

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:  
February 23, 2023

Program	Approach	Research	IND-Enabling	Early-Stage Clinical	Late-Stage Clinical	Partner
NTLA-2001: Transthyretin Amyloidosis	Knockout	▶				LEAD Inteilia <sup>*</sup> 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 <sup>*</sup> REGENERON
Hemophilia A	Insertion	▶				LEAD Inteilia <sup>*</sup> REGENERON
Research Programs	Knockout, Insertion, Consecutive Edits	▶				Inteilia
Research Programs	Various	▶				Inteilia <sup>**</sup> REGENERON SPRINGVISION

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

**AATD:** Alpha-1 Antitrypsin Deficiency

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## Our Pipeline



### Ex Vivo

CRISPR **creates** the therapy

IMMUNO-ONCOLOGY / AUTOIMMUNE DISEASE

Last updated:  
February 23, 2023

Program	Approach	Research	IND-Enabling	Early-Stage Clinical	Late-Stage Clinical	Partner
NTLA-6001: CD30+ Lymphomas	Allo CAR-T					
Acute Myeloid Leukemia / Solid Tumors	WT1-TCR					
Research Programs	Allo - Undisclosed					
Research Programs	Various					   
Other Novartis Programs	CAR-T, HSC, OSC	Undisclosed				 *** 

\*\*\* Milestones & royalties only

**CAR-T:** Chimeric Antigen Receptor T cells

**HSC:** Hematopoietic Stem Cells

**OSC:** Ocular Stem Cells

**TCR:** T Cell Receptor