

## Genetic analysis in gene therapy research workflows

Genetic analysis is at the forefront of improving the scientific understanding and development of safer and more effective gene therapies. Thermo Fisher Scientific offers one of the most comprehensive ranges of genetic analysis technologies. Our real-time PCR and Sanger sequencing tools included in this guide help enable reproducible research studies for greater insights throughout the gene therapy product development workflow. Learn more about the extensive Thermo Fisher portfolio of genetic analysis solutions.



### **Proof of concept**

### Identification of molecular lesion

Confirmation of mutational allele

Applied Biosystems<sup>™</sup> BigDye<sup>™</sup> Sanger sequencing reagents

Predesigned Applied Biosystems<sup>™</sup> TaqMan<sup>™</sup> genotyping

### **Correction of lesion**

Plasmid and vector construction

BigDye Sanger sequencing reagents

Custom TagMan assays

### **Animal models of disease**

Constructing model with genetic lesion

Invitrogen™ GeneArt™ CRISPR design tools

Invitrogen CRISPR/Cas9 gene editing tools

BigDye Sanger sequencing reagents

Applied Biosystems<sup>™</sup> SeqScreener<sup>™</sup> gene edit confirmation

### Colony management

Predesigned TaqMan genotyping assays

BigDye Sanger sequencing reagents

### Transgene uptake and route of administration

Applied Biosystems<sup>™</sup> Absolute Q<sup>™</sup> custom dPCR assays

TaqMan genotyping assays

### Phenotypic efficacy

Predesigned TaqMan gene expression assays and arrays

### Response biomarker identification

Singe-gene expression analysis

Predesigned TaqMan gene expression assays

### Expression signature analysis

Predesigned TaqMan gene expression arrays

### miRNA analysis

Applied Biosystems<sup>™</sup> TaqMan MicroRNA assays

Applied Biosystems™ TaqMan Advanced miRNA assays

## **Delivery vector design** and optimization

### Vector construction and packaging

Invitrogen™ GeneArt™ Custom Gene Synthesis

BigDye Sanger sequencing reagents

Custom TaqMan assays

### **MOI** titration and optimization

Cell line management

Applied Biosystems<sup>™</sup> CLA IdentiFiler<sup>™</sup> PCR amplification kits

Applied Biosystems<sup>™</sup> CLA GlobalFiler<sup>™</sup> PCR amplification kits

Virus detection and quantification

Custom TaqMan assays

Absolute Q dPCR assays

### **Vehicle toxicology**

Tissue damage gene expression

Predesigned TaqMan gene expression arrays

Custom TaqMan gene expression arrays

# **Delivery vehicle production**

### Quantifying gene transfer vehicles

Titrating functional entities

TaqMan dPCR assays

Custom TaqMan gene expression assays

Residual (unpackaged) vector quantification

TaqMan dPCR assays

### **Cell culture quality control**

Authenticating host cells

CLA IdentiFiler PCR amplification kits

CLA GlobalFiler PCR amplification kits

### Mycoplasma detection

Applied Biosystems™ MycoSEQ™ Mycoplasma Detection Assay

Custom fragment analysis primers

Applied Biosystems<sup>™</sup> TaqPath<sup>™</sup> BactoPure<sup>™</sup> Microbial **Detection Master Mix** 

### Contaminating microbial identification

Applied Biosystems<sup>™</sup> MicroSEQ<sup>™</sup> Rapid Microbial Identification System

TagPath BactoPure Microbial Detection Master Mix

BigDye Sanger sequencing reagents

TaqMan microbe assays

## **Pre-clinical development**

### Dosing optimization

Predesigned TaqMan genotyping assays

TaqMan dPCR assays

### Therapeutic efficacy

Gene expression analysis

Predesigned TaqMan gene expression assays and arrays

### Biomarker tracking

Custom TagMan assays

TagMan MicroRNA assays

TaqMan Advanced miRNA assays

### Toxicology and safety testing

Tissue damage gene expression

Predesigned TaqMan gene expression arrays

Custom TaqMan gene expression arrays

### Off-target phenotype screening

Applied Biosystems<sup>™</sup> TaqMan<sup>™</sup> Array Human Inflammation Panel

TaqMan human DNA damage and repair panels

### Confirmation of successful gene transfer

TaqMan genotyping assays

BigDye Sanger sequencing reagents

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