# A bicistronic AAV platform for dual-pathway modulation in AMD: Platform development and manufacturing readiness

Katie Binley[1], Andrew Osborne[1], Emily Warner[1], Laura Vaux[1], Chris Benwell[1], Peter Widdowson[1], Thomas Ciulla[1]

[1] Ikarovec Ltd, The NRP Innovation Centre, Norwich, United Kingdom, NR4 7GJ. | info@ikarovec.com

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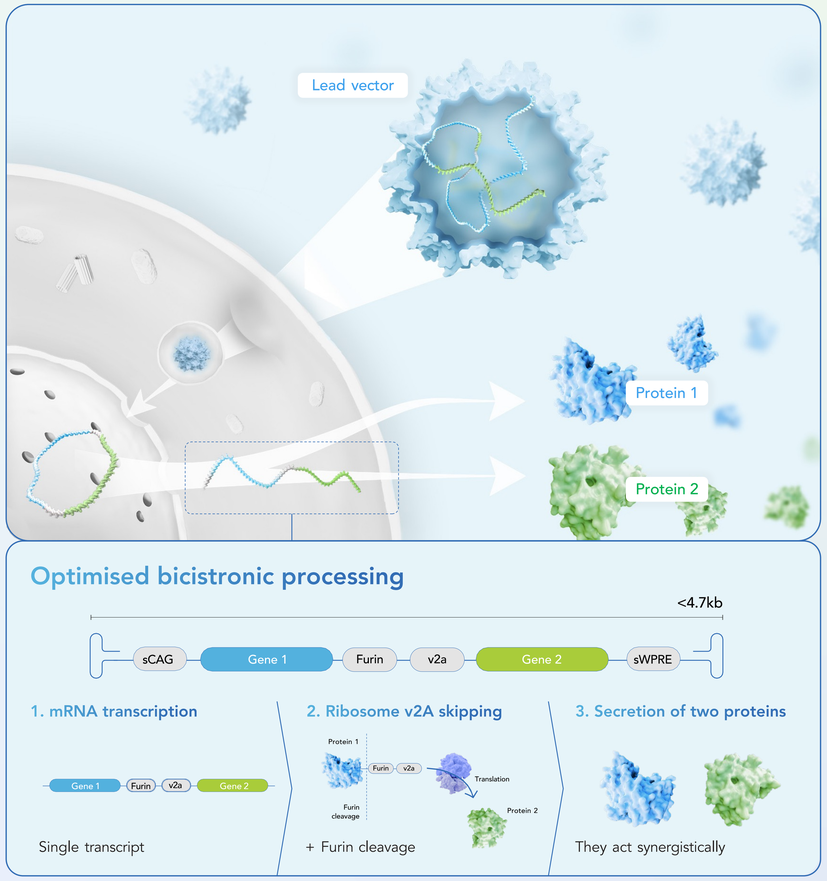
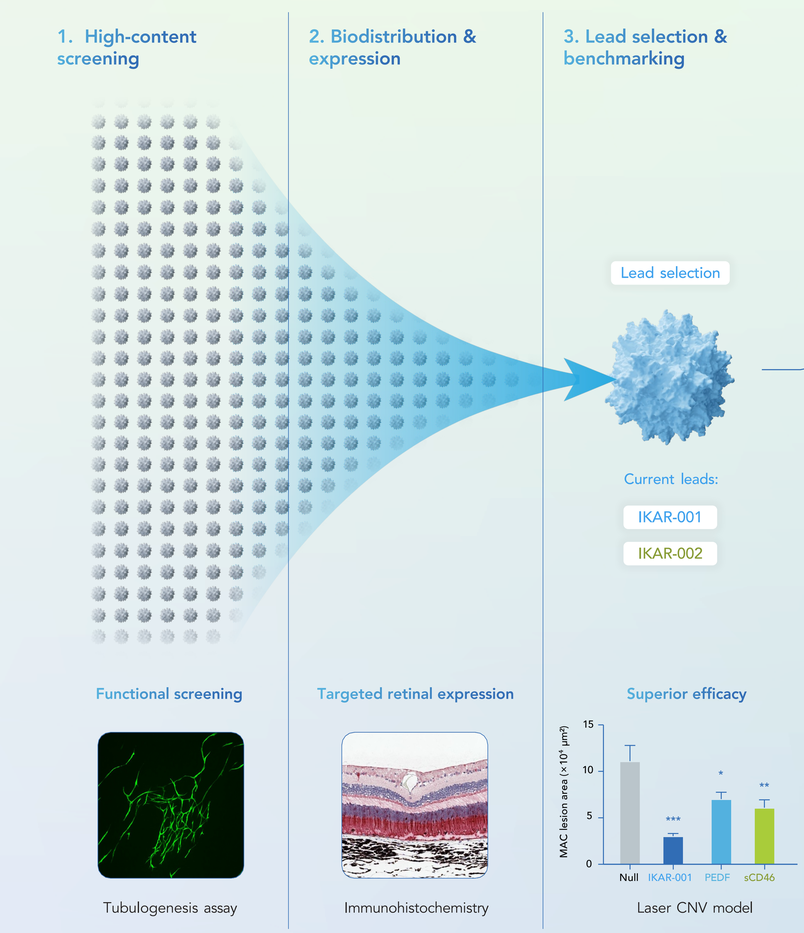
Age-related macular degeneration (AMD) involves multiple overlapping mechanisms requiring coordinated intervention. We developed a bicistronic AAV platform delivering secreted therapeutic proteins from a single vector. This poster describes construct engineering, screening, and manufacturing optimisation supporting clinical advancement.

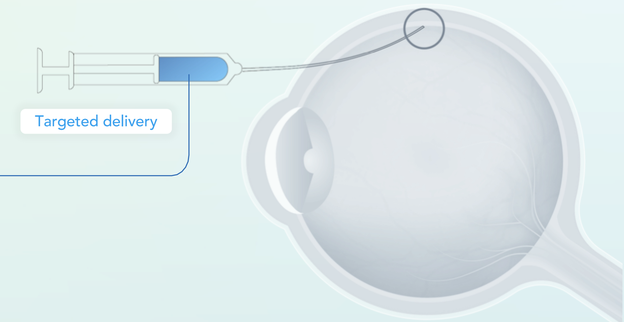
## 1 Bicistronic AAV platform for dual protein delivery




## 2 Streamlined discovery using a tiered screening strategy

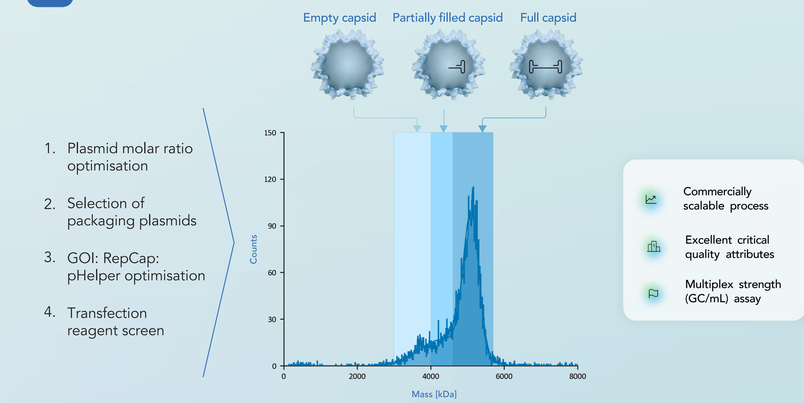



## 3 Sophisticated and targeted gene therapy delivery

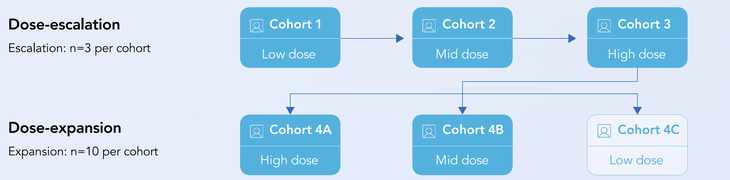



The platform's modular architecture enables targeted retinal delivery across multiple disease settings, with interchangeable construct components to address distinct pathogenic mechanisms.

## 4 Scalable bicistronic AAV suspension process

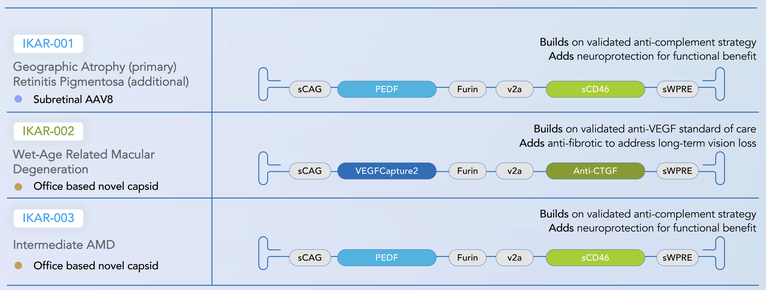



## 5 De-risked geographic atrophy clinical trial strategy




- Fast-progressor enrichment maximizes treatment effect detection; adaptive design with sensitive endpoints
- Early (12 week) & multiple biomarker inflection points
- Designed to demonstrate visual function preservation in GA; Fast Track designation pathway and breakthrough designation potential

## 6 Strategically designed pipeline of dual-pathway therapies




## 7 Executive Summary

- We have designed next generation bicistronic AAV vectors to treat GA, RP, wAMD and iAMD.
- Vector screening combined expression & biodistribution with high content efficacy models.
- An optimised commercially scalable manufacturing process yields vector with excellent critical quality attributes.
- These data support clinical development of lead IKAR-001 for GA and RP.
- Novel capsids for office-based delivery are under evaluation for IKAR-002 & 003 programs.

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