
Enabling a Healthier World

Lonza
Cell & Gene

Integrated Solutions for Cell and Gene Therapy from Discovery to Manufacturing

Products and Services

2022

Public



Golden road in surreal colorful forest

Lonza is the preferred global partner to the pharmaceutical, biotech and nutrition markets.

We work to enable a healthier world by supporting our customers to deliver new and innovative medicines that help treat a wide range of diseases. We achieve this by combining technological insight with world-class manufacturing, scientific expertise and process excellence.

Our unparalleled breadth of offerings enables our customers to commercialize their discoveries and innovations in the healthcare sector.

~16,000

Full-time employees

125

Years of history

37

Global sites

We Are an Integrated Solution Provider

Products, services and support to accelerate your CGT workflow

Our vision

Accelerating your
path to success

Worldwide network

Guarantees worldwide distribution, short lead time and quick response

Scalable technologies

Products and services available from discovery to commercialization, at a variety of grades and scales

Shorten time-to-market

Working with one trusted, innovative and experienced partner from discovery to commercialization to prevent unnecessary iteration cycles

Attuned products

Products and services designed to seamlessly work together for de-risking your process and improved cost control

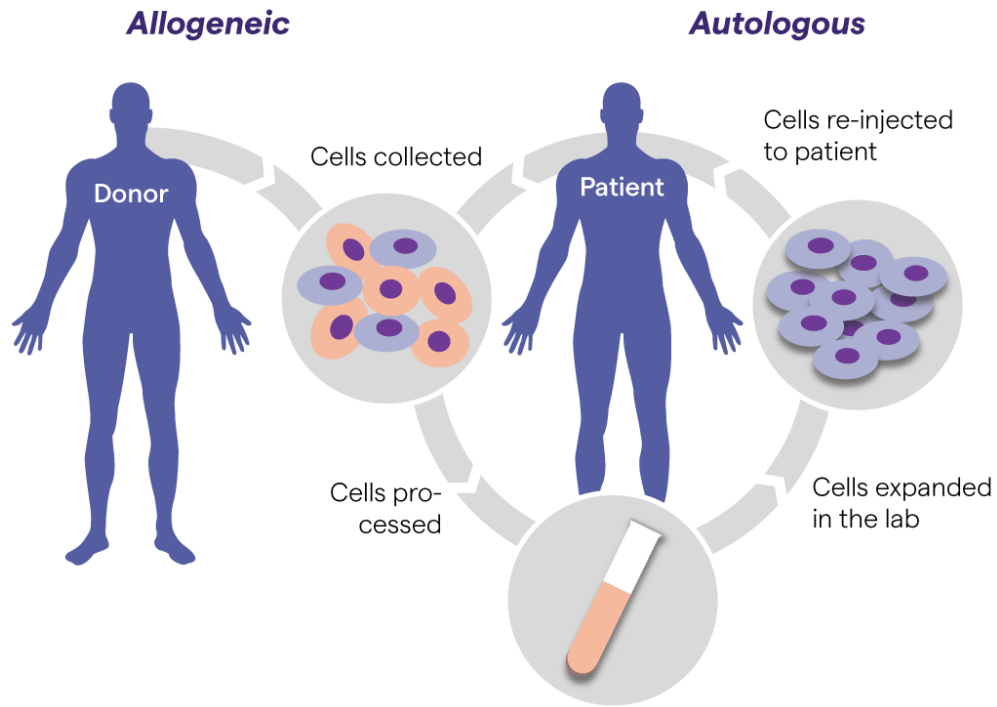
Outstanding support

Expertise in cell biology, transfection, process development, GMP manufacturing and regulatory requirements

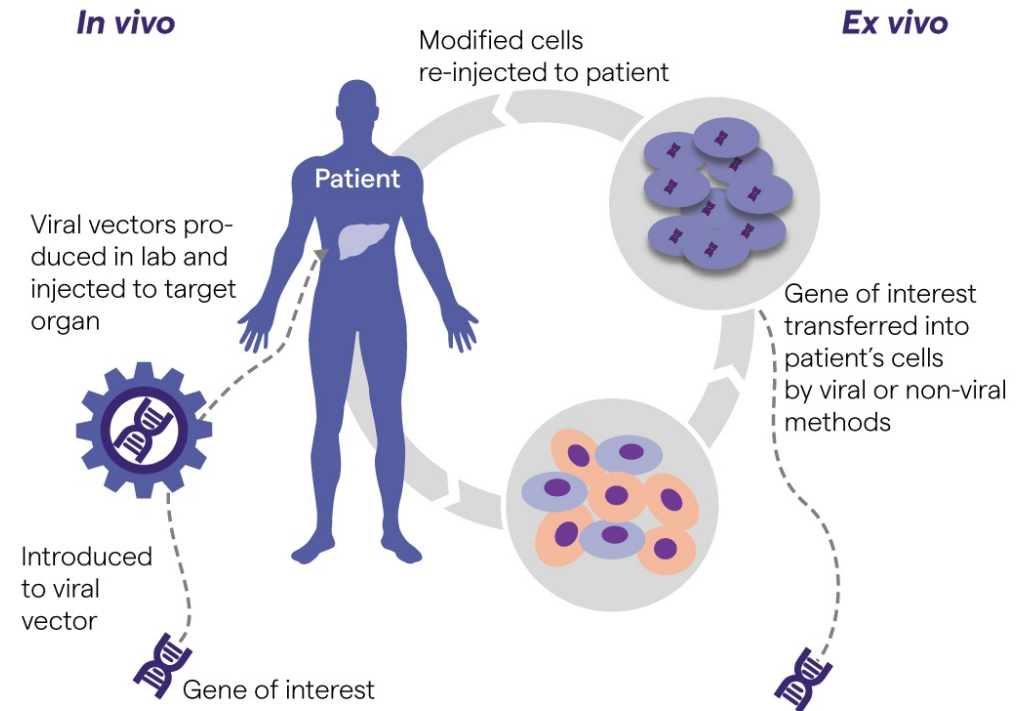
Cell and Gene Therapies Overview

Leading to “curative” therapies

Cell therapy Product = Cell



Gene therapy Product = Virus or gene modified cell to fix/replace faulty genes



The Key Challenges in Cell and Gene Therapies

Time to market, quality, quantity and costs



Discovery / translational

Research products working seamlessly together to accelerate development

Clinical trials

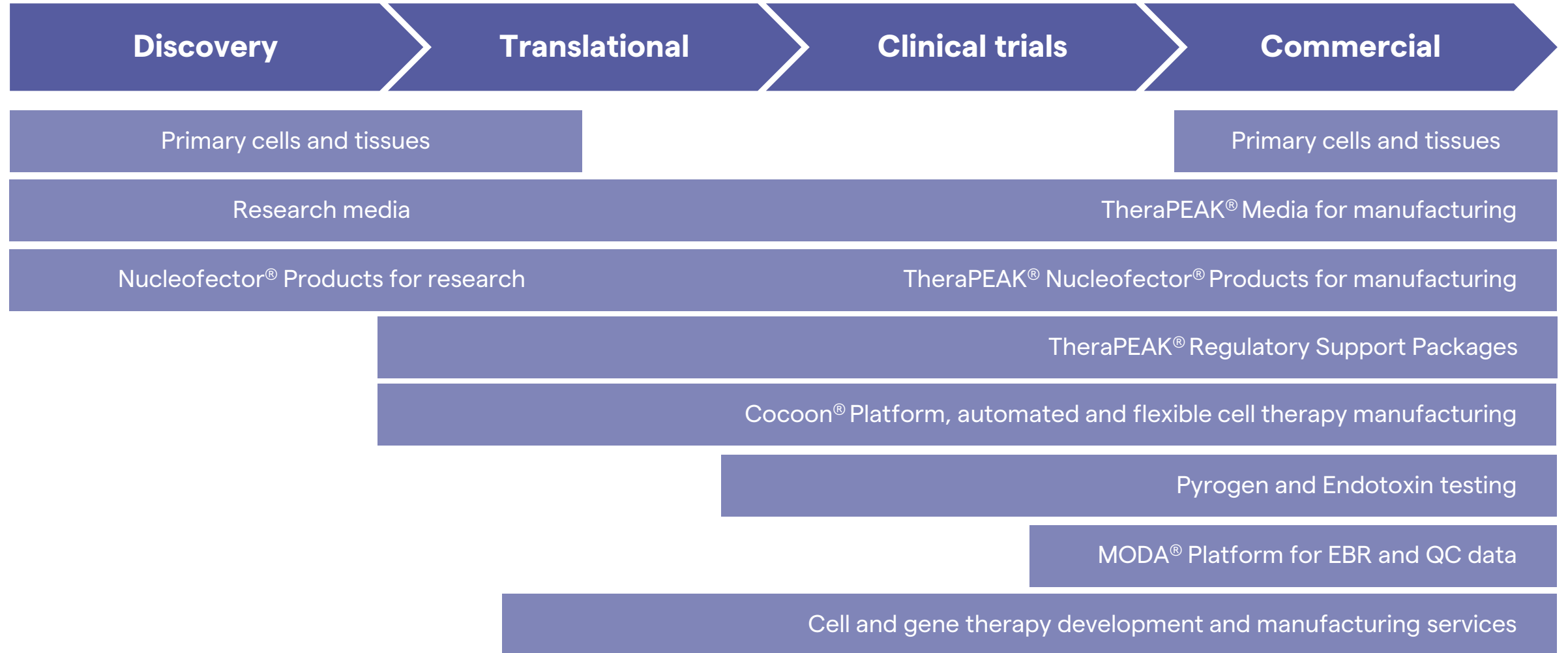
Product quality compliant with GMP manufacturing

Commercial

Easy scale-up solutions offered in various volumes and formats

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization

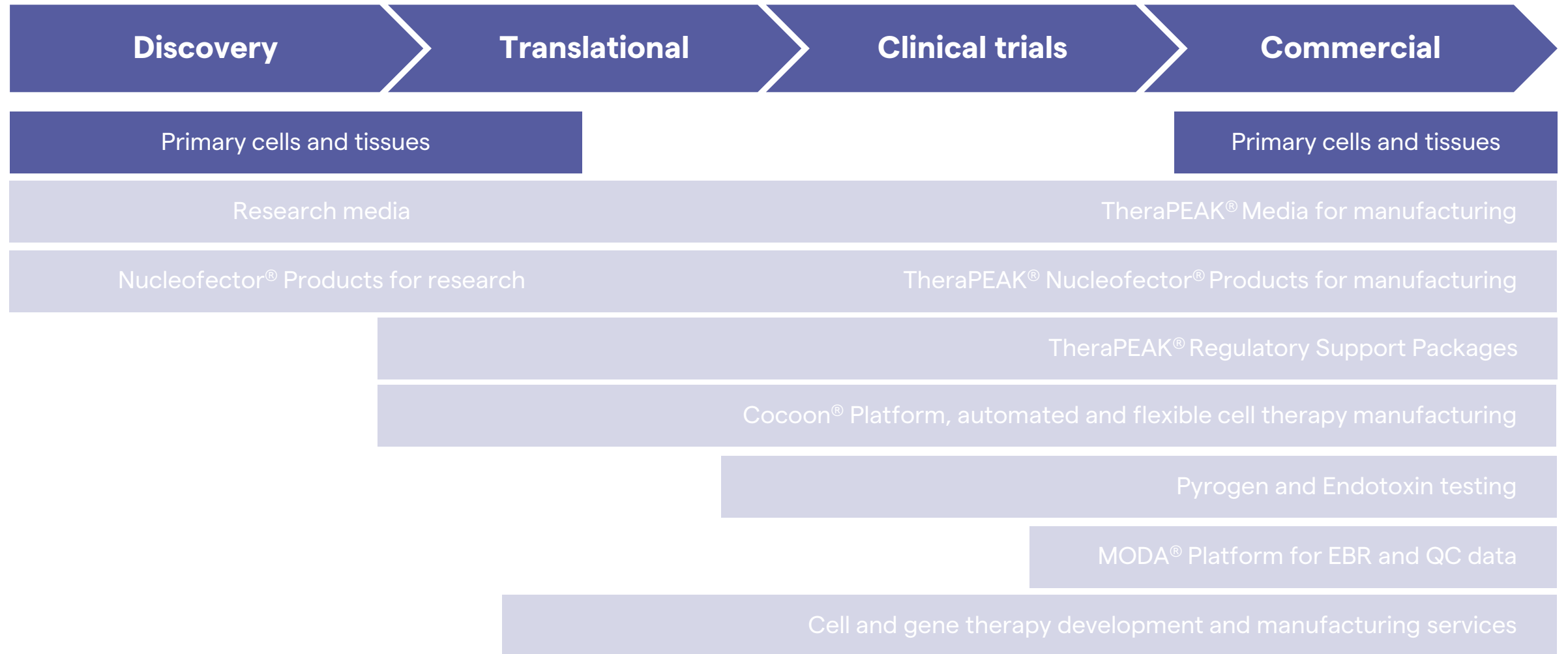


Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization

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Primary and Stem Cell Systems

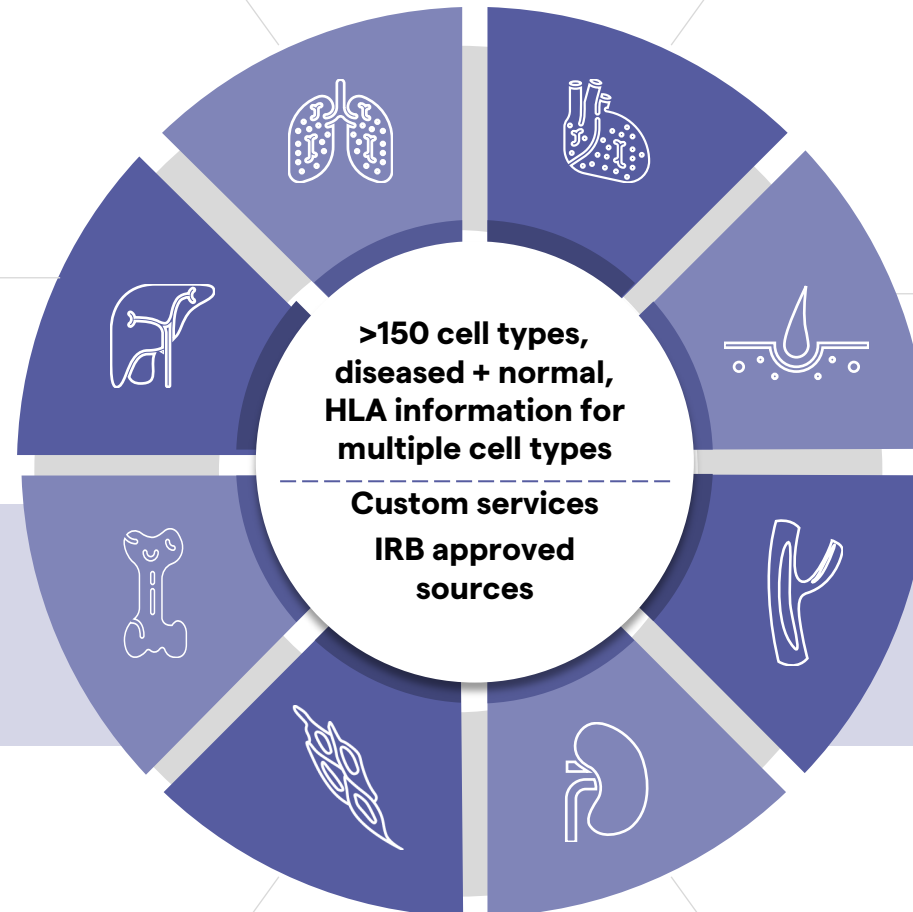
Broad portfolio offering with unmatched donor variety

- Bronchial epithelial cells
- Small airway epithelial cells
- Lung fibroblasts
- Bronchial smooth muscle cells

- Human/animal hepatocytes
- Kupffer cells
- Stellate Cells

- **(fresh) whole blood & bone marrow**
- **Mobilized blood and cells (CD34 & Co)**
- **Leukopaks (fresh and cryo)**

- Skeletal muscle cells



- Aortic endothelial cells
- Cardiac microvascular Endothelial cells
- Atrial cardiac cells
- Various fibroblasts from the heart and blood vessels

- Keratinocytes, melanocytes

- **T cells, B Cells, NK Cells, PBMCs, DCs, macrophages, monocytes, CD34+**

- Renal epithelial cells
- Cortical epithelial cells
- RPTECs

Primary and Stem Cell Systems

Broad portfolio offering with unmatched donor variety

Primary blood,
bone marrow,
and immune cells
for research

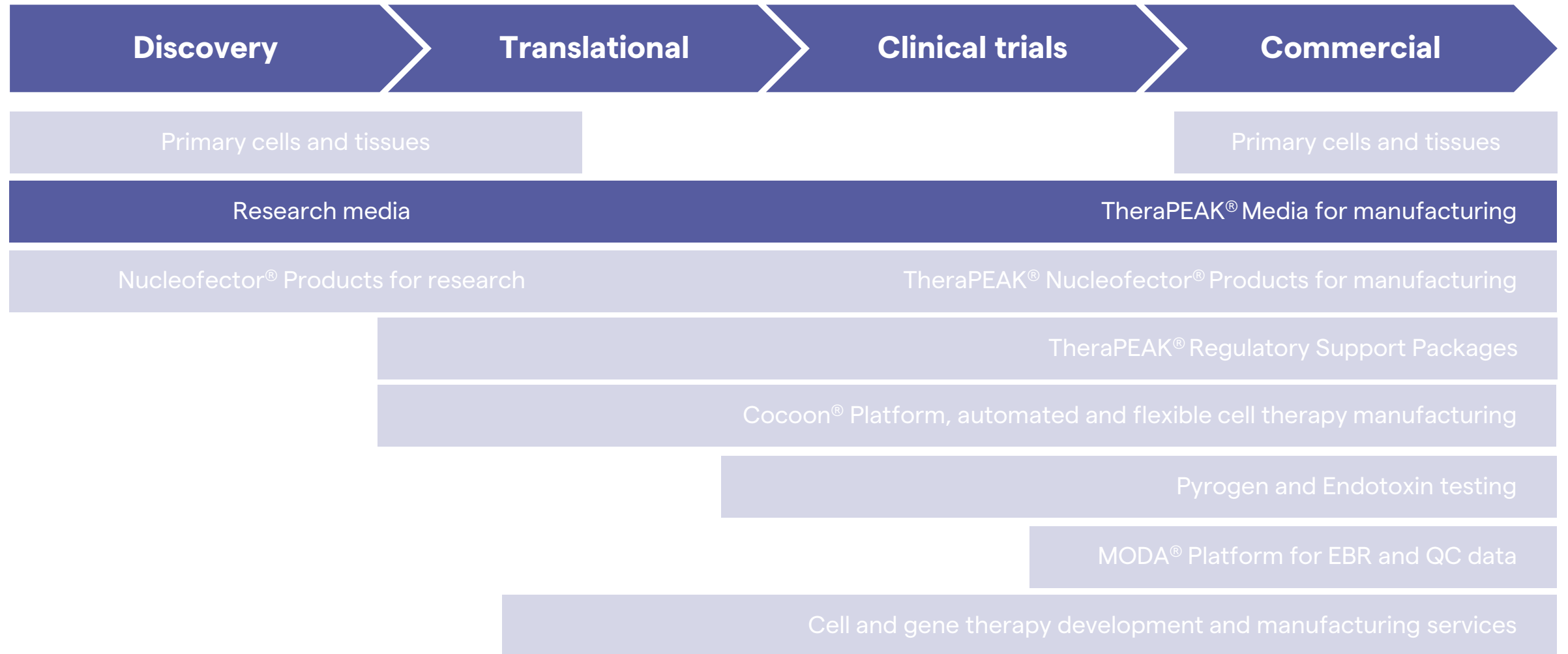
Fueling your next
discovery with
unmatched cell and
donor variety

- 1 Highest quality blood tissue, bone marrow tissue and cells plus custom services available to meet your specific needs
- 2 Unmatched donor and cell variety; HLA information for multiple cell types
- 3 X-VIVO® Serum-free Hematopoietic Cell Culture Media – with cell guarantee*
- 4 World-class scientific support team
- 5 *More than* cells – support for wide ranging workflows including transfection using Lonza's Nucleofector® Technology
- 6 Global supply reach
- 7 Full donor consent and IRB-approved collection facilities
- 8 Certificate of Analysis, SDS in multiple languages

* Lonza guarantees the performance of its primary cells only if appropriate Lonza recommended media and reagents are used exclusively and the recommended storage and use protocols are followed. Any modifications made to the recommended cell systems including the use of alternative media, reagents or protocols, will void cell and media performance guarantees. If you need assistance in selecting the appropriate media, reagents, or protocol, please contact Lonza Scientific Support.

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization

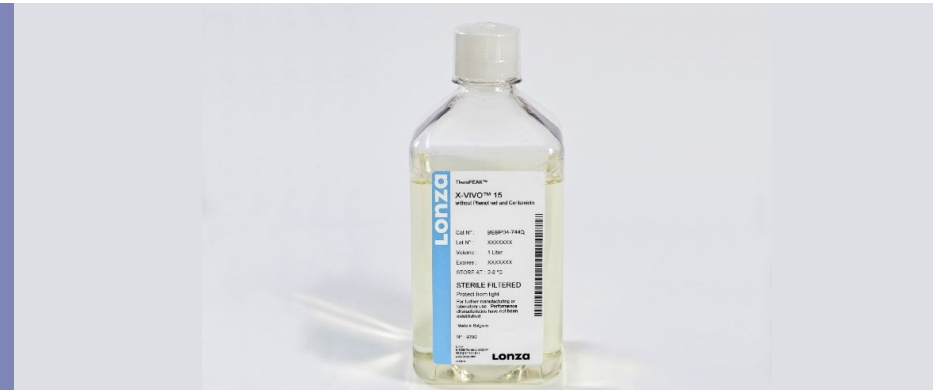


TheraPEAK® Medium and Reagents

GMP tools for cell and gene therapy applications

- > High performing
- > Serum-free
- > GMP produced media
- > Multiple media formulations for various cell and gene therapy applications

TheraPEAK® X-VIVO® Media Series



TheraPEAK® Media formulations are currently used in over 130 clinical trials globally

Product details

- Reduced variables with serum-free formulation
- Produced according to current GMP guidelines ensuring safe insertion to clinical processes
- Multiple formulations allowing seamless transition into customer specific processes
- Critical raw material in commercially FDA approved therapy

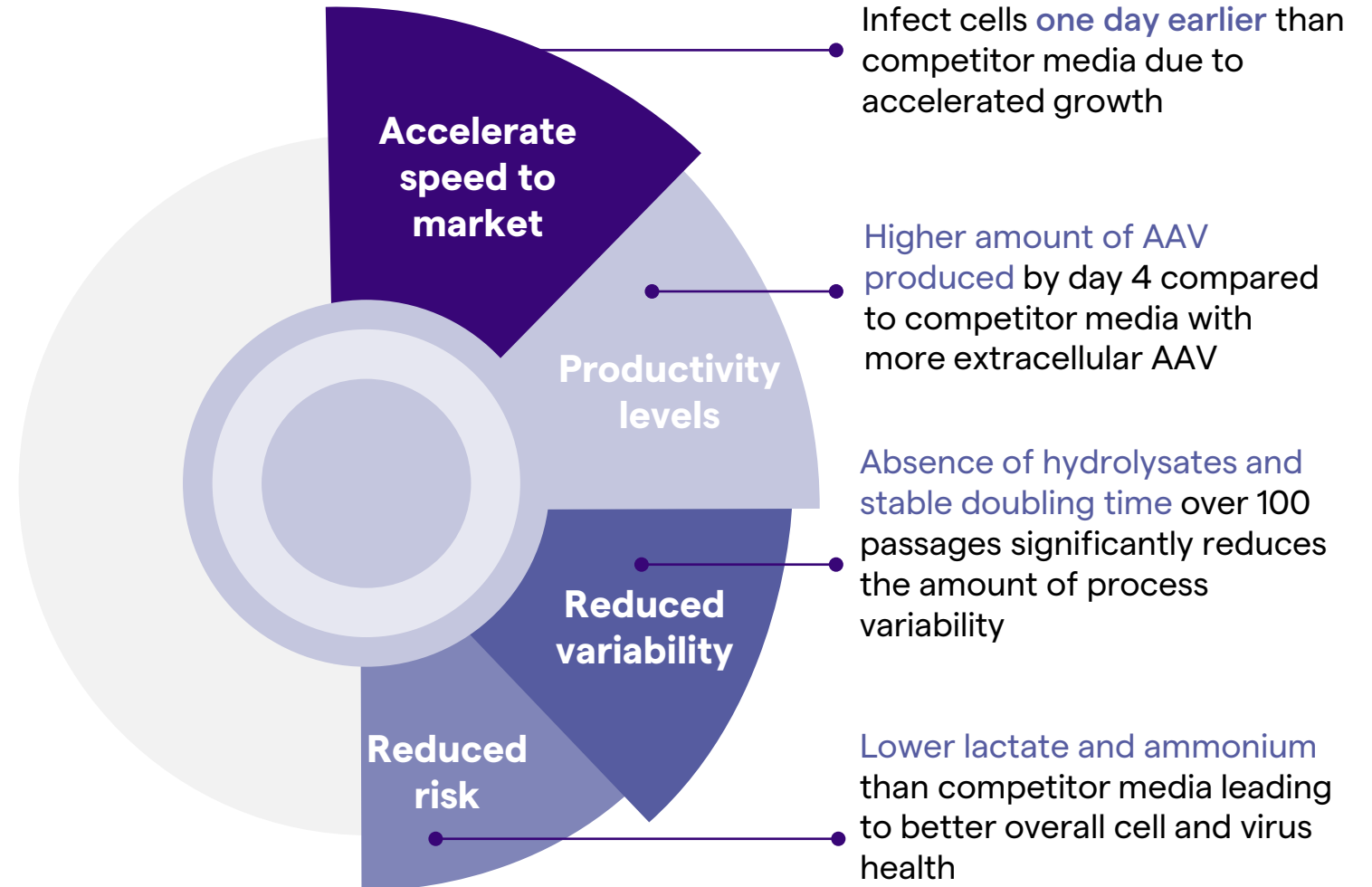
TheraPEAK® Medium and Reagents

GMP tools for cell and gene therapy applications

TheraPEAK® SfAAV® Medium

Serum-free, GMP produced medium for AAV production in Sf9 cells

- Chemically defined
- Non-animal origin
- Hydrolysate free
- Enhanced cell growth,
- Increased AAV production
- Easier downstream processing
- Drug master file soon available

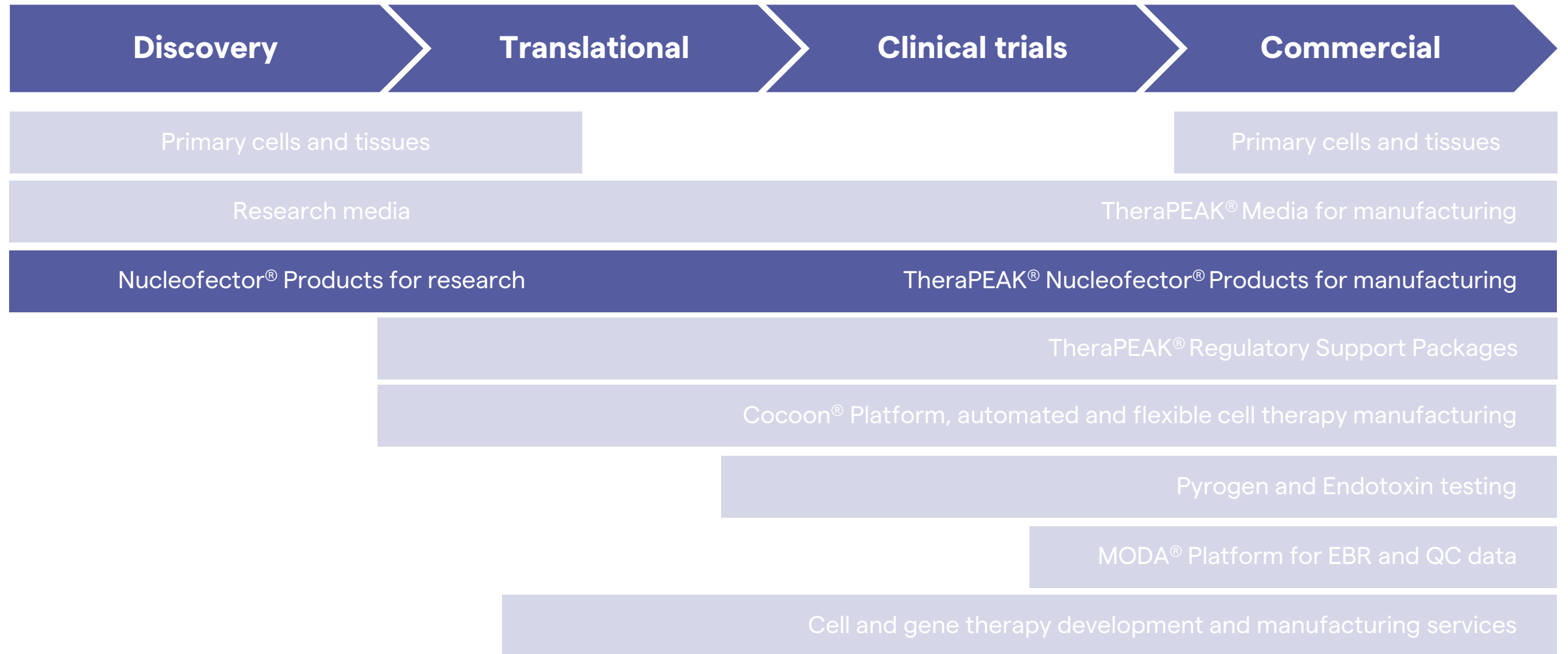


Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization

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Nucleofector® Technology

Scalable, non-viral transfection from research to manufacturing

Lonza

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State-of-the-art **electroporation** platform for **versatile and efficient** transfection and gene editing

More than **10,000 publications** using Nucleofector® Technology

Used in **leading labs** for *ex-vivo* gene therapy and CRISPR



X Unit

Ease-of-use
Intuitive operation software and predefined protocols

Research

Substrate variability
same conditions for DNA, RNA, RNPs

Unmatched scalability
from 1×10^4 up to 1×10^9 cells

Manufacturing

TheraPEAK® Consumables to support GMP manufacturing



LV Unit

4D-Nucleofector® LV Unit

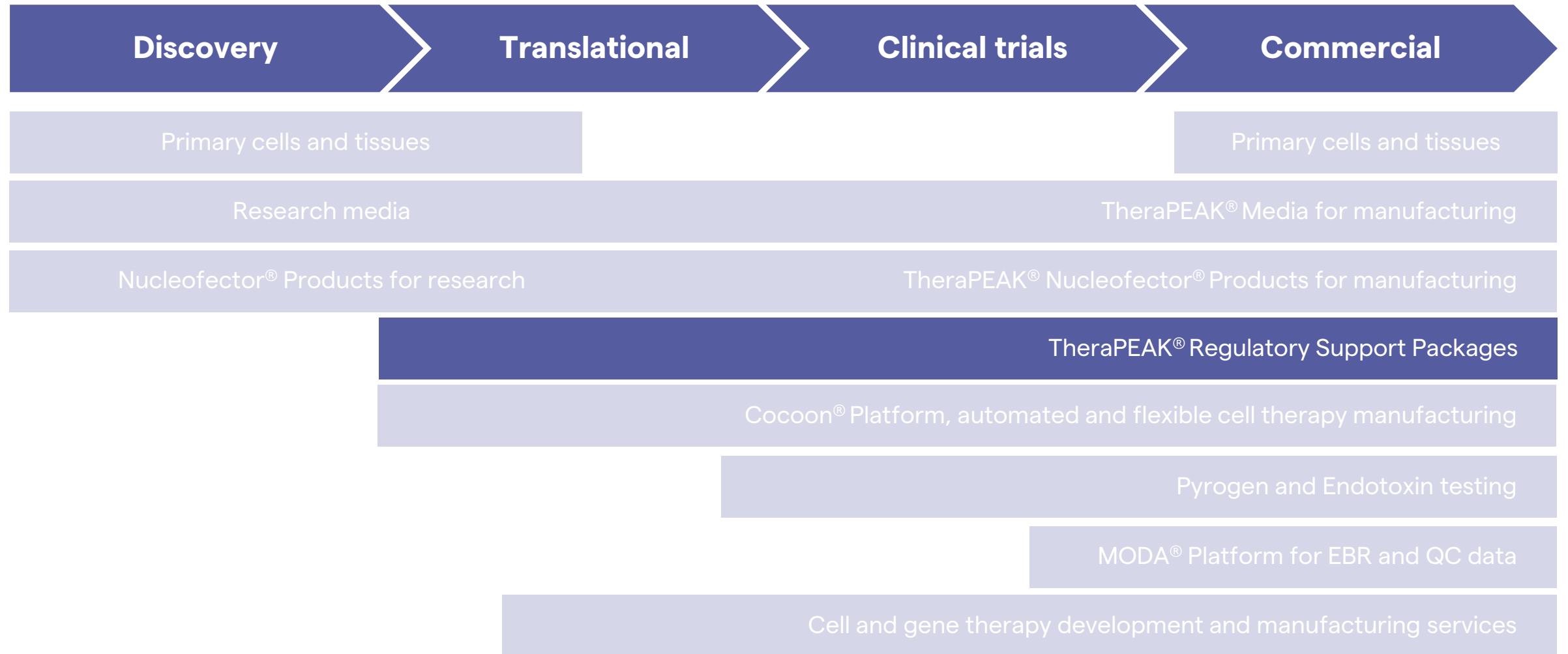
Supporting GMP manufacturing of autologous therapies



- 1 Based on efficient and robust Nucleofector® Technology
- 2 Allowing for **closed, scalable** transfection of **1×10^7 to 1×10^9 cells**
- 3 **Conditions transferable** from small scale units
- 4 Can be operated via **21 CFR Part 11** compatible software
- 5 IQOQ services
- 6 GMP grade TheraPEAK® Consumables
- 7 More than 5 early-stage clinical trials ongoing

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization



TheraPEAK® Regulatory Support Packages

Regulatory support along your journey from discovery to commercialization

Each TheraPEAK® Regulatory Support Package is a pre-developed information pack specific to the Lonza product used:

Packages available:

Level 1:
Available to all our customers

Level 2:
Beginning the clinical journey

Level 3:
Becoming a true partner

Included:

- Non-proprietary product information
- Basic change notifications
- High level company details for supplier qualification (paper audit)
- Authorization letter for Masterfile (if available)

- Critical raw material information
- Validation details
- Description of manufacturing and testing processes

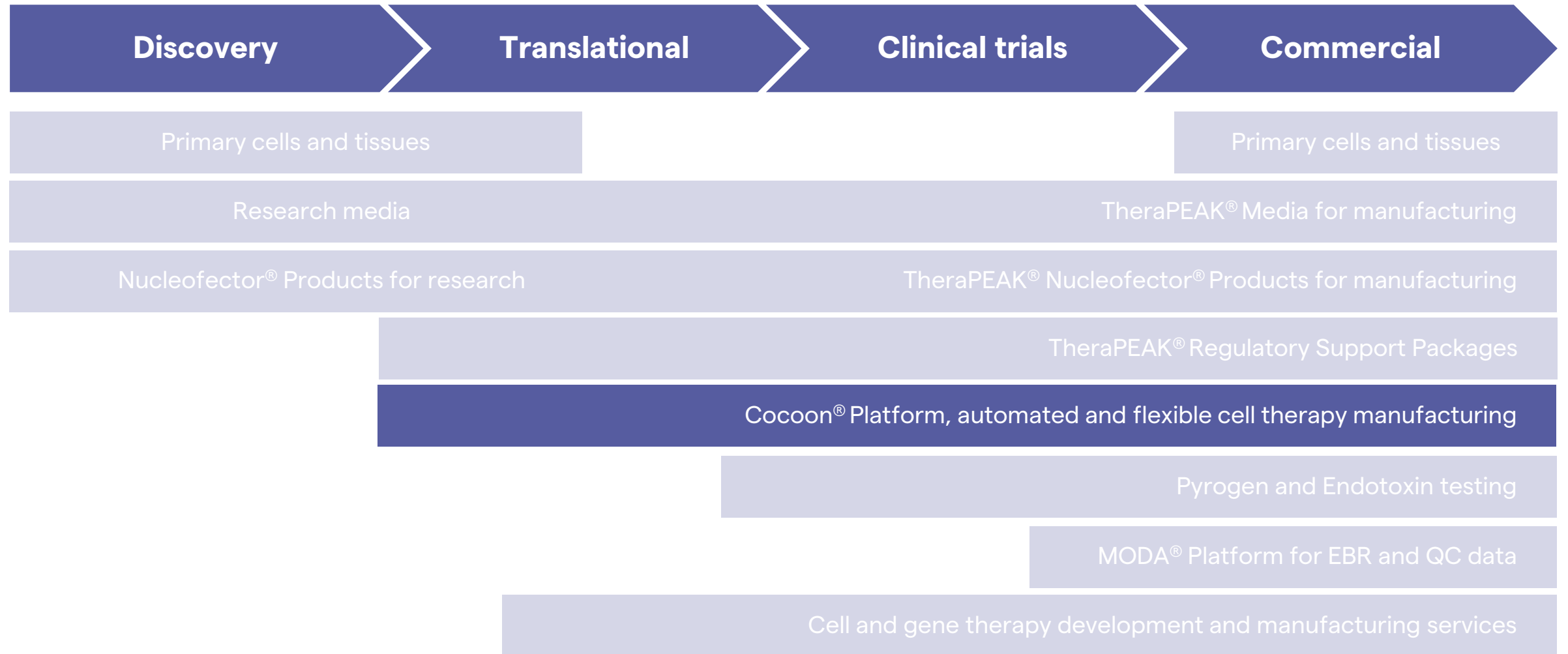
- Supply agreements and quality agreements (for custom products)
- Drug master file creation for local regulatory inconsistencies
- Customized support with regulator inquiries

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization

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Benefits of the Cocoon® Platform

Automated cell therapy manufacturing

> The Cocoon® Platform integrates multiple cell therapy manufacturing unit operations into a single consumable.



Integrated unit operations

> With the Cocoon® Platform, you can perform automated cell therapy manufacturing at a centralized location or at the point of care.



Functionally closed system



Superior scalability



In-process analytics

**Less labor intensive =
lower costs**

**High-quality product
manufactured every time**

**Easy tech transfer across
multiple sites**

**Reduced errors and
batch failures**

The Automated, Scalable Cocoon® Platform

Closed-process cell therapy manufacturing

1 Environmental unit

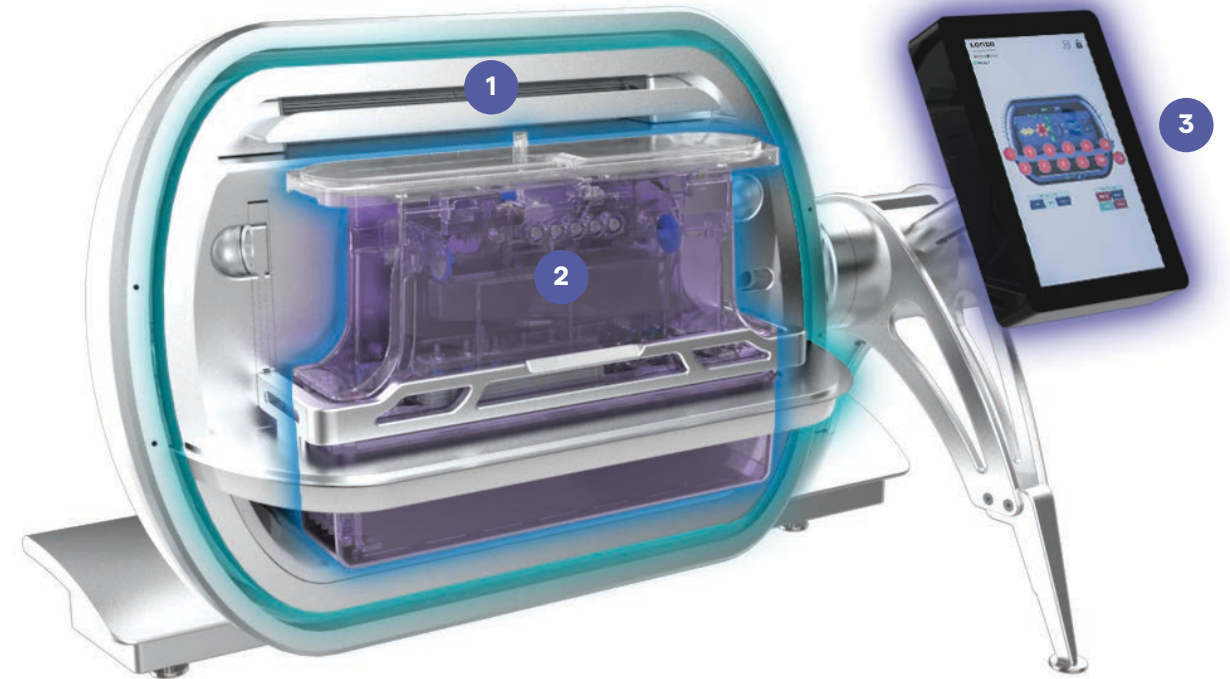
- Dual-zone temperature control (37°C & 4°C)
- Built-in bidirectional peristaltic pump
- Regulates CO₂ and Dissolved Oxygen
- Integrated magnet for cell enrichment

2 Single-use cassette

- Functionally-closed enabling closed-processing
- Process flexibility – suitable for suspension or adherent cells
- Integrated cold chamber (4°C) for internalizing all process reagents and consumables
- Apt for lenti- or gamma-retroviral transduction processes, as well as non-viral transfection with the 4D-Nucleofector® LV Unit

3 Cocoon® Software

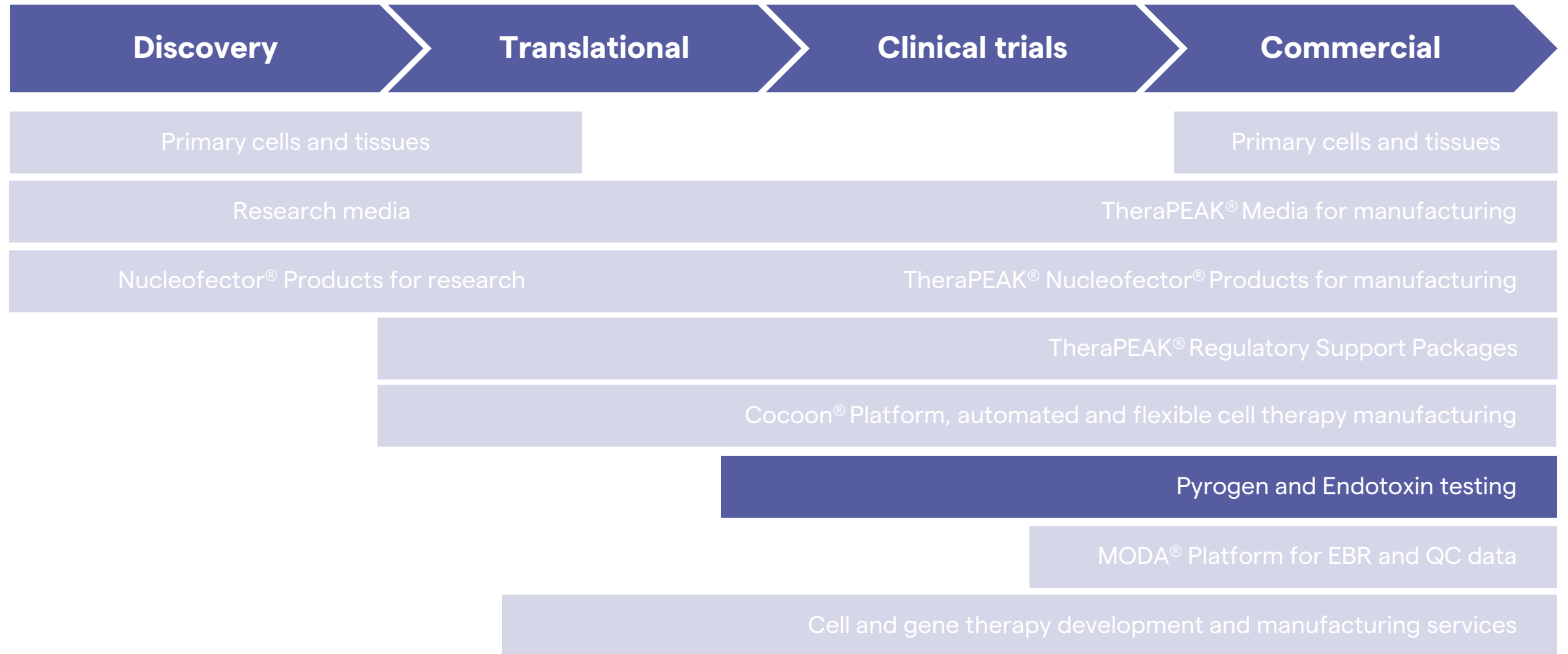
- Monitors and controls temperatures & gases
- Displays protocol design/control flow pathways
- Monitors in real-time pH/DO and performs in-process adjustments to maintain optimal culture conditions
- 21 CFR Part 11 & Annex 11 compliant, enabling product traceability/audit trails



*The Cocoon® Platform is qualified for clinical use including CE mark & FDA DMF.

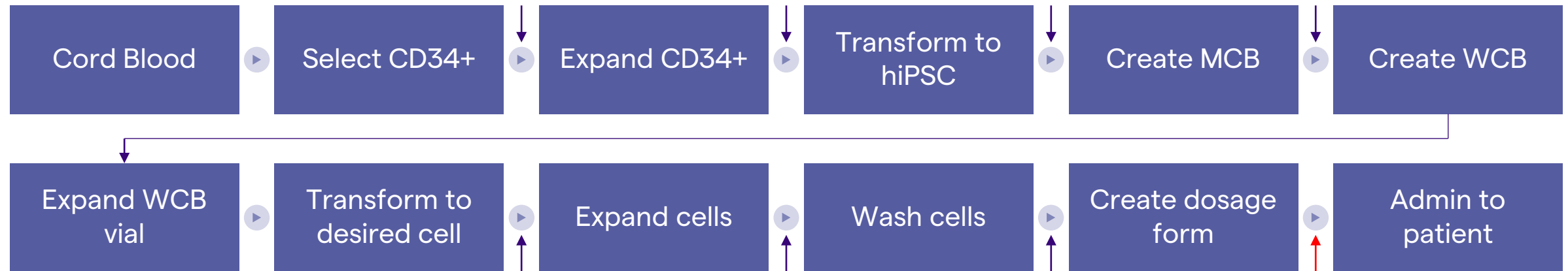
Integrated Solutions for Cell and Gene Therapy

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Pyrogen & Endotoxin Testing – Why and Where?

QC testing solutions for cell and gene therapy



01

Pyrogenicity and Endotoxin are quality attributes that must be monitored throughout CGT development.

- Raw materials (including water) must be tested for bacterial endotoxin contamination
- Pyrogenicity testing, either using tests for bacterial endotoxin or monocyte activation, is required prior to patient administration

02

Pyrogen and endotoxin contamination may have adverse effects on cell culture and patients.

- Cytotoxicity due to morphological changes and severe membrane damage
- Dependent on cell type and type of pyrogen
 - Little or no effect on skin cells
 - Large effect on hepatic cells

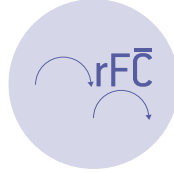
Pyrogen & Endotoxin Testing Platform

Complete testing solutions streamline QC to help ensure patient safety



Traditional Bacterial Endotoxin Tests (BET)

- PyroGent® Gel Clot Assays
- PyroGent® 5000 Turbidimetric Assays
- Kinetic-QCL® Chromogenic Assays



Sustainable Pyrogen & Endotoxin Tests

- PyroGene® Recombinant Factor C (rFC) Assays
- PyroCell® Monocyte Activation Test (MAT) Systems



Automation, Data Integrity & Accessories

- PyroTec® PRO Automated Endotoxin Testing System
- WinKQCL® Endotoxin Analysis Software
- Absorbance and Fluorescence Readers
- Consumables and Standards



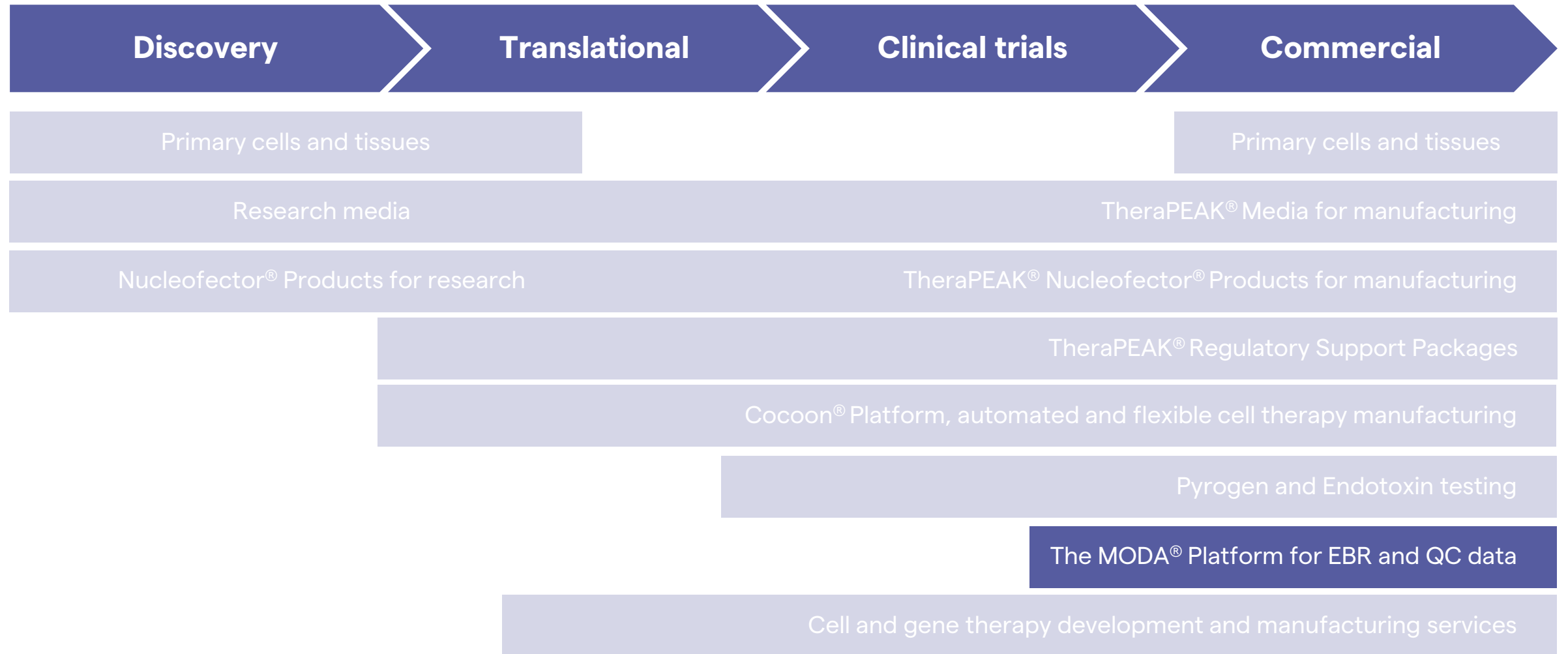
Expert Training & Support

- E-Learning Certification Modules
- QC Insider® Toolbox
- Testing Services
- Field Support Services
- Scientific Support
- Subject Matter Experts

Lonza has been providing endotoxin detection solutions and services since the 1970s

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization



The MODA® Platform – Digitalize CGT Quality and Manufacturing

More science. Less paper.®

Lonza

Cell & Gene



The MODA® Platform is built for CGT, to enable organizations to scale their manufacturing and enforce quality and compliance

MODA® helps gain complete **insight** into your manufacturing and QC operations.

MODA provides a cost-effective approach to accelerate implementation of an electronic batch record

The MODA[®] Platform – a Complete Solution to Manage Your Data

More science. Less paper.[®]

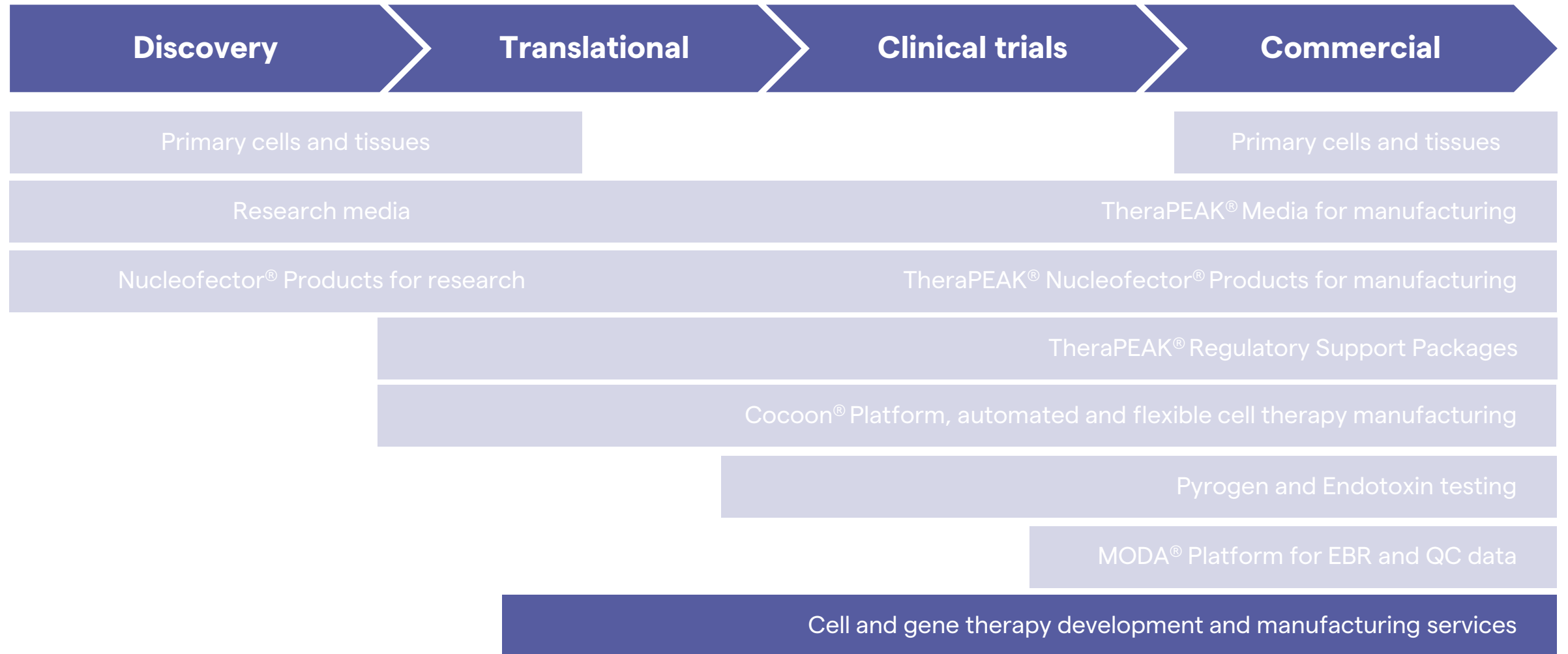


Track and Trace implementation for cell therapy manufacturing is the prerequisite to enable parallel processing and state of the art compliance for scale up of cGMP manufacturing.

- > Built for CGT processes – designed by the end user for the end user
- > Track and trace functionality
- > Minimize GMP risk – reduces human error, maximize utilization
- > Manages scale up from clinical to commercial
- > Review by exception – enable expedited product release
- > Accelerated implementation approach leveraging Lonza's expertise
- > Single source of data – integration to other systems and devices for automated data capture

Integrated Solutions for Cell and Gene Therapy

From discovery to commercialization



Cell and Gene Therapy Services

Process development capabilities and custom manufacturing

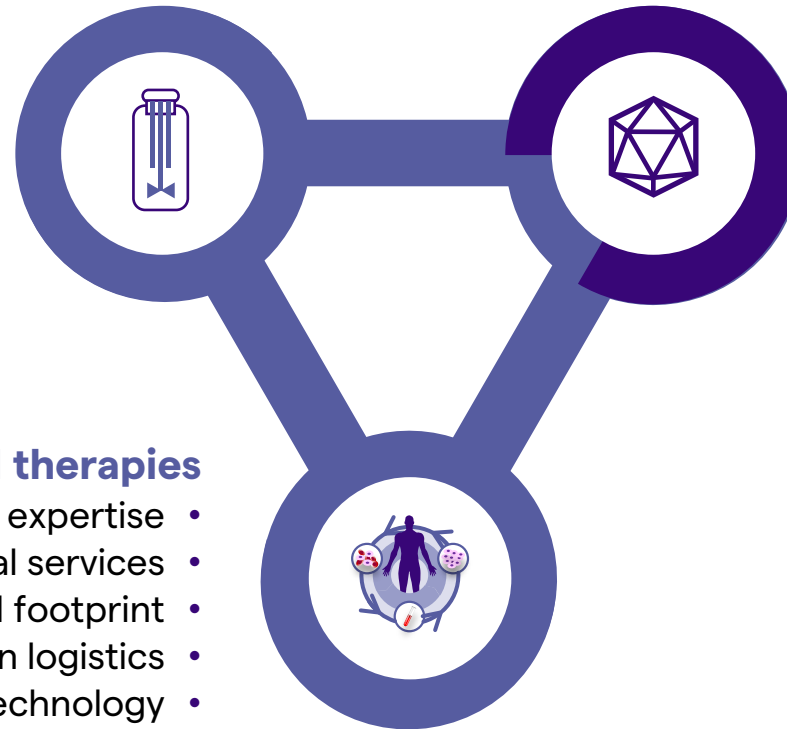
Tailored process and analytical development, cGMP manufacturing and regulatory services

Allogeneic cell therapies

- CAR-T/NK cells and exosomes Process Development – From concept to manufacturing
- Scale up to bioreactors
- iPSC generation and banking

Autologous cell therapies

- CAR-T, HSC, TIL Process Development expertise
- Bioanalytical services
- Global footprint
- Supply chain logistics
- Cocoon® Automation Technology



Viral vectors

- AAV, Lenti, Adeno, oncolytic viruses and other vectors
- GMP scales up to 40 X CS10 in adherent and 2,000L in suspension culture depending on vector type

Our Manufacturing Services

Tailored to pace your investments throughout the clinical stage

Preclinical and early phase

- Manufacturability assessment
- Process improvement & development
- Analytical assay development, qualification & validation
- Media optimization & development

Late Phase & commercial

- Global cGMP manufacturing capacity in 4 centers of excellence across 3 continents
- Global tech transfers
- Formulation & fill/finish
- Storage & distribution

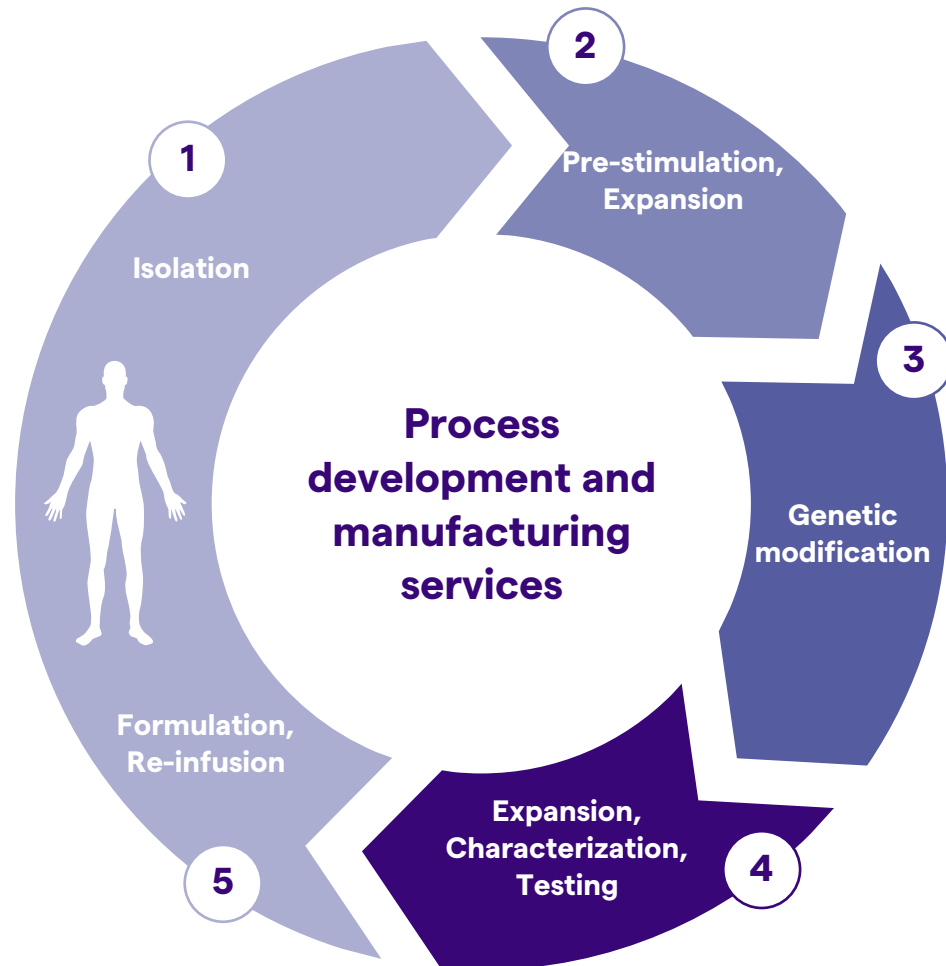
Support for optimized path to market

- Tissue acquisition services & MCB/WCB/MVB*
- Customized business and operation models, facility build-out
- Quality and regulatory set up for successful commercialization
- Regulatory consulting and services

*MCB: Master Cell Bank, WCB: Working Cell Bank, MVB: Master Viral Bank

Your Partner for Accelerating Your Cell and Gene Therapy

Summary



- **TheraPEAK® Media and Reagents (Steps 2, 4)**
Therapeutic cell-culture media enable robust, consistent and cost-effective processes
- **TheraPEAK® Nucleofector® Products (Step 3)**
Flexible non-viral transfection technology for various applications in cell and gene therapy
- **The Cocoon® Platform (Steps 2 – 4)**
Substantial cost reduction in manufacturing cell therapies through a functionally closed and automated platform. Provides flexibility and scalability for your process
- **Endotoxin testing (Steps 4 – 5)**
Regulatory compliant BET products and services ensure your cell therapy can be used with confidence
- **MODA® Solutions (Steps 4 - 5)**
Paperless, GMP-compliant data capture in laboratory and manufacturing, cost-effective and easy to implement
- **Cell and gene therapy development and manufacturing services (Steps 1 – 5)**
Extensive service offering from process development to GMP manufacturing
- **Primary cells and tissues – for workflow set-up and training**
Immune and adult stem cells readily available and cryopreserved, fresh blood and bone marrow or custom made-to-order products for your specific workflow needs
- **TheraPEAK® Regulatory Support Packages**
Pre-developed information packs specific to the Lonza GMP product used

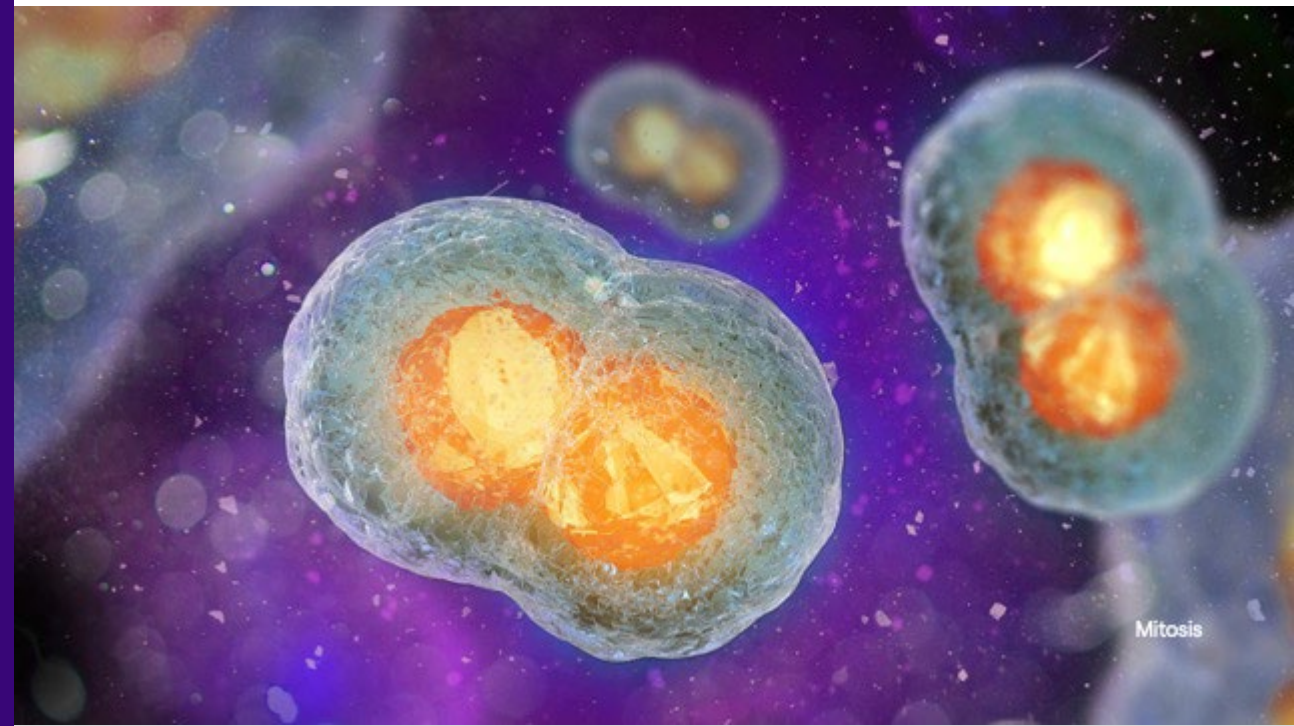
Products and Technologies

lonza.com/products-for-cell-and-gene-therapy

Services

lonza.com/cell-and-gene

Nucleofector® Technology and Primary Cell Systems are for research use only and are not intended for human therapeutic or diagnostic use. All TheraPEAK® Products are produced according to applicable GMP standards and follow the USP/EP guidance for cell and gene therapy raw materials. It is the end user's responsibility to ensure full compliance with local regulations in terms of safety assessments and effective removal prior to patient exposure. All trademarks belong to Lonza, registered in USA, EU or CH or to third party owners and used only for informational purposes. All third-party copyrights have been reproduced with permission or belong to Lonza. The information contained herein is believed to be correct and corresponds to the latest state of scientific and technical knowledge. However, no warranty is made, either expressed or implied, regarding its accuracy or the results to be obtained from the use of such information and no warranty is expressed or implied concerning the use of these products. The buyer assumes all risks of use and/or handling. Any user must make his own determination and satisfy himself that the products supplied by Lonza Group Ltd or its affiliates and the information and recommendations given by Lonza Group Ltd or its affiliates are (i) suitable for intended process or purpose, (ii) in compliance with environmental, health and safety regulations, and (iii) will not infringe any third party's intellectual property rights. For more details: www.lonza.com/legal.



Custom Services with Our Core Expertise



Cell Isolations from normal and diseased tissue



RNA/DNA isolation



Primary and cell line expansion and banking



Cell batch screening and characterization



Custom research media in bottles, kits, and bags



Learn more:
lonza.com/cellbioservices

Lonza has more than **30 years of experience in primary cell isolation** and related CellBio Services