

# Beacon Therapeutics

Transforming lives with pioneering  
ocular gene therapy

*Investing in Oxford: Transatlantic Healthcare Innovation Reception*

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Chief Executive Officer

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# Beacon Therapeutics: Transforming vision, transforming lives

Our mission: to save and restore vision in patients with blinding retinal diseases



## Competitive Pipeline

Breakthrough proprietary pipeline addressing rare and prevalent diseases including X-Linked Retinitis Pigmentosa (XLRP), dry AMD & inherited Cone Rod Dystrophy (CRD)



## Late-stage Asset

Late-stage program with compelling clinical data to date for XLRP, a devastating disease with no treatment available; 3 years from potential regulatory authorization



## Well Capitalized

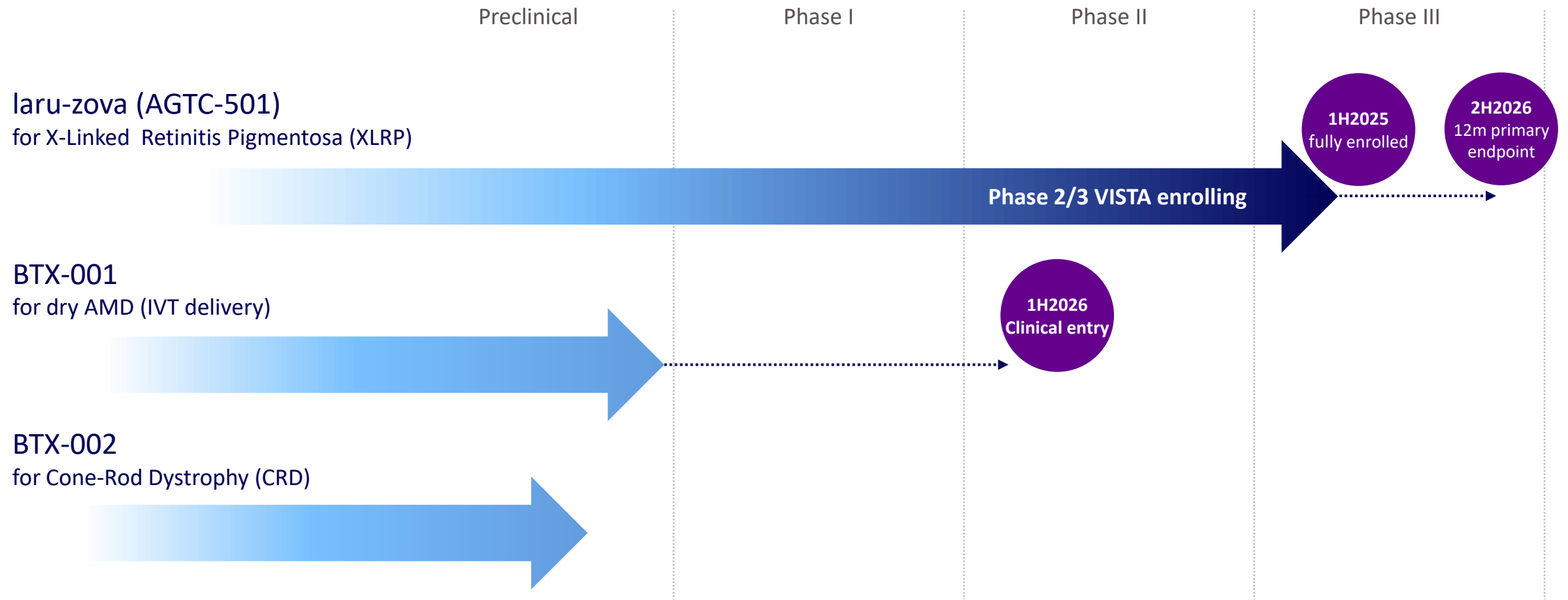
Backed by global blue-chip investors to drive our innovative therapies through clinical development and toward launch



## Ocular Gene Therapy Expertise

Experienced leadership team with proven success in developing and commercializing ocular therapies from concept to market

# Our ocular gene therapy pipeline addresses both rare and prevalent diseases with high unmet need



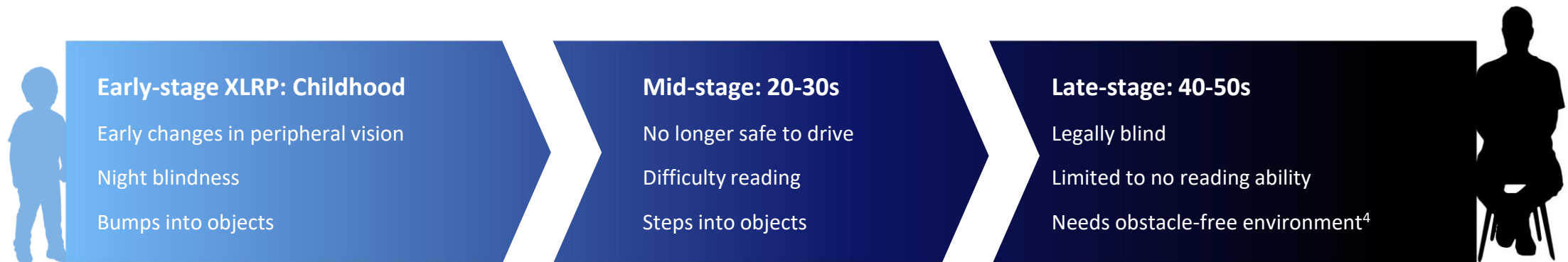
AMD = age-related macular degeneration. IVT=intravitreal. FPI=first patient in clinical study

# Lead asset addresses XLRP, a rare but devastating inherited retinal disease with no treatment



**X-Linked Retinitis Pigmentosa (XLRP) is one of the most common inherited retinal diseases**

└ 70-90% of XLRP cases are from **RPGR mutations**<sup>1,2</sup>

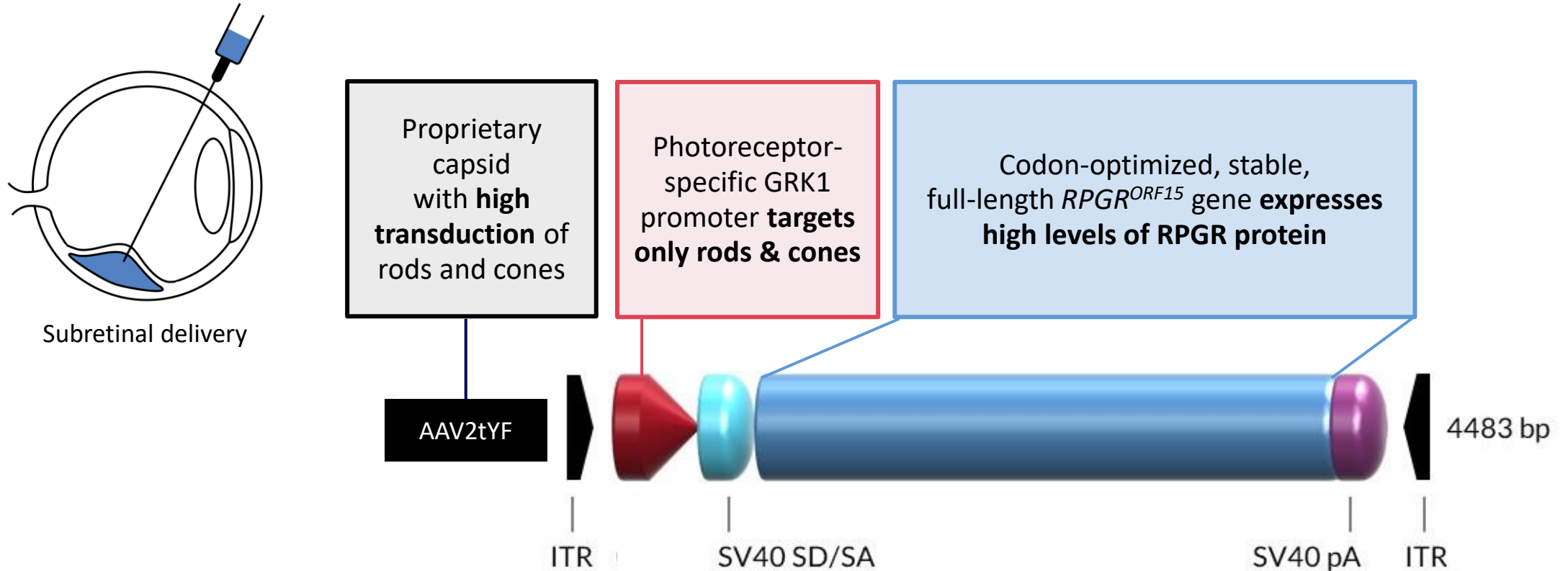


\*In the United States, Europe, and Australia.

XLRP = X-linked retinitis pigmentosa; RPGR=retinitis pigmentosa GTPase regulator

1. Birch DG, et al. *Transl Vis Sci Technol.* 2023;12:5.; 2. Nguyen XT, et al. *Int J Mol Sci.* 2020;21:835.; 3. Vinikoor-Imler LC, et al. *Ophthalmic Genetics.* 2022;43:581-8. 4. Cross N, et al. *Opth.* 2022;16:1993-2010.

# Laru-zova has potential to restore natural function of both rods and cones by delivering the full-length *RPGR*<sup>ORF15</sup> protein<sup>1,2</sup>



XLRP=X-linked retinitis pigmentosa; RPGR=retinitis pigmentosa GTPase regulator; AAV=adeno-associated virus; GRK1=rhodopsin kinase  
1. Cehajic-Kapetanovic J, et al. *Proc Natl Acad Sci U S A*. 2022;119(49):e2208707119.; 2. Wu Z, et al. *Hum Mol Genet*. 2015;24(14):3956-3970.

# Laru-zova has been well tolerated in 44 patients across three clinical trials to date

**years of data from 3 studies to date<sup>1-4</sup>**

Up to

5

Maximum tolerated dose and optimal location of bleb identified

No reports of serious or severe inflammation

No drug-related SAEs & only mild-to-moderate drug-related AEs in doses advanced in the development program

No immunogenicity pattern associated with AEs to date

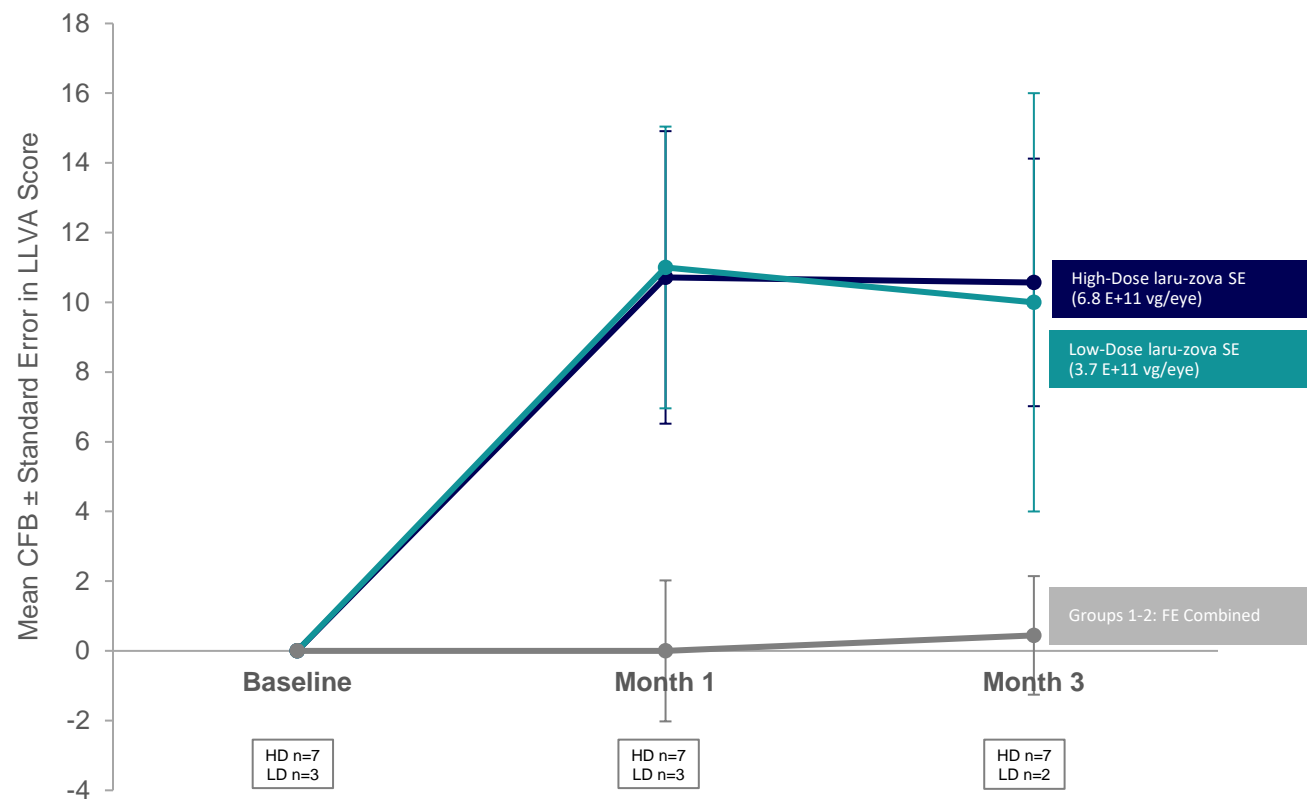
Vector shedding assessments collected in two studies showed negative or undetectable at all timepoints

1. Yang P, et al. Oral Presentation at Euretina Congress 2024, Barcelona, Spain.; 2. Sisk R, et al. Oral Presentation at AAO 2024 Conference, Chicago, Illinois.; 3. Stanga P. et al. Oral Presentation at FLORetina Congress 2024. Florence, Italy.; 4. Data on file, Beacon Therapeutics (USA), Inc.

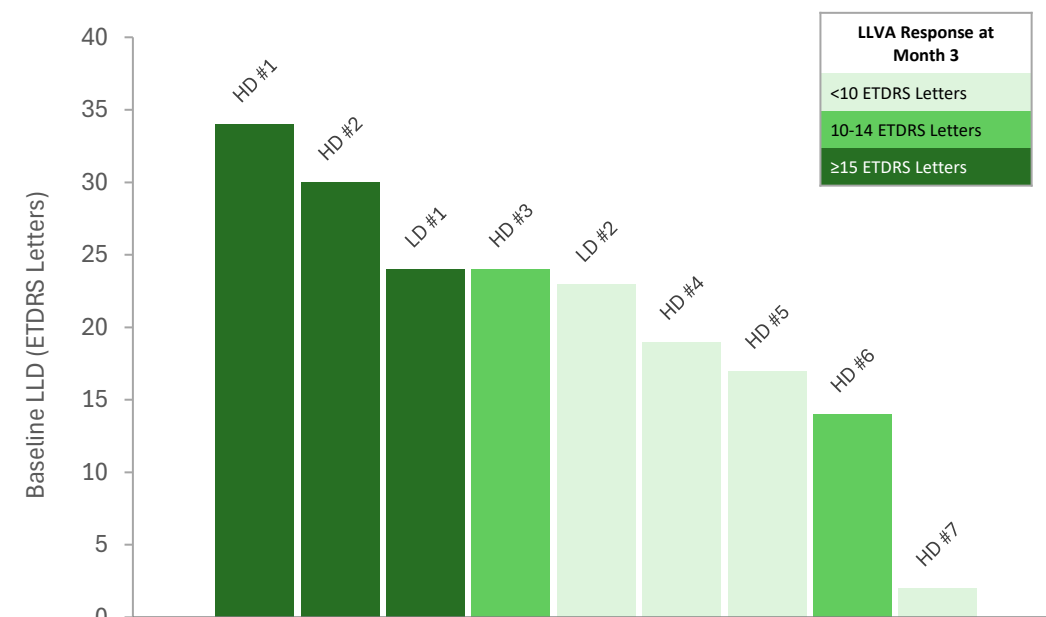
# Phase 2 DAWN efficacy:

## Early improvement in mean LLVA at month 3

Mean LLVA Change from Baseline  
(ETDRS Letters)



Baseline Low Luminance Deficit (LLD) in Study  
Eye By Subject with LLVA Response at Month 3

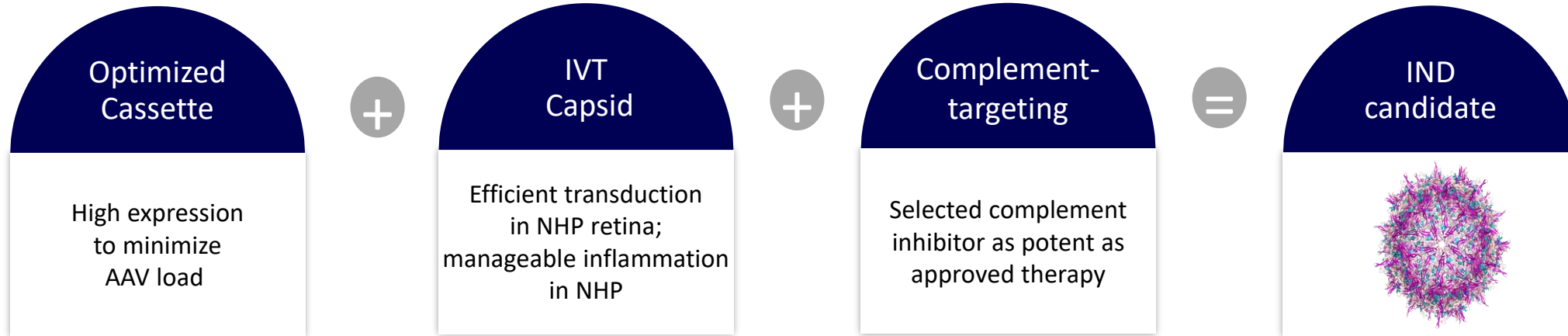


CFB = change from baseline; ETDRS = Early Treatment of Diabetic Retinopathy Study; LLVA = low luminance visual acuity; LLD = low luminance deficit; SE = study eye (newly treated); FE = fellow eye (previously treated); HD = high dose; LD= low dose

1. Yang P, et al. Oral Presentation at FLORetina-ICOOR 2024 Meeting, Florence, Italy.



# Dry AMD candidate BTX-001 progressing to IND



## BTX-001 gene therapy

targets de-risked complement pathway, combining convenience of IVT delivery with potential for sustained treatment effect seen with gene therapies

AAV=adeno-associated virus; IVT= intravitreal; NHP=non-human primate.



# Upcoming catalysts for XLRP (laru-zova) and dryAMD (BTX-001) programs



## Competitive Pipeline

Breakthrough proprietary pipeline addressing rare and prevalent diseases including X-Linked Retinitis Pigmentosa (XLRP), dryAMD & inherited Cone Rod Dystrophy (CRD)

**laru-zova Ph 2/3  
enrollment complete  
1H25**



## Late-stage Asset

Late-stage program with compelling clinical data for XLRP, a devastating disease with no treatment available; 3 years from potential regulatory authorization

**BTX-001  
Ph 1 clinical entry  
1H26**



## Well Capitalized

Backed by global blue-chip investors to drive our innovative therapies through clinical development and toward launch

**laru-zova Ph 2/3  
12M primary  
endpoint to support  
registration 2H26**



## Ocular Gene Therapy Expertise

Experienced leadership with proven success in developing and commercializing ocular therapies from concept to market