



Revolutionizing
Medicine

A Breakthrough
Scientific Discovery

Curative
Treatments

Targeting the
Underlying
Genetic Cause
of Disease

Investor Presentation
Barclay's Global Healthcare Conference

2018



Intellia
THERAPEUTICS

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Intellia Value Drivers

Genome
Editing



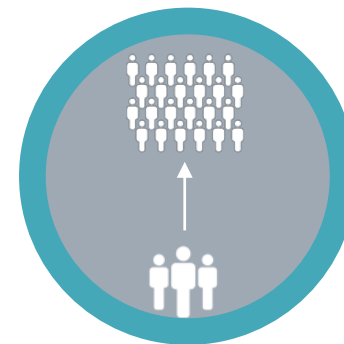
Proprietary
Delivery System



Diversified
Pipeline



Scalable
Application

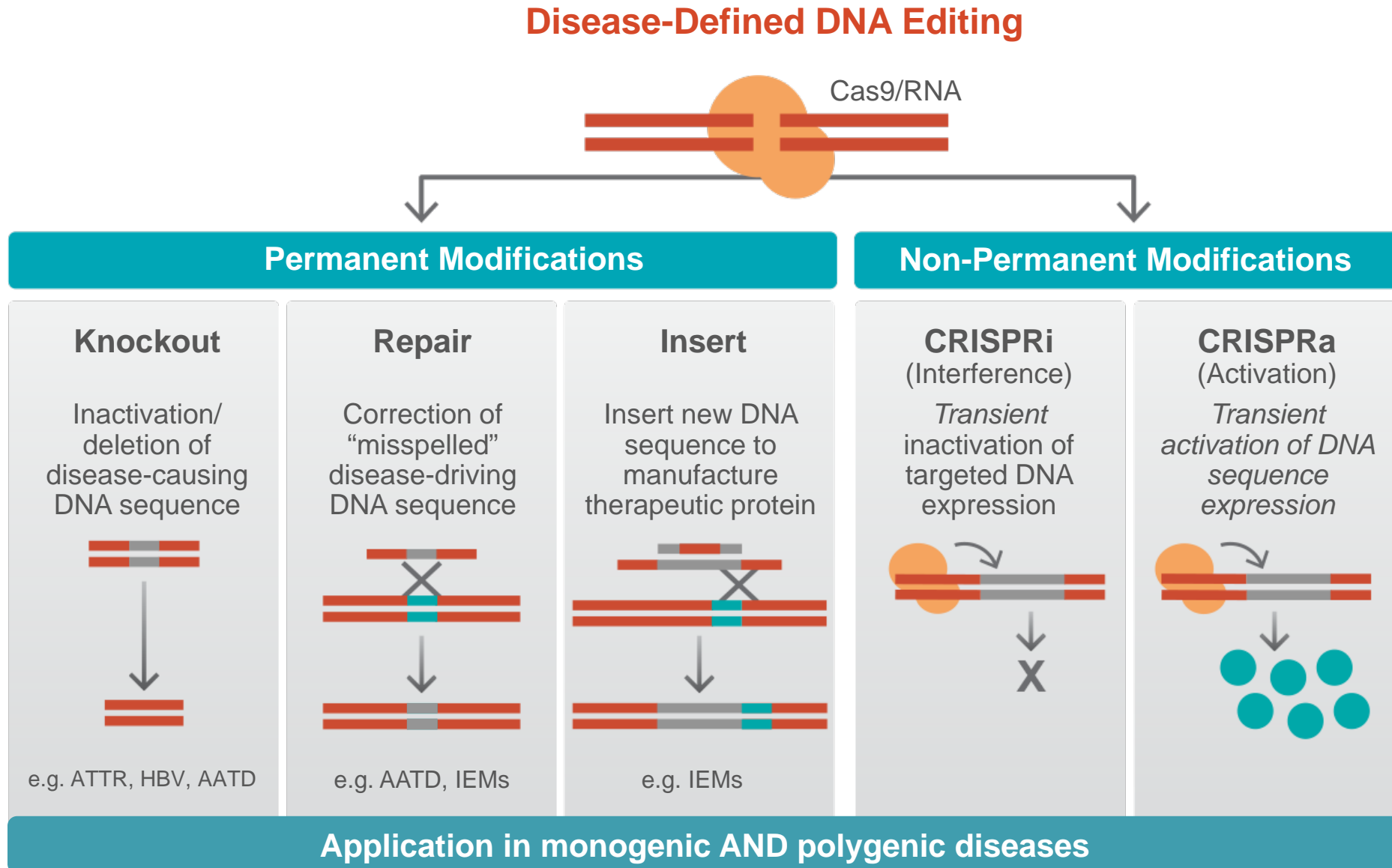


Committed
Partnerships

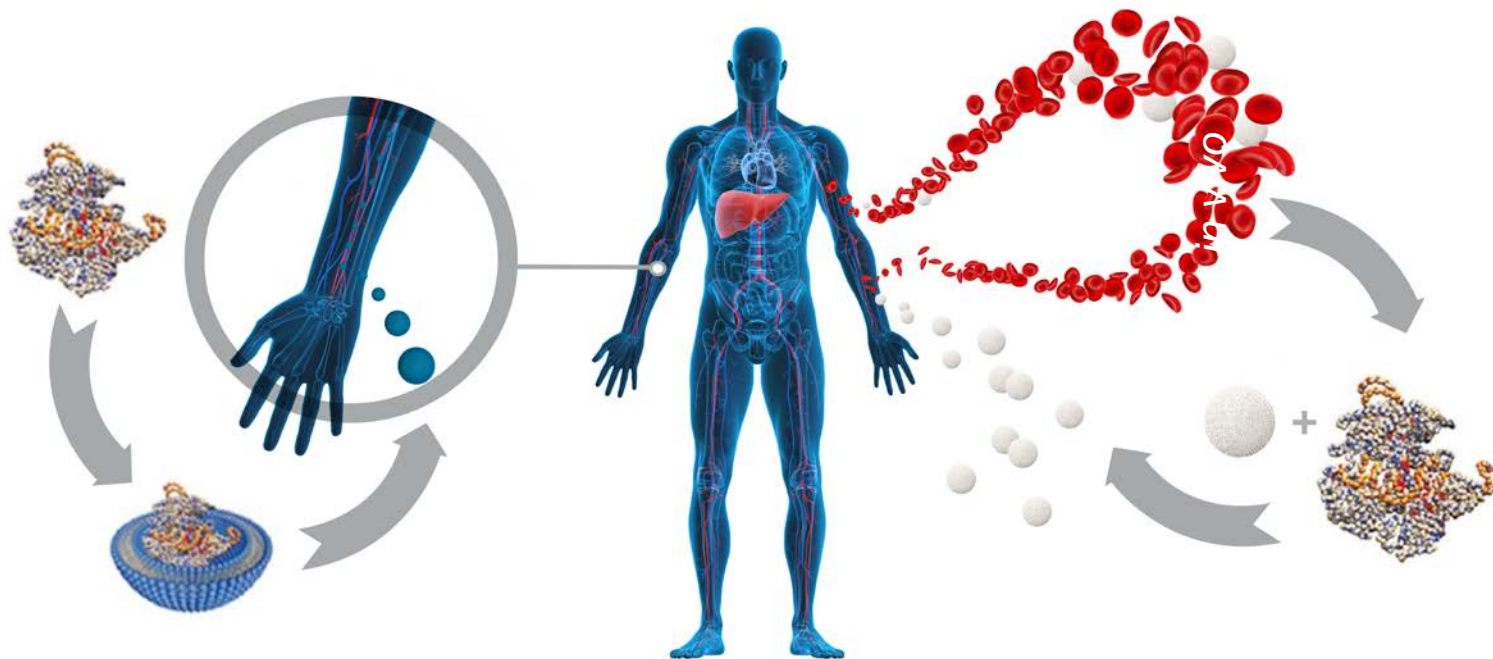


First company to demonstrate dose-dependent CRISPR/Cas9 liver editing in non-human primates

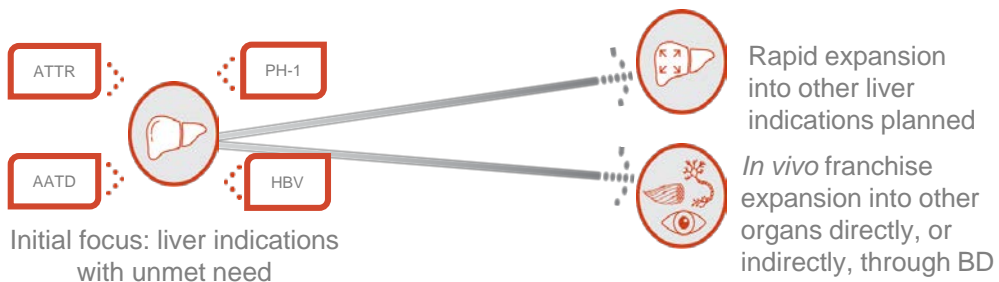
Genome Editing Approaches to Address Wide Spectrum of Genetic Diseases



Diversified Opportunities with *in vivo* and *ex vivo* Delivery



in vivo



ex vivo

Oncology



- T cell therapy
- NK cell therapy

Auto-Immune Disease

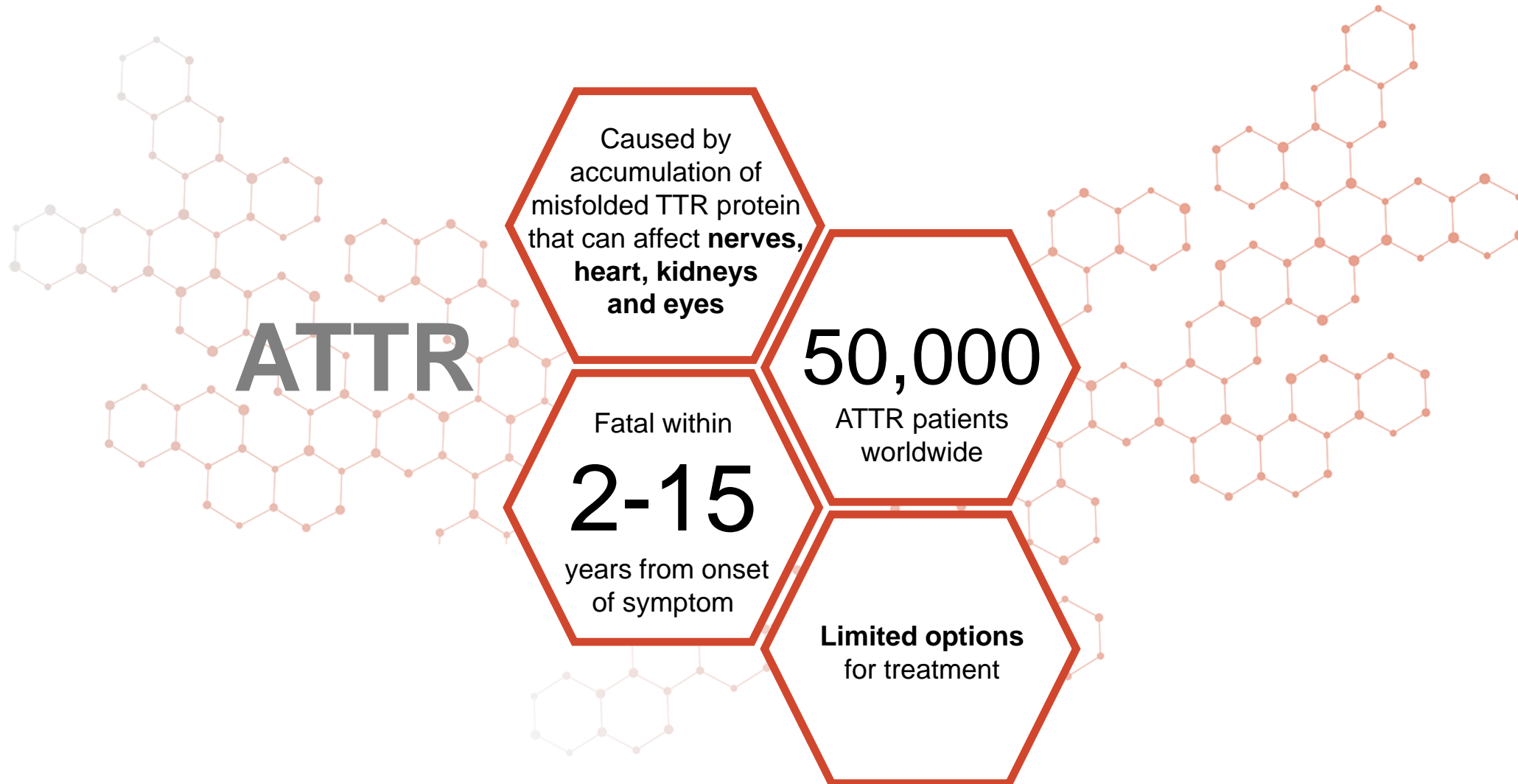


- Type 1 Diabetes
- Crohn's Disease

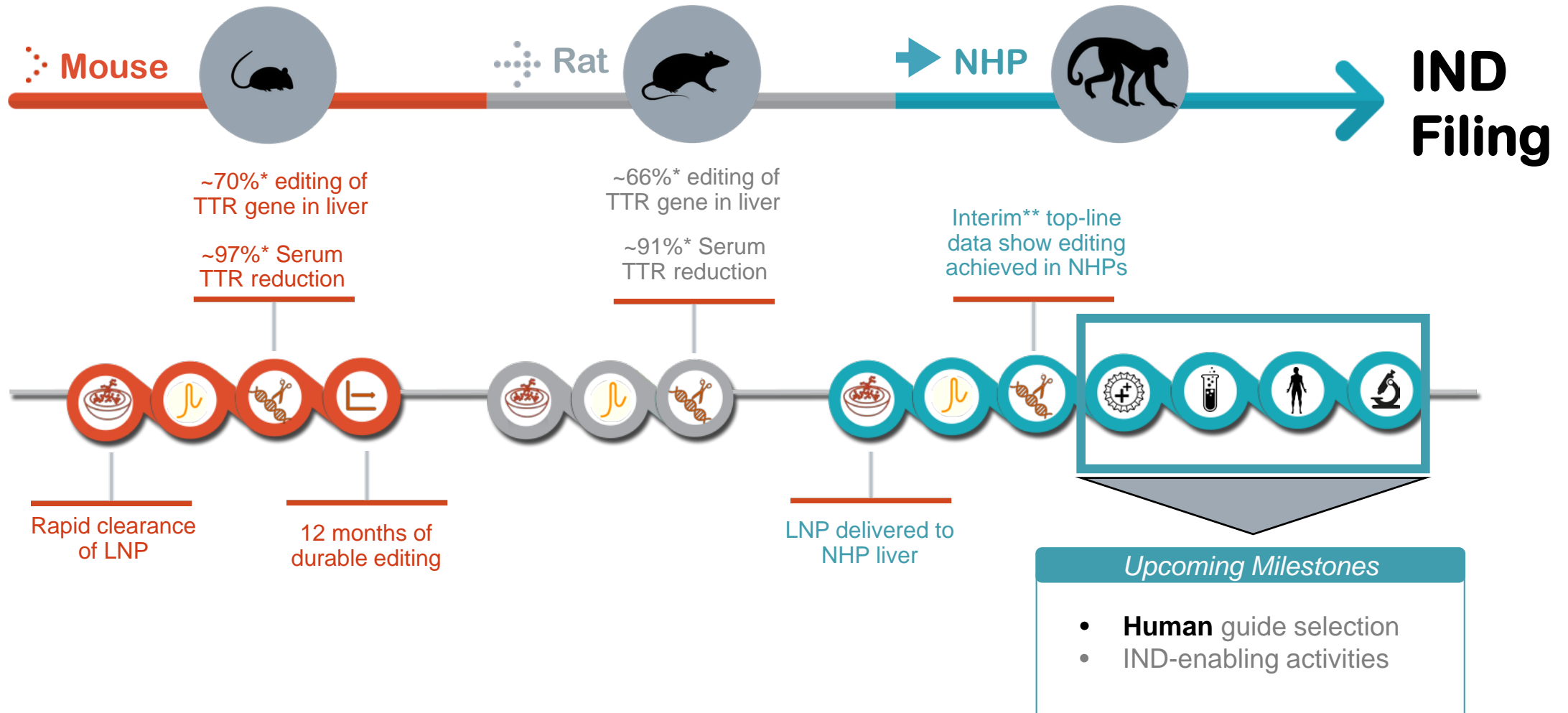
R&D Programs

	Programs	Program Lead	Status
in vivo	ATTR (Transthyretin Amyloidosis)	Intellia REGENERON	NHP Studies
	HBV (Hepatitis B)	Intellia	Animal Model Studies
	AATD (Alpha-1 Antitrypsin Deficiency)	Intellia	Guide Design & Evaluation
	PH-1 (Primary Hyperoxaluria Type 1)	Intellia	Guide Design & Evaluation
ex vivo	HSC (Hematopoietic Stem Cells)	Intellia NOVARTIS	Late Stage Preclinical Development
	CAR-T Cells (Chimeric Antigen Receptor)	NOVARTIS	Preclinical Development
	I-O (Immuno-Oncology)	extellia THERAPEUTICS	Preclinical Discovery
	AIID (Autoimmune and Inflammatory Diseases)	extellia THERAPEUTICS	Preclinical Discovery

Lead *in vivo* Program: ATTR



Roadmap to IND Filing



*Optimized

**Prior to optimization



LNP Delivery to Liver



Durability



Optimize Guide



Human Guide Selection



Edit in Liver



Guide Selection



Optimize Formulation



IND Enabling Activities

Intellia Recent Accomplishments

In non-human primates:

- First company to demonstrate dose dependent CRISPR/Cas9 editing in non-human primates
- First company to demonstrate CRISPR/Cas9 editing via lipid nanoparticles
- First company to demonstrate *in vivo* CRISPR/Cas9 editing at a disease-causing gene in NHP livers
- Showed that redosing increased genome editing levels in the liver
- Demonstrated that CRISPR/Cas9 leads to TTR serum protein reduction
- Observed a safe and well-tolerated profile following single and repeat dosing

In rodents:

- Demonstrated well-tolerated, year-long editing durability in *in vivo* mouse studies
- Demonstrated well-tolerated *in vivo* liver genome editing in rats

Path to clinic:

- Commenced characterization of human guides in *in vivo* NHP studies for identification of development candidate

Upcoming Goals

in vivo

- Nominate first Intellia development candidate
- Initiate IND-enabling activities for first development candidate
- Hold pre-IND meeting with regulatory authorities
- Intellia selection of hematopoietic stem cell target in Novartis collaboration
- Demonstrate *in vivo* insertion/repair edit in non-human primates
- Advance additional *in vivo* liver target(s)
- Progress delivery and editing on second organ in non-human primates

ex vivo

- Develop key data on generalized allogeneic engineered cell therapy
- Generate preclinical data on T cell candidate for immuno-oncology indication
- Deliver preclinical data for Treg-based candidate in auto-immune related disease

Industry Leading Investment Thesis



Scalable Application



Committed Partnerships



Proprietary Delivery System



Robust, Global IP Portfolio

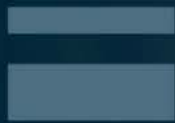


Diversified Pipeline



Strong Balance Sheet

Accelerating the development of life-transforming therapies



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NASDAQ: NTLA
www.intelliatx.com