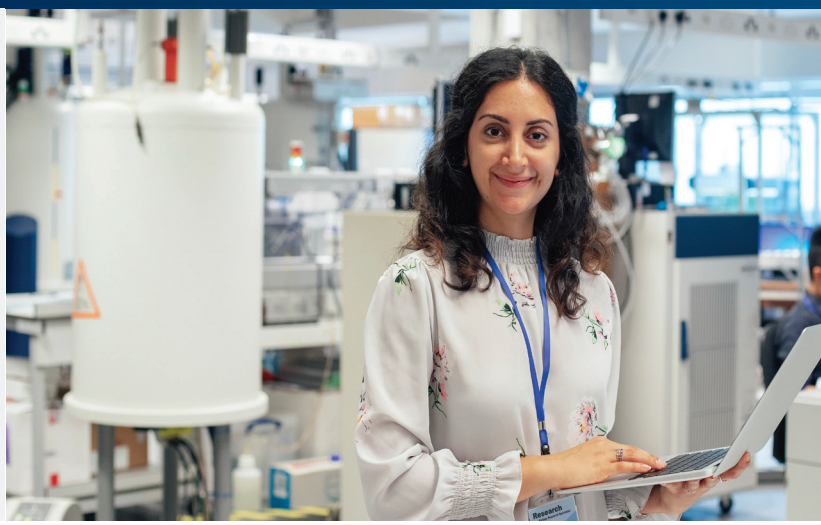
FACT SHEET

IRT and eClinical Technology Considerations for Cell and Gene Therapy

A case snapshot for personalized medicine

Introduction

Cell and gene therapy is a growing experimental technique that uses cells, proteins or genes from a patient or donor to treat or prevent disease.



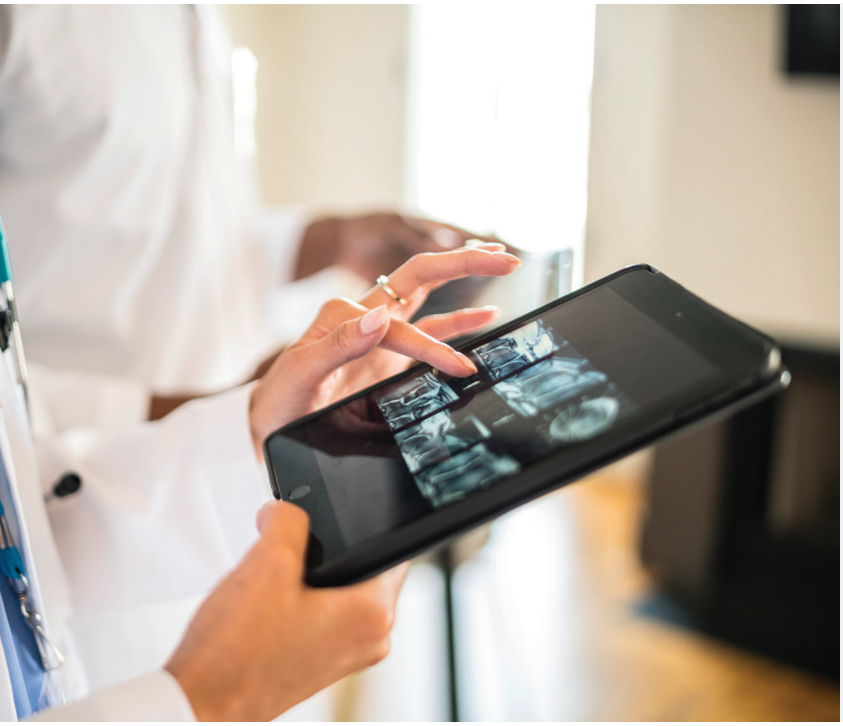
Cells are harvested and sent to a lab, which grows and develops these molecules into a personalized treatment for individual patients. A kit containing the altered cells – often in a bottle – is then returned to the drug depot, and sent to the site to be dispensed to the patient.

The hope is that eventually physicians will be able to treat a range of diseases including hereditary, heart and renal diseases; AIDS; and cancer, by infusing a gene into a patient's cells, as an alternative to medication based on small molecules, or biologics or surgery.

Bringing expertise to a new and complex clinical landscape

The logistical considerations for cell and gene therapy studies are very different from traditional clinical studies.

One of the most important considerations focuses on managing samples. At the patient's visit, there is still a kit or a treatment to be administered. Everything that happens before the treatment visits adds complexity:



creation of the treatment, accurately tracking its progress and location, and the associated numbers. Tracking the process of that therapy from inception through dispensing, these numbers include a sample identifier, a manufacturing number, a lot number, and a release number – all of which are unique to a patient. Managing these extra steps in the supply chain are critical to the smooth operation of the study and regulatory compliance.

Only with an IRT system from a specialty eClinical technology provider can a sponsor effectively navigate these new and complex steps throughout the supply chain. When a cell and gene therapy patient receives a specific therapy developed just for them, the IRT platform needs to be highly flexible and adaptive to allow for the unique processes for each cell therapy treatment.

Cenduit clients – in the vanguard of cell and gene therapy research

Our clients are consistently researching a number of gene therapy treatments. To illustrate, let's use the example of the numerous steps involved in tracking an apheresis collection bag. Until the point at which it becomes the actual IP, steps include:

- **Pre-assigning** a certain number of apheresis collection bags at random
- **Creating an apheresis form** in the IRT system in which bags are assigned individually
- **Capturing** the date of collection, volume, etc.
- **Marking a bag** as not used, used (and assigning a new IP number at the time of manufacture), damaged/lost, or shipped to the manufacturer
- **Tracking** the bag shipment
- **Treating the creation** of a bag of IP as a new drug, and conducting a "release," complete with individual expiry

We've also worked with other clients on studies in which the cell therapy treatment was handled similarly to a traditional drug trial in terms of dispensation. The client harvested stem cells collected from various donors, and stored and distributed the newly-created therapy to multiple patients, similar to a traditional drug.

A consultative approach to support each client's unique needs

Working in cell and gene therapy requires a much more consultative and flexible approach from our industry's IRT and eClinical solution providers. Sponsors have varying degrees of experience running cell and gene therapy trials. Our proven process assigns PMs based on the client's needs and level of complexity of the therapeutic area and indication. For clients who need extra support, our PMs provide a deeper level of consultation to help them navigate through the IRT build process. Many high-tech startups and emerging biopharmas (EBPs) have not delved into the details and logistics of how cell and gene therapy studies can be managed and how IRT design can be used to optimize this process. Regardless of a client's size or expertise, the way we support each study is founded on our three pillars of Quality, Innovation and Expertise to make the lives of the sponsor, site and patient easier.

At Cenduit we never try to fit cell and gene therapy studies – or any kind of study – into pre-defined functionality. The Cenduit IRT and Quantum Interactive™ agile design platforms and reporting tools support clients' most complex requirements. The flexibility and high degree of configurability of our library of existing components enables our teams to tailor each study build precisely to the protocol requirements, and gives us the ability to customize our system as needed as part of our standard offering.

In many cases, we've helped sponsors avoid making the mistake of creating serious data reconciliation problems. We've assisted in preventing the error of using a manual process, which could result in recording data in at least two different sources, which could have a negative impact on data validity.

THE EXPERIENCE TO HANDLE UNUSUAL REQUESTS FOR CELL AND GENE THERAPY TRIALS

In reality, there are numerous ways in which sponsors should conduct cell and gene therapy trials, and then there are ways in which they want to conduct them.

Keeping in mind that cell and gene therapy trials are already among the most complicated therapeutic areas, it's an eClinical technology provider's job to identify viable solutions, while staying firmly within the parameters of good clinical practice, regulatory compliance and patient safety.

MINI CASE STUDY

Gene therapy – what we accomplished

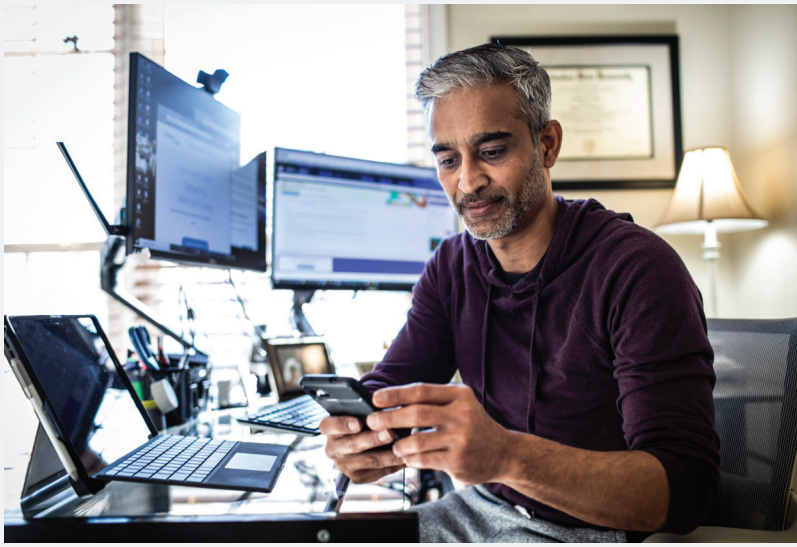
A client asked Cenduit to utilize an academic medical facility as a secondary depot, with the idea that someone would drive the treatment to the administering doctor's office. Working with the client closely, we helped to restrategize the following:

- Practical IRT programming: fundamental to tracking the correct status. In this study, a treatment bag was stored in liquid nitrogen (pre-use status), thawed and prepared by a secondary depot (ready to use status), and then sent to the site just in time for patient infusion.
- As soon as the treatment was thawed and prepared, an expiry date was dynamically created for each kit/ treatment.
- Cenduit IRT tracked all statuses and associated information, in addition to the object's physical location. Shipment status was used for tracking (in process, in transit, received).
- In addition to tracking the location and use of medication, Cenduit tracked the lifecycle of each treatment in this complex study.

Results

FLEXIBILITY AND STRICT CONTROL OF IRT-RELATED PROCESSES

Cenduit's ability to adapt amid trial complexity enabled us to strictly control the points of the process with which the IRT interfaced, while not interfering with study processes outside of the IRT system. Our expertise in over 1,500 worldwide trials enables our team to focus on these critical components, regardless of study complexity.



Innovating new clinical territory with quality

The world of gene therapy is still rapidly evolving. Sponsors are working diligently to learn how they can use best practices in trials to improve healthcare from the youngest to the eldest patient, across a wide range of indications.

Cenduit people, processes and technologies have already earned a place at the forefront of helping clients inaugurate bold new cell and gene therapy research programs, with reliable technology that is both configurable and customizable, throughout the full study lifecycle.

Contact us today, and let's begin a conversation about how we can help your organization leverage this important new area of research.

IQVIA TECHNOLOGIES

CONTACT US

Toll Free (US & Canada): 877-253-3080

Global Direct Dial : +1 610-871-0150 | UK: +44 140 334 2316

www.iqvia.com/oct