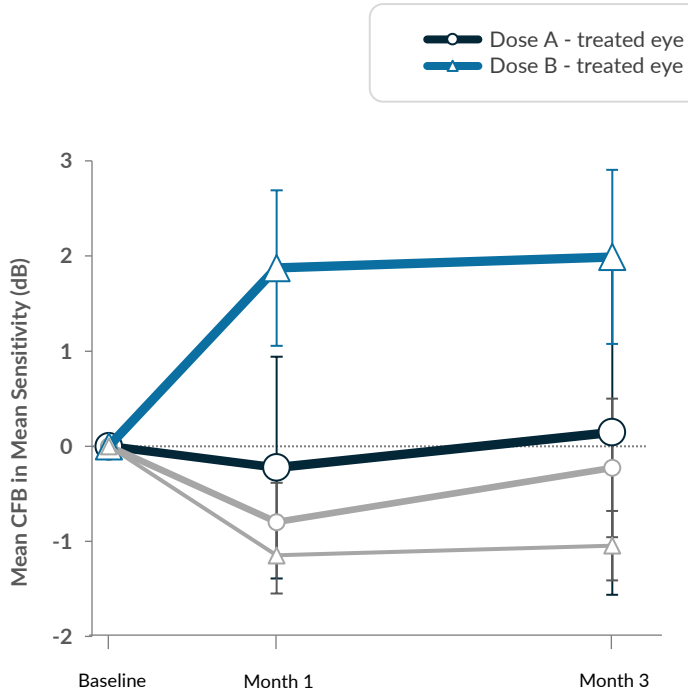
SKYLINE Primary Endpoint: Visual Sensitivity at Month 3

Robust improvements in visual sensitivity with a clear difference between dose groups

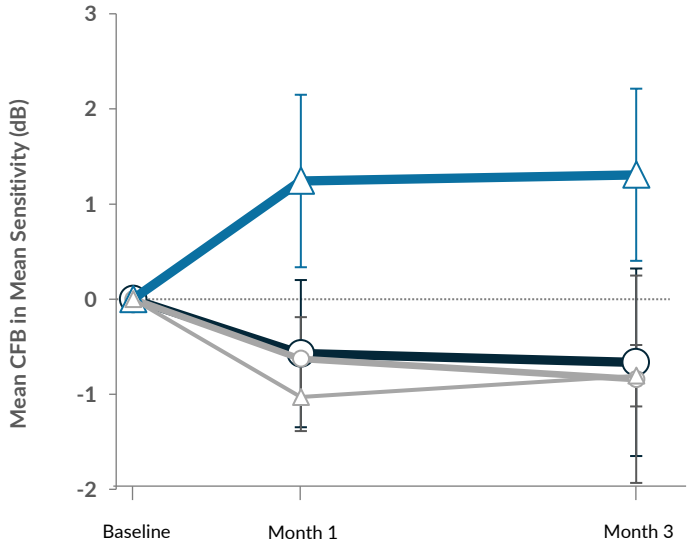
Patients (%) Achieving ≥7 dB improvement in ≥5 Loci at Month 3



All Patients Mean Sensitivity within Central 36 Loci



All Patients Mean Sensitivity within Bleb



Vertical error bars are ± 1 standard error. Sample sizes presented are the number of patients with baseline data and post treatment observations.

Microperimetry: Month 3 Interim Analysis

All groups N=13

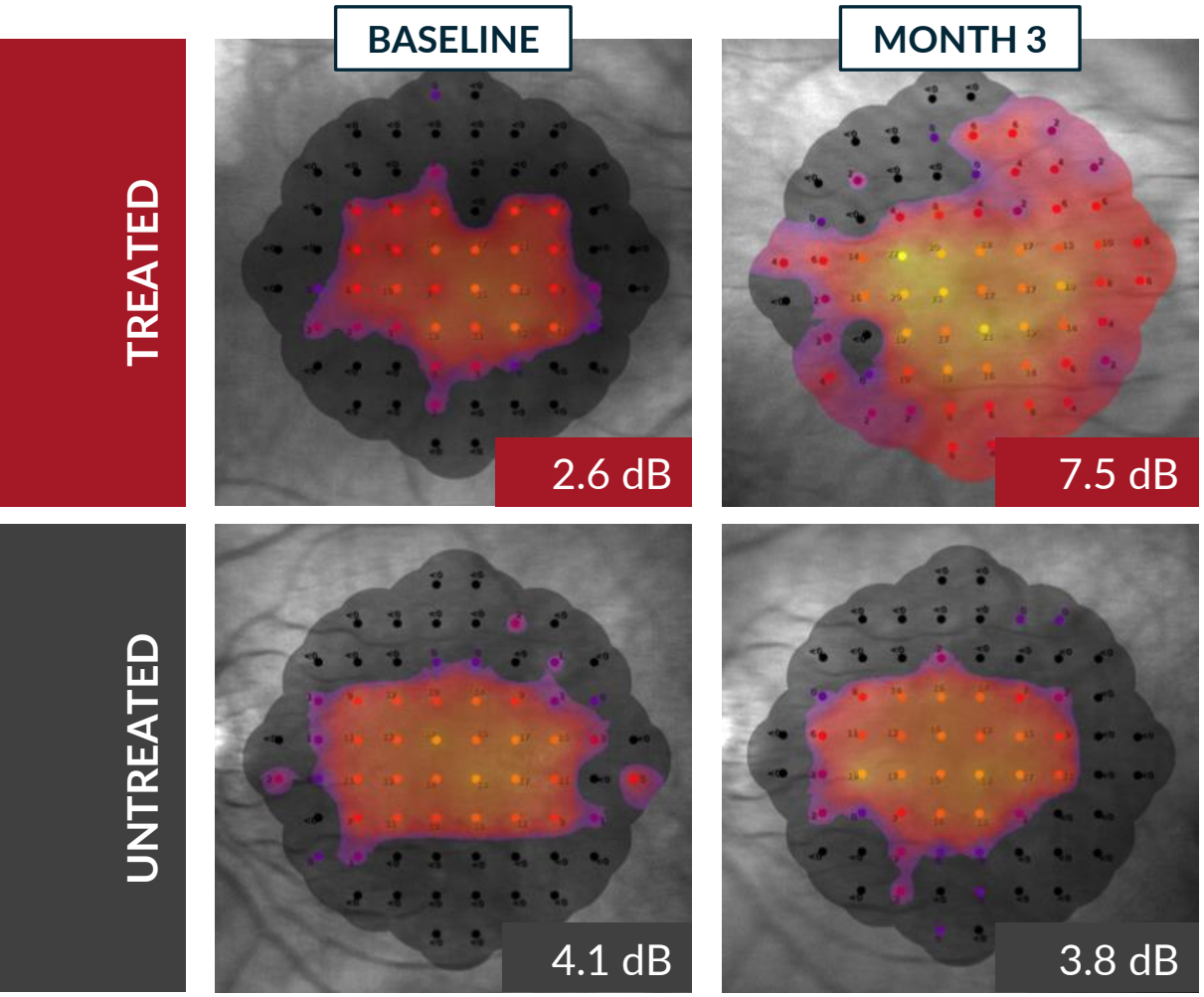
Six patients with several loci above 7dB also had significant improvements in overall sensitivity in the entire treated area compared to the untreated eye.

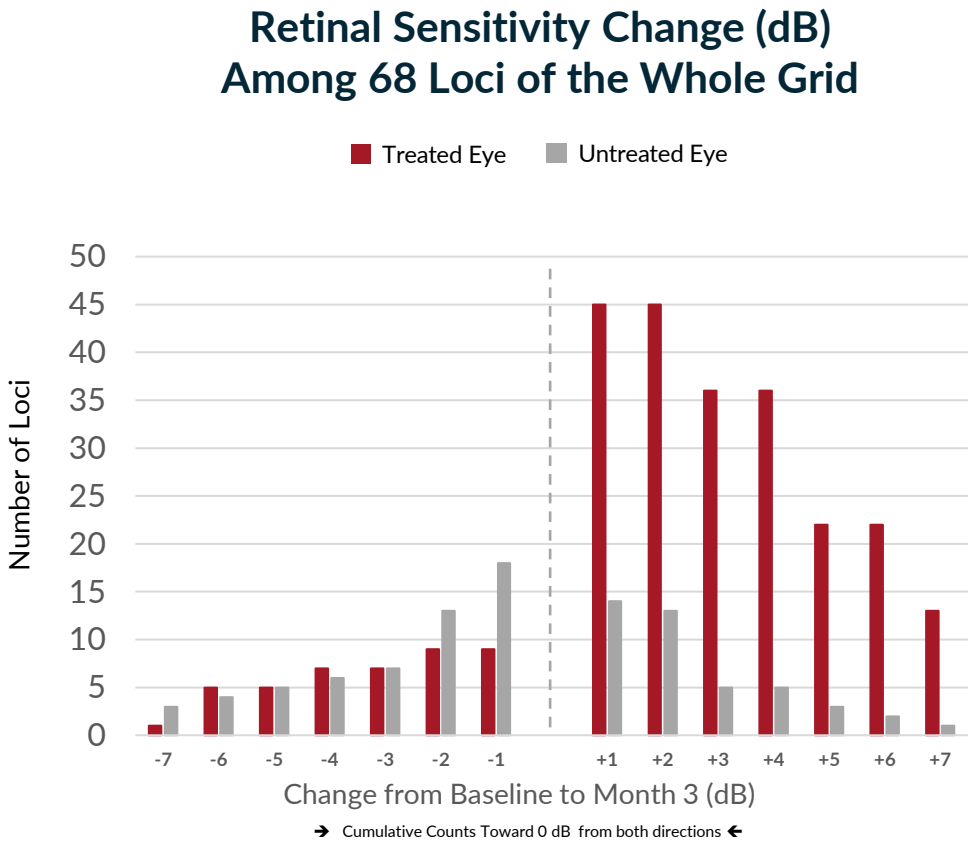
Change ≥7dB @ ≥5 Loci Within Grid		Mean Improvement Across Treated Area		Change ≥7dB @ Pre-Specified Loci	
Treated Eye	Untreated Eye	Treated Eye	Untreated Eye	Treated Eye	Untreated Eye
Yes—17	0	Yes—3.69	(0.61)	No—3	0
Yes—13	1	Yes—3.31	(0.25)	No—0	0
No—3	0	No—0.25	(1.22)	No—2	0
Yes—9	0	Yes—0.47	(1.46)	No—2	0
No—1	1	No—0.24	0.78	No—0	0
No—4	1	No—0.86	0.40	No—0	1
No—0	0	No—(3.62)	2.04	No—0	0
Yes—12	1	Yes—3.34	0.14	No—1	0
No—1	0	No—(3.30)	(2.10)	No—0	0
No		No		No	
Yes—9	1	Yes—1.59	(1.59)	No—2	0
Yes—13	0	Yes—2.62	(0.72)	No—4	0
No—2	0	No—(1.65)	(1.72)	No—0	0
6 responders		6 responders		0 responders	

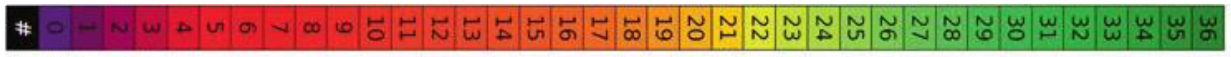
Very clear difference in loci response between treated and untreated eye.

Patient 2: Example of Patient with Improved Visual Sensitivity

Increase in both magnitude and AREA







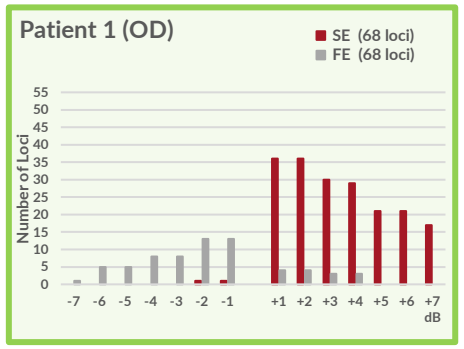
MAIA Color Scale (dB)

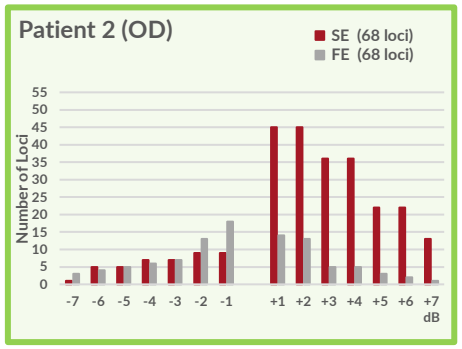
Retinal Sensitivity Change at Month 3 for the Whole Grid (dB)

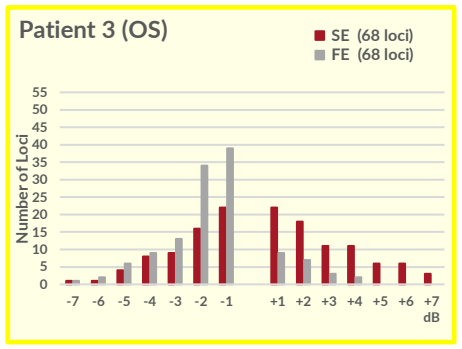
Double-cumulative histograms

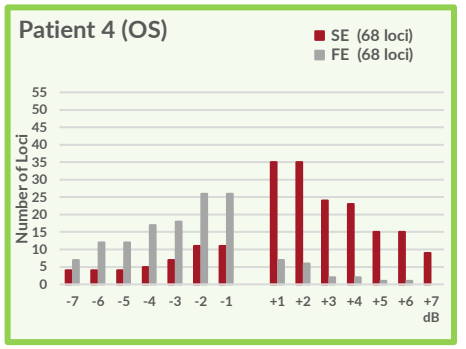
Treated eye (SE)

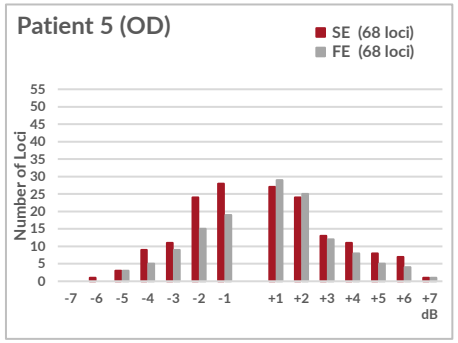
Untreated eye (FE)

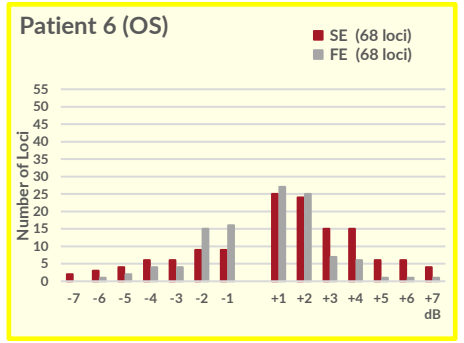


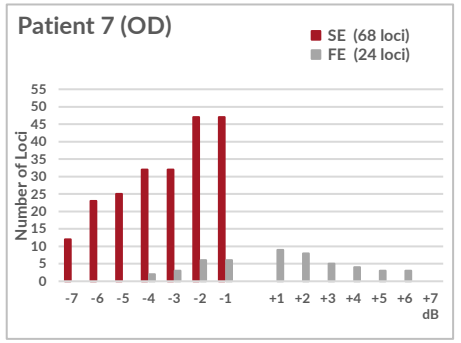


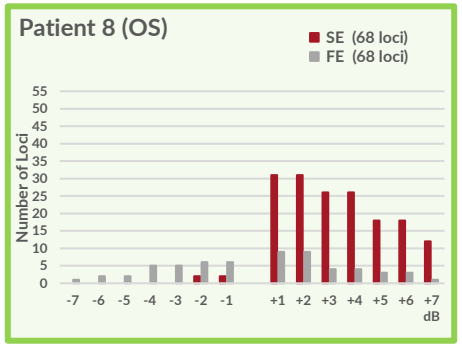


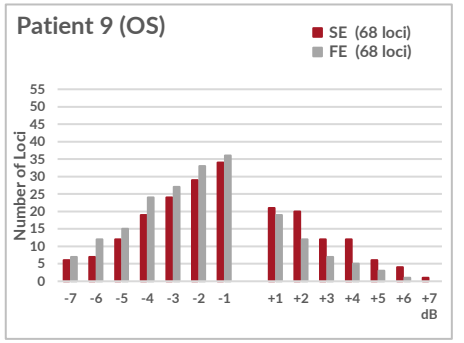


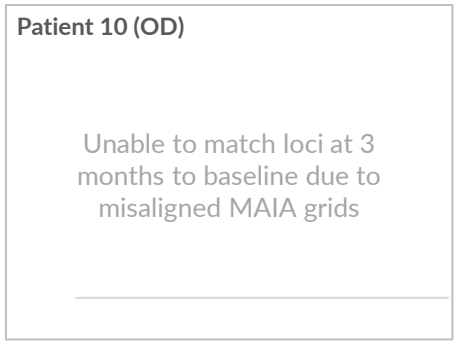


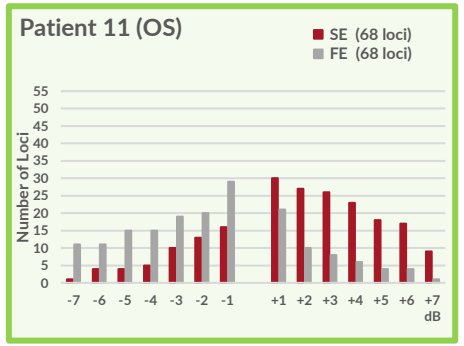


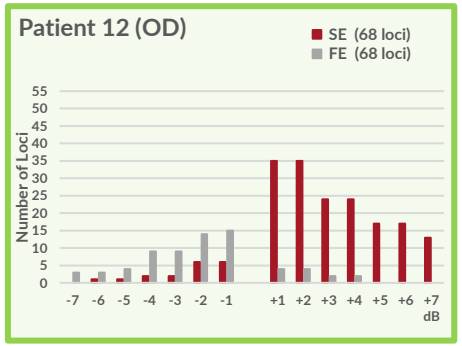


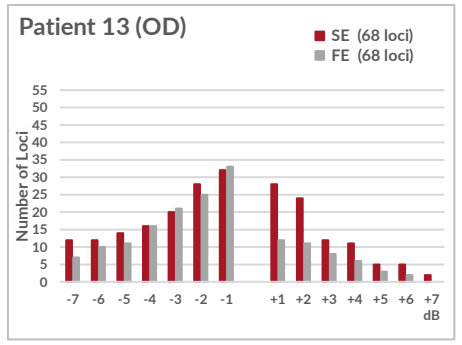












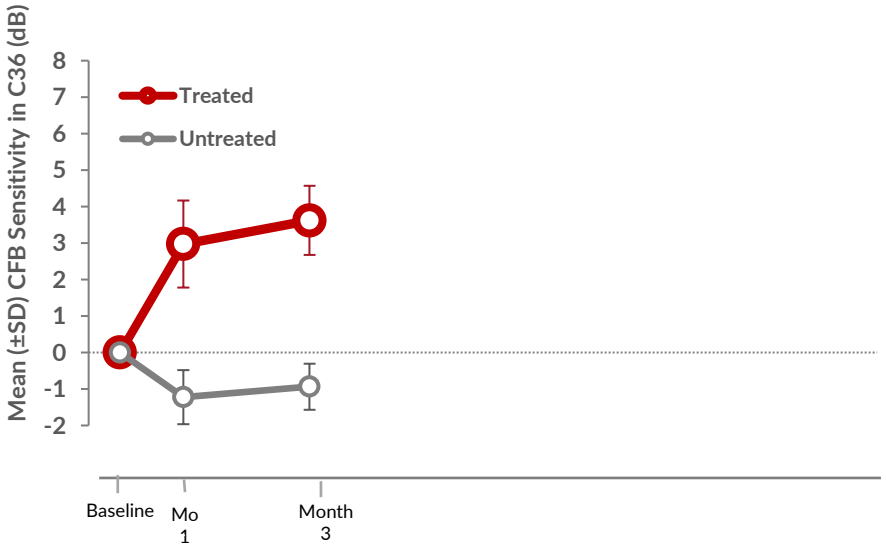
Six responders of 13 patients; 2 additional patients with positive changes.

Visual Sensitivity Change from Baseline Consistent Across Both Trials

Interim month 3 data for the sub-set of responders defined as patients with at least 5 loci improving by at least 7 decibels as measured by MAIA in both trials.

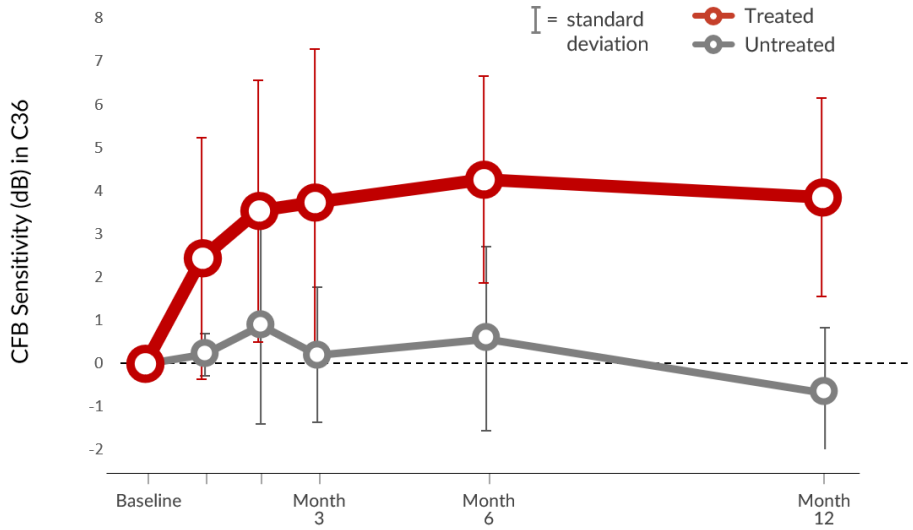
SKYLINE

Responders in both dose arms (n=6)



Phase 1/2

All responders, (n=6)

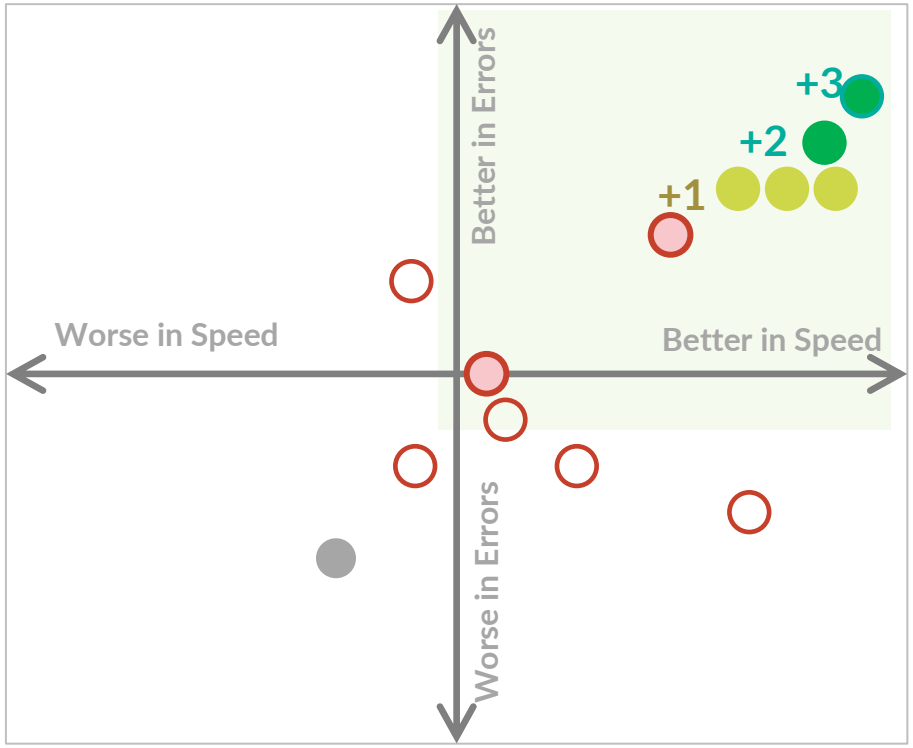


Both Skyline and the Phase 1/2 had similar and robust improvements in visual sensitivity.

Mobility Maze Data at Month 3

Seven patients had positive trends in improvement on speed and/or errors

Maze Challenge at Month 3



- A total of 7 (54%) patients showed some improvement in the maze challenge month 3:
  - 2 (15%) achieved the responder threshold of passing the maze with an improvement of 2 or more luminance levels
  - 3 (23%) improved 1 luminance level
  - 2 (15%) improved in speed without an increase of errors

Four of the six visual sensitivity responders also improved on the mobility maze.

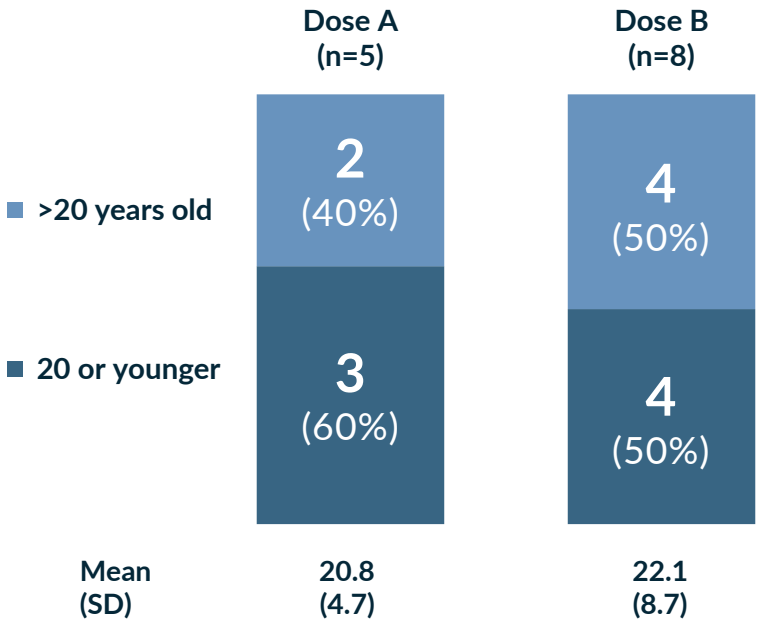
Patient Characteristics

Skyline trial demographics and baseline characteristics differ meaningfully from the original Phase 1/2 trial

Demographics

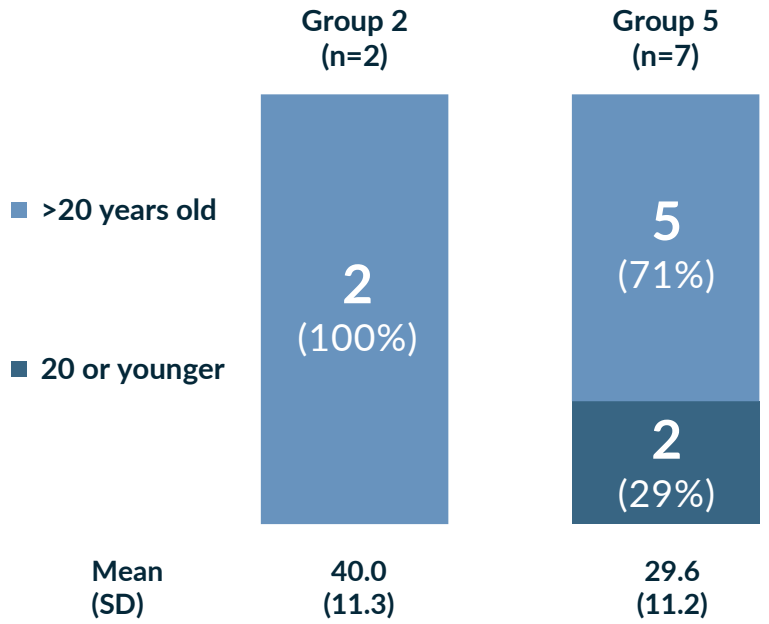
SKYLINE

- 100% males
- Ages 8 to 36 years old



PHASE 1/2

- 100% males
- Ages 19 to 48 years old



Baseline Characteristics

SKYLINE

	Dose A (N=5)		Dose B (N=8)		All (N=13)	
	SE*	FE#	SE	FE	SE	FE
BCVA ETDRS letters	67.4 (2.5)	72.8 (1.6)	66.5 (6.5)	71.1 (5.1)	66.8 (5.2)	71.8 (4.1)
Mean Sensitivity within bleb	5.2 (1.9)	5.1 (2.2)	4.4 (2.0)	4.3 (1.6)	4.7 (1.9)	4.6 (1.8)

PHASE 1/2

	Group 2 (n=2)		Group 5 (n=7)		Group 2+5 (N=9)	
	SE	FE	SE	FE	SE	FE
BCVA ETDRS letters	63.0 (1.4)	68.0 (1.4)	62.7 (7.5)	64.4 (9.7)	62.8 (6.5)	65.2 (8.5)
Mean Sensitivity within bleb	3.03 (n=1)	2.65 (n=1)	3.70 (2.42)	3.45 (2.36)	3.61 (2.25) (n=8)	3.35 (2.21) (n=8)

Skyline patients were younger with better baseline BCVA and better visual sensitivity than patients in the Phase 1/2 trial.

* SE – Study (treated) eye

FE - Fellow (untreated) eye

agtc

Safety Data

Favorable safety data

Safety Summary: Month 3 Interim Analysis

No clinically significant safety events related to study agent

- No SUSARs observed
- No endophthalmitis observed
- Majority of observed ocular AEs were non-serious
  - Favorable safety data in both dose groups and no apparent between dose difference
- 2 ocular SAEs were observed; neither related to study agent
  - Grade 3: A case of persistent decreased vision after surgery (related to surgery, not yet resolved)
  - Grade 3: A cased increased IOP (related to steroids, resolved with treatment)
- 1 non-ocular SAE observed
  - A case of asthma exacerbation (not-related, resolved)

Non-Serious Ocular AEs Related to Study Agent

All grade 2

MedDRA Preferred Term:	Dose A (N=5)	Dose B (N=8)	All Subjects (N=13)
Vitritis	1 (20%)	2 (25%)	3 (23%)
Eye pain	1 (20%)	0	1 (8%)

Ocular SAEs

None were determined to be related to study agent

Surgical and steroid issues are known and manageable

MedDRA Preferred Term:	Description:	Related to Study Agent	Related to Study Injection	Related to ConMed
IOP increased	Post-op D48, controlled with medications, resolved	No	No	**Yes** (Steroids)
Visual impairment	Borderline retinal structure at baseline, decrease in BCVA significant, resolving	No	**Yes**	No

Summary

Favorable interim safety data as of observed in both dose groups

Compelling improvements in visual sensitivity in Dose Group B observed at month 3 interim analysis

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    - Responders defined as patients with a 7 dB or greater improvement in at least 5 loci measured by MAIA microperimetry
  - Vista trial powered to be statistically significant for a 50% response rate
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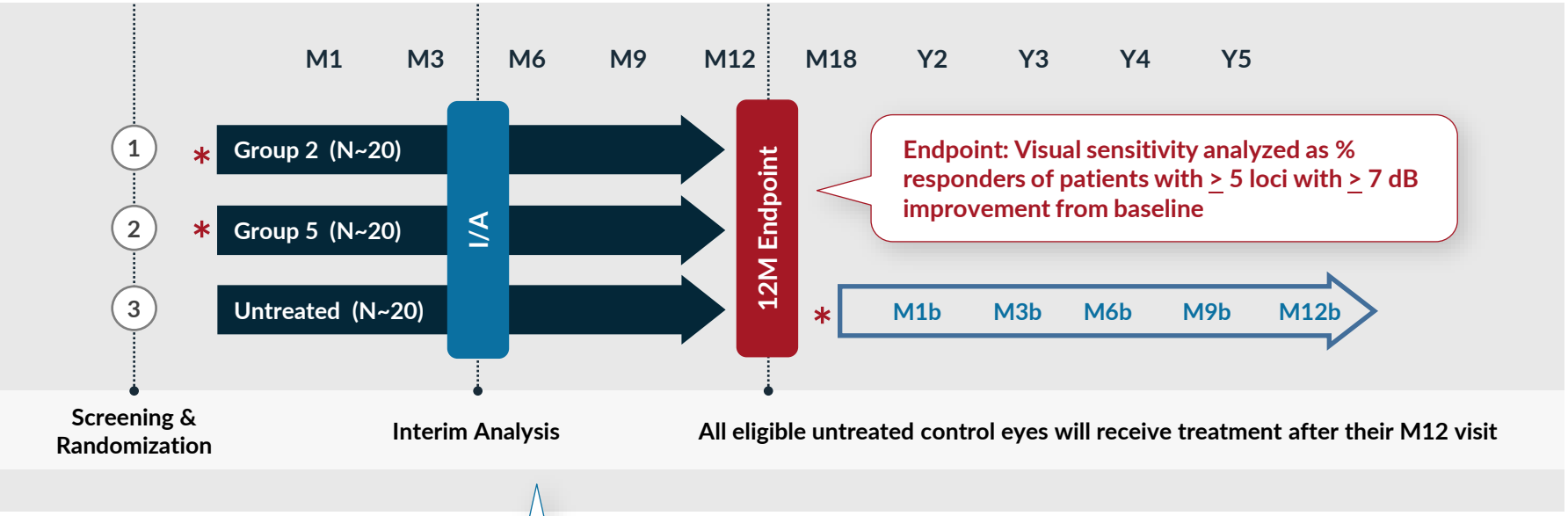
- Improvement trends in BCVA
  - Due to improved baseline for these patients, BCVA trends were less pronounced than in Phase 1/2
- Generally well tolerated
  - No clinically significant findings related to study agent

If Vista (Phase 2/3) data is consistent with Skyline and Phase 1/2 data, the Company intends to prepare a BLA filing.

* There were 5 patients in this group, but one patient was not evaluable.

Vista Clinical Trial

Phase 2/3 Trial Design: actively recruiting and pre-screening patients



At time of Vista interim analysis, we expect to also have M12 Skyline and M24 Phase 1/2 data to review with FDA to seek potential trial acceleration, including early dosing of second eye

- Two masked treatment arms and separate untreated control arm
- Pre-specified loci analysis will be incorporated as the primary endpoint in addition to other microperimetry assessments
- BCVA to continue as supportive secondary endpoint
- Ora-VNC™ mobility maze as additional supportive endpoint**
- Use of validated PRO survey

* Sub-retinal treatment

**Ora Visual Navigation Challenge (Ora-VNC™)

Question and Answer Period

Closing Remarks

Skyline Month 3 Interim Data Analysis

May 2022