TreeFrog therapeutics

In the stem cell jungle

TreeFrog Therapeutics helps decipher the wood from the trees in this series dedicated to all that is **Cell Therapy**

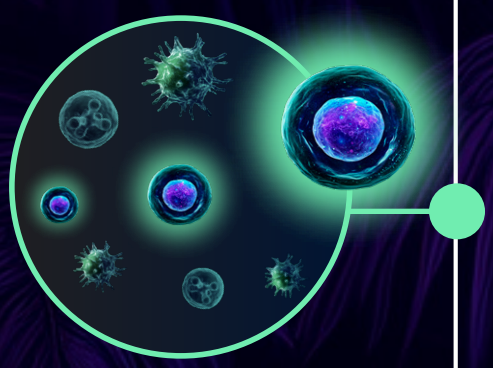
What are the challenges facing cell therapy today?

In factsheet one, we explained cell therapy. Here we look at some of the challenges facing companies striving to bring therapies to the millions in need.

Cell therapy is a revolutionary approach and holds immense promise for treating or curing a myriad of diseases, ranging from neurodegenerative diseases such as Parkinson's Disease, tissue regeneration, and diseases of the major organs such as the liver, pancreas and heart.

However, developing a microscopic therapy to treat diseases with cells is complex! Let's take a look at some of the challenges ... and how TreeFrog is responding to them.

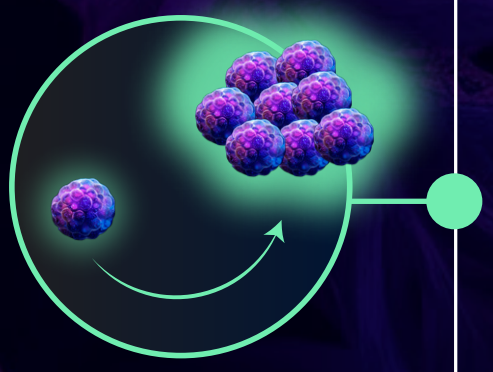
Technology & Scientific **Challenges**



Sourcing the right cell

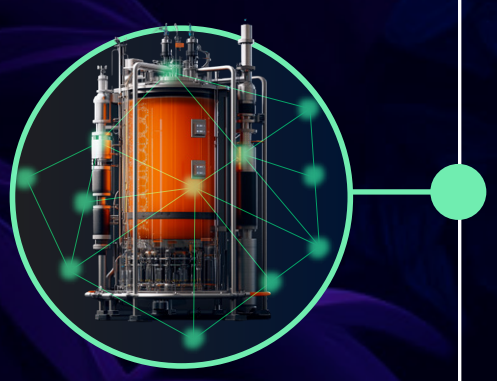
With an estimated 200 different cell types, there are many factors involved:

- The cell source needs to have robust and stable properties and be amenable to genetic manipulation.
- Whether sourcing from the patient or a donor, we need to ensure the cell source is ‘pure’, no damage exists or could happen when we manipulate it to put back in the patient.
- Sourcing from a healthy donor or using a type of stem cell, such as an induced pluripotent cell (iPSC) means the cell can perceive the body as ‘foreign’ so immunosuppressant therapy may be required.



Cell amplification and genomic purity

The number of cells needed for therapy ranges from hundreds of thousands to billions. The amplification of cells is very complex due to their size and fragility and scaling up the process to produce high-quality cells that ensure consistent quality, purity and potency is still a barrier for many programs.

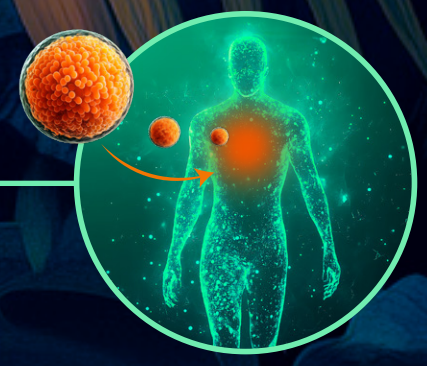


Characterization & quality control

Cells are living organisms, and developing a quality control system to monitor the therapy at each stage of development is very complex. Analytics, bioinformatics and imaging are areas that are progressing extremely fast to keep up and ensure quality and safety.

Cell survival & engraftment / transplant

Cell therapies can be delivered either directly on the patient's skin, or as injectables, infusion or graft. Ensuring that they survive and function effectively in the patient's body means monitoring and follow-up are essential.



Long-term safety and efficacy data

Building a robust body of evidence in safety and efficacy is important for getting regulatory approval and building confidence among healthcare providers and patients.

Immune challenges

Our immune system is extremely impressive and acts as a guardian against being attacked by damaged, diseased cells.
Cell Therapy is the transfer of healthy / engineered cells into the body. Depending on the source of the cell, immune suppression may be required and patients need to be carefully monitored.

Logistical Challenges

Storage, distribution and transportation of cell therapies is another challenge. For example, many cell therapies need to be cryopreserved. Ensuring the infrastructure is available for transport and at the healthcare facility is vital for the safety of the product.

Regulatory & Market Access Challenges

Regulatory

Cell therapies use complex novel technologies in both the mode of action and in the way they are manufactured. It is the role of the regulatory bodies to approve new therapies. New guidance and compliance standards are constantly being developed and put in place.

Cost & market access

Developing and manufacturing cell therapies is expensive due to the complexity and technology. Autologous therapies are developed for each patient, so have an additional cost compared to allogeneic which can produce batches for multiple patients. It is also not just the cost of manufacturing, as the delivery method and monitoring can have quite an impact. Working on cost-effectiveness while maintaining quality is a crucial challenge.
Regulatory, health authorities and insurance agencies, need to consider both the benefit/risk of the product in addition to assessing the overall cost of the product, treatment and follow-up versus ongoing care and costs if the patient does not receive the treatment.

What is TreeFrog therapeutics doing to address these challenges?

At TreeFrog Therapeutics, our purpose is clear – Cell Therapy for All. To achieve this, overcoming the challenges to get cell therapy to patients is at the centre of everything we do and we have broken through significant barriers so far to be successful.

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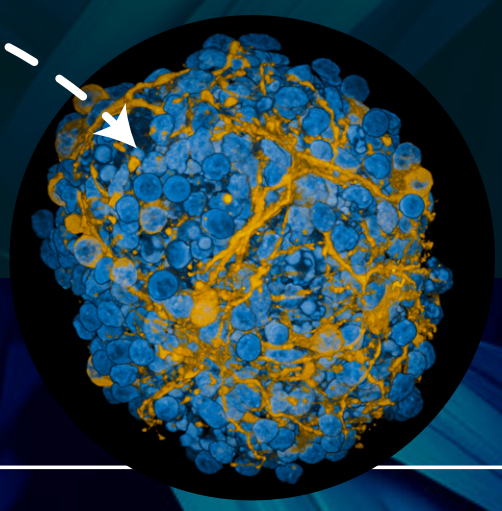
by TreeFrog therapeutics

Blending microfluidics and stem cell biology, our breakthrough technology addresses critical challenges of scale, genomic purity and quality, producing therapies that are safe & effective.

Learn more about the results in the paper by Philippe Cohen et al. in Biomaterials

- No impeller-induced shear stress
- Equal access to nutrients

Production of 1000s of capsules to protect cells per second
Seeding of large bioreactors
Billions and billions of cells in a single batch run



Delivering a unique 3D microtissue format that is in itself a microenvironment, providing protection to the cells and facilitating transplant

Ready-to-transplant 3D microtissue

In pre-clinical studies for Parkinson's disease, fast, full motor recovery was demonstrated at sixteen weeks and sustained at eight months with the 3D format of microtissues integrating well in the brain.

Read more on these results in Neurotherapeutics

We continue to work on further developing our technology and therapies to overcome all the challenges facing the industry today.

We also work with partners either in co-development or through licensing our technology to bring even more therapies to millions of patients.

TreeFrog therapeutics

Cell Therapy for All

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