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

**FORM 6-K**

**Report of Foreign Private Issuer  
Pursuant to Rule 13a-16 or 15d-16 of  
the Securities Exchange Act of 1934**

**For the month of April 2026**

**Commission File Number: 001-36622**

**PROQR THERAPEUTICS N.V.**

**Zernikedreef 9  
2333 CK Leiden  
The Netherlands**

**Tel: +31 88 166 7000**

(Address, Including Zip Code, and Telephone Number,  
Including Area Code, of Registrant's Principal Executive Offices)

Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F:

Form 20-F  Form 40-F

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1):

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7):

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On April 8, 2026, ProQR Therapeutics N.V. (“ProQR”) hosted a virtual analyst and investor event entitled “Expanding the Axiomer™ RNA Editing Opportunity Beyond AX-0810” to discuss developments of its current pipeline programs and announce two new programs, AX-0811 and AX-0422, including the selection of biliary atresia as the initial indication for Phase 2 development of AX-0810, along with a strategic overview. ProQR also released a press release entitled “ProQR Highlights Pipeline Expansion and Multiple Clinical Catalysts at Investor and Analyst Event.” Copies of the presentation and the press release are attached hereto as Exhibit 99.1 and Exhibit 99.2, respectively, and are incorporated herein by reference.

ProQR hereby incorporates by reference the information contained herein into ProQR’s registration statements on Form F-3 (File No. [333-282419](#), File No. [333-270943](#), File No. [333-263166](#) and File No. [333-285767](#)).

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**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.

**PROQR THERAPEUTICS N.V.**

Date: April 8, 2026

By: /s/ Dennis Hom  
Dennis Hom  
Chief Financial Officer

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INDEX TO EXHIBITS

Number	Description
<a href="#">99.1</a>	<a href="#">Presentation of ProOR Therapeutics N.V. for April 8, 2026 Analyst and Investor Event.</a>
<a href="#">99.2</a>	<a href="#">Press Release of ProOR Therapeutics N.V. on April 8, 2026.</a>



# EXPANDING THE AXIOMER™ RNA EDITING OPPORTUNITY BEYOND AX-0810

Nasdaq: PRQR | April 8, 2026



# Agenda

## Agenda

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### Welcome & Agenda

Sarah Kiely

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### Opening Remarks

Daniel A. de Boer

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### Platform and Pipeline Programs

Cristina Lopez Lopez  
Gerard Platenburg

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### Corporate Outlook

Dennis Hom

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### Q&A

Daniel A. de Boer  
Cristina Lopez Lopez  
Gerard Platenburg  
Dennis Hom

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## Speakers

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**Sarah Kiely**

*VP Investor Relations  
& Corporate Affairs*

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**Daniel A. de Boer**

*Founder & CEO*

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**Cristina Lopez Lopez, MD, PhD**

*Chief Medical Officer*

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**Dennis Hom**

*Chief Financial Officer*

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**Gerard Platenburg**

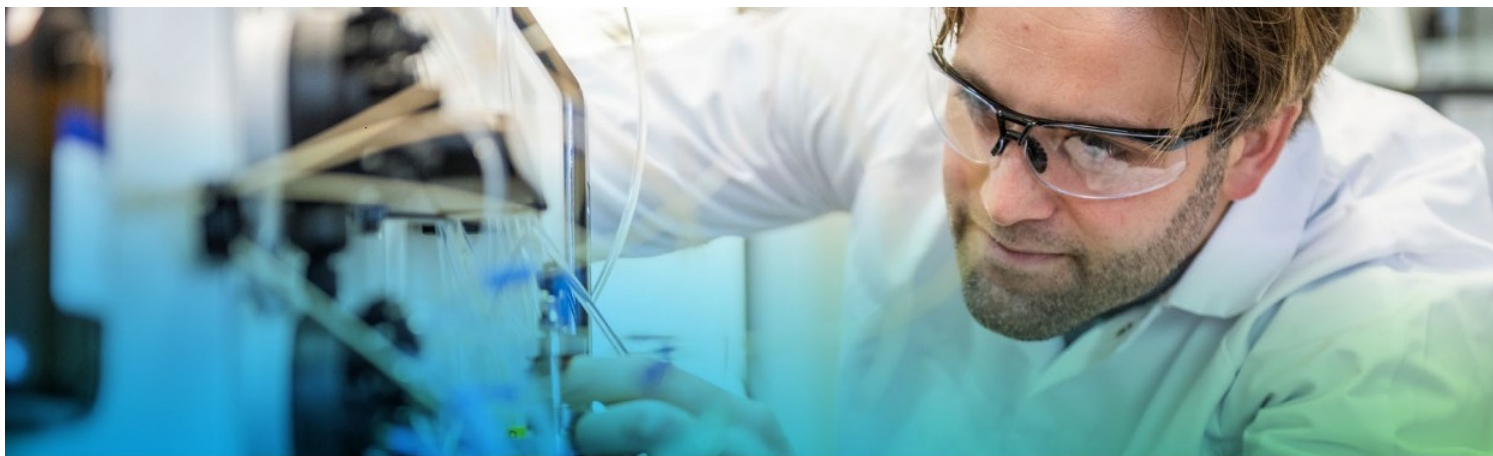
*Chief Scientific Officer*

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# Forward-looking statements

This presentation contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "continue," "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Such forward-looking statements include, but are not limited to, statements regarding our business, technology, strategy, preclinical and clinical model data; our initial pipeline targets and the upcoming strategic priorities and milestones related thereto, the continued advancement of our lead development pipeline programs, including approved, ongoing and planned clinical trials; expectations regarding the ongoing Phase 1 clinical trial of AX-0810 in NTCP for cholestatic diseases, including the planned trial design, and our ability to recruit for and complete a Phase 1 clinical trial for AX-0810, biliary atresia as our primary indication for AX-0810, the timing of top-line data readout for Phase 1 and Phase 1b of the clinical trial and the initiation of Phase 2 trial; expectations regarding the safety and therapeutic benefits of AX-0810, including the planned dosing levels and their efficacy; the anticipated timing of initial Phase 1 clinical data for our lead program, AX-0810, in H1 2026; our new pipeline targets, including the planned Phase 1 clinical trial of AX-0811 in NTCP for cholestatic diseases, our ability to recruit for and complete a Phase 1 clinical trial for AX-0811, an anticipated CTA filing and the Phase 1b cohort 1 data readout for AX-0811 pending regulatory clearance, expectations regarding the efficacy, clinical development timeline, and expected trial designs and development of AX-0422 and AX-2911, including the potential CTA filings and data readout pending regulatory clearance; clinical updates across multiple programs in 2026 and 2027; the therapeutic potential and development timeline regarding AX-0810, AX-0811, AX-0422, AX-2911 and AX-2402; the continued development and advancement of our Axiomer™ platform; the therapeutic potential of our Axiomer RNA editing oligonucleotides and product candidates; the timing, progress and results of our preclinical studies and other development activities, including the release of data related thereto; our patent estate, including our anticipated strength and our continued investment in it; our AI strategy and expectations regarding AI's ability to accelerate Axiomer discovery; our partnership with Ginkgo; and the potential of our technologies and product candidates. Forward-looking statements are based on management's

beliefs and assumptions and on information available to management only as of the date of this presentation. Our actual results could differ materially from those expressed or implied by these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our most recent annual report filed on Form 20-F. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by us and our collaborative partners whose operations and activities may be slowed or halted shortage and pressure on supply and logistics on the global market, economic sanctions and international tariffs; the likelihood of our preclinical and clinical programs being initiated and executed on timelines provided and reliance on our contract research organizations and predictability of timely enrollment of subjects and patients to advance our clinical trials and maintain their own operations; our reliance on contract manufacturers to supply materials for research and development and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the unpredictability of the duration and results of the regulatory review of applications or clearances that are necessary to initiate and continue to advance and progress our clinical programs; the ability to secure, maintain and realize the intended benefits of collaborations with partners, including the collaboration with Lilly; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in research and development; general business, operational, financial and accounting risks, and risks related to litigation and disputes with third parties; and risks related to macroeconomic conditions and market volatility resulting from global economic developments, geopolitical events and conflicts, high inflation, rising interest rates, tariffs and potential for significant changes in U.S. policies and regulatory environment. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law.  
Photo credits cover slide: <https://cindyjeurissen.com/>

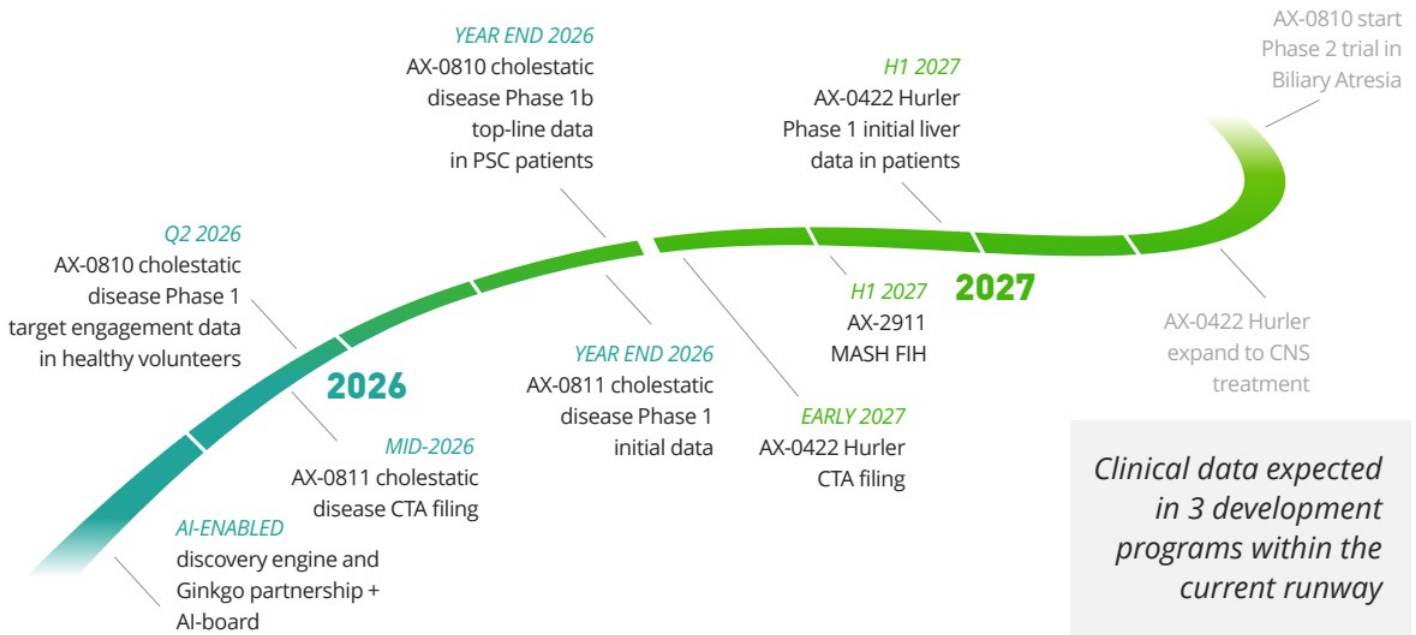


# Strategic Overview

*Presenter: Daniel A. de Boer*

# Multiple clinical catalysts within the runway

Core focus on cholestatic development, with value creating pipeline beyond





# Pipeline Programs

*Presenters: Cristina Lopez Lopez & Gerard Platenburg*

# Addressing unmet need in cholestatic diseases through NTCP modulation



## CHOLESTATIC DISEASE

- Biliary Atresia affects pediatrics early in life (~20,000 patients worldwide)
- Primary Sclerosing Cholangitis affects adults (~80,000 US+EU)
- No approved therapies and may require liver transplantation<sup>1,2</sup>



## BILE ACID TOXICITY

- Bile acid accumulation drives liver injury, leading to fibrosis and liver failure



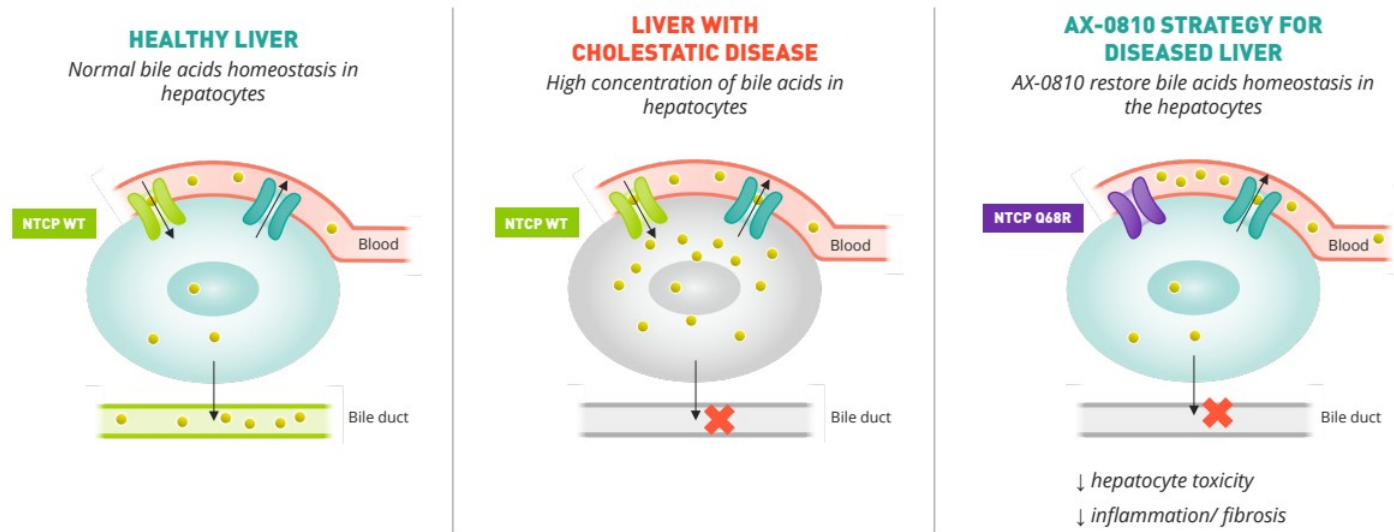
## NTCP MODULATION STRATEGY

- Human genetics supports NTCP modulation as hepato-protective mechanism to reduce bile acid reuptake and protect liver



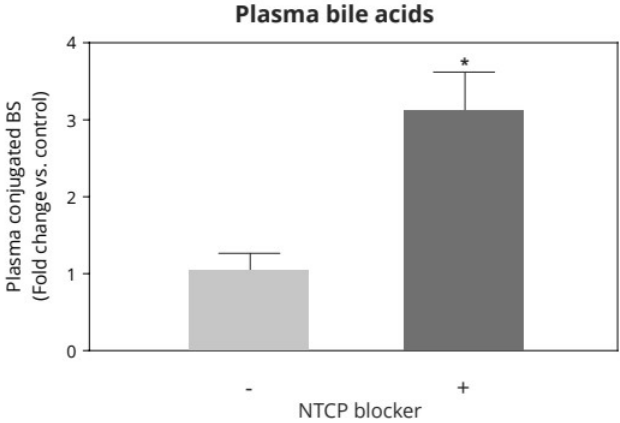
NTCP, sodium taurocholate co-transporting polypeptide. References: <sup>1</sup>Trivedi PJ, et al. Clin Gastroenterol Hepatol. 2022 Aug;20(8):1687-1700.e4; <sup>2</sup>Schreiber RA, et al. J Clin Med. 2022 Feb 14;11(4):999

# AX-0810 reduces bile acid accumulation in hepatocytes by modulating NTCP activity

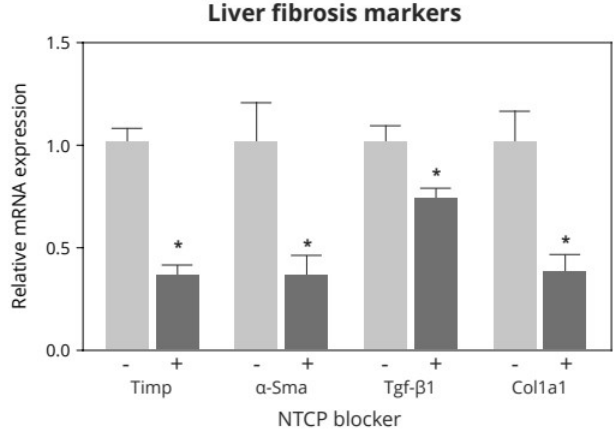


Halilbasic E, et al. J Hepatol. 2013 Jan;58(1):155-68; Nyholm I, et al. J Hepatol. 2025 Aug;83(2):440-452.

# NTCP modulation reduces fibrosis markers and elevates circulating bile acids



NTCP channel blocking **increases plasma bile acids concentrations**, up to 3-fold in cholestatic disease mouse model



**Pro-fibrotic markers show reduced expression** after NTCP channel blocking

Bulevirtide (Hepcludex) is a daily SC injected NTCP inhibitor approved for Hepatitis D. Slijepcevic D, et al. Hepatology. 2018 Sep;68(3):1057-1069.

# Assessing Safety, PK and Target Engagement of AX-0810 in First-in-Human Trial

Multiple ascending dose (MAD) N=33 (24 on treatment, 9 on placebo)



DMC safety reviews before proceeding to next dose and dose escalation is sequential during the dosing phase

## Treatment

AX-0810 GalNAc conjugated editing oligonucleotide

## Objectives

- Assess safety, tolerability, and PK of AX-0810
- Confirm target engagement as measured by biomarkers

## Key endpoints

- Change in bile acids levels
- Bile acids profile
- TUDCA challenge
- Liver biomarkers

## Phase 1 progressing

- ✓ Initial AX-0810 data demonstrate no safety signals and pharmacokinetics consistent with non-clinical models
- Target engagement data on track for H1 2026

CTA, Clinical Trial Application; DMC, Data Monitoring Committee; MAD, Multiple Ascending Dose; PK, Pharmacokinetics; TUDCA, Tauroursodeoxycholic acid; AX-0810 CTA has been approved in Europe (October 2025).

# Biliary atresia is a severe pediatric disease with no approved therapies



## DIAGNOSIS

*Pediatric: in the first weeks of life*



## POPULATION

*Approximately 20,000 patients worldwide*



## SYMPTOMS

*Jaundice, poor weight gain, pale stool, dark urine*



## PROGRESSION

*Rapid progression to cirrhosis and portal hypertension early in life*



## STANDARD OF CARE

*Kasai procedure as first-line therapy*



## SIGNIFICANT UNMET NEED

*No approved pharmacological treatments; 60-80% require liver transplant despite Kasai*

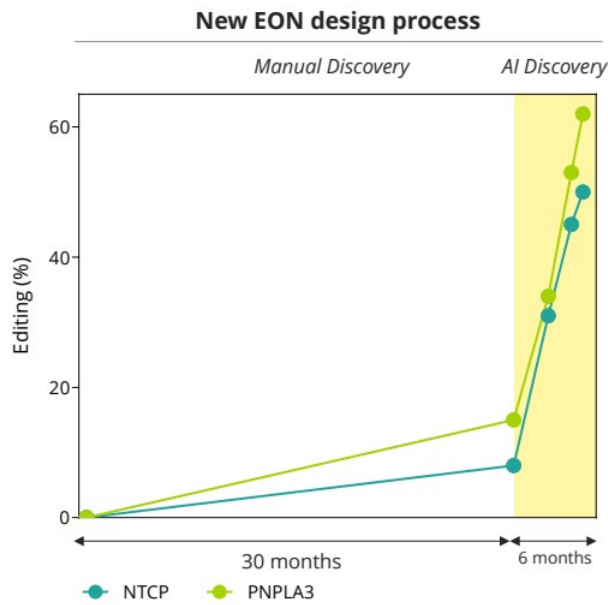
Adike A, et al. Expert Rev Gastroenterol Hepatol. 2018 Oct;12(10):1025-1032; Verklade HJ, et al. J Hepatol. 2016 Sep;65(3):631-42; Sundaram SS, et al. Liver Transpl. 2017 Jan;23(1):96-109; Raghu VK, et al. Liver Transpl. 2021 May;27(5):711-718; NORD, 2019, Japanese Biliary Atresia Society. Japanese Biliary Atresia Registry (JBAR). <https://jbas.net/en/national-registration/>.

# Biliary atresia as primary indication for AX-0810

BILIARY ATRESIA				
SEVERITY AND UNMET NEED	BIOLOGICAL RATIONALE	CLINICAL DE-RISKING	CENTRALIZED CARE	REGULATORY PATHWAY
Leading cause of pediatric liver transplantation and no approved therapies	AX-0810 targets the key driver of liver injury in the disease	No comorbidities and limited confounding factors	Patients concentrated in specialized centers	Pediatric guidance and orphan designation potential

# AI-guided EON design accelerates discovery

~90% faster discovery and up to 6× improvement in EON performance



Trained on 12+ years of **PROPRIETARY AXIOMER DATA**

Trained on experimentally-validated editing outcomes of numerous EONS and targets



AI enables discovery of **BETTER-PERFORMING EONS**

Models trained on our in-house data generate EONS with higher editing efficiency and greater sequence diversity



Robotics-enabled HTS **ACCELERATES DESIGN-TEST CYCLES**

Enabling rapid iteration per target and amplifying AI-driven learning through continuous model improvement

# AX-0811: AI-enabled next-gen RNA editing therapy targeting NTCP for cholestatic diseases

## ~3x higher editing efficiency

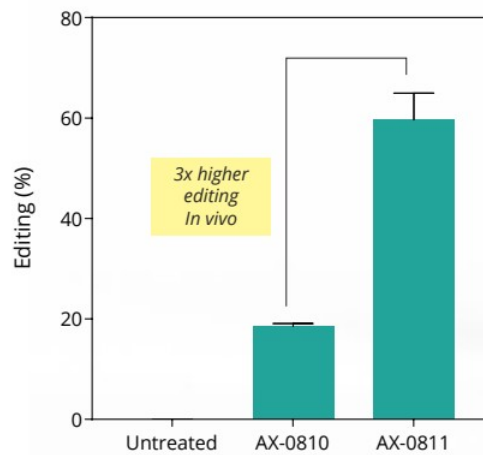
- Improved efficacy and dosing convenience over AX-0810

## Upcoming development milestones

- AX-0811 program advancing rapidly, with CTA filing in mid-2026
- Initial clinical data in healthy volunteers expected before year-end 2026

## Editing of *hNTCP* in livers of humanized mice

*SC administration, GalNAc EONs, 30mg/kg, 10 doses, n=3-4, D24, dPCR, Mean, SEM*



# Assessing Safety, PK and Target Engagement of AX-0811 in First-in-Human Trial

Multiple ascending dose (MAD) N=33 (24 on treatment, 9 on placebo)



DMC safety reviews before proceeding to next dose and dose escalation is sequential during the dosing phase

## Treatment

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## Objectives

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- Confirm target engagement as measured by biomarkers

## Key endpoints






- Change in bile acids levels
- Bile acids profile
- TUDCA challenge
- Liver biomarkers

## CTA enabling activities ongoing

- CTA filing in mid 2026
- Target engagement data expected by year-end 2026

CTA, Clinical Trial Application; DMC, Data Monitoring Committee; MAD, Multiple Ascending Dose; PK, Pharmacokinetics; TUDCA, Tauroursodeoxycholic acid

# ProQR building a leading NTCP franchise in cholestatic disease

NTCP FRANCHISE				
AX-0810			AX-0811	
				
<p><i>Rapid path to patients with <b>high unmet medical need</b></i></p>	<p><i>Establishes clinical validation</i></p>	<p><i>Awareness amongst <b>physicians and centers of excellence</b></i></p>	<p><i><b>AI-discovered next-gen candidate</b> with enhanced efficiency</i></p>	<p><i>Optimized Dose and scalability</i></p>

# AX-0422 RNA editing therapy to address Hurler Syndrome



## HURLER SYNDROME

- Most severe form of MPS1
- Early onset, multi-symptom disease
- Progressive deterioration, high morbidity
- Current therapies do not address all comorbidities and have limitations



## IDUA DEFICIENCY

- W402X mutation (c. 1293G>A; p.W402X) is present in up to 60% of patients with severe phenotype<sup>1</sup>
- Causes IDUA deficiency, leading to toxic accumulation of GAGs



## CLINICAL DE-RISKING

- AX-0422 corrects the W402X mutation back to WT
- Restores endogenous enzyme production, leading to GAGs clearance
- Potential to impact systemic and CNS disease



GAGs: glycosaminoglycans; MPS1: Mucopolysaccharidosis type I. <sup>1</sup>Baldo G, et al, 2018, <https://doi.org/10.1111/cge.13224>

# Increases in IDUA enzymatic activity drive meaningful clinical impact

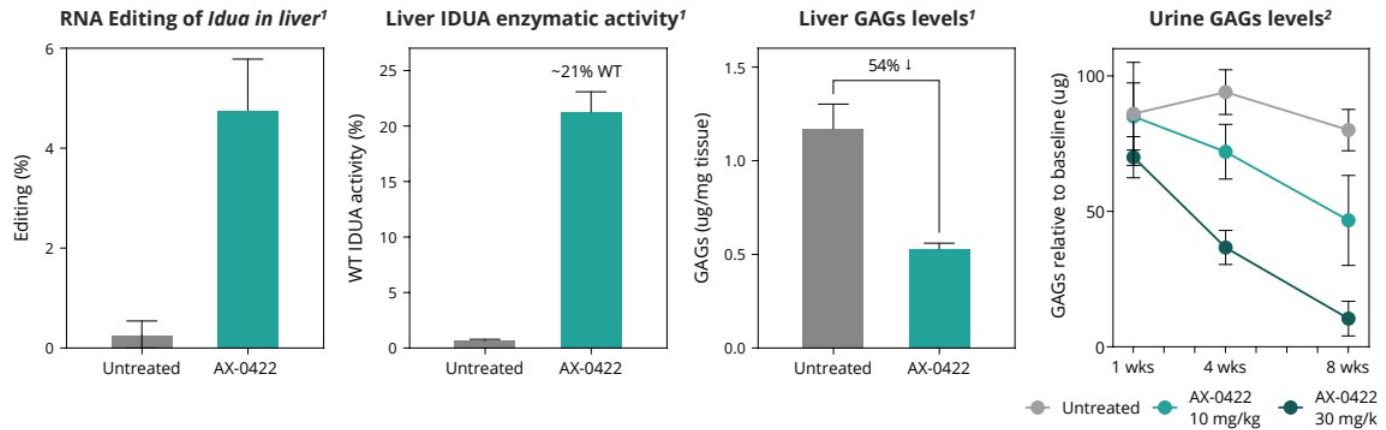
Severity

	Scheie	Hurler-Scheie	Hurler
Diagnosis	Teens	Childhood	< 18 months
Life expectancy	Normal	20 yo	10 yo
Enzymatic activity in fibroblasts (% of WT) <sup>1</sup>	<b>0.8%</b>	<b>0.3%</b>	<b>0.2%</b>

A restoration of 1-15% of normal IDUA enzymatic function<sup>2</sup> can improve phenotype

<sup>1</sup>Oussoren E, et al. *Mol Genet Metab.* 2013 Aug;109(4):377-81; <sup>2</sup>Kakkis ED, et al. *N Engl J Med.* 2001 Jan 18;344(3):182-8.

# RNA editing achieves therapeutically meaningful enzyme restoration in *Idua* mouse model



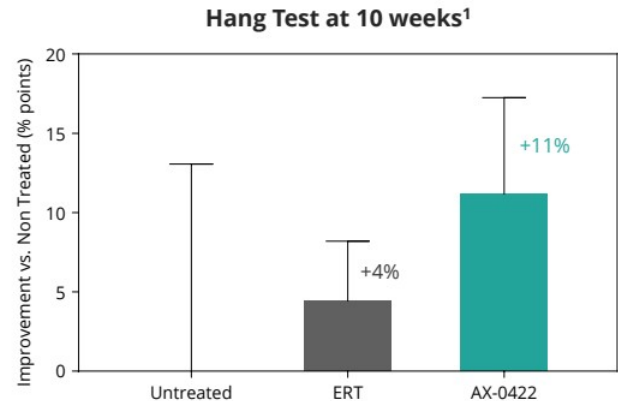
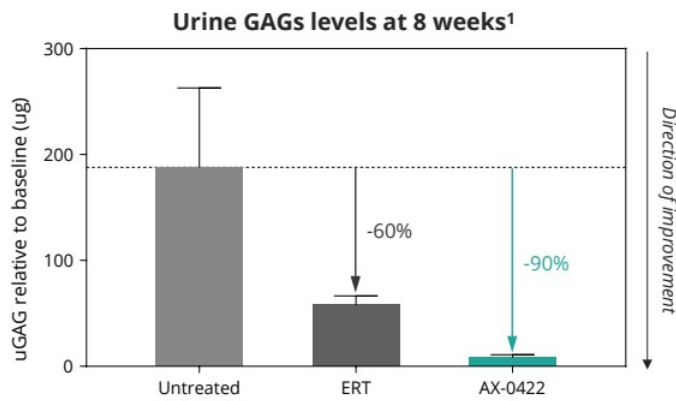
Following SC delivery, targeted editing of the W402X mutation restores ~21% IDUA activity, driving substantial liver GAG reduction and dose-dependent normalization of urinary GAGs - supporting potential for disease-modifying benefit

<sup>1</sup>AX-0422 surrogate treatment of *Idua*-W392X mice, SC, 30 mg/kg, Q1W until 8 wks, data at 8 weeks, n=6, mean, SEM; <sup>2</sup>AX-0422 surrogate treatment of *Idua*-W392X mice, SC, 10 and 30 mg/kg, Q1W until 4 wks, n=4-6, mean, SEM

# AX-0422 shows differentiated activity vs standard of care in *Idua* mouse model



Greater biomarker reduction and functional improvement vs ERT

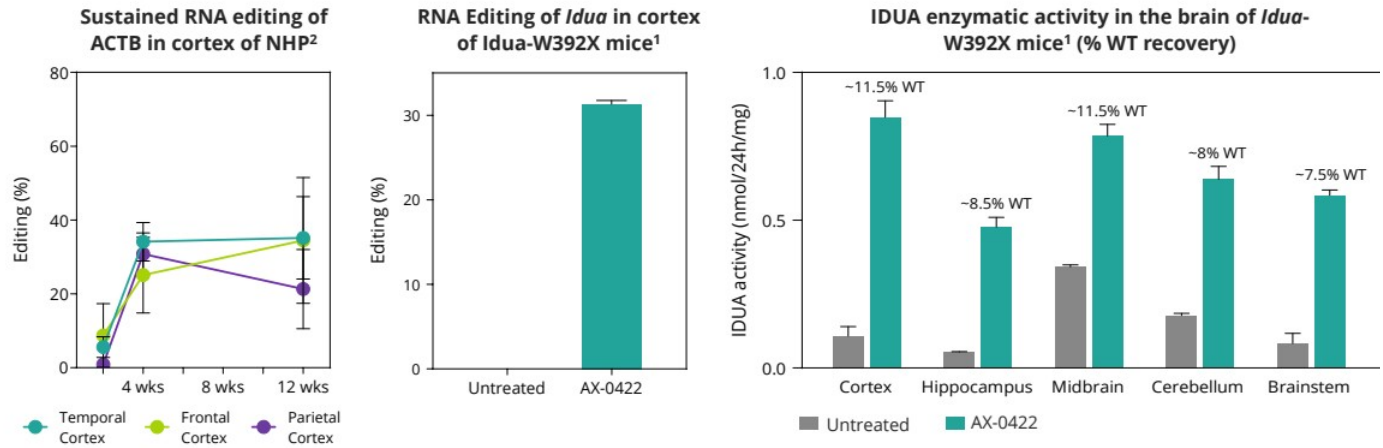


AX-0422 delivers reduction in urinary GAGs compared to ERT, approaching biomarker normalization

AX-0422 shows improvement in motor skills test compared to ERT

<sup>1</sup>*Idua*-W392X mice, AX-0422 surrogate treatment: SC, 30 mg/kg, ERT (Laronidase) treatment: IV, 0.58 mg/kg, Q1W until 4 wks, n=6, mean, SEM

# AX-0422 achieves robust, durable CNS editing with functional enzyme restoration



- Axiomer results in sustained CNS editing of up to 12 weeks (single dose, NHP)
- Following ICV delivery, efficient editing in Hurler disease model leads to broad enzyme restoration across brain regions (~7–12% of WT)
- Levels consistent with disease-modifying potential in Hurler syndrome

<sup>1</sup>AX-0422 surrogate treatment of *Idua*-W392X mice ICV, 250µg, single dose, n=6, 4 weeks, ddPCR, mean, SEM / western blot, mean, SEM; <sup>2</sup>IT administration, 10.6mg AX-0422 surrogate treatment, single dose, n=3, up to 12 weeks, ddPCR, mean, SD

# AX-0422 positioned to redefine the standard of care in Hurler Syndrome

*Positioned to deliver systemic and neurological benefit through a single, targeted mechanism*



## DIFFERENTIATED APPROACH

- Impact liver and neurological driven symptoms
- Convenient, infrequent dosing potential
- Avoids limitations of SoC



## HIGH AXIOMER PLATFORM POTENTIAL

- RNA editing restores endogenous enzyme production
- Preclinical data show relevant enzyme restoration, biomarker and functional improvement

# AX-0422 preliminary clinical development

*A two-step approach with liver delivery followed by CNS delivery*

## Subcutaneous administration for Liver



★  
Interim  
biomarker readout  
in H1 2027

## Intrathecal administration for CNS



- Primary objective: safety, tolerability
- Secondary: pharmacokinetics
- Exploratory PD and clinical measures: plasma IDUA enzyme activity and protein level; HS and DS levels
- Development candidate selected
- CTA filing in early 2027
- First-in-human trial clinical biomarker data in patients in H1 2027

DS: dermatan sulfate; HS: heparan sulfate

# AX-2911 RNA editing therapy to address metabolic dysfunction-associated steatohepatitis (MASH)



## MASH

- Highly prevalent and increasing worldwide
- Progression to cirrhosis, liver cancer and liver-related mortality
- Limited treatment options<sup>1</sup> highlight the significant unmet medical need, particularly in lean MASH patients



## PNPLA3 I148M

*Patatin-like phospholipase domain-containing<sup>3</sup> variant*

- Strongest genetic risk factor for disease progression
- ~50% of MASH patients<sup>2-4</sup>
- Associated with higher liver fat, NASH risk, and fibrosis progression
- Carriers may show reduced response to GLP-1 agonists<sup>5</sup>



## RESTORING WT-LIKE PNPLA3

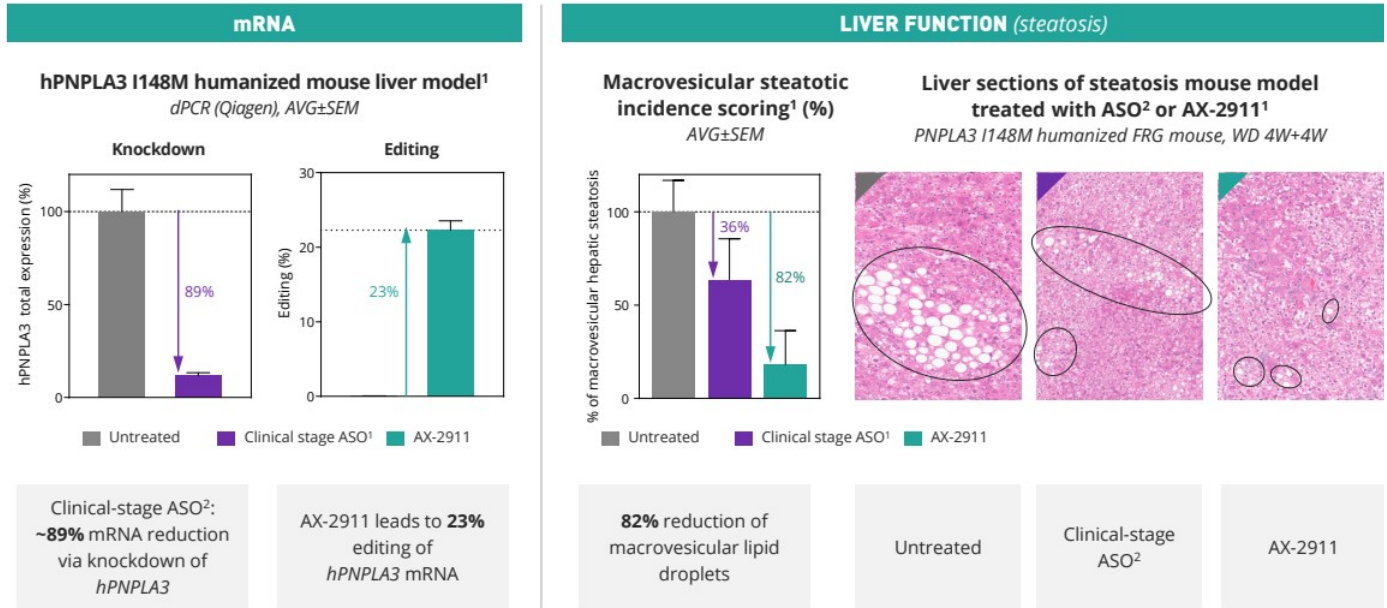
- AX-2911 restores PNPLA3 I148M (Met→Val) function
- Targets MASH primary genetic driver, unlike metabolic therapies
- Broad potential, including GLP-1-low response and lean MASH patients



<sup>1</sup>Sandireddy R, et al. *Front Cell Dev Biol.* 2024 Jul 16;12:1433857; <sup>2</sup>Tsedendorj Yumchinsuren et al., 2025; <sup>3</sup>Sookoian Silvia et al., 2011; <sup>4</sup>Souza Matheus et al., 2024; <sup>5</sup>Chen, Yunzhi et al, 2020

# Editing has functional advantage over knockdown

*AX-2911 substantially reduces liver fat vs clinical-stage ASO<sup>2</sup>*



# AX-2911 development strategy

Exploring an Investigator-Initiated Trial (IIT) in China



## OBJECTIVE

*Generate early proof-of-concept in patients*

*De-risk the program and inform development strategy*



## ACCELERATED APPROACH

*Parallel preparation for global CTA/IND development*



## EXPECTED TIMELINE

*FIH in H1 2027*

*Interim readouts to guide next steps*

# ProQR development pipeline and milestones

	TARGET	AXIOMER APPLICATION	DISCOVERY	NON-CLINICAL	CLINICAL	MILESTONES	ESTIMATED POPULATION
<b>DEVELOPMENT PIPELINE</b>							
<b>AX-0810</b> <i>for Cholestatic diseases</i>	NTCP	Modulate				Target engagement data 1H 2026	~100K patients
<b>AX-0811</b> <i>for Cholestatic diseases</i>	NTCP	Modulate				Target engagement data in 2026	
<b>AX-0422</b> <i>for Hurler Syndrome</i>	IDUA	Correct				CTA filing early 2027; Clinical biomarkers in H1 2027	~500-1000 patients
<b>AX-2911</b> <i>for MASH</i>	PNPLA3	Correct				FIH H1 2027	~8M patients
<b>AX-2402</b> <i>for Rett syndrome</i>	MECP2 R270X	Correct					~5K
<b>PARTNERED PIPELINE</b>							
10 undisclosed targets (option to expand to 15)			<i>Progress undisclosed</i>				



**IT'S IN  
OUR RNA**



## ProQR Highlights Pipeline Expansion and Multiple Upcoming Clinical Catalysts at Investor and Analyst Event

- AX-0810 clinical target engagement data in healthy volunteers on track for this quarter; biliary atresia selected as initial Phase 2 indication
- Additional programs advancing toward the clinic, including AX-0811 and AX-0422
- Axiomer platform supporting multiple additional clinical data readouts within current runway

LEIDEN, Netherlands & CAMBRIDGE, Mass., April 8, 2026 – ProQR Therapeutics N.V. (Nasdaq: PRQR) (ProQR), a company dedicated to changing lives through transformative RNA therapies based on its proprietary Axiomer™ RNA editing technology platform, today highlighted key updates from its virtual Investor and Analyst event hosted today.

During the event, ProQR provided an overview of its RNA editing pipeline and development strategy, emphasizing its lead clinical program AX-0810, expansion of its pipeline with two new programs announced, and multiple clinical data readouts within its current runway.

“Today’s event highlighted the progress we are making across our pipeline and the strength of the Axiomer platform,” said Daniel A. de Boer, Founder and Chief Executive Officer of ProQR. “We look forward to the target engagement data with AX-0810 later this quarter and are excited about additional programs approaching the clinic, building a pipeline with multiple clinical readouts in the runway. At the same time, we are continuing to enhance our discovery capabilities, through AI-enabled and automated approaches, which are already supporting the advancement of next-generation programs.”

**AX-0810 Advancing Toward Target Engagement Data**

AX-0810, ProQR’s lead RNA editing program targeting NTCP, remains on track to report target engagement data from healthy volunteers in the first half of 2026. The company also announced the selection of biliary atresia as the initial indication for Phase 2 development, based on strong biological rationale, high unmet need, and the anticipated development path.

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## Pipeline Expansion

ProQR highlighted continued expansion of its pipeline, including:

- AX-0811, a next-generation NTCP program for cholestatic diseases generated by ProQR's AI-enabled discovery engine, with CTA filing expected in mid 2026 and initial clinical data anticipated by year-end 2026;
- AX-0422, targeting IDUA for Hurler syndrome, with CTA filing expected in early 2027 and initial clinical data anticipated in the first half of 2027;
- AX-2911, targeting PNPLA3 for MASH, advancing toward early clinical data generation with plans for a first-in-human (FIH) investigator-initiated trial (IIT) in China in H1 2027.

## Advancing the Axiomer Platform

ProQR also discussed continued advancement of the Axiomer platform, including the application of AI-enabled discovery and high-throughput screening supported by a partnership with Ginkgo Bioworks, to support the design and optimization of RNA editing therapeutics.

ProQR expects to deliver multiple clinical data readouts across its pipeline within its current cash runway, which extends into mid-2027.

A replay of the webcast and the presentation slides are available on ProQR's website, [www.proqr.com](http://www.proqr.com), under "Events".

## About Axiomer™

ProQR is pioneering a next-generation RNA base editing technology called Axiomer™, which could potentially yield a new class of medicines for diverse types of diseases. Axiomer™ "Editing Oligonucleotides", or EONs, mediate single nucleotide changes to RNA in a highly specific and targeted way using molecular machinery that is present in human cells called ADAR (Adenosine Deaminase Acting on RNA). Axiomer™ EONs are designed to recruit and direct endogenously expressed ADARs to change an Adenosine (A) to an Inosine (I) in the RNA – an Inosine is translated as a Guanosine (G) – correcting an RNA with a disease-causing mutation back to a normal (wild type) RNA, modulating protein expression, or altering a protein so that it will have a new function that helps prevent or treat disease.

## About ProQR

ProQR Therapeutics is dedicated to changing lives through the creation of transformative RNA therapies. ProQR is pioneering a next-generation RNA technology called Axiomer™, which uses a cell's own editing machinery called ADAR to make specific single nucleotide edits in RNA to reverse a mutation or modulate protein expression and could potentially yield a new class of medicines for both rare and prevalent diseases with unmet need. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind.

Learn more about ProQR at [www.proqr.com](http://www.proqr.com).

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## Forward Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "continue," "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Such forward-looking statements include, but are not limited to, statements regarding our business, technology, strategy, preclinical and clinical model data; our initial pipeline targets and the upcoming strategic priorities and milestones related thereto; our new pipeline targets, the continued advancement of our lead development pipeline programs, including approved, ongoing and planned clinical trials; expectations regarding the ongoing Phase 1 clinical study of AX-0810 in NTCP for cholestatic diseases; expectations regarding the safety and therapeutic benefits of AX-0810, including the planned dosing levels and their efficacy; the anticipated timing of initial Phase 1 clinical data for our lead program in healthy volunteers, AX-0810, in H1, 2026, and clinical updates across multiple programs in 2026; the anticipated development path in relation to the selection of biliary atresia as the initial indication for AX-0810 Phase 2 development; our new pipeline targets, including the planned Phase 1 clinical trial of AX-0811 in NTCP for cholestatic diseases, our ability to recruit for and complete a Phase 1 clinical trial for AX-0811, an anticipated CTA filing and the Phase 1b cohort 1 data readout for AX-0811 pending regulatory clearance, expectations regarding the efficacy, clinical development timeline, and expected trial designs and development of AX-0422 and AX-2911, including the potential CTA filings and data readout pending regulatory clearance; clinical updates across multiple programs in 2026 and 2027; the therapeutic potential and development timeline regarding AX-0810, AX-0811, AX-0422, AX-2911 and AX-2402; the anticipated benefits from our partnership with Ginkgo Bioworks; the continued development and advancement of our Axiomer™ platform; the therapeutic potential of our Axiomer RNA editing oligonucleotides and product candidates; the timing, progress and results of our preclinical studies and other development activities, including the release of data related thereto; our patent estate, including our anticipated strength and our continued investment in it; and the potential of our technologies and product candidates; and our cash runway. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Our actual results could differ materially from those expressed or implied by these forward-looking statements for many reasons, including, without limitation, the risks, uncertainties and other factors in our filings made with the Securities and Exchange Commission, including certain sections of our most recent annual report filed on Form 20-F. These risks and uncertainties include, among others, the cost, timing and results of preclinical studies and clinical trials and other development activities by us and our collaborative partners whose operations and activities may be slowed or halted shortage and pressure on supply and logistics on the global market, economic sanctions, U.S. government shutdown and international tariffs; the likelihood of our preclinical and clinical programs being initiated and executed on timelines provided and reliance on our contract research organizations and predictability of timely enrollment of subjects and patients to advance our clinical trials and maintain their own operations; our reliance on contract manufacturers to supply materials for research and development and the risk of supply interruption from a contract manufacturer; the potential for future data to alter initial and preliminary results of early-stage clinical trials; the unpredictability of the duration and results of the regulatory review of applications or clearances that are necessary to initiate and continue to advance and progress our clinical programs; the ability to secure, maintain and realize the intended benefits of collaborations with partners, including the collaboration with Lilly; the possible impairment of, inability to obtain, and costs to obtain intellectual property rights; possible safety or efficacy concerns that could emerge as new data are generated in research and development; general business, operational, financial and accounting risks, and risks related to litigation and disputes with third parties; and risks related to macroeconomic conditions and market volatility resulting from global economic developments, geopolitical events and conflicts, high inflation, rising interest rates, tariffs and potential for significant changes in U.S. policies and regulatory environment. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future, except as required by law.

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