White Paper

Empowering rare disease patients in the design of personalized therapy clinical trials

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Summary

The advent of CAGT products has not only revolutionized the way doctors treat health conditions, but also provided great hope to patients, especially those living with rare diseases. Moreover, these therapies embody the concept of personalized medicine much more than other,conventional medicines.

The involvement of expert patients and the consideration of PROs in the clinical trial process will continue to generate richer, more reflective data that assesses the full value of the therapy to patients themselves.

Digital tools can facilitate the collection of patient-reported outcomes, and, at the same time, allow researchers to provide the necessary clinical trial updates to patients. The enhanced and streamlined communication between researcher and patient will provide greater transparency and the insights gathered can help to shape future clinical trial design and drug development.

Introduction

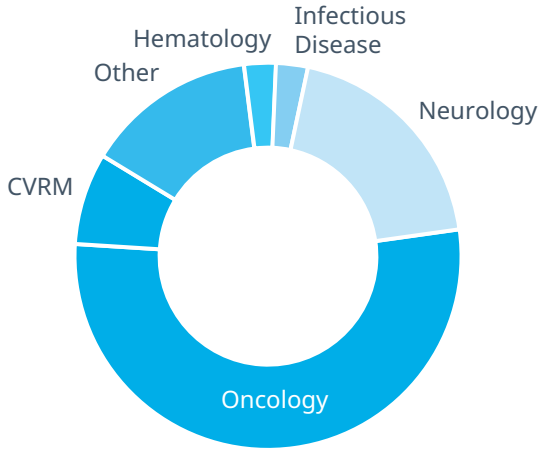
Cell and gene therapy (CAGT) has revolutionized medicine by providing doctors with the means to treat medical conditions once considered “untreatable” whilst giving hope to patients living with conditions that cannot be treated with traditional small molecule medicines.

Cell therapy and gene therapy are often used interchangeably; however, they are distinctive therapy areas. As its name suggests, cell therapy involves using live, intact cells that come from a donor or the patient themselves, to treat certain medical conditions. However, gene therapy involves the use of genetic material to treat or prevent certain medical conditions[1]. Some CAGT therapies aren’t purely cell or gene therapies, but may overlap, e.g, gene-modified cell therapies. All of these specialized therapeutic approaches are often referred to collectively as CAGT.

Therapeutic application of CAGT

Advancements in CAGT have led to its application in many adult and pediatric therapeutic areas including oncology, hematology, cardiovascular, renal and metabolism (CVRM), neurology, and infectious diseases[2].

Figure 1: Common therapeutic areas treated by CAGT



Source: IQVIA Analytics

Oncology

A notable (and successful) early therapeutic application of CAGT is the use of Chimeric Antigen Receptor (CAR) — T cell therapy in the treatment of selected blood cancers (e.g., acute lymphoblastic leukemia [ALL], multiple myeloma, B-cell lymphoma, follicular lymphoma, etc.). As of May 2023, there are six US Food and Drug Administration (FDA)-approved CAR-T cell therapies[3], four of which are approved for use in the Asia Pacific (APAC) region. Some of the CAR-T products have demonstrated great clinical success, where as many as 90% of B-cell acute lymphoblastic leukemia patients were shown to have complete remission[4]. These early successes have led to a surge in the research and development of CAGT, with the APAC region being at the forefront of the field. Several countries, including Japan, China, India and Singapore, have made significant investments to develop their national CAGT R&D infrastructure[5,6].

"In a complete remission, treatment has successfully cleared detectable cancer cells from the body, and the individual no longer show any signs and symptoms of cancer[7]."

— Definition of complete remission

Rare disease

The application of CAGT has also begun to have a sizable impact on the treatment of rare diseases. Although definitions vary around the world, a rare disease is a medical condition that affects relatively few people in a population, i.e., fewer than 200,000 people as defined by the US Orphan Drug Act; or fewer than 1 in 2000 people in the European Union (EU)[8]; or fewer than 1 in 5000 people in India as suggested by the Organization for Rare Disease India (ORDI)[9]. These diseases, though individually rare, affect 400–475 million people globally – most of these people may not obtain a formal diagnosis in their lifetime[10]. Many of these rare diseases are life-threatening with 62% of these individuals having a reduced life expectancy[11]. Moreover, 95% of these rare diseases do not have an effective, active treatment[10], thus the advent of CAGT may provide the "magic bullet" that people living with rare diseases are searching for.

"The World Health Organization defined 'rare disease' as one that impacts fewer than 6.5 –10 per 10,000 individuals[9]."

For example, the application of CAGT therapies has brought great advances in the treatment of spinal muscular atrophy (SMA)[4], hemophilia A and B[5,6], where missing proteins responsible for the disease presentation are effectively restored[12-14]. These early successes are paving the way for the development of more novel CAGTs to treat other rare diseases.

As of May 2023, excluding simple cellular infusion therapies, there are five FDA-approved CAGT products for rare diseases[15]: Luxturna (voretigene neparvovec-rzyl), Rethymic (allogeneic processed thymus tissue-agdc), Skysona (elivaldogene autotemcel), Zolgensma (onasemnogene abeparvovec-xioi) and Zynteglo (betibeglogene autotemcel).

Clinical trials (conventional therapies)

Before novel therapies are made available to patients, they must undergo a series of clinical trials in order to determine whether they are effective and safe to be used in patients. Clinical trials generally comprise four distinct phases[16], depending on the stage of development of the treatment, each with its own specific purpose. The idea of these sequential trials is to gradually build our understanding of and confidence in the new treatment:

- Phase I: This is the first time the medication is introduced to people. Phase I trials are conducted to find the highest dosage of this treatment that can be given without causing serious side effects in individuals. These trials usually only enroll a few participants.
- Phase II: Once the medication is deemed safe enough to proceed with further testing, the next step is to check whether it works in the intended target disease. The patient population is larger, and there are often several different doses. If it is deemed effective without causing many side effects in the patient population then phase III trials can begin. Safety information is collected continuously.
- Phase III: During this phase, the treatment will be evaluated against existing medications to determine if the new therapy will be a useful new option for patients. If the phase III trials are successful, the development data will be submitted for regulatory approval. Safety information is collected continuously.

- Phase IV: In this phase, registered medication will be monitored over a longer period to enhance our understanding of its safety and effects over the long-term. Sponsors and investigators may start new clinical trials with the new medication to check where it fits in the treatment plans for their patients.

Clinical trials (CAGT)

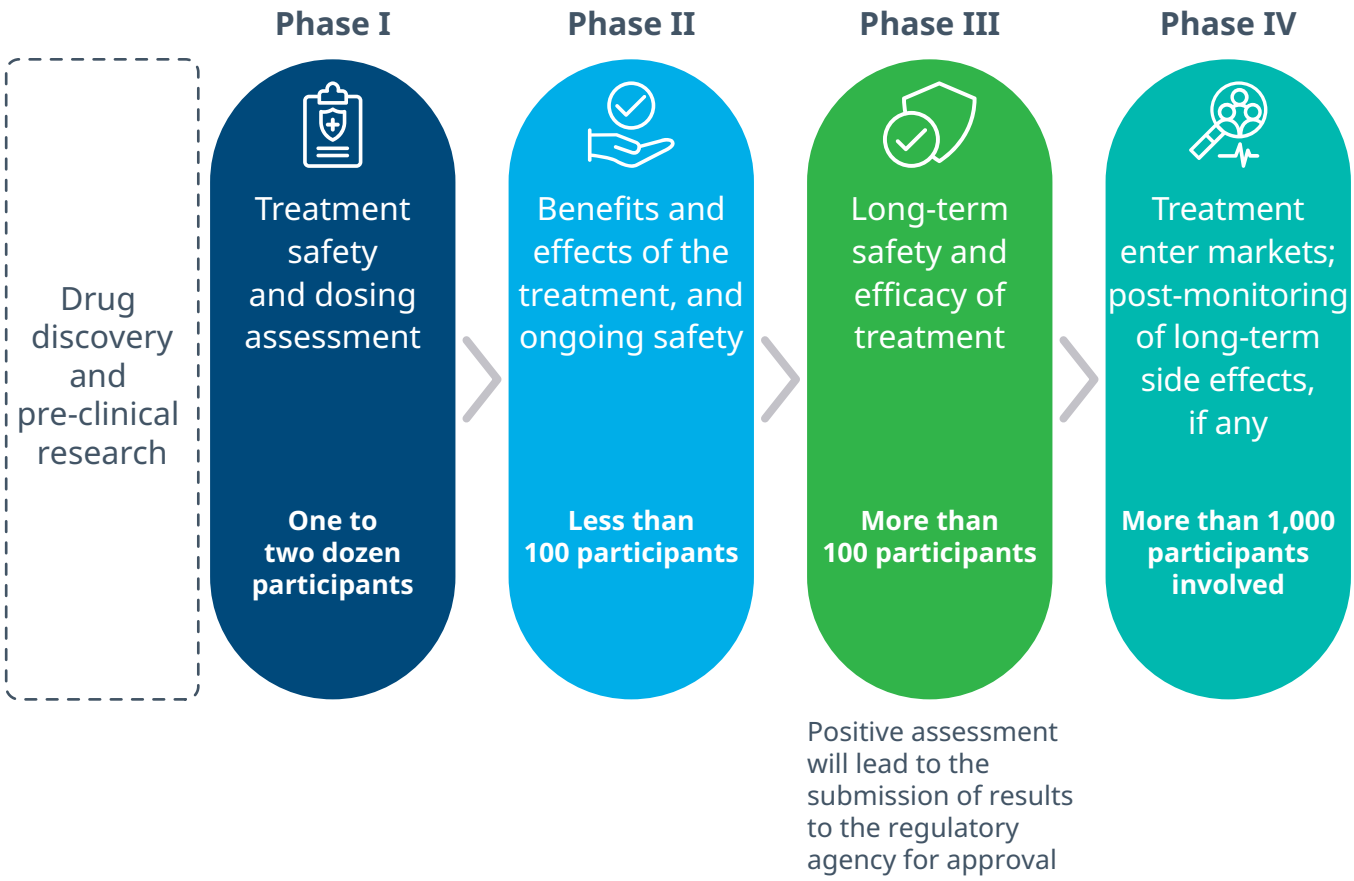
As of May 2023, there are over 3,200 active CAGT trials (early phase I to phase III), of which, 13.9% studies are in phase III. The number of active CAGT trials has almost doubled in five years[18].

Just like conventional therapies, CAGTs must undergo similar rigorous trials before they can be approved. There are some considerations, which we discuss below.

Specific challenges associated with CAGT (and rare disease) clinical trials

Clinical trials assessing CAGT and other treatments for rare diseases come with their own set of specific challenges. The most fundamental challenge to sponsors and investigators is the difficulty in finding eligible participants to take part in the trials. These diseases are often rare, with small, possibly largely undiagnosed patient populations who may lack the documented clinical profile needed to enter the trial[19]. Moreover, the clinical sites need to be equipped with the necessary tools for the day-to-day operations of the clinical trial, and relevant personnel with the right skillset to manage the process, adhering to strict protocols and follow up requirements. For example,

Figure 2: The four phases of clinical trials[17].



frequent, regular follow-ups are required post-infusion of CAR-T products when the risk of complications is highes[20], further adding to the operational complexity and demands on the patient and caregivers during such clinical trials.

Besides the difficulties in finding appropriate clinical sites and eligible participants for these trials, there is an additional layer of complexity. CAGT trials come with strict, often country-specific regulations that must be adhered to before a new CAGT product can enter clinical trials. Some APAC countries tend to follow US and EU regulations, while others like Singapore have developed their own[21].

Beyond the varied regulatory environment, there are also significant logistical demands associated with CAGT clinical trials. For instance, in autologous (i.e., cell obtained from the patient) CAR-T clinical trials, there is a requirement for a designated leukapheresis center in order to isolate white blood cells from patients for these cells to be genetically modified[22] – such centers may not be available away from the large urban hospitals and CAR-T therapy often requires international export, import and storage of isolated and modified cells, presenting logistical and customs challenges.

Figure 3: Rare diseases

Importance of patient-reported outcomes in CAGT (and rare disease) clinical trials

Historically, clinical trials have relied heavily on clinical measures and physicians' observations to assess the safety and effectiveness of the new treatment. However, in recent years, there has been increasing recognition of the importance of the patient's voice to help provide relevant insights that may not be part of the researchers' core considerations[23]. There is a need for analysis of patient-reported outcomes to determine what degree of symptom improvement is meaningful for patients themselves[24].

"We can't tailor make every single trial completely unique for each individual because we do have to answer the data questions. But we do also need to remember these are people with lives."

— Organization for Rare Disease India

Patient-reported outcomes (PROs), as the name suggests, are directly reported by the patient and are often taken "as is" without further interpretation by a clinician or the researcher-in-charge. These outcomes often relate to the patient's health, feelings, quality of life or functional status associated with the state of care or treatment provided[25].

Some scales may be in the form of simple lines that are marked by the patient so that their general health or feelings, e.g., pain (see Figure 4a), can be assigned a single number. Other PROs have a more complex structure, and they may offer multiple choices to the person completing them (see Figure 4b).

Historically, PROs have not been seen as reliable measures of treatment efficacy by regulators and have hence not influenced regulatory decisions for conventional pharmaceutical products. However, this is changing. A growing number of organizations are recognizing their importance - this is especially true of clinical trials involving rare disease patients[28]. The heightened recognition of the importance of hearing directly from patients in clinical trials has led to the US FDA publishing a guidance document for the pharmaceutical industry titled, "Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labelling Claims"[29]. This amplifies the narrative that the patient's voice is key during the clinical trial process.

Figure 4a – The visual analogue scale (VAS), used by investigators to understand how much pain the patient is in[26].

Note: A mark is placed on the line at the point that represents the level of pain observed. This is measured in millimeter from left anchor 'no pain' to generate a pain score.
The word 'distress' replaces 'pain' to create a distress scale

Figure 4b – A physical function questionnaire[27], used by investigators to assess the impact the condition is having on the patient's daily activities.

PROMIS SF V1.2 - PHYSICAL FUNCTION 8B

Physical Function - Short Form 8b

Please respond to each question or statement by marking one box per row.

		Without any difficulty	With a little difficulty	With some difficulty	With much difficulty	Unable to do
1	Are you able to do chores such as vacuuming or yard work?	☐	☐	☐	☐	☐
2	Are you able to go up and down stairs at a normal pace?	☐	☐	☐	☐	☐
3	Are you able to go for a walk of at least 15 minutes?	☐	☐	☐	☐	☐
4	Are you able to run errands and shop?	☐	☐	☐	☐	☐
		Not at all	**Very little**	**Somewhat**	**Quite a lot**	**Cannot do**
5	Does your health now limit you in doing two hours of physical labor?	☐	☐	☐	☐	☐
6	Does your health now limit you in doing moderate work around the house like vacuuming, sweeping floors or carrying in groceries?	☐	☐	☐	☐	☐
7	Does your health now limit you in lifting or carrying groceries?	☐	☐	☐	☐	☐
8	Does your health now limit you in doing heavy work around the house like scrubbing floors, or lifting or moving heavy furniture?	☐	☐	☐	☐	☐

Figure 5: Young boy texting in hospital

Project Patient Voice and the 21st Century Cures Law

When it comes to rare diseases, patients often present with debilitating symptoms and any slight improvement to a rare disease patient's quality of life can be just as important to them as a "cure". There is, thus, a growing emphasis on quality of life as a clinical trial outcome measure, as any recorded improvements or even a simple plateau in progression may be life-changing for this group of patients[30].

In the US, the FDA introduced ***Project Patient Voice*** to give patients, caregivers, and healthcare professionals, a digital platform to examine patient-reported outcomes that were collected from clinical trials of approved cancer treatments[31]. The information provided gives patients a clearer picture of what to expect when being treated with a prescribed treatment - all of which empower patients to have informed conversations with their doctors on their preferred treatment plan. We hope that ***Project Patient Voice*** will extend beyond cancer clinical trials to clinical trials involving other conditions (i.e., rare diseases) to provide the relevant information to the necessary stakeholders, as and when required.

Click here to find out more about Project Patient Voice and 21st Century Cures Law

Building on ***Project Patient Voice,*** the US also introduced a law to help accelerate medical product development to bring novel innovations to patients more efficiently. Aptly named the ***21st Century Cures Law***, the law enhances the ability of researchers to modernize clinical trial designs by incorporating clinical outcome assessments that will help accelerate the development and review of revolutionary medical products[32]. Adaptation of this law to the APAC context, could potentially lead to faster introduction of novel medical treatments to the patients in the region. The traditional sequence of clinical trial phases is therefore likely to change, e.g., applications for market registration might be made much earlier than phase III, and with data from only a small group of treated patients. In these situations, regulators will usually mandate additional safety follow-up.

Calling on the expert patient

While PROs do uncover some key insights previously unknown to the researcher, the quality and the clarity of the information can be enhanced if these insights are clearly communicated by a well-informed patient.

Such individuals are often referred to as "expert patients", and they hold a wealth of knowledge having significant experience living with their diagnosed conditions. These individuals are highly aware, educated about the condition and can articulate the needs of their own and their fellow patients to doctors or clinical trial investigators, needs that may be extremely important to them, but may not have been considered or prioritized by healthcare professionals[33]. An example of this is a symptom such as "moderate" nausea or fatigue, which may be a low priority for the physician, but may have a significant impact to the patient's quality of life.

"Many healthcare providers don't understand rare diseases as they don't see them very often. I find I spend a lot of time explaining the disease to them. So, unlike routine medicine, you're the one who must educate the doctor because they don't know your child."

— Caregiver, Organization for Rare Disease India

Patient input into CAGT (and rare disease) clinical trial design

To better understand the importance of patient input into CAGT (and rare disease) clinical trial design, we conducted an interview with a prominent patient organization, ORDI from India. The insights gathered from the interviews supported the notion that the patient voice is crucial in shaping clinical trial design.

For instance, while the recruitment of patients may be deemed as a major challenge to clinical trial investigators, they are also faced with the possibility of patients dropping out of the study. This is because some study designs require multiple in-person follow-ups post-treatment administration which can become a major burden for participants. Clinical trial sponsors and investigators thus need to balance potentially contradictory issues. They need sufficient patient follow-up detail to ensure robust study efficacy and safety data, at the same time avoiding overwhelming participants with trial procedures, therefore leading to patients' drop out[19].

A representative from ORDI corroborated this narrative; with patients highlighting how some clinical trial processes are not the most patient friendly. This could be in the form of mandatory reporting as required by regulators that may not be aligned to what patients or their caregivers can do. In addition, there may be instances where there is a misalignment of expectations on the clinical trial processes between the sponsor and patients. Such gaps in the clinical trial process could potentially overwhelm participants leading to patients' drop out.

Moreover, FDA guidelines also highlight the importance of patient retention in clinical trials. Each study must meet the minimum trial participant numbers for it to progress through the various clinical trial phases. Each and every dropout potentially jeopardizes the chance that a life-changing drug will not enter the market[34]. As such, there is an inherent and critical need to better understand the patient perspective to help design clinical trials that reduce the risk of discontinuation.

"I think many rare disease patients have settled on the fact that there may not be a complete cure [for their condition]. It is more so to do with whether a treatment can stop progression of the disease. Even if it's not doing good, it should not create any additional problems."

— Caregiver, Organization for Rare Disease India

Considerations when embarking on the clinical trial journey as a rare disease patient

Before deciding whether to participate in a clinical trial, patients must have access to as much information as possible. Patients (and their caregivers) must have enough understanding that they can adequately weigh the benefits and risks associated with the trial and the treatment in question before enrolling. Much of this information can be found in the informed consent form, a mandatory item provided for all clinical trials.

However, informed consent forms provided to patients i.e., and/or carers may be too technical in nature. It is thus advisable for patients to reach out to their physician or relevant patient support groups when in any doubt.

"Clinical trial itself is a very scary word for many patients and you know there is a lot of negativity built upon this."

— Caregiver, Organization for Rare Disease India

Roles of patient organizations in bridging the information divide

Patient organizations (POs) are organizations set up to represent and support patients and their families. Generally, they will support one specific condition or group of conditions, but in rare disease, POs may exist that support the needs of all rare disease patients within their region. They provide credible information and can provide emotional and practical support throughout the patient journey, from diagnosis to long-term care. One specific area where POs can provide a great deal of support is by making patients aware of upcoming or ongoing clinical trials and then facilitating the patient and caregivers' understanding of the requirements, potential benefits and risks of a trial in simple, patient-friendly language. The position of the PO as an impartial third-party, with no commercial interest in the clinical trial means that their advice is very trustworthy.

"In India, how you communicate trial information is more important than what is being communicated."

— Organization for Rare Disease India

Promoting clinical trial transparency using digitalized tools

To help patients make more informed decisions on clinical trial participation, some clinical trial sites use digitalized tools to promote greater transparency in the clinical trial process. One such tool is IQVIA Patient Recruitment Services which provides patients with all the relevant clinical data and trial information that helps them make informed decisions when they are considering trial participation. Such tools can augment recruitment efforts driven by rare disease patient registries, site networking, patient organizations and online forums.

Besides providing patients with relevant clinical trial information to facilitate their enrolment process, digitalized tools can also be employed during the clinical trial process. These tools coupled with remote clinical monitoring devices, will allow patients to share any PROs post-therapy administration. At the same time, researchers will be able to share trial updates with the participants thereby ensuring greater clinical trial transparency. Such two-way communication between patients and researchers can be used as a standard during the clinical trial process and will generate better clinical insights that can help shape more positive patient outcomes in the long run. Moreover, the convenience of these tools bring to the patient may enhance patient retention in the follow-up phase.

It is important to note that there may be challenges associated with the implementation of such tools in clinical trial sites. For example, internet and technological access may be limited in the patients' own homes. If sponsors provide equipment, and patients have Internet access, trial sites then train those involved in their use to familiarize themselves with the electronic data collection process before they use them.

"50 – 60% of [rare disease patients in India] have access to technology, but sometimes it's the language barrier. Face-to-face [contact] is better if you need clarification"

— Organization for Rare Disease India

Conclusion

Patient centricity is essential to ensure success in CAGT trials. The involvement of expert patients and the consideration of PROs in the clinical trial process will continue to generate richer, more reflective data that assesses the full value of the therapy to patients themselves.

"With the patient, for the patient."

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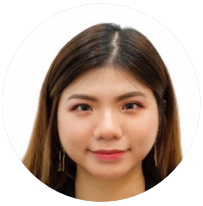

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Prasanna is the co-founder and executive director of ORDI, an umbrella organization that represents more than 7000 diseases in India. Through ORDI, he established the first nationwide Rare Disease Helpline in India to provide information and advice to patients with rare diseases needing medical or non-medical assistance. He also co-founded the Lysosomal Storage Disorder Support Society India, which is the first national level parent support group for children with ultra-rare disease. Outside of ORDI, he is the parent to Nidhi who is India's first Pompe patient.

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