

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): April 27, 2026**

**INTELLIA THERAPEUTICS, INC.**  
(Exact name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-37766**  
(Commission  
File Number)

**36-4785571**  
(IRS Employer  
Identification No.)

**40 Erie Street, Suite 130**  
**Cambridge, Massachusetts**  
(Address of Principal Executive Offices)

**02139**  
(Zip Code)

**Registrant's Telephone Number, Including Area Code: (857) 285-6200**

**Not Applicable**  
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock (Par Value \$0.0001)	NTLA	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

## Item 7.01 Regulation FD Disclosure.

On April 27, 2026, Intellia Therapeutics, Inc. (the “Company”) issued a press release titled “Intellia Therapeutics Reports Positive Phase 3 Results in Hereditary Angioedema, Marking a Global First for *In Vivo* Gene Editing” and presented topline data from the HAELO Phase 3 trial of lonvoguran ziclumeran (“lonvo-z”) for the treatment of hereditary angioedema (“HAE”) in a presentation titled “Phase 3 HAELO Clinical Trial: Topline Data for Lonvo-z.” A copy of the press release and presentation are furnished as Exhibits 99.1 and 99.2, respectively, to this Current Report on Form 8-K and are incorporated herein by reference.

*The information under this Item 7.01, including Exhibits 99.1 and 99.2 hereto, are being furnished herewith and shall not be deemed “filed” for the purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall such information be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.*

## Item 8.01. Other Events.

### *Updates about Lonvo-z for the Treatment of HAE*

On April 27, 2026, Intellia announced positive topline results from the global Phase 3 HAELO clinical trial of lonvo-z in HAE. Lonvo-z is an *in vivo* CRISPR gene editing candidate that is designed as a one-time treatment, administered in an outpatient setting, to inactivate the *kallikrein B1* (“*KLKB1*”) gene to permanently lower kallikrein and bradykinin levels.

HAELO is a randomized, double-blind, placebo-controlled Phase 3 trial designed to evaluate the efficacy and safety of a one-time 50 milligram dose of lonvo-z in adults and adolescents aged 16 years and older with Type I or Type II HAE. Key endpoints of the trial focused on the number of HAE attacks experienced by patients, quality of life, safety and tolerability.

A total of 80 patients were enrolled, with 52 receiving lonvo-z and 28 receiving placebo. Of the total population, 49% of patients were enrolled in the United States and 71% were on long-term prophylaxis (“LTP”) therapy at study entry. Patients on LTP therapy were required to discontinue those therapies in the weeks prior to dosing. After week 28, patients had the option to participate in a blinded crossover to receive lonvo-z, if they previously received placebo, or placebo, if they previously received lonvo-z. As of the data cutoff (February 10, 2026), the median follow-up for enrolled patients is 7.5 months. As of the data cutoff, all patients who received lonvo-z at baseline or in crossover after week 28 remained LTP free.

Key findings from HAELO include:

### Efficacy of Lonvo-z

- The trial met its primary endpoint. For the six-month efficacy evaluation period (weeks 5 to 28), a one-time infusion of lonvo-z reduced attacks by 87% versus placebo, with a mean monthly attack rate of 0.26 in the lonvo-z arm compared with 2.10 in the placebo arm ( $p < 0.0001$ ).
- The trial met all of its key secondary endpoints with statistical significance ( $p < 0.0001$ ). These included a 62% rate of patients who were entirely attack free and therapy free in the lonvo-z arm for the six-month efficacy evaluation period, compared with 11% of patients in the placebo arm.
- All patients in the lonvo-z arm experienced a reduction in attack rate from baseline. For the 38% of patients in the lonvo-z arm who were not attack free during the primary observation period, a mean attack rate reduction of 72% was observed.

### Safety of Lonvo-z

- Favorable safety and tolerability data were observed for lonvo-z. The most common treatment emergent adverse events (“TEAEs”) during the primary observation period (infusion through week 28) were infusion-related reactions (“IRRs”), headache and fatigue.
- All TEAEs reported as of the data cutoff were mild or moderate (Grade 1 or Grade 2) and there were no serious adverse events observed in the lonvo-z arm.
- All reported IRRs were mild or moderate and were transient.
- There was no meaningful difference between the lonvo-z and placebo arms in clinical chemistries. A single Grade 2 ALT elevation was observed in the lonvo-z arm that self-resolved in one week.
- As of the data cutoff, the safety and tolerability data observed in patients participating in the crossover following week 28 was consistent with the safety and tolerability observed during the primary observation period.

Also on April 27, 2026, Intellia announced that it initiated a rolling biologics license application (“BLA”) submission to the U.S. Food and Drug Administration to seek regulatory approval of lonvo-z for the treatment of HAE. Intellia is preparing to complete the BLA filing in the second half of 2026 and for a potential U.S. launch of lonvo-z in the first half of 2027, if approved.

### **Forward-Looking Statements**

This Current Report on Form 8-K and certain of the materials furnished or filed herewith contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, but are not limited to, express or implied statements regarding Intellia’s beliefs and expectations regarding: the success and advancement of its program for lonvoguran ziclumeran or “lonvo-z” (formerly known as NTLA-2002) for the treatment of hereditary angioedema (“HAE”), including its plan to complete the submission of a biologics license application (“BLA”) for lonvo-z in the second half of 2026, its expectations regarding review and approval of that BLA, and its expectations regarding a potential U.S. launch of lonvo-z in the first half of 2027; and the potential of one dose of lonvo-z to offer prolonged freedom from both attacks and the need for ongoing therapy.

Any forward-looking statements in this current report on Form 8-K 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: uncertainties related to the conduct of clinical studies and other development and commercialization requirements for its product candidates, including lonvo-z, including risks related to the ability to develop and successfully commercialize lonvo-z or any of Intellia’s product candidates; risks related to Intellia’s ability to protect and maintain its intellectual property position; risks related to Intellia’s relationship with third parties, including its contract manufacturers, collaborators, licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical study results will not be positive; and risks related to the potential delay of planned clinical trials or regulatory filings due to regulatory feedback or other developments. 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 current report on Form 8-K is as of the date of the report, and Intellia undertakes no duty to update this information unless required by law.

## **Item 9.01 Financial Statements and Exhibits.**

### (d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press Release dated April 27, 2026 titled “Intellia Therapeutics Reports Positive Phase 3 Results in Hereditary Angioedema, Marking a Global First for <i>In Vivo</i> Gene Editing”</a>
99.2	<a href="#">Presentation dated April 2026 titled “Phase 3 HAELO Clinical Trial: Topline Data for Lonvo-z”</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Intellia Therapeutics, Inc.

Date: April 27, 2026

By: /s/ John M. Leonard

Name: John M. Leonard

Title: Chief Executive Officer and President



\*\*\* CONFIDENTIAL \*\*\*

**Intellia Therapeutics Reports Positive Phase 3 Results in Hereditary Angioedema, Marking a Global First for *In Vivo* Gene Editing**

- *Phase 3 HAELO trial of lonvoguran ziclumeran (lonvo-z) met primary and all key secondary endpoints; favorable safety and tolerability data observed*
- *Single dose of lonvo-z freed most patients from both attacks and ongoing therapy for six-month efficacy evaluation period, demonstrating its potential to be the first and only one-time HAE treatment*
- *Rolling biologics license application (BLA) submission initiated with the U.S. Food and Drug Administration (FDA); anticipate U.S. launch in the first half of 2027, if approved*
- *Intellia to host webcast today at 8:00 a.m. ET*

CAMBRIDGE, Mass., April 27, 2026 – [Intellia Therapeutics, Inc.](#) (Nasdaq: NTLA), a leading biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies, today announced positive topline results from the global Phase 3 HAELO clinical trial of lonvo-z (formerly known as NTLA-2002) in hereditary angioedema (HAE). HAE is a rare genetic condition in which patients experience recurrent and potentially life-threatening swelling (angioedema) attacks in various parts of their body, including the face, upper airway, abdomen and extremities due to an overproduction of bradykinin. Designed as a one-time treatment that is administered in an outpatient setting, lonvo-z is an *in vivo* CRISPR gene editing candidate that is intended to inactivate the *kallikrein B1 (KLKB1)* gene to permanently lower kallikrein and bradykinin levels.

Intellia separately announced today that it has initiated a rolling BLA submission to the FDA to seek regulatory approval. The company is preparing for a potential U.S. launch of lonvo-z in the first half of 2027.

“As the first Phase 3 data reported for an *in vivo* gene editing therapy, today’s HAELO results represent a profound milestone for Intellia, the broader CRISPR and precision medicine fields and, most importantly, the HAE community,” said John Leonard, M.D., Intellia President and Chief Executive Officer. “For those patients who have spent years battling unpredictable breakthrough swelling attacks, anxiety about their next attack or the many burdens associated with chronic prophylactic treatment, lonvo-z represents a potential paradigm shift in treatment. These data affirm lonvo-z’s potential, with one dose, to offer prolonged freedom from both attacks and the need for ongoing therapy.”

“We extend our deep gratitude to the many patients, caregivers and clinicians who have helped advance gene editing science by participating in our clinical trials. It is because of their contribution that we are advancing toward our first potential approval, with the goal of making lonvo-z available to U.S. patients in the first half of 2027,” Dr. Leonard concluded.

### **HAELO Topline Results**

HAELO is a randomized, double-blind, placebo-controlled Phase 3 trial designed to evaluate the efficacy and safety of a one-time 50 milligram dose of lonvo-z in adults and adolescents aged 16 years and older with Type I or Type II HAE. Key endpoints of the trial focused on the number of HAE attacks experienced by patients, quality of life, safety and tolerability.

A total of 80 patients were enrolled, with 52 receiving lonvo-z and 28 receiving placebo. Of the total population, 49% of patients were enrolled in the United States and 71% were on long-term prophylaxis (LTP) therapy at study entry. Patients on LTP were required to discontinue those therapies in the weeks prior to dosing.

Key findings from HAELO include:

- The trial met its primary endpoint. For the six-month efficacy evaluation period (weeks 5 to 28), a one-time infusion of lonvo-z reduced attacks by 87% versus placebo, with a mean monthly attack rate of 0.26 in the lonvo-z arm compared with 2.10 in the placebo arm ( $p < 0.0001$ ).
- The trial met all of its key secondary endpoints with statistical significance ( $p < 0.0001$ ). These included a 62% rate of patients who were entirely attack free and therapy free in the lonvo-z arm for the six-month efficacy evaluation period, compared with 11% of patients in the placebo arm.
- Favorable safety and tolerability data were observed for lonvo-z. The most common treatment emergent adverse events (TEAEs) during the primary observation period (infusion through week 28) were infusion-related reactions, headache and fatigue. All TEAEs reported as of the data cutoff (February 10, 2026) were mild or moderate and there were no serious adverse events observed in the lonvo-z arm.
- As of the data cutoff, all patients who received lonvo-z at baseline or in crossover after week 28 remained LTP free.

Additional clinical data from HAELO will be presented at the 2026 European Academy of Allergy and Clinical Immunology Congress (EAACI), taking place June 12-15 in Istanbul, Türkiye (abstract #100217).

“Despite the availability of several HAE treatments, many patients continue to experience significant burdens related to the disease, including breakthrough attacks and challenges associated with chronic treatment,” said Aleena Banerji, M.D., Professor at Harvard Medical School, Director of Clinical Care, Center for Drug and Vaccine Allergy at Massachusetts General Hospital, and a HAELO principal investigator. “The results we are seeing from lonvo-z demonstrate its potential to eliminate the need for chronic medication and related challenges. If approved as a one-time treatment, I would expect lonvo-z to be an appealing option for many patients.”

### **Webcast Information**

The company will host a conference call and webcast today at 8:00 a.m. ET to discuss the topline results. To join the webcast, please visit the Events page of the Investors & Media section on Intellia’s website at [intelliatx.com](http://intelliatx.com). A replay of the webcast will be available for approximately 90 days.

### **About Lonvo-z**

Based on Nobel Prize-winning CRISPR/Cas9 technology, lonvo-z has the potential to become the first one-time treatment for hereditary angioedema (HAE). Lonvo-z is an *in vivo* CRISPR gene editing candidate that is intended to permanently lower kallikrein by inactivating the *kallikrein B1 (KLKB1)* gene with a single dose. Lonvo-z has received five notable regulatory designations: Orphan Drug and RMAT Designation by the U.S. Food and Drug Administration (FDA), the Innovation Passport by the U.K. Medicines and Healthcare products Regulatory Agency (MHRA), Priority Medicines (PRIME) Designation by the European Medicines Agency, as well as Orphan Drug Designation (ODD) by the European Commission.

### **About Hereditary Angioedema**

Hereditary angioedema (HAE) is a rare, genetic disease characterized by severe, recurring and unpredictable inflammatory attacks in various organs and tissues of the body, which can be painful, debilitating and life-threatening. It is estimated that one in 50,000 people are affected by HAE. There are preventative and on-demand treatment options to help manage the condition, including long- and short-term prophylaxis used to prevent swelling attacks. Current treatment options often include lifelong therapies, which may require chronic intravenous (IV) or subcutaneous (SC) administration as often as twice per week or daily oral administration to ensure constant pathway suppression for disease control. Despite chronic administration, breakthrough attacks still occur. Kallikrein inhibition is a clinically validated strategy for the preventative treatment of HAE attacks.

## About Intellia Therapeutics

Intellia Therapeutics, Inc. (Nasdaq: NTLA) is a leading clinical-stage biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies. The company's mission is to transform the lives of people with severe diseases by developing and commercializing potentially curative treatments. With deep scientific, technical and clinical development experience, Intellia aims to reset the standard for medicine by durably treating the root causes of disease. Learn more at [intelliatx.com](https://intelliatx.com) and follow us [@intelliatx](https://twitter.com/intelliatx).

## Forward-Looking Statements

This press release contains “forward-looking statements” of Intellia Therapeutics, Inc. (“Intellia” or the “Company”) 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 beliefs and expectations concerning: the success and advancement of its program for lonvoguran ziclumeran or “lonvo-z” (also known as NTLA-2002) for the treatment of hereditary angioedema (“HAE”), including its plan to complete the submission of a biologics license application (“BLA”) for lonvo-z in the second half of 2026, its expectations regarding review and approval of that BLA, and its expectations regarding a potential U.S. launch of lonvo-z in the first half of 2027; and the potential of one dose of lonvo-z to offer prolonged freedom from both attacks and the need for ongoing therapy.

Any forward-looking statements in this press release 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: uncertainties related to the conduct of clinical studies and other development and commercialization requirements for its product candidates, including lonvo-z, including risks related to the ability to develop and successfully commercialize lonvo-z or any of Intellia’s product candidates; risks related to Intellia’s ability to protect and maintain its intellectual property position; risks related to Intellia’s relationship with third parties, including its contract manufacturers, collaborators, licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical study results will not be positive; and risks related to the potential delay of planned clinical trials or regulatory filings due to regulatory feedback or other developments. 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, as well as discussions of potential risks, uncertainties, and other important factors in Intellia's other filings with the Securities and Exchange Commission, including its quarterly report on Form 10-Q. All information in this press release is as of the date of the release, and Intellia undertakes no duty to update this information unless required by law.

***Investor Contact:***

Jason Fredette  
Vice President, Investor Relations and Corporate Communications  
Intellia Therapeutics, Inc.  
[jason.fredette@intelliatx.com](mailto:jason.fredette@intelliatx.com)

***Media Contact:***

Mike Tattory  
Vice President  
LifeSci Communications  
[mtattory@lifescicomunications.com](mailto:mtattory@lifescicomunications.com)

###

# Phase 3 HAELO Clinical Trial: Topline Data for Lonvo-z

April 27, 2026



**KIM**  
Living with Hereditary  
Angioedema



## 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 successfully develop and commercialize lonvoguran ziclumeran ("lonvo-z"), formerly known as NTLA-2002, for the treatment of hereditary angioedema ("HAE"); our ability to achieve upcoming objectives, including completing the submission of a biologics license application for lonvo-z for the treatment of HAE in the second half of 2026, and successfully launching lonvo-z for the treatment of HAE in the U.S. in the first half of 2027; the potential commercial opportunities for lonvo-z and our other product candidates, including the value and market potential for lonvo-z and the potential of lonvo-z to eliminate attacks and ongoing therapy with one treatment; and our ability to complete our current lonvo-z priorities to prepare for a successful launch, including scaling a field sales and reimbursement teams, finalizing pricing, and finalizing a contracting strategy.

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 the ability to successfully develop and commercialize lonvo-z or any of our other product candidates; risks related to Intellia's ability to protect and maintain its intellectual property position; risks related to Intellia's relationship with third parties, including our contract manufacturers, licensors and licensees; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to the authorization, initiation and conduct of preclinical and clinical studies and other development requirements for our product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical trial results will not be positive; risks related to the development and advancement of *in vivo* and *ex vivo* technologies for pipeline expansion and collaborations; risks related to Intellia's future financial condition and our ability to fund our operations; risks related to Intellia's collaborations with Regeneron or our other collaborations not continuing or not being successful; and risks related to Intellia's ability to execute its strategic plans, including completing pivotal clinical trials and commercial launch of its product candidates. 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 of 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.

## Today's Speakers



**Dr. John Leonard**, President and Chief Executive Officer  
*Intellia Therapeutics*



**Dr. David Lebwohl**, Executive Vice President and Chief Medical Officer  
*Intellia Therapeutics*



**Dr. Marc Riedl**, Professor of Medicine, Clinical Director of the U.S. Hereditary Angioedema Association (HAEA) Angioedema Center  
*University of California San Diego; HAELO principal investigator*

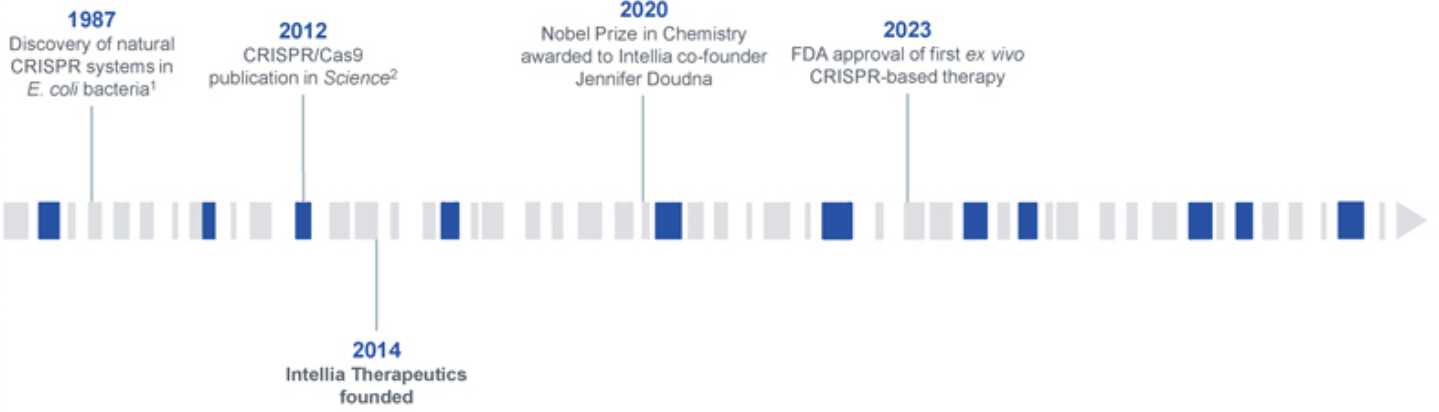
# Introduction

**Dr. John Leonard**

*President & CEO, Intellia Therapeutics*



# CRISPR is Coming of Age



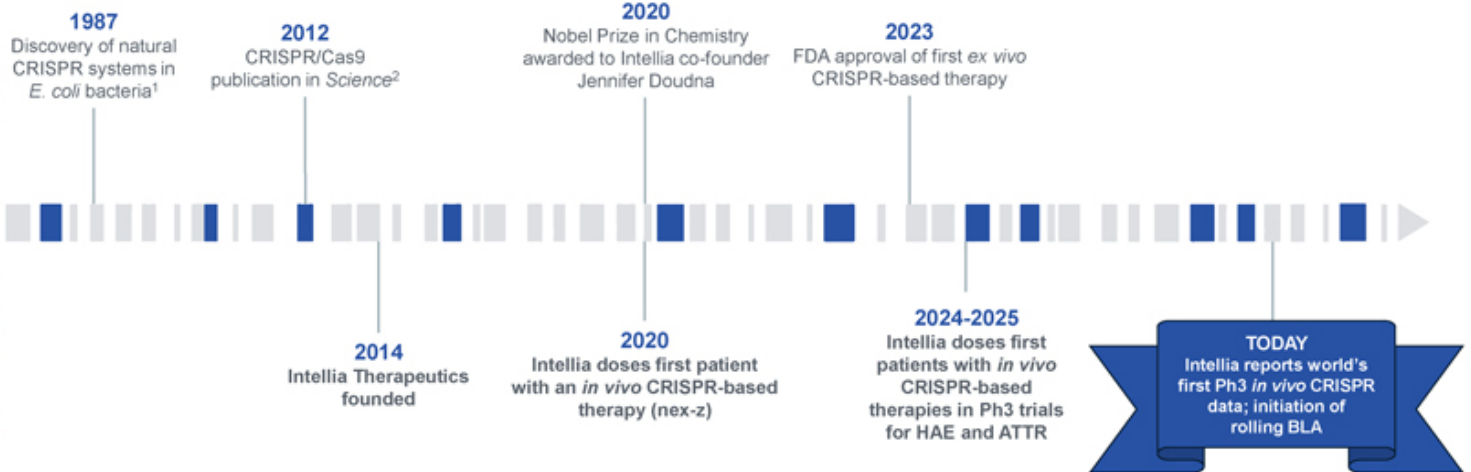
5 1. Gostimskaya I. *Biochemistry (Mosc)*, 2022 Aug;87(8):777-788. 2. Jinek et al. *Science*, 2012 Aug 17;337(6096):816-21.  
Cas9: CRISPR-associated protein 9, CRISPR: clustered regularly interspaced short palindromic repeats



## Intellia's Mission Statement

*To transform the lives of people with severe diseases by developing and commercializing potentially curative treatments.*

# CRISPR is Coming of Age



7 1. Gostimskaya I. *Biochemistry (Mosc)*, 2022 Aug;87(8):777-788. 2. Jinek et al. *Science*, 2012 Aug 17;337(6096):816-21.  
ATTR: transthyretin amyloidosis; BLA: biologics license application; Cas9: CRISPR-associated protein 9; CRISPR: clustered regularly interspaced short palindromic repeats; FDA: U.S. Food and Drug Administration; HAE: hereditary angioedema

# Intellia's Pipeline Advancing Toward Significant Near-Term Milestones

Program	Indication	Research/ Preclinical	Early-Stage Clinical	Late-Stage Clinical	BLA Submission	
Lonvo-z <sup>1</sup>	Hereditary Angioedema (HAE)					
Nex-z <sup>2</sup>	Transthyretin Amyloidosis with Polyneuropathy (ATTRv-PN)					
	Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM)					
REGV131-LNP1265 <sup>3</sup>	Hemophilia B					
AVC-201 AVC-203	Acute Myeloid Leukemia (AML) B-cell malignancies					
Other Ongoing Research Programs	Various					 

Lead refers to lead development and commercial party.

1. Lonvo-z (lonvoguran ziclumeran), formerly referred to as NTLA-2002. 2. Nex-z (nexiguran ziclumeran), formerly referred to as NTLA-2001; Regeneron shares in approximately 25% of worldwide development costs and commercial profits for the ATTR program and has an option to enter into a co-promotion agreement for the U.S. commercialization. 3. Hemophilia B is being advanced solely by Regeneron; Intellia is eligible for milestones and royalties. 4. AVC-201 and AVC-203 are wholly owned by AvenCell and utilize proprietary allogeneic cell engineering technology licensed from Intellia. 5. Intellia is advancing both wholly owned and partnered programs.

# About HAE and Lonvo-z

Dr. David Lebwohl

*Chief Medical Officer, Intellia Therapeutics*

HAE: hereditary angioedema

**Intellia**  
THERAPEUTICS

# Hereditary Angioedema (HAE): Currently a Lifelong Condition with Significant Burden

## Rare, genetic and life-threatening disease

- Caused by a hereditary deficiency or dysfunction of the C1 inhibitor protein that leads to an imbalance in the kallikrein-kinin system and an overproduction of bradykinin
- Patients experience unpredictable, recurrent, painful and potentially life-threatening swelling attacks<sup>1,2</sup>
- Symptoms often begin in the first decade of life and typically worsen in puberty<sup>3,4</sup>
- Attacks can be triggered by stress, trauma, infection, fatigue and hormones<sup>2</sup>

“

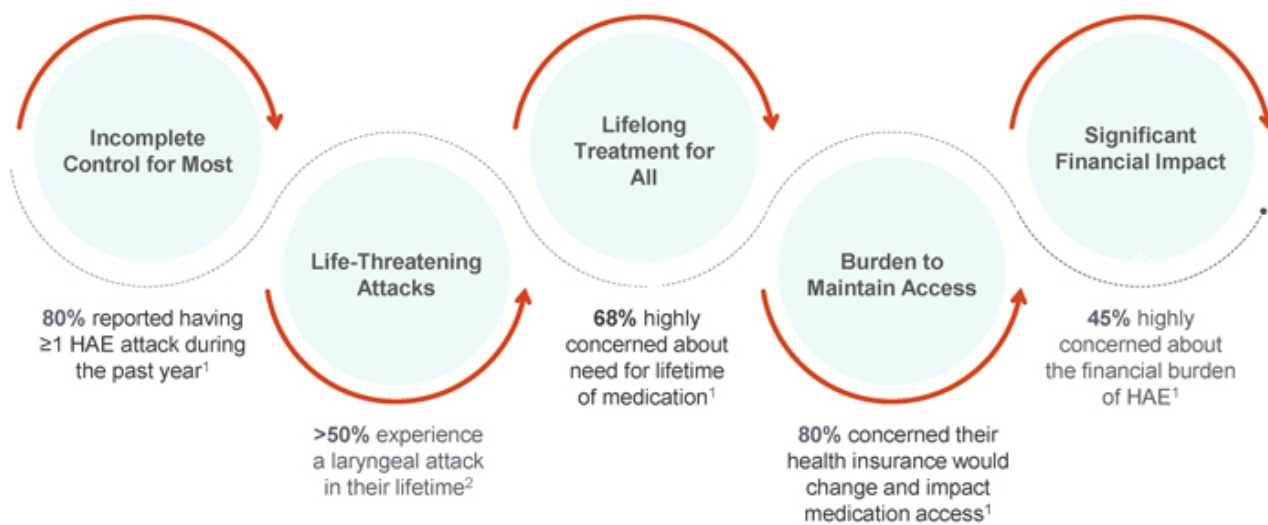
“The fear is always there — a tickle in your throat, and you think, ‘Do I have a cold, or is this a swell?’”

KIM  
Living with HAE

Inteija  
THERAPEUTICS

# Despite Available Treatments, Many Patients are Unable to Break the Chronic Cycle of Managing Their HAE

## Patient-Reported Burdens



# The Cumulative Costs for Chronic HAE Treatments are Sizable

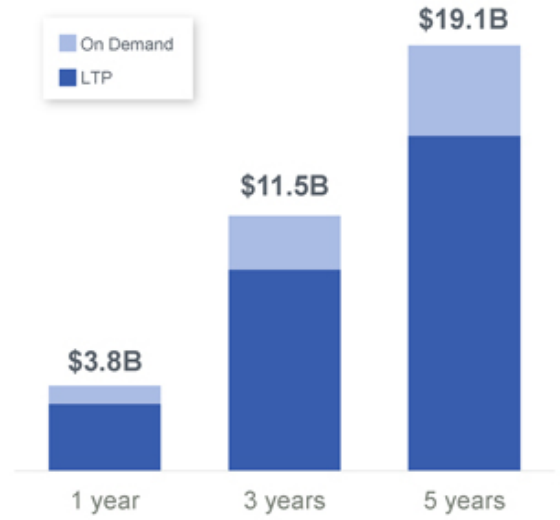
U.S. Treated Patients with Type 1 & 2 HAE<sup>1</sup>



Average Age of HAE Diagnosis in U.S.<sup>2</sup>



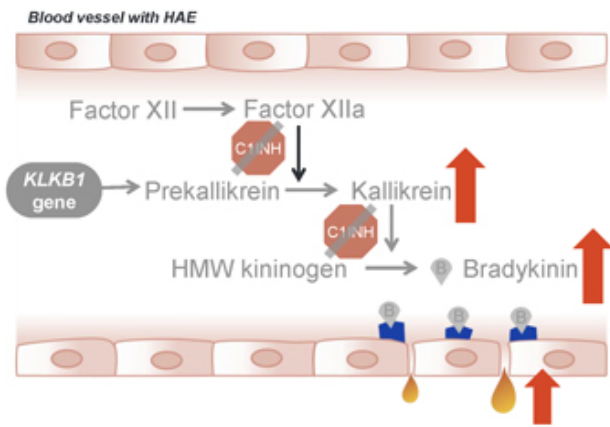
Cumulative U.S. Healthcare System Costs for Chronic and On-Demand HAE Therapies<sup>3</sup>



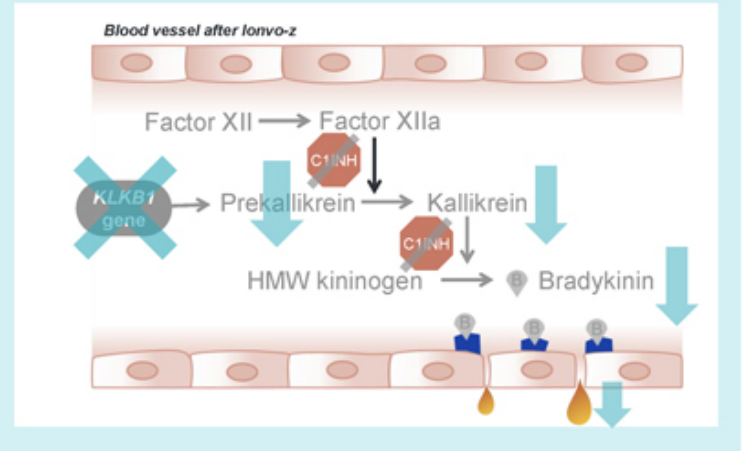
1. Castaldo et al. *Allergy Asthma Immunol*, 2025. 2. Banerji et al. *Annals of Allergy, Asthma & Immunology*, 2020. 3. IPD Analytics. *Market & Financial Insights*. Published April 2025. Assumes weighted average cost of LTP agents of \$684,000 and total on demand HAE market size \$812M; excludes annual price increases. B: billion; HAE: hereditary angioedema; LTP: long-term prophylaxis

# Lonvo-z is an Investigational One-Time HAE Treatment Intended to Permanently Inactivate the *KLKB1* Gene

In HAE, C1 inhibitor deficiency imbalances the kallikrein-kinin system (KKS), leading to excess protein production and debilitating swelling attacks

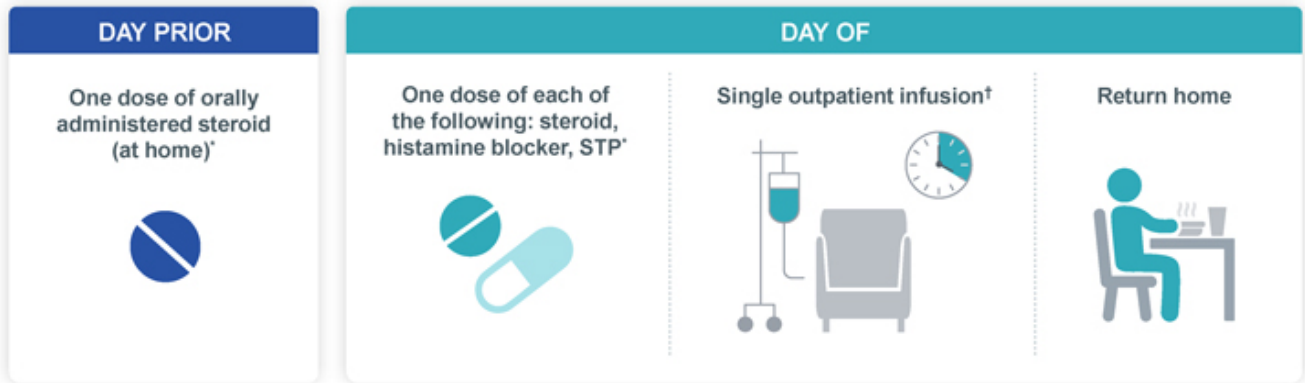


Lonvo-z was designed to stop HAE attacks: Inactivating *KLKB1* gene resets the KKS to stop excess protein production and HAE swellings



13 Adapted from Zuraw BL. *N Engl J Med*. 2008 Sep 4;359(10):1027-36. This presentation includes data for an investigational product not yet approved by regulatory authorities. C1INH: C1 esterase inhibitor; HAE: hereditary angioedema; HMW: high-molecular weight; *KLKB1*: kallikrein B1

# Lonvo-z is Designed to be Administered in an Outpatient Setting in Two to Four Hours

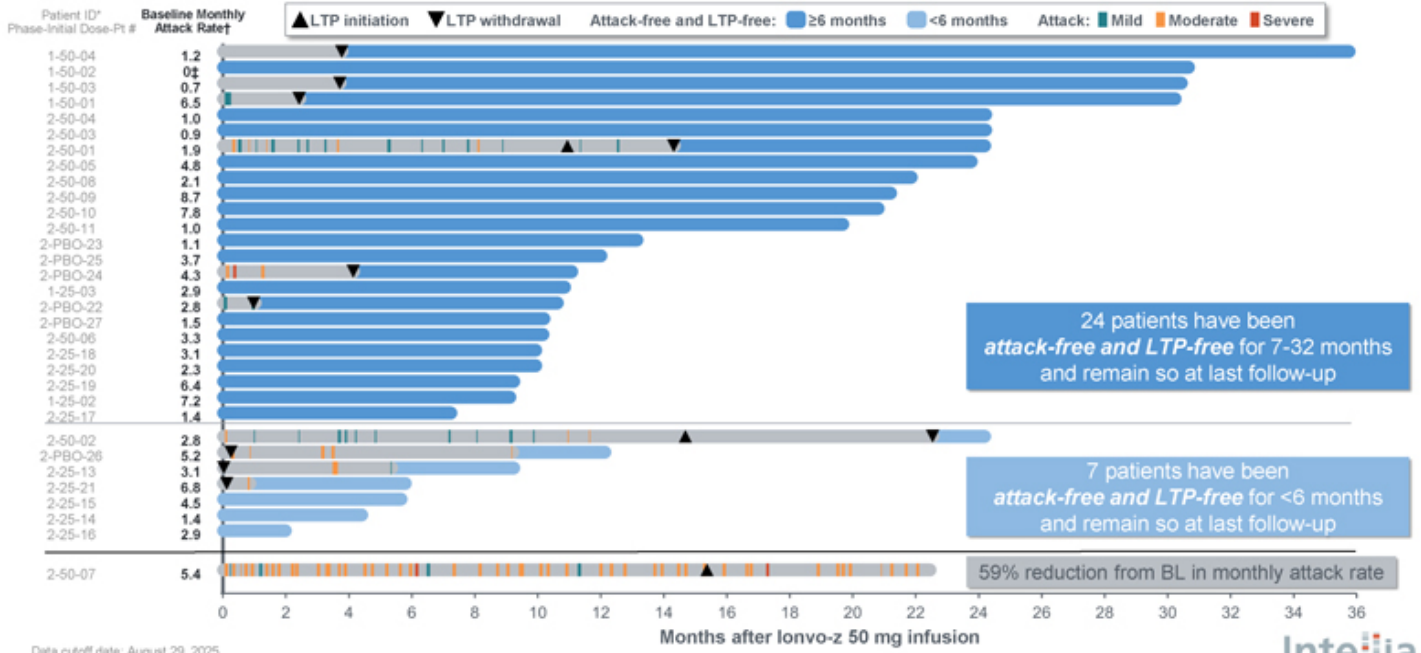


This schedule reflects the HAELO Phase 3 clinical trial protocol. Schedule may vary if approved.

HAELO Phase 3 dosing regimen pictured above

\* Pretreatment medication includes: oral dexamethasone on the day prior to treatment, followed by a pre dose regimen within 1-2 hours prior to treatment of IV or oral corticosteroid, histamine blocker. One IV or oral H1 blocker and one IV or oral H2 blocker. Patients are also administered an STP per standard of care for HAE patients undergoing a procedure. † lonvo-z is being developed for administration as a 2-4-hour infusion. Patient time at treatment center may vary depending on pre- and post-administration clinical and office procedures. STP: Short-term prophylaxis

# Pooled Analysis of Phase 1/2 Clinical Data: After Becoming Attack-Free and LTP-Free for ≥6 Months, All Patients Maintained Their Response



24 patients have been *attack-free and LTP-free* for 7-32 months and remain so at last follow-up

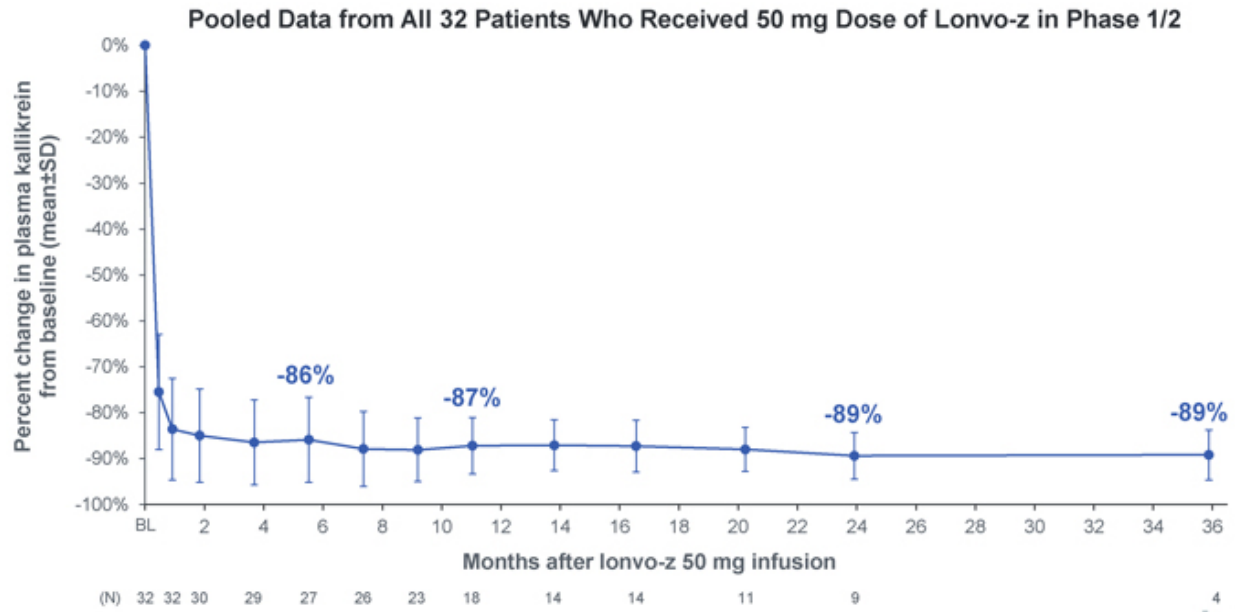
7 patients have been *attack-free and LTP-free* for <6 months and remain so at last follow-up

59% reduction from BL in monthly attack rate

Data cutoff date: August 29, 2025

Phase 1 eligibility was determined by historical attack period. \* Patient IDs align with prior Phase 1 and Phase 2 publications. † Baseline is defined as the screening period (50 mg initial dose or 25 mg to 50 mg) or for PBO to 50 mg as the time from informed consent to 50 mg infusion or start of any LTP, whichever occurred first. ‡ Patient had 0.9 attacks per month in the 3 months prior to screening. This presentation includes data for an investigational product not yet approved by regulatory authorities. BL: baseline; LTP: long-term prophylaxis; mg: milligram; PBO: placebo; Pt: patient.

# No Waning of Effect Observed: Kallikrein Reduction has Remained Deep and Durable



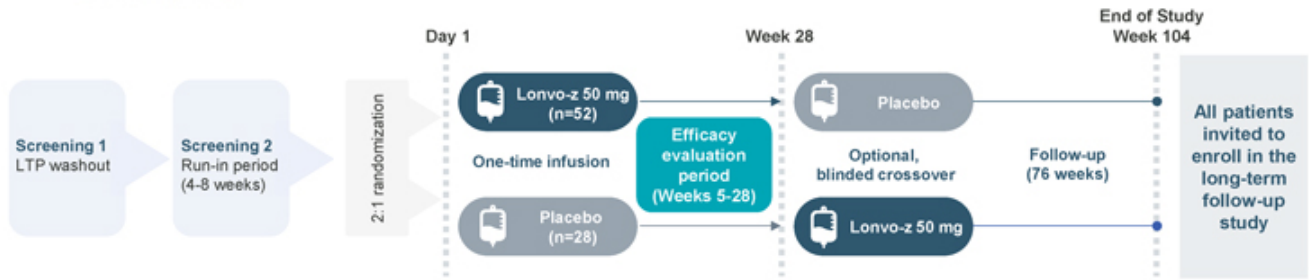
# HAELO Phase 3 Clinical Data

**Dr. Marc Riedl**

*Professor of Medicine, Clinical Director of the U.S. Hereditary Angioedema Association (HAEA) Angioedema Center at the University of California San Diego; HAELO principal investigator*

**Intellia**  
THERAPEUTICS

# A Placebo-Controlled, Double-Blind, Randomized Phase 3 Trial of Lonvo-z as a One-Time HAE Treatment



### Stratification

Baseline number of investigator-confirmed HAE attacks per month from Screening 2 to Randomization

### Primary Endpoint

Time-normalized number of investigator-confirmed HAE attacks from Weeks 5 through 28

### Key Secondary Endpoints

- Time-normalized number of investigator-confirmed HAE attacks requiring on-demand treatment from Weeks 5 through 28
- Time-normalized number of moderate or severe investigator-confirmed HAE attacks from Weeks 5 through 28
- Investigator-confirmed HAE attack-free status from Weeks 5 through 28
- Change from baseline to Week 28 in AE-QoL Questionnaire total score

**Pre-specified primary analysis when  $\geq 60$  patients reach Week 28**

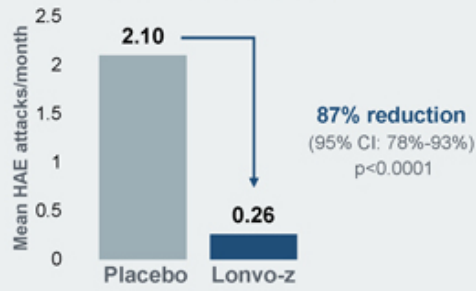
## HAELO Phase 3 Demographics and Baseline Characteristics

Demographic Characteristics	Lonvo-z (n=52)	Placebo (n=28)
Age, median years (range)	42 (23 – 71)	40 (19 – 76)
Female, n (%)	35 (67%)	20 (71%)
Enrolled in United States, n (%)	26 (50%)	13 (46%)
Hereditary angioedema type, n (%)		
Type 1	49 (94%)	25 (89%)
Type 2	3 (6%)	3 (11%)
Long-term prophylaxis at study entry, n (%)	35 (67%)	22 (79%)
Lanadelumab	25 (48%)	12 (43%)
C1 esterase inhibitor	5 (10%)	3 (11%)
Berotralstat	4 (8%)	1 (4%)
Garadacimab	1 (2%)	3 (11%)
Other	2 (4%)	3 (11%)
On-Demand therapy only, n (%)	17 (33%)	6 (21%)
Historic typical attack severity, n (%)		
Mild	7 (14%)	5 (18%)
Moderate	30 (58%)	20 (71%)
Severe	15 (29%)	3 (11%)
Monthly attack rate during run-in, mean (SD)*	3.5 (1.8)	3.5 (1.9)

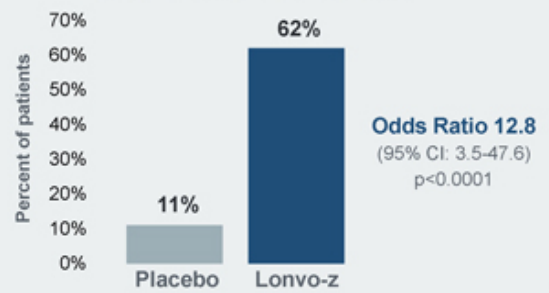
**Median follow-up for enrolled patients: 7.5 months**

# HAELO Trial Achieved its Primary and All Key Secondary Endpoints

Mean Attack-Rate Reduction Weeks 5 - 28  
(Primary Endpoint) <sup>1,2</sup>



Attack Free Weeks 5 - 28  
(Key Secondary Endpoint) <sup>2</sup>

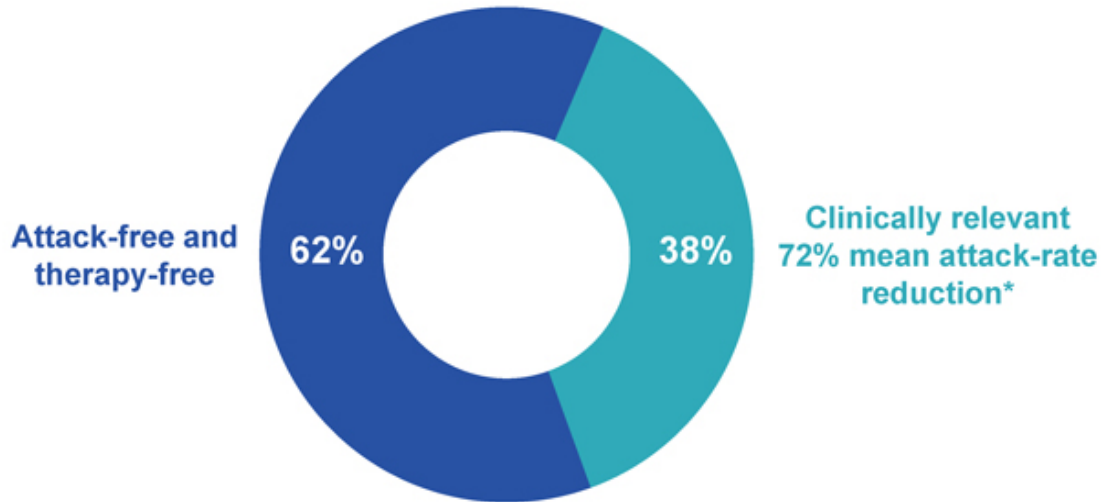


All other key secondary endpoints were achieved with high statistical significance (p < 0.0001)

Data cutoff: February 10, 2026

1. Model-based HAE attack rate using a Poisson regression model with Pearson Chi-square scaling of standard error with treatment arm and baseline attack rate (> 3 vs ≤ 3 attacks/month) as covariates. 2. Includes assessments up to and including Week 28, or the latest assessment prior to the data cut-off date for patients who did not reach Week 28. CI: confidence interval; HAE: hereditary angioedema

# 100% of Patients in Lonvo-z Arm Experienced Attack-Rate Reductions from Baseline During Weeks 5-28

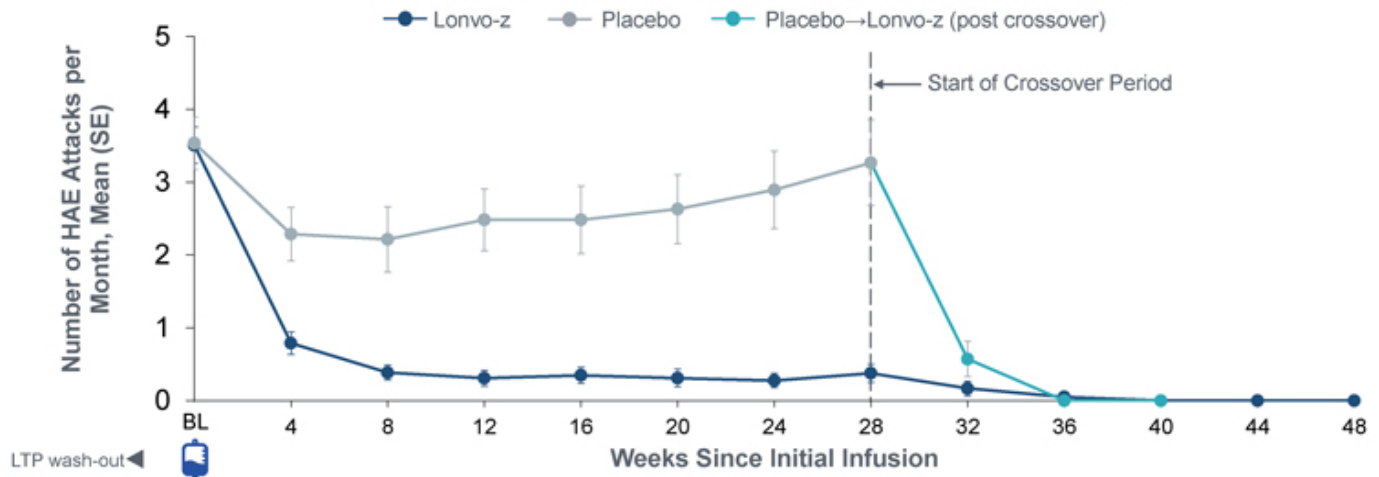


All patients who received lonvo-z at baseline or in crossover were LTP-free

\* Patients with ≥1 attack during weeks 5-28  
Data cutoff: February 10, 2020  
Post-hoc, exploratory analysis. LTP: long-term prophylaxis

# Attack-Rate Reduction Observed Quickly Following Lonvo-z Dosing; Further Reduction Observed in Early Crossover Data Following Week 28

Mean Monthly Investigator-Confirmed HAE Attack Rate Over Time



	BL	4	8	12	16	20	24	28	32	36	40	44	48
Lonvo-z (n)	52	52	52	52	52	52	50	44	43	20	8	6	3
Placebo (n)	28	28	28	27	27	27	25	21	0	0	0	0	0
Placebo→Lonvo-z (n)	0	0	0	0	0	0	0	0	20	12	3	0	0

Data cutoff: February 10, 2026

All eligible patients in the placebo arm received lonvo-z crossover infusion between Week 28 and Week 32. During the efficacy evaluation period, two patients in the placebo arm restarted LTP due to a high number of attacks. For these patients, data were censored at the time of LTP initiation and only attacks occurring prior to LTP initiation were included in the analysis. Both patients discontinued LTP before receiving the lonvo-z crossover infusion. HAE: hereditary angioedema; LTP: long-term prophylaxis; mg: milligram; SE: standard error

## Favorable Safety and Tolerability Data

Primary Observation Period (Weeks 1 – 28)	Lonvo-z (N=52)	Placebo (N=28)
<b>TEAEs in ≥10% of patients, n (%)</b>		
Infusion-related reaction	32 (62%)	5 (18%)
Headache	10 (19%)	3 (11%)
Fatigue	7 (14%)	3 (11%)
Nasopharyngitis	7 (14%)	9 (32%)
Back pain	6 (12%)	3 (11%)
Upper respiratory tract infection	6 (12%)	2 (7%)
<b>Serious TEAEs</b>	<b>0</b>	<b>1 (4%)*</b>
<b>Grade ≥3 TEAEs</b>	<b>0</b>	<b>0</b>

- No SAEs or Grade ≥3 TEAEs reported in lonvo-z arm
- All IRRs were mild or moderate and were transient
- No meaningful difference between arms in clinical chemistries; single Grade 2 ALT elevation observed in lonvo-z arm that self-resolved in one week
- Consistent safety and tolerability data observed in crossover following week 28 as of data cutoff

## HAELO Data Summary

- Novel one-time investigational treatment
- Primary and all key secondary endpoints achieved. During the six-month efficacy observation period:
  - 87% attack-reduction rate for lonvo-z vs. placebo
  - 62% of patients entirely free from attacks (and therapy)
- All patients in lonvo-z arm saw a reduction in attack rate from baseline
- Early crossover data trending favorably with attack rates approaching zero in both trial arms
- Favorable safety and tolerability data

# Advancing Toward Lonvo-z's Planned Launch

**Dr. John Leonard**

*President & CEO, Intellia Therapeutics*

## Additional HAELO Perspectives



### Significant Patient Enthusiasm

80 patients enrolled  
(original target:  $\geq 60$ )

All patients dosed within  
nine months

~70% of patients washed  
out of LTP to enroll



### Diverse Mix of Patients

Multi-national trial with  
~50% of enrolled patients  
in U.S.; broad age range

Population includes patients:

*With complete HAE control;  
partial control at entry*

*Who were on LTP and/or  
on-demand therapies at entry*









### Extensive (and Still Expanding) Phase 3 Database

Longest Phase 3 trial  
undertaken in HAE to date,  
once completed

Largest cohort of patients  
receiving proposed label dose  
(50 mg of lonvo-z)

# Lonvo-z: Unique Potential to Eliminate Attacks and Ongoing Therapy with One Treatment

## PHASE 3 CROSS-TRIAL COMPARISON\*

Product	% of Patients Attack & Therapy Free*	% Attack Reduction vs. Placebo*	% of Patients Attack Free*	Lifetime Dosing Burden
<b>lonvo-z</b> (investigational)	<b>62%</b>	<b>87%</b>	<b>62%</b>	<b>1x</b> infusion 
berotralstat <sup>1</sup>	0%	44%	n/a	Daily oral tablets 
C1 est. inh. <sup>2</sup>	0%	87%	n/a	104 injections / year 
donidalorsen <sup>3</sup>	0%	55-81%	35-53%	7-13 injections / year 
garadacimab <sup>4</sup>	0%	89%	62%	13 injections / year 
lanadelumab <sup>5</sup>	0%	74-87%	31-44%	13-26 injections / year 

*For illustrative purposes only.*

Chronic long-term prophylaxis (LTP) therapies

\* This graphic includes data from the blinded time periods of distinct clinical trials with their own enrollment criteria and methodologies. Cross-trial comparisons have inherent limitations and should be interpreted with caution. 1. berotralstat label. 2. C1 esterase inhibitor label. 3. donidalorsen label. 4. garadacimab label. 5. lanadelumab label.

## Preparing for a Successful Launch in 1H 2027\*

- ✓ Established core commercialization team
- ✓ Deployed field medical team
- ✓ Finalized overall launch strategy
- ✓ Commenced payer engagement
- ✓ Continued patient advocacy group/medical society engagements
- ✓ Finalized distribution model for launch
- ✓ Identified potential treatment centers
- ✓ Initiated rolling BLA submission with FDA

✓ 2025 Accomplishment    ✓ 2026 Accomplishment

### Priorities ahead...

Complete BLA submission

Scale field sales and reimbursement teams

Finalize pricing

Finalize contracting strategy

## Summary



HAELO trial **achieved its primary and all key secondary endpoints** with favorable safety and tolerability data

# #1

**Global first** for *in vivo* gene editing



Intellia advancing rapidly toward potential approval and **first planned launch** in 1H 2027\*



## Thank You!

We extend our gratitude to the patients, caregivers, and families who have taken part in the HAELO clinical trial; a decision not taken lightly and rooted in trust and hope.

A sincere thank you to the HAELO study investigators, site coordinators and staff whose commitment and hard work made this study possible.

We also express our appreciation to the U.S. Hereditary Angioedema Association (HAEA) and HAE International (HAEi) for their invaluable support and partnership throughout this journey.

This milestone is a shared achievement that we could not have achieved without the unwavering support of the HAE community.

Q & A



Intelia  
THERAPEUTICS



**Intellia**  
THERAPEUTICS