MULTI-REGIONAL CLINICAL TRIALS
THE MRCT CENTER OF BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

Cell and Gene Therapies

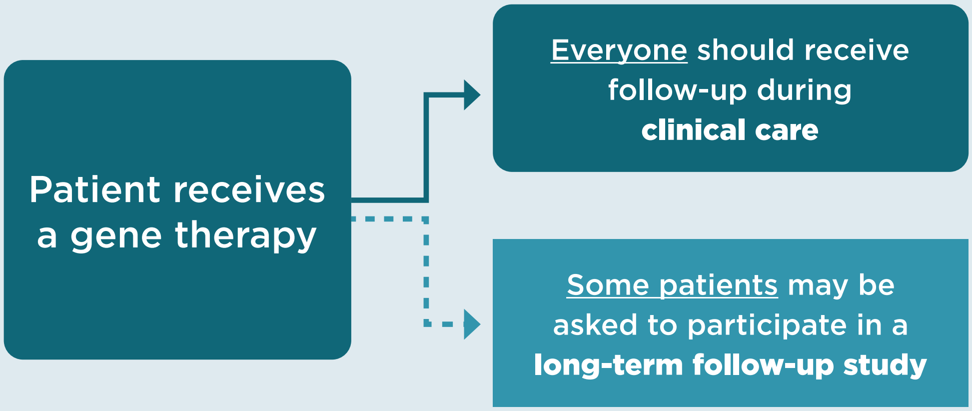
Patient Resource

Toolkit for Supporting the Design, Conduct, and Reporting of Long-term Follow-up Studies for Gene Therapies

---

Long-term follow-up studies involve extended monitoring of patients after they receive gene therapy. This guide explains why long-term follow-up studies are important and the choices you may have.

After receiving a gene therapy, you may be asked to participate in a long-term follow-up study.[i]



---

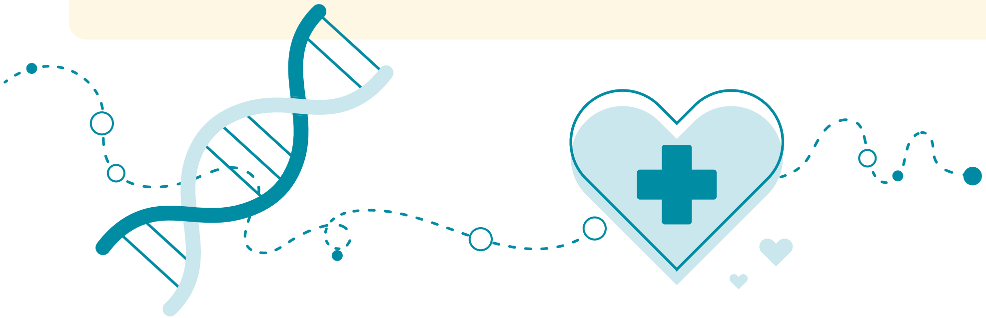
[i] If you are receiving gene therapy in a clinical trial, it may include a long-term follow-up component. You may also be eligible for a long-term follow-up study if you receive gene therapy in clinical care or through an expanded access pathway.

Why are long-term follow-up studies important for many gene therapies?

- Many gene therapies are designed to have long-lasting effects.
- Gene therapies are a new class of drugs and long-term effects are not completely understood.
- Long-term follow-up is conducted to monitor your health so that any issues that arise can be addressed quickly.
- Long-term follow-up studies help researchers better understand the safety of gene therapies over time, so that care for current and future patients can be improved.

After receiving gene therapy, your overall health and well-being should be supported and monitored.

- Your healthcare providers will provide **clinical care and follow-up**, potentially for a long time (e.g., for 5 years, 15 years, or even for a lifetime), after receiving gene therapy.
- Your overall well-being is important, and includes not just your physical condition, but **mental health and nutrition** as well.



What will **participation** in a long-term follow-up study involve?

The **intensity and duration** of participation in a long-term follow-up study **will vary**. You will be given detailed information, including any benefits and risks, and you will have time to ask questions.

Depending on the specific gene therapy, **follow-up** may include:

- Clinic or virtual visits
- Blood tests, imaging, biopsies
- Health questionnaires or surveys

Some **possible benefits** of participating in a long-term follow-up study are:

- Ongoing monitoring of your health
- Early identification of safety concerns
- Receiving important information that affects your medical care
- Contributing to science to benefit other patients
- Opportunities to ask questions and receive updates

There may also be burdens and risks involved with participating:

- Burdens in terms of **time and potential costs** (e.g., associated with travel)
- **Risks** associated with clinical tests conducted to monitor your health

Your Choice and Your Rights

- **You have the choice** whether to participate in a long-term follow-up study.
- **You can say yes or no.**
- To make your decision, **ask questions** about the benefits, risks, and burdens of participation.
  - You can also **ask about alternatives** to monitor your health instead of participating in a long-term follow-up study.
- You may choose to **withdraw** from the long-term follow-up study at any time and for any reason.
  - Please note that withdrawing from a long-term follow-up study does not remove the gene therapy from your body.

Key Contact Information

You should be given information about whom to contact for:

- Reporting health concerns
- Questions about results or follow-up questions
- Withdrawing from the study

You may have many questions about your specific Long-Term Follow-Up study. You have a right to ask and to know the answers.

All your questions are good questions!
This list just provides examples.

Questions you may want to ask:

- **How long will the long-term follow-up study last?**
- **How will my health be monitored (appointments, lab tests, imaging, surveys, sample collection, etc.)? Is this monitoring different from my regular clinical care, or the same?**
- **What is the visit schedule?**
  - Are there any in-person visits? If so, where do the visits take place?
  - Can I go to my local doctor’s office, or do I need to travel to a medical center or centralized research site?
  - Can I do any visits from home (telehealth or home nursing visit)?
- **Will participation in a long-term follow-up study affect my regular medical care?**
  - Will I need to give separate consent each time samples are collected, or does my initial consent cover all collections?
    - What if I need samples for my own clinical care?
- **What happens if I move or change doctors?**
- **If researchers discover a safety issue with the gene therapy I received, how and when will I be told?**
- **Will I receive my individual test results, and when can I expect them?**
- **Will I receive a summary of the long-term follow-up study results, and when can I expect them?**
- **What resources are available to support my physical and mental health and nutrition?**
- **Is there any reimbursement for costs related to being in the long-term follow-up study, like transportation, parking, or childcare?**

---

* Please note that this Patient Tool does not include reference citations; these can be found in the main Toolkit.

To cite this resource, please use the suggested citation for the main Toolkit:
Carolyn Riley Chapman, Emina Berbić, Mena Shaikh, Barbara E. Bierer, and the MRCT Center Long-Term Follow-up Working Group. 2026. *Toolkit for Supporting the Design, Conduct, and Reporting of Long-Term Follow-Up Studies for Gene Therapies*, v. 2.0 Cambridge and Boston, MA: The Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard. https://mrctcenter.org/LTFUToolkit

Thank you for your interest in this Patient Resource. If you have any questions or suggestions, please reach out to the MRCT Center at mrct@bwh.harvard.edu using "LTFU Toolkit" in the subject line.