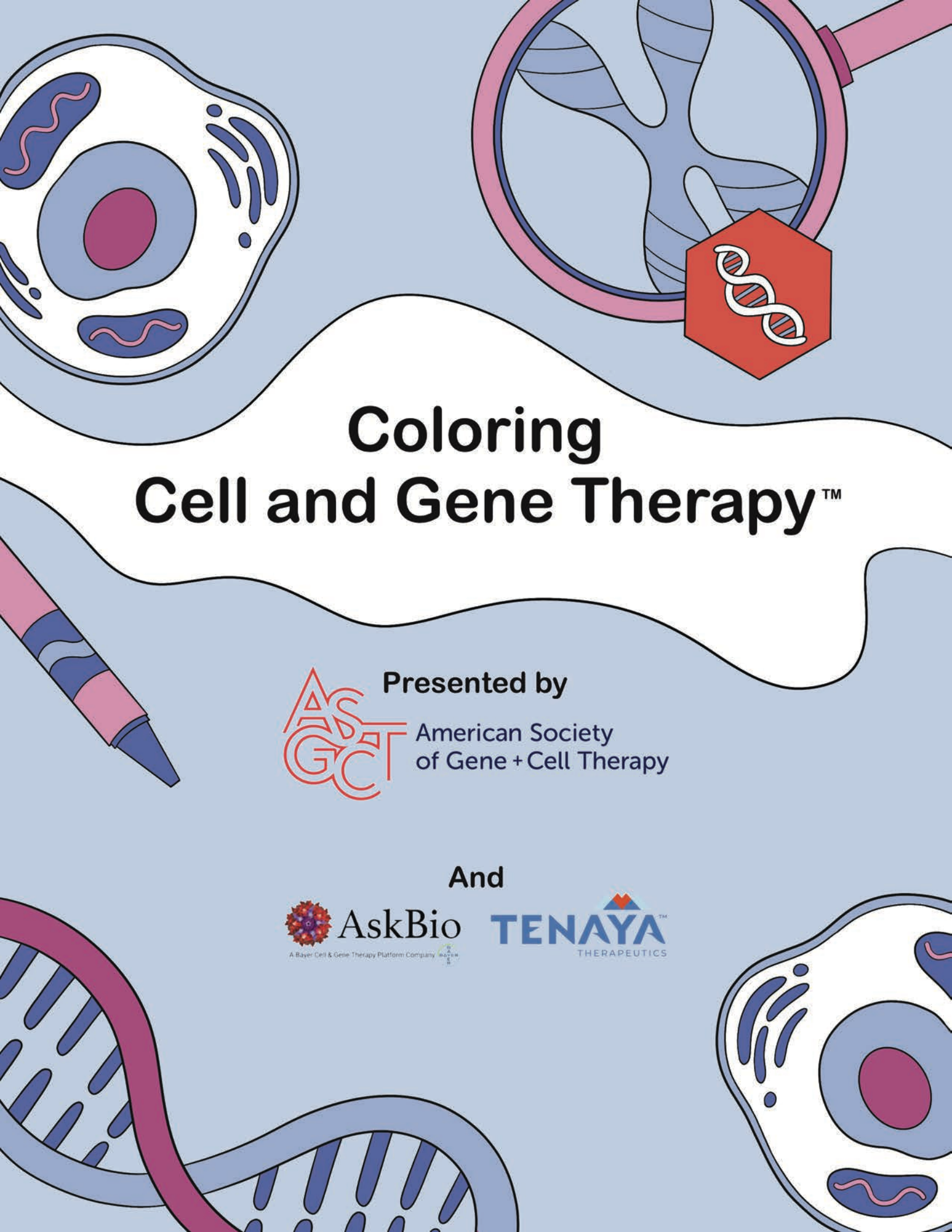


Coloring Cell and Gene Therapy™

Presented by

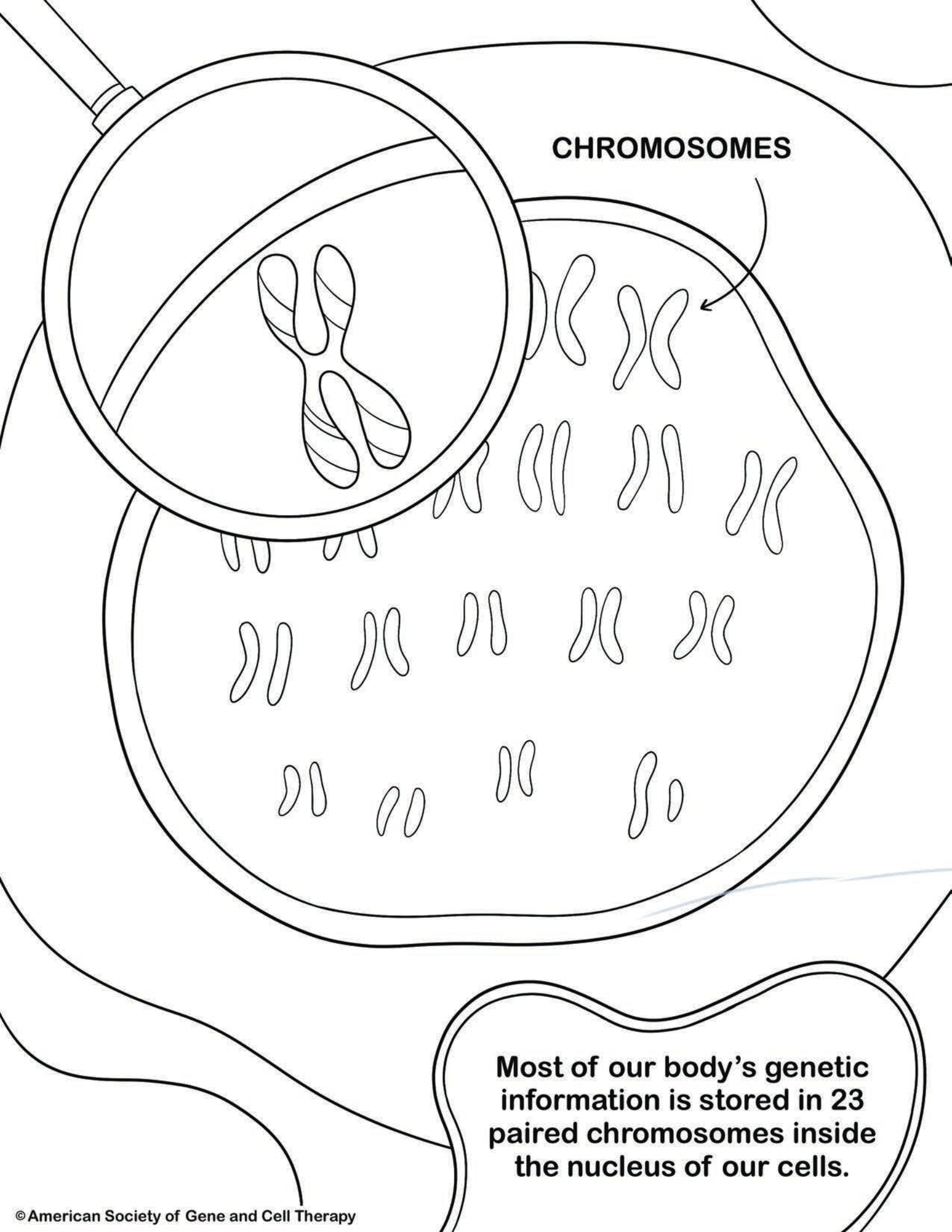
American Society of Gene + Cell Therapy

And

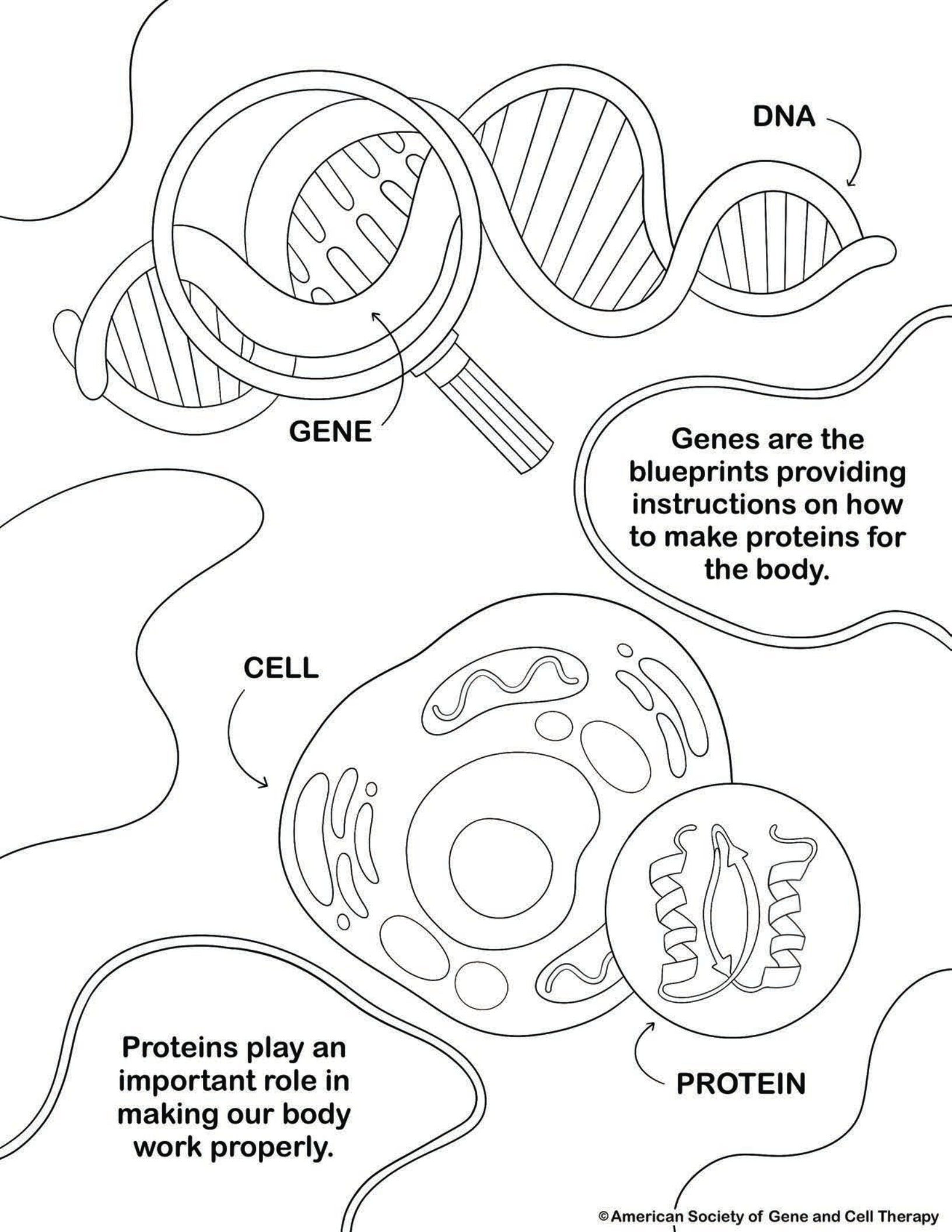
AskBio

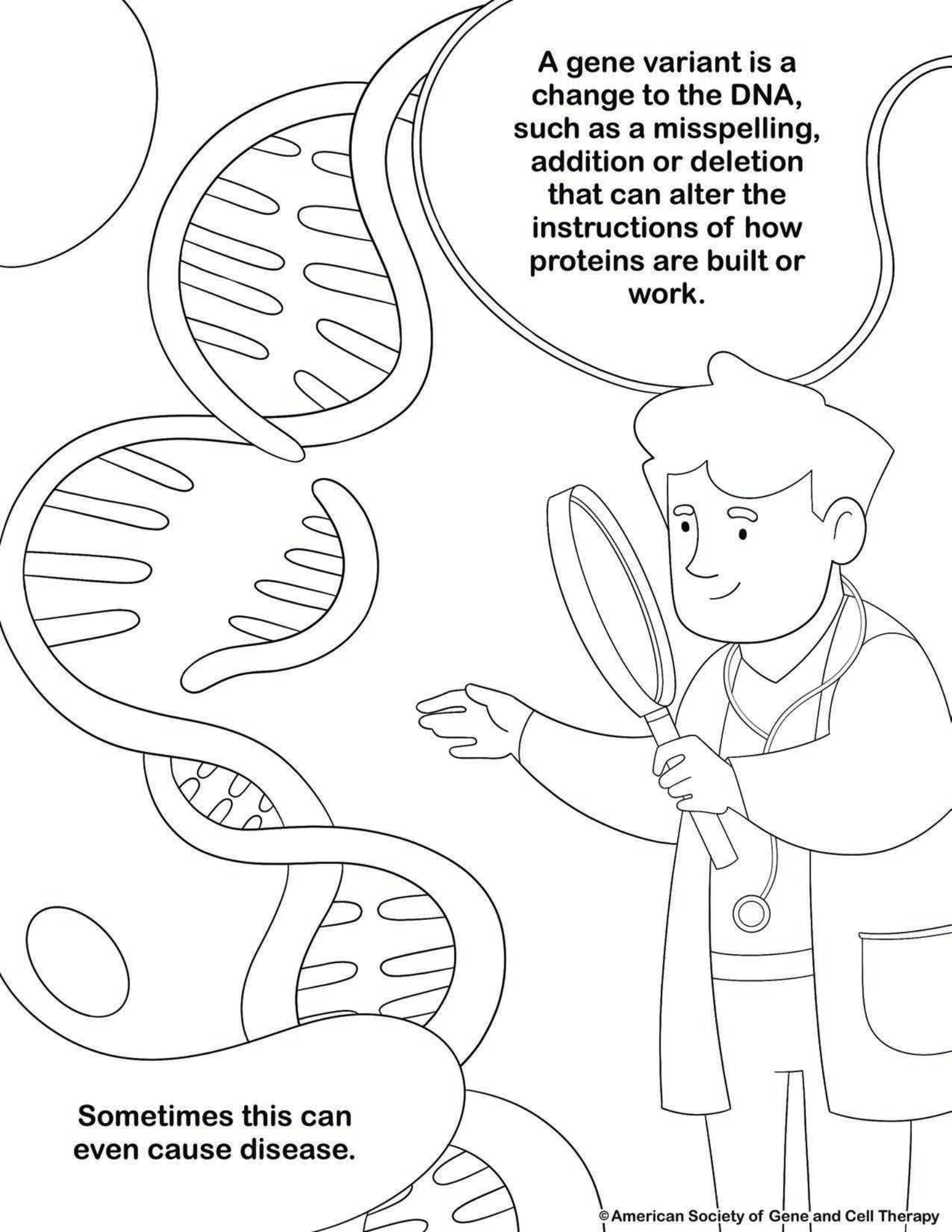
A Bayer Cell & Gene Therapy Platform Company

TENAYA™ THERAPEUTICS

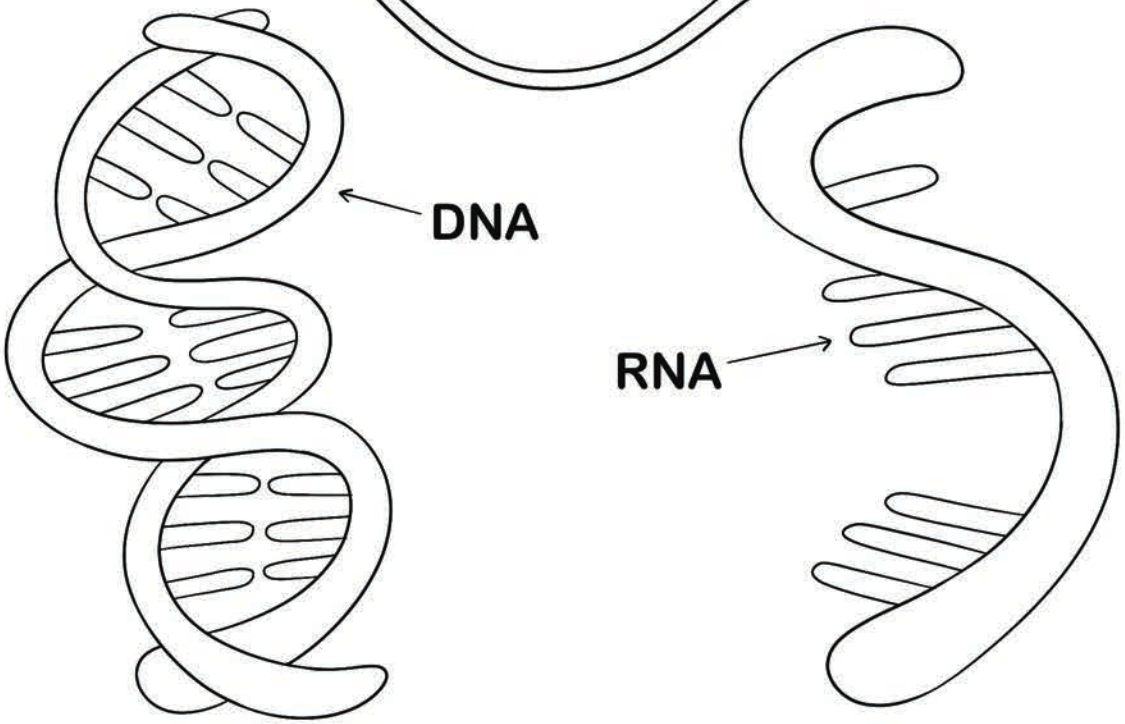


Most of our body's genetic information is stored in 23 paired chromosomes inside the nucleus of our cells.

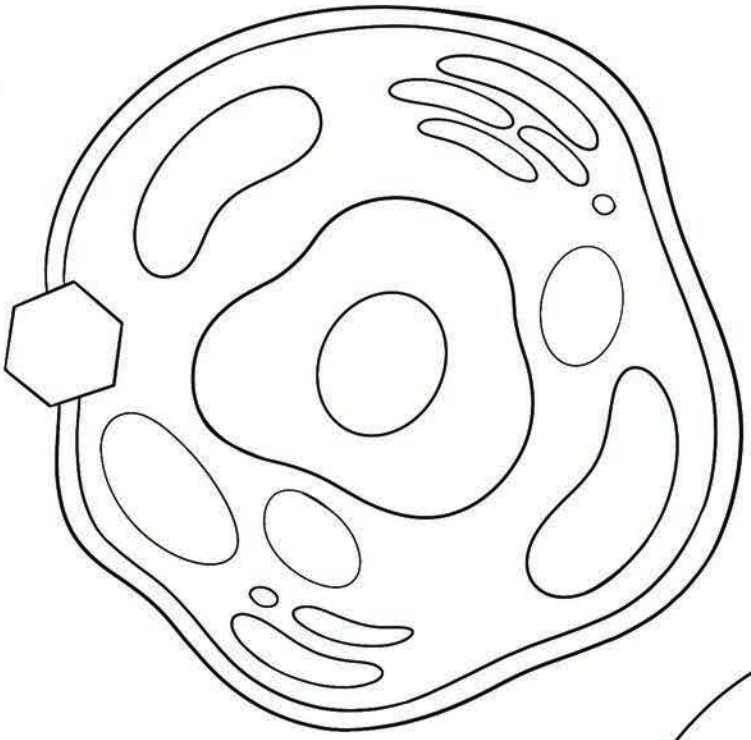


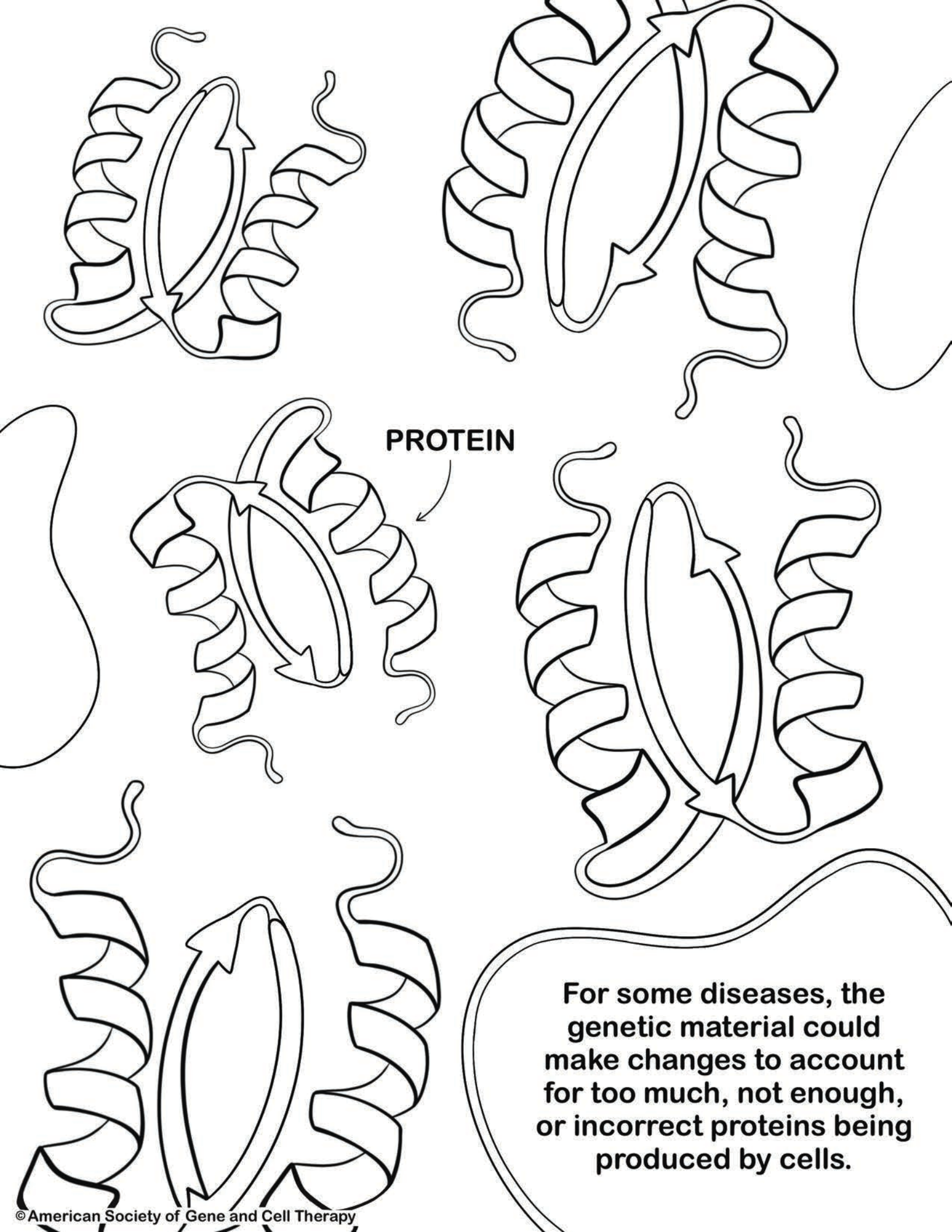


Gene therapy is the use of genetic material, such as DNA or RNA, to treat or prevent disease.



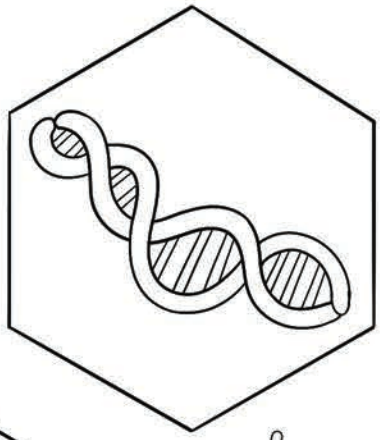
It can address a gene variant by delivering genetic material with instructions to change how a protein— or group of proteins— is produced by the cell.



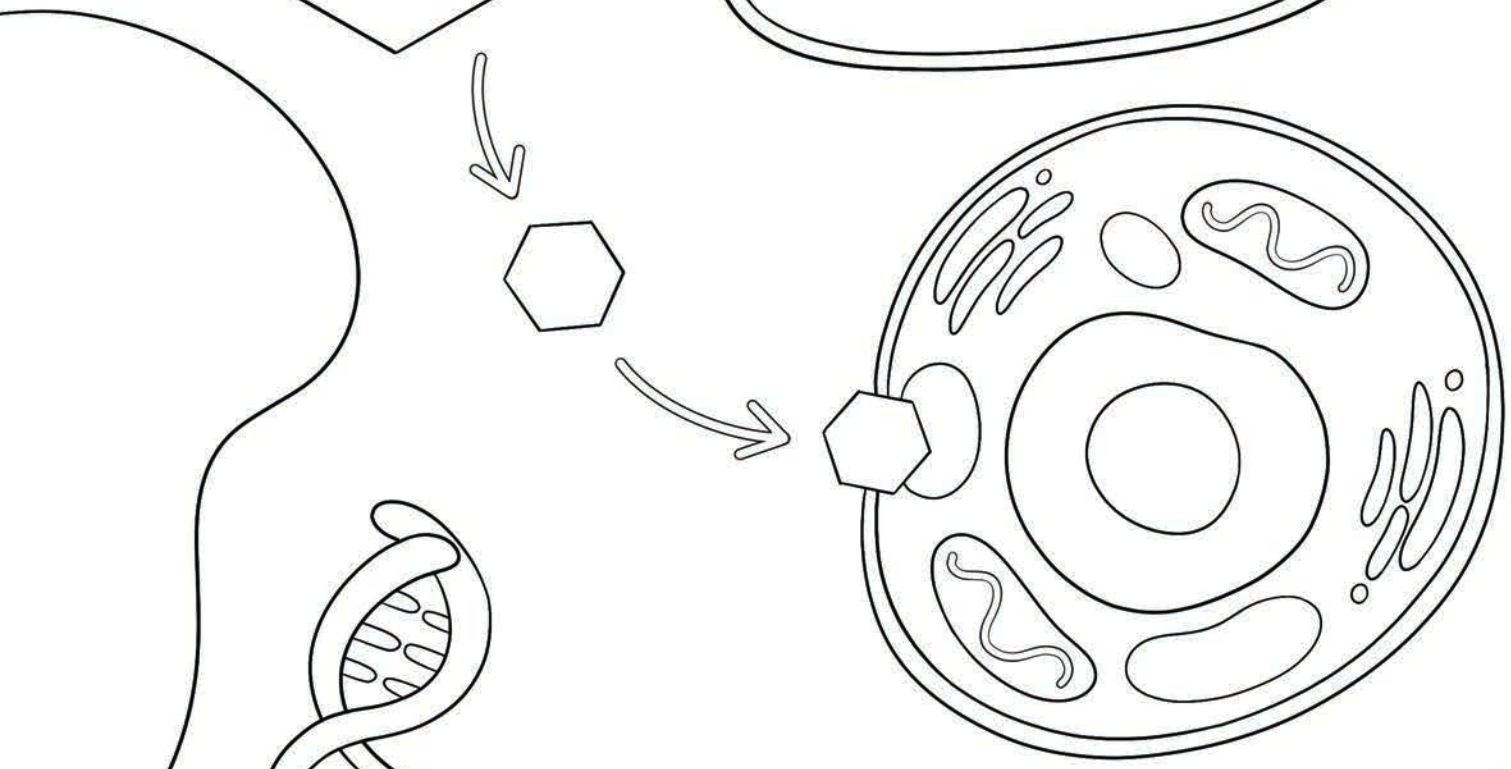


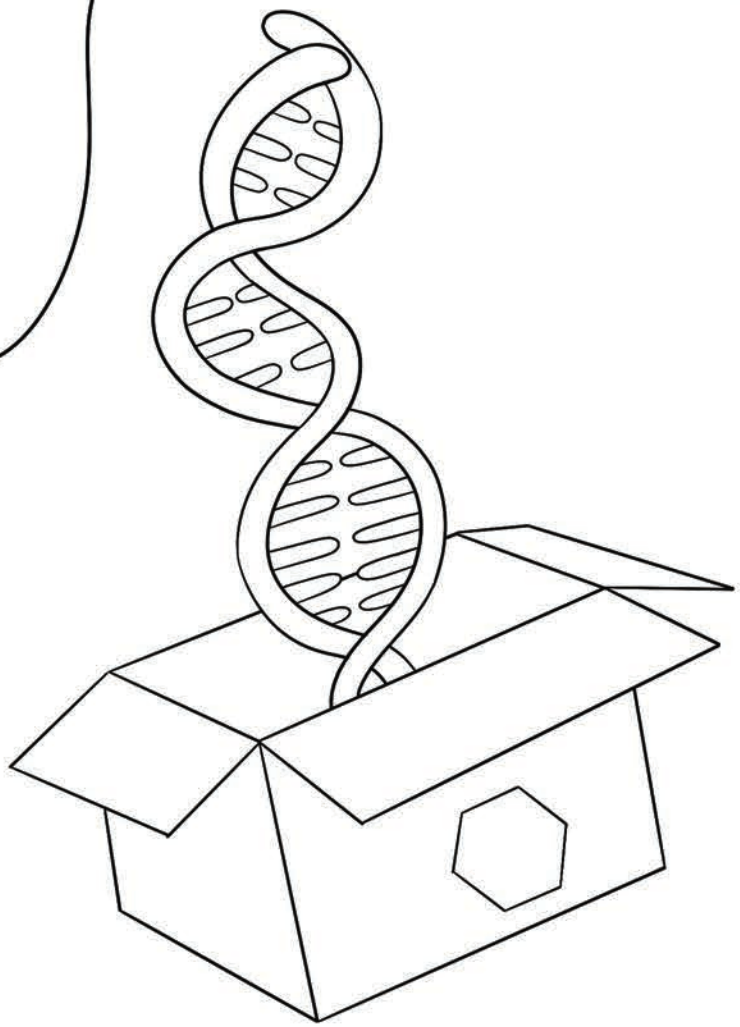
For some diseases, the genetic material could make changes to account for too much, not enough, or incorrect proteins being produced by cells.

VECTOR

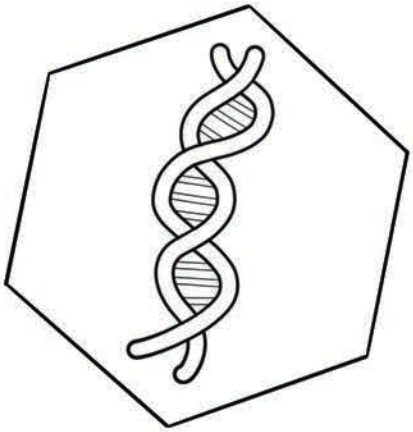


The new genetic material, such as a working copy of a gene, is delivered into the cell using a vector.





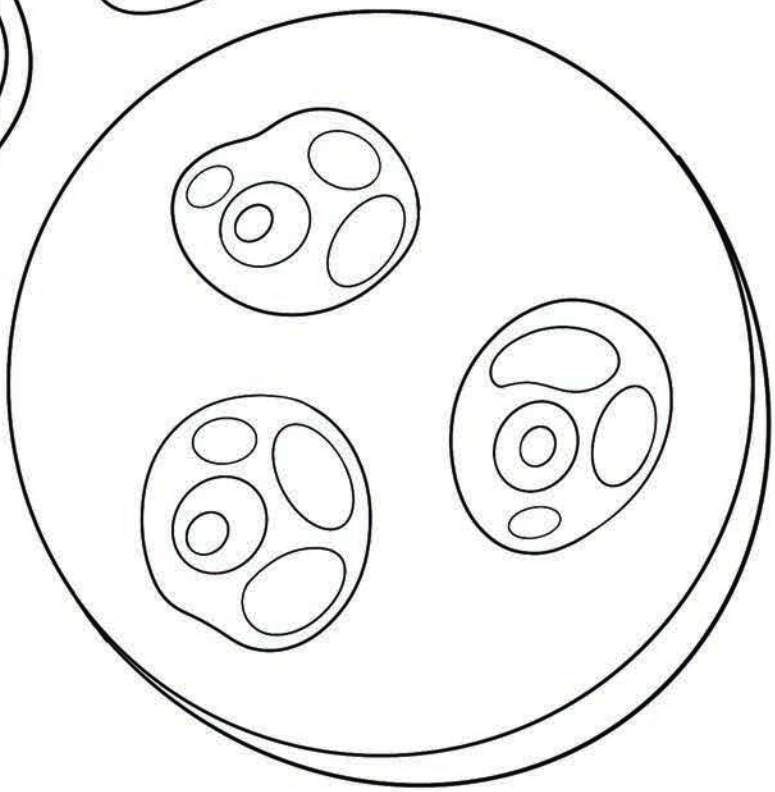
A vector is like a package and inside is a specific message for cells. Viruses can be used as vectors because they have evolved to be very good at getting into cells.

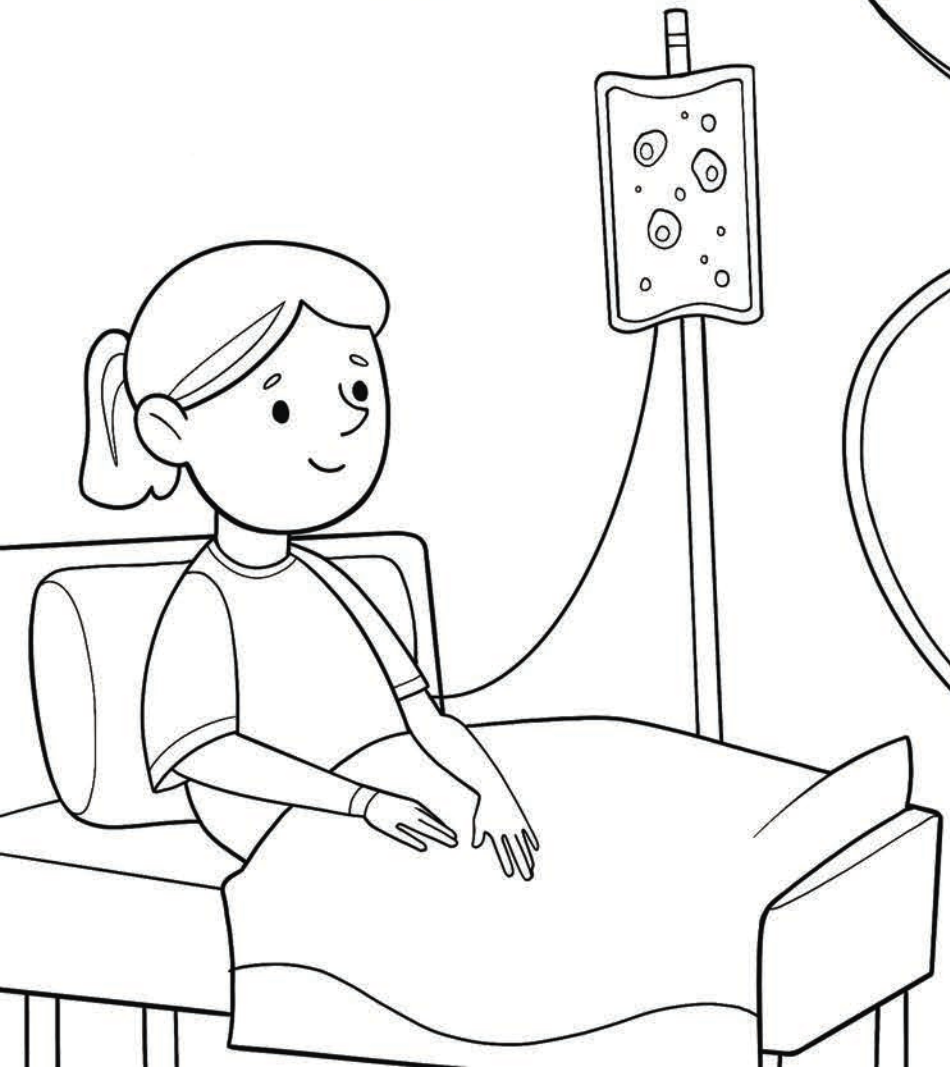


Scientists have learned to remove the viral genes that could cause illness, and keep the virus's useful parts to treat or prevent disease.

Ex Vivo

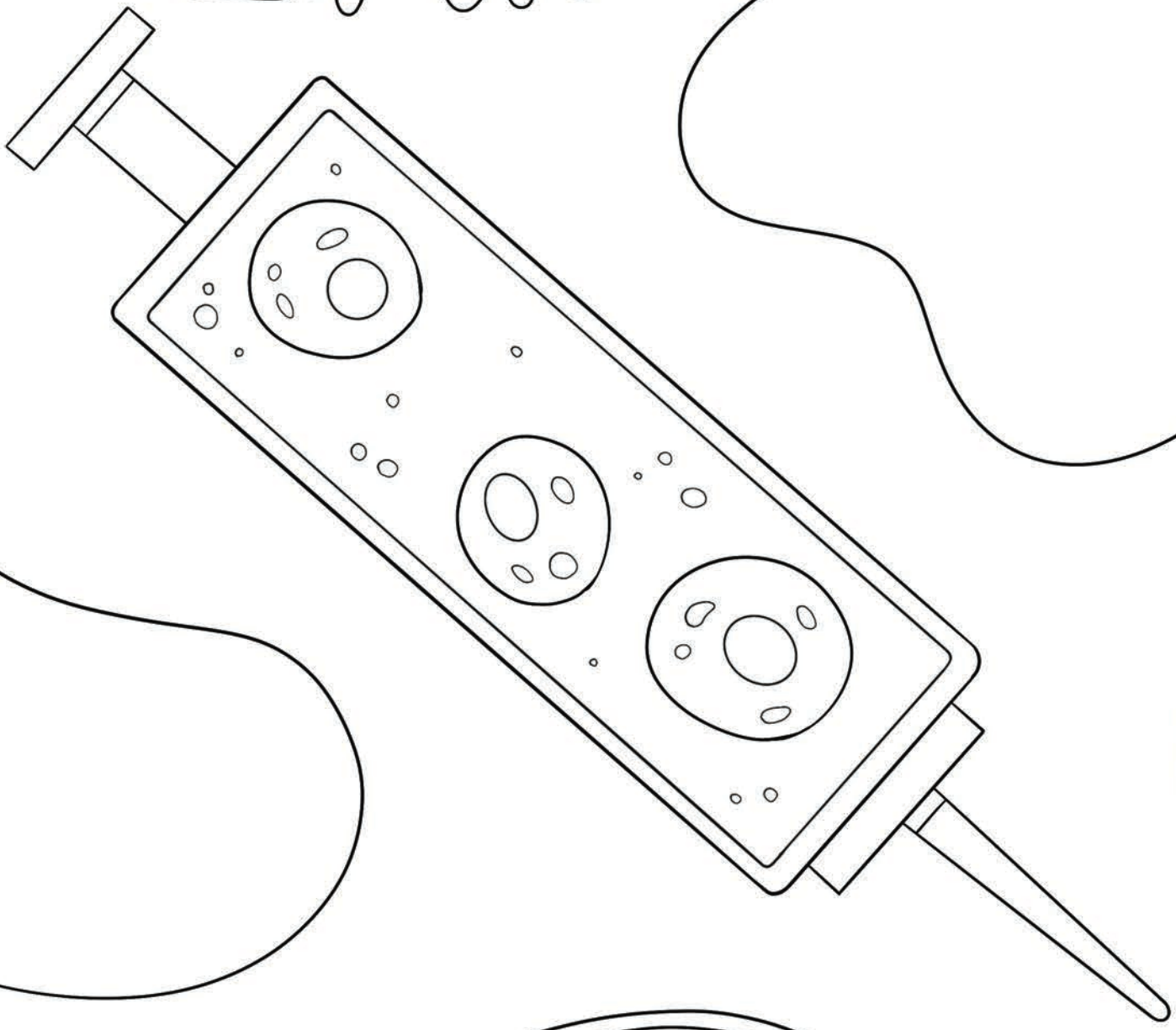
Ex vivo (outside the body) treatment removes the person's own cells and delivers the genetic material to these cells in a specialized lab.





The modified cells are then returned to the body to help it function better.

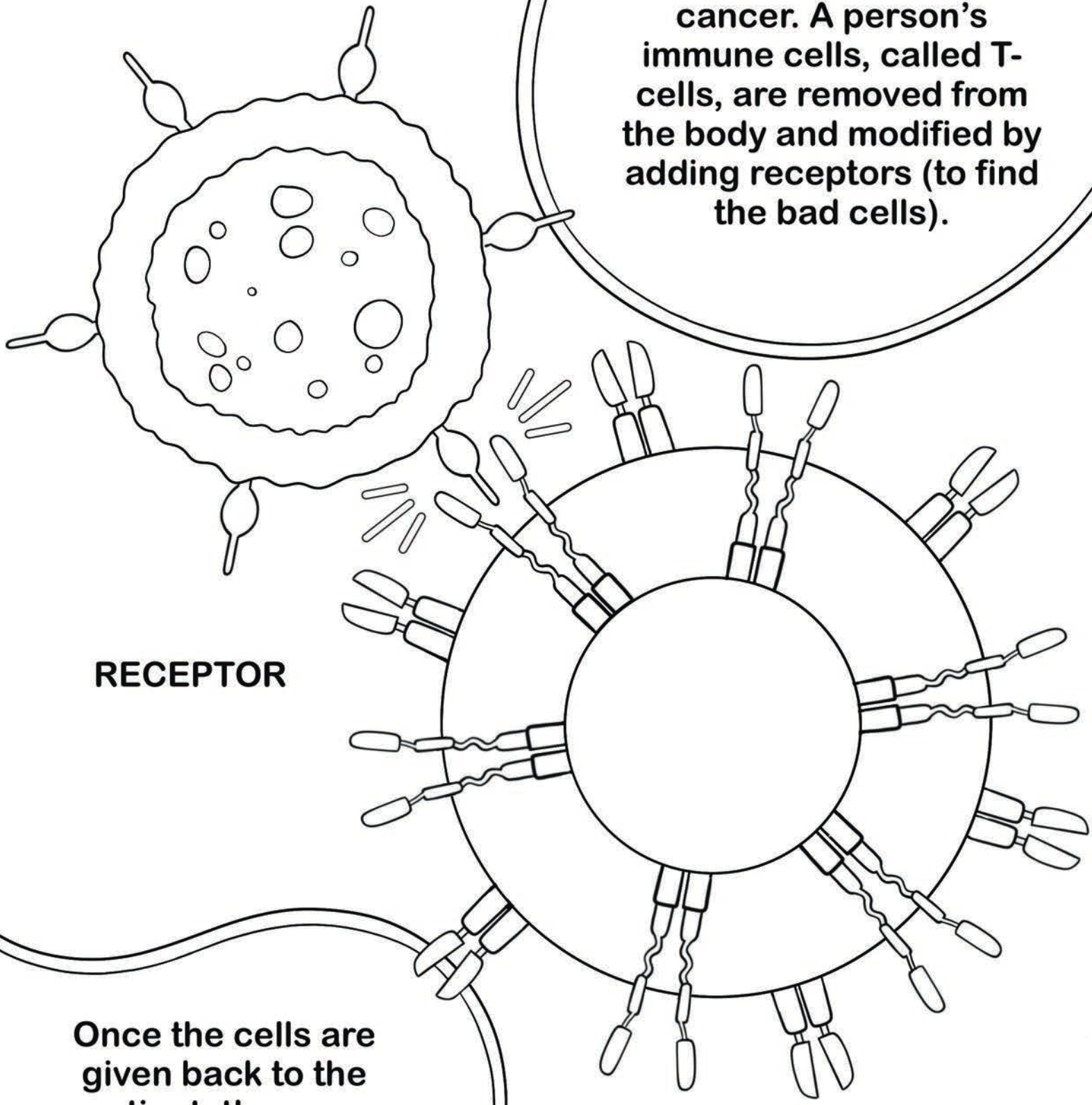
In Vivo



***In vivo* (inside the body) treatment means the genetic material is delivered directly into the person such as through an injection or infusion.**

CAR T-cell therapy is used to treat certain forms of cancer. A person's immune cells, called T-cells, are removed from the body and modified by adding receptors (to find the bad cells).

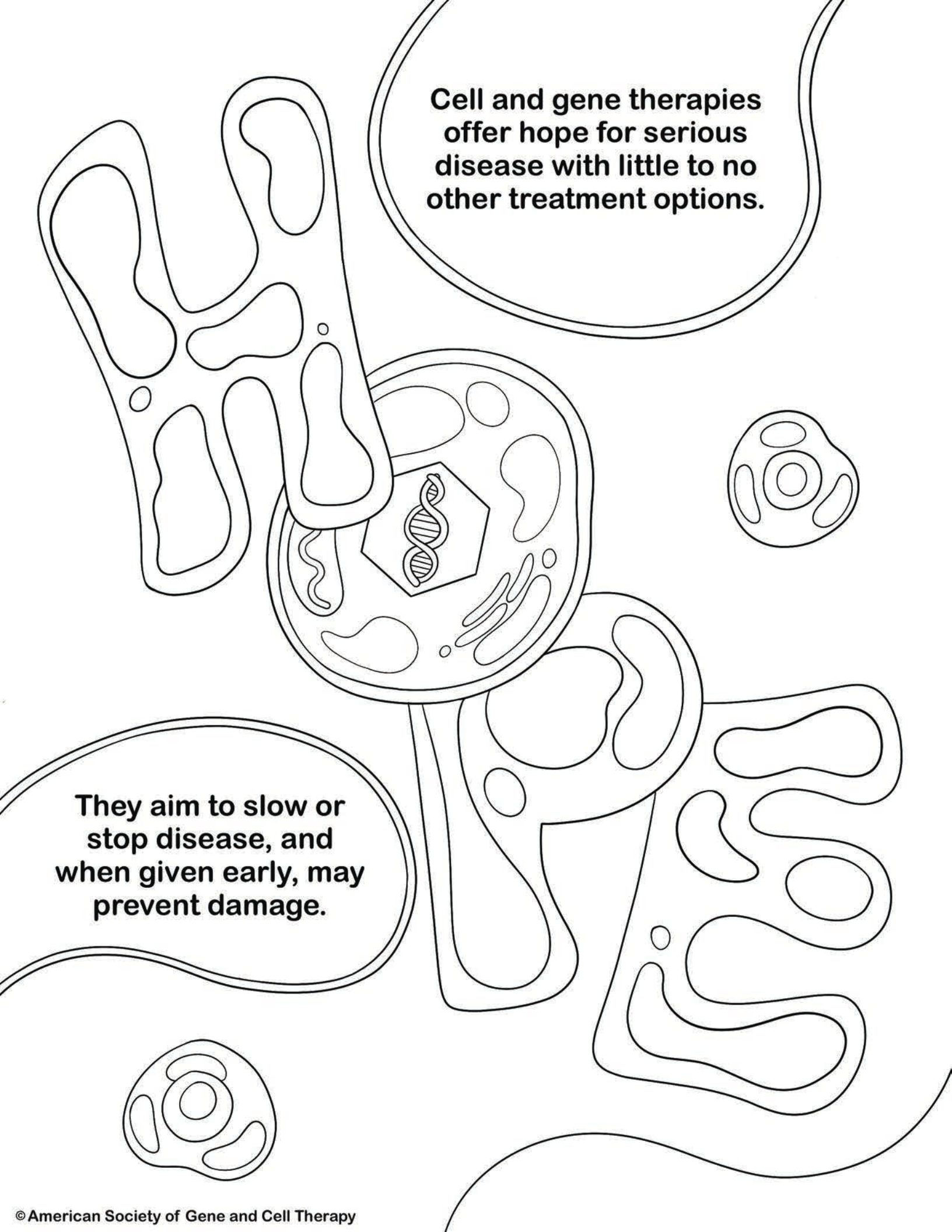
CANCER CELL

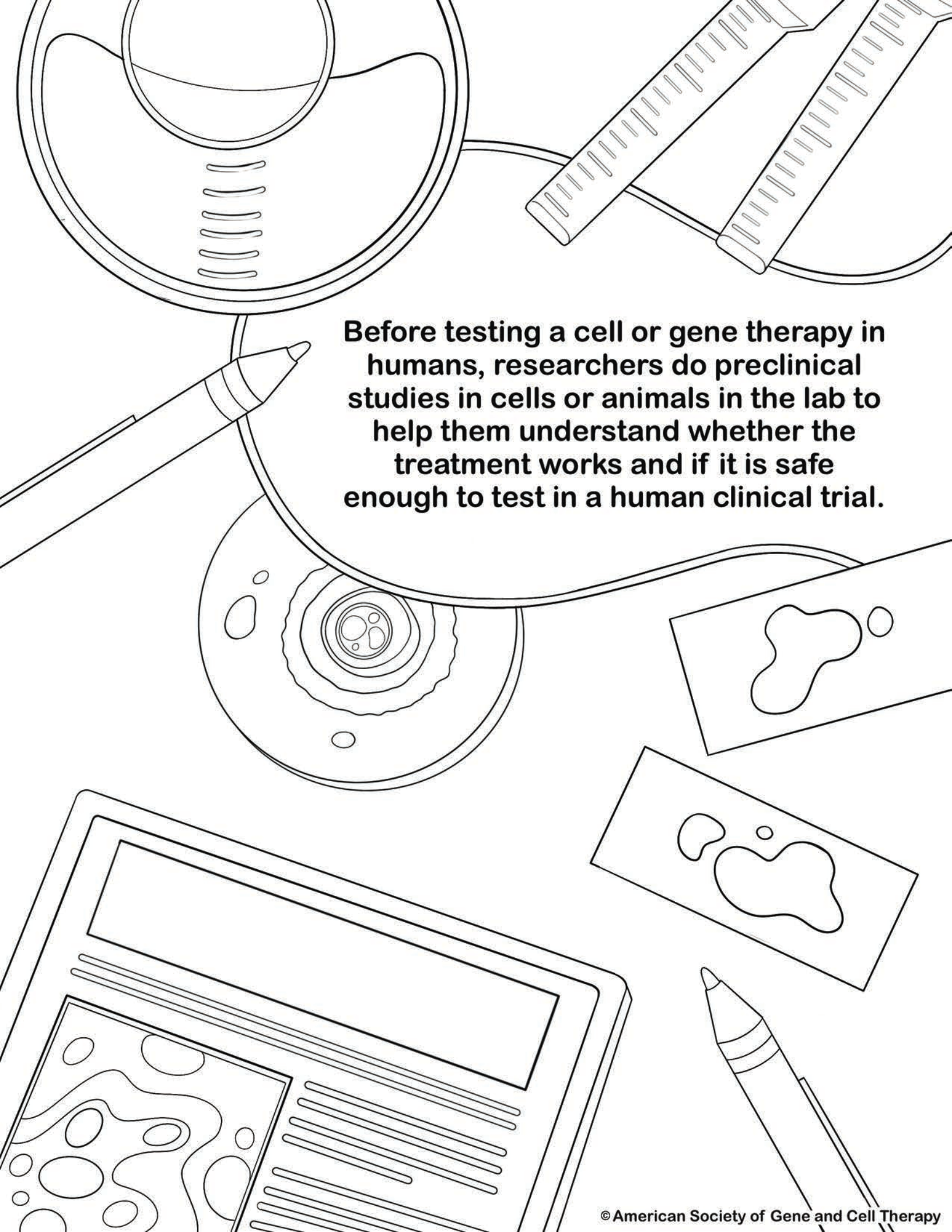


RECEPTOR

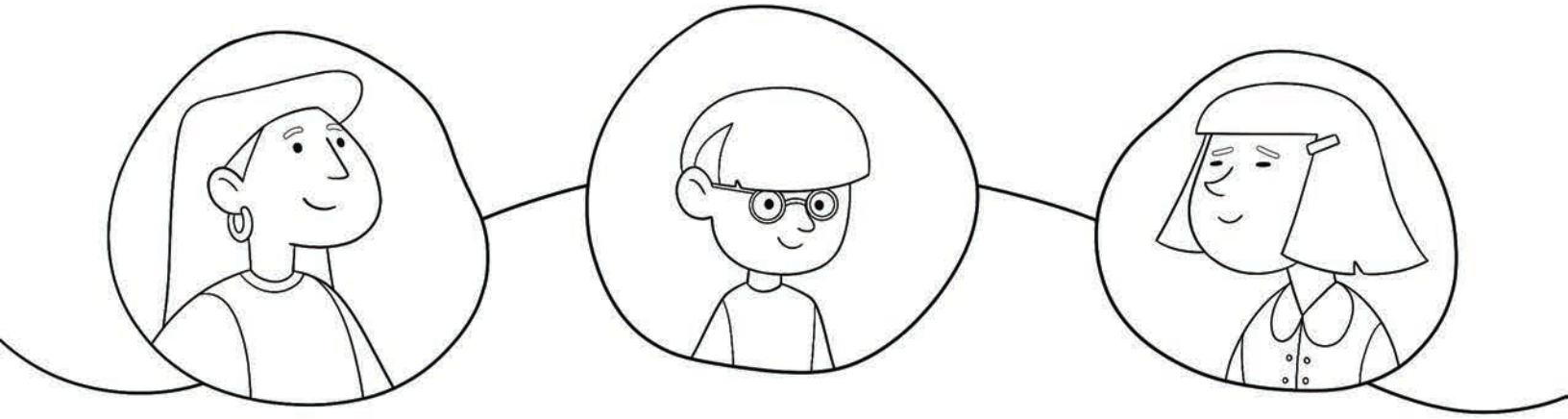
MODIFIED T-CELL

Once the cells are given back to the patient, they can recognize and kill cancer cells.

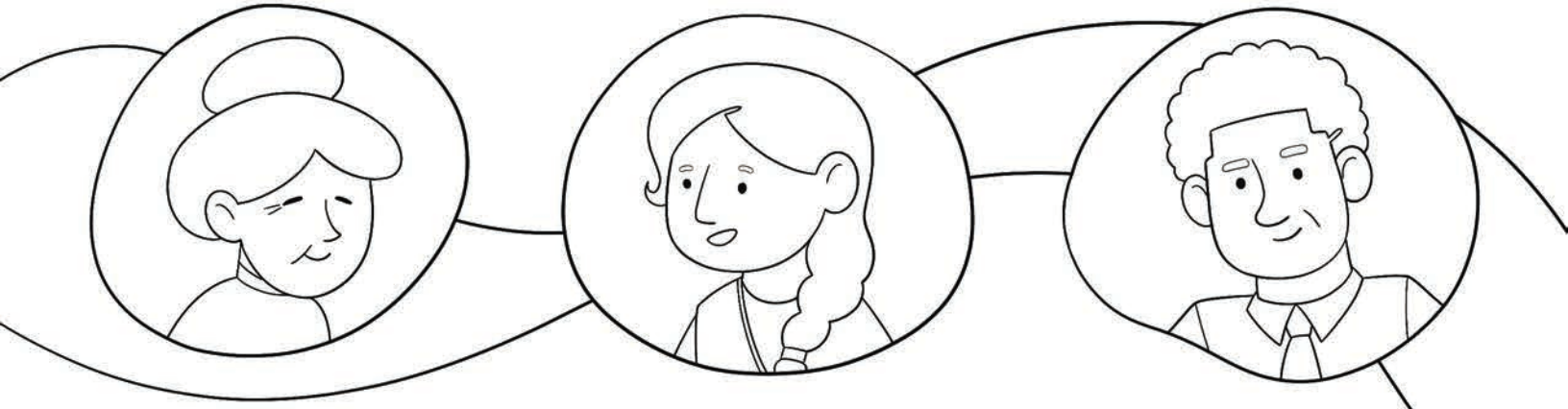




Before testing a cell or gene therapy in humans, researchers do preclinical studies in cells or animals in the lab to help them understand whether the treatment works and if it is safe enough to test in a human clinical trial.



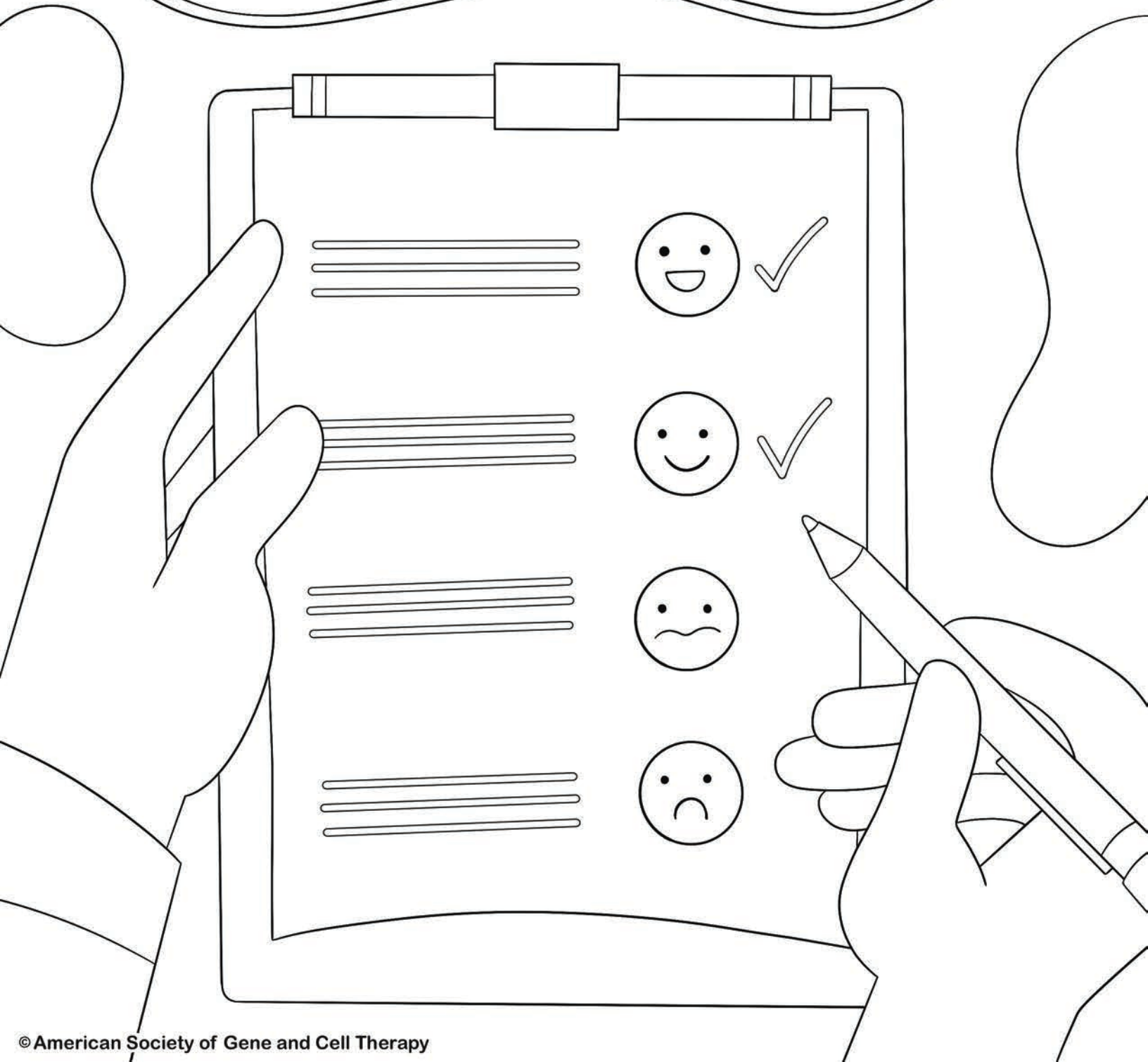
Clinical trials are studies that test the therapy in a small number of people to see how it works in the body and whether it is safe and effective.



This part of the research process is complex and may take years before the U.S. Food and Drug Administration (FDA) can approve a new treatment to be ready for more people.

To search for clinical trials, visit clinicaltrials.gov.

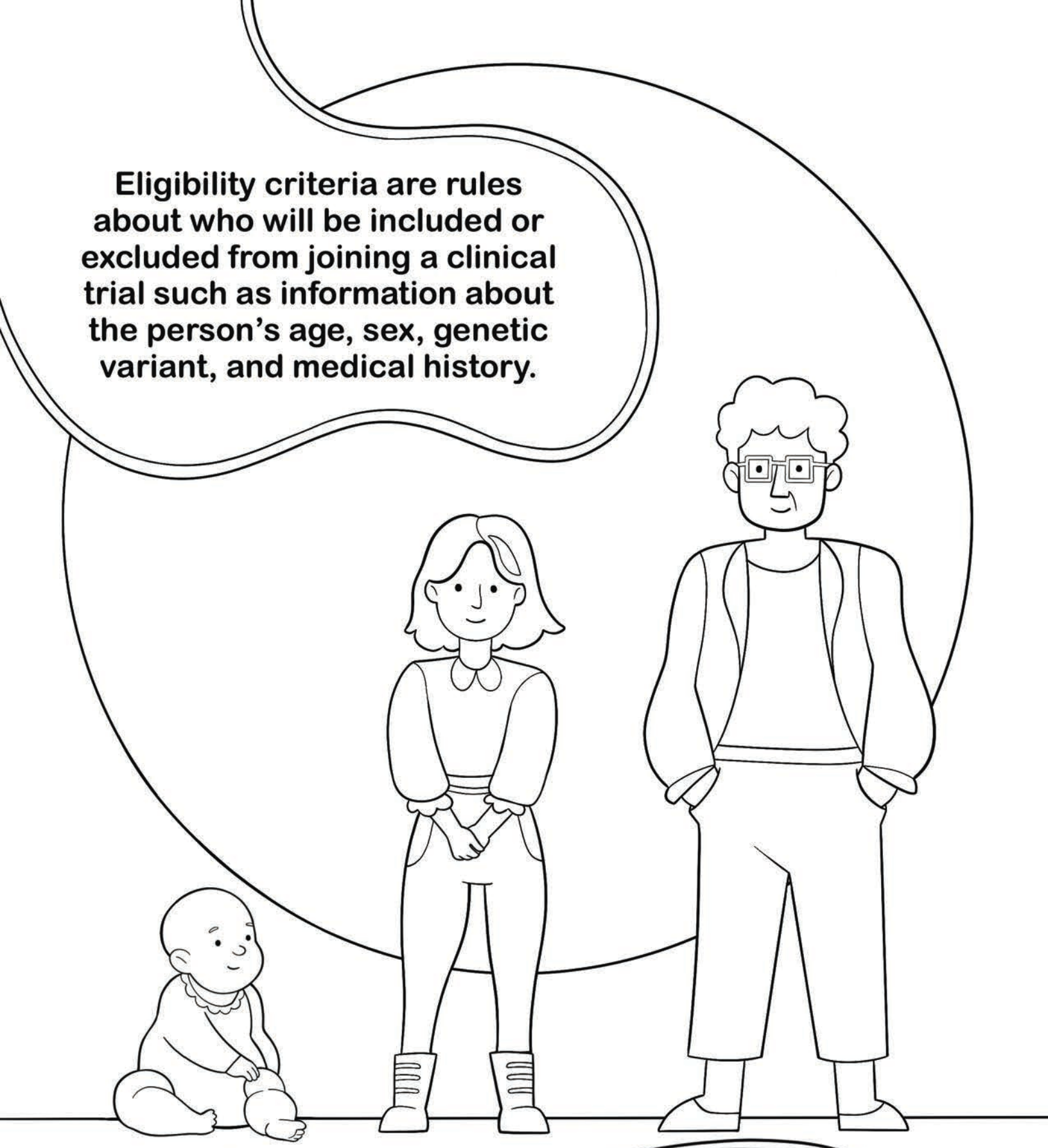
Cell and gene therapies come with unique risks that are important to understand before participating. For example, it is not a guaranteed cure, there may be unknown effects, and joining a clinical trial could limit future eligibility for other treatments.



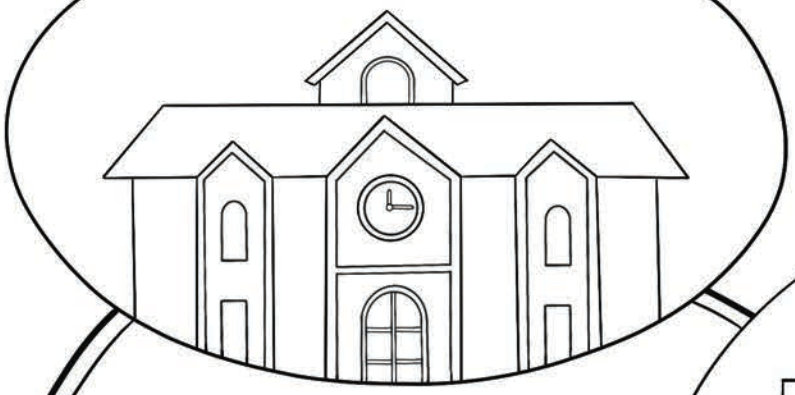
Informed consent is an important step before joining a clinical trial.

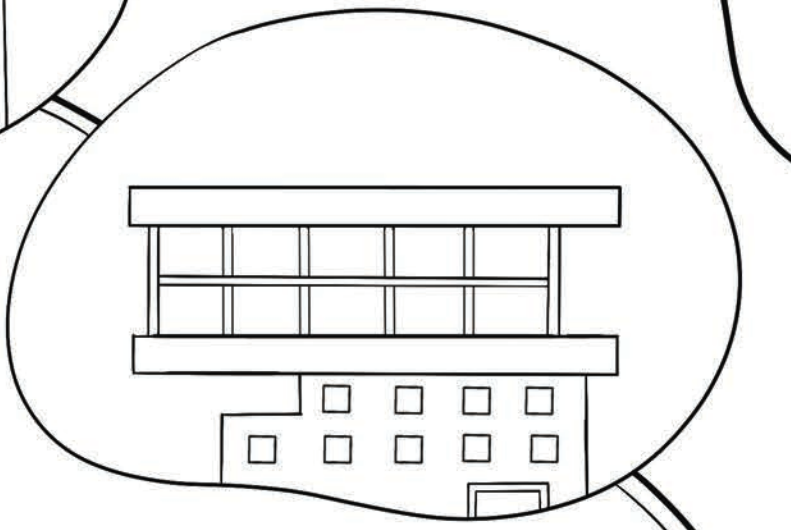
During an informed consent discussion the researchers explain the study, the participant's rights, and what to expect before, during and after— all to help them decide if they want to take part in the study.

Eligibility criteria are rules about who will be included or excluded from joining a clinical trial such as information about the person's age, sex, genetic variant, and medical history.

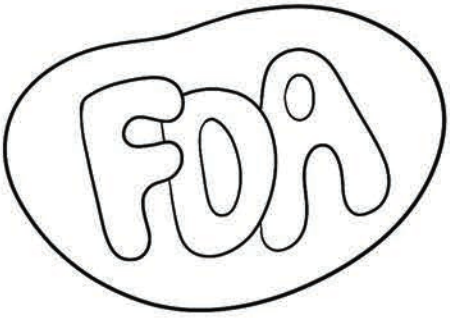


They help researchers better understand the results to know if the treatment works.





People from universities, biotech companies, hospitals, patient advocacy groups and the FDA all work together to research, test, review and deliver safe and effective cell and gene therapies to patients.



Today, the FDA has approved many cell and gene therapies that use special cells or helpful genes to treat certain cancers, blood disorders, and many different rare genetic diseases that affect the eyes, muscles, brain and more.

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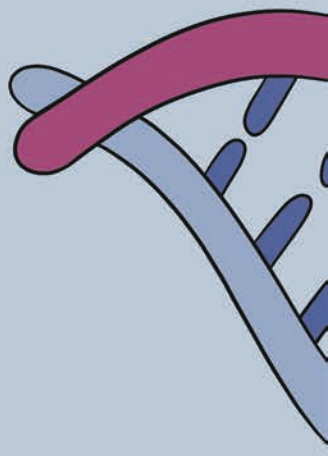

asgct.org
For more resources, please visit
patienteducation.asgct.org

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