ADARx PHARMACEUTICALS



Focused on Developing Next-Generation siRNA Therapeutics Designed to Treat Broad Spectrum of Diseases

September 2026

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This presentation and the accompanying oral commentary contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended.

All statements contained in this presentation and the accompanying oral commentary, other than statements of historical facts, are forward-looking statements, including statements regarding the potential benefits, effectiveness and safety of our product candidates and technology platforms; the design, progress and results of our research and development programs, preclinical studies and clinical trials; our anticipated clinical and regulatory milestones, data readouts, filings and trial initiations; target product profiles of our product candidates, including anticipated efficacy, dosing regimens, and therapeutic windows; the translatability and predictability of our preclinical results and clinical data; the competitive positioning of and market opportunities for our product candidates; our collaboration and commercialization efforts, our business strategies, plans and objectives; and our anticipated growth, operating results and financial performance. These statements involve substantial known and unknown risks, uncertainties and other factors that may cause our actual results, timing of results, levels of activity, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements. Forward-looking statements may be identified by words such as “anticipate,” “believe,” “continue,” “could,” “design,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “project,” “should,” “target,” “will,” “would” or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words.

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ADARx Pharmaceuticals

Developing next-generation siRNA therapeutics designed to treat a broad spectrum of diseases

BROAD siRNA PIPELINE

- **5 wholly-owned, internally generated clinical + advanced preclinical programs** for chronic diseases

ADARx siRNA CANDIDATES W/ KEY DESIGN FEATURES

- **Designed for Potency, precision and durability**
- Focus on targets where deep & durable suppression can lead to meaningful clinical benefit

PROPRIETARY TECHNOLOGY

- **Core technologies** developed to optimize siRNA sequences and targeted tissue delivery
- **Reproducibility demonstrated across multiple clinical programs,** potentially enables scalable, efficient pipeline expansion



ADVANCING THE POTENTIAL OF siRNA

- **Hepatic: designed to increase depth and durability of clinical response**
- **Extrahepatic**: ability to **selectively target 6 tissue types outside the liver**

KEY COLLABORATION

AbbVie collaboration in neuroscience, immunology, oncology; **$335M** upfront and several billion in potential milestones + royalties

abbvie | ADARx PHARMACEUTICALS

EXPERIENCED LEADERSHIP

- **siRNA therapeutic development pioneers** on leadership team
- Supported by broad group of blue chip investors

ADARx PHARMACEUTICALS

Cash, cash equivalents and short-term investments of ~$427 million as of June 30, 2026

Shared Vision of Building Next-Generation siRNA Therapeutics Company

Management team with deep experience in RNAi medicine



Zhen Li, PhD
President and
Chief Executive Officer



Ryan Fisk, MBA
Chief Financial Officer and
Chief Business Officer



A. Robert MacLeod, PhD
Chief Scientific Officer



Rui Zhu, PhD
Chief Technology Officer



Rob Ackles
Chief People Officer



Christopher Claeboe, PhD, MBA
Senior Vice President
of Operations



Jiang Bian, JD
Vice President, Head of Legal

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Board of Directors

Zhen Li, PhD
President & CEO

Erez Chimovits, MBA
Partner, OrbiMed

Ting Feng, PhD
Partner, Sirona Capital

Simeon George, MD, MBA
CEO & Managing Partner, SR One

Carl L. Gordon, PhD, CFA
Managing Partner, OrbiMed

Ying Huang, PhD
Former CEO, Legend Biotech

Judith J. Li, MBA
Partner, Lilly Asia Ventures

Ricky Sun, PhD, MBA
Partner, Bain Capital Life Sciences

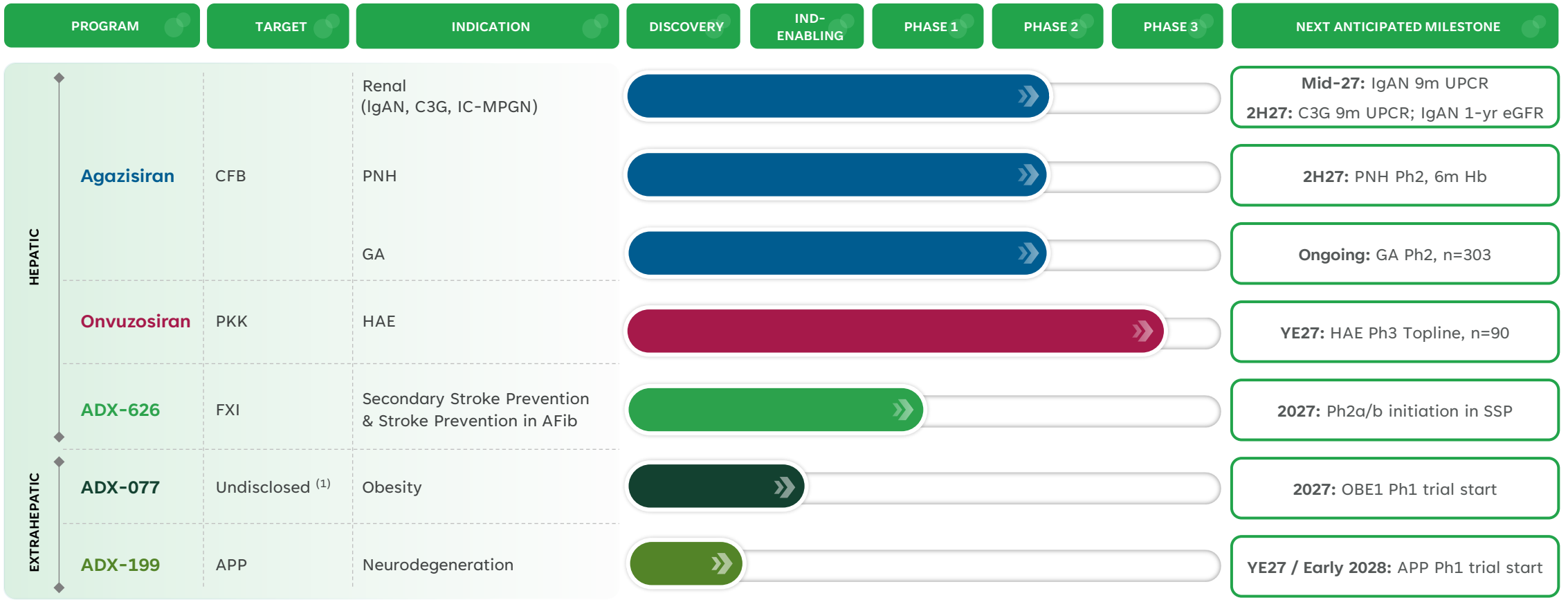
Mary Tagliaferri, MD
CMO & SVP, Nektar Therapeutics

Chen Yu, MD, MBA
Founding Managing Partner, TCGX

$352.5M raised to date from blue chip biotech investors

Investors

ADARx Pipeline of Next-Generation siRNA Programs



1) A novel, undisclosed target in adipose tissue, which we refer to as "OBE1", whose reduction in expression has been shown to reduce adiposity, with the objective to reduce body weight and fat while preserving lean mass. For additional details, see the section titled "ADX-077—siRNA preclinical therapeutic candidate for obesity via a novel undisclosed target in adipose tissue"

AFib = Atrial Fibrillation; APP = Amyloid-β Precursor Protein; C3G = Complement 3 Glomerulopathy; CFB = Complement Factor B; eGFR = Estimated Glomerular Filtration Rate; FXI = Factor XI; GA = Geographic Atrophy; HAE = Hereditary Angioedema; Hb = Hemoglobin; IC-MPGN = Immune Complex-Mediated Membranoproliferative Glomerulonephritis; IgAN = IgA Nephropathy; PKK = Prekallikrein; PNH = Paroxysmal Nocturnal Hemoglobinuria; SSP = Secondary Stroke Prevention; UPCR = Urine Protein-Creatinine Ratio

abbvie *Collaboration and Option*

Discovery-stage extrahepatic programs leveraging ADARx's RNA discovery expertise and siRNA technology across indications in neuroscience, immunology and oncology

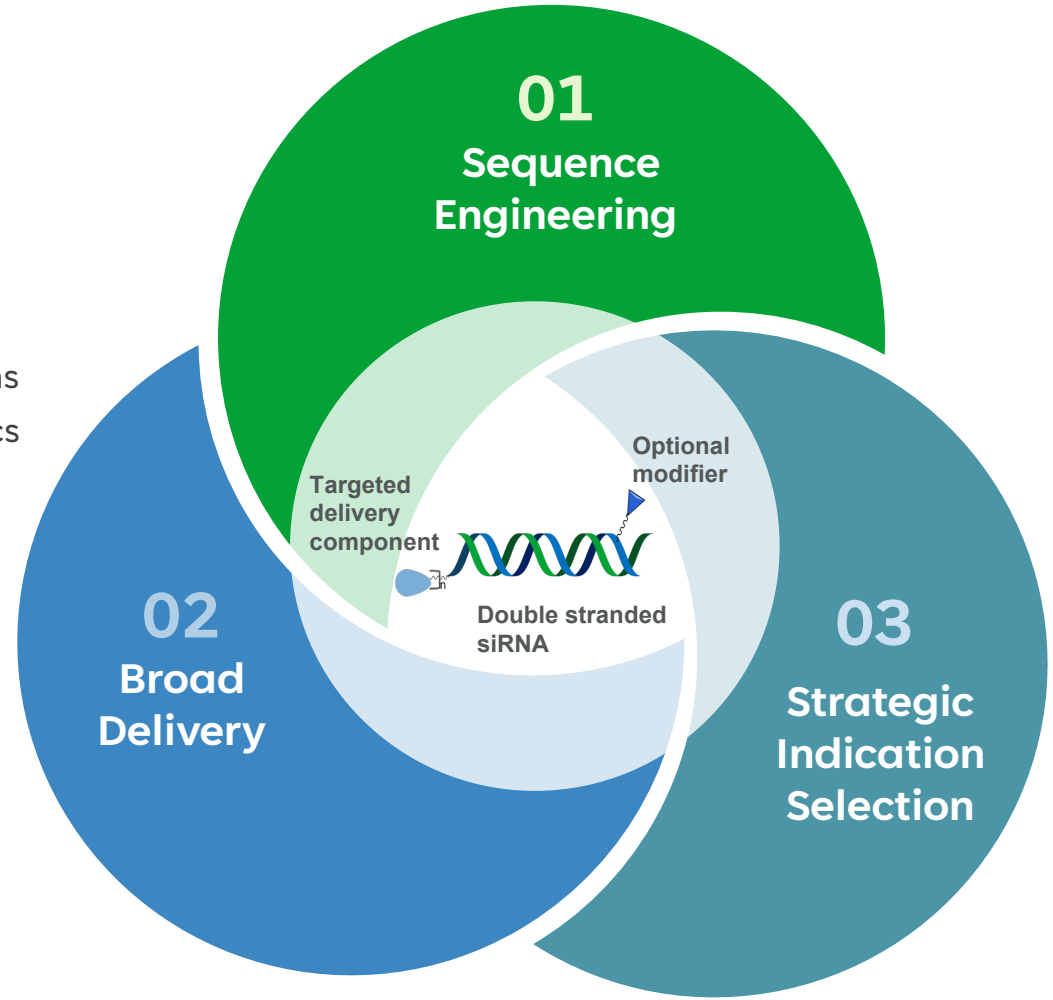
ADARx PHARMACEUTICALS

ADARx Differentiation: Technology and Strategy

Core proprietary technologies developed to optimize our siRNA sequences and delivery to both hepatic & extrahepatic tissues

- MST™ designed with the goal of consistently achieving highest possible inherent potency and durability of siRNA sequences
  - Powered by unique insights into oligonucleotide–enzyme interactions
  - Tuning kinetics and thermodynamics through rational design
  - Enabled by proprietary computational platform

- CTD™ Platform unlocking extrahepatic tissue access
  - Designed to enable targeted delivery beyond the liver



- Prioritizing chronic diseases where MST/CTD-enabled, deep & durable target suppression could yield meaningful clinical benefit
  - Prudent stewards of capital focusing pipeline on potentially transformative outcomes

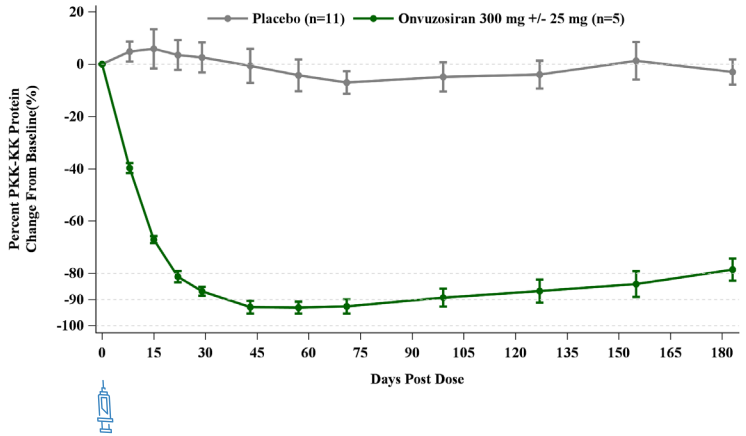
MST = mRNA Silencing Technology; CTD = Cell Targeted Delivery

Reproducibility Demonstrated Across Multiple Clinical Programs

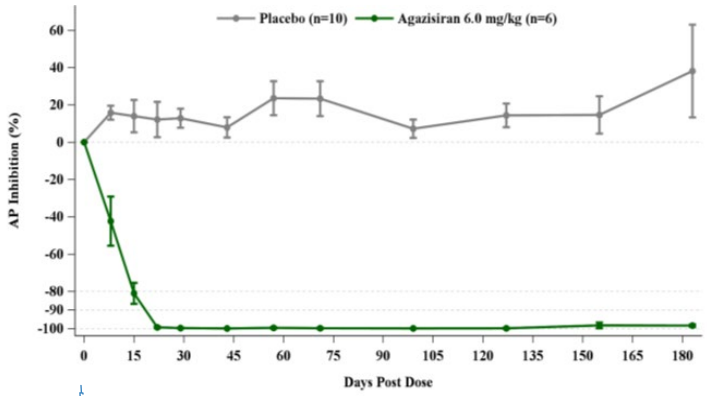
Observed quality, reproducibility and speed potentially enables scalable & efficient pipeline expansion

CLINICAL DATA

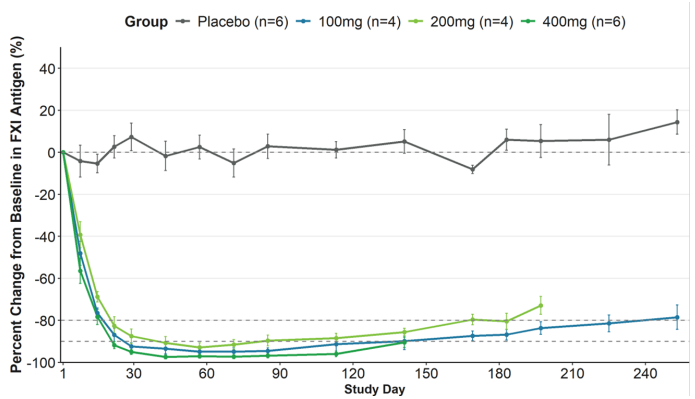
Onvuzosiran
PKK



Agazisiran
CFB



ADX-626
FXI



Quality Clinical Data Observed to Have:

- Precise, deep and durable activity
- Well-tolerated across completed clinical studies

Observed Reproducibility

- Platform has delivered consistent performance in clinical studies

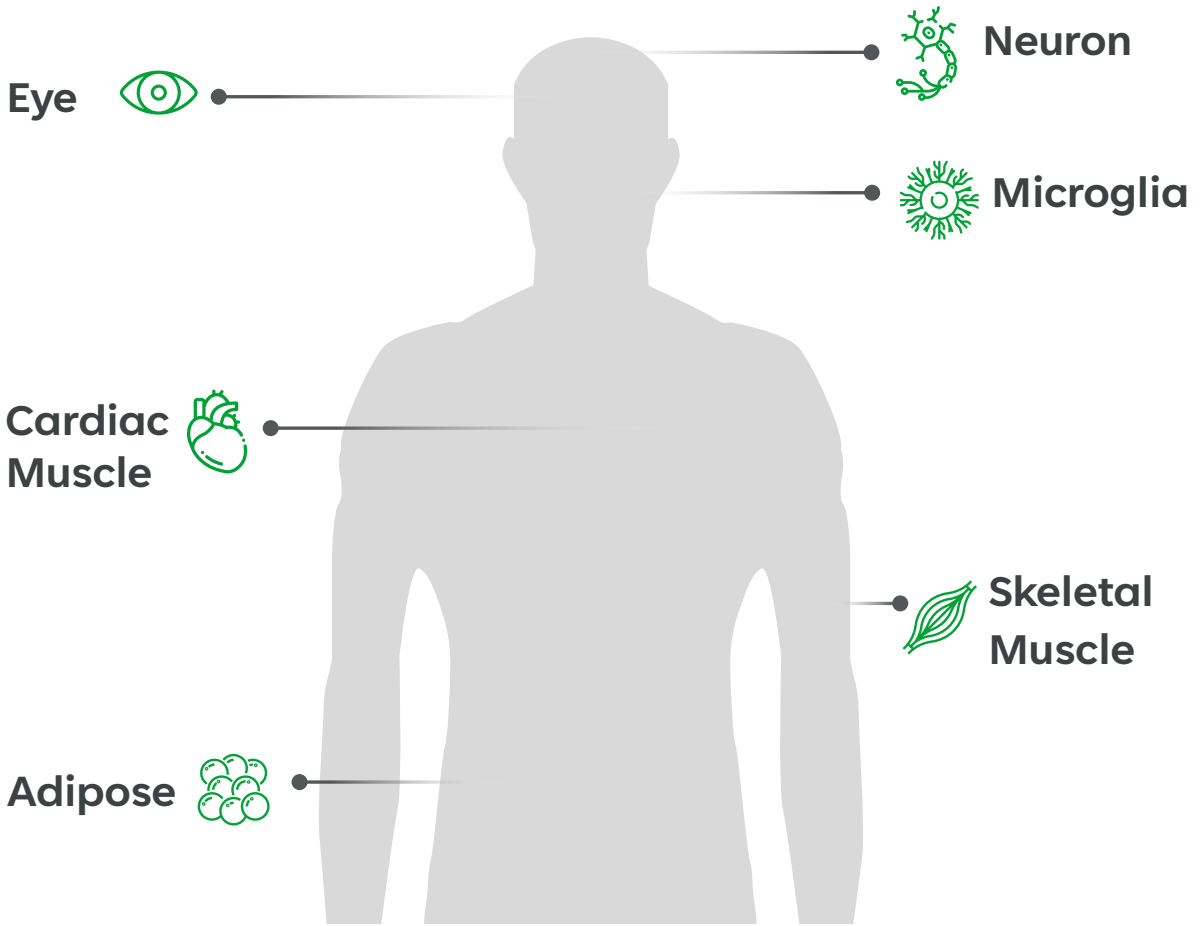
Capital-Efficiency & Speed

- Therapeutic candidates have progressed from discovery to development candidate nomination in as little as 9 months

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Unlocking Extrahepatic Tissue Access for siRNA May Drive Future Pipeline Expansion

ADARx technology has Shown the ability to selectively target six extrahepatic tissues



ADARx strategic focus developed to overcome siRNA limitations:

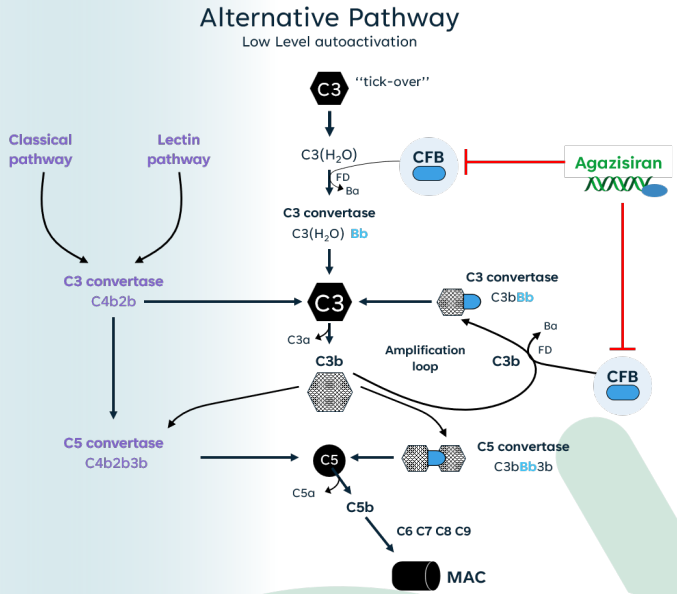
- ***Extrahepatic-targeted***: Designed to overcome key barriers to extrahepatic targeting:
  - Deliver to the target tissue / cell type
  - Design & engineer a highly potent, efficient siRNA payload
- ***Hepatic-targeted***: Designed to increase depth and durability of response of hepatic tissue-directed therapies
- ***Bispecific approaches***: Developing bispecific-siRNA technology designed to enable the suppression of multiple disease-causing proteins simultaneously with a single construct

ADARx PHARMACEUTICALS

Agazisiran

A potent long-acting siRNA silencing complement factor B (CFB) mRNA for the treatment of multiple complement-mediated diseases

Agazisiran Has Pipeline-in-a-Product Potential with Three Ph2 Trials Ongoing



Adapted from: Vivarelli 2024

Encouraging clinical data

- We believe FDA's approval of the complement factor B (CFB) inhibitor FABHALTA® validates CFB as a target in the complement pathway, which is implicated in the pathophysiology of a broad range of complement-mediated diseases
- Designed to suppress liver-derived CFB to selectively inhibit AP activation
- Participants in a Phase 1 trial who were administered a single SC dose of agazisiran observed to have up to 99.9% AP activity suppression for 6 months
- Well-tolerated with doses up to 6.0mg/kg, no treatment-related serious adverse events (SAEs) in Phase 1[1]

Target profile*

- SC administered with Q3M and Q6M targeted dosing intervals
- Pipeline-in-a-product potential to treat (1) renal diseases (IgAN, C3G, IC-MPGN); (2) PNH; and (3) GA secondary to AMD
- Generally well-tolerated with no treatment-related SAEs

Large market opportunities

- IgAN: ~200K WW incidents per year; ~210K affected individuals in the US and ~190K in Europe
- C3G/IC-MPGN: ~1,750 patients in US, similar number for IC-MPGN patients; Our competitors have reported approximately three times higher C3G patient numbers based on epidemiology research
- PNH: ~100K to ~300K patients WW; Most face a lengthy path to diagnosis, and a majority diagnosed do not receive complement inhibitor therapy
- GA: ~5M patients WW

Multiple Phase 2 trials ongoing

- Multiple Phase 2 trials ongoing to evaluate the efficacy and safety of agazisiran in (1) IgAN, C3G and IC-MPGN, (2) PNH and (3) GA secondary to AMD
- Meaningful cadence of data anticipated in 2027 and 2028

*Target product profile is provided for illustrative purposes only, and may not be achieved in clinical development

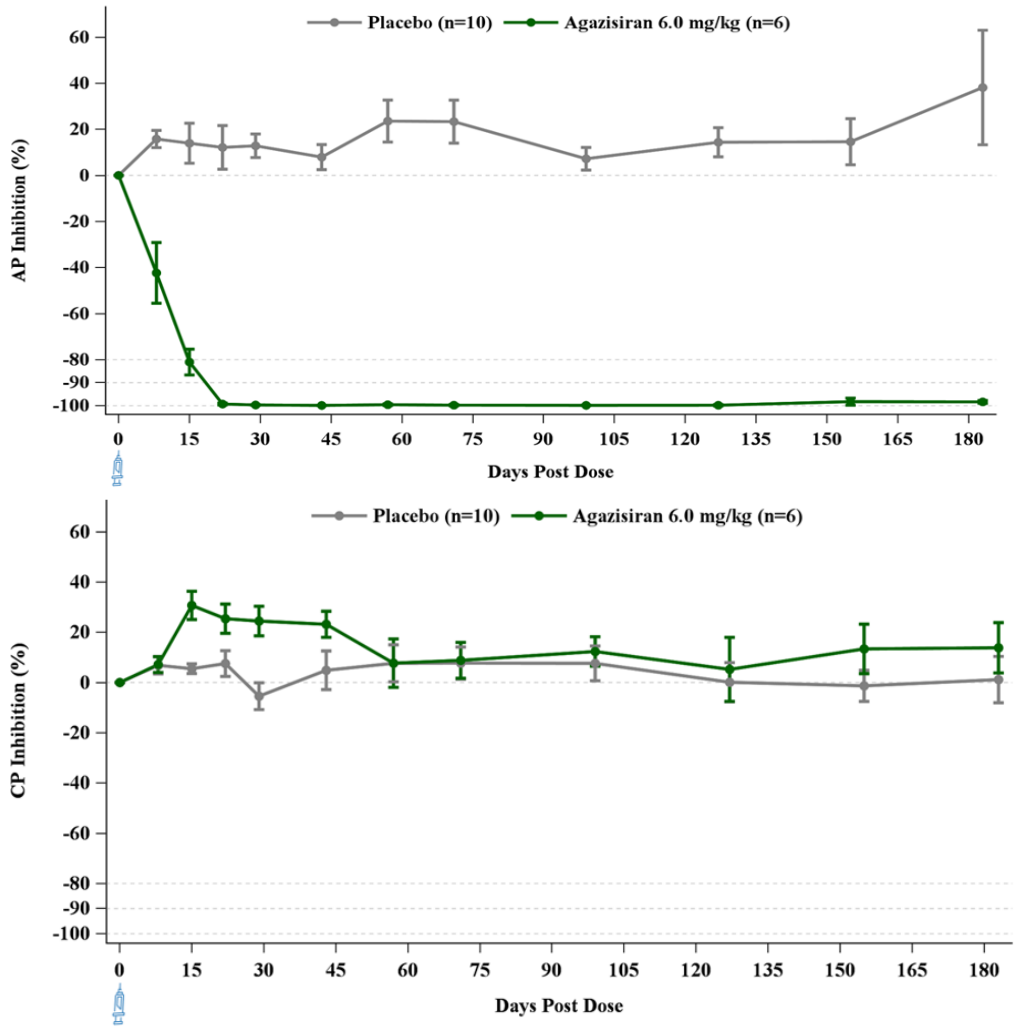
AMD = age-related macular degeneration; AP = Alternative Pathway; C3G = Complement 3 Glomerulopathy; CFB = Complement Factor B; GA = Geographic Atrophy; IC-MPGN = Immune Complex-Mediated Membranoproliferative Glomerulonephritis; IgAN = IgA Nephropathy; PNH = Paroxysmal Nocturnal Hemoglobinuria

1) As of the June 8, 2026 safety cutoff date

Agazisiran Ph1 Data Supports Potential for Both Duration & Depth of AP Activity Inhibition

- At 6.0 mg/kg, we observed near-complete inhibition of AP after a single SC injection of agazisiran
  - A mean inhibition of 99.9% of the complement AP activity was achieved at nadir
  - Suppression sustained for at least 6 months with a mean reduction of 98.3% and 93.5% through 6mo in AP & CFB
  - No observed impact on the complement CP activity
- No infections due to encapsulated organisms reported
- Well-tolerated with doses up to 6.0 mg/kg, no treatment-related serious adverse events (SAEs) in Phase 1*

Clinical Phase 1 data



Agazisiran effects on AP activity, and CP activity in healthy participants at 6 mg/kg

* As of the June 8, 2026 safety cutoff date

Agazisiran Clinical Development Program: Multiple Phase 2 Trials Ongoing with Cadence of Data Anticipated in 2027 and 2028

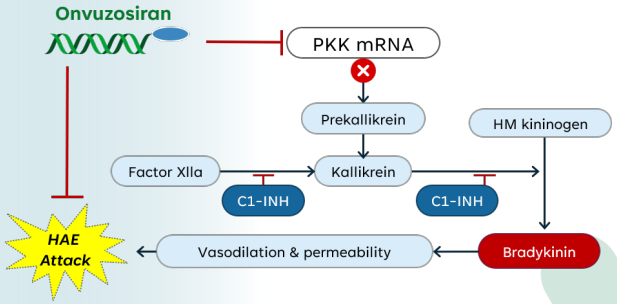
Program	Trial Design	Protocol Summary	Endpoints
Renal Diseases (IgAN, C3G, IC-MPGN)	• Global, multi-center, open-label multiple dose Phase 2 POC (N=45) • Evaluates SC agazisiran across IgAN, C3G and IC-MPGN	IgAN: Agazisiran 300 mg Q3M N=15 IgAN: Agazisiran 400 mg Q6M N=15 C3G or IC-MPGN: Agazisiran 300 mg Q3M N=15	• **Primary:** safety and tolerability • **Key Secondary:** change from baseline in UPCR • **Exploratory Endpoint:** change from baseline in eGFR over time
	• China Phase 2 trial evaluating 400 mg SC Q6M dosing regimen in IgAN (N=30) • Includes agazisiran monotherapy and in combination with an APRIL / BAFF inhibitor	IgAN: Agazisiran 400 mg Q6M N=15 IgAN: Agazisiran 400 mg Q6M + APRIL/BAFF N=15	• **Primary:** safety and tolerability • **Key Secondary:** change from baseline in UPCR • **Exploratory Endpoint:** change from baseline in eGFR over time
PNH	• Phase 2 study in patients with PNH and residual anemia • Evaluates safety and tolerability of SC Agazisiran dosed every three months • Two cohorts	PNH: Agazisiran 300 mg Q3M N = Up to 24	• **Primary:** safety and tolerability • **Key Secondary:** hemoglobin, LDH, plasma CFB and AP activity
GA	• Randomized, masked, placebo-controlled Phase 2 study in GA secondary to AMD (N=303) • Evaluates SC agazisiran versus placebo over a 24-month treatment / follow-up period	Agazisiran: 400 mg Q3M up to 1 year and Q6M up to 18 months N=202 Placebo: Q3M up to 1 year and Q6M up to 18 months N=101	• **Primary:** slope of GA area change by fundus autofluorescence from baseline to Month 12 • **Key Secondary:** GA progression through Month 24; retinal structural measures

ADARx PHARMACEUTICALS

Onvuzosiran

A potent long-acting siRNA silencing PKK mRNA for the prevention of HAE attacks

Onvuzosiran Targeting >75% Attack-free Rate with Q6M/Q3M Dosing



The bradykinin cascade and its role in HAE*

*Adapted from: Farkas & Varga. (2011). Clinical, cosmetic and investigational dermatology. 4. 61-8.

Encouraging clinical data

- Onvuzosiran is designed to selectively target and degrade PKK mRNA in the liver and prevent protein synthesis, thereby reducing PKK protein levels and preventing activation of bradykinin
- In Phase 1 at 6 mg/kg, we observed that a single SC injection reduced circulating PKK/KK levels by a mean of 94% at nadir and maintained 84% mean suppression at 6 months, with no treatment-related SAEs[1]
- In Phase 2a, all participants remained attack-free for 3 months following the initial dose, with one patient remaining attack-free for 18 consecutive months on Q6M dosing after one mild attack at Day 153[2]

Target profile*

- Onvuzosiran Target Product Profile*:
  - >90% reduction in monthly attacks
  - >75% patients attack-free
  - Q6M or Q3M SC dosing to minimize treatment burden
  - Well-tolerated, with favorable safety profile

Market opportunity

- HAE affects ~1 in 50,000 people worldwide
- HAE market, including acute and prophylactic treatment, was ~$3.1 billion in 2024 and is estimated to reach ~$6.8 billion WW by 2032,[3] with prophylactic treatment representing a significant majority

Phase 3 STOP-HAE trial ongoing

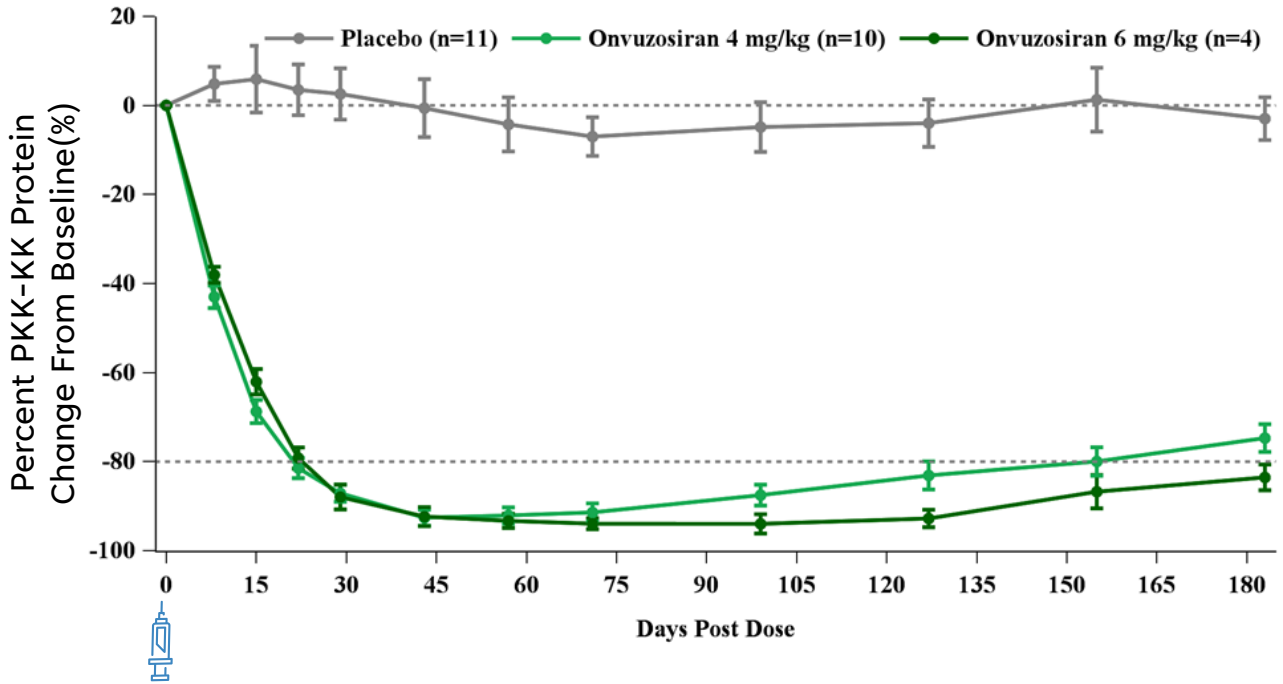
- A Phase 3, randomized, double-blind, placebo-controlled study at 300mg Q6M and 240mg Q3M is ongoing
- Topline data anticipated by YE27

*Target product profile is provided for illustrative purposes only, and may not be achieved in clinical development
PKK = Prekallikrein
1) As of the August 5, 2026 safety cutoff date; 2) As of the August 19, 2026 data cutoff date; 3) Evaluate Pharma June 9, 2026

ADARx PHARMACEUTICALS

Onvuzosiran Observed Deep and Durable Target Knockdown

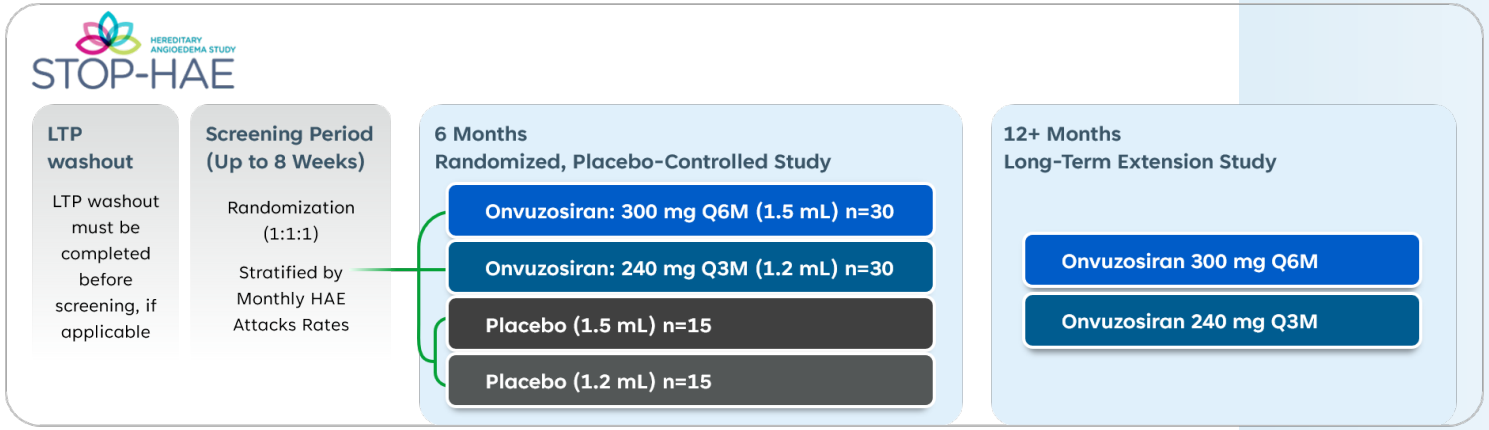
Phase 1 clinical data in healthy volunteers at select dose cohorts



- At the 6 mg/kg dose level, we observed that a single SC injection reduced circulating PKK/KK levels by a mean of 94% at nadir and maintained 84% mean suppression at 6 months
- Three of the four participants reached below LLOQ* for PKK/KK levels at nadir
- Well-tolerated with doses up to 6.0 mg/kg, no treatment-related SAEs[1]

LLOQ: Lower Limit of Quantification
1) As of the August 5, 2026 safety cutoff date

STOP-HAE: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Onvuzosiran Targeting Prekallikrein in Subjects With Hereditary Angioedema



Patient Population

- HAE Type I or Type II
- ≥18 years old
- ≥1 HAE attack in the first 4 weeks of Screening or ≥2 HAE attacks in 8 weeks of Screening,
- ≥ 3 attacks anytime during screening allows direct randomization
- Access to acute medication(s) for attacks

Primary Endpoint

- Monthly HAE attacks rate in 300mg Q6M vs PBO

Key Secondary Endpoints

- Monthly HAE attacks required acute therapy in onvuzosiran vs PBO
- Monthly moderate to severe HAE attacks in onvuzosiran vs PBO
- % Attack free in 6M in onvuzosiran

Exploratory Endpoints

- Safety and tolerability
- PK/PD
- AE-CT/AE-QoL

Visits

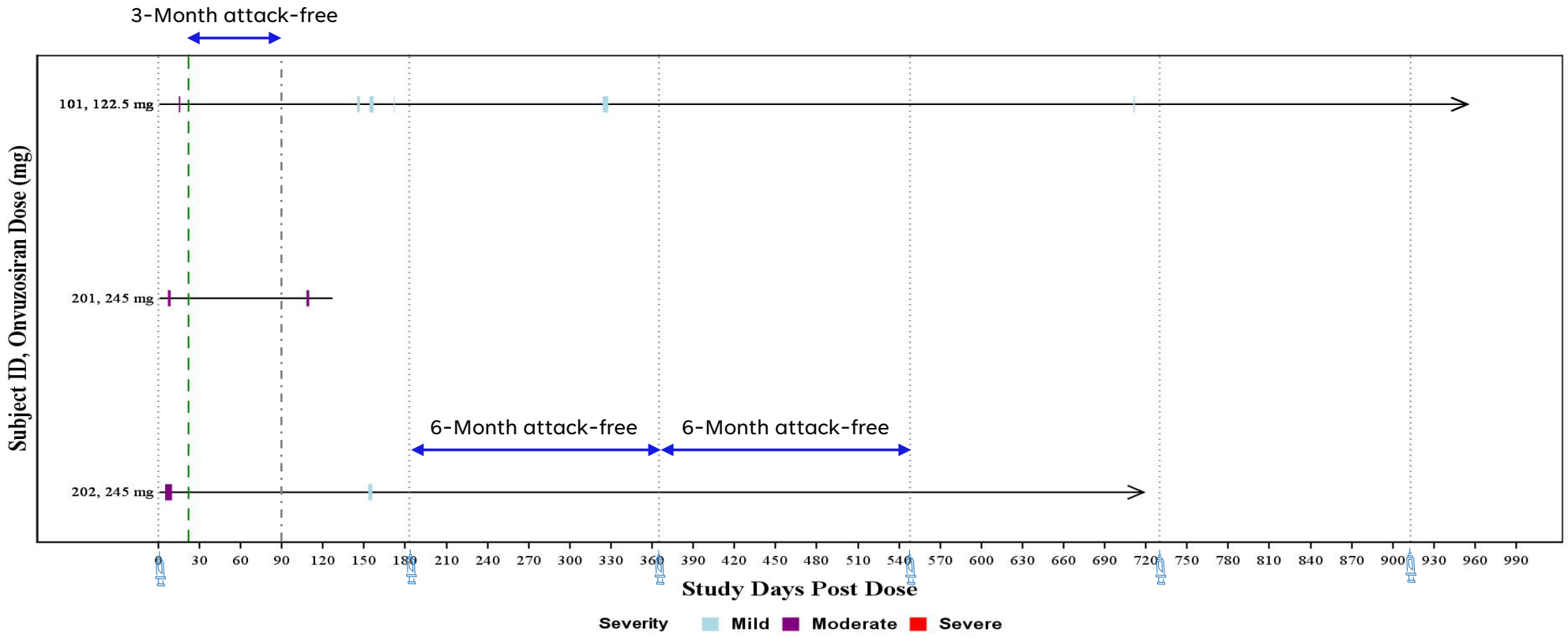
- Screening, Day 1 (Dose 1), w3, w5, w9, w13(Dose 2), w17, w21, w25(EOS)

- Phase 3 global study ongoing
- 300 mg Q6M Cohort:
  - Phase 1 support: 94% mean (88-97%) reduction at nadir, 84% mean (78-92%) reduction at 6mo at 300 mg +/-25 mg Q6M
- 240 mg Q3M Cohort:
  - Observed 100% Q3M attack-free rate in Phase 2a after the first dose[1]
    - Achieving attack control after the initial dose is generally considered the most challenging phase of treatment
    - Both our data and broader industry experience[2] suggest that attack-free rates improve with subsequent dosing

STOP-HAE topline data expected by YE2027

1) Interim data as of the August 19, 2026 data cutoff date
2) J ALLERGY CLIN IMMUNOL PRACT, Volume 13, Issue 2 p378-387.E2 February 2025

Onvuzosiran: Interim Phase 2a Results



- Phase 2a: Dosed at Q6M, all were doses either significantly lower than in Phase 3 or less frequent (*Phase 3 dose cohorts: 300 mg Q6M or 240 mg Q3M*)
- All three patients (3/3) were attack-free for 3 months after the initial dose (starting from Day 22*), supporting that patients on 240 mg Q3M cohort will potentially achieve attack-free status
- No attacks observed when PKK & KK reduction >80%
- Patient 202 (245mg, Q6M) remained attack-free for a consecutive 18 months after one mild attack at Day 153
- Generally well-tolerated, consistent with the safety results observed in Phase 1, with no SAEs reported in participants dosed with onvuzosiran

ADARx PHARMACEUTICALS

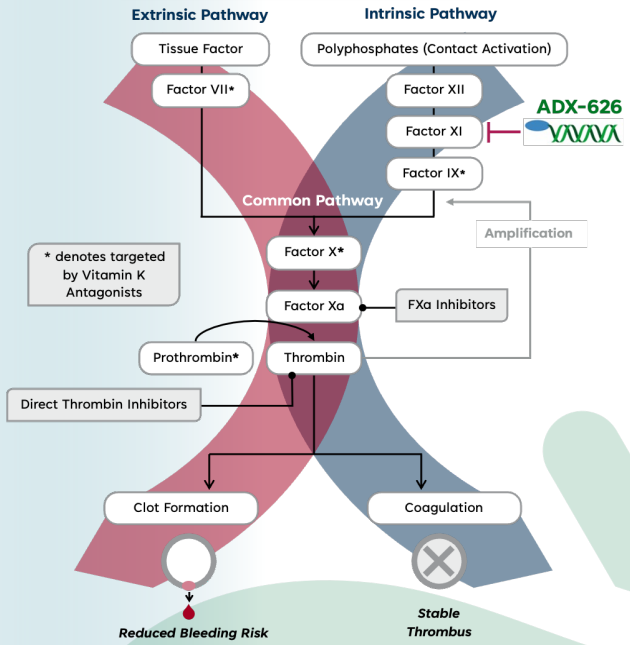
Interim data as of the August 19, 2026 cutoff date

* Per feedback from FDA, for onvuzosiran Phase 3 study, HAE attacks before Day 22 will be excluded from primary endpoint analysis, consistent with the siRNA proposed mechanism of action.

ADX-626

A potent long-acting siRNA silencing coagulation Factor XI (FXI) for secondary stroke prevention with additional clinical potential for stroke prevention in atrial fibrillation

ADX-626 is a Potent & Long-acting siRNA Targeting FXI in Development for SSP and AFib



Mechanism of antithrombotic activity without bleeding risk with FXI inhibitors compared to other anticoagulants

Genetically & clinical validated target

- FXI is a key component of the intrinsic coagulation pathway
  - Plays an important role in thrombus propagation/growth
  - Less critical for the initial clot formation that largely depends on the extrinsic pathway and platelet function
- Third-party Phase 3 asundexian trial provides POC for targeting FXI for secondary stroke prevention
- We believe this supports the potential to achieve antithrombotic activity with low risk of bleeding

Encouraging clinical data

- In an ongoing Phase 1 study, after single SC dose of ADX-626, observed a mean reduction of 97.4% and 95.4% at nadir in FXI protein and FXI activity, respectively[1]; continuing to explore higher doses
- Well-tolerated with no SAEs[2]

Target profile*

- Deep and sustained FXI inhibition with infrequent SC administration
- Well-tolerated with favorable safety profile

Large unmet need

- SSP: ~ 12M stroke patients every year: ~6M non-cardioembolic ischemic strokes, 20%-30% will suffer a recurrent event
- AFib: ~12M patients in US and ~60M WW; ~35% will have a stroke at some point in their life

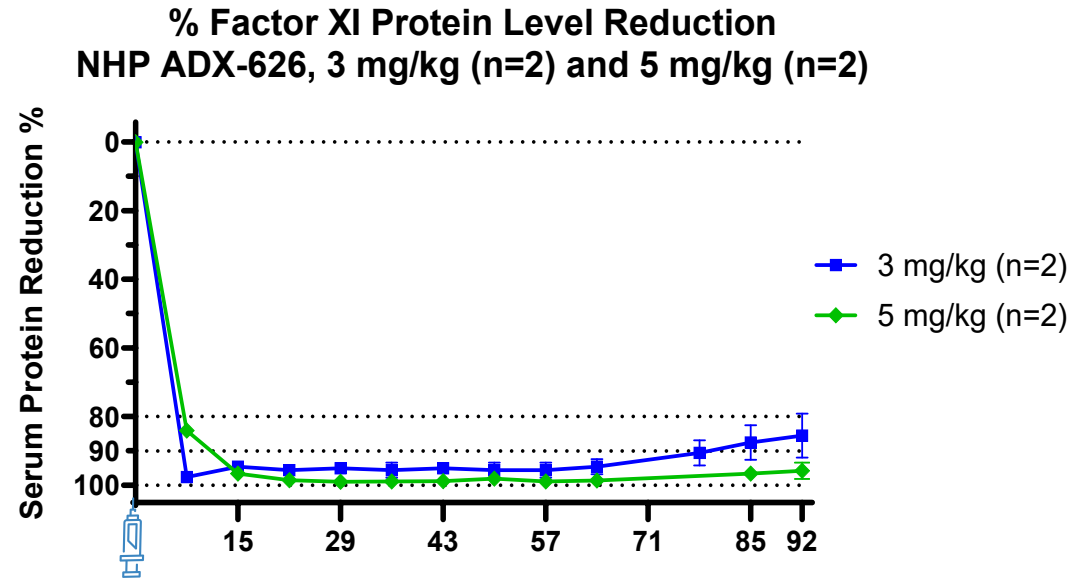
Phase 2 planning underway

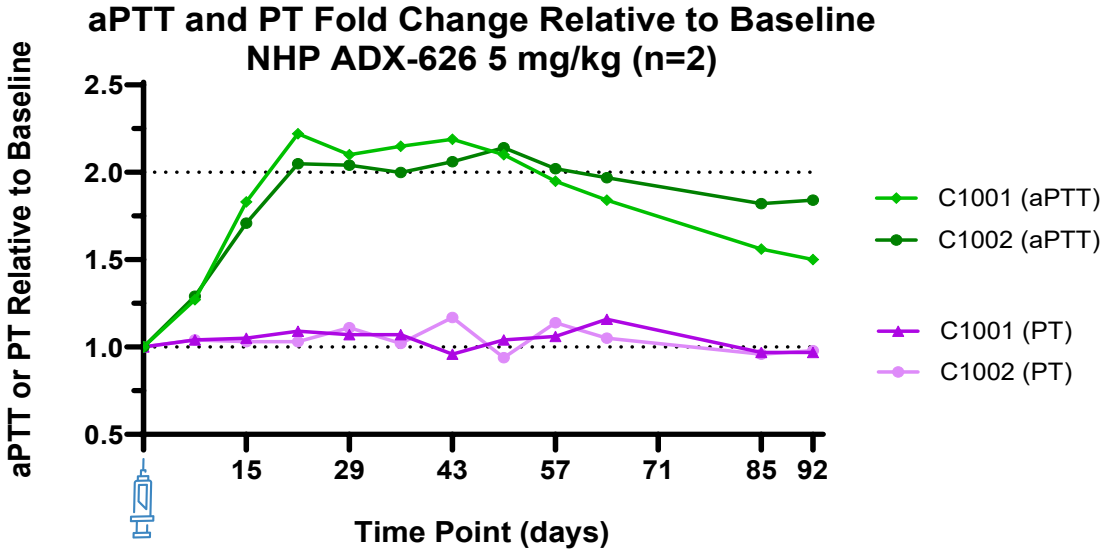
- Intend to pursue Phase 2 development in secondary stroke prevention, as well as exploratory studies in stroke prevention in atrial fibrillation patients
- Plan to initiate a Phase 2a/b clinical trial in SSP in 2027, with Phase 2a 3-month PD data expected by YE2027

*Target product profile is provided for illustrative purposes only, and may not be achieved in clinical development
SSP = Secondary Stroke Prevention; AFib = Atrial Fibrillation
1) Based on data received as of August 12, 2026; 2) As of the June 16, 2026 safety cutoff date

ADX-626 NHP Data: Observed that a Single SC Injection Demonstrated Deep and Sustained FXI Protein Suppression in NHP Study

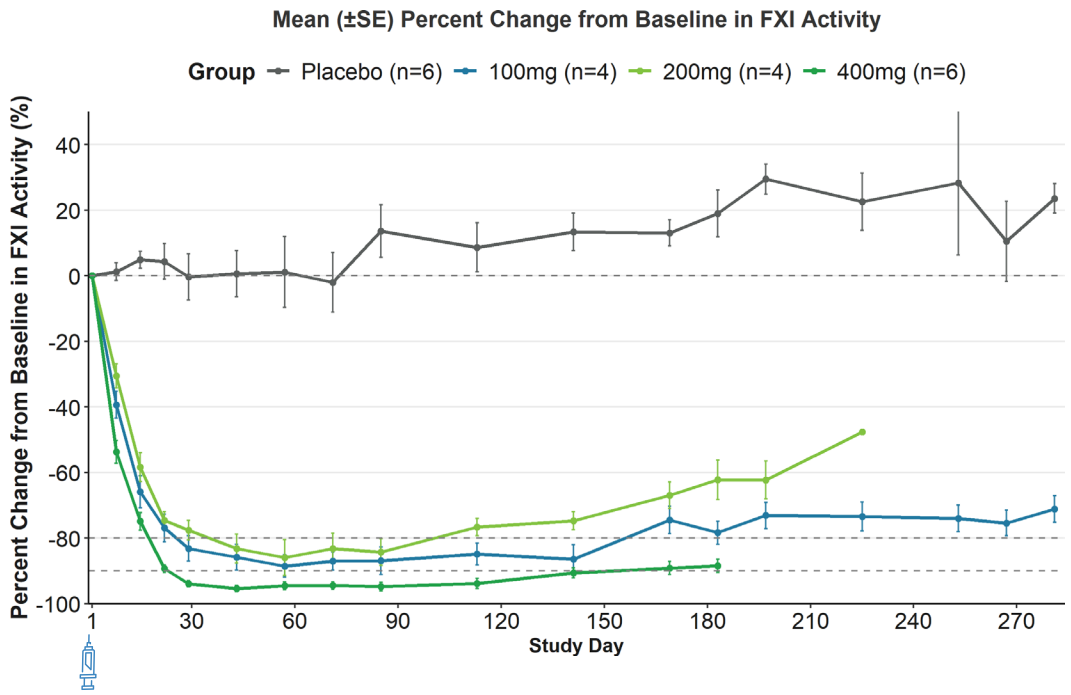
- **Single SC dose** achieved **near-complete, sustained reduction** of FXI protein in NHP (up to 99.6% reduction of FXI protein at Day 29 and maintained >95% reduction of FXI protein through Day 92)
- **Consistent >2-fold increase in intrinsic coagulation pathway activity**, supporting criticality of deep FXI suppression
- **No effect observed on prothrombin time (PT)**
  - PT increases have been linked to bleeding risk
- **GLP tox study completed**; top dose (100mg/kg) established as **NOAEL**

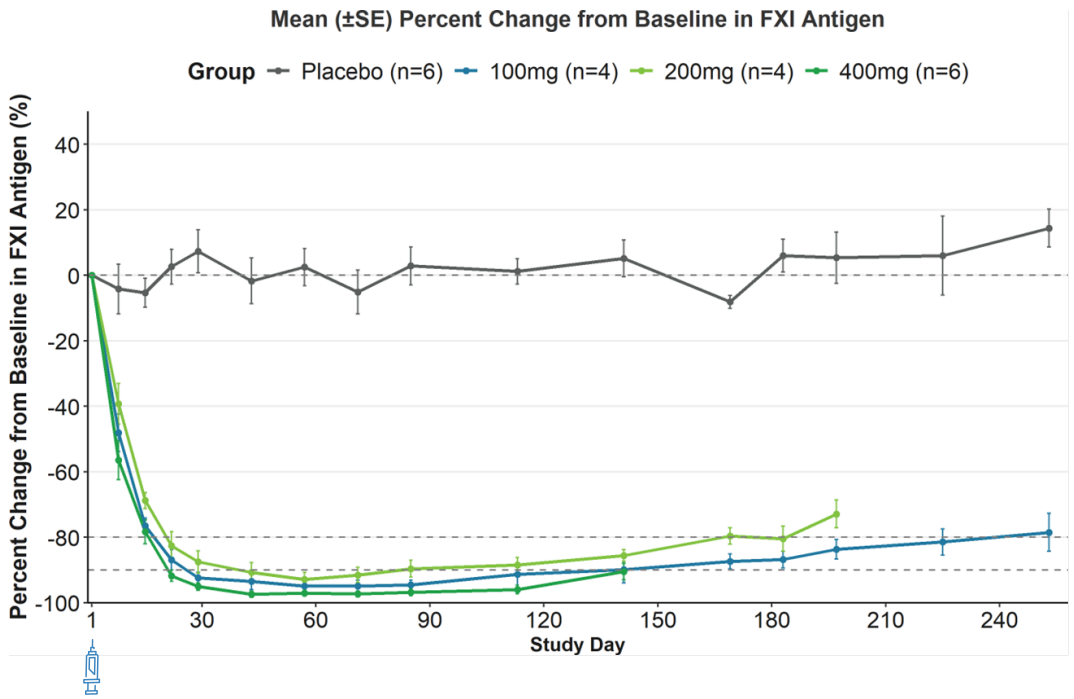




ADARx PHARMACEUTICALS

ADX-626: Deep and Durable Target Reduction Observed in Ongoing Phase 1 Study[1]





- Deep target reduction: We observed that a single SC dose at 400 mg dose reduced the mean of 97.4% and 95.4% at nadir in FXI protein and FXI activity, respectively (Four of the six participants were below LLOQ for FXI activity)
- Long duration: Achieved long duration with potential Q6M dosing
- Well-tolerated with no SAEs[2]
- Continuing to explore higher doses and intend to pursue Phase 2 development in secondary stroke prevention, as well as exploratory studies in stroke prevention in atrial fibrillation patients

ADARx PHARMACEUTICALS

1) Based on data received as of August 12, 2026; 2) As of the June 16, 2026 safety cutoff date

ADX-077

siRNA preclinical therapeutic candidate for obesity via a novel undisclosed target in adipose tissue

ADX-077 Designed to Selectively Inhibit a Novel Target in Adipose Tissue with Encouraging Weight Loss Data in Obese NHPs

Novel approach targeting weight loss with preservation of lean mass

- ADX-077 was designed to selectively inhibit an undisclosed target ("OBE1") in adipose tissue
- OBE1 reduces adiposity through mechanism distinct from appetite suppression associated with GLP-1 receptor agonism
- Observed meaningful body weight reduction in obese NHP and DIO mice while preserving lean mass

Target profile*

- Significant weight reduction with minimal impact on lean mass
- Infrequent dosing intervals
- Well-tolerated with favorable safety profile

Large unmet need

- Obesity remains a leading factor for chronic disease and affects 890M+ adults worldwide
- Despite the increasing use of GLP-1 based therapies, clinical limitations include:
  - Lean mass loss
  - Tolerability constraints
  - Frequent dosing
  - High discontinuation rates

IND-enabling stage

- Manufacturing clinical supplies to support our FIH study is ongoing
- Plan to initiate Phase 1 clinical trial in 2027, subject to receipt of regulatory clearance
- Initial clinical data from this planned Phase 1 clinical trial are anticipated in 2028

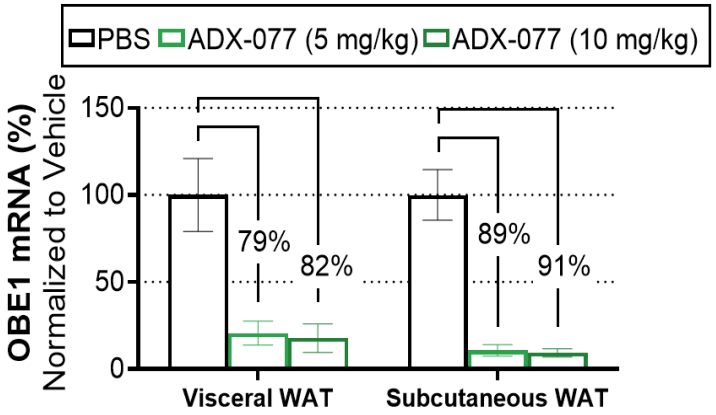
ADARx PHARMACEUTICALS

*Target product profile is provided for illustrative purposes only, and may not be achieved in clinical development

Observed Deep, Durable Fat Loss With Lean Muscle Preserved in DIO Mice

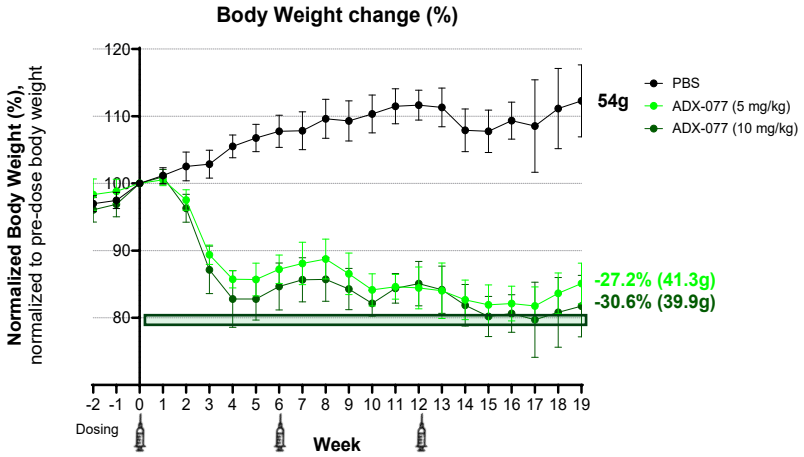
Deep target mRNA knockdown

OBE1 mRNA Knockdown in Adipose



Meaningful durable weight loss (Q6W SC)

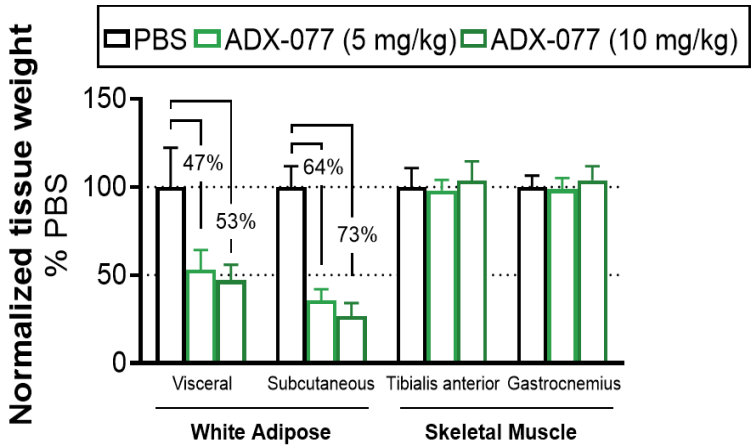
Percent Body Weight



*Mean body weight of mice on a normal diet at week 40 is 41.2 ± 4.5 g

Robust fat reduction, observed without lean mass loss

White Adipose and Skeletal Muscle



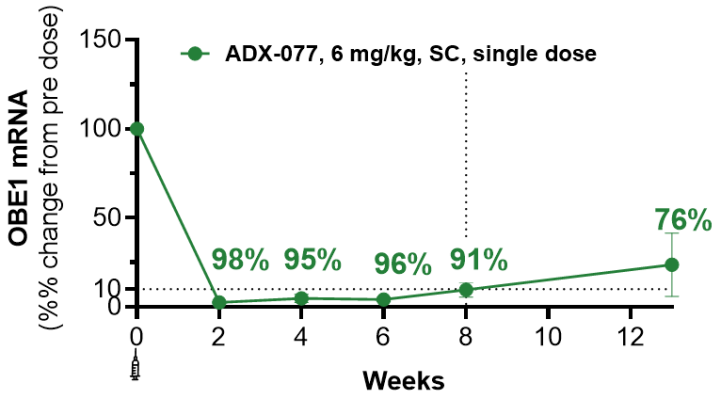
- Observed deep target mRNA reduction
- Durable weight loss observed
- Meaningful and durable reduction observed in both visceral and subcutaneous fat with no lean mass loss
- Well-tolerated with repeat-dosing
- Similar robust results in *db/db* mice suggest potential in patients with obesity and diabetes

ADARx PHARMACEUTICALS

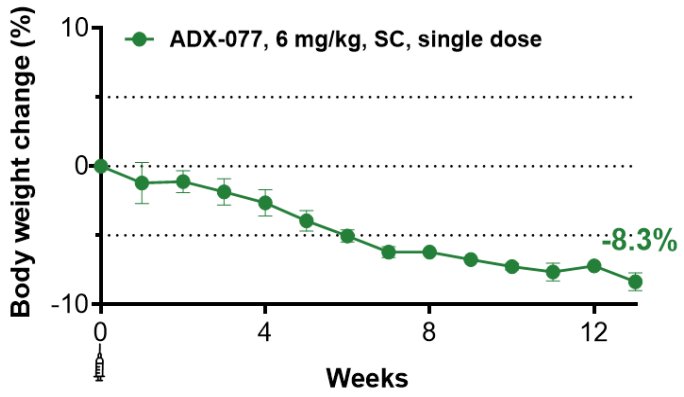
ADX-077: Encouraging NHP Data

Meaningful Effect on Body Weight and Fat Reduction Observed in Spontaneous Obese NHPs

OBE1 mRNA Knockdown in Adipose

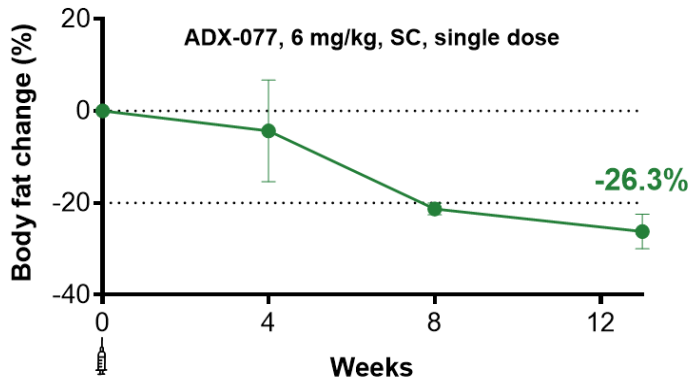


Body Weight

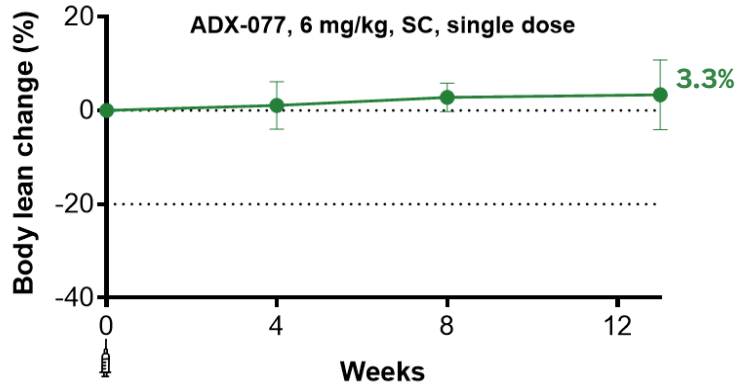


Starting NHP BMI=42-53

Body Fat Change



Body Lean Change



- **ADX-077**
  - Steady weight loss and meaningful fat mass reduction observed in obese NHPs
  - Preserved lean mass
- **Competitive with semaglutide in Obese NHP[1] on fat loss without impacting lean muscle***

[1]Mastaitis, J. W. et al. Nature Communications volume 16, Article number: 4377 (2025)

*The data presented above are based on cross-study comparisons and are not based on any head-to-head experiments. Differences exist between study designs, subject characteristics and other factors, and caution should be exercised in drawing any conclusions from a comparison of the data across studies as cross-study comparisons are inherently limited and such data may not be directly comparable.

ADARx PHARMACEUTICALS

ADX-199

siRNA preclinical therapeutic candidate for selectively silencing and degrading APP mRNA in neurons for neurodegenerative diseases

ADX-199 is Designed to Selectively Silence and Degrade APP mRNA with Potent and Durable Reduction Observed Across Various Brain Regions in NHPs

Extrahepatic delivery into neurons

- ADX-199 is an siRNA therapeutic candidate engineered to selectively reduce APP mRNA in neurons

Validated target

- Human genetic evidence indicates that elevated levels of APP protein or its cleavage products, including amyloid-ß peptides, are closely linked to increased risk of early-onset neurodegenerative diseases

Large market opportunity

- APP has been proposed as a target for Alzheimer's disease (AD) and CAA
- ~7.4M Patients in US aged 65+ live with Alzheimer's disease, projected to reach ~11.2M by 2040
- CAA represents a significant unmet need with no currently approved disease-modifying therapies
- The prevalence of moderate-to-severe CAA is estimated to be somewhere between 25%-50% of AD patients, and 7-23% in the general population

Encouraging preclinical data

- We observed, in NHPs, deep APP mRNA reduction across various brain regions at 3 months
- Observed potent and durable reduction of target APP protein and its downstream neurotoxic Aβ42 peptide with a single dose
- Observed preferential activity in the CNS tissue over peripheral tissues
- Generally well-tolerated following a single 60mg IT dose

Advancing into IND-enabling studies

- Advancing into IND-enabling studies and plan to initiate a Phase 1 clinical trial at the end of 2027 or in early 2028
- Initial clinical data from the Phase 1 clinical trial is anticipated in 2028

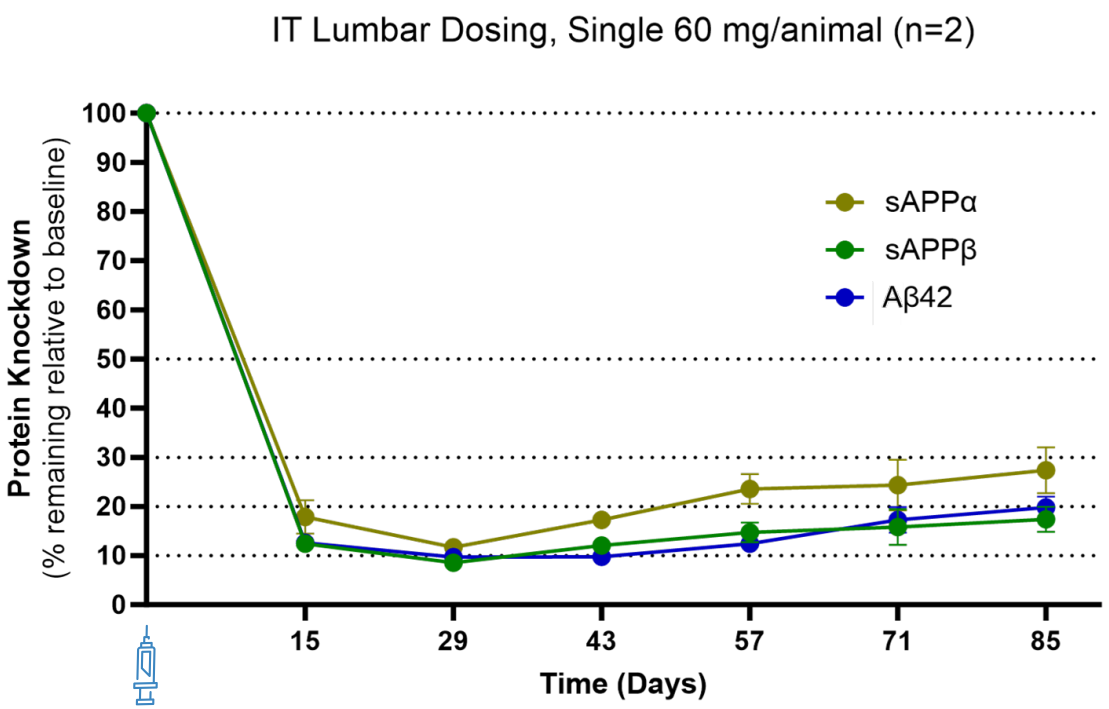
ADARx PHARMACEUTICALS

APP = Amyloid-β Precursor Protein; CAA = Cerebral Amyloid Angiopathy

ADX-199: Engineered to Selectively Reduce APP mRNA in Neurons for Neurodegenerative Diseases

Observed Deep and Durable Target Suppression in NHPs

ADX-199 in NHP Study

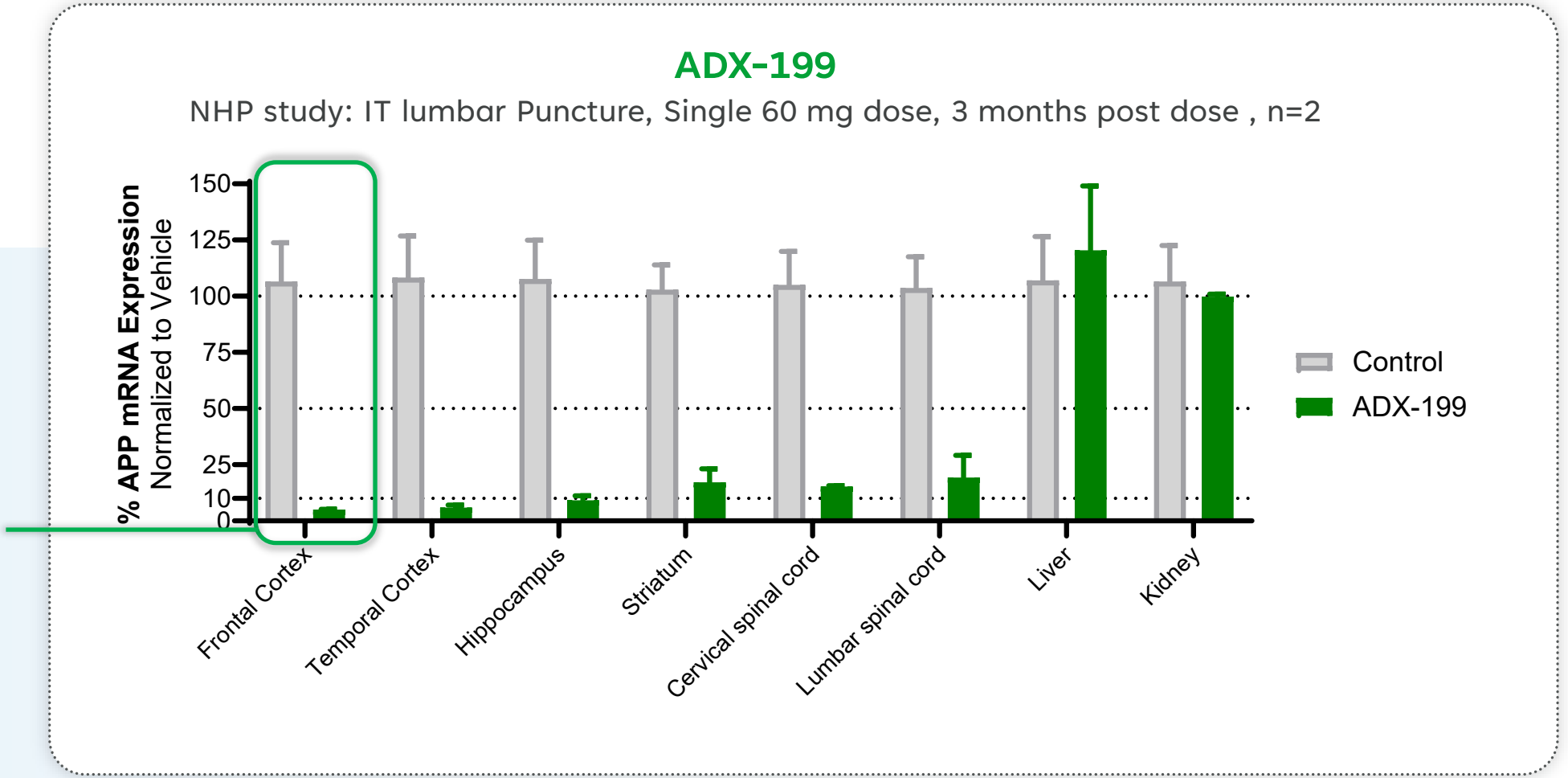


- Targeting a well validated neurodegenerative disease driver (APP)
- Achieved potent and durable reduction of target APP protein and its downstream neurotoxic Aβ42 peptide with a single dose
- Generally well-tolerated following a single 60mg IT dose

ADARx PHARMACEUTICALS

Preferential APP mRNA Knockdown Observed in NHP

We observed deep APP mRNA reduction across various brain regions at 3 months, including **94.9%** knockdown activity in the frontal cortex, a key brain region implicated in Alzheimer's disease



Observed preferential activity in the CNS tissue over peripheral tissues

ADARx Summary

Internally Generated, Wholly Owned Programs Designed to Efficiently Deliver siRNA to Both Hepatic & Extrahepatic Tissues

HEPATIC

Agazisiran (CFB, Ph2)

A potent long-acting siRNA silencing CFB mRNA for the treatment of multiple complement-mediated diseases

- In Phase 1, observed up to 99.9% mean inhibition of complement AP activity at nadir after single SC injection, with reduction sustained through 6M
- Well-tolerated, no treatment-related SAEs in Ph1[1]
- Multiple Phase 2 trials ongoing with meaningful cadence of data anticipated in 2027 and 2028

Onvuzosiran (PKK, Ph3)

A potent long-acting siRNA silencing PKK mRNA for the prevention of HAE attacks

- In Phase 2a, 100% attack-free for 3 months, with one patient remaining attack-free for 18 consecutive months on Q6M dosing after one mild attack at Day 153[2]
- Generally well-tolerated with no SAEs reported in subjects dosed with onvuzosiran[3]
- Phase 3 STOP-HAE trial ongoing with topline data expected by YE27

ADX-626 (FXI, Ph1)

A long-acting siRNA silencing coagulation FXI for secondary stroke prevention with additional clinical potential for stroke prevention in AFib

- In Phase 1, at 400 mg dose, observed deep FXI mean reduction (97.4%) at nadir in FXI protein activity with SC dosing Q6M[4]; continuing to explore higher doses
- Well-tolerated with no SAEs
- Preparing for Phase 2 development in SSP and exploratory studies in SPAF

EXTRAHEPATIC

ADX-077 (OBE1, IND-enabling)

siRNA preclinical therapeutic candidate for obesity via a novel undisclosed target in adipose tissue

- In obese NHPs, a single SC injection resulted in 8.3% overall bodyweight reduction at Week 13, biased to fat mass while preserving lean mass
- Well-tolerated in NHPs at 50mg/kg
- IND-enabling studies ongoing; Anticipated P1 trial start: 2027

ADX-199 (APP, Preclinical)

siRNA therapeutic candidate for selectively silencing and degrading APP mRNA in neurons for neurodegenerative diseases

- In NHPs, a single injection resulted in deep APP mRNA reduction across various brain regions at 3 months, including 94.9% knockdown activity in the frontal cortex
- Advancing into IND-enabling studies; P1 trial start anticipated by YE27 / early 2028

abbvie | ADARx PHARMACEUTICALS

Discovery-stage extrahepatic programs leveraging ADARx's RNA discovery expertise and siRNA technology across indications in neuroscience, immunology and oncology

We believe demonstrated reproducibility across multiple programs enables scalable and efficient pipeline expansion

ADARx PHARMACEUTICALS

1) As of the June 8, 2026 safety cutoff date; 2) As of the August 19, 2026 cutoff date; 3) As of the August 5, 2026 safety cutoff date; 4) Based on data received as of August 12, 2026

Meaningful Cadence of Data Milestones Anticipated Across Our Portfolio

Program	2027	2028
Agazisiran CFB	**Mid-27:** IgAN Ph2, 9m UPCR reduction data **2H27:** PNH Ph2, 6m Hb readout **2H27:** IgAN Ph2, 1-yr eGFR stabilization **2H27:** C3G Ph2, 9m UPCR reduction	**1H28:** IgAN China combo, 9m UPCR reduction
Onvuzosiran HAE	**YE27:** STOP-HAE Ph3 Topline, n=90	**2028:** HAE FDA submission[1]
ADX-626 FXI	**2027**: FXI potential Ph2a/b trial start in SSP **YE27:** FXI Ph2a, 3m PD data readout in SSP patients	
ADX-077 Obesity	**2027:** OBE1 Ph1 trial start	**2028:** OBE1 Ph1 data readout
ADX-199 APP	**YE27 / Early 2028:** APP Ph1 trial start	**2028:** APP Ph1 data readout

In addition, certain near-term development milestones across AbbVie collaboration programs
Cash, cash equivalents and short-term investments of ~$427 million as of June 30, 2026

ADARx PHARMACEUTICALS

UPCR = Urine Protein-Creatinine Ratio; eGFR = Estimated Glomerular Filtration Rate; Hb = Hemoglobin; PD = Pharmacodynamics; SSP = Secondary Stroke Prevention
1) Subject to receipt of positive data from STOP-HAE Phase 3 trial

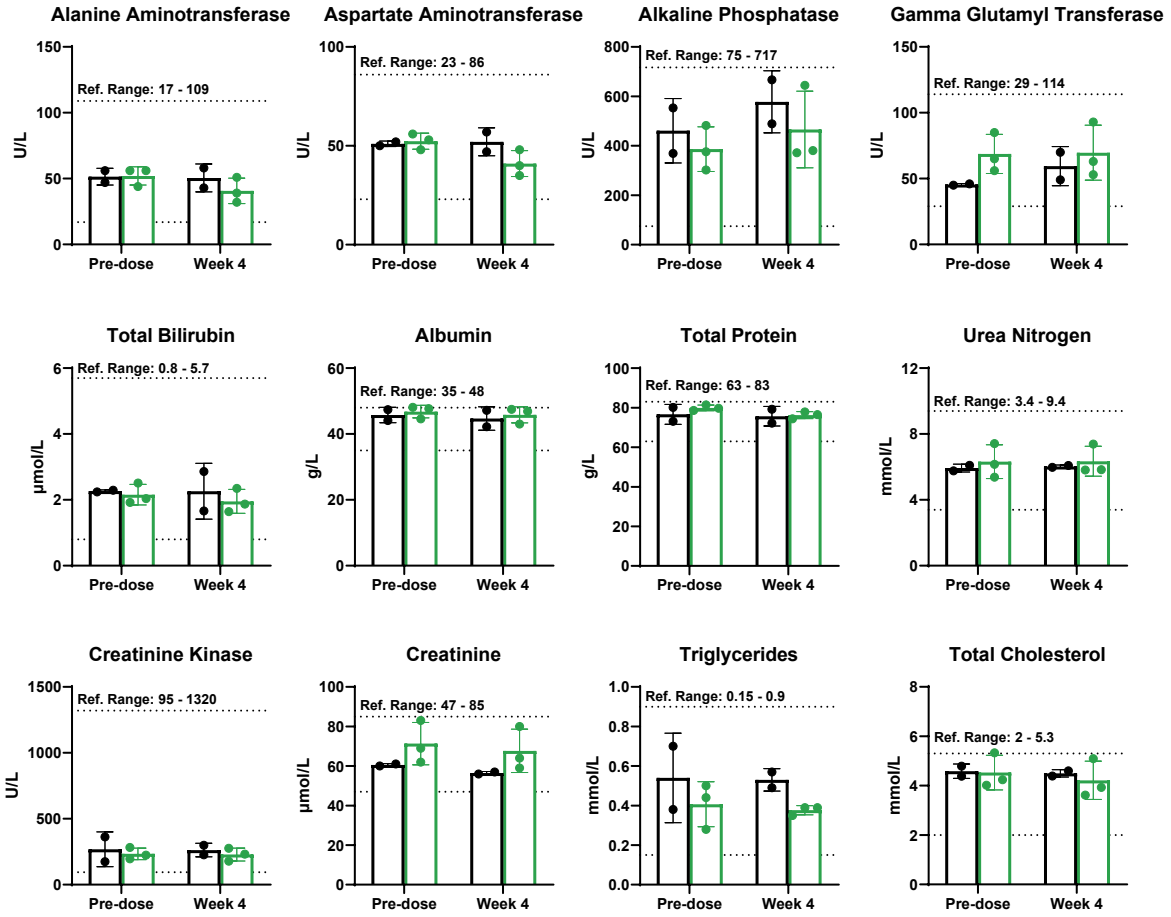
Thank you!

Innovative. Efficient. Focused.

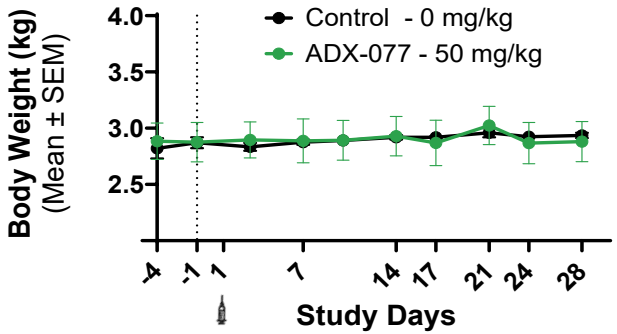
Appendix

Explorative Tox Study: ADX-077 Was Well Tolerated in NHP at 50 mg/kg

Clinical Chemistry



Body Weight



Observed no:

- treatment-related effect on clinical observations
- undesired body weight reduction in lean NHPs
- undesired clinical pathology parameters
- impacts on GI and tolerability issues