XLRP Data Summary Month 12 High Dose Groups May 2021

Forward-Looking Statements

This presentation contains forward-looking statements that reflect AGTC's plans, estimates, assumptions and beliefs. Forward-looking statements include statements regarding the company's (i) clinical and pre-clinical programs, including the proposed Phase 2/3 (Vista) trial design for XLRP, the anticipated timeline for initiating and reporting data in the Vista trial and the Phase 1/2 expansion (Skyline) trial for XLRP, (ii) ability to enroll patients (ii) regulatory progress with FDA, (iii) potential growth and market opportunities, and (iv) ability to be competitive. 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," "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: AGTC cannot predict when or if it will obtain regulatory approval to continue to progress its clinical trials, commercialize a product candidate or receive reasonable reimbursement; uncertainty inherent in clinical trials and the regulatory review process; risks and uncertainties associated with drug development and commercialization; risks related to the COVID-19 outbreak that may delay clinical trial enrollment; gene therapy is still novel with only a few approved treatments so far; 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 the Company's most recent Annual Report on Form 10-K and subsequent periodic reports filed with the SEC. Given these uncertainties, you should not place undue reliance on these forward-looking statements, which speak only as of the date of this presentation. Except as required by law, AGTC assumes no obligation to update or revise these forward-looking statements, whether as a result of new information, new events or otherwise.

Attendees and Agenda

- Attendees
  - Sue Washer CEO
  - Matthew Feinsod, Executive VP of Global Strategy and Development
  - Mark Shearman CSO
- Agenda
  - Introductions and Overview
  - Update on XLRP product candidate clinical development
  - Q&A
  - Closing Remarks

Key Takeaways

- Group 5 & Group 6 doses have responders at Month 12
  - 50% response rate (4/8) in patients who met Phase 2/3 inclusion criteria
    - Vista trial was powered to be statistically significant for a 50% response rate
  - One Group 5 patient who was a responder at Month 6, not a responder at Month 12
- BCVA continues to show supportive evidence of a biological response at Month 12
  - Clear difference between treated eye and untreated eye
- Early-look data shows two Group 4 patients with continued response at Month 24
  - Evidence for continued durability of response to treatment
  - Only three patients have reached Month 24; third patient was never a responder

Updated Data for New Interim Analysis

All Evaluable Centrally-Dosed Patients

Dose Group	C36 Change ≥7db @ ≥5 Loci at Month 6	Number of Responders	C36 Change ≥7db @≥5 Loci at Month 12	Number of Responders
2#	Yes	1/1	No	0/1
4	No	2/7	No	2/7
	Yes		Yes	
	No		No	
	No		No	
	Yes		Yes	
	No		No**	
	No		No	
5	No	3/7	No	2/7
	Yes		No	
	Yes		Yes	
	No**		No**	
	Yes		Yes	
	No		No	
	No		No	
6	No**	2/4	No**	2/4
	No**		No**	
	Yes		Yes	
	Yes		Yes	

**One patient does not meet new inclusion criteria; so responders are 2/7 or 28%

Additional two patients do not meet strict criteria but treated eye is statistically better than untreated eye

**Three patients do not meet new inclusion criteria; so responders are 4/8 or 50%

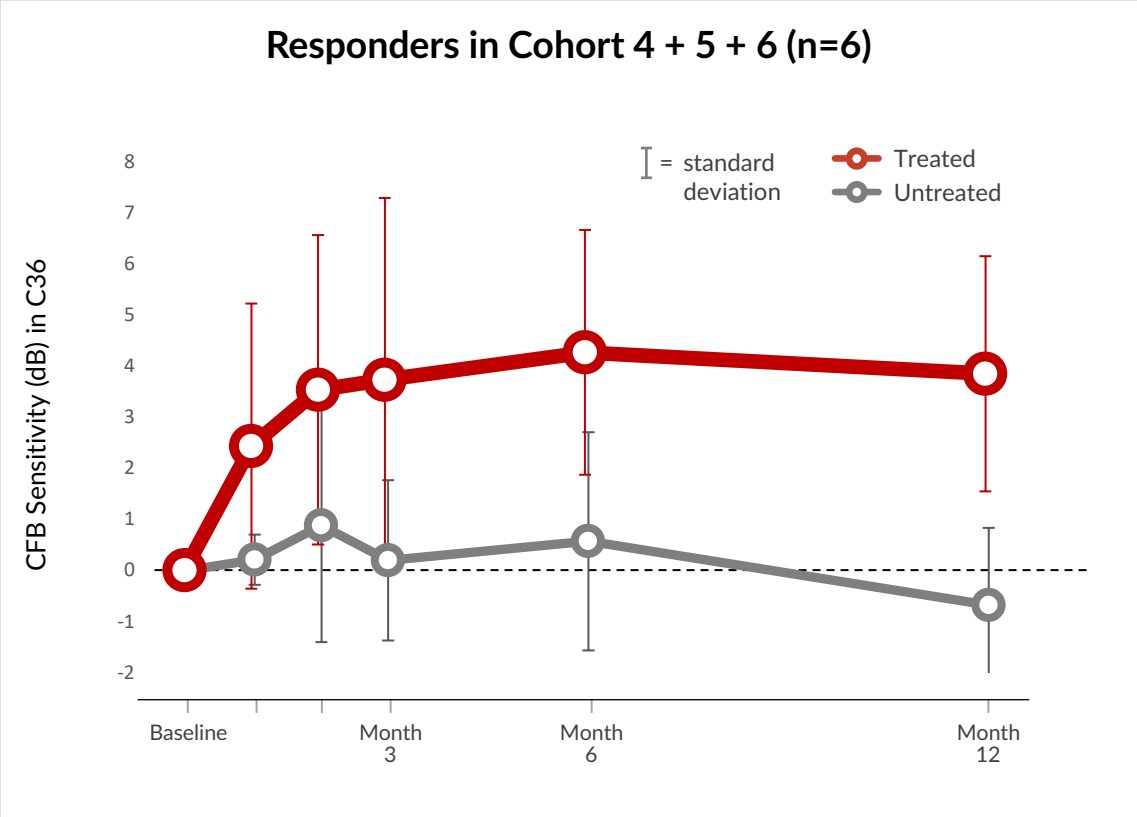
Additional patient does not meet strict criteria but treated eye is statistically better than untreated eye

There is an additional Group 2 patient with no microperimetry data available

Microperimetry – Six Responders at Month 12

Groups 4-6

Increased mean sensitivity relative to baseline across **the central 36 loci**



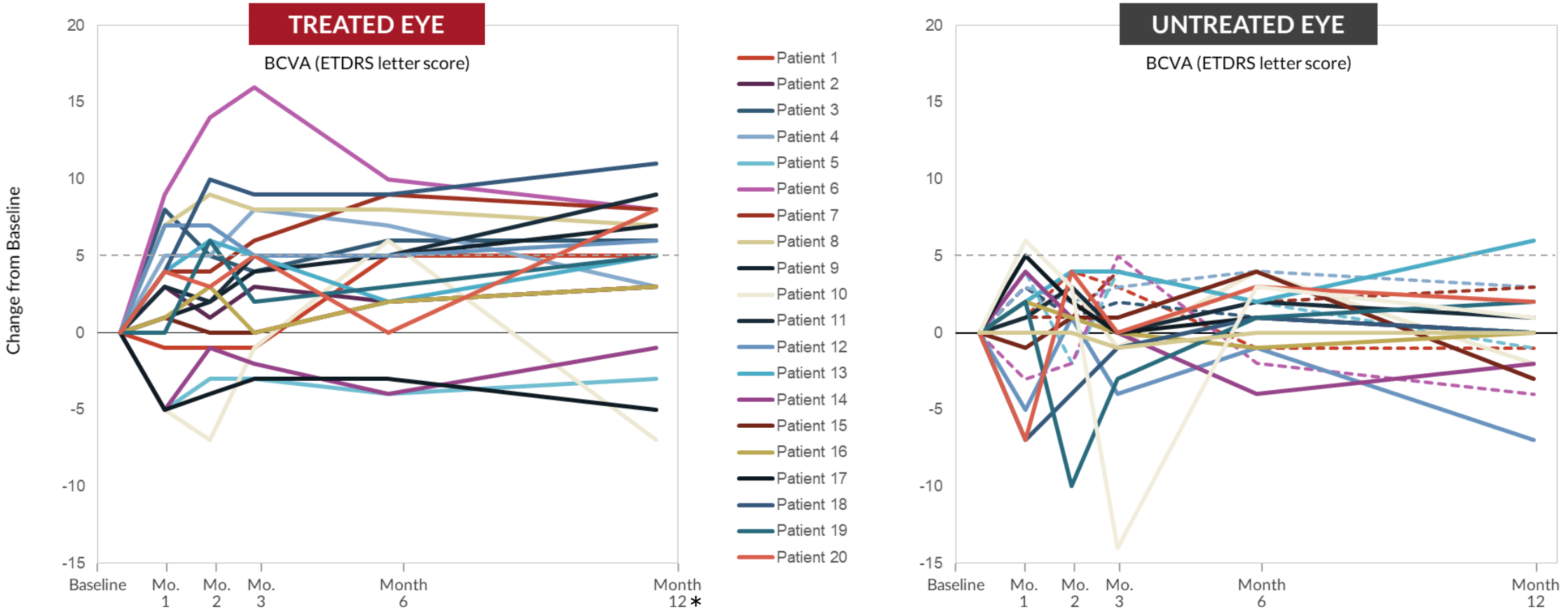
Responder identified as patient with ≥ 7 dB improvement in sensitivity at ≥ 5 loci in central 36 loci of perimetry grid at Month 12

Patients 4, 8, 12, 14, 19 & 20

BCVA – Individual Patient Data at Month 12

All Groups, N=20

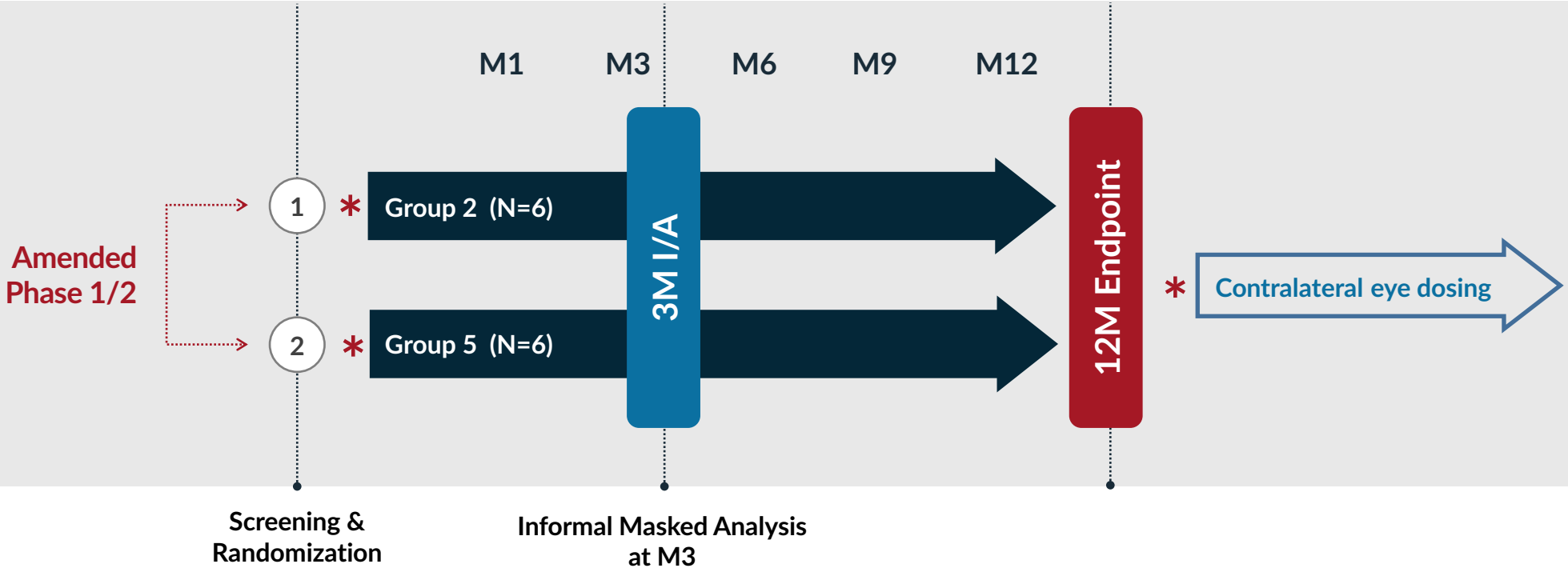
Supportive evidence of statistically significant improved visual acuity across all centrally-dosed groups



* 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)

Skyline Clinical Trial

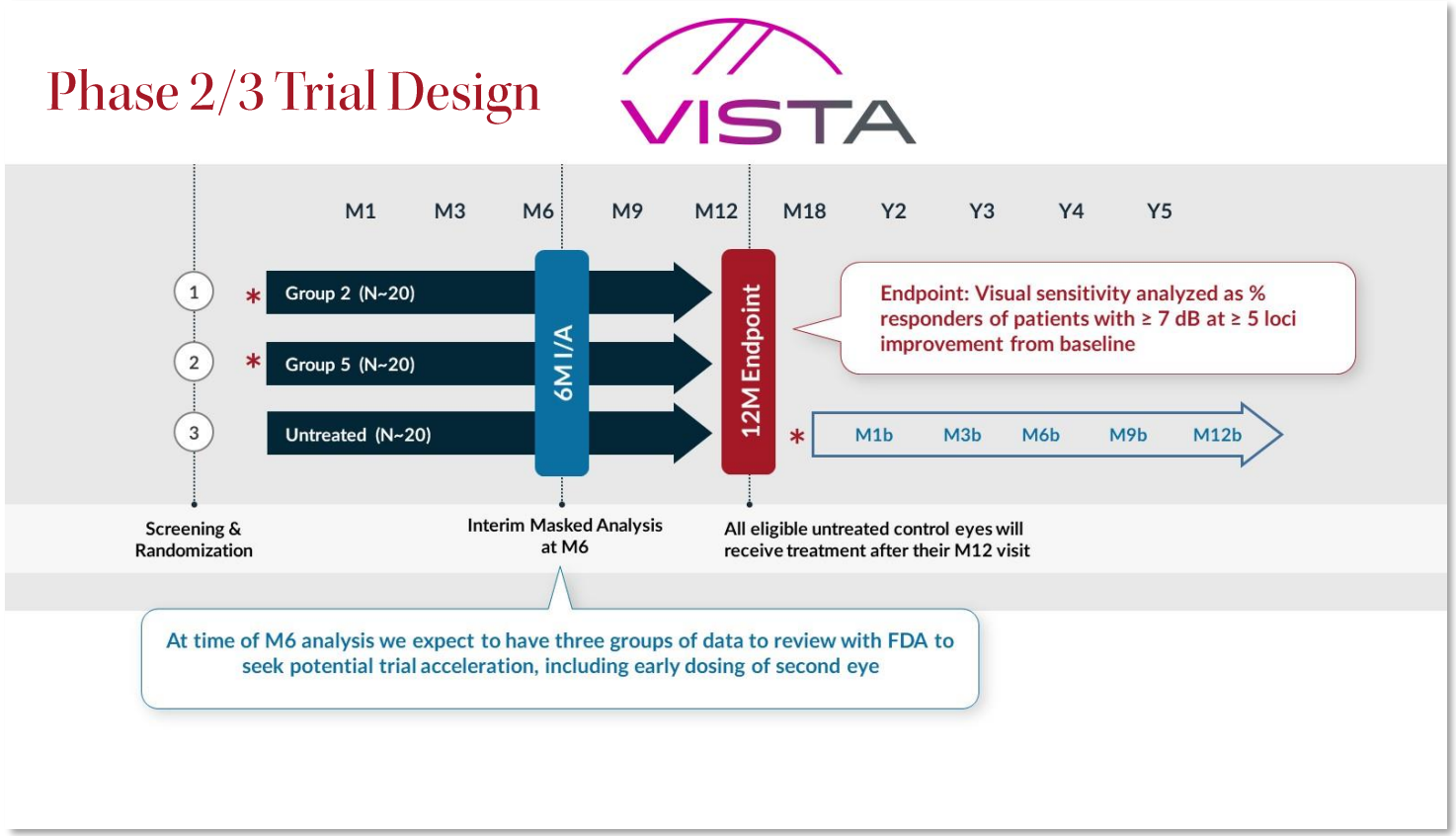
Phase 1/2 Expansion. First path to verify correlation of visual sensitivity changes to mobility maze outcomes and to maintain patient & site engagement



* Sub-retinal treatment

Vista Clinical Trial

Phase 2/3 Trial Design



- 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

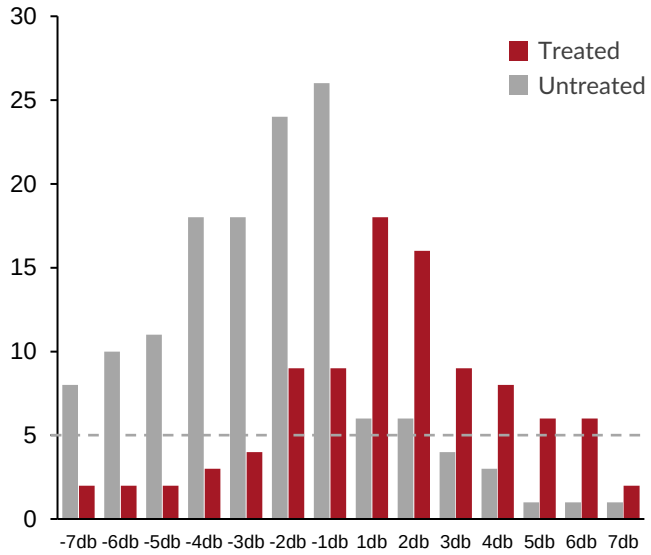
**Ora Visual Navigation Challenge (Ora-VNC™)

Patient-by-Patient Data for Responders– Group 5, Month 12

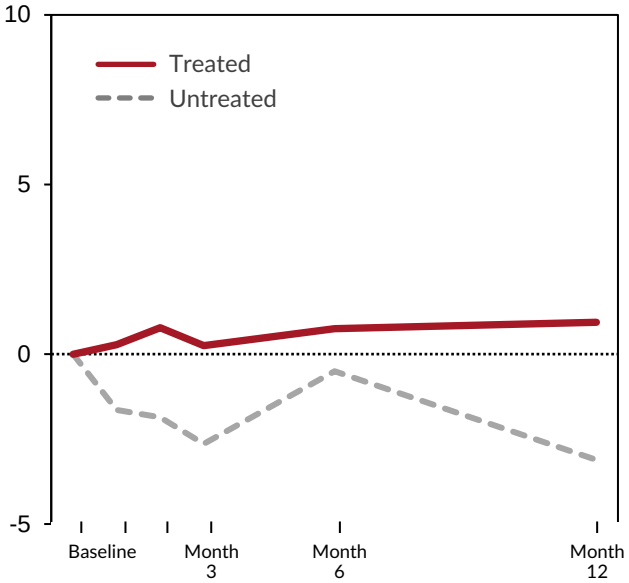
Patient 10

Statistical Improvement by K-S Test*

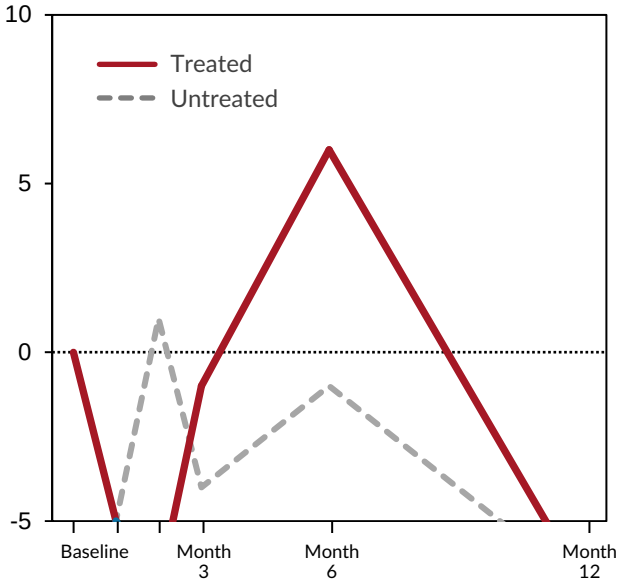
Retinal Sensitivity Change (db)



C36 Retinal Sensitivity Change (db)



Visual Acuity Change (ETDRS letters)



Age	Study Eye	Baseline VA	Baseline Sensitivity
19	**OD**	**OD: 68** OS: 75	**Treated: 6.4 dB** Untreated: 7.8 dB

* P = 0.00009 at Month 12

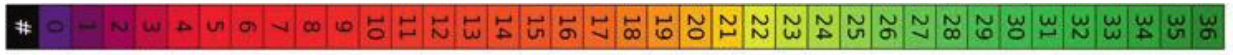
Patient numbering based on chronology of dosing

agtc

Patient 10

Statistical Improvement by K-S Test

	BASELINE	MONTH 3	MONTH 6	MONTH 12
TREATED	6.37 dB	7.09 dB	6.54 dB	7.20 dB
UNTREATED	7.81 dB	6.63 dB	7.72 dB	6.50 dB



MAIA Color Scale (dB)

Bleb covered central area apart from area on far right

Mean Sensitivity within Bleb

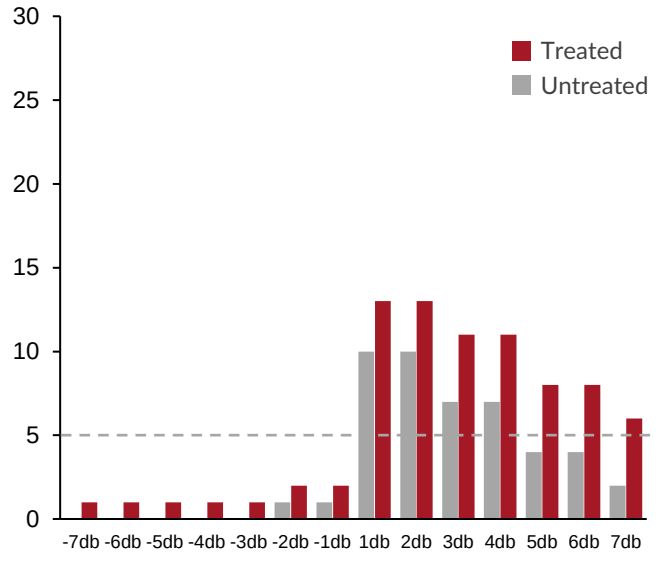
Patient numbering based on chronology of dosing

agtc

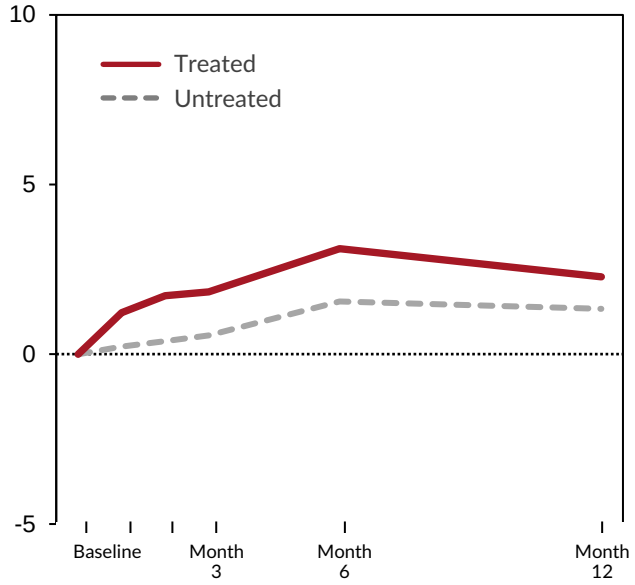
Patient 12

RESPONDER

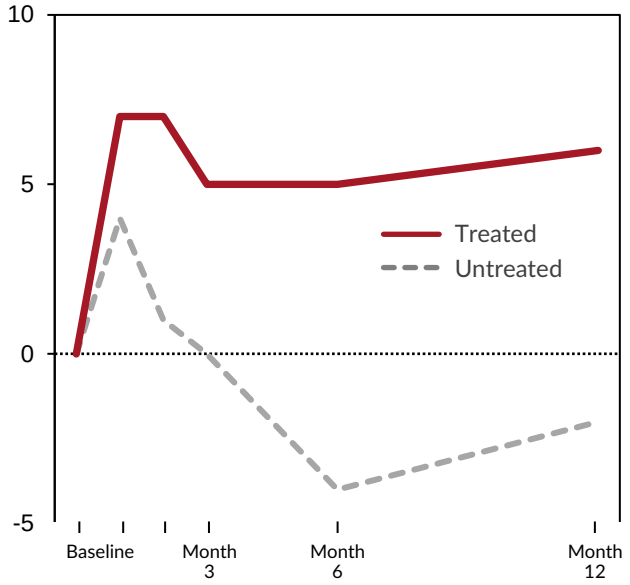
Retinal Sensitivity Change (db)



C36 Retinal Sensitivity Change (db)



Visual Acuity Change (ETDRS letters)



Age	Study Eye	Baseline VA	Baseline Sensitivity
45	**OS**	OD: 57 **OS: 59**	**Treated: 3.0 dB** Untreated: 2.5 dB

Patient numbering based on chronology of dosing

agtc

Patient 12

RESPONDER

	BASELINE	MONTH 3	MONTH 6	MONTH 12
TREATED	3.03 dB	4.45 dB	5.61 dB	4.52dB
UNTREATED	2.53 dB	2.67 dB	4.07 dB	3.60 dB

Bleb covered top right half

MAIA Color Scale (dB)

Mean Sensitivity within Bleb

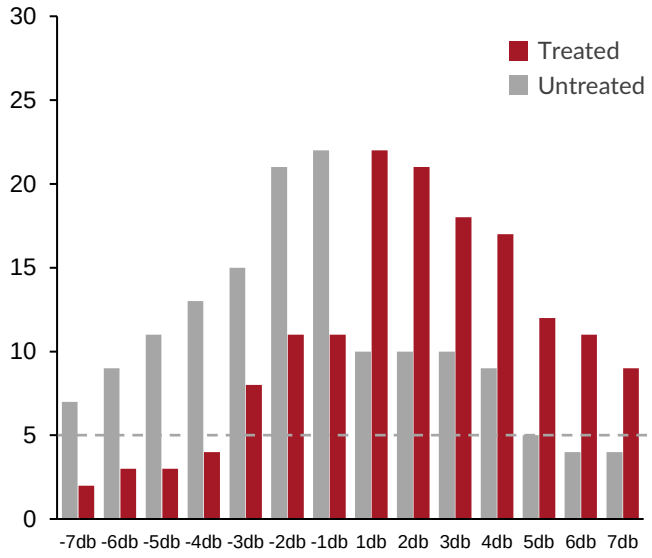
Patient numbering based on chronology of dosing

agtc

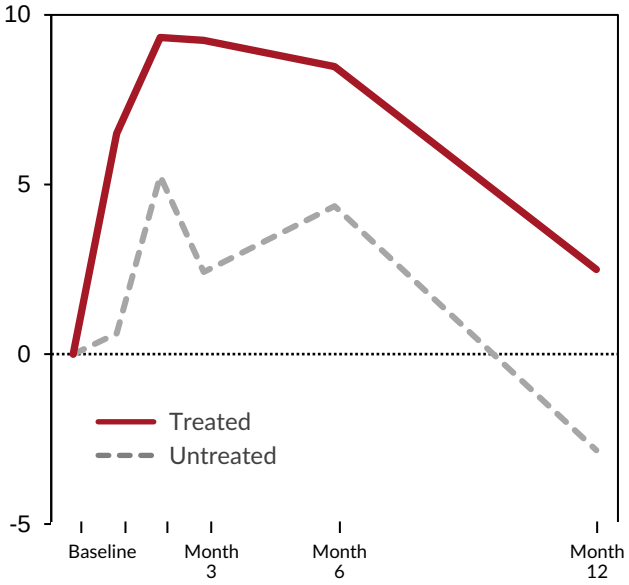
Patient 14

RESPONDER

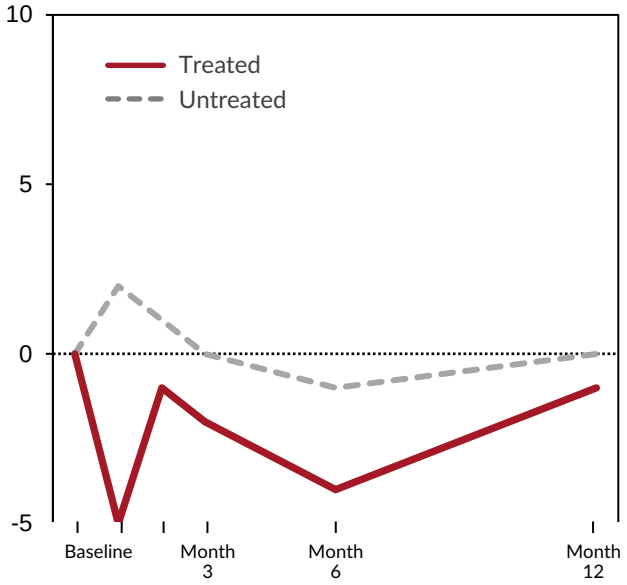
Retinal Sensitivity Change (db)



C36 Retinal Sensitivity Change (db)



Visual Acuity Change (ETDRS letters)



Age	Study Eye	Baseline VA	Baseline Sensitivity
21	**OD**	**OD: 67** OS: 70	**Treated: 6.4 dB** Untreated: 6.2 dB

Patient 14

RESPONDER

	BASELINE	MONTH 3	MONTH 6	MONTH 12
TREATED	6.44 dB	16.03 dB	15.09 dB	7.96 dB
UNTREATED	6.19 dB	8.03 dB	9.81 dB	3.65 dB

Bleb covered entire area

MAIA Color Scale (dB)

Mean Sensitivity within Bleb

Patient numbering based on chronology of dosing

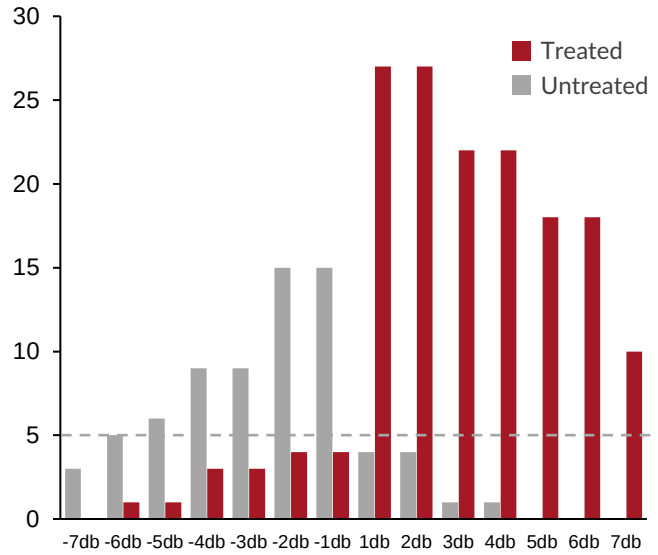
agtc

Patient-by-Patient Data for Responders– Group 6, Month 12

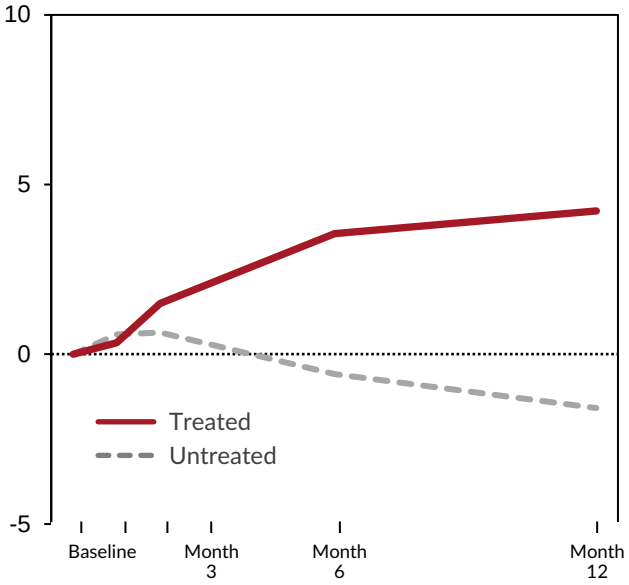
Patient 19

RESPONDER

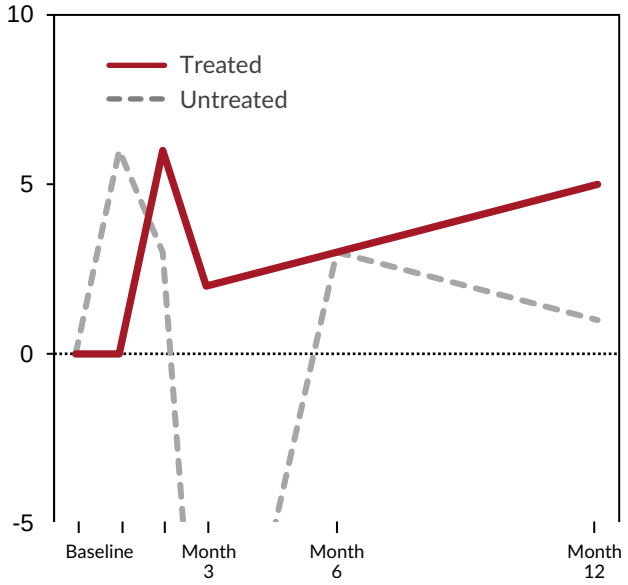
Retinal Sensitivity Change (db)



C36 Retinal Sensitivity Change (db)



Visual Acuity Change (ETDRS letters)



Age	Study Eye	Baseline VA	Baseline Sensitivity
19	**OD**	**OD: 70** OS: 71	**Treated: 2.9 dB** Untreated: 3.4 dB

Patient 19

RESPONDER

	BASELINE	MONTH 2	MONTH 6	MONTH 12
TREATED	2.88 dB	3.74 dB	5.24 dB	6.15 dB
UNTREATED	3.37 dB	3.46 dB	2.75 dB	2.16 dB

Bleb covered entire area

MAIA Color Scale (dB)

Mean Sensitivity within Bleb

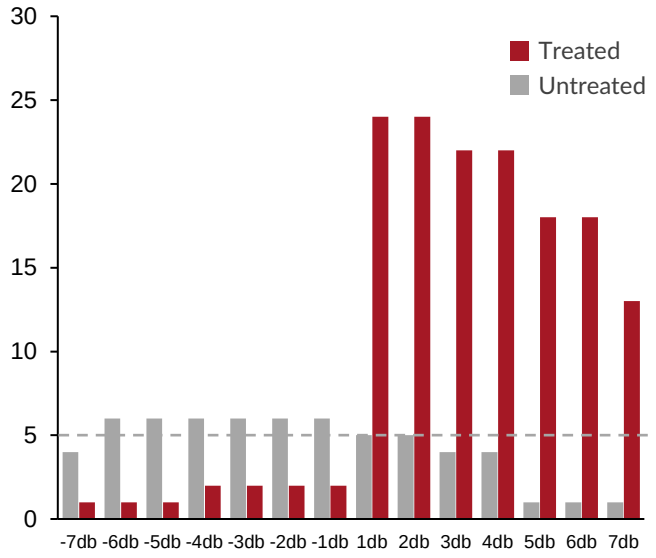
Patient numbering based on chronology of dosing

agtc

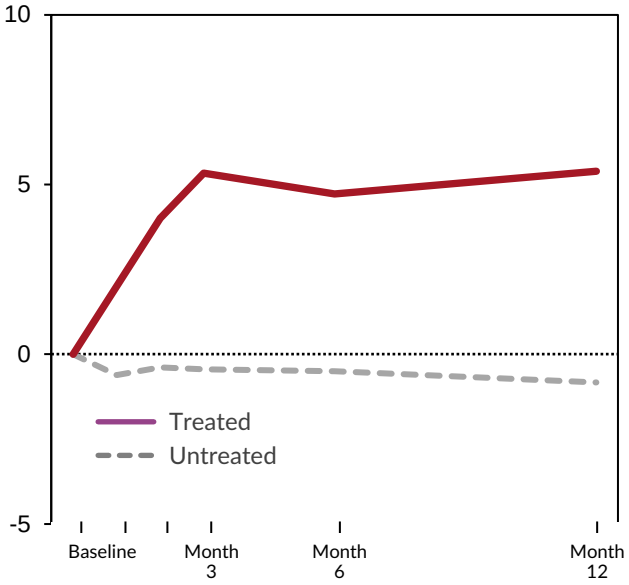
Patient 20

RESPONDER

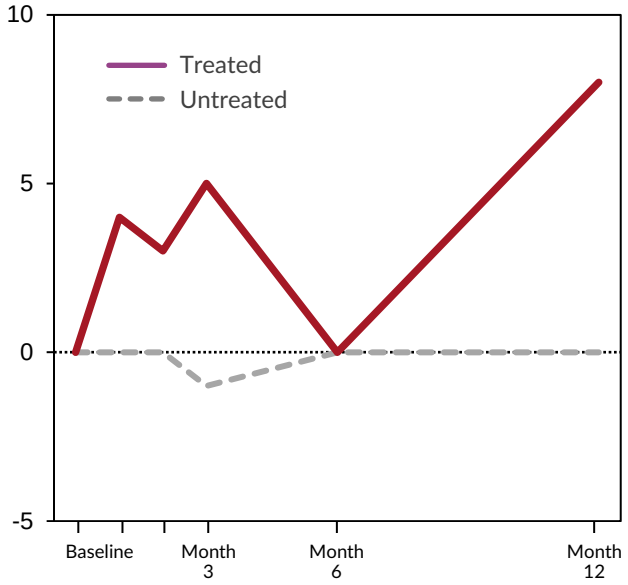
Retinal Sensitivity Change (db)



C36 Retinal Sensitivity Change (db)



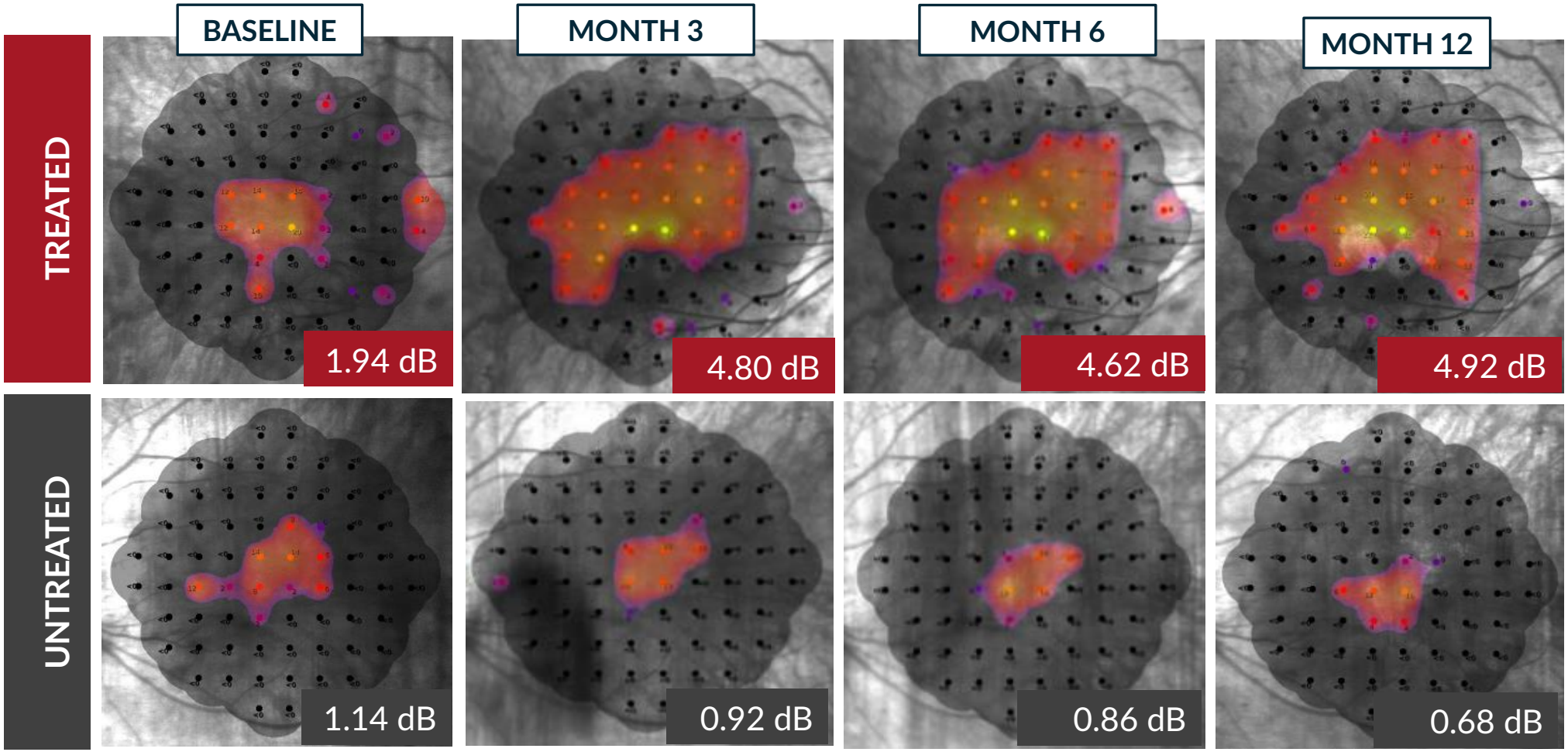
Visual Acuity Change (ETDRS letters)



Age	Study Eye	Baseline VA	Baseline Sensitivity
30	**OD**	**OD: 64** OS: 70	**Treated: 1.9 dB** Untreated: 1.1 dB

Patient 20

RESPONDER



Bleb covered entire area

Improvements in the ***intensity*** and ***area of sensitivity*** for the treated eye (top row) versus untreated eye (bottom row); sensitivity values are from within bleb

Mean Sensitivity within Bleb

Patient numbering based on chronology of dosing

Early Look Patient Data – Group 4, Month 24

Durability of Response Beyond One Year

Group 4

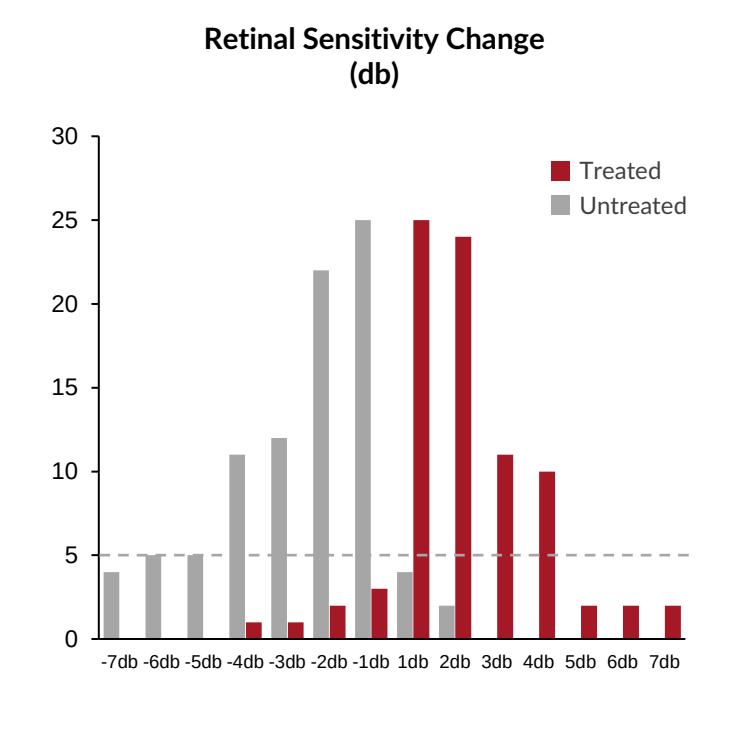
- Three Group 4 patients have reached Month 24, two maintain a durable effect
  - Patient 3 showed statistically different profiles in treated eye versus untreated eye
  - Patient 4 satisfied 7/5 criteria
  - The third patient was not a responder at Month 12

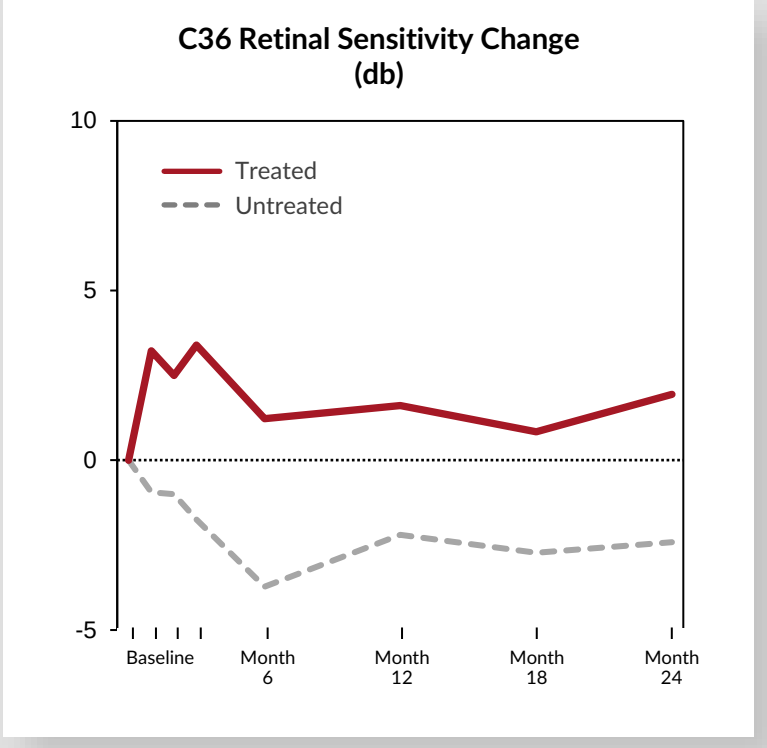
Subject	Repeatability Coefficient C36	Delta24M	Month 24 Responder
Patient 3	2.99	4.36111	Y
Patient 4	2.99	4.80556	Y

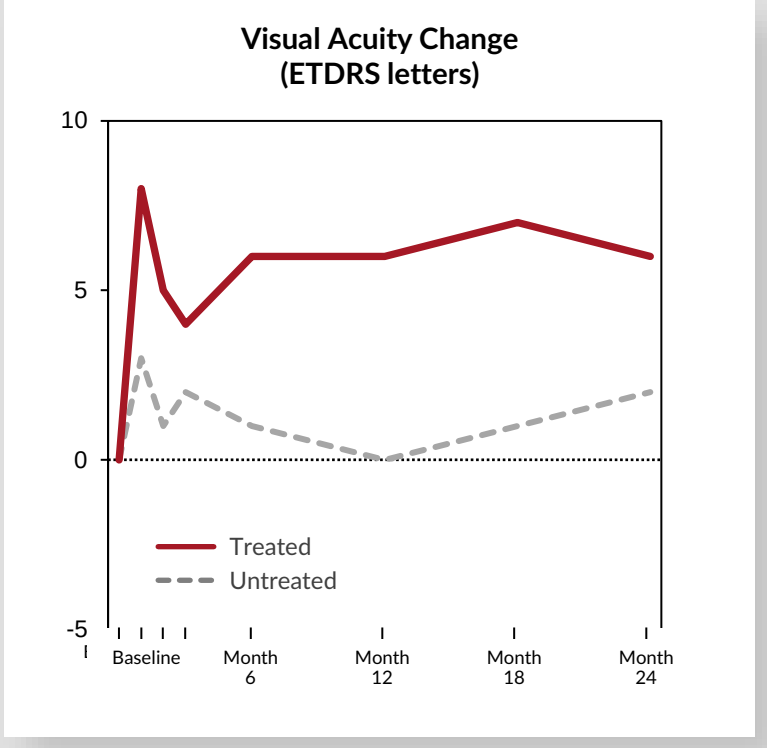
Such continued durability data has yet to be reported by XLRP competitors

Patient 3

Statistical Improvement by K-S Test*







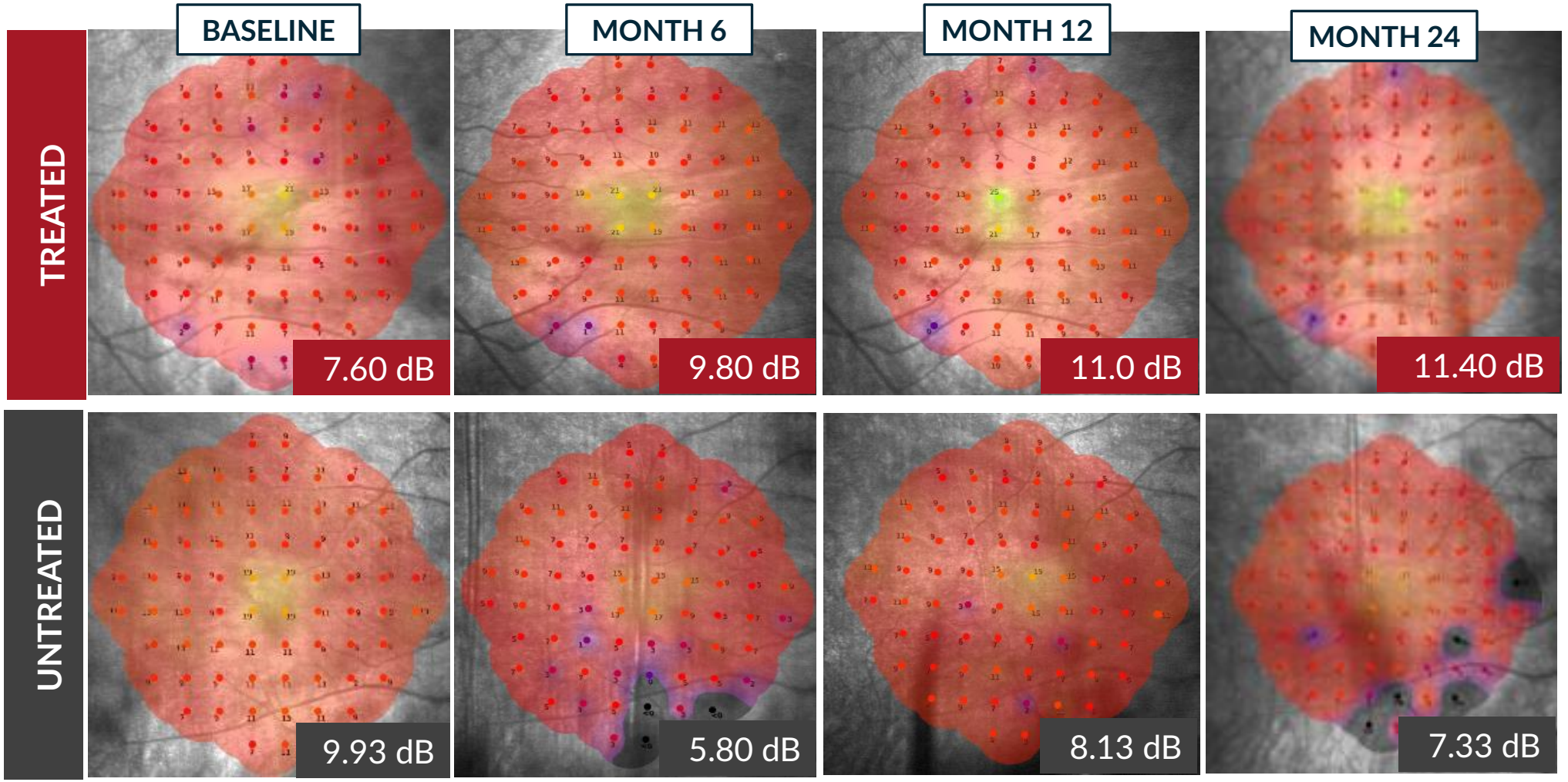
Age	Study Eye	Baseline VA	Baseline Sensitivity
18	**OS**	OD: 67 **OS: 49**	**Treated: 7.6 dB** Untreated: 9.9 dB

* P = 0.00065 at Month 12

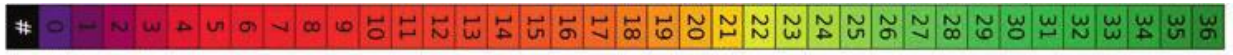
Patient numbering based on chronology of dosing

Patient 3

Statistical Improvement by K-S Test



Bleb covered bottom right



MAIA Color Scale (dB)

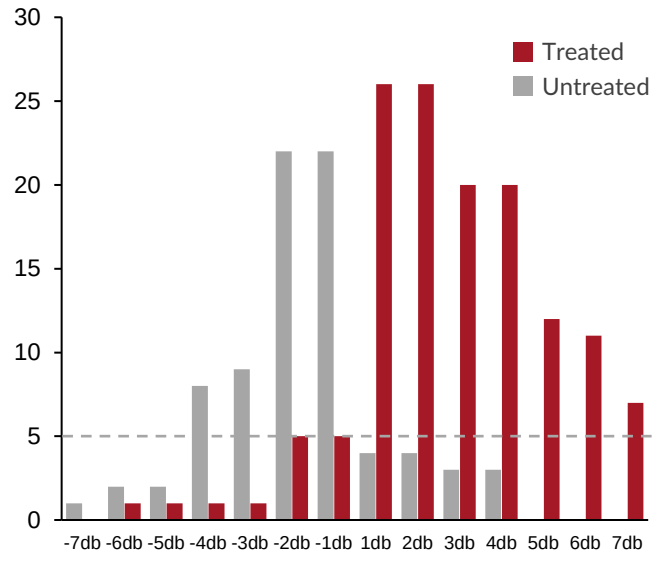
Mean Sensitivity within Bleb

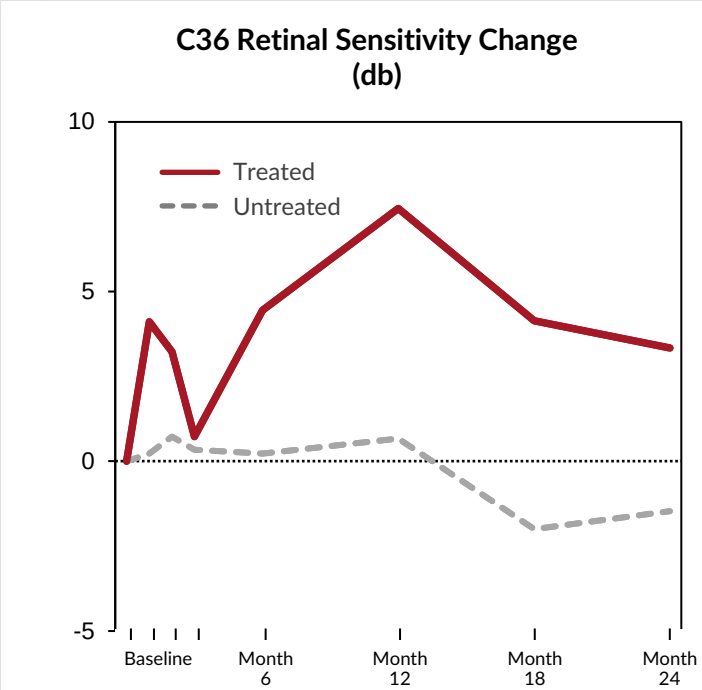
Patient numbering based on chronology of dosing

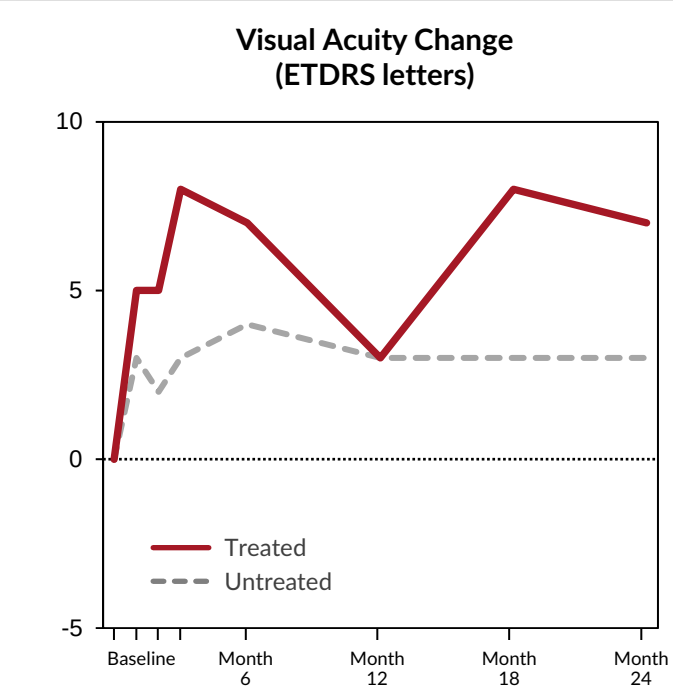
agtc

Patient 4

RESPONDER





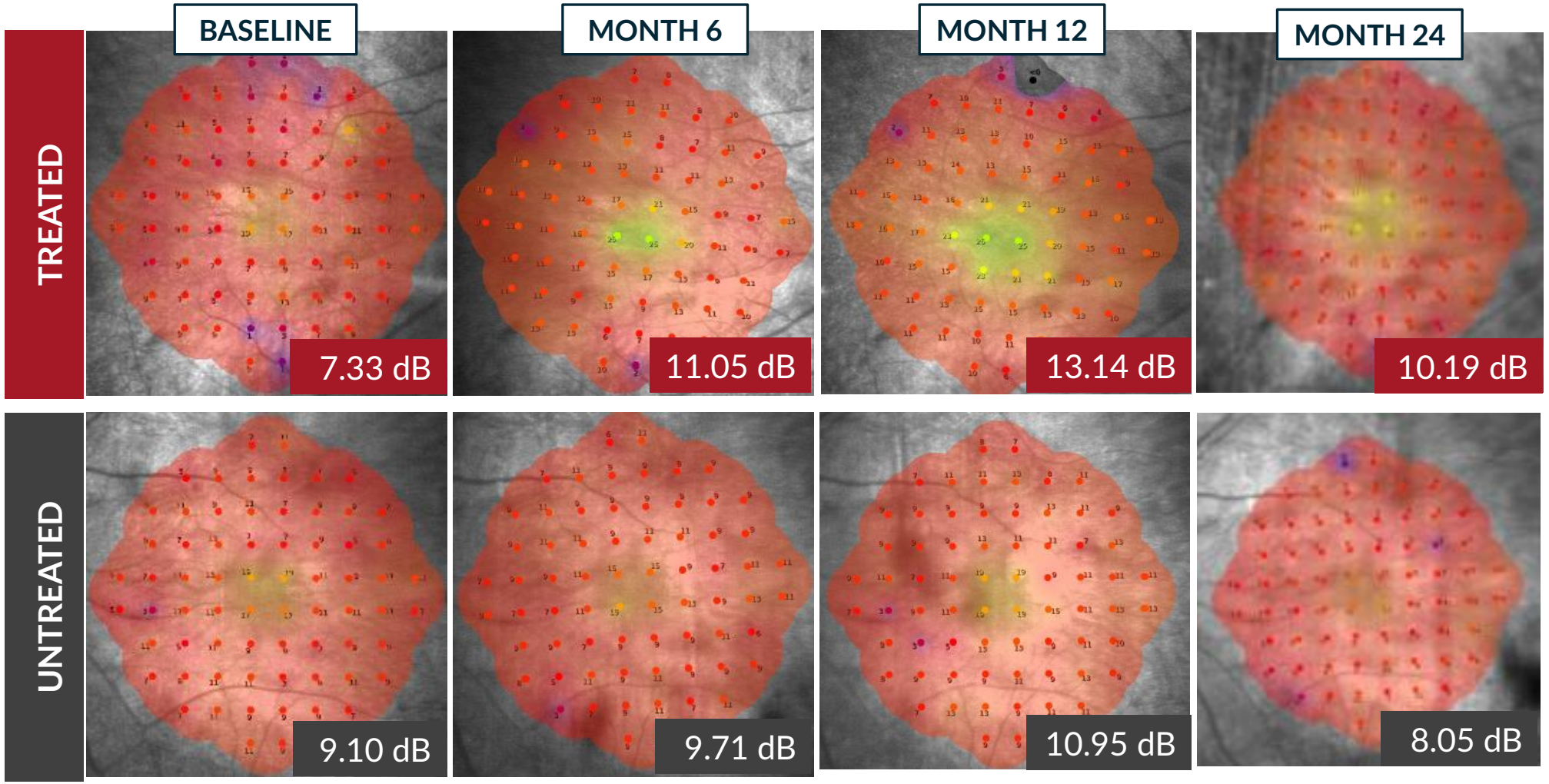


Age	Study Eye	Baseline VA	Baseline Sensitivity
18	**OD**	**OD: 62** OS: 66	**Treated: 7.3 dB** Untreated: 9.1 dB

Patient numbering based on chronology of dosing

Patient 4

RESPONDER



Bleb covered middle left

Mean Sensitivity within Bleb

Patient numbering based on chronology of dosing

agtc

Key Takeaways

- Group 5 & Group 6 doses have responders at Month 12
  - 50% response rate (4/8) in patients who met Phase 2/3 inclusion criteria
    - Vista trial was powered to be statistically significant for a 50% response rate
  - One Group 5 patient who was a responder at Month 6, not a responder at Month 12
- BCVA continues to show supportive evidence of a biological response at Month 12
  - Clear difference between treated eye and untreated eye
- Early-look data shows two Group 4 patients with continued response at Month 24
  - Evidence for continued durability of response to treatment
  - Only three patients have reached Month 24; third patient was never a responder

Upcoming XLRP Clinical Milestones

- Present 12-month trial results from the Phase 1/2 at AAO in November 2021
  - Dr. Robert Sisk, Cincinnati Eye Institute
- Provide Skyline trial results from the 3-month masked interim analysis in 4Q 2021
- Provide Skyline trial results from the 12-month data in 3Q 2022
- Provide Vista trial results from the 6-month masked interim analysis in 4Q 2022

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