HELIOS-A Phase 3 Study of Vutrisiran

Full 9-Month Results

April 19, 2021

Alnylam PHARMACEUTICALS

Agenda

Welcome

- Christine Lindenboom
  Senior Vice President, Investor Relations & Corporate Communications

Introduction

- John Maraganore, Ph.D.
  Chief Executive Officer

Disease Overview & HELIOS-A 9-Month Results

- Akshay Vaishnaw, M.D., Ph.D.
  President of R&D

Commercial Preparedness & Next Steps

- Yvonne Greenstreet, MBChB, MBA
  President and Chief Operating Officer

Q&A Session

Alnylam Forward Looking Statements

This presentation contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, including expectations regarding our ability to achieve our "Alnylam P5x25" strategy, the potential expansion of the ATTR amyloidosis franchise, plans for additional global regulatory filings and the continuing product launches of our approved products, the filing of and review by FDA of an NDA for vutrisiran and the timing of additional regulatory filings, the timing of 18-month data from the HELIOS-A study and the potential timing of a U.S. launch of vutrisiran, the potential market opportunity in hATTR amyloidosis and the key drivers of potential market expansion with. Actual results and future plans may differ materially from those indicated by these forward-looking statements as a result of various important risks, uncertainties and other factors, including, without limitation: the direct or indirect impact of the COVID-19 global pandemic or any future pandemic on our business, results of operations and financial condition and the effectiveness or timeliness of our efforts to mitigate the impact of the pandemic; our ability to discover and develop novel drug candidates and delivery approaches and successfully demonstrate the efficacy and safety of our product candidates; the pre-clinical and clinical results for our product candidates; actions or advice of regulatory agencies and our ability to obtain and maintain regulatory approval for our product candidates, as well as favorable pricing and reimbursement; successfully launching, marketing and selling our approved products globally; delays, interruptions or failures in the manufacture and supply of our product candidates or our marketed products; obtaining, maintaining and protecting intellectual property; our ability to successfully expand the indication for ONPATTRO in the future; our ability to manage our growth and operating expenses through disciplined investment in operations and our ability to achieve a self-sustainable financial profile in the future without the need for future equity financing; our ability to maintain strategic business collaborations; our dependence on third parties for the development and commercialization of certain products, including Novartis, Regeneron and Vir; the outcome of litigation; the risk of government investigations; and unexpected expenditures; as well as those risks more fully discussed in the "Risk Factors" filed with our most recent Annual Report on Form 10-K filed with the SEC and in our other SEC filings. If one or more of these factors materialize, or if any underlying assumptions prove incorrect, our actual results, performance, timelines or achievements may vary materially from any future results, performance or achievements expressed or implied by these forward-looking statements. All forward-looking statements speak only as of the date of this presentation and, except as required by law, we undertake no obligation to update such statements.

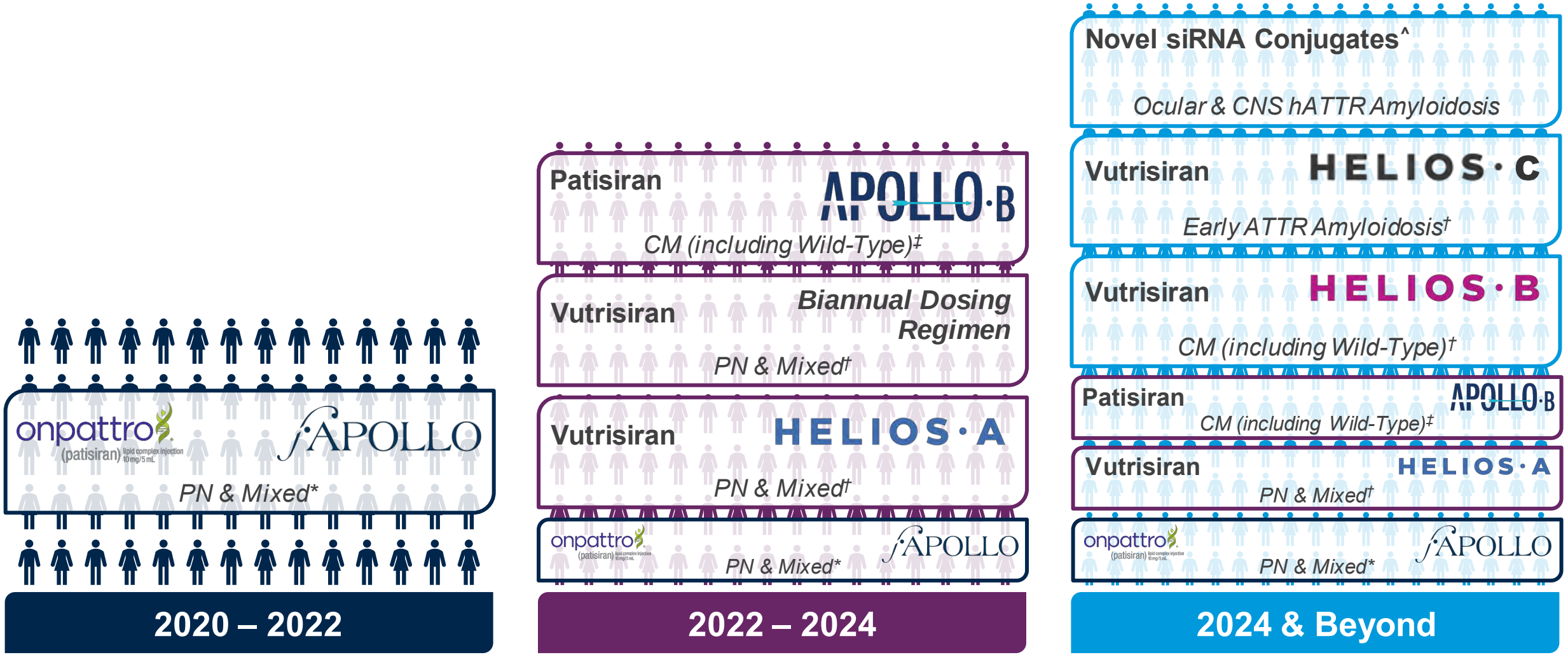
John Maraganore, Ph.D.

Chief Executive Officer

Introduction

Alnylam ATTR Amyloidosis Franchise

Potential to Expand Value to Patients Globally for Many Years to Come



* ONPATTRO is approved in the U.S. and Canada for the treatment of the PN of hATTR amyloidosis in adults, and in the EU, Japan and other countries for the treatment of hATTR amyloidosis in adults with stage 1 or 2 PN; ‡ ONPATTRO has not been approved by the FDA, EMA, or any other regulatory agency for cardiac manifestations of amyloidosis. No conclusions can or should be drawn regarding its safety or effectiveness in this population

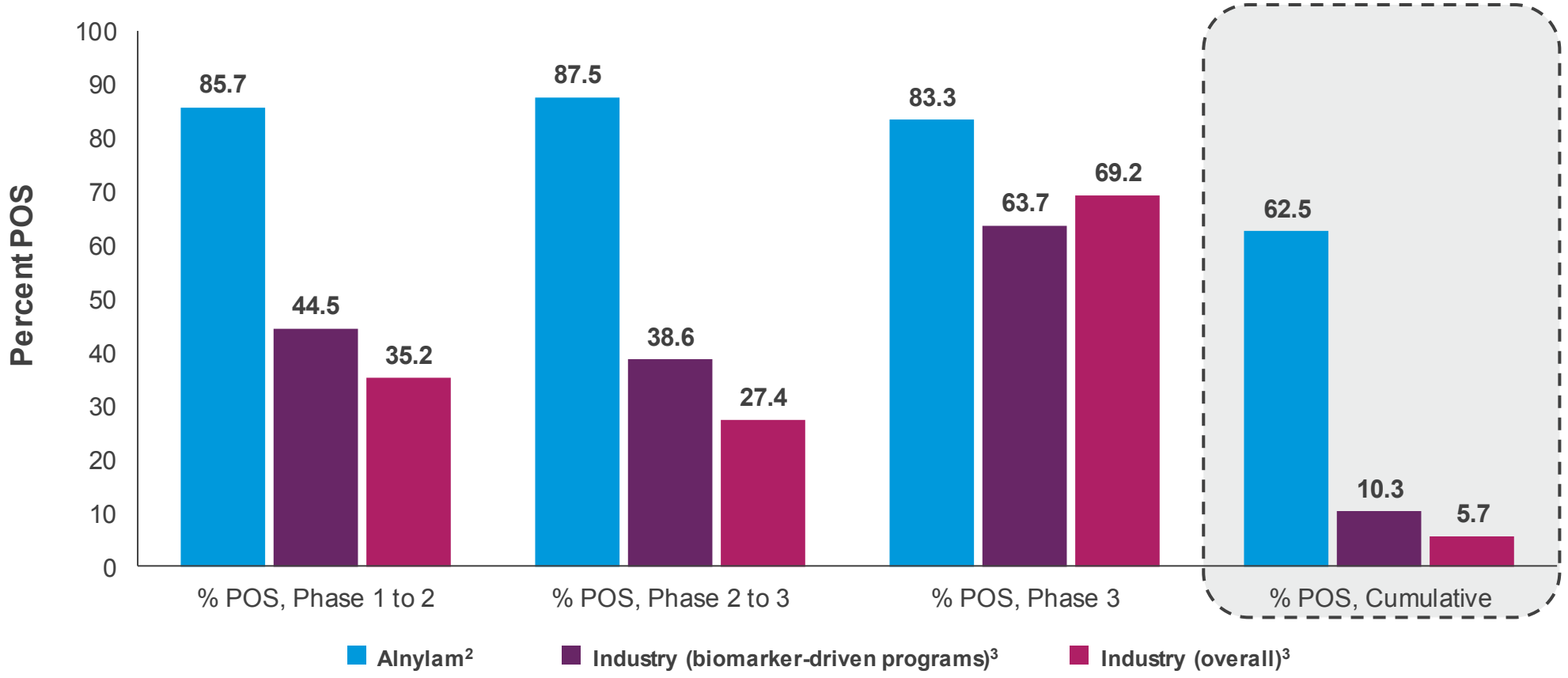
† Vutrisiran is an investigational agent and has not been approved by the FDA, EMA, or any other regulatory agency and no conclusions can or should be drawn regarding its safety or effectiveness; additional studies and future development possible; ^ Novel siRNA conjugate development candidates for ocular or CNS hATTR amyloidosis not yet selected

Intended to be illustrative and not intended to represent specific estimates of patient numbers

Productivity of Alnylam RNAi Therapeutic Platform

Comparison of Historical Industry Metrics to Alnylam Portfolio[1]

Probability of Success (POS) by Phase Transition



[1] Analysis as of January 2021; Past rates of Alnylam and industry respectively may not be predictive of the future
[2] Alnylam programs biomarker-driven at all stages of development (100%); figures include ALNY-originated molecules now being developed by partners
[3] Wong et al., Biostatistics (2019) 20, 2, pp. 273–286

Additional Launches Planned Over Next 12-24 Months

2018	2019	2020	2020-2021	2022-2023	
onpattro® (patisiran) lipid complex injection 10 mg/5 mL	GIVLAARI® (givosiran) injection for subcutaneous use 189 mg/mL	OXLUMO™ (lumasiran) for injection 94.5 mg/0.5 mL	**Leqvio®** (inclisiran)	**Vutrisiran**	**Fitusiran[5]**
ONPATTRO is indicated in the U.S. for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults[1]	GIVLAARI is indicated in the U.S. for the treatment of adults with acute hepatic porphyria[2]	OXLUMO is indicated in the U.S. for the treatment of primary hyperoxaluria type 1 to lower urinary oxalate levels in pediatric and adult patients[3]	Leqvio is approved in the EU for the treatment of adults with hypercholesterolemia or mixed dyslipidemia[4] *CRL in U.S. related to inspection*	ATTR amyloidosis *Positive HELIOS-A Phase 3 Results* *NDA submitted*	Hemophilia *Two of three Phase 3 studies fully enrolled*

[1] ONPATTRO is approved in U.S. and Canada for the PN of hATTR amyloidosis in adults, and in EU, Japan and other countries for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy. For additional information on ONPATTRO, see Full Prescribing Information
[2] GIVLAARI is approved in the U.S., Brazil and Canada for the treatment of adults with acute hepatic porphyria (AHP), and in the EU for the treatment of AHP in adults and adolescents aged 12 years and older. For additional information on GIVLAARI, see Full Prescribing Information
[3] OXLUMO is approved in the U.S. and EU for the treatment of primary hyperoxaluria type 1 in all age groups. For additional information on OXLUMO, see Full Prescribing Information
[4] Novartis has obtained global rights to develop, manufacture and commercialize inclisiran under a license and collaboration agreement with Alnylam Pharmaceuticals, a leader in RNAi therapeutics
[5] Sanofi Genzyme is leading and funding development of fitusiran and will commercialize fitusiran, if successful
Anticipated dates of launch based on current development timelines for investigational therapeutics and assuming positive pivotal study data and regulatory approval

Alnylam

P5X25

Patients: Over 0.5 million on Alnylam RNAi therapeutics globally

Products: 6+ marketed products in rare and prevalent diseases

Pipeline: Over 20 clinical programs, with 10+ in late stages and 4+ INDs per year

Performance: ≥40% revenue CAGR through YE 2025

Profitability: Achieve sustainable non-GAAP profitability within period

Akshay Vaishnaw, M.D., Ph.D.

President of R&D

Disease Overview & HELIOS-A 9-Month Results

Cece
Living with hATTR amyloidosis (USA)

ATTR Amyloidosis

Rare, Progressively Debilitating, and Often Fatal Disease

Description

Caused by misfolded TTR protein that accumulates as amyloid deposits in multiple tissues including heart, nerves, and GI tract[1]

Hereditary ATTR (hATTR) Amyloidosis

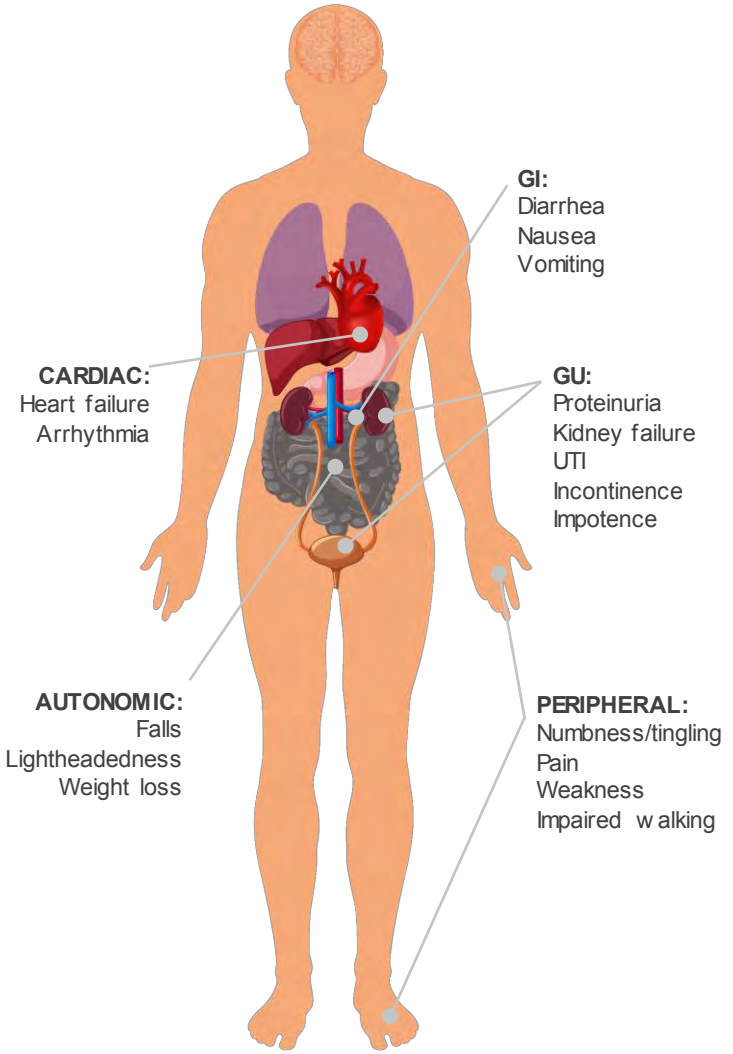
~50,000

patients worldwide*

Wild-Type ATTR (wtATTR) Amyloidosis

~200,000 – 300,000

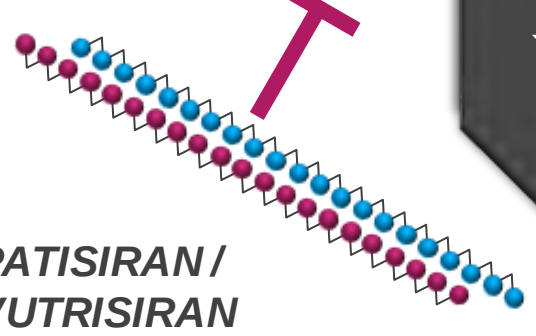
patients worldwide



[1] Coelho T, et al. N Engl J Med. 2013;369(9):819-829
* Ando, et al. Orphanet J Rare Dis, 2013; Ruberg, et al. Circulation, 2012

RNAi Therapeutic Hypothesis in ATTR Amyloidosis

Silencing TTR Gene Expression Can Potentially Address Underlying Cause of Disease



PATISIRAN / VUTRISIRAN

siRNA sequences selected to silence both variant and wild-type TTR

Production of variant and wild-type TTR in liver*

Reduce circulating TTR

Prevent or clear tissue amyloid deposits

Halt or improve progressive manifestations of disease

* >95% of TTR in circulation produced in liver

Alnylam's TTR Amyloidosis Franchise

Approved Treatment Option and Investigational Programs

onpattro® (patisiran) lipid complex injection 10 mg/5 mL

ONPATTRO® (patisiran) is an **Approved RNAi Therapeutic** for Treatment of **Polyneuropathy of hATTR Amyloidosis***

About ONPATTRO

- Approved in over 30 countries
- IV administration, once every 3 weeks
- Patisiran also in clinical development as potential treatment for ATTR amyloidosis with cardiomyopathy‡

Vutrisiran

Vutrisiran is an **Investigational RNAi Therapeutic** for Potential **Treatment of ATTR Amyloidosis†**

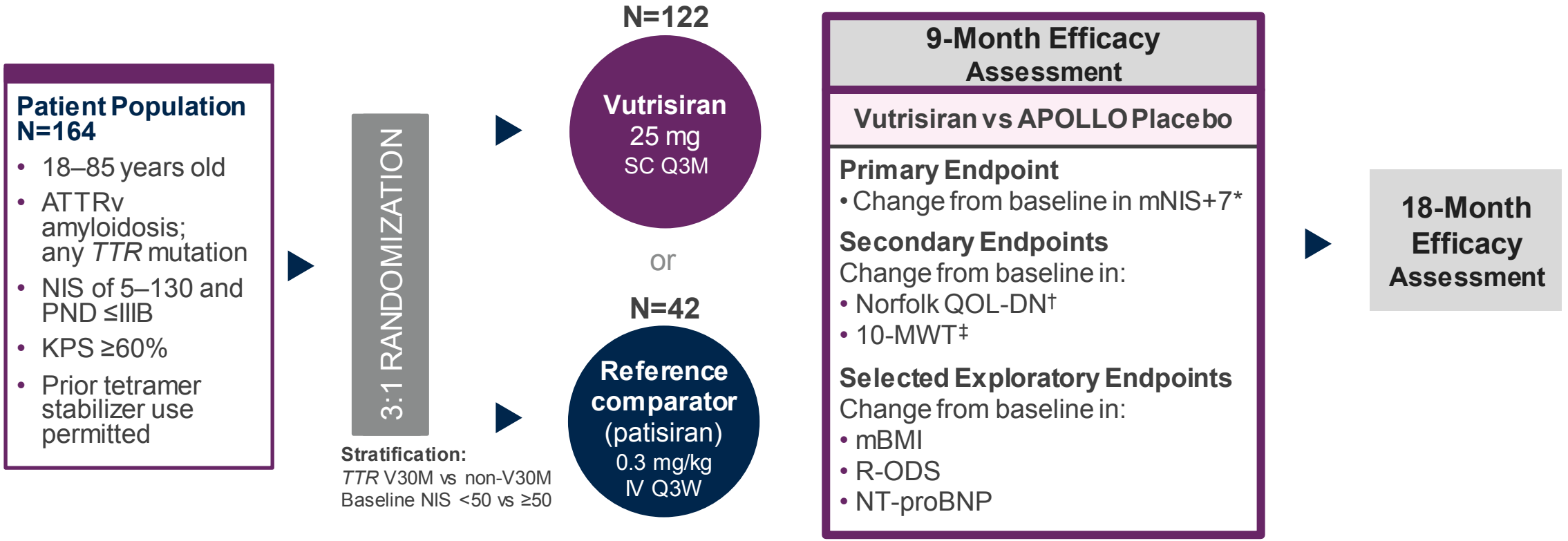
About Vutrisiran

- Subcutaneous administration, once every 3 months
  - Exploring biannual dosing regimen
- Pre-filled syringe (PFS) presentation
- Positive HELIOS-A Phase 3 results
  - NDA submitted April 2021

* ONPATTRO is approved in the U.S. and Canada for the polyneuropathy of hATTR amyloidosis in adults, in the EU, Switzerland and Brazil for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy, and in Japan for the treatment of transthyretin (TTR) type familial amyloidosis with polyneuropathy; ‡ Patisiran has not been approved by the FDA, EMA, or any other regulatory agency for cardiac manifestations of amyloidosis. No conclusions can or should be drawn regarding its safety or effectiveness in this population;
† Vutrisiran is an investigational agent and has not been approved by the FDA, EMA, or any other regulatory agency and no conclusions can or should be drawn regarding its safety or effectiveness

Vutrisiran HELIOS·A Phase 3 Study

Randomized, Open-Label Study in Hereditary ATTR Amyloidosis Patients with Polyneuropathy



*Higher scores of mNIS+7 indicate more neurologic impairment (range, 0 to 304). †Higher scores of Norfolk QOL-DN indicate worse quality of life (range, -4 to 136). ‡10-meter walk test speed (m/s) = 10 meters/mean time (seconds) taken to complete two assessments at each visit, imputed as 0 for patients unable to perform the walk; lower speeds indicate worse ambulatory function.

10-MWT, 10-meter walk test; ATTRv, transthyretin-mediated amyloidosis (v for variant); IV, intravenous; KPS, Karnofsky performance status; mBMI, modified body mass index; mNIS+7, modified Neuropathy Impairment Score +7; NIS, Neuropathy Impairment Score; Norfolk QOL-DN, Norfolk Quality of Life-Diabetic Neuropathy; NT-proBNP, N-terminal pro–brain natriuretic peptide; PND, polyneuropathy disability; Q3M, every 3 months; Q3W, every 3 weeks; R-ODS, Rasch-built overall disability scale; SC, subcutaneous; TTR, transthyretin.

Baseline Demographic and Disease Characteristics

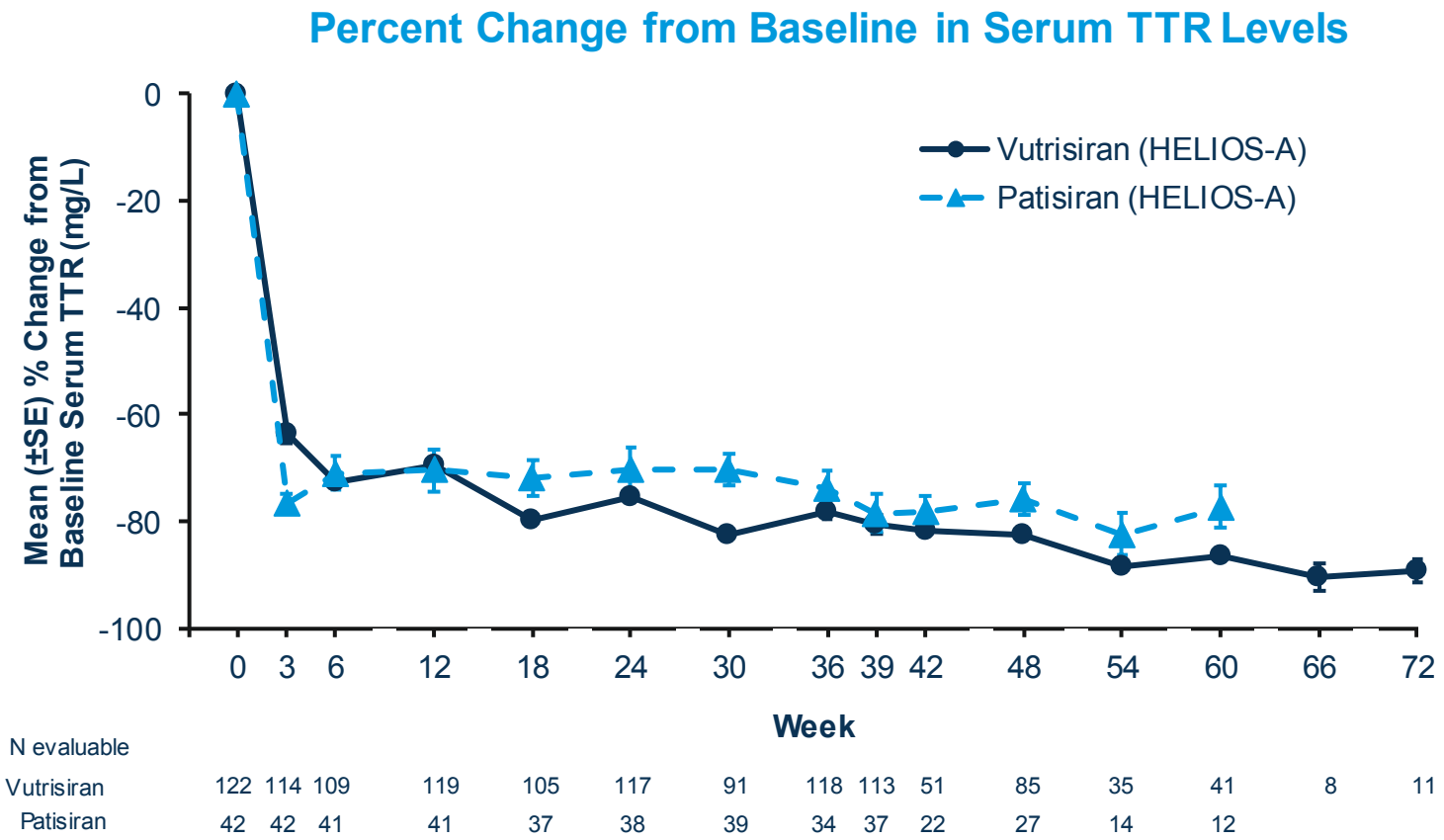
Characteristic	APOLLO	HELIOS-A	
	Placebo N=77	Vutrisiran N=122	Patisiran (N=42)
Age (years), median (range)	63 (34, 80)	60 (26, 85)	60 (31, 81)
Males, n (%)	58 (75)	79 (65)	27 (64)
TTR genotype, n (%)			
V30M	40 (52)	54 (44)	20 (48)
Non-V30M	37 (48)	68 (56)	22 (52)
NIS, mean (range)	57 (7, 126)	43 (5, 127)	43 (6, 116)
PND score*, n (%)			
I: preserved walking, sensory disturbances	20 (26)	44 (36)	15 (36)
II: impaired walking but can walk without stick or crutch	23 (30)	50 (41)	17 (40)
IIIA: walk with 1 stick or crutch	22 (29)	16 (13)	7 (17)
IIIB: walk with 2 sticks or crutches	11 (14)	12 (10)	3 (7)
Cardiac subpopulation, n (%)†	36 (47)	35 (29)	13 (31)

Adams et al., *AAN*, April 2021. *One patient (1.3%) in APOLLO placebo group had a PND score IV defined as confined to wheelchair or bedridden (not shown on the slide). †Cardiac subpopulation was defined as patients who had pre-existing evidence of cardiac amyloid involvement (baseline left ventricular wall thickness ≥1.3 cm and no aortic valve disease or hypertension in medical history).

NIS, Neuropathy Impairment Score; PND, polyneuropathy disability; TTR, transthyretin.

Rapid and Sustained Reduction in Serum TTR Levels with Vutrisiran Similar to Patisiran in HELIOS-A

- Vutrisiran achieved a mean steady-state* serum TTR reduction from baseline of 83% (SD: 14%)



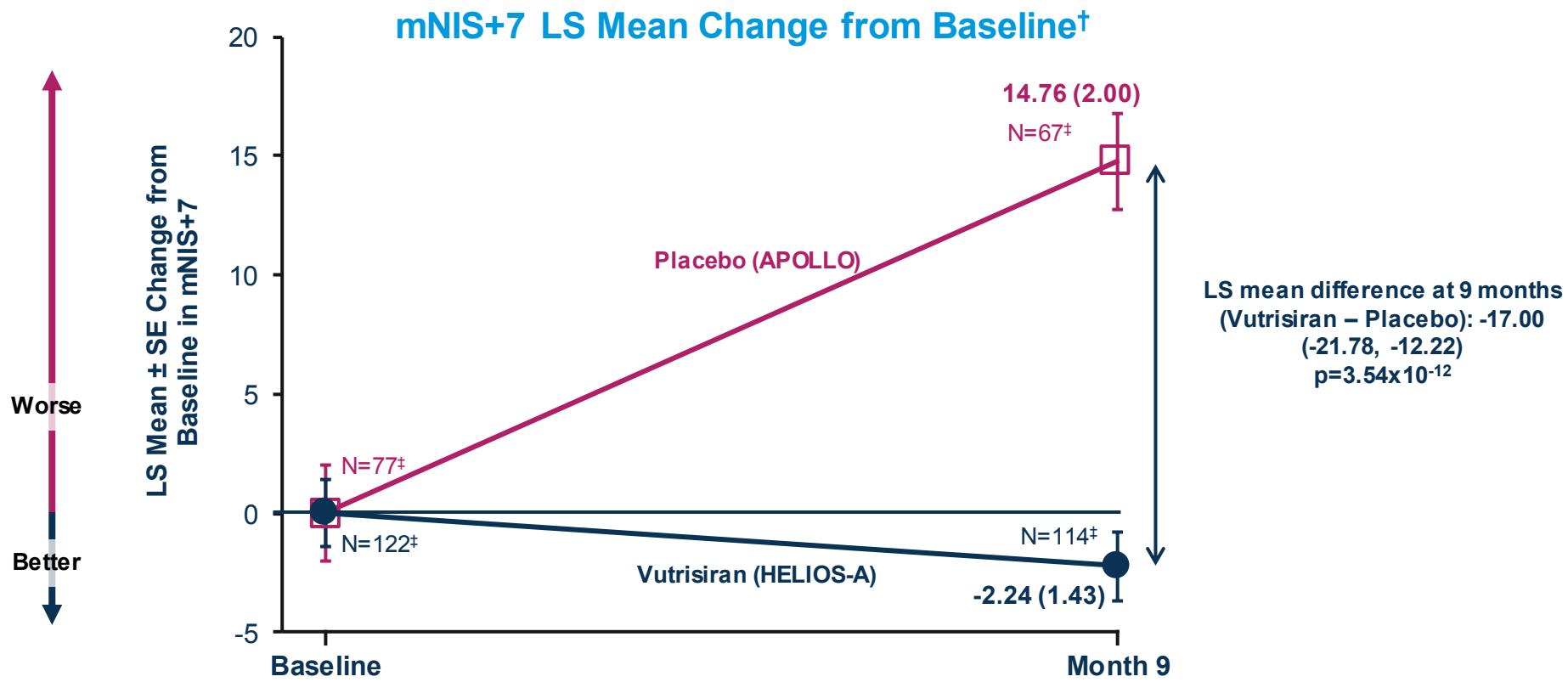
Adams et al., *AAN,* April 2021

*Steady state w as measured using Day 211 samples for vutrisiran.

SD, standard deviation; SE, standard error; TTR, transthyretin.

Significant Improvement in Neuropathy Impairment with Vutrisiran

- Vutrisiran achieved statistically significant improvement in mNIS+7 at 9 months, compared with external placebo group
  - Improvements across all pre-specified patient subgroups* and components of mNIS+7 (data not shown)



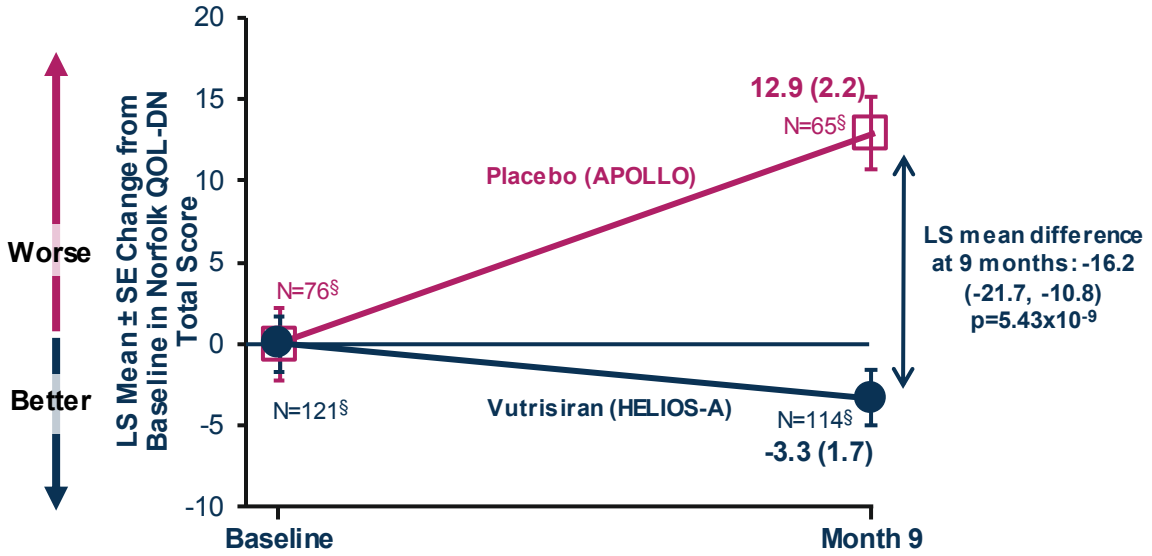
Adams et al., *AAN*, April 2021 (updated to reflect that consistency of treatment effects in vutrisiran and patisiran groups in HELIOS-A data not shown at AAN is shown in this presentation). *Pre-specified patient subgroups included age (<65 or ≥65), sex, race, region, baseline NIS (<50 or ≥50), previous tetramer stabilizer use, genotype(V30M or non-V30M), FAP stage (I or II and III), cardiac subpopulation (baseline left ventricular w all thickness ≥1.3 cm and no aortic valve disease or hypertension in medical history),†mITT population. **At baseline, the mean (±SD) mNIS+7 w as 60.6 (36.0) in the vutrisiran group and 74.6 (37.0) in external placebo group.** ‡Number of evaluable patients.

LS, least squares; mITT, modified intent-to-treat; mNIS+7, modified Neuropathy Impairment Score +7; SD, standard deviation; SE, standard error.

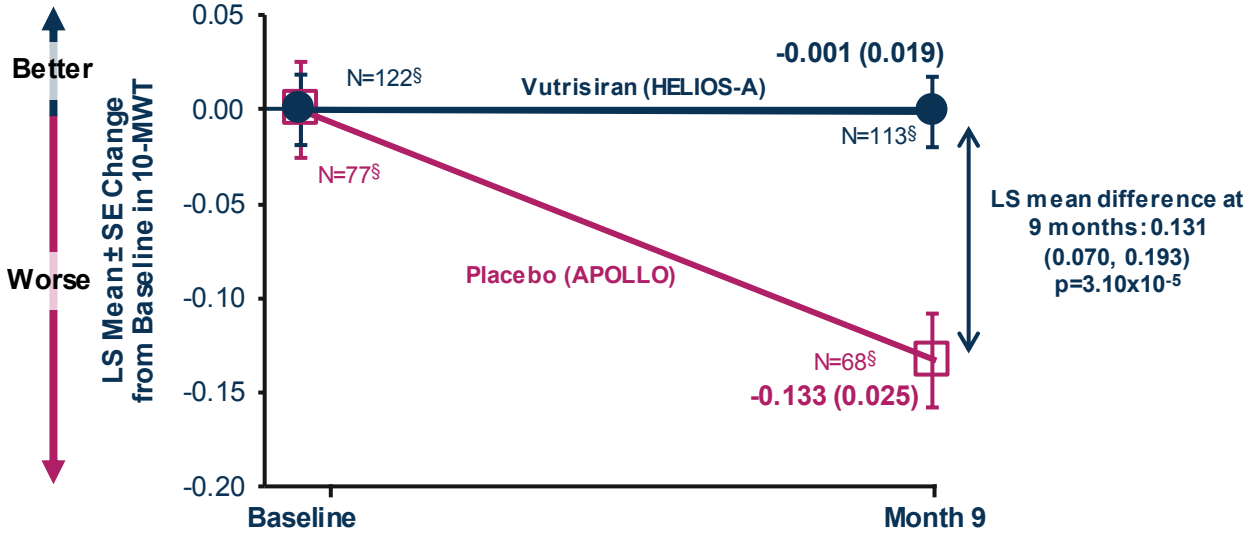
Significant Improvement in Quality of Life and Gait Speed with Vutrisiran

- Vutrisiran achieved statistically significant improvement in Norfolk QOL-DN and gait speed measured by 10-MWT at 9 months, compared with external placebo group
  - Improvements across all pre-specified patient subgroups* and domains of Norfolk QOL-DN (data not shown)

Norfolk QOL-DN LS Mean Change from Baseline†



10-MWT LS Mean Change from Baseline‡



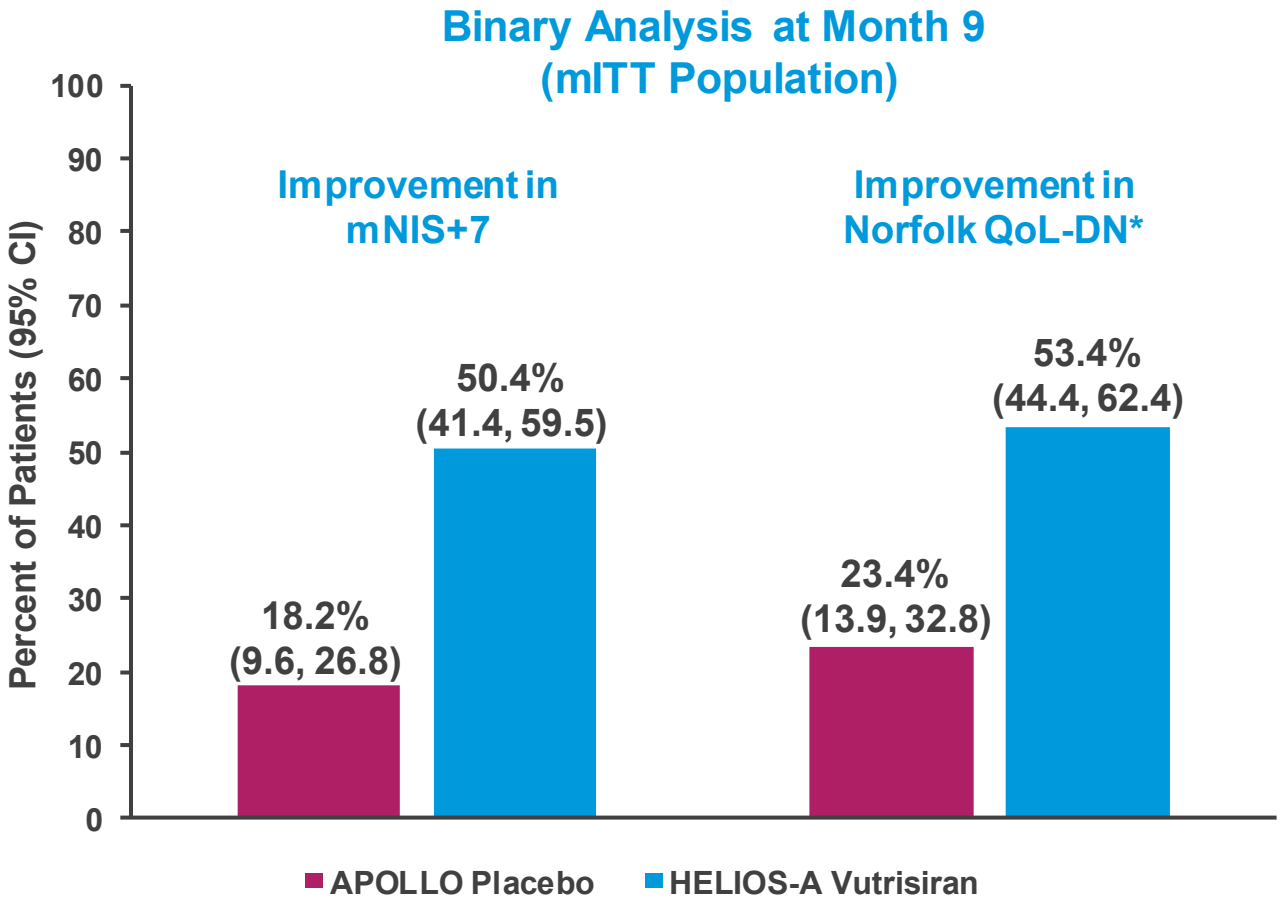
Exploratory endpoints at 9 months, including change from baseline in R-ODS and mBMI, demonstrated improvements compared with external placebo group (data not shown)

Adams et al., *AAN*, April 2021 (updated to reflect that consistency of effects in vutrisiran and patisiran groups of HELIOS-A and NT-proBNP exploratory data not shown at AAN is shown in this presentation). *Pre-specified patient subgroups included age (<65 or ≥65), sex, race, region, baseline NIS (<50 or ≥50), previous tetramer stabilizer use, genotype (V30M or non-V30M), FAP stage (I or II and III), cardiac subpopulation (baseline left ventricular wall thickness ≥1.3 cm and no aortic valve disease or hypertension in medical history),† mITT population. **At baseline, the mean (±SD) Norfolk QOL-DN score was 47.1 (26.3) in the vutrisiran group and 55.5 (24.3) in the external placebo group.** ‡mITT population. At baseline, the mean (±SD) 10-MWT was 1.006 (0.393) in the vutrisiran group and 0.790 (0.319) in the external placebo group. §Number of evaluable patients.

10-MWT, 10-meter walk test; LS, least squares; mBMI, modified body mass index; mITT, modified intent-to-treat; Norfolk QOL-DN, Norfolk Quality of Life-Diabetic Neuropathy; R-ODS, Rasch-built overall disability scale; SD, standard deviation; SE, standard error.

Evidence of Reversal of Polyneuropathy Disease Manifestations

Majority of Patients Showed Improvement in Neuropathy Impairment and Quality of Life, Relative to Baseline*



*Improvement defined as patients with a decrease in total score from baseline to Month 9

HELIOS-A and APOLLO Month 9 Efficacy Results

Post Hoc Cross-Study Comparison

Endpoint	HELIOS-A		APOLLO	
	Vutrisiran (N=122) LS mean change from baseline (95% CI)	Vutrisiran-Placebo LS mean difference (95% CI)	Patisiran (N=148) LS mean change from baseline (95% CI)	Patisiran-Placebo LS mean difference (95% CI)
mNIS+7*	-2.2 (-5.0, 0.6)	-17.0 (-21.8, -12.2)	-2.0 (-5.0, 0.9)	-16.0 (-20.7, -11.3)
Norfolk QOL†	-3.3 (-6.6, -0.1)	-16.2 (-21.7, -10.8)	-7.5 (-10.5, -4.6)	-15.0 (-19.8, -10.2)
10-MWT‡ (m/s)	-0.001 (-0.038, 0.036)	0.131 (0.070, 0.193)	0.05 (0.013, 0.086)	0.156 (0.099, 0.213)

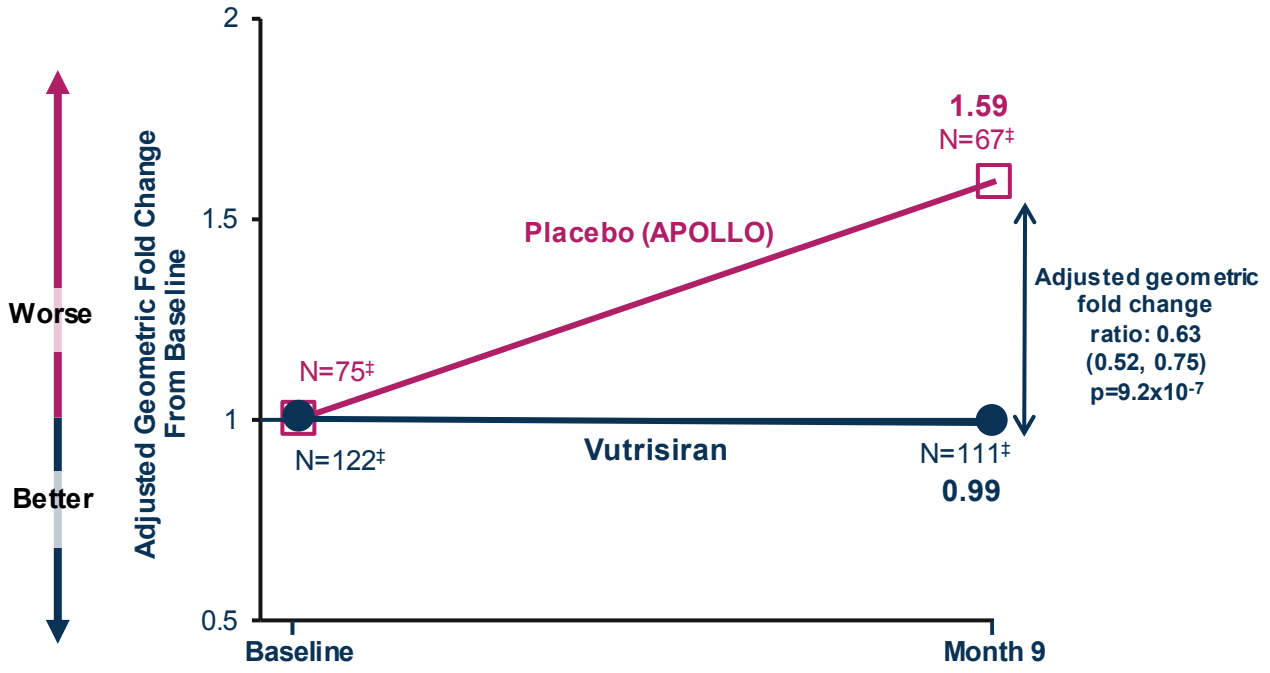
- Vutrisiran efficacy in HELIOS-A similar to that seen with patisiran in APOLLO study
- Patisiran efficacy in HELIOS-A similar to that previously observed in APOLLO study
  - Mean change from baseline for the HELIOS-A patisiran arm was -1.41 for mNIS+7, 0.1 for Norfolk and -0.039 m/s for 10-MWT**

*Higher scores of mNIS+7 indicate more neurologic impairment (range, 0 to 304). †Higher scores of Norfolk QOL-DN indicate worse quality of life (range, -4 to 136). ‡10-meter walk test (10-MWT) speed (m/s) = 10 meters/mean time (seconds) taken to complete two assessments at each visit, imputed as 0 for patients unable to perform the walk; lower speeds indicate worse ambulatory function.
**HELIOS-A patisiran arm not intended for statistical testing vs. vutrisiran for mNIS+7, Norfolk or 10-MWT endpoints; results presented as arithmetic means per statistical analysis plan.

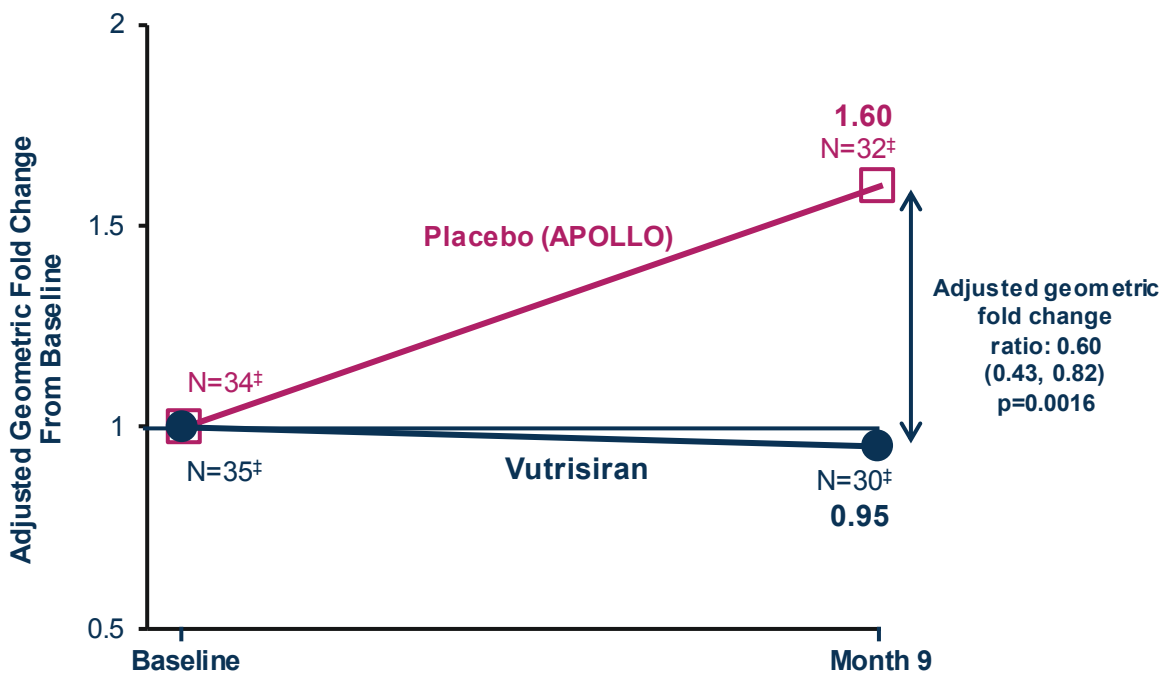
Improvement in NT-proBNP with Vutrisiran vs External Placebo Group

Exploratory Endpoint

Change from Baseline in NT-proBNP (mITT Population)*



Change from Baseline in NT-proBNP (Cardiac Subpopulation)†



*NT-proBNP is a measure of cardiac stress, with higher values indicating a greater level of cardiac stress. At baseline, NT-proBNP geometric mean (SE) was 273.0 (42.2) ng/ml in the vutrisiran group (N=122) and 531.3 (86.7) ng/L in APOLLO placebo (N=75) group. †Cardiac subpopulation was defined as patients who had pre-existing evidence of cardiac amyloid involvement (baseline left ventricular wall thickness ≥1.3 cm and no aortic valve disease or hypertension in medical history). **At baseline, NT-proBNP geometric mean (SE) was 772.8 (195.0) ng/L in the vutrisiran cardiac subpopulation group (N=35) and 771.1 (151.1) ng/L in APOLLO placebo cardiac subpopulation (N=34) group.** ‡Number of evaluable patients.

CI, confidence interval; mITT, modified intent-to-treat; NT-proBNP, N-terminal pro–brain natriuretic peptide; SE, standard error.

HELIOS-A Safety Summary*

Acceptable Safety Profile of Vutrisiran

The majority of AEs were mild or moderate in severity

- No drug-related discontinuations or deaths
- Two study discontinuations (1.6%) due to AEs in the vutrisiran arm by Month 9, both due to deaths, neither of which was considered related to study drug
  - One death due to COVID-19 pneumonia and the other due to iliac artery occlusion
- Two SAEs deemed related to vutrisiran by investigators:
  - Dyslipidemia and urinary tract infection
- AEs ≥10% in vutrisiran group included diarrhea, pain in extremity, fall, and urinary tract infections
  - Each of these events occurred at a similar or lower rate compared with external placebo group
- Injection site reactions were reported in five patients (4.1%) receiving vutrisiran
  - All were mild and transient
- No safety signals regarding liver function tests, hematology or renal function related to vutrisiran

At Least One Event, n (%)	APOLLO†	HELIOS-A	
	Placebo (N=77) PY=96.1	Vutrisiran (N=122) PY=131.3	Patisiran (N=42) PY=43.2
AEs	75 (97.4)	114 (93.4)	40 (95.2)
SAEs	31 (40.3)	21 (17.2)	17 (40.5)
Severe AEs	28 (36.4)	15 (12.3)	12 (28.6)
AEs leading to treatment discontinuation	11 (14.3)	2 (1.6)	3 (7.1)
AEs leading to stopping study participation	9 (11.7)	2 (1.6)	2 (4.8)
Deaths	6 (7.8)	2 (1.6)	3 (7.1)

Adams et al., *AAN*, April 2021

*Cumulative safety data from first dose of study drug to data cut-off date (November 10, 2020). †External reference to reflect the type of disease-related events commonly reported in this population.

AE, adverse event; PY, patient-years; SAE, serious AE.

HELIOS-A 18-Month Data to Further Assess Vutrisiran Impact

18-Month Results Expected Late 2021

Additional Secondary Endpoints

- Extend mNIS+7, Norfolk QOL, and 10-MWT dataset beyond 9 months with longer follow-up
- Assess new secondary endpoints: mBMI, R-ODS, serum TTR reduction

Exploratory Endpoints

- Further characterize potential benefit of vutrisiran on cardiac manifestations of disease

Exploratory endpoint	Measures
NT-proBNP	Cardiac stress
Left ventricular wall thickness	Cardiac amyloid burden
Longitudinal strain	Cardiac function
Technetium scintigraphy	Cardiac amyloid burden

HELIOS-A Month 9 Data Summary

- HELIOS-A is a phase 3, global, open-label study of vutrisiran 25 mg SC Q3M in patients with hATTR amyloidosis with polyneuropathy

- Vutrisiran met primary and both secondary endpoints at 9 months
  - Statistically significant improvements in neuropathy impairment (mNIS+7), quality of life (Norfolk QOL-DN), and gait speed (10-MWT), compared with external placebo group
  - Majority of patients showed improvement in neuropathy impairment and QOL, relative to baseline
  - Consistent treatment effects observed in vutrisiran and patisiran groups of HELIOS-A; also consistent with patisiran effect observed in APOLLO study

- Vutrisiran also demonstrated improvement in NT-proBNP compared with external placebo group

- Vutrisiran has acceptable safety profile and favorable benefit:risk profile

- HELIOS-A will continue to investigate efficacy and safety of vutrisiran through 18-month treatment period and extension period

SC, subcutaneous, Q3M, every 3 months; hATTR, hereditary transthyretin mediated amyloidosis; mNIS+7, modified Neuropathy Impairment Score +7; Norfolk QOL-DN, Norfolk Quality of Life-Diabetic Neuropathy; 10-MWT, 10-meter walk test; QOL, quality of life; NT-proBNP, N-terminal pro–brain natriuretic peptide

Yvonne Greenstreet, MBChB, MBA

President and Chief Operating Officer

Commercial Preparedness & Next Steps

Vutrisiran Planned Next Steps

File NDA

Initiate q6M Data Generation Early 2021

HELIOS-A Topline 18-Month Results Late 2021

U.S. Launch Early 2022

hATTR Amyloidosis Market Opportunity

Estimated Disease Prevalence*†

~ 50,000 patients worldwide

NEUROLOGIC PHENOTYPE
> 50% have cardiomyopathy

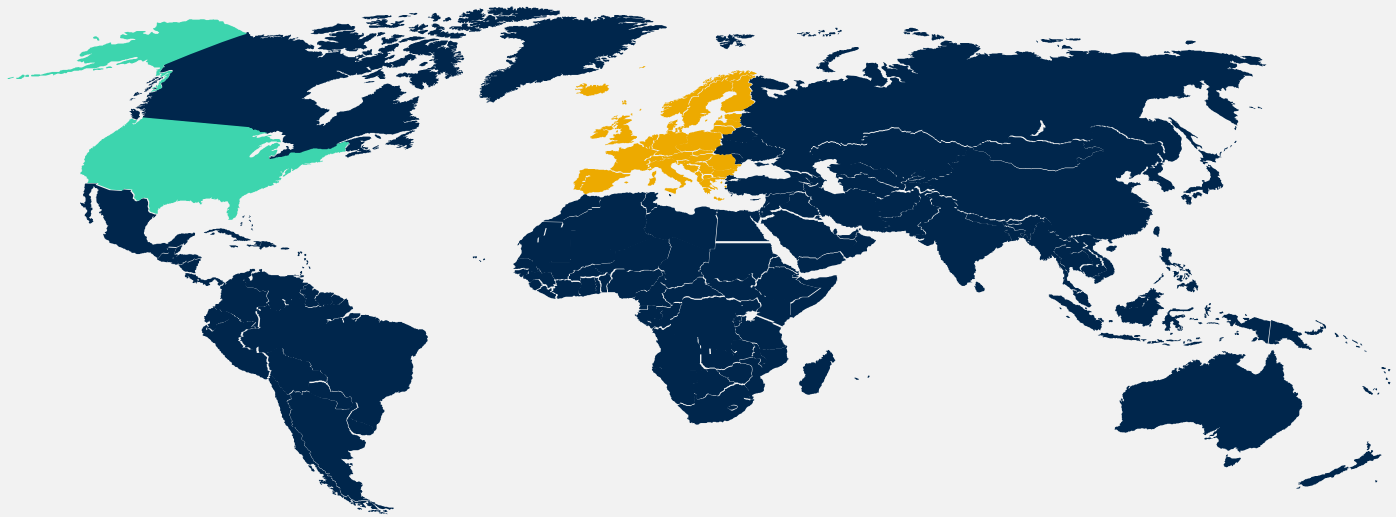
CARDIAC PHENOTYPE†
> 50% have neuropathy

PN & MIXED†

20K to 30K worldwide
~ 10K diagnosed‡

10K to 15K in U.S.
< 3K diagnosed

5K to 10K in EU
~ 2K diagnosed



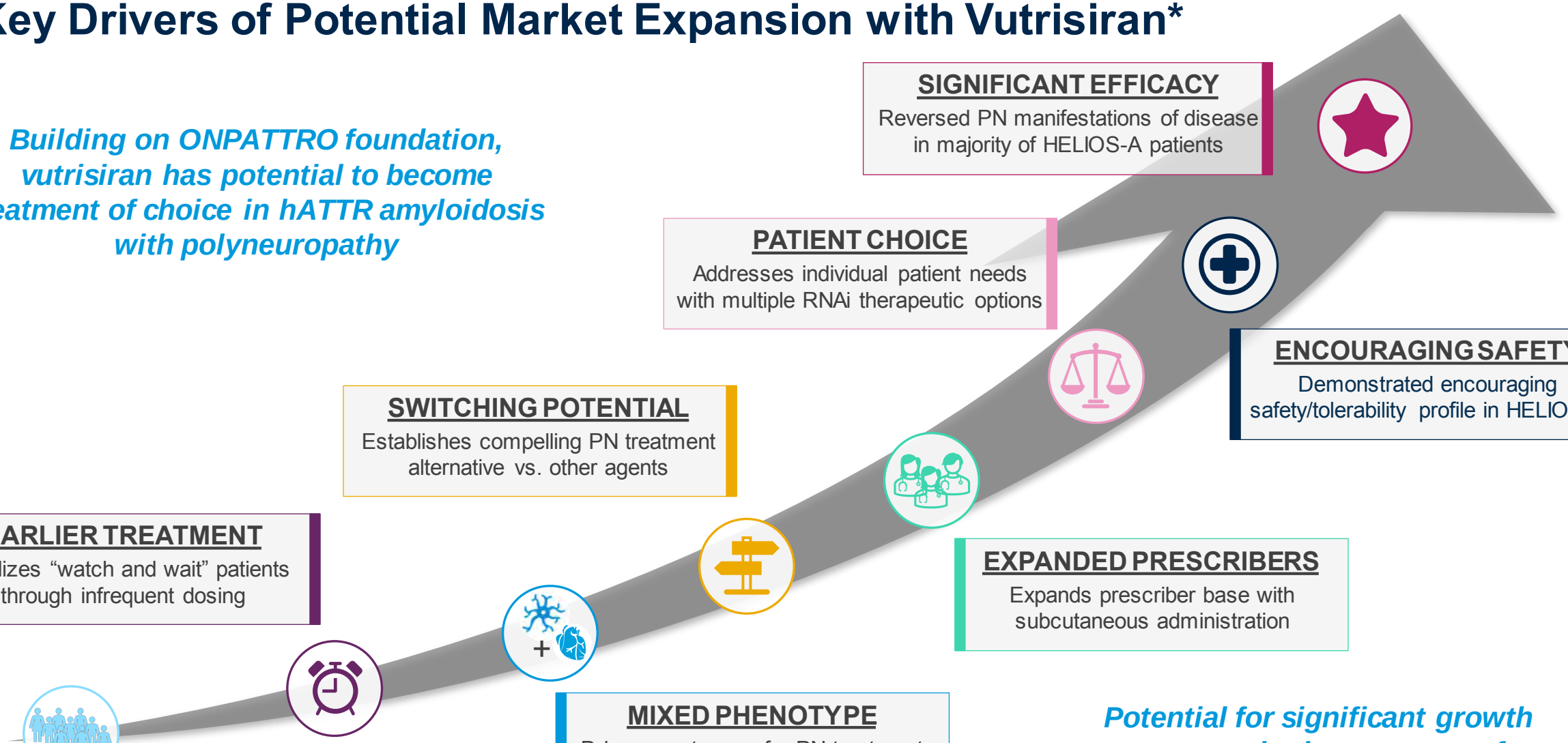
* Based on Alnylam estimates from interviews with key opinion leaders, THAOS registry, recent clinical trials and literature

† ONPATTRO is approved in U.S. and Canada for the PN of hATTR amyloidosis in adults, and in EU, Japan and other countries for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy. For additional information on ONPATTRO, see Full Prescribing Information

‡ Current diagnosis rates difficult to confirm and may be lower in initial launch years

Key Drivers of Potential Market Expansion with Vutrisiran*

Building on ONPATTRO foundation, vutrisiran has potential to become treatment of choice in hATTR amyloidosis with polyneuropathy



SIGNIFICANT EFFICACY
Reversed PN manifestations of disease in majority of HELIOS-A patients

PATIENT CHOICE
Addresses individual patient needs with multiple RNAi therapeutic options

ENCOURAGING SAFETY
Demonstrated encouraging safety/tolerability profile in HELIOS-A

SWITCHING POTENTIAL
Establishes compelling PN treatment alternative vs. other agents

EXPANDED PRESCRIBERS
Expands prescriber base with subcutaneous administration

EARLIER TREATMENT
Mobilizes "watch and wait" patients through infrequent dosing

MIXED PHENOTYPE
Drives greater use for PN treatment within mixed phenotype population

BROAD ACCESS
Continues innovative approach with payers, ensuring broad access

Potential for significant growth opportunity in treatment of hATTR amyloidosis with polyneuropathy with global approvals of vutrisiran

*Vutrisiran is an investigational RNAi therapeutic, market expansion is pending regulatory review and approval as well as other commercial factors

HELIOS-A 9-Month Results

Q&A Session

Alnylam