

ST JAMES'S
HOSPITAL



Wellcome - HRB
Clinical Research Facility
at St James's Hospital

The background features a photograph of a modern, multi-story building with large glass windows and light-colored stone or concrete panels. A large, stylized red circular graphic is overlaid on the right side of the image, partially obscuring the building. The overall design is clean and professional.

Your Journey Through the CRF

A Patient Guide to Cell and Gene Therapies

Welcome to the Clinical Research Facility

Welcome to the Clinical Research Facility (CRF) here at St James's Hospital. In this document, we will explain

- What Cell and Gene Therapies are
- How Cell and Gene Therapies work
- What you can expect if you will receive a Cell or Gene therapy at the CRF

To reassure you on your journey through the CRF, it is just like other wards in the hospital. It has patient treatment rooms, a pharmacy, and is staffed by doctors, nurses and pharmacists, among others. Medical research is conducted at the CRF where new, cutting-edge treatments are made available to patients through clinical trials. Sometimes, patients will also visit the CRF to receive medicines which are just newly available.

For more information on what a clinical trial at the CRF means, please read [here](#).



Since it opened in 2013, the CRF has hosted 251 studies, with 11,250 patients taking part in clinical research.



Disease Areas
19

St. James's Hospital is Ireland's largest acute academic teaching hospital. We support a wide range of studies in multiple disease areas.



Investigators
123

We collaborate with a wide range of investigators across a variety of disease spectrums.



Regulated Clinical Trials
81

This includes early Phase I /II to Phase III and medical device studies.



Clinical Studies
132

This includes observational studies and interventional studies.

What Are Cell and Gene Therapies?

- Cell and Gene Therapies are different from the usual medicines you may be familiar with
- They might be potential cures for conditions where there are currently no or limited treatments
- Cell and Gene Therapies treat some rare diseases. They represent a big step forward in how diseases are treated
- It is essential for patients to be aware of these therapies. This awareness helps patients make informed decisions about participating in clinical trials and receiving treatment with Cell and Gene Therapies

Cell and Gene Therapies Explained Gene Therapy

- Genes determine how our bodies look and work. For instance, they decide our eye colour and height
- Sometimes, genes don't work correctly, leading to diseases
- Gene Therapy involves changing the genes inside our body to treat or stop diseases
- It can replace a faulty gene or add a new one
- Gene Therapy is currently being used to conditions like haemophilia, and spinal muscular atrophy
- At the CRF, we have conducted Gene Therapy clinical trials and treated patients with Gene Therapies that are now available as treatments



Gene therapy patient stories

Please click on these links to read about patients who have received Gene Therapies at the CRF.

Spinal Muscular Atrophy

[Click here](#)

Haemophilia

The CRF, in collaboration with the National Coagulation Centre, is the only centre in Ireland to have conducted gene therapy studies in haemophilia.

[Click here](#)



Cell Therapy

- Cell Therapy fixes cells that are not working properly or uses cells to deliver treatment throughout the body
- Living cells are transferred into patients through their bloodstream. These living cells direct the patient's own immune cells towards disease
- These cells can come from the patient themselves or from a donor

In 2021, a patient received CAR-T therapy at the CRF, the first such treatment available in Ireland.

Previously, people had to travel abroad to access CAR-T.

One patient said he was thrilled to be able to access this treatment in Ireland. *"I feel like I was on the edge of a cliff about to fall off and I've been thrown a rope and I'm going to grab it with both hands. It has not been an easy road for me and my family, but now I feel like I have a fighting chance."*

[Click here for more information](#)



The CRF will be running CAR-T clinical trials in the coming months and years.

Patient Journey Through the CRF

When you come to the CRF (Clinical Research Facility), it could be for one of two reasons:

- Cell or Gene Therapy **Trial**: You might be participating in a research study where new cell or gene therapies are being tested.
- Cell or Gene Therapy **Treatment**: Alternatively, you could be here to receive an approved cell or gene therapy. In this case, the focus is on providing you with an established treatment.

Your journey through the CRF will vary depending on whether you are part of a **trial** or receiving **treatment**. These differences are explained here:



Clinical Trial Journey



1



If you are interested in taking part, you will be referred by your doctor to the doctor conducting the clinical trial. You will have a meeting with this doctor where they will explain all aspects of the trial to you, and you will have the opportunity to ask questions. You will be given a patient information leaflet, with all the trial information, to take home with you.

2



If you are happy to be part of the trial, you will be asked to return and sign the informed consent form.

3



A screening visit will be scheduled where health assessments will be conducted to see if you are eligible to take part.

4



Randomisation period: If you are eligible, you will then be randomly assigned to either the placebo (no active agent) or study drug group.

5



You will return to the CRF multiple times throughout treatment for a specified period of time. The schedule of visits, phone calls, and your time commitment will have been explained to you before consenting to the trial.

6



You will return to the CRF during a follow-up period where you will be monitored but will not be receiving the study drug.

7



In some trials, if you complete the randomisation period of the trial, you may have the option to join an 'open label' period of the trial. If you did not receive the study drug previously, you can now receive it.

8



Some trials may have a further 'extension' period where you can continue receiving the drug. You will still need to visit the CRF occasionally and receive phonecalls.

Treatment Journey



1



If you, or someone you care for, are coming to the CRF for treatment (that is not part of a clinical trial), your doctor will discuss this with you in advance

2



You will receive an appointment to visit the CRF for treatment.

3



The CRF provides care like that provided in the general hospital. On arriving, you will be greeted at reception and seated in the waiting area. A nurse will then come to meet you for your appointment.

4



Depending on your treatment, you may need further tests, which can be done here at the CRF.

5



Like a general hospital, you will be taken care of by a team of doctors and nurses in a patient treatment room. These treatment rooms are used for giving medicines and have a bed if you need to stay overnight.

6



The CRF has an on-site pharmacy with a 'clean room'. This means that your medication will be prepared nearby and delivered to the CRF nurses promptly so that it can be given to you.

7



After your treatment has finished, you may have to return to the CRF for follow-up appointments. Your CRF nurse will let you know if this is the case.

Frequently Asked Questions About Clinical Trials



How to access the CRF

If you are a patient of St James's Hospital, please ask your doctor if there is a trial suitable for you.

If you are a patient at another hospital and you are interested in a trial that is open here, then please ask your doctor to refer you to the doctor at St James's Hospital who is conducting the trial.



What is a clinical trial?

Clinical trials are medical research trials involving research on a particular disease area using a medicine. Clinical trials aim to improve treatments for patients and enhance their quality of life.

Most trials involve testing a new type of drug or method of treatment. Every trial is led by a medical doctor and a research team who will look after you during your treatment. Our team here in the CRF is highly skilled and will provide you with the care and support you need.

There are several phases of clinical trials: Phase 1, Phase 2, Phase 3 and Phase 4, depends on the stage of drug development. Most clinical trials are randomised and they involve two or more treatment arms; this means that you will be randomly assigned to one of the treatments.

Randomisation ensures that the trial is fair and that the results are reliable. You may not be assigned to the new treatment arm and this might disappoint you.

Some trials may involve a placebo. A placebo can look like a real drug but it does not affect the body. Placebos are used when there is

no standard treatment to compare the new treatment to. Clinical trials are highly regulated and follow a long and careful research process to make sure they are as safe as possible.



Who can take part in a clinical trial?

All trials have guidelines about who can and cannot take part. These are sometimes called eligibility or inclusion criteria. For example, a trial may only include people with a certain type of disease or a certain age group. Trials also have guidelines about who cannot take part. These are called exclusion criteria.

Your doctor or research nurse can tell you if a certain trial is suitable for you.



If I am interested in participating in a trial, what is the next step?

If you are interested in participating in a clinical trial, then please ask your doctor if there are any suitable trials for you. If there is a suitable trial, then you will be provided with a patient information leaflet (PIL) and informed consent form.

This is a detailed document that tells you everything about the trial. The trial will be fully explained to you by your doctor and research nurse.

You will be advised to take the PIL home and discuss it with your family and friends and a follow-up appointment will be made for you.

When you attend for the follow-up appointment, the trial will be discussed again and if you wish to participate then you will sign the informed consent form.

You will then be 'screened' for the trial; this

involves various tests and assessments and this may take a few weeks. If you are deemed eligible, then you will be randomised to one of the treatment arms on the trial and your treatment will commence.



How will I benefit?

Taking part in a trial means you may benefit from a new treatment that might not be available, except in the trial setting. The new treatment may work better than the standard treatment.

You will be closely monitored throughout the trial by the research team and there will be a research nurse that you can liaise with. Whilst you are on a clinical trial you may have to attend the hospital more frequently and have more frequent blood tests or scans. Some patients find this reassuring.



Are there any risks or drawbacks?

With any trial, there is a small risk that the treatment could harm you or not work. You could get unpleasant or unexpected side effects, which is important to report to your study team. During the trial, researchers try to reduce these risks as much as possible.

Taking part in a trial may involve additional visits to the hospital which may be an inconvenience for you and your family.



Will it cost me anything to participate in a clinical trial?

There will be no additional costs to you. Any additional tests associated with the trial will be paid for by the trial sponsor. You may have to

visit the hospital more frequently as part of the trial and in this case, your travel expenses may be reimbursed. Please talk to the research nurse about this.



How long do trials last?

Clinical trials can last from one year to many years. Depending on the phase of the trial there may be hundreds or even thousands of patients enrolled on it at many hospitals all over the world.

Your participation in the trial may last for several years as we will continue to follow-up with you long after your treatment has stopped.



Can I withdraw from a trial?

You are free to withdraw from a clinical trial at any time. Your participation is entirely voluntary. You will need to inform your research team of your desire to withdraw and they will discuss other treatment options with you. Your decision to withdraw will not affect your future care.



What about non-clinical trial studies?

The CRF is involved in regulated studies as discussed in the questions above and also involved in non-regulated studies which could be disease registries, biobank type studies, physiotherapy interventional studies.



What is a biobank?

Watch our video below to find out more:

<https://vimeo.com/417373652>



What is the Future of Cell and Gene Therapies in Ireland

Cell and Gene Therapies hold promise for treating diseases where little or no other treatments exist. The CRF enables access to patients to receive these treatments when they need them. It is likely that more Cell and Gene Therapies will become available over the coming years. However, just because a Cell or Gene Therapy is being studied in clinical trials, or has become available as a treatment, does not guarantee that it will be available to Irish patients. Patient representative groups, medical teams, the Irish government and pharmaceutical companies continue to work towards timely and efficient access to Cell and Gene Therapies for Irish patients who need them. There are several places where you can keep up to date on the availability of Advanced Therapies and their availability.

Written Information

Plain language summary of gene therapy [Click here](#)



Cell and Gene Drugs Licensed in the US
[Click here](#)



Treatment updates from SMA Ireland
[Click here](#)



Podcasts

Gene therapy podcast [Click here](#)



Treatment updates from Irish Haemophilia Society
[Click here](#)



Cell and gene therapy podcast
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OrphaNet Rare Diseases Portal [Click here](#)



Patient experience of gene therapy
[Click here](#)

