Gene Therapy Solutions

Drug Development Continuum

Addressing Needs Across the

Discovery

Design & Create

 Gene Knock-in/out Pluripotent stem cells

Exploring

response

T-cell response

Target potential Off-target effects

Innate immune

Confirm targeting CAR-T construct Confirming targeting Biodistribution

Optimizing

Optimizing design

Profile for toxicities

Pre-Clinical

Development

Efficiency of delivery & transfection Safety & toxicity

- Characterizing immune response
- Combination therapy studies

Time to results

Manufacture

Therapeutics

Manufacturing/

Biomanufacturing

Analytical Testing

Potency Dosing

- Characterizing the T-cell response

Efficient workflow Modernized Analytics

• Robust and Reproducible

- Cell quality/purity Sterility/Contamination

Ph I / Ph II / Ph III • Safety, Efficacy,

Human Testing

Clinical trials

Clinical

Response

Development

Profiling Response

Characterizing

innate &

Characterizing response to treatment

- adaptive response

Post Market

Monitoring

Monitoring for

Pharmacovigilance

adverse events

Monitoring

Durability

Toxicities

Navigating The

identification

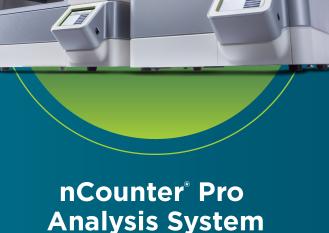
Quality & Consistency

Product Functionality & Efficacy

Regulatory Compliance

Assay Modernization





in a single tube.

800+ plex pathway-based

gene expression analysis

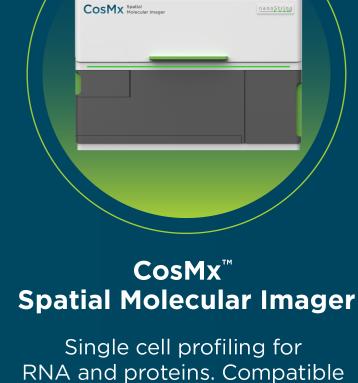
Dosing/Safety/ Toxicity

Many Challenges



Three Platforms. Unlimited Potential





Testing • Improve time to results

• Characterize early and

drug development.

Consolidate &

concurrently throughout

Modernize

- customize assays

Achieve consistent in-process results Design manufacturing efficiencies for speed • Develop menu of assays using reliable platform

Improve

Product

Quality

- Fully automated workflow

nCounter® Pro

Fully automated workflow for rapid analytics

with a single FFPE or Fixed/Fresh Frozen tissue section. Characterize upstream and

downstream of manufacturing • Identify the right

drug target & candidate

of response, and safety

biodistribution and MOA

• Discover biomarkers

Accurately measure

- with spatial platforms

Simple High Quality

• 24 hour sample

multiplexing

State of

Enables a

21 CFR Part 11

Environment

to data, 15 minutes

lab time, and true

the art encryption standards

> **Protect User Data**

Data Integrity

• Reproductible

• Limit of

detection:

15 zeptomole

with over 6 logs

dynamic range

The nCounter Pro has

multiple systems in place

that help ensure the

integrity and security

of your data.





Standardized

Easy Networking

Controlled

Access

Cell Quality Sterility Dosing Tansfection Purity Contamination Potency Confirmation Diversity nCounter® Stem Cell Characterization Panel ISP, Mesenchymal Tissue Specific

Differentiation

Status

Modernize With Molecular Characterization

Standardized Purpose-built Panels For Assay Development

nCounter® CAR-T Characterization Panel

Allogenic-Autologus CAR-T Cell Therapies

Sterility

Contamination

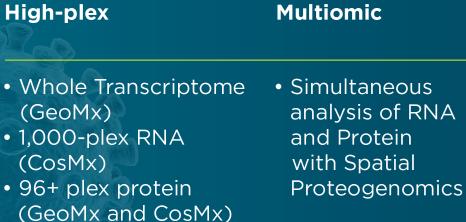
nCounter ® Gene Therapy Optimization Panel



Viral and Non-viral

Pluripotency

GeoMx Digital Spatial Profiler & CosMx Spatial Molecular Imager



Morphologically resolved data

Catalyze the next revolution Using Spatial Biology

Toxicity TCR

Lineage

Specification

Integration

Viral Viral

Stemness

Secretion



• Single cell and subcellular resolution • Receptor-ligand and 1-10 cells (CosMx) Process up to 40 slides/week

Multicellular

resolution:

(GeoMx)

20-200 cells

 Phenotyping • Transgene detection in cellular and subcellular context

Applications

Biodistribution

Biomarker Discovery

• Treatment Response

Transfection Efficacy

cell-cell interactions

Toxicity and Safety



Specialized Data



To learn



Translational scientist with expertise across all areas of Cell and Gene Therapy

more, visit nanostring.com

nanoString