

# Cell & Gene Therapy Solutions

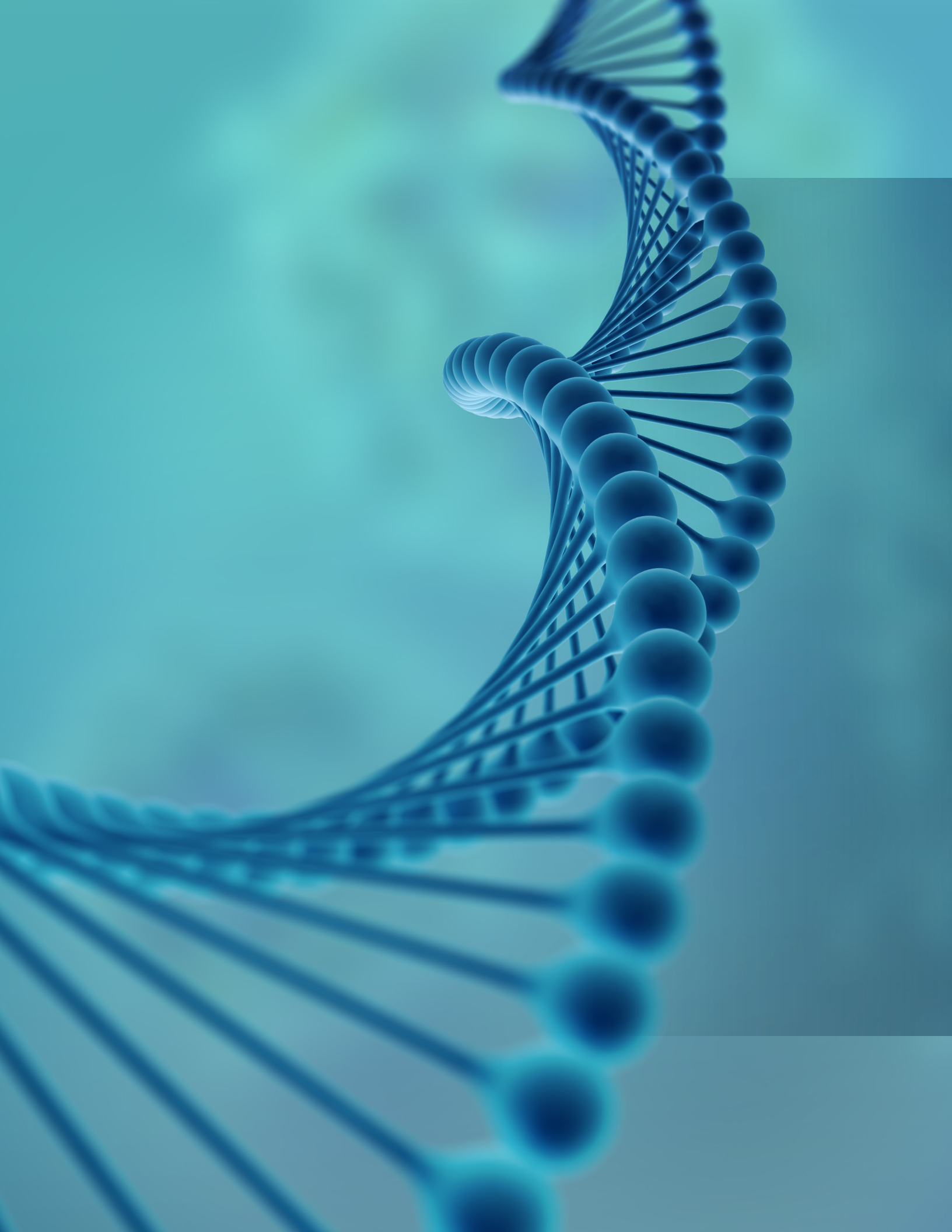
CAR-T • Stem Cell • Gene Therapy



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—●●●●●—<sup>®</sup>



# Cell & Gene Therapy

## Powerful Tools to Accelerate Drug Development

Cell & Gene Therapies (CGTs) are a transformative class of medicines addressing treatment needs for cancer, rare diseases, neurodegenerative diseases, and regenerative medicine just to name a few. The field of CGT is rapidly advancing with widespread interest, dedicated research and investments in clinical trials.

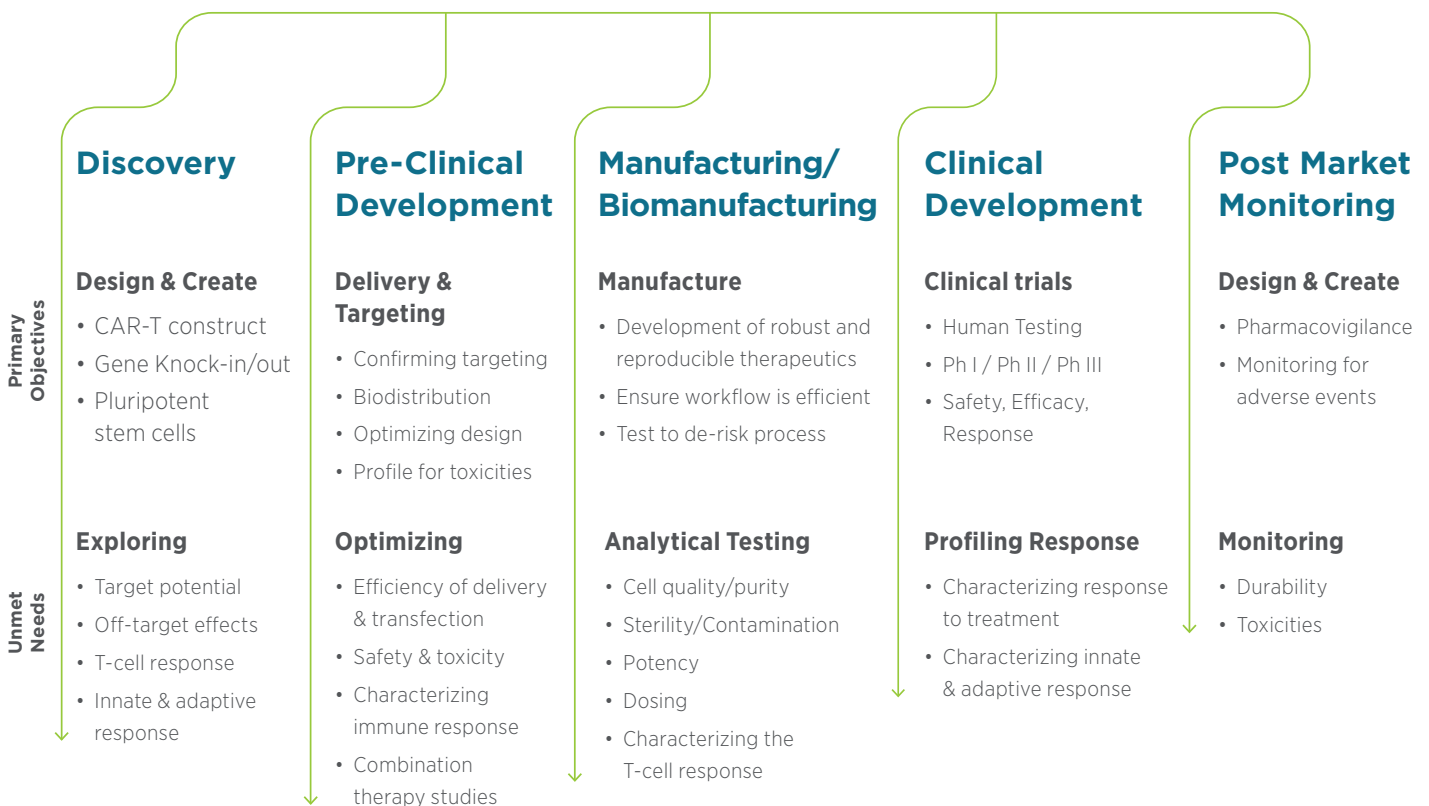
Although CGTs have made a significant and positive impact on healthcare, scientists are still facing marked challenges at every step of the process. Understanding the unique needs, addressing the challenges, and creating novel solutions is our passion at NanoString. Partnering with the community to ensure innovation, quality and reproducibility are woven into the entire process to ultimately improve the quality of life for those who endure the challenges of disease.

- Understanding needs across Discovery to Biomanufacturing to Clinical Trials
- Addressing challenges through collaborative innovation
- Identifying novel solutions that advance the field



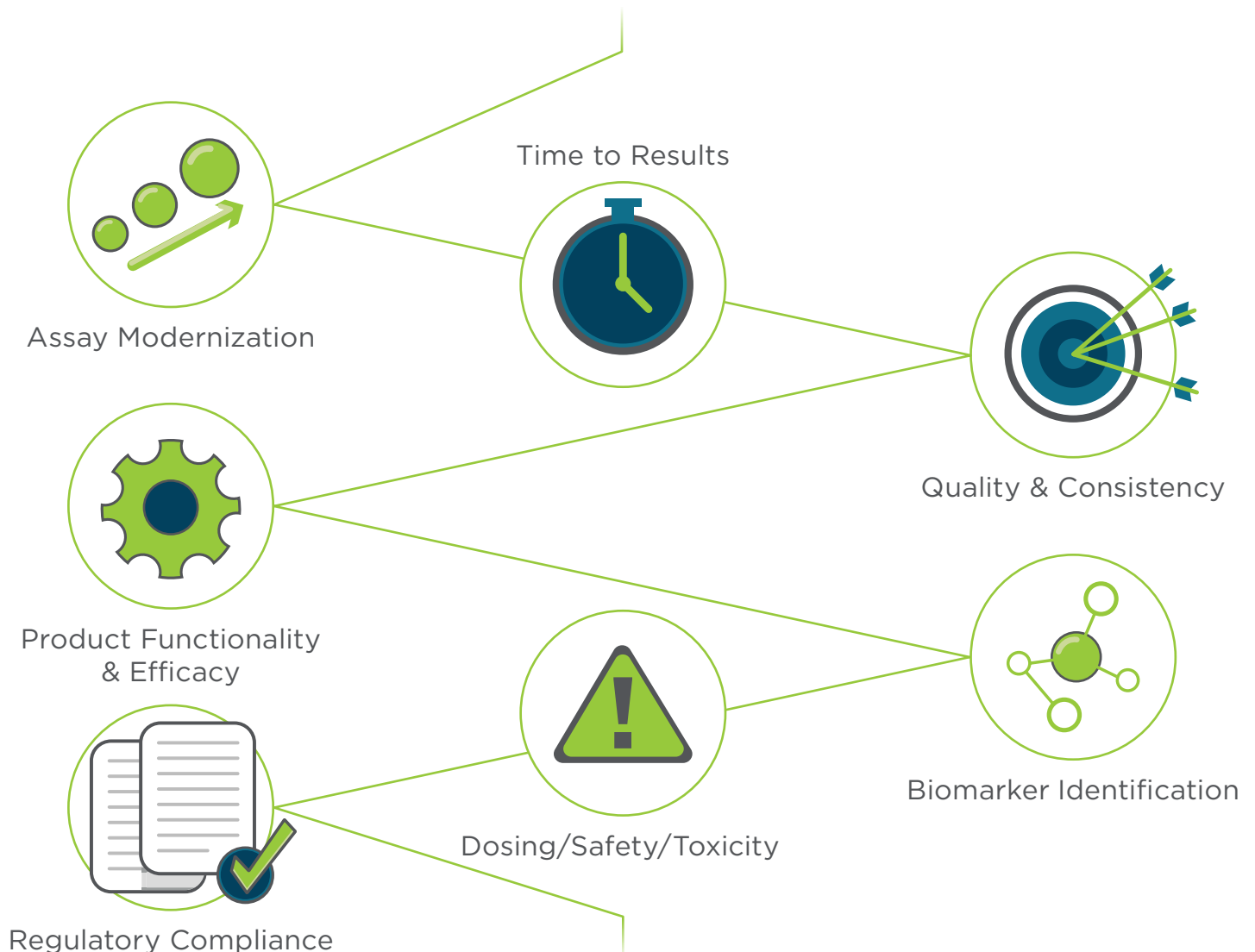
# Addressing Needs

Addressing the unique needs of each team of scientists involved in creating CGTs while keeping the bigger picture in mind we believe is a big part of what is needed to ultimately deliver quality treatments.



# Navigating Challenges

Understanding the myriad of complex challenges including those that fall outside of the scientific realm and taking on each challenge methodically is necessary to bring speed and efficiencies to the process.



# Creating Novel Solutions

## Three Platforms. Unlimited Potential.

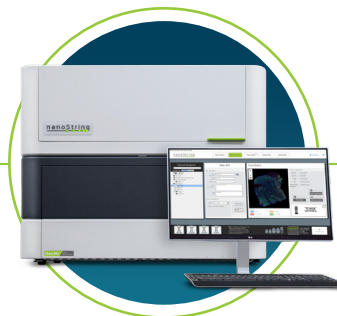
NanoString's integrated platforms and analytics can help tackle challenges and alleviate risks in the drug development process—from discovery to commercialization. Each platform with its uniquely

NanoString barcoded technology and distinct value supports the needs at each step in the process by providing biological insights and characterization on various scales.



### nCounter<sup>®</sup> Pro Analysis System

800+ plex Pathway-based gene expression profiling in a single-tube. Compatible with full spectrum sample types.



### GeoMx<sup>®</sup> Digital Spatial Profiler

Morphological, spatial context for whole transcriptome and proteomics experiments from a single slide.



### CosMx<sup>™</sup> Spatial Molecular Imager

Single cell profiling of RNA and Proteins with subcellular resolution from a single slide.

#### Modernize Testing

- Improve time to results
- Characterize early and concurrently throughout drug development.
- Consolidate & customize assays

#### Improved Product Quality

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

#### Characterize upstream and downstream of manufacturing

- Identify the right drug target & candidate
- Discover biomarkers of response, and safety
- Accurately measure biodistribution and MOA with spatial platforms

# Data Analytics

## Options for Discovery and Decision Making

Having access to a comprehensive range of analysis tools and services transforms your valuable data to bring insights, confirm specifications or deliver exciting results. With on-system and secure cloud-based data

analysis tools, expert bioinformatics support, and data analysis services, you can expedite analysis and accelerate discoveries.

### Rosalind/nSolver Cloud-based analysis tool

- Pathway Analysis
- Cell Type Profiling
- TCR Diversity
- Available at no-additional charge with some fee-based modules
- Secure platform with flexibility for internal or external collaboration
- Quality control

### Biostats Partnership NanoString R&D collaboration

- Project/workplan guided analysis
- Biomarker and signature development
- Utilizes extensive analysis toolbox & internal expertise

### Customized Reports Rosalind-NanoString collaboration

- Designed to specific request
- Biomanufacturing focused
- Single or multi-assay readout
- Locked report capabilities

### AtoMx™ Spatial Informatics Platform

- A cloud-based, integrated informatics ecosystem for spatial biology
- Share, collaborate, visualize, analyze, and store data anytime and anywhere
- Scalable data storage to manage large datasets
- Minimal investment in computing hardware & infrastructure
- Custom analysis modules the CosMx platform



# nCounter Pro

## nCounter Pro Analysis System delivers Speed and Quality data

The nCounter Pro technology incorporates a simple and quick workflow delivering highly reproducible data. The instrument incorporates advanced cyber security controls, audit trail and record maintenance, and is in compliance with 21 Code of Federal Regulations (CFR) Part 11. When combined with the purpose-built suite of standardized gene expression panels modernizing analytical assays and integrating characterization early and often throughout the process, nCounter provides value for the discovery and preclinical teams, biomanufacturing and translational biomarker researchers alike.

## nCounter<sup>®</sup> Pro Analysis System



### Simple

- 24 hours from sample to data, 15 minutes lab time, and true multiplexing



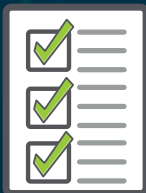
### High Quality

- Reproducible with over 6 logs dynamic range
- Limit of detection: as little as 1 transcript per cell in a 100ng sample



### Secure

- Enables a 21 CFR Part 11 environment
- State of the art encryption standards



### Standardized

- Pre-built panels provide a standardized tool for assay development
- Purpose driven content simplifies assay development

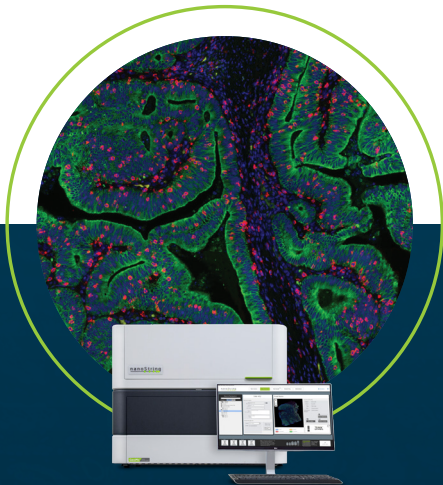


# Spatial Resolution

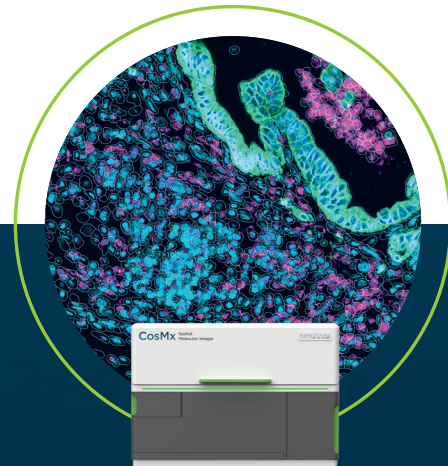
## GeoMx and CosMx Platforms address advanced Spatial Biology applications

NanoString's revolutionary spatial biology solutions combine high-plex and high-throughput spatial analysis of RNA and protein expression data at the multi-cellular, single-cell and sub-cellular level enabling essential breakthrough insights into important questions surrounding functionality and efficacy.

**GeoMx**<sup>®</sup> Digital Spatial Profiler



**CosMx**<sup>™</sup> Spatial Molecular Imager



### High-plex

- Whole Transcriptome (GeoMx)
- 1,000-plex RNA (CosMx)
- 96+ plex protein (GeoMx and CosMx)

### Multiomics

- Simultaneous analysis of RNA and Protein with Spatial Proteogenomics

### Morphologically resolved data

- Multicellular resolution: 20-200 cells (GeoMx)
- Single cell and subcellular resolution 1-10 cells (CosMx)
- Process up to 40 slides/week with GeoMx DSP

### Novel Applications

- Biodistribution
- Biomarker Discovery
- Treatment Response
- Toxicity and Safety
- Transfection Efficacy
- Receptor-ligand and cell-cell interactions
- Phenotyping
- Transgene detection in cellular and subcellular context

# Gene Expression

## for Modernized Assay Development

Pre-built application specific panels provide the starting point for assay development and allow for standardized molecular characterization on the nCounter Analysis System.

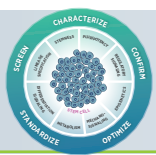
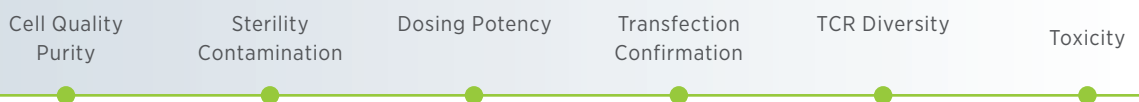
Standardized panels allow for early characterization and collaboration between discovery and biomanufacturing teams and into clinical trial follow-up. Capable of replacing outdated and time-consuming assays, nCounter gene expression panels bring efficiencies to the challenging workflow by consolidating multiple cumbersome assays to the single platform, introducing speed and robust reliability that ensures quality and performance.

- Each pre-built panel has been designed to address important biological questions for comprehensive product characterization.
- Available in human and mouse versions suitable for all aspects of the development process.
- Customizable with a Panel Plus spike-in with up to 55 user defined genes, transgene inserts or microbial genes for detecting contamination.



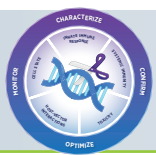
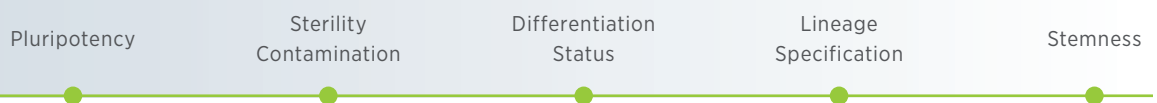
### CAR-T Characterization

#### Allogeneic-Autologus CAR-T Cell Therapies



### Stem Cell Characterization

#### IPS, Mesenchymal Tissue Specific



### Gene Therapy Optimization

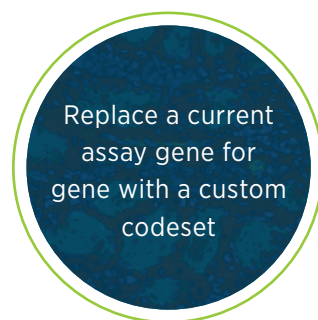
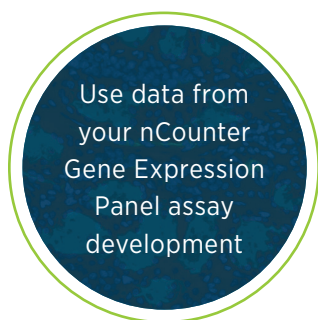
#### Viral and Non-viral



# Custom CodeSets

## for Refined Assay Solutions

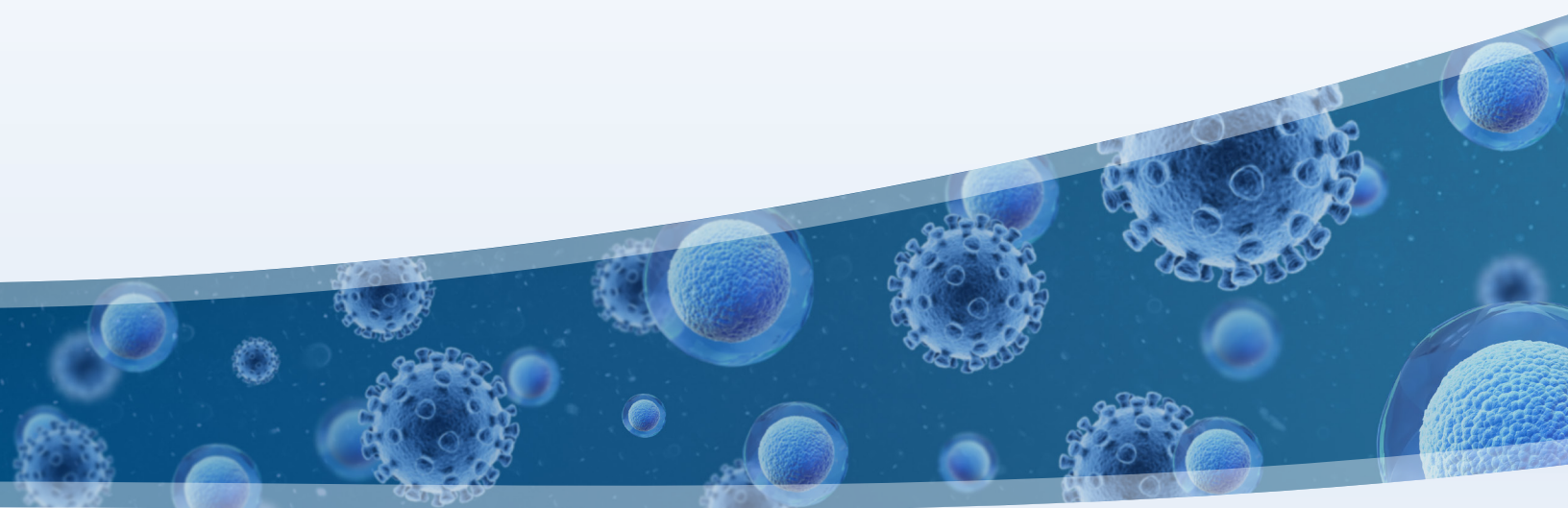
Design your own multi-plex custom or high throughput assay. With help from NanoString's bioinformatics scientists you can create small gene sets for single-use studies or ongoing in-process analytical testing.



<b>Custom CodeSet</b>	Refine and create your final assay with individual custom panels targeting as little as 20 genes or as many as 800.
<b>PlexSet™ Reagents</b>	Design high throughput assays plexing 6-96 genes with up to 8 samples per well for up to 9,216 data points per run.
<b>Panel Plus</b>	Customize any nCounter panel with up to 55 user defined genes.

## Assay Development

Contact us today to find out how we can support your projects and advance your therapeutic programs. Visit [nanosttring.com](https://nanosttring.com)



For more information, please visit [nanosttring.com](https://nanosttring.com)

**NanoString Technologies, Inc.**

530 Fairview Avenue North  
Seattle, Washington 98109

T (888) 358-6266  
F (206) 378-6288

[nanosttring.com](https://nanosttring.com)  
[info@nanosttring.com](mailto:info@nanosttring.com)

**Sales Contacts**

United States [us.sales@nanosttring.com](mailto:us.sales@nanosttring.com)  
EMEA: [europa.sales@nanosttring.com](mailto:europa.sales@nanosttring.com)

Asia Pacific & Japan [apac.sales@nanosttring.com](mailto:apac.sales@nanosttring.com)  
Other Regions [info@nanosttring.com](mailto:info@nanosttring.com)

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