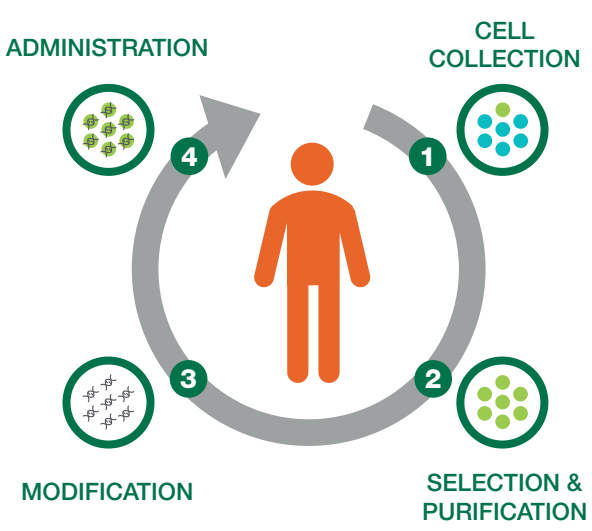
Autologous Vs. Allogeneic Cell and Gene Therapies

Cell and gene therapies (CGT) are at the frontline of clinical research and offer the potential to transform treatment for serious medical conditions. There are two types of cell therapies in development: autologous and allogeneic. Each provides distinct clinical and production advantages and challenges, and therapeutic capabilities.

AUTOLOGOUS VS. ALLOGENEIC CELL THERAPY PROCESS



AUTOLOGOUS CELL THERAPY

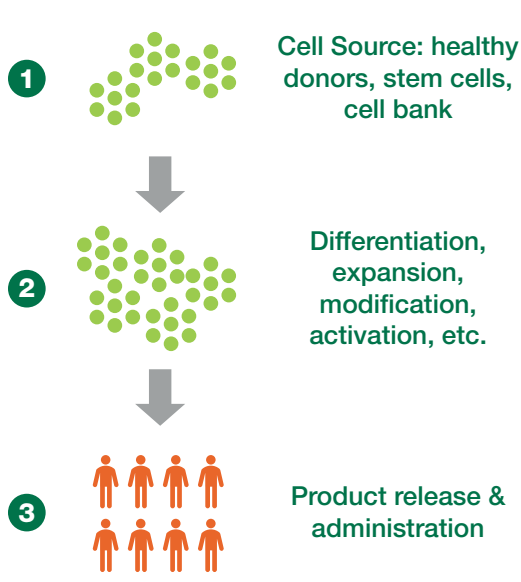
"Personalized" Therapies

1. Cells are **collected** directly from an **individual** patient
2. The patient's cells undergo **selection and purification**
3. Cells are genetically **modified and amplified**
4. The autologous therapy is administered back to the **same patient**

ALLOGENEIC CELL THERAPY

"One to Treat Many"

1. Banked cells are pre-screened and preserved to develop many treatments from a **single source**
2. The banked cells undergo **differentiation, expansion, modification,** and **activation**
3. The allogeneic therapies are delivered to treatment sites globally and administered to **many patients**



AUTOLOGOUS VS. ALLOGENEIC COMPARISON

AUTOLOGOUS THERAPIES		ALLOGENEIC THERAPIES
• Proven technology • Potential lifeline for rare and ultra-rare diseases that can elicit long-term responses in the patient's body for years	STATUS OF TECHNOLOGY	• Still under clinical investigation
• Cells are easily accessible and are collected from patients directly • There is no need to find a matched donor • Genetic disposition to the disease must be screened since this may limit the therapeutic benefit	STARTING MATERIAL	• Lack of availability and high cost of clinical grade cell lines
• Prior treatment and the underlying disease can impact the quality of patient cells • The quantity of patient cells collected can be low	CELL YIELD	• Healthy pre-screened donors and cell banks provide a large quantity of quality cells
• Batches derived from individual patients means high initial product variability and low predictability of end-product quality • Difficult to achieve reproducible results across multiple products	CELL HETEROGENEITY	• Batches derived from the same donor means low initial product variability and high predictability and consistency of end-product quality
• 1 batch = 1 dose • High operating costs • Extremely expensive and time-consuming to develop • Regulatory guidance already known	MANUFACTURING	• 1 batch = 100s and 1,000s of doses • Cost can be significantly lower per dose • Need higher throughput technology to optimize, standardize, and scale production • Regulatory guidance still under development
• Can take up to 12 weeks to develop and ship back to patients	TIME TO DELIVERY	• Off-the-shelf products are readily available and can be rapidly administered to patients
• Only a few treatment centers globally • High product cost leads to low engagement with payers or insurance companies and national health providers • Total amount of time and cost required from pre- to post-treatment is not feasible	PATIENT ACCESSIBILITY	• Off-the-shelf products can be available at any patient center globally • Lower product cost • Less time needed at treatment centers and ultimately a more affordable experience
• Low risk of adverse immune reactions and GvHD post-transplant	IMMUNOLOGICAL RISK	• High risk of autoimmune events post-transplant; residual non-modified foreign cells in the final product may trigger an immune reaction

The Cell and Gene Therapy Partner of Choice

Development of cell and gene therapies is complicated, with every product presenting a unique set of challenges. It's critical to have an experienced partner to help you engage with regulators and design the study early on. Having executed trials for the first gene therapy approved in Europe, Veristat set the standard for other therapies in development and remains a leader in this specialized area today. With our global team of scientific experts offering strategic insights and custom-tailored solutions, Veristat provides comprehensive end-to-end services, from pre-IND to submissions and approvals.

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