



Revolutionizing
Medicine

A Breakthrough
Scientific Discovery

Curative
Treatments

Targeting the
Underlying
Genetic Cause
of Disease

Q3 2017 Earnings Presentation

October 31, 2017

Intellia
THERAPEUTICS

Intellia Therapeutics Legal Disclaimers

This presentation contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia") within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, but are not limited to, express or implied statements regarding Intellia's future financial or business performance, conditions, plans, prospects, trends or strategies and other financial or business matters; Intellia's ability to advance and expand the CRISPR/Cas9 technology to develop into human therapeutic products, as well as Intellia's CRISPR/Cas9 intellectual property portfolio; Intellia's ability to achieve stable liver editing; effective genome editing with a single treatment dose; the potential timing and advancement of Intellia's preclinical studies, including continuing non-human primate studies, and clinical trials; Intellia's ability to replicate results achieved in Intellia's preclinical studies in any future studies, including human clinical trials; the potential development of the *ex vivo* cell therapeutics through Intellia's eXtellia division, including the development of next-generation T cell therapies that address unmet needs in hematological and solid tumors, immuno-oncology and auto- immunity; the intellectual property position and strategy of Intellia's licensors; actions by government agencies; the impact of Intellia's collaborations with Ospedale San Raffaele, Novartis and Regeneron on Intellia's development programs; the potential timing of regulatory filings regarding Intellia's development programs; the potential commercialization opportunities, including value and market, for product candidates. Any forward-looking statements in this presentation are based on management's current expectations and beliefs of future events, and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: risks related to Intellia's ability to protect and maintain Intellia's intellectual property position; risks related to the ability of Intellia's licensors to protect and maintain their intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for Intellia's product candidates; the risk that any one or more of Intellia's product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies will not be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Novartis or Regeneron will not continue or will not be successful. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Intellia's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Intellia's most recent quarterly report on Form 10-Q filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in Intellia's other filings with the Securities and Exchange Commission. All information in this presentation is as of the date of the presentation, and Intellia Therapeutics undertakes no duty to update this information unless required by law.

Today's Presenters



Nesson Bermingham, Ph.D.
CEO, President and Founder



John Leonard, M.D.
EVP, Research & Development



Graeme Bell
EVP & Chief Financial Officer

Agenda

☰ Welcome and overview

R&D update

Financial results

Upcoming milestones

Q&A



Business and R&D Update

Business Update

- Collaboration revenue of \$7.3M
- R&D investment of \$17.5M
- 135 focused R&D personnel
- 34 general & administrative support staff
- 70:30 ratio of R&D : G&A capital deployment
- Cash balance of \$222M
- Loss per share of \$0.44

R&D Update

- Early results in non-human primates showed, for the first time with CRISPR/Cas9, liver genome editing of over 20 percent in certain animals
 - Dose-dependent levels of liver genome editing in non-human primates, with ability to redose
 - Well-tolerated safety profile
 - Confirmed rapid clearance of all components of the LNP system, as seen in rodent studies
- Demonstrated *in vivo* maintenance of liver genome editing in mice through 12 months
- Made progress in evaluating *in vivo* delivery by LNPs to a second organ, with successful genome editing by CRISPR/Cas9 in the central nervous system (cerebellum and striatum) in mice

Agenda

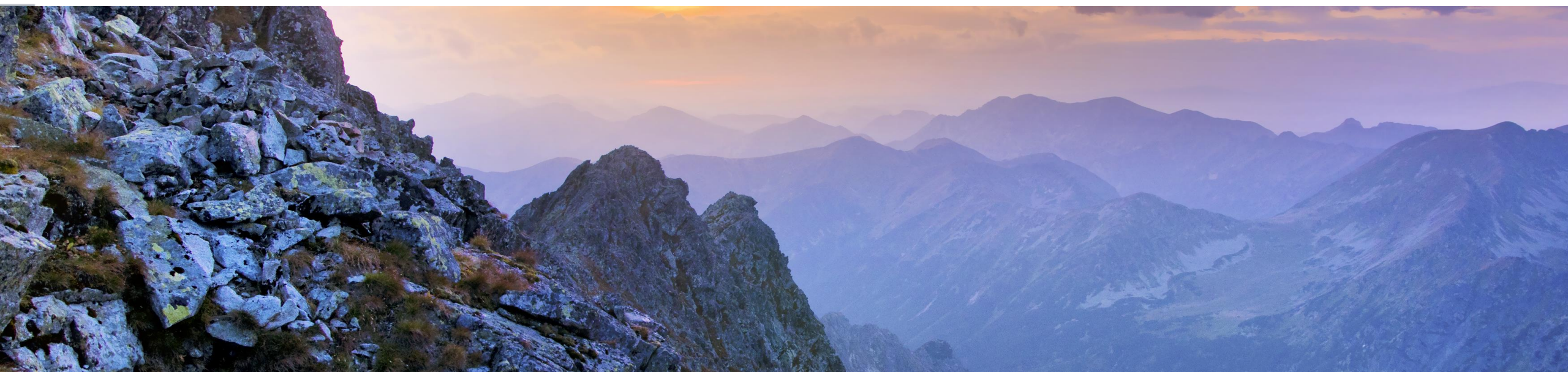
Welcome and overview

⊞ R&D update

Financial results

Upcoming milestones

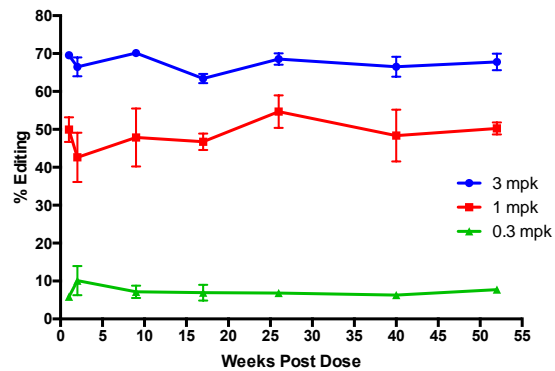
Q&A



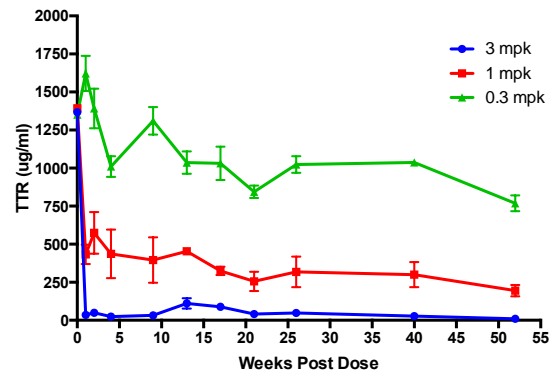
Editing Effect Durability Mouse Study

High Editing Effect Durability *in vivo* After Single Dose; Persistent Effect for Full 12 Months

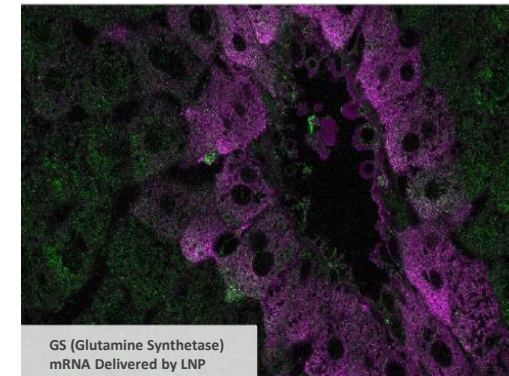
⚡ Liver Editing Durable at 12 months



⚡ Low Serum TTR Levels Persist



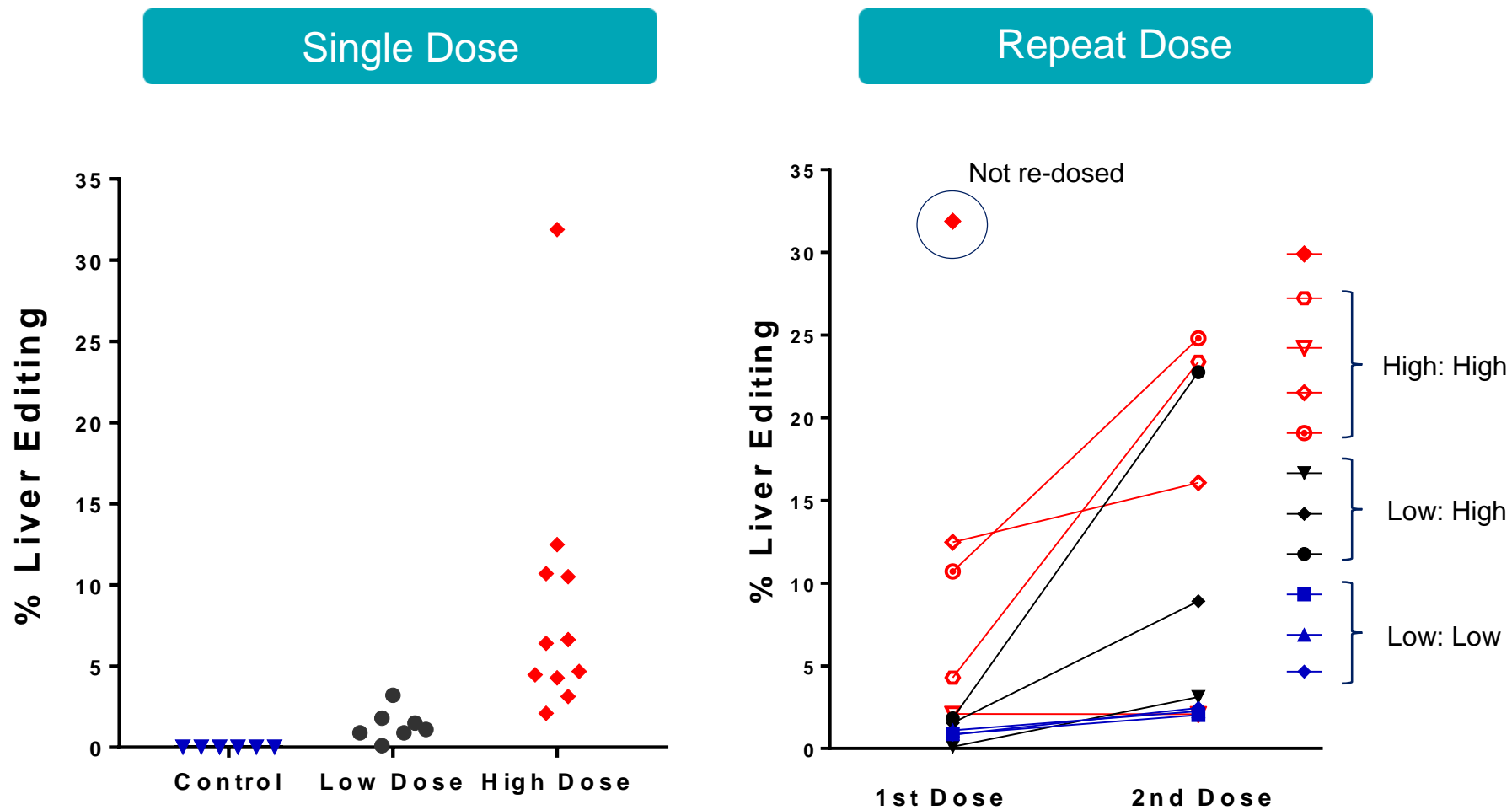
⚡ Edited Liver Cell Population Linked To Durability



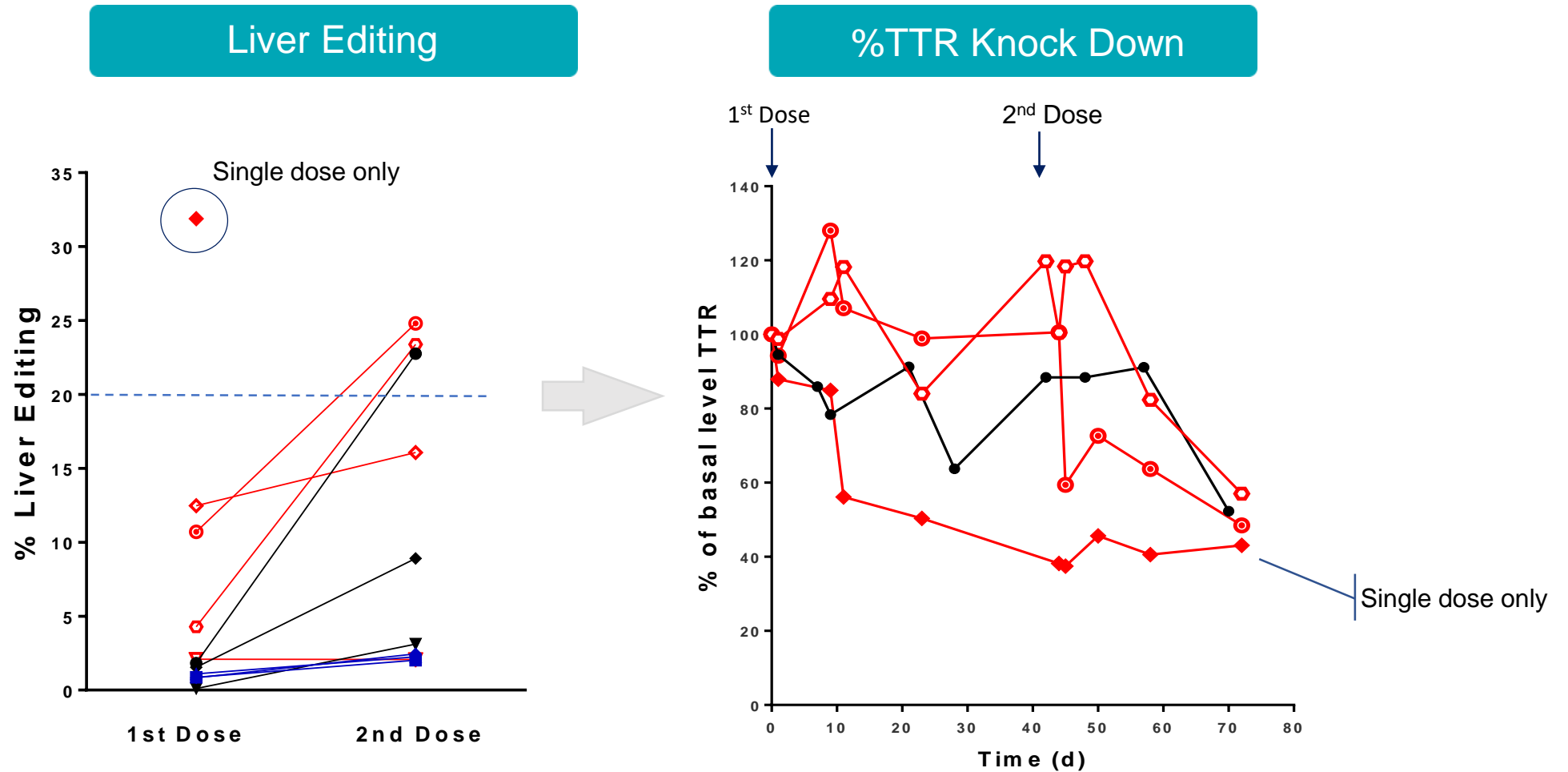
Cas9 expression is transient, with durable editing effect

- Durable and stable liver editing for duration of 12 month study
- ~97% reduction in serum TTR levels, ~70% liver editing
- Stem cell editing likely drives durability

Editing Achieved with Single and Repeat Dose of LNP in NHPs



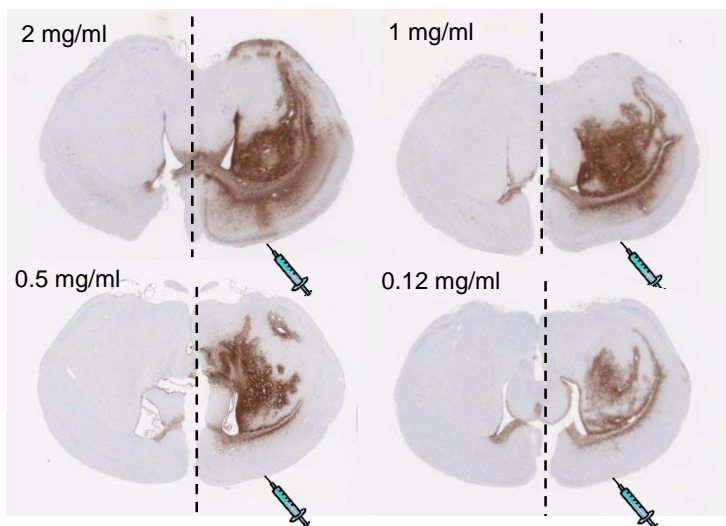
NHP Study - Higher Editing Resulted in Higher Reduction of TTR serum



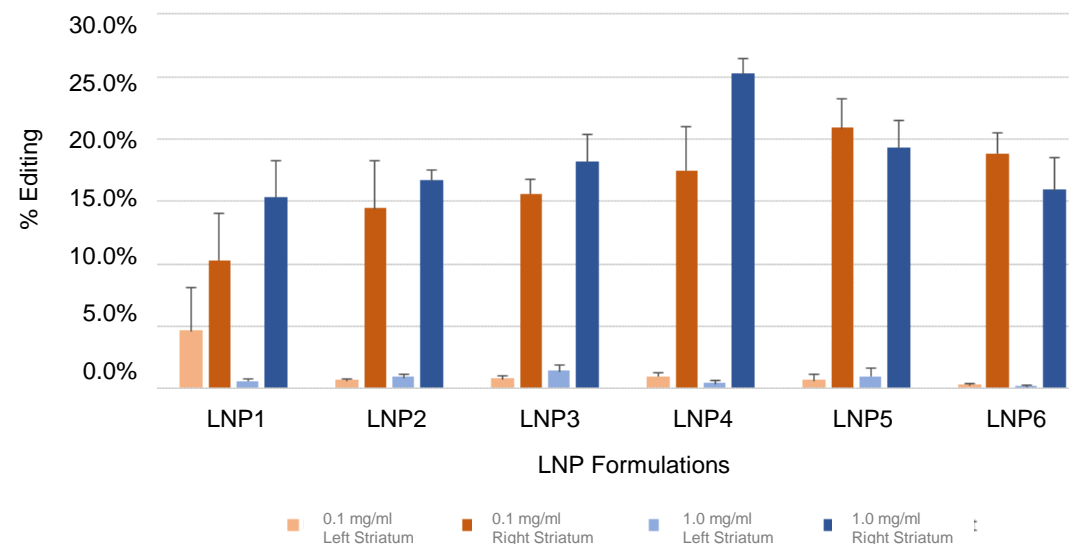
in vivo Potential in CNS

LNP-mediated CNS Delivery and Editing Observed in Mice in Collaboration with Dr. Beverly Davidson

LNP Dose Response Observed in CNS



1-28% Editing in Striatal and Cerebellar Tissue



LNP-mediated CNS delivery led to protein expression and editing in mouse

- Single, local administration was well-tolerated with no behavioral changes
- Uptake is seen in the cerebellum and striatum, and results in genome editing
- Significant opportunities for CNS-specific improvements of LNP formulation and guide selection

Agenda

Welcome and overview

R&D update

⊞ **Financial results**

Upcoming milestones

Q&A



Statement of Operations

	Three Months Ended September 30		Nine Months Ended September 30	
	2017 \$M	2016 \$M	2017 \$M	2016 \$M
Collaboration Revenue	7.317	4.869	19.449	10.852
Operating Expenses				
- Research and development	17.481	7.861	46.477	20.509
- General and administrative	5.711	4.705	17.812	11.680
Total operating expenses	23.192	12.566	64.289	32.189
Operating loss	(15.875)	(7.697)	(44.840)	(21.337)
Interest income	0.519	0.215	1.260	0.266
Net loss	(15.356)	(7.482)	(43.580)	(21.071)
Loss per share	(0.44)	(0.22)	(1.25)	(1.16)
Shares outstanding	35,189	34,316	34,945	18,098

\$'M, except net loss per share
Weighted average shares outstanding, basic and diluted in '000

Balance Sheet

	September 30 2017 \$M	December 31 2016 \$M
Cash and cash equivalents	222.264	273.064
Total assets	249.170	298.969
Total liabilities	73.323	89.132
Total stockholders equity	175.847	209.837

Agenda

Welcome and overview

R&D update

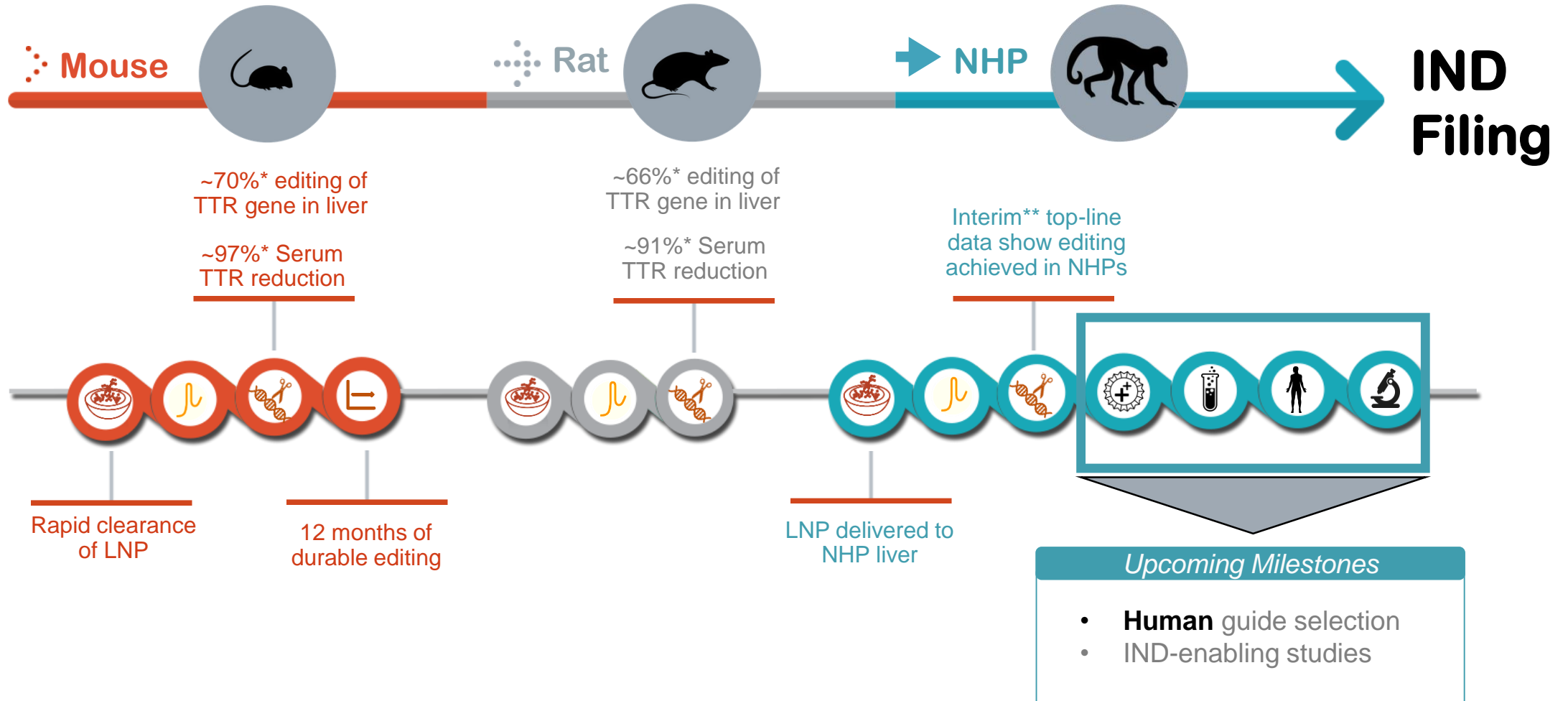
Financial results

⊞ Upcoming milestones

Q&A



Roadmap to IND Filing



Key Accomplishments & Activities

Key Accomplishments

- Successfully delivered CRISPR/Cas9 to non-human primate livers via proprietary LNP delivery system
- Achieved CRISPR/Cas9 editing at the target genome site in the liver of NHPs following a single dose
- Demonstrated that CRISPR/Cas9 editing level translated into TTR serum reduction
- Showed that redosing increased genome editing levels in the liver
- Observed a safe and well-tolerated profile following single and repeat dosing

Upcoming Activities

- Nominate first Intellia development candidate
- Initiate IND-enabling activities
- Demonstrate *in vivo* insertion/repair edit in non-human primates
- Generate preclinical data on T cell candidate for immuno-oncology indication

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

Agenda

Welcome and overview

R&D update

Financial results

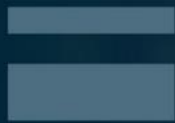
Upcoming milestones

 Q&A



Question & Answer





Intellia
THERAPEUTICS



NASDAQ: NTLA
www.intelliatx.com