

Key focus areas ^[1]

At Novartis, we are reimagining medicine in an effort to produce breakthroughs and address major unmet needs for patients with devastating diseases, including genetic disorders and certain deadly cancers. These efforts are done in collaboration with scientists, physicians, academia, industry partners, and patients.

In 2017, Novartis received approval of the first CAR-T cell therapy which is both a cell therapy and a gene therapy. Currently, there are three key focus areas for research and development of potentially transformative cell and gene therapies at Novartis: AAV-based therapies, CAR-T cell therapies and CRISPR-based technologies.

AAV-based¹

AAV (adeno-associated virus)-based therapies have the power to deliver new or working copies of a missing or nonworking gene to human cells. As AAV seems not to be associated with any disease, it is considered a safe and attractive vector for gene delivery. Novartis is exploring the use of AAV-based therapies across neurology and ophthalmology by delivering new genes into patient cells.

CAR-T²

Individualized CAR (chimeric antigen receptor) T-cell therapy uses a patient's own immune system to fight certain types of cancers. A patient's T-cells are extracted and reprogrammed outside of the body to recognize and fight cancer cells and other cells expressing a particular antigen. CAR-T cell therapies are currently approved by various regulatory bodies to treat certain forms of advanced B-cell blood cancers. Novartis has a deep CAR-T pipeline. Our focus is to broaden the impact of cell therapy in oncology by going deeper in B-cell malignancies, potentially reaching patients in other hematological cancers and solid tumors and researching potential next-generation CAR-Ts that focus on new targets and utilize new technologies.

[Learn more about CAR-T cell therapy](#) ^[2]

CRISPR-based³

CRISPR (clustered regularly interspaced short palindromic repeats)-based technologies create double-stranded breaks in DNA, acting like a pair of molecular scissors that can be used to insert, remove, or replace specific pieces of a person's existing DNA. Novartis is conducting early research on the use of CRISPR-based technologies across hematology and

ophthalmology to potentially treat diseases by correcting genetic defects.

Learn more about the areas where these therapies become effective

[View the infographic](#) ^[3]

Footnotes:

References

1. Daya S, Berns KI. Gene therapy using adeno-associated virus vectors. *Clin Microbiol Rev.* 2008;21(4):583–593.
2. Tisagenlecleucel prescribing information. East Hanover, New Jersey, USA: Novartis Pharmaceuticals Corporation; May 2018.
3. Ran FA, et al. Genome engineering using the CRISPR-Cas9 system. *Nat Protoc.* 2013;8(11):2281–2308.

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Links

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[3] <https://www.novartis.com/news/media-library/cell-and-gene-key-focus-areas>