



Bota-vec for the treatment of X-Linked Retinitis Pigmentosa (XLRP)

- Phase 3 complete (n=95)
- Phase 3 conducted at 32 of the leading global sites for Inherited Retinal Disease (IRD) in North America and Europe
- Granted PRIME and Fast Track designations, and US and EU Orphan Drug Designations
- BLA and MAA filing preparation under way



Bota-vec Opportunity Overview: High-Value, De-Risked Program with Potential Near-Term Approval and Launch



Totality of Phase 3 data demonstrates benefit of treatment

- Significant improvements in each of the domains of vision: **functional vision, retinal function and visual function**
- Multiple statistically significant secondary endpoints demonstrate clinically meaningful benefit
- Convergent positive signals across objective (LLVA, perimetry) and subjective (PROs) measures
- Safety profile expected and manageable across both Phase 1 and Phase 3 studies (n= 137) with further improved immune safety demonstrated in the Phase 3 (n=95)



High Unmet Need

- Bota-vec addresses a severe unmet need with no approved disease-modifying therapies
- Demonstrates improvement in each domain of vision in an otherwise inexorably degenerative disease resulting in complete blindness
- Patients report life changing impact of therapy resulting from meaningful improvement in vision.



Commercial Potential

- Significant market >20,000 US and EU
- **~ \$1.7 billion* projected peak sales and ~ \$7.7 billion** cumulative sales over 10 years**
- Low cost of goods (<\$10k per patient) high margin product
- **Strong launch anticipated** ~ several hundred patients identified for treatment across a small number of centers of excellence
- 32 of these leading sites participated in the global Phase 3. >60 surgeons trained for optimal delivery.
- And several hundred patients are known to be waiting for treatment



Regulatory Pathways

- Consistent, highly statistically significant, clinically meaningful Phase 3 data across secondary endpoints supports positive approvability dialogue
- **EMA:** MeiraGTx intends to file MAA based on totality of data; EU PRIME and ODD designations
- **FDA:** MeiraGTx intends to return to FDA to discuss the potential route to approval
- Precedent for totality-of-evidence with a trend in primary approvals in rare genetic diseases and previous IRD treatment, EU and US
- Strong support and engagement from patient advocacy groups (FFB, retina UK, FB), physicians, >30 global KOLs, and patients KOLs and patient advocacy groups lobbying for approval across US, EU and Japan



MEIRAGTx

*, ** Revenue projections are from investment bank and demand forecasts using Luxturna pricing, 30% GTN as benchmark

Bota-vec status: awaiting BLA and MAA filings

Largest global development program in a severe Inherited Retinal Disease (IRD) completed with compelling data indicating statistically significant and clinically meaningful benefits across every domain of vision supporting global approval:

- **Phase 3 complete (n= 95)** conducted at 32 of the leading global sites for IRD sites in N. America UK EU and Japan
 - Novel primary endpoint (VMA, Maze) while not achieving statistical significance showed a positive trend with **treated patients 2.4x more likely to respond** than untreated
 - Statistically significant and clinically meaningful **improvements** demonstrated in secondary endpoints in **all domains of vision**
 - **Significant clinically meaningful responder analysis** comparing treated and untreated patients using any endpoints in each of the domains of vision
 - **Significant PRO data** – particularly ability to function in low light, independence, emotional well being, strong concordance of scripted interviews with clinical data
 - **Strong safety profile** - expected and manageable across both Phase 1 and Phase 3 studies (**n= 137**) with further improved immune safety demonstrated in the Phase 3 (n=95)
- **Japan** four Phase 3 patients treated in Japan with 100% response rate, PI reported an extraordinary 40-letter gain in one patient LLVA
- **Phase1/2 study (n=42)** supportive of safety as well as efficacy

Commercial Manufacturing Status:

- **PPQ complete:** MeiraGTx is the commercial manufacturer of bota-vec. PPQ is complete with 3 successful batches of each DS and DP produced; several hundred vials are available for commercial release.
- **Commercial CMC/QC licenses in hand:** MeiraGTx has been granted commercial licenses for both viral vector manufacturing and QC facilities for AAV-RPGR manufacturing as well as QC.

Strong physician and patient advocacy group (FFB) support for approval of bota-vec

Foundation for Fighting Blindness (FFB) Open Letter

In June 2025, the FFB published a public letter signed by 30 leading retinal specialists urging J&J to seek approval for bota-vec based on “life changing improvements after treatment in the Phase 3 LUMEOS trial”.

“The vision improvements in LUMEOS have been life changing for many patients. Both objective measures and subjective reports demonstrate clear and meaningful efficacy... **The Foundation and trial investigators feel it is incumbent upon J&J to urgently pursue regulatory approval** to make the vision-restoring treatment available to the global XLRP community.”

Reference: FFB Public Letter, June 2025

Patient Voices from LUMEOS Trial Quoted in FFB Letter

“**The improvement... has been nothing short of remarkable.** ...I would do anything within my power to have the same life-changing opportunity extended to my untreated eye.”

“The treatment has dramatically improved my eyesight. **I used to need a cane at night... and now I no longer need to use a cane.**”

“**I saw stars for the first time** and clouds at night! It was amazing.”

“Tasks that were once frustrating... like moving around safely in dim rooms or walking outdoors after sunset, **have become noticeably easier... restored a sense of independence.**”

Bota-vec commercial opportunity:

Launch into concentrated, educated KOL market with commercial supply manufactured in-house at MeiraGTx

Projected cost-effective launch into a concentrated group of educated physicians and patients globally

- Most IRD patients are seen by a **small community of connected KOLs globally**
- We believe a very **large majority, (~ 80%) of RPGR patients globally**, are under the care of such physicians at **~ 40-50 sites US, EU, and Japan**
- This includes the **32 sites who participated in the Bota-vec Phase 3 study**, and all of these global KOLs are known to MeiraGTx through our decade old relationships with the Moorfields Eye Hospital in London and other leading hospitals
- **Over 60 surgeons were trained in bota-vec** delivery for the Phase 3 – there were minimal surgical complications and the safety profile was as expected and improved in the Phase 3 compared to the Phase 1
- KOLs and patient advocates globally are **lobbying for approval** with patients waiting for treatment globally
- **Several hundred patients already identified** in patient registry and at leading hospitals globally
- **Commercial material for release:** PPQ successful with several hundred vials available at MeiraGTx for potential commercial launch
- **MeiraGTx is the commercial manufacturer** with end-to-end **control of drug supply and guaranteed capacity** to support a rapid launch. MeiraGTx holds manufacturing and QC commercial licenses in UK (MHRA) and in Ireland (HPRA)



Phase 3 LUMEOS study of botaretigene sparoparvovec (bota-vec)

Summary of Data

Source: Oral presentation at FFB ARVO 2025
meeting and publication in preparation



Bota-vec Phase 3 Pivotal Study Results

Consistent and Robust Efficacy Across Objective & Subjective Measures

These Endpoints Reflect Significant and Clinically Meaningful Improvement in Every Domain of Vision

Category	Functional Vision		Retinal Function			Visual Function	Safety
Assessment	VMA* (binocular) PRIMARY	PRO: LLQ Extreme lighting domain score	Static perimetry responder (central 30 degrees)	Static perimetry responder (full field)	Retinal sensitivity (central 10 degrees)	Low luminance visual acuity (LLVA, ETDRS letters)	Intraocular inflammation
Results	↑ [†]	↑ [‡]	↑ [‡]	↑ [‡]	↑ [‡]	↑ [‡]	Expected, acceptable, and manageable
Treatment difference (±95% CI)	13.4% (-3.6%, 30.5%) <i>P</i> value = 0.247	7.29 (2.29, 12.38) <i>P</i> value = 0.006	38.1% (21.4%, 54.7%) <i>P</i> value = 0.001	36.6% (18.2%, 54.9%) <i>P</i> value = 0.001	1.24 dB (0.54, 1.94) <i>P</i> value = 0.001	4.75 ETDRS letters (1.64, 7.86) <i>P</i> value = 0.003	

*VMA is a novel assessment first used in this study. [†]Results trending in anticipated direction. [‡]*P*<0.05

Full analysis set population (observed data). Includes participants randomized to both doses.

BCVA, best-corrected visual acuity; bota-vec, botaretigene sparoparvovec; ETDRS, Early Treatment Diabetic Retinopathy Study; LLQ, low luminance questionnaire; LLVA, low luminance visual acuity; PRO, patient-reported outcomes; VMA, vision-guided mobility assessment.

Bota-vec Phase 3 Pivotal Study Results: Highly statistically significant and clinically meaningful benefit in multiple secondary endpoints every domain of vision

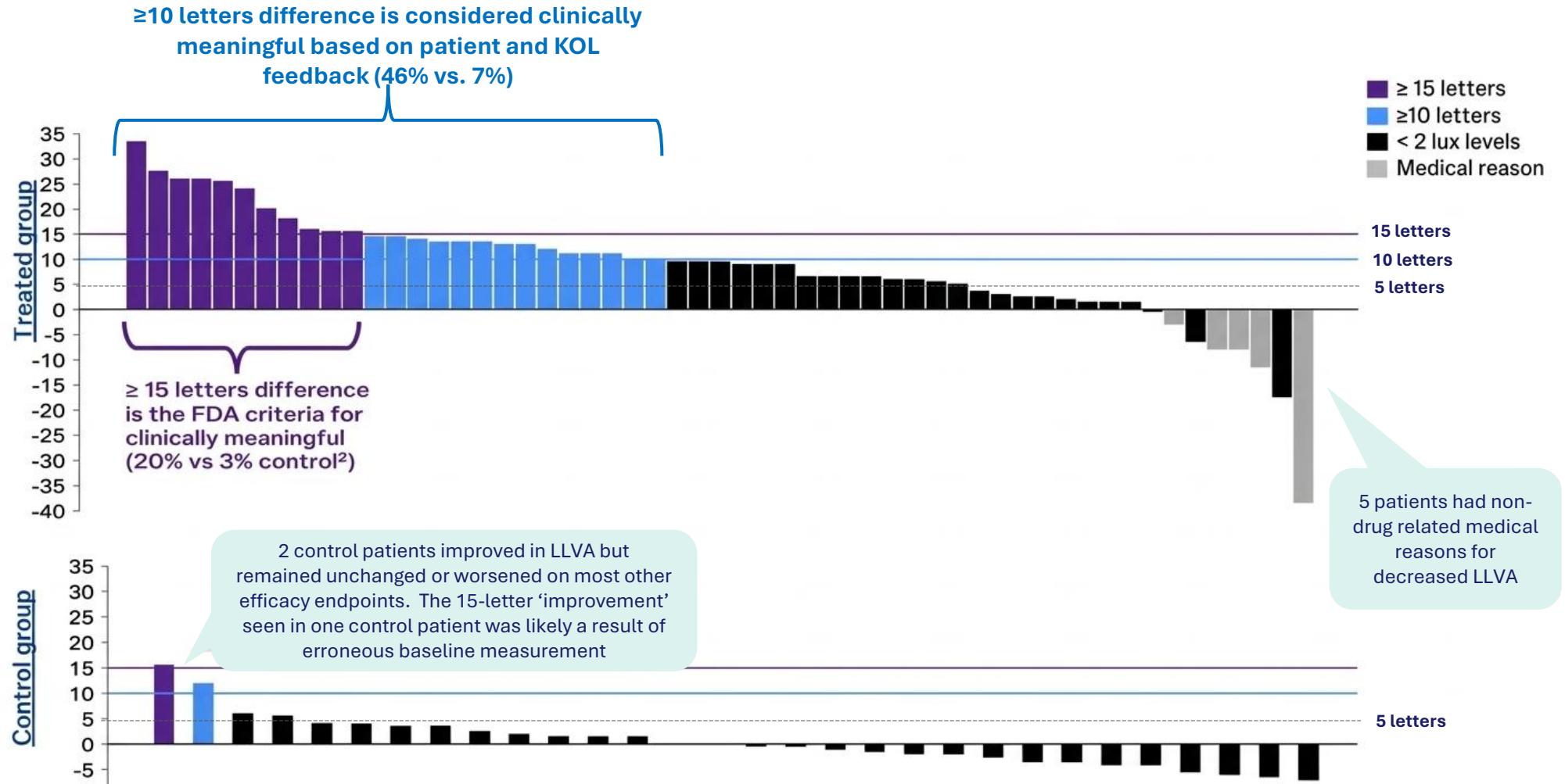
Primary Endpoint	Pooled treated (n = 55)	Control group (n = 30)	Between-group difference (pooled treated group minus control) (95% CI)	P-value
VMA responders ¹ (binocular), %	27.3%	13.3%	13.4% (-3.6%, 30.5%)	0.247 [†]
Secondary Endpoints				
Change in MRS10 ⁴ , LS mean (SE)	0.88 (0.22)	-0.36 (0.29)	1.24 (0.54; 1.94)	0.001
Pointwise responders in full visual field ² , %	58.2%	23.3%	36.6% (18.2%, 54.9%)	0.001
Pointwise responders in the central 30 degrees ² , %	47.3%	10.0%	38.1% (21.4%, 54.7%)	0.001
Change in mLLQ Extreme lighting domain score, LS mean (SE)	1.80 (1.50)	-5.49 (2.05)	7.29 (2.20; 12.38)	0.006
Change in LLVA ⁴ , LS mean (SE)	6.86 (0.94)	2.11 (1.26)	4.75 (1.64; 7.86)	0.003
Change in MRS90 ⁴ , LS mean (SE)	-0.00 (0.19)	-0.93 (0.26)	0.92 (0.29; 1.55)	0.004

Significant improvements in Patient Reported Outcomes (PRO) questions related to everyday function and quality of life:

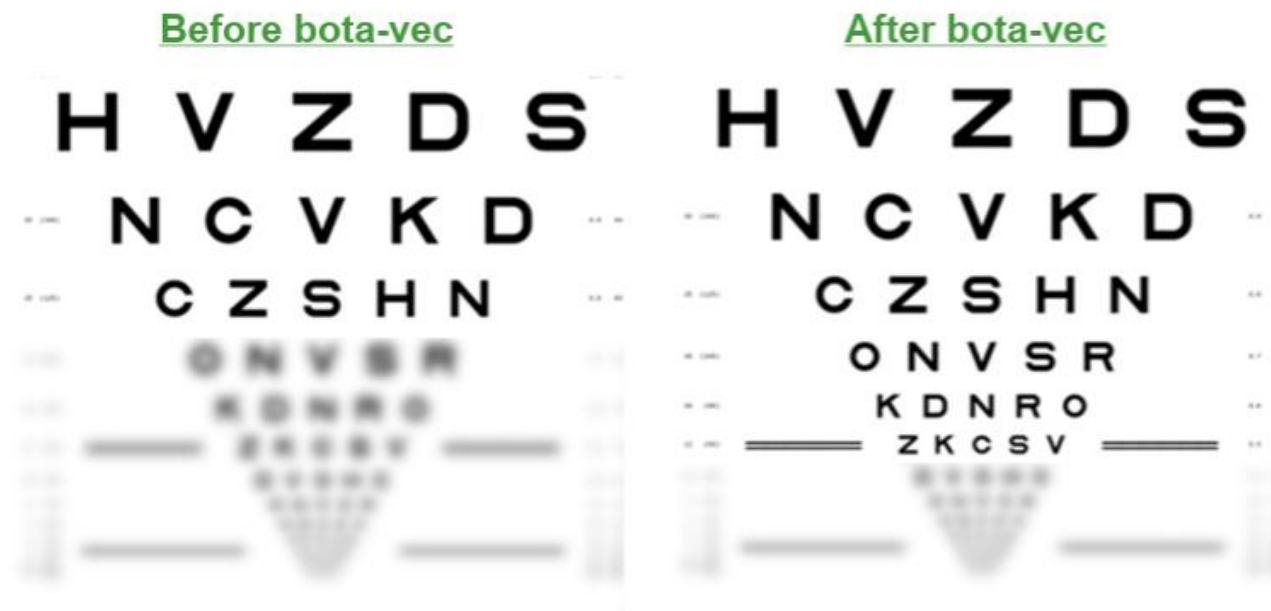
- **IVI-A:** significant improvement in total score vs. control at week 52 (**p=0.024**) with greater significance in the emotional well being questions (**p=0.005**)
- **Modified Low Luminance Questionnaire MLLQ** showed statistically significant improvement (**p=0.006**) with improvements in questions relating to mobility (**p= 0.001**), general dim lighting (**p= 0.007**) and emotional distress (**p= 0.019**) being highly significant
- **Notable concordance** between PRO interviews and other clinical data

Impressive results for Visual Acuity measured in low light levels (LLVA) with 46% of treated patients achieving ≥ 10 letter gain on ETDRS chart compared to 7% for untreated controls

Change in Low Luminance Visual Acuity (ETDRS letters) in worse-seeing eye for individual patients at week 52



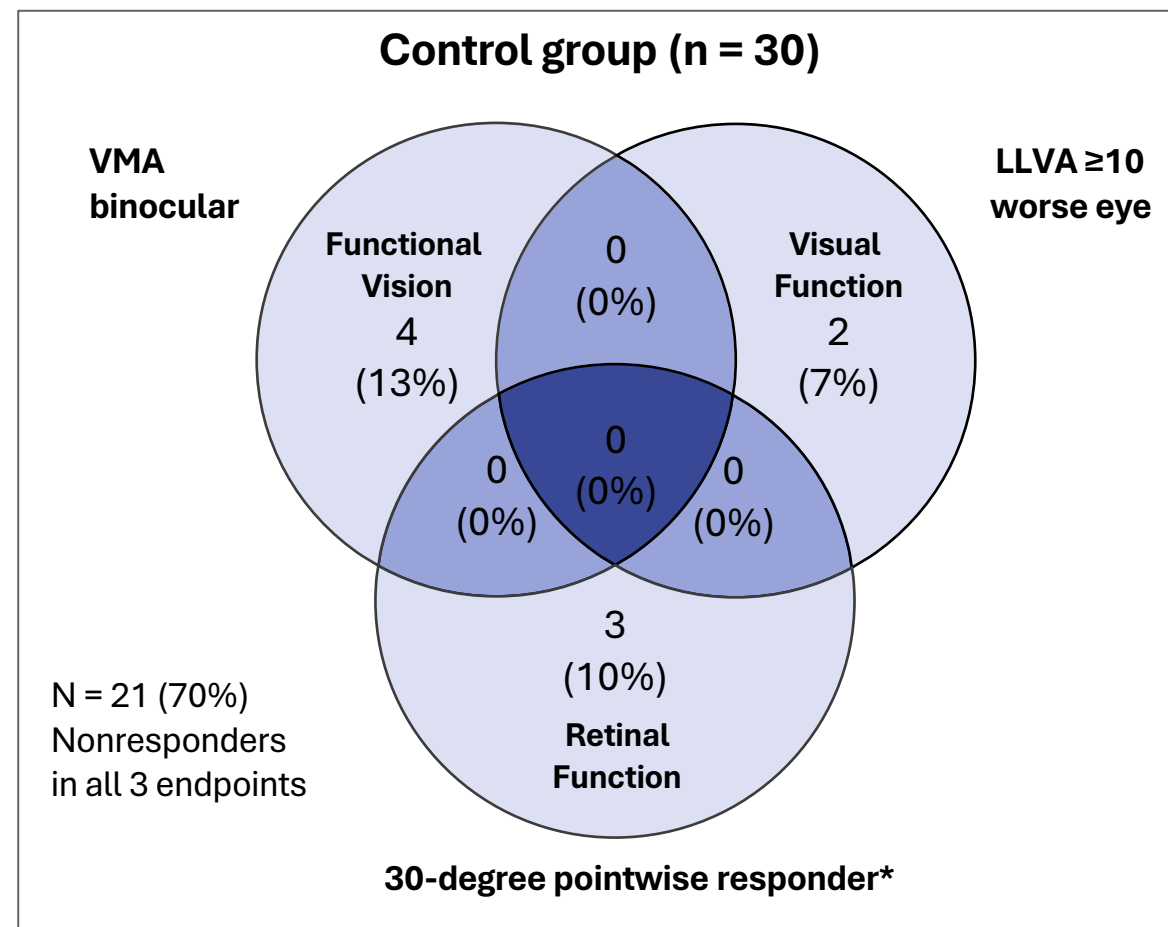
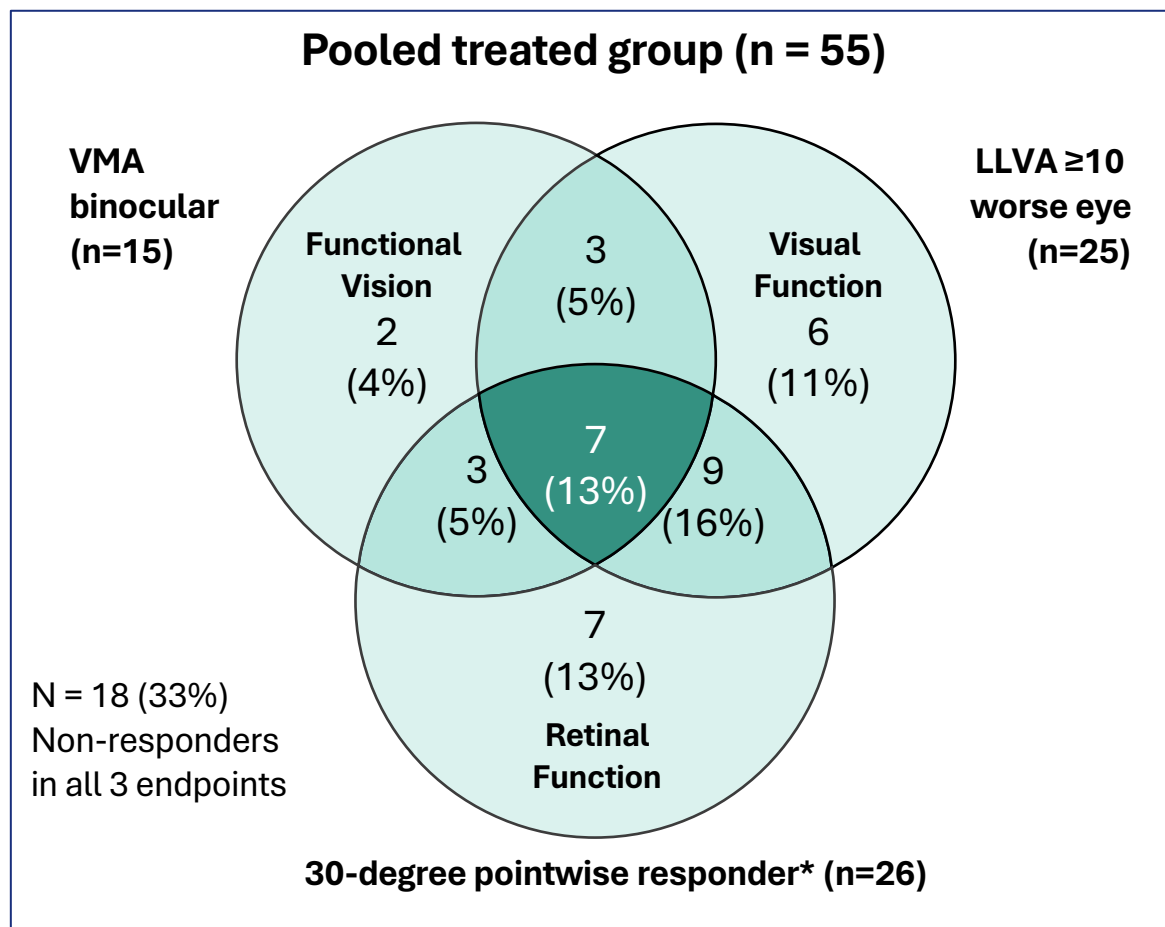
Illustrative Depiction of Effect of bota-vec Treatment: 15 letters = 3 lines on the ETDRS chart



- 15 letters is equivalent to 3 lines on the eye chart
- From a functional standpoint in everyday life, a 3-line improvement in visual acuity means **you can now stand twice as far away from an object and still see it clearly**

- Example of 15 letter visual acuity improvement from 0.8 logMAR or 20/125 (left image) to 0.5 logMAR or 20/60 (right image)

40% (22/55) of Treated Patients Showed Improvement in ≥ 2 Endpoints Compared to 0% in Controls



Multi-Endpoint Responder Analysis: Comprehensive Results

FF = Full Field; Pt = Pointwise; binoc = binocular.

Consistent 25–40% treatment benefit across ALL endpoint combinations with most combinations showing 0% response in control arm

SECTION 1: VMA + LLVA COMBINATIONS

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
VMA binoc. + LLVA binoc. (≥ 10)	40.0%	0%	+40.0%
VMA binoc. + LLVA worse eye (≥ 10)	27.3%	0%	+27.3%
VMA binoc. + LLVA binoc. (≥ 15)	29.1%	0%	+29.1%
VMA binoc. + LLVA worse eye (≥ 15)	25.5%	0%	+25.5%

SECTION 2: VMA + STATIC PERIMETRY

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
VMA binoc. + Pointwise 30° binoc.	32.7%	3.3%	+29.4%
VMA binoc. + Pointwise 30° worse eye	25.5%	0%	+25.5%
VMA worse eye + Pointwise FF binoc.	30.9%	0%	+30.9%
VMA worse eye + Pointwise FF worse eye	29.1%	0%	+29.1%

SECTION 3: LLVA + STATIC PERIMETRY

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
LLVA worse eye (≥ 10) + Pt 30° binoc.	43.6%	3.3%	+40.3%
LLVA binoc. (≥ 10) + Pt 30° binoc.	36.4%	6.7%	+29.7%
LLVA worse eye (≥ 10) + Pt FF binoc.	41.8%	3.3%	+38.5%
LLVA binoc. (≥ 15) + Pt 30° worse eye	29.1%	0%	+29.1%

Conclusions

- Large Phase 3 study, 32 leading global sites (n=95). Although the novel primary endpoint was not statistically significant, there was a positive trend with the treated group 2.4x more likely to respond than untreated.
- Unprecedented clinically meaningful and statistically significant **improvements** were observed in **all three domains of vision** **(i)** retinal function (static perimetry and microperimetry); **(ii)** functional vision under low luminance (mLLO); and **(iii)** visual function (LLVA). Prior to studies with AAV-RPGR, stabilization of vision was expected and by regulators, this sort of strong improvement across all lines of vision was not previously known to be possible.
- Safety profile of bota-vec was as expected and manageable, no new safety signals in the Phase 3 with improved inflammatory profile and reduced surgery related A/Es.
- Supported by Phase 1/2 dose escalation and expansion study (n=43) for safety and efficacy.
- The Phase 3 study investigators strongly support seeking regulatory approval for bota-vec globally, signing an open letter from the Foundation for Fighting Blindness (FFB)