Intellia is Leading the Gene Editing Revolution

Corporate Overview
June 2024

MILTON
Living with ATTR amyloidosis with cardiomyopathy
Intellia Therapeutics’ Legal Disclaimer

This presentation contains “forward-looking statements” of Intellia Therapeutics, Inc. ("Intellia", "we" or "our") 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 about Intellia’s beliefs and expectations regarding: our ability to build a world-class gene editing toolbox to develop an unsurpassed gene editing pipeline; the safety, efficacy and advancement of our clinical programs for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis, NTLA-2002 for the treatment of hereditary angioedema ("HAE") and NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease pursuant to our clinical trial applications ("CTA") and investigational new drug ("IND") submissions, including the expected timing of data releases, regulatory filings, and the initiation and completion of clinical trials, including initiating the Phase 3 study for the treatment of ATTR amyloidosis with polynuropathy in 2024, presenting updated data from the ongoing Phase 1 study of NTLA-2001 in 2024, initiating the Phase 3 clinical trial for NTLA-2002 for HAE in 2024, presenting additional data from the Phase 2 portion of the Phase 1/2 study of NTLA-2002 in 2024, and dosing the first patient in the Phase 1 study of NTLA-3001 in 2024; the execution of its strategic priorities for 2024-2026, including the completion of patient enrollment for pivotal studies of NTLA-2001 and NTLA-2002, the planned BLA submission for NTLA-2002 for HAE in 2026, demonstrating human proof-of-concept for targeted in vivo gene insertion, initiating clinical development for its allogeneic ex vivo program, demonstrating preclinical proof-of-concept of editing in tissues outside the liver, and advancing DNA writing technology; the ability to generate data to initiate clinical trials and the timing of CTA and IND submissions; the advancement, expansion and acceleration of our CRISPR/Cas9 technology and related technologies, including DNA writing, base editing, manufacturing and delivery technologies, to advance and develop additional candidates and treatments; our ability to demonstrate our platform's modularity and replicate or apply results achieved in preclinical studies, including those in its NTLA-2001, NTLA-2002 and NTLA-3001 programs, in any future studies, including human clinical trials; our ability to optimize the impact of our collaborations on our development programs, including, but not limited to, collaborations with Regeneron Pharmaceuticals, Inc. (“Regeneron”), including our co-development programs for ATTR amyloidosis and hemophilia A, with AvenCell Therapeutics, Inc. (“AvenCell”) for the development of universal CAR-T cell therapies, with SparingVision SAS (“SparingVision”) for the development of ophthalmic therapies, with ReCode Therapeutics, Inc. (“ReCode”) for the development of novel genomic medicines for the treatment of cystic fibrosis, with Kyverna Therapeutics, Inc. (“Kyverna”) for the development of KYV-201, and with ONK Therapeutics Ltd. (“ONK”) for the development of engineered NK cell therapies; the potential commercial opportunities, including value and market, for our product candidates, including the potential of NTLA-2001, NTLA-2002 and NTLA-3001 to be a single-dose treatment, the potential of NTLA-2001 to halt and reverse disease and result in lifelong, stable TTR reduction, the potential of NTLA-2002 to provide extensive and continuous reduction in kallikrein activity and eliminate significant treatment burden; and the potential of NTLA-3001 to achieve normal human levels of alpha-1 antitrypsin protein and halt progression of lung disease; and our use of capital and other financial results.

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 our ability to protect and maintain our intellectual property position; risks related to valid third party intellectual property; risks related to our relationship with third parties, including our licensors and licensees; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to regulatory agencies’ evaluation of regulatory filings and other information related to our product candidates; uncertainties related to the authorization, initiation and conduct of studies and other development requirements for our product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; risks related to the development and/or commercialization of any of Intellia’s or its collaborators’ product candidates, including that they may not be successfully developed and commercialized; risks related to the results of preclinical or clinical studies, including that they may not be positive or predictive of future results; risks related to the development of novel platform capabilities, including technologies related to editing in tissues outside the liver, base editing and DNA writing; risks related to Intellia’s reliance on collaborations, including that its collaborations with Regeneron, AvenCell, SparingVision, ReCode, Kyverna, ONK or its other collaborations 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 Annual Report on Form 10-K and Quarterly Report on Form 10-Q 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 on its cover page, and Intellia undertakes no duty to update this information unless required by law.
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Intellia is Leading a New Era of Medicine

Turning Nobel-Prize-Winning Science into Medicine

• Poised to bring first-ever *in vivo* CRISPR therapy to market
• Initiated first-ever, pivotal Phase 3 program for an *in vivo* CRISPR therapy
• Plan to initiate two additional *in vivo* Phase 3 studies in 2024

100+ patients dosed with Intellia’s investigational *in vivo* CRISPR-based therapies

Robust pipeline of *in vivo* and *ex vivo* programs

Comprehensive gene editing toolbox
Advancing a Full-Spectrum Genome Editing Company

CRISPR-Based Modular Platform

EMPLOY NOVEL EDITING AND DELIVERY TOOLS

In Vivo
CRISPR is the therapy

Ex Vivo
CRISPR creates the therapy

Genetic diseases

Immuno-oncology
Autoimmune diseases

REWIRE & REDIRECT CELLS

Fix the Target Gene
Intellia is Developing Potentially Curative Gene Editing Treatments to Transform the Lives of Patients

**Full-Spectrum Strategy**
Pipeline of *in vivo* and *ex vivo* CRISPR-based therapies for life-threatening diseases with high unmet need

**Clinically Validated Modular Platform**
Modular technology enables a reproducible path to drug discovery and development

**Deploying Novel Tools**
Continued innovation across editing and delivery modalities for future therapeutic applications
Therapeutic Strategies to Treat Life-Threatening Diseases Have Advanced Over Time

INNOVATION TIMELINE

Small Molecule Drugs  Biologics  RNAi  Gene Therapy  Genome Editing

PROTEINS  RNA  DNA
Gene Editing Starts with CRISPR/Cas9, a Two-Part, Programmable System

**FOUNDATIONAL CRISPR MACHINERY**

1. **Guide RNA (gRNA)**
   - Identifies genetic target

2. **Cas Protein**
   - Responsible for the targeted DNA editing and provides platform for other enzymatic activities

**INSIDE CELL NUCLEUS**

**KEY FEATURES OF CRISPR/CAS9 SYSTEM**

- Selectivity
- High potency
- Address any site
- Target multiple DNA sites
CRISPR/Cas9 and Derivative Gene Editing Technologies Can Be Used to Make Any Type of Edit

**INTELLIA’S EDITING TOOLS**

- **DERIVATIVE TECHNOLOGIES**
  - Base Editor
  - DNA Writer
  - Other Technologies

**KNOCKOUT**
Inactivation/deletion of disease-causing DNA sequence

**INSERT**
Insert new DNA sequence to manufacture therapeutic protein

**REPAIR**
Correction of “misspelled” disease-driving DNA sequence

INTELLIA SELECTS THE BEST TOOL FOR EACH THERAPEUTIC APPLICATION
A Tailored Approach to Maximize the Reach of Gene Editing Across Multiple Tissues

**INTELLIA’S DELIVERY TOOLS**

- **LNP:** Liver-targeted
- **LNP:** Bone marrow-targeted
- **AAV**
- **Other technologies**

**TARGET TISSUES**

- **LNP**s are well-suited for delivery to the liver and blood cells
  - Liver
  - Bone Marrow

- AAV and other technologies are well-suited for delivery to other tissues
  - CNS/PNS
  - Eye
  - Muscle

*This is a representative list of target tissues and does not include all future opportunities.*
CRISPR-Based Editing Technologies are a Promising New Therapeutic Modality

Potential of CRISPR-Based Editing Technologies

- Treat patients at the root cause of their disease
- Single dose treatment with potential lifelong benefit
- Reduce burden to the healthcare system over a patient’s lifetime
CRISPR-Cas9 In Vivo Gene Editing for Transthyretin Amyloidosis

Julian D. Gillmore, M.D., Ph.D., Ed Gane, M.B., Ch.B., Jorg Taubel, M.D., Justin Kao, M.B., Ch.B., Marianna Fontana, M.D., Ph.D., Michael L. Maitland, M.D., Ph.D., Jessica Seitzer, B.S., Daniel O’Connell, Ph.D., Kathryn R. Walsh, Ph.D., Kristy Wood, Ph.D., Jonathan Phillips, Ph.D., Yuanxin Xu, M.D., Ph.D., Adam Amaral, B.A., Adam P. Boyd, Ph.D., Jeffrey E. Cehelsky, M.B.A., Mark D. McKee, M.D., Andrew Schiermeier, Ph.D., Olivier Harari, M.B., B.Chir., Ph.D., Andrew Murphy, Ph.D., Christos A. Kyratsous, Ph.D., Brian Zambrowicz, Ph.D., Randy Soltys, Ph.D., David E. Gutstein, M.D., John Leonard, M.D., Laura Sepp-Lorenzino, Ph.D., and David Lebwohl, M.D.
1. Execute pivotal trials for first two *in vivo* CRISPR-based therapies
   - Complete patient enrollment for pivotal studies of NTLA-2001 and NTLA-2002
   - Planned BLA submission for NTLA-2002 for HAE in 2026

2. Launch next wave of *in vivo* and *ex vivo* clinical programs
   - Demonstrate human proof-of-concept for targeted *in vivo* gene insertion
   - Initiate clinical development for first allogeneic *ex vivo* program

3. Deploy new gene editing and delivery modalities
   - Demonstrate preclinical proof-of-concept of editing in tissues outside the liver
   - Advance DNA writing technology

Intellia’s Strategic Priorities for 2024 – 2026
## Upcoming 2024 Key Clinical Program Milestones

<table>
<thead>
<tr>
<th>Program</th>
<th>Milestones</th>
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<tbody>
<tr>
<td><strong>NTLA-2001</strong> &lt;br&gt;<strong>ATTR</strong></td>
<td>- Dose first patient in pivotal Phase 3 MAGNITUDE trial for ATTR-CM in Q1 2024 &lt;br&gt;- Continue to open new sites and enroll patients &lt;br&gt;- Initiate a pivotal Phase 3 study for ATTRv-PN by year-end &lt;br&gt;- Present updated data from the ongoing Phase 1 study in 2024</td>
</tr>
<tr>
<td><strong>NTLA-2002</strong> &lt;br&gt;<strong>HAE</strong></td>
<td>- Present updated data from the Phase 1 portion in 2024 &lt;br&gt;- Present data from the Phase 2 portion in 2024 &lt;br&gt;- Initiate the Phase 3 study in 2H 2024, subject to regulatory feedback</td>
</tr>
<tr>
<td><strong>NTLA-3001</strong> &lt;br&gt;<strong>AATD</strong></td>
<td>- Dose first patient in Phase 1 study of NTLA-3001 in 2024</td>
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</table>
## Broad Development Pipeline Fueled by Robust Research Engine

<table>
<thead>
<tr>
<th>PROGRAM</th>
<th>APPROACH</th>
<th>Research and Preclinical</th>
<th>Early-Stage Clinical</th>
<th>Late-Stage Clinical</th>
<th>PARTNERS</th>
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<tr>
<td><strong>In Vivo: CRISPR is the therapy</strong></td>
<td></td>
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<tr>
<td>NTLA-2001: Transthyretin Amyloidosis</td>
<td>Knockout</td>
<td></td>
<td></td>
<td></td>
<td>Intellia Therapeutics, Regeneron</td>
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<tr>
<td>NTLA-2002: Hereditary Angioedema</td>
<td>Knockout</td>
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<td></td>
<td></td>
<td>Intellia Therapeutics</td>
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<tr>
<td>NTLA-3001: AATD-Lung Disease</td>
<td>Insertion</td>
<td></td>
<td></td>
<td></td>
<td>Intellia Therapeutics</td>
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<tr>
<td>Hemophilia A / B**</td>
<td>Insertion</td>
<td></td>
<td></td>
<td></td>
<td>Intellia Therapeutics, Regeneron, LEAD</td>
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<tr>
<td>Research Programs</td>
<td>Knockout, insertion or repair</td>
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<td></td>
<td></td>
<td>Intellia Therapeutics</td>
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<tr>
<td>Research Programs</td>
<td>Tissues outside the liver</td>
<td></td>
<td></td>
<td></td>
<td>Intellia Therapeutics, Regeneron, SparkVision</td>
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<tr>
<td><strong>Ex Vivo: CRISPR creates the therapy</strong></td>
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<tr>
<td>Research Programs</td>
<td>Allogeneic and other</td>
<td></td>
<td></td>
<td></td>
<td>Intellia Therapeutics, Vencell, Kyverna, Onk</td>
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</table>

Lead refers to lead development and commercial party
* Intellia is advancing both wholly owned and partnered programs.
** Hemophilia A program is in the research stage; Hemophilia B is being advanced by Regeneron – Intellia is eligible for milestones and royalties.
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2. *In Vivo* Portfolio
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In Vivo

CRISPR is the therapy

GENETIC DISEASES

Strategic Advantages:

Potential curative therapy from a single dose

Systemic non-viral delivery of CRISPR/Cas9 provides transient expression and potential safety advantages

Potential for permanent gene knockout or gain of function by targeted insertion

Capable of delivering to multiple tissue types for various therapeutic applications
Modular Delivery Platform Enables Rapid and Reproducible Path to Clinical Development

LNP Delivery System:

- **gRNA identifies genetic target**
- **TTR gRNA**
- **KLKB1 gRNA**
- **Target-specific gRNA**
- **Cas9 mRNA**
- **AAA**

**Key Advantages of LNP Delivery**

- Clinically-proven delivery to liver
- Large cargo capacity
- Transient expression
- Biodegradable
- Low immunogenicity
- Well-tolerated
- Redosing capability
- Scalable synthetic manufacturing
- Tunable to other tissues
NTLA-2001 for Transthyretin (ATTR) Amyloidosis

About ATTR Amyloidosis
• Caused by accumulation of misfolded TTR protein
• Primarily affects the nerves and/or the heart
• Chronic dosing is required with current treatment options

Our Approach
Knock out TTR gene with a single-dose CRISPR-based treatment
• Reduces wild-type and mutant TTR protein
• Aims to address polyneuropathy and cardiomyopathy

Key Advantages Includes Potential to:
• Halt and reverse disease with deep and consistent TTR reduction
• Be a single-dose treatment
• Expect lifelong, stable TTR reduction
## ATTR Amyloidosis: Large Commercial Opportunity with Significant Unmet Need

### NTLA-2001

**Potential to be the best-in-class TTR reduction agent and only single-dose treatment**

<table>
<thead>
<tr>
<th>Prevalence(^{1,2})</th>
<th>50,000</th>
<th>(~200-500K)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>ATTRv patients worldwide</td>
<td>ATTRwt patients worldwide</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Life Expectancy(^3)</th>
<th>2-7 years</th>
<th>10+ years</th>
</tr>
</thead>
<tbody>
<tr>
<td>after diagnosis for ATTR-CM patients</td>
<td>after diagnosis for ATTRv-PN patients</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Disease Burden(^4)</th>
<th>Patients experience <strong>highly burdensome symptoms</strong>, including heart failure, shortness of breath, muscle weakness and sensory deficits</th>
</tr>
</thead>
</table>

<table>
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<tr>
<th>Commercial Opportunity(^{5,6})</th>
<th>$11B+</th>
<th>$450K+</th>
</tr>
</thead>
<tbody>
<tr>
<td>global market size expected by 2029</td>
<td>average annual cost of TTR reduction treatment in the U.S.</td>
<td></td>
</tr>
</tbody>
</table>

2. Compiled from various sources.
5. GlobalData 2023
6. Redbook 2023
NTLA-2001 Phase 1 Study in ATTR Amyloidosis

Two-part, open-label, multicenter study in adults with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN) or ATTR amyloidosis with cardiomyopathy (ATTR-CM)

Intervention: Single-dose CRISPR/Cas9-based gene editing therapy administered via an intravenous (IV) infusion

**PART I: Single-Ascending Dose Escalation**

- **ATTRv-PN patients (n=36)**
  - 0.1 mg/kg (n=3)
  - 0.3 mg/kg (n=3)
  - 0.7 mg/kg (n=3)
  - 1.0 mg/kg (n=6)

- **ATTR-CM patients (n=36)**
  - 0.7 mg/kg NYHA Class I/II (n=3)
  - 0.7 mg/kg NYHA Class III (n=6)
  - 1.0 mg/kg NYHA Class I/II (n=3)

**PART II: Dose Expansion**

- 55 mg NYHA Class I/II (n=12)
- 80 mg NYHA Class I/II (n=5)
- 55 mg NYHA Class III (n=12)
- 55 mg NYHA Class I/II (n=12)

**PRIMARY OBJECTIVES**

Evaluate safety, tolerability, PK and PD
- Measure serum TTR levels

**SECONDARY OBJECTIVES**

Evaluate efficacy on clinical measures of:
- Neurologic function in subjects with ATTRv-PN
- Cardiac disease in subjects with ATTR-CM

Clinicaltrials.gov ID: NCT04601051
## Most Frequent Treatment-Emergent Adverse Events

### TEAEs by Maximum Toxicity Grade and Preferred Term Reported in >5% of All ATTRv-PN and ATTR-CM Patients (N=65)

<table>
<thead>
<tr>
<th>AE, Preferred Term, n (%)</th>
<th>Any Grade</th>
<th>Grade 1</th>
<th>Grade 2</th>
<th>Grade ≥3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Infusion-related reaction</td>
<td>25 (38)</td>
<td>10 (15)</td>
<td>14 (22)</td>
<td>1 (2)</td>
</tr>
<tr>
<td>Headache</td>
<td>12 (18)</td>
<td>12 (18)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Diarrhea</td>
<td>11 (17)</td>
<td>10 (15)</td>
<td>1 (2)</td>
<td></td>
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<tr>
<td>Back pain</td>
<td>7 (11)</td>
<td>7 (11)</td>
<td></td>
<td></td>
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<tr>
<td>COVID-19 infection</td>
<td>6 (9)</td>
<td>5 (8)</td>
<td>1 (2)</td>
<td></td>
</tr>
<tr>
<td>Cardiac failure</td>
<td>6 (9)</td>
<td>2 (3)</td>
<td>2 (3)</td>
<td>2 (3)</td>
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<tr>
<td>Upper respiratory tract infection</td>
<td>6 (9)</td>
<td>6 (9)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>AST increased</td>
<td>5 (8)</td>
<td>3 (5)</td>
<td>1 (2)</td>
<td>1 (2)</td>
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<tr>
<td>Dizziness</td>
<td>5 (8)</td>
<td>5 (8)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Fatigue</td>
<td>5 (8)</td>
<td>5 (8)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Muscle spasms</td>
<td>5 (8)</td>
<td>4 (6)</td>
<td>1 (2)</td>
<td></td>
</tr>
<tr>
<td>Vision blurred</td>
<td>5 (8)</td>
<td>5 (8)</td>
<td></td>
<td></td>
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<tr>
<td>Atrial flutter</td>
<td>4 (6)</td>
<td></td>
<td>1 (2)</td>
<td>3 (5)</td>
</tr>
<tr>
<td>Constipation</td>
<td>4 (6)</td>
<td>2 (3)</td>
<td>2 (3)</td>
<td></td>
</tr>
<tr>
<td>Rash</td>
<td>4 (6)</td>
<td>4 (6)</td>
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</table>

- Data cutoff May 11, 2023.
- Patients reporting more than one AE related to NTLA-2001 are counted only once using the maximum toxicity grade. AEs coded to preferred term using Medical Dictionary for Regulatory Activities (MedDRA), version 23.0 for PN and version 24.0 for CM. Interim data presented are from the initial 65 of 72 patients dosed. Results from the final 7 patients enrolled after the data cutoff will be reported at a future date.
- This presentation includes data for an investigational product not yet approved by regulatory authorities.
Regardless of Baseline TTR Levels, NTLA-2001 Led to Consistently Low and Sustained Absolute Serum TTR in All Patients

Data cutoff May 11, 2023.

Figure notes: Results for each dose level are shown out to the last time point with complete follow-up for the entire cohort. Interim data presented excludes the 0.1 mg/kg cohort from the dose-escalation of the polyneuropathy arm. The three patients in the 0.1 mg/kg cohort have been re-dosed at 55 mg and results will be shared in a future presentation.

The 55 mg and 80 mg doses are the fixed doses corresponding to 0.7 mg/kg and 1.0 mg/kg, respectively.

This presentation includes data for an investigational product not yet approved by regulatory authorities.
A Phase 3, Randomized, Double-blind, Placebo-controlled Study to Evaluate NTLA-2001 in Patients with ATTR Amyloidosis with Cardiomyopathy (ATTR-CM)

**Key Eligibility Criteria:**
- Adult patients with diagnosis of either hereditary or wild-type ATTR-CM
- NYHA Class I – III
- NT-proBNP baseline ≥ 1000 pg/mL

**Stratification:**
- NAC stage
- TTR genotype: wild-type vs. mutant
- Concomitant tafamidis use vs. no tafamidis

**Primary Endpoint**
- Composite endpoint of CV-related mortality and CV-related events

**Key Secondary Endpoints**
- Serum TTR
- KCCQ-OS score

**Study Duration:**
- Dependent on occurrence of pre-specified number of CV events and a minimum of 18 months follow-up
- Majority of patients are expected to have ≥ 30 months of follow-up for the primary analysis

Clinicaltrials.gov ID: NCT06128629
NTLA-2002 for Hereditary Angioedema (HAE)

About HAE
- Genetic disease characterized by recurring, severe and unpredictable swelling in various parts of the body
- Despite availability of existing therapies, significant unmet need persists
- Chronic dosing is required with current treatment options

Our Approach
Knock out *KLKB1* gene with a single-dose CRISPR-based treatment
- Reduce kallikrein activity to prevent attacks

Key Advantages Includes Potential to:
- Be a single-dose treatment
- Provide extensive and continuous reduction in kallikrein activity
  - Intended to minimize the risk of breakthrough attacks
- Eliminate significant treatment burden
# HAE: Large Commercial Opportunity with Significant Unmet Need

**Prevalence**

| HAE patients worldwide | 150,000+ |

**Diagnosis**

| Symptom onset typically occurs by 12 years old | 20 years old |

| average age of diagnosis |

**Disease Burden**

| 50-60% |

| patients continue to have HAE attacks despite existing therapies |

| • Attacks can result in hospitalizations |

| • Patients subject to lifetime of attack risk and chronic treatment |

**Commercial Opportunity**

| $6B+ |

| global market size expected by 2029 |

| $500K+ |

| annual U.S. cost of leading prophylactic treatment |

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2. Farkas et al. Allergy. 2017. 72;300-313


4. GlobalData 2023

5. Redbook 2023
NTLA-2002 Phase 1/2 Trial Design

International, multicenter study to assess safety, tolerability, PK, PD and effect of NTLA-2002 on attacks in adults with Type I or Type II HAE

Total Enrollment:
Up to 55 patients, age 18 and older

Intervention:
Single dose administered via an intravenous (IV) infusion

PHASE 1
Open-Label, Single-Ascending Dose
- 75 mg (n=3)
- 50 mg (n=4)
- 25 mg (n=3)

PHASE 2
Expansion study to confirm recommended dose
- 50 mg (n=10)
- 25 mg (n=10)
- Placebo arm (n=5)

KEY ENDPOINTS
- Evaluate safety and tolerability
- Change in plasma kallikrein protein and activity levels
- Change in attack rates (Phase 2)
**NTLA-2002 Continues to Be Well-Tolerated Across All Dose Levels**

<table>
<thead>
<tr>
<th>TEAEs Occurring in ≥2 Patients</th>
<th>25 mg (n=3)</th>
<th>50 mg (n=4)</th>
<th>75 mg (n=3)</th>
<th>All Patients (N=10)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Any TEAE</td>
<td>3</td>
<td>4</td>
<td>3</td>
<td>10</td>
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<tr>
<td>Infusion-related reaction</td>
<td>2</td>
<td>2</td>
<td>3</td>
<td>7</td>
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<tr>
<td>COVID-19</td>
<td>3</td>
<td>1</td>
<td>2</td>
<td>6</td>
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<td>Fatigue</td>
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<td>Upper respiratory tract infection</td>
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<td>Myalgia</td>
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<td>3</td>
<td>3</td>
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<td>Oropharyngeal pain</td>
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<td>1</td>
<td>3</td>
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<td>Abdominal discomfort</td>
<td>0</td>
<td>2</td>
<td>0</td>
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<tr>
<td>Headache</td>
<td>0</td>
<td>0</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>Viral upper respiratory tract infection</td>
<td>0</td>
<td>0</td>
<td>2</td>
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With a median follow-up time of 20.1 months:

- No treatment-emergent AEs ≥ Grade 3
- No treatment-emergent SAEs
- No AESIs other than IRRs
- No liver enzyme elevations or platelet count decreases > Grade 1
- No clinically significant shifts in coagulation parameters

Data cutoff date: 12Feb2024

This presentation includes data for an investigational product not yet approved by regulatory authorities.
A Single Dose of NTLA-2002 Continues to Show Dose-Dependent and Durable Reductions in Plasma Kallikrein Protein Over Time

Data cutoff date: 12Feb2024.
Baseline value is the average of 2 samples on separate days during the screening period and 1 predose on study Day 1. Only visits completed by all patients within a cohort are presented.

Asterisks indicate the start of additional ongoing follow-up since the previous datacut of 17Feb2023.
This presentation includes data for an investigational product not yet approved by regulatory authorities.
A Single Dose of NTLA-2002 Led to a 98% Reduction in HAE Attack Rate, With 8 of 10 Patients Attack-Free in Post-Primary Observation Period

Data cutoff date: 12Feb2024.
A month is defined as 28 days.
This presentation includes data for an investigational product not yet approved by regulatory authorities.
NTLA-3001 for Alpha-1 Antitrypsin Deficiency (AATD)-Associated Lung Disease

About AATD
• Genetic disorder leading to progressive lung and/or liver disease
• >60K AATD patients in the U.S.
• ~250K AATD patients globally

Our Approach
Targeted insertion of a functional *SERPINA1* gene into the albumin locus
• Continuous expression of functional AAT protein at normal levels

Key Advantages
• Designed to be a single-dose treatment
• Aims to achieve normal human levels of AAT protein and halt progression of lung disease

---

1 Remih et al. Curr Opin Pharmacol 2021; 59:149-156
2 Brantly M. Clin Chem. 2006; 52:2180-2181
* In severe AATD patients defined as individuals with PiZZ genotype.
Durable Production of Physiologic Levels of hAAT Through One Year in NHP

Insertion Platform Enables Targeted, Stable Gene Insertion in the Albumin Locus

Precisely Create Insertion Site

Deliver Insertion Template

Gene of Interest

(Factor 9) (SERPINA1)

LNP

AAV

Human AAT (hAAT) Expression

Circulating hAAT (µg/mL)

Time (weeks post insertion)

0 4 8 12 16 20 24 28 32 36 40 44 48 52

0 500 1000 1500 2000

Insertion (n=3)

Buffer control (n=3)

Normal range

Therapeutically relevant

1 Stoller & Aboussouan. The Lancet, 2005
Normal range: ~1000-2700 µg/mL, or 20-53 µM; Therapeutically relevant: 571 µg/mL, or 11 µM

This presentation includes data for an investigational product not yet approved by regulatory authorities.
Significant Opportunities to Unlock Full Potential of *In Vivo* Platform

### CRITERIA USED TO SELECT POTENTIAL FUTURE CANDIDATES:

- Unmet need
- Population size
- Technical feasibility

### Potential Liver Development Programs

**RARE DISEASES**
- Blood disorders
- Lysosomal storage diseases
- Metabolic diseases

**PREVALENT DISEASES**
- Chronic viral diseases
- Dyslipidemia
- Hypertension
- NASH

### Unlocking Full Potential of Genome Editing

**TARGET TISSUES**
- Bone marrow
- CNS/PNS
- Eye
- Heart
- Muscle
- Lung

**Expansion into tissue-specific diseases**

---

* This is a selection of potential liver targets and does not represent all future opportunities.
** Individual targets could be developed by Intellia, Regeneron or through collaborations.
*** In collaboration with SparingVision
**** In collaboration with ReCode
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2. *In Vivo* Portfolio

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**Ex Vivo**

**CRISPR creates the therapy**

**Strategic Advantages:**

Utilizing proprietary CRISPR engineering platform to create differentiated cell therapies for IO and AI diseases

Targeting modalities, such as TCR, with broad potential in multiple indications

Focused on reproducing natural cell physiology for potential improvements to safety and efficacy in immuno-oncology
Proprietary Engineering Platform to Power Next-Generation Engineered Cell Therapies

LNP-BASED CELL ENGINEERING PLATFORM

Highly efficient sequential editing | Optimal cell performance | Scalable manufacturing process

ENABLES VERSATILE SOLUTIONS BY “MIXING AND MATCHING,” INCLUDING:

Cell Type
- HSCs, T cells
- NK cells, Macrophages

Targeting Modality
- TCRs
- CAR-Ts, Universal CARs

Rewiring Instructions
- Immune-enhancing edits
- Novel targets
### Differentiated Approach to Cell Therapy Genome Engineering

<table>
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<th>Gene Editing Approach</th>
<th>Delivery</th>
<th>Editing Mode</th>
<th>Knockout (KO)</th>
<th>Insertion</th>
<th>Key Questions From Preclinical Data</th>
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<td>LNP</td>
<td>Sequential</td>
<td>Cleavase or Base Editor</td>
<td>CRISPR insertion</td>
<td>Minimize random DSB?</td>
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<td>Other Approaches</td>
<td>Electroporation</td>
<td>Electroporation</td>
<td>Simultaneous</td>
<td>Base Editor</td>
<td>Lenti/Retroviruses</td>
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</table>

**Key Questions From Preclinical Data**

- Minimize random DSB?
- Minimize random insertion?
- Minimize genotoxicity risk?

---

LNP-based, sequential process + Precise CRISPR KOs & insertion(s) = Quality cell product

Slide based on preclinical data disclosed by Intellia. Intellia’s cell product to be further explored in additional preclinical and clinical studies.
Sequential Editing with LNP Approach Minimizes Translocations While Retaining Robust Cell Viability and Expansion

ddPCR assay to detect TRAC-TRBC translocations

LNP Approach: Cell Expansion at D10

% TRAC-TRBC translocated cells

No Edit  Electroporation  Simultaneous LNP  Sequential LNP

Fold Expansion

No Edit  Simultaneous  Sequential
Three Immune Concerns Must Be Addressed by Allogeneic Cell Therapies

1. **Graft-versus-host disease (GvHD)**
   - T cell receptor (TCR) from allogeneic T cells recognizes and kills recipient (host) cells.
   - Largely solved with knockout (KO) of endogenous TCR

2. **Rejection via host T cells**
   - Human leukocyte antigen (HLA) molecules must match between donor and recipient to prevent rejection from:
     - Host CD8 (HLA class I) T cells
     - Host CD4 (HLA class II) T cells

3. **Rejection via host natural killer (NK) cells**
   - NK cells will attack cells that lack HLA-I expression or have low HLA-I.
   - No validated solution yet
Intellia’s Differentiated Allogeneic Approach Aims to Address All Three Immune Concerns

Key Potential Advantages
✓ Approach is applicable to CAR and TCR
✓ Solves for host NK and T cell rejection
✓ Avoids long-term immunosuppression

Intellia’s Editing Strategy
1a. Knockout endogenous TCR
1b. Insert target CAR or TCR
2a. Knockout HLA Class II
2b. Knockout HLA-A only
3. Partial HLA Class I match

Main Objective of Edit
 Prevent Graft-versus-Host Disease (GvHD)
 Direct T cell for tumor killing
 Prevent CD4-mediated rejection
 Prevent CD8-mediated rejection
 Block NK cell activation and avoid NK-mediated rejection
Realizing the Promise of Gene Editing

At Intellia, we innovate every day to make CRISPR-based medicines a reality for patients.

This is just the beginning of the gene editing revolution.
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<td><em>Ex Vivo</em> Portfolio</td>
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<td>Persistence of <em>In Vivo</em> Edits</td>
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<td>3</td>
<td><em>In Vivo</em> Editing of Hematopoietic Stem Cells</td>
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<td>4</td>
<td>Intellia’s Allogeneic Solution</td>
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<td>5</td>
<td>Platform: Identifying Potent and Highly Specific Guide RNAs</td>
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<td>6</td>
<td>Strategic Collaborations</td>
</tr>
<tr>
<td>7</td>
<td>Abbreviations</td>
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</table>
Re-dosing with Intellia’s LNP Delivery Platform

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Clinical Proof-of-Concept that Redosing with Intellia’s LNP-Delivered Gene Editing Technology Led to a Targeted Additive Pharmacodynamic Effect

Data cutoff April 12, 2024.
This presentation includes data for an investigational product not yet approved by regulatory authorities.
Follow-on Dosing Was Well-Tolerated and Did Not Lead to Any Safety Findings

<table>
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<th>Preferred Terma, n (%)</th>
<th>0.1 mg/kg + 55 mg (n=3)</th>
<th>Maximum CTCAE Toxicity Grade</th>
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<td>Any TEAE</td>
<td>2 (67%)</td>
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<td>COVID-19</td>
<td>1 (33%)</td>
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<tr>
<td>Fatigue</td>
<td>1 (33%)</td>
<td>1</td>
</tr>
<tr>
<td>Hand fracture</td>
<td>1 (33%)</td>
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<tr>
<td>Headache</td>
<td>1 (33%)</td>
<td>1</td>
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<tr>
<td>Infusion-related reaction</td>
<td>1 (33%)</td>
<td>1</td>
</tr>
<tr>
<td>Nausea</td>
<td>1 (33%)</td>
<td>1</td>
</tr>
<tr>
<td>Vulvovaginal candidiasis</td>
<td>1 (33%)</td>
<td>2</td>
</tr>
</tbody>
</table>

- 8 – 12 months of follow-up for patients who received a follow-on dose
- No clinically significant changes in liver enzymes, platelets or coagulation parameters

Data cutoff April 12, 2024. Note: For each preferred term, patients reporting more than one adverse event are counted only once. A Grade 1 AE of “Hereditary neuropathic amyloidosis” was reported for one patient at the time of the data cutoff and confirmed to be entered in error. Adverse events are coded to preferred term using MedDRA, version 26.0. This presentation includes data for an investigational product not yet approved by regulatory authorities.
Persistence of *In Vivo* Edits

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Protein Reduction Remains Unchanged Following PHx Murine Model of Liver Regeneration

* Similar results obtained for control and LNP when sham surgery was performed. Next generation sequencing (NGS) analysis to evaluate the frequency of insertion and deletion events (edits)
Gene Insertion Provides a Durability Advantage Over Conventional AAV Episomes in a PHx Murine Model of Rapid Liver Growth
In Vivo Editing of Hematopoietic Stem Cells

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Editing HSCs *In Vivo* Requires LNPs with Bone Marrow Tropism

LNPs designed, formulated and tested *in vivo* to identify compositions with enhanced delivery to bone marrow and HSCs.

- **Liver-tropic LNP**
  - Infusion of LNP
  - Infusion of LNP

- **Bone marrow-tropic LNP**
  - Infusion of LNP
  - Infusion of LNP
Editing of Mouse Bone Marrow and HSCs is Durable Through At Least One Year

- Editing was similar across all time points assessed, in both whole bone marrow and HSCs populations
- Results highlight the potential for a single-course, long-lasting therapy

Editing Over One Year Period

![Bar chart showing editing over one year period](chart.png)

- **Buffer**
- **Whole bone marrow**
- **Hematopoietic stem cells**

* Lin-Sca-1+c-Kit+CD34-Flk2- cell population
Editing of Mouse Bone Marrow and HSCs Increases with Multidosing

- Non-immunogenic LNP delivery platform may enable stepwise “treat-to-target” approach

---

* Blood. 2017;130(17):1946-1948

** Lin-Sca-1+c-KIT+CD34-Flk2+ cell population

---

Level predicted to be curative for SCD*
Intellia’s Allogeneic Solution
# Immune Concerns Unaddressed by Current Allogeneic Solutions

## Intellia’s Approach

Intellia’s approach employs an intense lymphodepletion regimen followed by a knockout (KO) of HLA-I (B2M), KO HLA-I & express NK inhibitor (HLA-E),KO HLA-II & partial HLA Class I match to avoid rejection of cell therapy by host CD8 T cells, host CD4 T cells, host NK cells, and to avoid profound immunosuppression.

<table>
<thead>
<tr>
<th>Approach</th>
<th>Employ intense lymphodepletion regimen</th>
<th>Knockout (KO) HLA-I (B2M)</th>
<th>KO HLA-I &amp; express NK inhibitor (HLA-E)</th>
<th>Intellia’s Approach</th>
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<tr>
<td>Avoid rejection of cell therapy by host CD8 T cells</td>
<td>✔</td>
<td>✔</td>
<td>✔</td>
<td>✔</td>
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<tr>
<td>Avoid rejection of cell therapy by host CD4 T cells</td>
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<td>✔</td>
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<tr>
<td>Avoid rejection of cell therapy by host NK cells</td>
<td>✔</td>
<td>✗</td>
<td>✗</td>
<td>✔</td>
</tr>
<tr>
<td>Avoid profound immunosuppression</td>
<td>✗</td>
<td>✔</td>
<td>✔</td>
<td>✔</td>
</tr>
</tbody>
</table>

Slide based on preclinical data disclosed by Intellia. Intellia’s cell product to be further explored in additional preclinical and clinical studies.
Allo TCR-T Cells Resist NK Cell Killing for at Least 90 Days *In Vivo*

**B2M Knockout T cells**

>90% B2M KO T cells killed by NKs within 24h

**Allo TCR-T Cells**

Minimal Allo T cell rejection in the presence of NK cells

Days 0-90

IVIS imaging to detect Luciferase+ T cell signal

Mice engrafted with human NK cells or mock treated

Luciferase+ T cells

Signal from Live B2M KO T Cells

Signal from Live Allo TCR-T Cells

Mock mice

NK engrafted mice

Mock mice

NK engrafted mice

Days Post T Cell Injection

Days Post T Cell Injection
Platform: Identifying Potent and Highly Specific Guide RNAs

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Comprehensive gRNA Specificity Assessment: An Off-Target Workflow

1: Discovery of Potential Off-Target Edits

- Computational prediction
- SITE-Seq Genomic DNA digest
- GUIDE-seq Cell-based DNA repair

Aggregate ALL potential off-target genomic loci

2: Cell-based Validation of True Off-Target Edits by Deep Sequencing

- Multiplex panel for NGS
- Targeted Amp-Seq NGS follow-up

**Ex Vivo**
- Cell drug product

**In Vivo**
- Edit cells *in vitro* using drug product formulation
- Therapeutically relevant human primary cell type(s) (multiple donors)
- Dose range to exceed projected therapeutic exposure (≥10X)
Limited Overlap in Discovered Off-Target Loci by Three Leading Methods

1: Discovery of Potential Off-Target Edits

- Computational prediction
- SITE-Seq Genomic DNA digest
- GUIDE-seq Cell-based DNA repair

Aggregate ALL potential off-target genomic loci

658 non-overlapping potential off-target loci

Data above is a representative example
Off-Target Workflow In Practice: Representative Example

1: Discovery of Potential Off-Target Edits

658 non-overlapping potential off-target loci

SITE-Seq

GUIDE-Seq

Cas-OFFinder

431

178

37

14

2: Validation of Off-Target Edits in Cells

Multiplex panel for NGS

Targeted Amp-Seq NGS follow-up

In Vivo Programs

- Dose responses using drug product formulation
- Therapeutically relevant human primary cell type(s) (2 donors)
- Dose range to exceed projected therapeutic exposure (≥10X)
- Validation: off-target indels detected in edited cells

Data above is a representative example
Validation of Off-Target Editing in Primary Human Hepatocytes at Supersaturating LNP CRISPR Concentrations to Maximize Sensitivity

- **658 potential off-target loci**
- **7 validated off-target (OT) loci**
  - 2 in introns and 5 in intergenic regions

- SITE-Seq discovered **100%**
- GUIDE-Seq and Cas-OFFinder discovered the same 3 out of 7 validated off-target loci **43%**
- Eliminate gRNA with validated off-target indels in regions of the genome associated with cancer

Data above is a representative example
In Vitro: No Detectable Off-Target Editing with Pharmacologic Concentration of sgRNA
Strategic Collaborations

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Growing Intellia’s Impact on Patients Through Strategic Collaborations

Genetic diseases

Ophthalmology

Cystic Fibrosis

In Vivo

Ex Vivo

IO (Universal CAR-T)

Autoimmune diseases (CD19 CAR-T)

IO (NK Cells)
Collaborations Helping to Accelerate the Development of CRISPR-Based Therapies

**REGENERON**

Collaboration Overview:

- **Up to 15 in vivo targets** with a mix of co-developed and licensed programs
  - Liver-centric product development

- **ATTR (in vivo knockout):** Intellia is lead party; Regeneron will share 25% of costs and profits

- **Hemophilia A (in vivo insertion):** Regeneron is lead party; Regeneron will share 65% of costs and profits

- **In vivo targets exclusively developed by Regeneron:**
  - Up to $320M in milestones per target
  - High single to low double-digit royalties

- **Non-exclusive license to certain platform IP** for up to 10 ex vivo CRISPR products in defined cell types

- **New research collaboration as of September 2023**
  to develop treatments for neurological and muscular diseases
Abbreviations
# Abbreviations

<table>
<thead>
<tr>
<th>Abbreviation</th>
<th>Full Form</th>
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<tr>
<td>AAT</td>
<td>alpha-1 antitrypsin</td>
</tr>
<tr>
<td>AATD</td>
<td>alpha-antitrypsin deficiency</td>
</tr>
<tr>
<td>AAV</td>
<td>adeno-associated virus</td>
</tr>
<tr>
<td>AE</td>
<td>adverse event</td>
</tr>
<tr>
<td>AESI</td>
<td>adverse event of special interest</td>
</tr>
<tr>
<td>AI</td>
<td>autoimmune disease</td>
</tr>
<tr>
<td>AST</td>
<td>aspartate transaminase</td>
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<td>ATTR amyloidosis</td>
<td>transthyretin amyloidosis</td>
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<td>ATTR-CM</td>
<td>ATTR amyloidosis with cardiomyopathy</td>
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<td>ATTRv-PN</td>
<td>hereditary ATTR amyloidosis with polyneuropathy</td>
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<td>B2M</td>
<td>beta-2-microglobulin</td>
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<td>BLA</td>
<td>biologics license application</td>
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<td>CAR-T</td>
<td>chimeric antigen receptor T cells</td>
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<td>CNS</td>
<td>central nervous system</td>
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<td>CTCAE</td>
<td>Common Terminology Criteria for Adverse Events</td>
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<td>CV</td>
<td>cardiovascular</td>
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<tr>
<td>ddPCR</td>
<td>digital droplet polymerase chain reaction</td>
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<tr>
<td>DSB</td>
<td>double strand break</td>
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<td>GvHD</td>
<td>graft-versus-host disease</td>
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<td>EC90</td>
<td>concentration inducing 90% of maximal effect</td>
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<td>FOD</td>
<td>follow-on dose</td>
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<td>Gr</td>
<td>grade</td>
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<td>guide RNA</td>
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<td>hemophilia A/B</td>
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<td>human leukocyte antigen class E</td>
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<td>immuno-oncology</td>
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<td>infusion-related reaction</td>
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<td>Kansas City Cardiomyopathy Questionnaire-Overall Summary</td>
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<td>kallikrein B1</td>
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<td>lipid nanoparticle</td>
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<td>MedDRA</td>
<td>Medical Dictionary for Regulatory Activities</td>
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<td>messenger RNA</td>
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<td>National Amyloidosis Centre</td>
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<td>nonalcoholic steatohepatitis</td>
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<td>non-human primate</td>
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<tr>
<td>NK</td>
<td>natural killer</td>
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<td>NT-proBNP</td>
<td>N-terminal-pro-B-type natriuretic peptide</td>
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<td>NYHA</td>
<td>New York Heart Association</td>
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<td>PD</td>
<td>pharmacodynamics</td>
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<td>PHx</td>
<td>partial hepatectomy</td>
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<td>PK</td>
<td>pharmacokinetics</td>
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<td>PNS</td>
<td>peripheral nervous system</td>
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<td>Pt</td>
<td>patient</td>
</tr>
<tr>
<td>SAE</td>
<td>serious adverse event</td>
</tr>
<tr>
<td>SE</td>
<td>serious event</td>
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<td>sickle cell disease</td>
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<td>standard deviation</td>
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