Navigating the Challenges of a Program in T-Cell Therapies

It is an exciting time in the world of Regenerative Medicine and Advanced Therapies. New and innovative products are being developed every day, particularly in the field of T-Cell therapies. However, with new breakthroughs come new challenges. CTI has significant experience working with adoptive T-cell technologies, and consequently has a history of experience successfully navigating these challenges.

Understand the Regulatory Process...

In the recent past, one of the most common questions we receive regarding T-cell therapy programs is how to navigate the Recombinant DNA Advisory Committee (RAC) submission process. The RAC was originally formed to provide oversight of gene transfer technologies, and while it has been around since 1974, the process for submitting and going through the review process can be a daunting one, particularly for new or small companies or departments. To add to the complexity, in April 2016, the NIH amended the review process for human gene transfer protocols, requiring a full RAC review of only specific kinds of protocols. Then, in August of 2018, an announcement was published in the Federal Register putting a hold on new RAC submission acceptances and asking for public comment on additional proposed changes to the process. Familiarizing yourself or working with a partner who is experienced with relevant regulatory submissions, the most

...Including Accelerated Approval Programs

In addition to mastering the process for RAC submission and review, it is beneficial to have a full understanding of accelerated approval programs which could significantly shorten your program's time to market. In 2017, regulatory authorities made major progress around the development of a comprehensive regenerative medicine and advanced therapy policy framework. From these discussions came the Regenerative Medicine Advanced Therapies (RMAT) designation, which is different from other accelerated approval programs (like Breakthrough and Fast Track) and can facilitate speed to market for innovative therapies. To be able to take advantage of RMAT and other accelerated designations, it is important to fully understand their requirements, the differences between them, and the application processes. Working with an experienced consultant or CRO who has a deep understanding of the intricacies of the process could save significant time getting your product to market.

Be Realistic and Proactive with Site Start-up

Site start-up can be challenging in any type of clinical trial, but add in the potential for additional subcommittee approvals, such as Institutional Biosafety Committees, at the site level and labor-intensive start-up processes and it can seem a monumental challenge. However, it doesn't have to be. The most common challenges with site start-up can be overcome if you choose your sites carefully, familiarize yourself with the subcommittee requirements of each chosen site, and plan your budget realistically. The competition for sites experienced in advanced therapy research is high and there is a lot of time-intensive labor required during the start-up phase of these complicated programs, so both of these factors drive up site costs and delay timelines. If you have a realistic expectation of site costs and timelines, you can more effectively plan for an efficient and successful start-up process.

Get Involved

As the old saying goes, "it's not what you know, but who you know." In the case of running a program in adoptive T-cell technologies, it's both. Gene therapy is a progressing field, and CAR-T is an new community in the space. Networking with the stakeholders in the community and really engaging with the patients impacted by your program is crucial to setting yourself up for success.

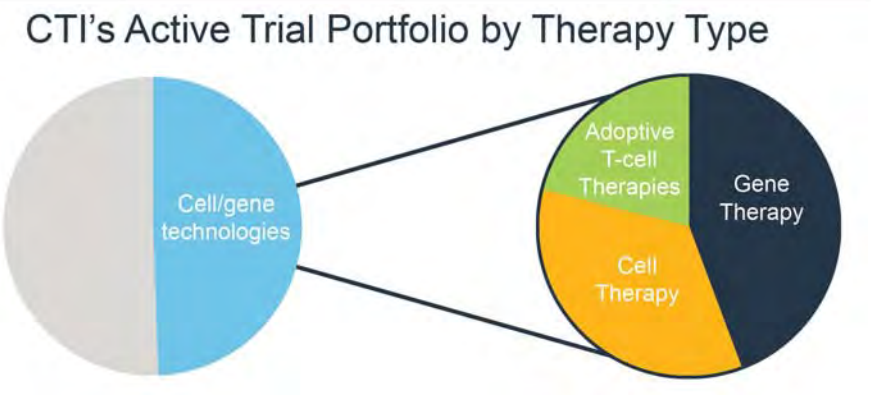
Start Early with Manufacturing Discussions

Lastly, when it comes to CAR-T technologies, we often hear our clients expressing frustration about issues with the manufacturing process. While CTI is not a contract manufacturing organization (CMO), our experience working in this space has given us insight into the frustrations the process can bring. Manufacturing challenges can negatively impact both study initiation and enrollment timelines. For autologous products, effectively aligning product availability with patient availability and investigational site resource availability is crucial. Add in traceability requirements and the CMO quickly becomes a major player in the conduct of the research trial. Our advice is that it is never too early to begin discussions with your contract CMO, because it almost always takes longer to figure out than you think it will.

How CTI Can Help

Approximately 50% of CTI's active programs involve innovative advanced therapy

interventions, and of those programs, 20% involve adoptive T-cell technologies.



We are extremely experienced with RAC submissions under both the old and evolving systems, including familiarity with RAC Freedom of Information Act (FOIA) redaction policies and RAC reporting requirements at study start and throughout the study, safety reporting, and annual reporting. We have pre-existing relationships with most of the sites known as having experience in the space, which translates to an understanding of their subcommittees, including how often they meet, and when. We have relationships and regularly work with innovators and key opinion leaders, engage in dialogue with patient advocacy groups, participate in industry associations such as the Alliance for Regenerative Medicine (ARM), and sponsor and speak at industry meetings such as Stem Cell on the Mesa, RegenMed Investor Day, and the Advanced Therapies Summit.

Because CTI is therapeutically focused, we are one of the first and few providers that have partnered with sponsors meeting early industry milestones in this arena such as the first marketing approvals of true advanced therapies and management of programs that fall under new regulatory guidelines.

If you would like more information about how CTI can help you with your program with T-cell therapies, contact us here **and we would be happy to have further discussions with you.**

Additional Highlights

Upcoming Meetings We'll be Attending

CAR-TCR Summit
Boston, MA
September 4-6

Bio Spain
Seville, Spain
September 25-27

Society for the Study of Inborn Errors of Metabolism (SSIEM) Symposium
Athens, Greece
September 4-7

European Sleep Research Society (ESRS)
Basil, Switzerland
September 25-28

Nordics Life Science Conference
Stockholm, Sweden
September 10-12

Outsourcing in Clinical Trials (OCT) Southern California
San Diego, CA

Annual Meeting
Houston, TX
September 12-15

Cell Therapy World Asia 2018
Seoul, Korea
September 18-20

Global Genes
Irvine, CA
October 3-4

Cell and Gene Meeting on the Mesa
La Jolla, CA
October 3-5

Join Our Team!

We are currently seeking qualified individuals to join our team!

Search Open Positions

CTI Cares

This month, the CTI family is raising money to support 'JDRF', a charitable organization that works to improve outcomes and support those with Type 1 Diabetes.

Learn more and donate

New Hires & Promotions

CTI is thrilled to welcome all of our new employees, and to congratulate our recently promoted employees!

View New Hires and Promotions

CTI in the News:

CTI Clinical Trial and Consulting Services Announces Collaboration with Sernova Corp. for US Phase I/II Cell Pouch Clinical Trial

CTI Clinical Trial and Consulting Services (CTI) announces its collaboration with Sernova Corp. (TSX-V: SVA) (OTCQB: SEOVF) (FSE: PSH) as Contract Research Organization (CRO) to assist Sernova with the execution of its US Phase I/II clinical trial “Safety, Tolerability and Efficacy Study of Sernova's Cell Pouch™ for Clinical Islet Transplantation.”

Read on »

Our mailing address is:
CTI Clinical Trial & Consulting
100 E RIverCenter Blvd.
Suite 1600
Covington, KY 41011