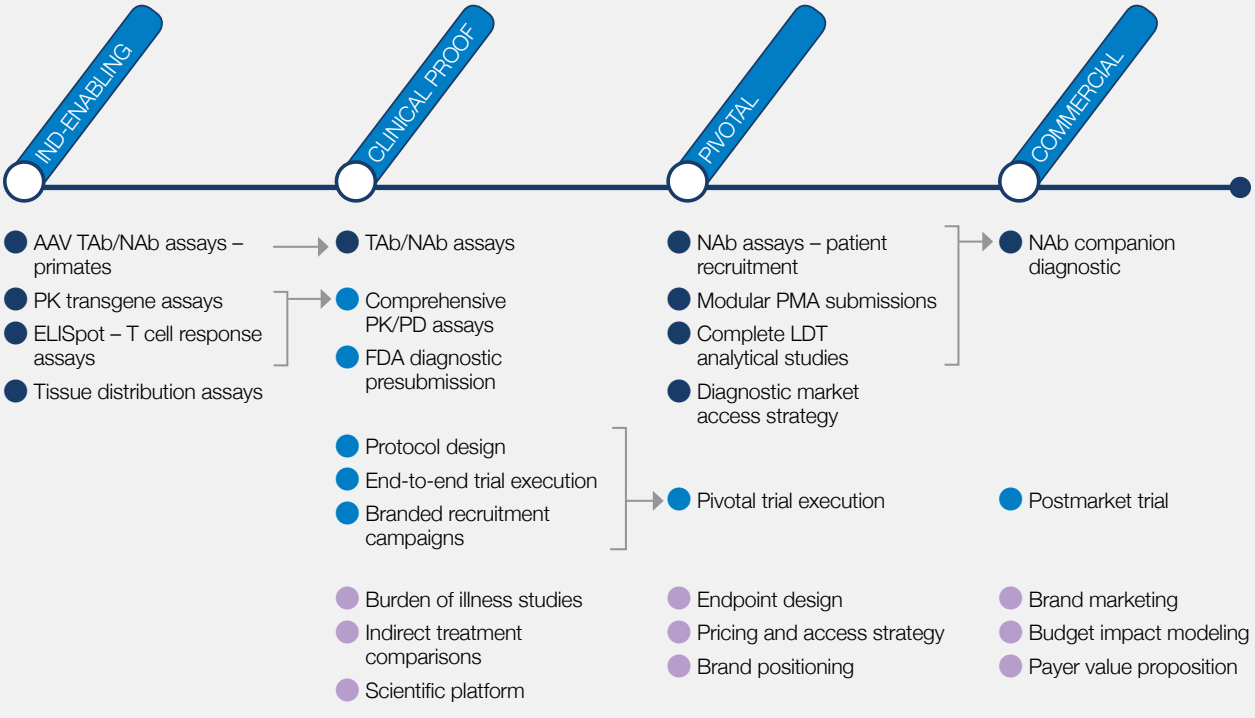
PRECISION for medicine

Gene Therapy Solutions

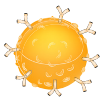
Successfully delivering a gene therapy to market requires an integrated approach, scientific expertise, and the right partner. With leadership in immunogenicity, a regulatory team that coordinates therapeutic and companion diagnostic strategy, and a focus on executing end-to-end clinical trials in oncology and rare disease, Precision is positioned to guide successful gene therapy development.

Delivering evidence at every critical inflection point



Biomarker Assays

Leadership in immunogenicity and biomarker assays



T cells

B cells

Viral Vectors

Gene therapy development requires specialized testing capabilities. Precision provides comprehensive capabilities to execute assays from preclinical through clinical trials and can execute assays under all relevant quality systems.

- Characterization of T cell responses via ELISpot and Intracellular Cytokine Staining (ICS)
- Characterization of binding and neutralizing antibodies to AAV serotypes
- Cell-based NAb screening for study enrollment (human and NHP) and commercial CDx support
- Tissue distribution of viral vectors via ddPCR

Custom assay development, validation, and global sample testing under any quality system (GxP, CLIA, ISO)

Advanced informatics to support analytical methods, long-term assay performance tracking, and agency-compliant data sets

Comprehensive sample protection global infrastructure

- Custom kitting
- Transport management
- 24/7 online tracking and reporting
- Processing and storage of any tissue type
- Strategically created lab infrastructure to enable global real-time processing with uniform quality

Real-time sample processing from
55+
COUNTRIES

Samples managed:
25
MILLION

Cell and nucleic acid isolations:
~2
MILLION

Global Clinical Trials

Full support for Rare and Orphan Disease trial execution

Rare disease trials target small patient populations spread over a wide geographic area. Such unique challenges require an experienced team.

- A targeted patient-centric approach
- Regulatory excellence in program development and agency engagement
- Statistical expertise for small populations and complex endpoints
- Masterful clinical execution meeting the unique challenges of rare disease

ROAR AWARDS

Winner of the ROAR Awards "Best Orphan Drug CRO" for 2 consecutive years.

100%

DATA SUBMISSION ACCEPTANCE

Unparalleled expertise in rare and orphan disease

EXPERIENCE WITH

OVER **50** RARE DISEASES

OVER **100** ORPHAN PROJECTS

α1-Antitrypsin deficiency (A1ATD)	Mitochondrial Diseases
Light Chain Amyloidosis	Multiple Myeloma
Alport Syndrome	Multiple Sclerosis
Amyotrophic lateral sclerosis	Netherton Syndrome
Calciphylaxis; Uremic Calciphylaxis	Niemann-Pick Type C1 Disease
Cushing Syndrome	Paroxysmal Nocturnal Hemoglobinuria (PNH)
Cystic Fibrosis	Phenylketonuria (PKU)
Dravet Syndrome	Polycythemia Vera
Duchenne Muscular Dystrophy	Prader-Willi Syndrome
Eisenmenger Syndrome	Primary Lymphedema
Eosinophilic Esophagitis	Primary Biliary Cirrhosis, Primary Sclerosing Cholangitis
Fabry Disease	Primary Hyperoxaluria
Fatty Acid Oxidation Disorder	Pulmonary Arterial Hypertension (PAH)
Friedreich's Ataxia	Raynaud's Phenomenon; Systemic Sclerosis
Gaucher Disease	Retinal Vein Occlusion; Uveitis
Growth Hormone Deficiency	Stargardt Disease
Hereditary Inclusion Body Myopathy	Valley Fever
Hereditary Angioedema (HAE)	Wilson's Disease
Idiopathic Pulmonary Fibrosis (IPF)	X-Linked Myotubular Myopathy
Macular Degeneration	IL-18 Driven Autoinflammatory Conditions

Companion Diagnostics Development

End-to-end diagnostics and regulatory process experience

Emerging FDA guidance on companion diagnostic considerations has created the need to formulate a clear strategy for diagnostic development that supports therapeutic time to market.

Precision develops and executes strategies for assays, trials, and CDx together.

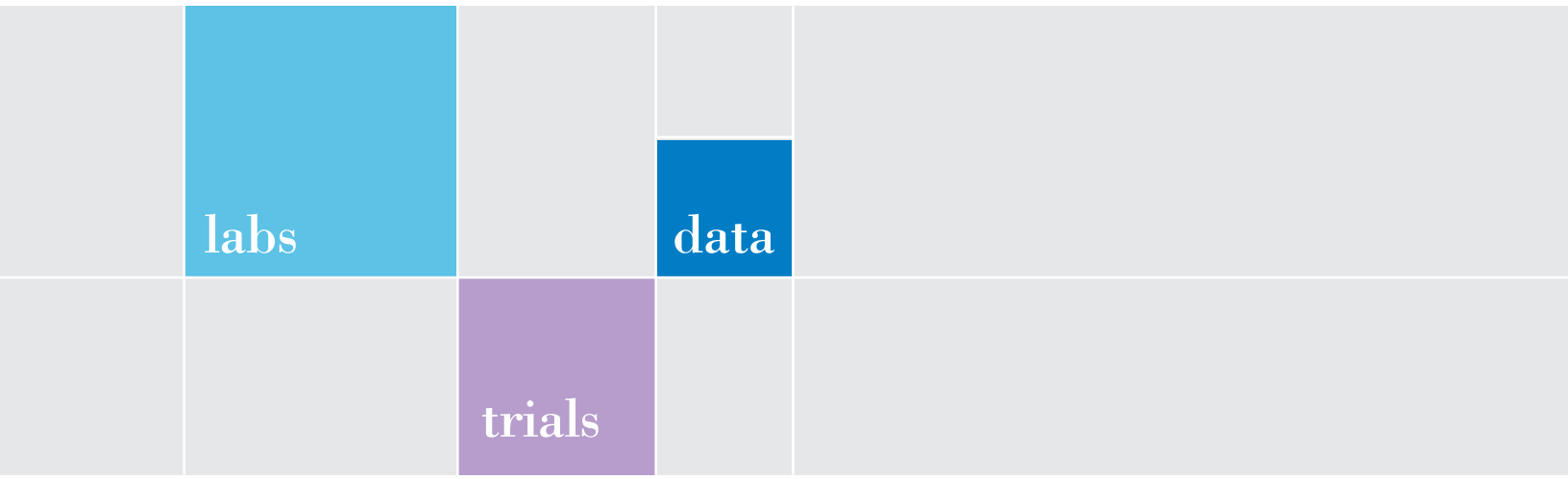
Fully compliant (CLIA) cell-based NAb assays for AAV companion diagnostic and commercial use

CDx / LDT Development

- Over 20 strategies, including regulatory, clinical trials, quality planning, and market access
- Co-development timelines of treatment and CDx
- Expertise with assays for CDx

Modular PMAs

- 7 successful Modular PMAs for IVDs (6 in parallel)
- Over 100 PMA Supplements and Annual Reports, eg, postmarket studies, assay changes, software versions



To learn how Precision for Medicine can accelerate your trial, please contact us at info@precisionformedicine.com or visit precisionformedicine.com.

PRECISION for medicine
shift the curve

Rev. 02