

We are building a pipeline of *in vivo* and *ex vivo* therapies, as well as continuing to develop innovative modular platform capabilities.

Our Pipeline



In Vivo CRISPR **is** the therapy

GENETIC DISEASES

Last updated:
January 5, 2023

| Program | Approach | Research | IND-Enabling | Early-Stage Clinical | Late-Stage Clinical | Partner |
|--------------------------------------|--|----------|--------------|----------------------|---------------------|--|
| NTLA-2001: Transthyretin Amyloidosis | Knockout | ▶ | | | | LEAD Inteilia [*] REGENERON |
| NTLA-2002: Hereditary Angioedema | Knockout | ▶ | | | | Inteilia |
| NTLA-2003: AATD-Liver Disease | Knockout | ▶ | | | | Inteilia |
| NTLA-3001: AATD-Lung Disease | Insertion | ▶ | | | | Inteilia |
| Hemophilia B | Insertion | ▶ | | | | LEAD Inteilia [*] REGENERON |
| Hemophilia A | Insertion | ▶ | | | | LEAD Inteilia [*] REGENERON |
| Research Programs | Knockout, Insertion, Consecutive Edits | ▶ | | | | Inteilia |
| Research Programs | Various | ▶ | | | | Inteilia ^{**} REGENERON SPRINGVISION |

* Lead development and commercial party ** Rights to certain *in vivo* targets

AATD: Alpha-1 Antitrypsin Deficiency

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Ex Vivo

CRISPR **creates** the therapy

IMMUNO-ONCOLOGY / AUTOIMMUNE DISEASE

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|---------------------------------------|--------------------|-------------|--------------|----------------------|---------------------|---|
| OTQ923 / HIX763: Sickle Cell Disease | HSC | ▶ | | | | *** Inte:ia THERAPEUTICS NOVARTIS |
| NTLA-6001: CD30+ Lymphomas | Allo CAR-T | ▶ | | | | Inte:ia THERAPEUTICS |
| Acute Myeloid Leukemia / Solid Tumors | WTI-TCR | ▶ | | | | Inte:ia THERAPEUTICS |
| Research Programs | Allo - Undisclosed | ▶ | | | | Inte:ia THERAPEUTICS |
| Research Programs | Various | ▶ | | | | Inte:ia THERAPEUTICS VENCELL kyverna ONK THERAPEUTICS |
| Other Novartis Programs | CAR-T, HSC, OSC | Undisclosed | | | | *** Inte:ia THERAPEUTICS NOVARTIS |

*** Milestones & royalties only

CAR-T: Chimeric Antigen Receptor T cells

HSC: Hematopoietic Stem Cells

OSC: Ocular Stem Cells

TCR: T Cell Receptor