

Our Pipeline

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

Program	Approach	Research	Candidate Selection	IND-Enabling	Early-Stage Clinical	Late-Stage Clinical	
<i>In Vivo: CRISPR is the therapy</i>							
NTLA-2001: Transthyretin Amyloidosis	Knockout	[Progress bar]					LEAD Intellia [®] + REGENERON
NTLA-2002: Hereditary Angioedema	Knockout	[Progress bar]					Intellia [®]
Hemophilia A and B	Insertion	[Progress bar]					LEAD REGENERON + Intellia [®]
Research Programs	Knockout, Insertion, Consecutive Edits	[Progress bar]					Intellia [®]
Research Programs	Various	[Progress bar]					Intellia [®] REGENERON [®]
<i>Ex Vivo: CRISPR creates the therapy</i>							
OTQ923 / HIX763: Sickle Cell Disease	HSC	[Progress bar]					NOVARTIS Intellia [®] ***
NTLA-5001: Acute Myeloid Leukemia	WT1-TCR	[Progress bar]					Intellia [®]
Solid Tumors	WT1-TCR	[Progress bar]					Intellia [®]
Undisclosed Programs	Undisclosed	[Progress bar]					Intellia [®]
Other Novartis Programs	CAR-T, HSC, OSC	Undisclosed					NOVARTIS Intellia [®] ***



* Lead development and commercial party
CAR-T: Chimeric Antigen Receptor T cells

** Rights to certain *in vivo* targets
HSC: Hematopoietic Stem Cells

*** Milestones & royalties
OSC: Ocular Stem Cells

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