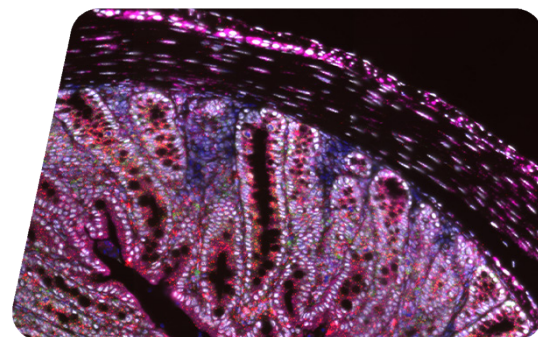


Accelerate AAV-based Therapeutic Development

Visualize Biodistribution with the Power of RNAscope™ Probes



Characterize Biodistribution, Cellular Tropism, and Transduction Efficiency

RNAscope™ ISH technology is an ideal solution for detecting AAV vector DNA and therapeutic transgene mRNA expression with morphological context, along with protein markers addressing critical questions of tissue biodistribution, persistence, cellular tropism and vector promoter activity.

- **Measure** abundance of AAV+ cells in target tissues and track vector persistence over time.
- **Quantify** RNA expression of any vector cargo like CRISPR/Cas9, guide RNAs, or other non-coding RNAs.
- **Demonstrate** therapeutic efficacy within vector transduced cells and tissues.
- **Easily Scale** from small animal models to non-human primates and distinguish human transgenes from homologous host transcripts.

Probe Design Strategy

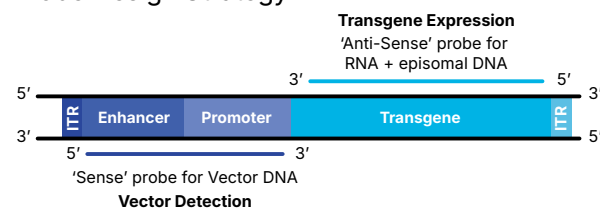


Figure 01A. RNAscope probes are designed to target unique regions within the promoter of the AAV vector DNA or the transgene mRNA of the viral construct.

Simultaneous Detection of AAV Vector DNA and Transgene mRNA

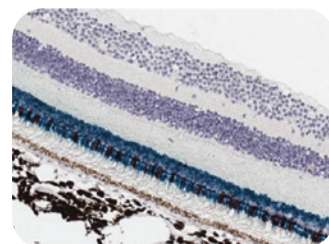


Figure 01B. Retina cells were transduced with AAV-GFP. RNAscope™ 2.5 HD Duplex assay was used for simultaneous detection of rhodopsin (rod cells; green) and opsin (cone cells; red).

Ready-to-Use Popular Gene Therapy Probes

	RNAscope	BaseScope
Promoter	CAG	
	CBA	
	CMV	-
	Synapsin	AAV
Enhancer	CMV	Alb
	CMV-CAG	TTR
	CMV-CBA	TTR-EN
	Fos	
WPRES	WPRES	
	CBA-WPRES	
	CAG-WPRES	WPRES3
	CDH-WPRES	
PolyA	SV40polyA	bGH-polyA
		SV40-polyA
		bGH-polyA-NPBB
Reporters	EGFP	
	GFP	AAV-EGFP
	GFP-Jellyfish	gRNA-GFP
	mClover3	pAAV-GFP
	TurboGFP	pTR-CB-GFP
	EYFP	
	Luciferase	
	Cherry	
	Tomato	
	sYFP	—

Visualize Biodistribution — More Informative than qPCR

Morphology-based biodistribution is extremely important. While qPCR-based methods provide average values for copy number from extracted nucleic acids, the RNAscope technology provides morphology-based quantification.

FDA strongly recommends biodistribution studies to characterize engineered products⁽¹⁾. RNAscope *in situ* hybridization assay is the most powerful spatial method available within the methods provided.

Powerful Visualization at Single-Cell and Single-Molecule Sensitivity and Specificity

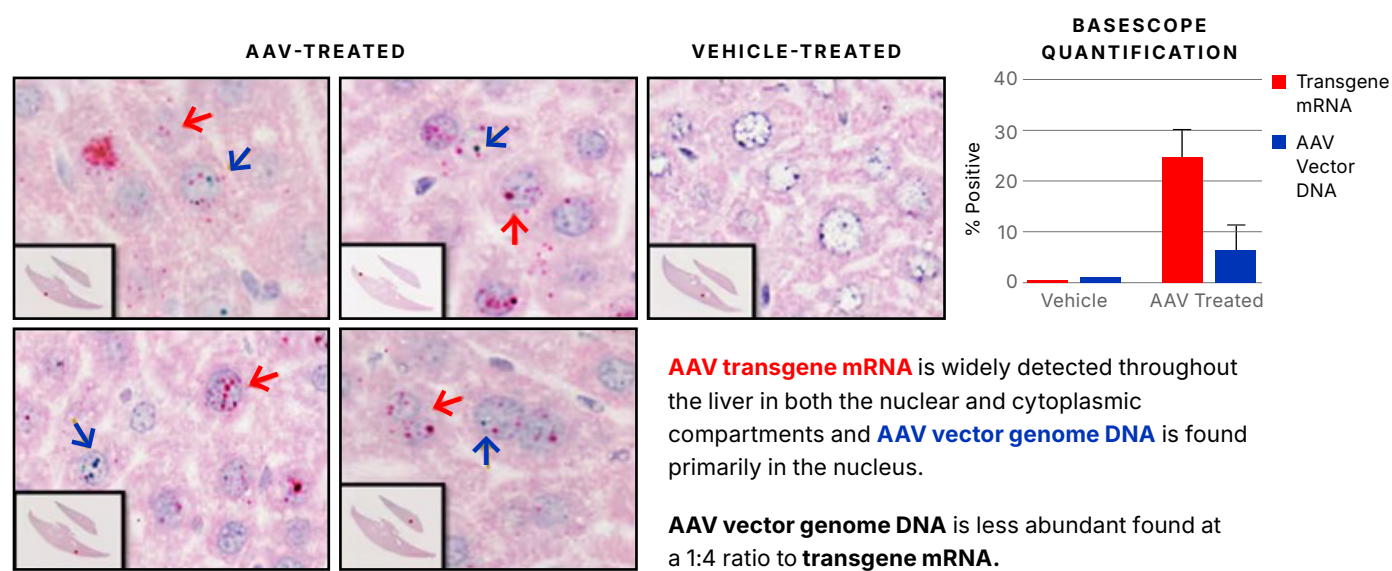
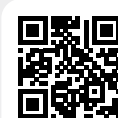


Figure 02. BaseScope™ assay allows visualization and quantification of transgene expression (red arrow) and AAV vector (blue arrow) presence, in treated liver samples.

Accelerate your pre-clinical programs in gene therapy with ready-to-use catalog probes. Getting started is easy!

Easy Probe Selection: Select the genes you want from an extensive online catalog and custom design services.

Expert Technical Support: Experienced technical specialists in RNA spatial assays will help you get up and running quickly.



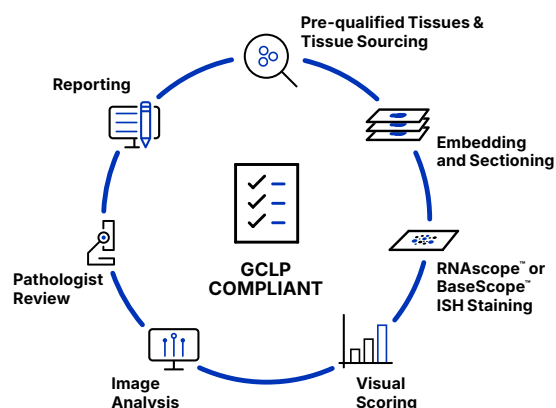
Contact Us

For a custom probe for your region of interest by submitting your proprietary sequence for new probe request.

Professional Assay Services

Our Professional Assay Services offer you direct access to the developers of RNAscope in situ hybridization (ISH) technology. Accelerate your journey in preclinical, and clinical development with the promise that we will provide consistent and trustworthy data to drive your projects forward.

Contact us for a Free Project Consultation.



(1)S12 Nonclinical Biodistribution Considerations for Gene Therapy Products; Guidance for Industry; U.S. Department of Health and Human Services Food and Drug Administration; Center for Drug Evaluation and Research (CDER); Center for Biologics Evaluation and Research (CBER). May 2023; ICH-Safety.

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