REGULATORY AND STRATEGIC DEVELOPMENT EXPERTISE IN ADVANCED THERAPIES

Advanced therapies hold the bold promise of providing state-of-the-art therapeutic approaches to combat diseases that are still out of reach of traditional drugs and biologic therapies. Our work with innovators, regulators, key opinion leaders, and investigators in this area gives us unique insight into the challenges and hurdles that are facing the industry and positions Medpace as a key partner to help accelerate our client's product through the development process.

Advanced Therapy Medicinal Products (ATMPs) are medicines for human use that utilize genes, cells, and/or tissue-engineered products to diagnose, prevent, or treat injury and disease. Although ATMP is strictly an EU designation, the term is roughly interchangeable with gene therapy products and human cellular and tissue-based products (HCT/P) in the US, and regenerative medicinal products in Japan. Cumulatively, these products are referred to as "advanced therapies."

Advanced therapies are at the cutting edge of technological advances in drug development. Through their ability to repair specific genes, tissues, and organs, these therapies offer the promise of groundbreaking new opportunities in a myriad of challenging and complex research areas to treat the world's most devastating diseases.

GENE THERAPY MEDICINES

- Contain nucleic acids (eg, genes) that produce therapeutic, prophylactic, or diagnostic effects by regulating, repairing, replacing, adding, or deleting genetic sequences that cause disease
- Delivery of DNA to target cells may involve use of viral or bacterial vectors, or other mechanisms
- Targeting devastating and complex diseases characterized by genetic defects such as cystic fibrosis, hemophilia, and muscular dystrophy

SOMATIC CELL THERAPY MEDICINES

- Contain autologous or allogeneic cells or tissues that have been modified so they can be used to repair, regenerate, or replace human tissue
- Engineering of cells or tissues involves substantial manipulation to achieve the required properties. The cells or tissues may be viable or non-viable and may contain additional substances including cellular products, chemicals, or matrices

TISSUE-ENGINEERED MEDICINES

- Contain autologous or allogeneic cells or tissues that have been modified so they can be used to repair, regenerate, or replace human tissue
- Engineering of cells or tissues involves substantial manipulation to achieve the required properties. The cells or tissues may be viable or non-viable and may contain additional substances including cellular products, chemicals, or matrices

COMBINATION THERAPIES

- Incorporate cells or tissues with one or more medical devices as an integral part of the product
- Cells (somatic cell therapy or tissue engineered product) embedded within a biodegradable matrix or scaffold (classified as a medical device) being used to increase new bone formation in regions of atrophy would be an example of a combination therapies

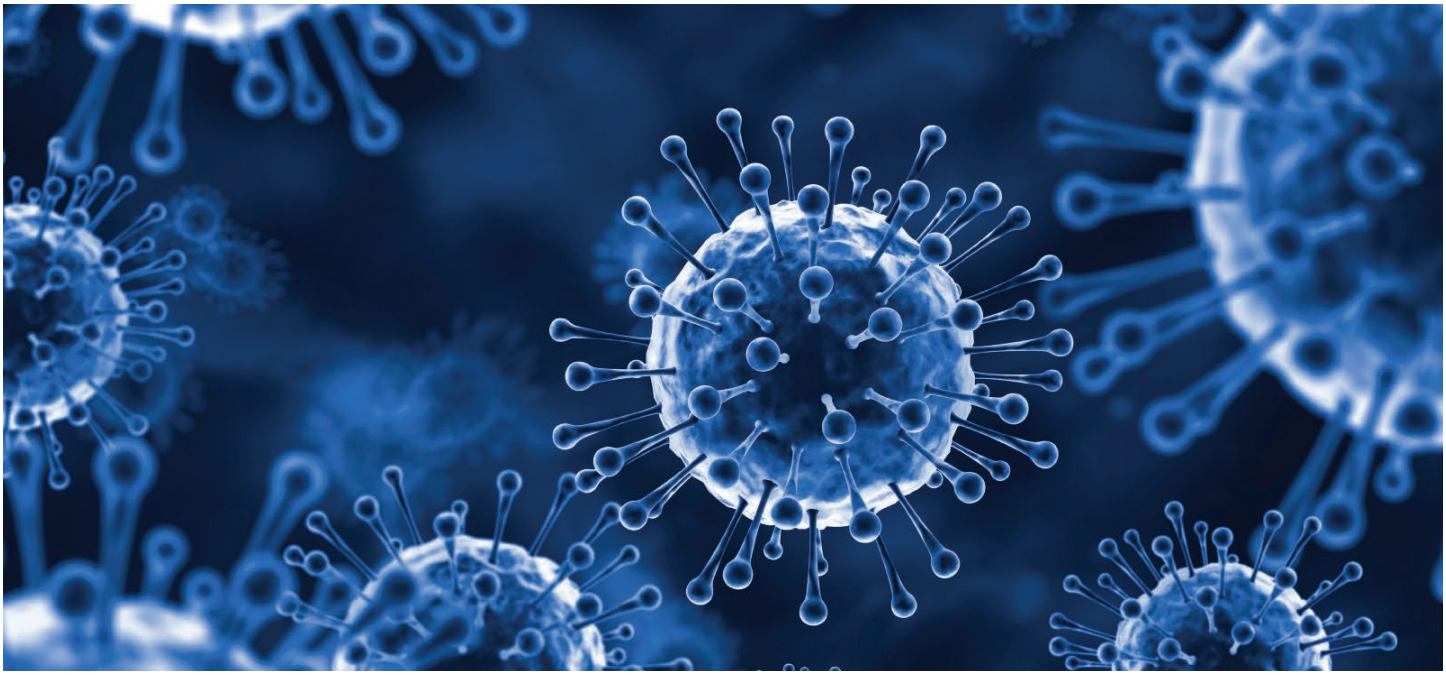
Advanced therapies are known to be especially complex and challenging products to develop. The complexity, in large part, is due to the fact that the technology and science of these novel therapeutics is moving faster than the existing regulations can accommodate. Regulators do recognize the need for close collaboration with biotechnology and pharmaceutical companies during the development process and have provided a number of scientific and financial incentives to encourage and accelerate the progression of these important life-changing therapies.

REGULATORY SUPPORT FOR THE DEVELOPMENT OF ADVANCED THERAPIES

- Classification of advanced therapies – either through formal processes with the Committee for Advanced Therapies of EMA or through early interactions with the FDA, consultation options are available to Sponsors to confirm that their product meets scientific criteria for advanced therapy classification and to provide information regarding the appropriate regulatory framework
- Orphan drug designation – advanced therapies are often developed for rare diseases and can benefit from orphan designation and the associated incentives
- Adaptive and expedited pathways – approaches available in the US, EU, and Japan to accelerate and support product development and marketing approval thereby improving timely access for patients to new medicines (e.g., RMAT, PRIME)
- Scientific advice and protocol assistance – evaluation of available nonclinical, manufacturing, and clinical data at all stages of advanced therapy development and consultation on study designs to effectively generate robust evidence of a therapy's benefits and risks
- EU SME status – financial, administrative, and procedural incentives offered to micro-, small-, and medium-sized enterprises (SMEs) once registered with the EMA
- Guidance documents – regular issuance of documents outlining the current thinking of regulatory agencies on nonclinical, manufacturing, clinical, and regulatory topics to aid in the development of advanced therapies

MEDPACE SERVICES

- Medpace can advise, support, and assist clients to efficiently navigate the regulatory challenges of their advanced therapy development program by leveraging scientific subject matter experts and regulatory strategists with in-depth knowledge of the latest global regulations related to advanced therapy products.
- Development planning for advanced therapies in all major regions with strategic incorporation of applicable regulatory and competitive intelligence at national and regional levels
- Critical review and gap analysis of the development program, including nonclinical, quality, clinical pharmacology, clinical, and device considerations
- Integrated support for scientific advice meetings with regulatory authorities at all stages of product development, including early development planning (eg. ATMP classification in the EU and INTERACT meetings in the US)
- Development and review of global regulatory documents to facilitate effective clinical trial applications
- Guidance for genetically modified organism (GMO)-related processes, including development and review of GMO documentation and applications in the EU member states
- Strategic support for accelerated development of advanced therapies, including support for regulatory pathways and designations in all regions



MEDPACE SCIENTIFIC AND STRATEGIC DEVELOPMENT & REGULATORY AFFAIRS

Medpace's Scientific & Strategic Development (SSD) and Global Regulatory Affairs groups work hand-in-hand to facilitate the rapid development of safe and effective therapeutics for our clients. Our teams are comprised of global product development experts, strategists, regulatory project managers, regulatory writers, and regulatory operations colleagues, providing unrivaled regulatory support across all areas of clinical development through early discovery to marketing authorization.

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a global full-service clinical research organization providing Phase I-IV core development services for drug, biologic, and device programs. With medical and regulatory expertise in multiple therapeutic specialties. Medpace has assembled therapeutically focused teams to execute at every level of the company's operations, providing complete and seamless drug and device development services.

MAKING THE COMPLEX
SEAMLESS™