Corporate Overview

June 2015
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. There are a number of important factors that could cause actual results to differ materially from the results anticipated by these forward-looking statements. These important factors include our ability to discover and develop novel drug candidates and delivery approaches, successfully demonstrate the efficacy and safety of our drug candidates, obtain, maintain and protect intellectual property, enforce our patents and defend our patent portfolio, obtain regulatory approval for products, establish and maintain business alliances; our dependence on third parties for access to intellectual property; and the outcome of litigation, as well as those risks more fully discussed in our most recent quarterly report on Form 10-Q under the caption “Risk Factors.” If one or more of these factors materialize, or if any underlying assumptions prove incorrect, our actual results, performance 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.
RNAi Therapeutics

A New Class of Innovative Medicines

• Harness natural pathway
  ◦ Catalytic mechanism
  ◦ Mediated by small interfering RNA or “siRNA”

• Therapeutic gene silencing
  ◦ Any gene in genome
  ◦ Distinct mechanism of action vs. other drug classes
  ◦ Unique opportunities for innovative medicines

• Clinically validated platform
  ◦ Human POC in multiple programs
    – Papers in *NEJM* and *Lancet*
1. Liver-expressed target gene
   - Involved in disease with high unmet need
   - Validated in human genetics
   - GalNAc-siRNA enables SC dosing with wide therapeutic index

2. POC achieved in Phase 1
   - Blood-based biomarker with strong disease correlation
     - e.g., Serum TTR, thrombin generation, hemolytic activity, LDL-C, HBsAg levels

3. Definable path to approval and market
   - Established endpoints
   - Focused trial size
   - Large treatment effect
   - Collaborative approach with physicians, regulators, patient groups, and payers
Alnylam Strategic Therapeutic Areas (STAr)

**Genetic Medicines**
- Genetically validated liver targets for rare orphan diseases
- High unmet needs in focused markets
- SC dosing
- Alnylam direct commercial in NA and EU
- Genzyme alliance for ROW commercial  
  - Through end-2019

**Cardio-Metabolic Disease**
- Genetically validated liver targets for dyslipidemia, NASH, type 2 diabetes, and hypertension
- Development path in genetically defined, high unmet need subpopulations  
  - Access to larger populations thereafter
- Emerging genetics
- qM or qQ SC dosing
- Partnership opportunities

**Hepatic Infectious Disease**
- Liver pathogen and/or host targets
- Sub-acute duration of treatment (~12 mo)
- Multiple siRNAs possible, if needed
- Defined opportunities with very large markets
- qM or qQ SC dosing
- Partnership opportunities
# Alnylam Development Pipeline

<table>
<thead>
<tr>
<th>Category</th>
<th>Discovery</th>
<th>Development</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
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Transthyretin-Mediated Amyloidosis (ATTR) Program

Unmet Need and Product Opportunity

Progressive, debilitating monogenic disease
- ATTR is significant orphan disease
  - ~50,000 Patients worldwide
- Clinical pathology
  - Onset ~40 to >60 yr; fatal within 2-15 years
  - Two predominant forms
    - Familial amyloidotic polyneuropathy (FAP)
    - Familial amyloidotic cardiomyopathy (FAC)
- Halting disease progression remains unmet need
  - Liver transplantation required early
  - TTR stabilizers provide modest benefit

Mutant transthyretin (TTR) is genetic cause
- Autosomal dominant with >100 defined mutations
- Misfolds and forms amyloid deposits in nerves, heart, other tissues

RNAi opportunity as potentially transformative therapy
- Knockdown disease-causing protein
- Aim to halt progression, possibly achieve regression
- Value proposition supported by pharmacoeconomics and cost of disease burden
- Concentrated provider base and active patient community
RNAi Therapeutics for ATTR Amyloidosis
Patisiran and Revusiran

Patisiran for Familial Amyloidotic Polyneuropathy (FAP)
- Intravenous administration
- Positive Phase 2 results in FAP patients
- Phase 2 Open-Label Extension (OLE) study ongoing
  - Clinical endpoints every 6 months
  - Positive 12-month data reported at AAN, April 2015
  - 18-month data expected in late 2015
- APOLLO Phase 3 trial ongoing
  - Over 40 sites in over 15 countries
  - Expect APOLLO to enable NDA submission ~2017

Revusiran for Familial Amyloidotic Cardiomyopathy (FAC)
- Subcutaneous administration
- Positive Phase 2 study results
  - TTR cardiac amyloidosis patients
- Phase 2 Open-Label Extension (OLE) study ongoing
  - Clinical endpoints every 6 months
  - Report data at least once annually
- DISCOVERY study ongoing
  - Prevalence of TTR mutations in suspected cardiac amyloidosis
    - Multi-center study; up to 1,000 patients
- ENDEAVOR Phase 3 trial ongoing
Patisiran Phase 2 OLE Preliminary Study Results*
Change in mNIS+7 at 12 Months

Evidence that neuropathy progression halted at 12 months
• Mean decrease of 2.5 points observed
• Compares favorably with 13 to 18 point increase estimated from historical data sets
• Similar result in patients with/without concurrent TTR stabilizer therapy
• Sustained TTR knockdown through 16 months (mean max KD of 88%; max KD up to 96%); well tolerated out to 17 months
  ◦ No drug-related SAEs
  ◦ Mild flushing (22.2%) and infusion-related reactions (18.5%) most common adverse events
  ◦ No significant lab findings, no discontinuations

*Data as of March 13, 2015
Adams, AAN Apr. 2015
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<th>Diflunisal Phase 3</th>
<th>Patisiran Phase 2 OLE</th>
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<tr>
<td>ΔmNIS+7 at 6 mos</td>
<td>Mean (SEM)</td>
<td>PBO: 8.7 ± 2.0</td>
<td>PBO: 7.4 ± 6.9</td>
<td>-1.4 ± 2.1</td>
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<td>8.9 ± 5.7 (linear)</td>
<td>Drug: 2.5 ± 2.9</td>
<td>Drug: 2.3 ± 6.0</td>
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<td>10.3 ± 5.7 (non-linear)</td>
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<td>ΔmNIS+7 at 12 mos</td>
<td>Mean (SEM)</td>
<td>PBO: 17.3 ± 3.5</td>
<td>PBO: 12.6 ± 4.0</td>
<td>-2.5 ± 2.9</td>
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<td>NA (linear)</td>
<td>Drug: 6.6 ± 3.7</td>
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*Data as of March 13, 2015
Adams, AAN Apr. 2015
**Phase 3 Study Design**

**Patient Population**
- FAP: any TTR mutation, Stages 1 and 2
- Neurological impairment score (NIS) of 5-130
- Prior tetramer stabilizer use permitted

**Primary Endpoint**
- mNIS+7 at 18 months

**Secondary Endpoints**
- Norfolk QOL-DN
- NIS-weakness
- mBMI
- 10-meter walk test
- COMPASS-31

**Exploratory Endpoints**
- EQ-5D QOL
- NIS+7
- Serum TTR levels
- Cardiac assessments
- Grip strength
- Rausch-built Overall Disability Scale

**Statistical Considerations**
- Placebo-estimated mNIS+7 progression rate of 17.8 points/yr derived from natural history study of 283 FAP patients
- Study with 90% power to detect as little as 37.5% difference in ΔmNIS+7 between treatment groups with 2-sided alpha=0.05
- Blinded interim analysis of variance for sample size adjustment

*All completers eligible for patisiran treatment on Phase 3 OLE study*
Revusiran Phase 2 Study Results

Open label, multi-dose study in TTR cardiac amyloidosis patients

- Includes patients with FAC and senile systemic amyloidosis (SSA)
- Up to 98.2% TTR knockdown with similar effects toward WT and mutant protein
- Generally well tolerated
  - Transient mild ISRs in 15% patients; 1 SAE (LFT increase to ~4x ULN in 1 patient, resolved with continued dosing)
  - No study discontinuations and no changes in renal function, other lab chemistries, or hematologic parameters

Results as of March 15, 2015; Gilmore, ACC, March 2015
Patient Population
- Documented TTR mutation, including V122I or other
- Amyloid deposits on biopsy (cardiac or non-cardiac)
- History of heart failure
- Evidence of cardiac amyloid involvement by echocardiogram

2:1 RANDOMIZATION

Revasiran 500mg SC qD x 5, then qW for 18 mos

OR

Placebo SC qD x 5, then qW for 18 mos

Co-Primary Endpoints
- Change in 6-MWD at 18 months compared to baseline
- Percent reduction in serum TTR over 18 months

Secondary Endpoints
- Composite CV mortality and CV hospitalization
- Change in NYHA class
- Change in Kansas City Cardiomyopathy Questionnaire (KCCQ)

All completers eligible for revusiran treatment on Phase 3 OLE study

Statistical Considerations
- Placebo-estimated decline in 6-MWD of ~140 meters over 18 months in natural history study of 137 FAC patients (N=39 for 6-MWD data)
- 90% Power to detect as little as 39% difference in 18 mo change from baseline 6-MWD between treatment groups with significance level of p <0.05
- Un-blinded interim analysis for futility when ~50% of patients reach 18 mos
Hemophilia and Rare Bleeding Disorders Program
Unmet Need and Product Opportunity

High unmet needs in hemophilia and rare bleeding disorders (RBD)

• Hemophilias are recessive X-linked monogenic bleeding disorders
  ◦ Hemophilia A: loss of function in Factor VIII
    – >40,000 Patients in EU/U.S.
  ◦ Hemophilia B: loss of function in Factor IX
    – ~9,500 Patients in EU/U.S.

• Segments of high unmet need remain
  ◦ E.g., “Inhibitor” patients\(^1,2\)
    – 2,000 Patients in major markets; up to 6,000 WW
    – >15-25 Bleeds/year; >5 in-hospital days/year
    – ~$300,000/year avg. cost; up to $1M/year

• Hemophilia A and B represent >$9B market
  ◦ Premium pricing established
  ◦ Value supported by pharmacoeconomics
  ◦ Well organized patient advocacy
  ◦ Significant opportunity for global expansion

\(^1\) WFH 2012 Global Survey; \(^2\) Antunes et al., Haemophilia. 20:65-72 (2014)
Antithrombin and ALN-AT3 Program

Antithrombin (AT) is genetically defined target
- AT is key natural anticoagulant
  - Inactivates Factor Xa and thrombin
  - Attenuates thrombin generation
- Human AT deficiency associated with increased thrombin generation
- Expressed in liver; circulates in plasma

Co-inheritance of thrombophilic traits in hemophilia\(^1\)
- Associated with milder bleeding, reduced factor requirements, fewer complications
- Includes heterozygous
- Antithrombin deficiency
- Factor V\(_{\text{Leiden}}\)
- Protein C deficiency
- Protein S deficiency

ALN-AT3 in clinical development
- Extensive pre-clinical efficacy and safety data in hemophilia models\(^2\)
- Orphan drug status in U.S./EU (HA/HB)
- Positive initial Phase 1 results; new Phase 1 data at ISTH, June 20-25
- Additional data late ’15


\(^{2}\)Seghal et al., Nat Med, doi:10.1038/nm.3847
ALN-AT3 Phase 1 Study
Initial Evidence for Potential Correction of Hemophilia Phenotype

Potent and durable AT knockdown at low microgram/kilogram (mcg/kg) SC doses
• Up to 70% AT knockdown in hemophilia subjects in second dose cohort (45 mcg/kg); durable, lasting ~60 days

Increased thrombin generation
• Up to 334% increase in thrombin generation; mean increase of 112 ± 38% at AT knockdown >50% (p<0.05)

ALN-AT3 generally well tolerated in both healthy volunteers and hemophilia subjects
• No SAEs; all AEs mild or moderate, and transient; no discontinuations

Study ongoing; additional results expected in mid- and late 2015

Akinc, Goring Coagulation Conference, January 2015, Data as of Jan. 6, 2015
ALN-AT3 Phase 1 Study
Initial Evidence for Potential Correction of Hemophilia Phenotype

**Improved whole blood clotting as measured by thromboelastometry (ROTEM®)**
- Measures clotting time and clot strength in whole blood following tissue factor stimulus
  - Highly sensitive to coagulation defects
- In single, most advanced subject in 45 mcg/kg cohort, ALN-AT3 results in marked improvement

**Initial evidence for impact on bleeding**
- Subject remains bleed free for up to 47 days, compared to historical ABR of 10-12 bleeds/yr

**Method Description**

Qualitative interpretation (InTEG)

![Diagram of clotting process](image-url)
ALN-AT3 Phase 1 Study
Initial Evidence for Potential Correction of Hemophilia Phenotype

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<th>%AT KD</th>
<th>CT₁ (min)</th>
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<td><strong>Day 1</strong></td>
<td>1%</td>
<td>21 ± 5</td>
<td>24 ± 7</td>
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Qualitative interpretation (InTEG)

- **Normal**
- **Hemophilia**

Akinc, Goring Coagulation Conference, January 2015
Data as of Jan. 6, 2015

¹Clotting Time; ²Clot Formation Time
ALN-AT3 Phase 1 Study
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<td>Day 21</td>
<td>57%</td>
<td>9 ± 0.2</td>
<td>5 ± 0.1</td>
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</table>

$^1$Clotting Time; $^2$Clot Formation Time

Akinc, Goring Coagulation Conference, January 2015
Data as of Jan. 6, 2015
Complement Disease Program
Unmet Need and Program Opportunity

Wide range of complement-mediated diseases
- Excessive complement activity drives disease pathophysiology in many indications
  - Paroxysmal nocturnal hemoglobinuria (PNH)
  - Atypical hemolytic uremic syndrome (aHUS)
  - Neuromyelitis optica (NMO)
  - Myasthenia gravis (MG)
  - Many others
- Soliris™ (eculizumab) is blockbuster drug
  - >$1.5B in reported 2013 sales
  - >$2.1B in reported 2014 sales

New therapeutic options needed
- Consistent level of efficacy
  - Inadequate complement inhibition demonstrated in 49% of eculizumab-treated patients; correlated with poorer clinical outcomes¹
- SC delivery for more tolerable treatment regimen
- Reduce access barriers

Complement C5 and ALN-CC5 Program

Complement C5 is genetically validated target
- Key component of terminal pathway
  - C5 cleavage releases C5a; initiates membrane attack complex (MAC) formation
- Human C5 deficiency associated with minimal complications
  - Increased susceptibility to Neisserial infections
  - Also, many C5-deficient mouse strains
- Majority expressed in liver; circulates in plasma

Complement C5 is clinically validated target
- Eculizumab is anti-C5 Mab
- Approved in PNH and aHUS
  - In PNH, >80% inhibition of hemolytic activity associated with clinical benefit\(^1\)
- Potential advantages of synthesis inhibition vs. protein binding approach

ALN-CC5 in clinical development
- Pre-clinical efficacy in animal models
- Phase 1/2 study ongoing; Initial SAD Phase 1/2 data at EHA, June 11-14
- Additional data late ’15

\(^1\)Hillmen et al., NEJM; 350:552-9 (2004)
ALN-CC5 Pre-Clinical Efficacy
Potent C5 Knockdown and Complement Activity Inhibition

SC Administration of ALN-CC5 in non-human primates for over 7 months
• Potent dose-dependent C5 knockdown up to 99.2%
  ◦ Mean maximum knockdown of 98.4% ± 0.7%
  ◦ Expect qM dosing regimen in humans based on translation of ESC-GalNAc-siRNA conjugates
• Potent inhibition of complement activity up to 96.9%; closely correlated with C5 knockdown
  ◦ Mean maximum inhibition of CAP ELISA of 95.1% ± 0.93%
  ◦ Mean maximum inhibition of serum hemolytic activity of 88.0% ± 6.1%
ALN-CC5 Phase 1/2 Study
Dose-Escalation Study Including Patients with PNH

Study Design
- Randomized, double-blind, placebo-controlled SAD study in healthy volunteers (up to N=32)

Primary Objective
- Safety and tolerability of single dose in healthy volunteers

Secondary Objectives
- PK/PD and clinical activity
  - C5 levels, hemolysis suppression

Study Design
- Randomized, double-blind, placebo-controlled MAD study in healthy volunteers (up to N=28)

Primary Objective
- Safety and tolerability of multi-dose in healthy volunteers

Secondary Objectives
- PK/PD and clinical activity
  - C5 levels, hemolysis suppression

Study Design
- Open-label MAD study in PNH patients (N=8)

Primary Objective
- Safety and tolerability of multi-dose in PNH patients

Secondary Objectives
- PK/PD and clinical activity
  - C5 levels, hemolysis suppression
  - LDH reduction
ALN-CC5 Pre-clinical Efficacy
Single Dose Data in NHP

SC Administration of ALN-CC5 in non-human primates (0.2, 1, 5, 25 mg/kg)
- Potent dose-dependent C5 knockdown up to 97.8%; mean maximum knockdown of 95.9% ± 1.4%
- Dose-responsive inhibition of complement hemolytic activity
Acute Intermittent Porphyria (AIP) Program
Unmet Need, Product Opportunity, and Program Status

**AIP is autosomal dominant disorder**
- Ultra-rare orphan disease
  - ~5,000 Patients with annual attacks U.S./EU
  - ~500 Patients with recurrent attacks U.S./EU

**High unmet need and cost**
- Patients present with acute or recurrent attacks
- Limited treatment options
  - Blood-derived hemin given IV via central line
  - No prophylactic treatment to prevent attacks

**Opportunity to treat and prevent porphyria attacks**
- Orphan disease with substantial morbidity
- Value supported by significant burden of disease
- Study ongoing in patients
  - Prospective observational study to monitor attacks

**RNAi therapeutics to halt disease symptoms**
- Targets ALAS-1, upstream of genetic defect
- Blocks toxic intermediates (ALA/PBG)
- ALN-AS1 to treat and/or prevent attacks

**ALN-AS1 in clinical development**
- Proof of concept and efficacy in animal models
- Phase 1 study ongoing; initial data early ’16
AAT Deficiency-Associated Liver Disease
Unmet Need, Product Opportunity, and Program Status

**Alpha-1 liver disease is autosomal disorder**
- Commonest mutant allele (PiZZ) homozygosity results in liver cirrhosis
- Z-AAT protein misfolds and aggregates in hepatocytes
  - Leads to liver injury, fibrosis, cirrhosis, hepatocellular carcinoma (HCC)
- Rare orphan disease
  - ~120,000 people in U.S./EU homozygous for Z allele
  - ~10% have associated liver pathology (alpha-1 liver disease)

**High unmet need and cost**
- Occurs in both children and adults
- Limited treatment options
  - Supportive care
  - Liver transplantation (in case of advanced cirrhosis)

**Opportunity to treat and prevent alpha-1 liver disease**
- Orphan disease with substantial morbidity
- Value supported by significant burden of disease

**RNAi therapeutics to prevent synthesis of disease-causing protein**
- ALN-AAT targets alpha-1-antitrypsin gene in PiZZ patients

**ALN-AAT in clinical development**
- Proof of concept and efficacy in animal models
- CTA filed; Phase 1 start late ’15
- Initial data in early ’16
Genetic Medicine Partnership
Global Commercialization for Value Creation

Transformational alliance to expand and accelerate global product value

- Includes Alnylam Genetic Medicine products that achieve human POC through end-2019
- $700M upfront investment; 12% Alnylam ownership
- Alnylam retains North America and EU
  - Leads development and commercialization
- Genzyme obtains ROW and certain additional rights
  - Includes co-development and co-commercialization for revusiran in NA/EU
  - Alnylam receives R&D funding, milestones, and royalties
# Alnylam Development Pipeline

<table>
<thead>
<tr>
<th>GENETIC MEDICINES</th>
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# CARDIO-METABOLIC DISEASES

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# HEPATIC INFECTIOUS DISEASES

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</table>
PCSK9 and ALN-PCSsc Program

Unmet need in hypercholesterolemia

- Elevated LDL-C validated risk factor for coronary heart disease (CHD)
- 34 million Americans have hypercholesterolemia (> 240 mg/dL)
- Recent clinical studies
  - Many patients on statins do not meet LDL-C goal
  - Lower LDL-C is better (IMPROVE-IT)
- Multiple genetically defined patient subgroups

PCSK9 is genetically validated target

- GOF mutations associated with hypercholesterolemia and premature CHD
- LOF mutations associated with hypocholesterolemia and decreased CHD risk

Blazing e al Am. Heart J; 168:205 (2014)
ALN-PCSsc Pre-Clinical Efficacy
Potent PCSK9 Knockdown and LDL-C Lowering

Highly durable PCSK9 knockdown and LDL-C reduction
- Single dose in NHP
- Up to 96% PCSK9 knockdown, up to 77% LDL-C lowering; absence of statins
- Highly durable effects, supports once-monthly or possibly once-quarterly dosing
  - >50% LDL-C lowering maintained for over 3 months in 10 mg/kg group
ALN-PCSsc Phase 1 Study

Study Design
• Randomized, single-blind, placebo-controlled, single ascending dose (SAD) and multi-dose (MD), subcutaneous dose-escalation study
  ◦ 3:1, ALN-PCSsc vs. placebo
• Up to 76 volunteer subjects with elevated baseline LDL-C (≥100 mg/dL)
  ◦ MD phase to also include subjects both on and off statin co-medication

Treatment Regimen
• SD or MD (qM x 2)

Study Objectives
• Primary: safety and tolerability
• Secondary: PK, clinical activity (knockdown of PCSK9 compared to baseline and % reduction of LDL-C)

Status
• Phase 1 enrolling
• Initial clinical data expected mid-’15
  • ESC meeting, August 29-September 2
# Alnylam Development Pipeline

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Hepatitis B Virus (HBV) Infection

Chronic HBV (CHB) infection is significant WW problem
- One third of world population infected
- 400M people with chronic disease
  - 25M in U.S./EU
- Most unaware of infection
- High prevalence expected for next 3 decades
  - Despite availability of HBV vaccine

Clinical manifestations severe
- Chronic inflammation leading to cirrhosis and hepatocellular carcinoma (HCC)

Current therapies not curative and have significant limitations
- Reduce viral load, resulting in improved liver histology, decrease in cirrhosis and HCC but do not eliminate virus

Future therapies aim to enable “functional cure”
- Regain sustained immune control over infection, with eventual elimination of viral cccDNA

http://www.hepb.org/hepb/statistics.htm
**RNAi Therapeutics for HBV**

**Efficacy in HBV-Infected Chimpanzees**

**Dose-dependent antiviral response with intra-subject ascending doses**

- Mean $2.9 \log_{10}$ decrease in viral DNA day 2-6 post 0.5 mg/kg dose
  - $>4 \log_{10}$ reduction in circulating viral DNA achieved in highest titer animal
- Mean $2.0 \log_{10}$ reduction in HBsAg at 0.5 mg/kg dose
  - Up to 2.3 $\log_{10}$ reduction achieved

---

*low titer animal dropped below LLOQ from day 23-day 98

Meyers, TIDES, May 2014
ALN-HBV Development Candidate (DC)
Potent ESC-GalNAc-Conjugate for SC Administration

Potent, multi-log HBsAg knockdown in murine model

- Mouse model with AAV-HBV vector
- ALN-HBV DC achieves potent knockdown of HBsAg
  - Single SC dose of siRNA at 3 mg/kg; specificity confirmed with control siRNA
  - Up to $3.9 \log_{10}$ reduction; mean $1.8 \log_{10}$ reduction 5-10 days after single dose
- IND filing expected in late ’15

![Graph showing HBsAg levels over time for ALN-HBV and Control siRNA](image)
Guidance and Goals
3 STArs
3 Marketed Products
10 Clinical Programs
4 Late Stage Programs
<table>
<thead>
<tr>
<th><strong>Alnylam 2015 Pipeline Goals</strong></th>
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<tbody>
<tr>
<td><strong>2015</strong></td>
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<tr>
<td><strong>Early</strong></td>
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* Early is Q1-Q2, Mid is Q2-Q3, and Late is Q3-Q4; **IND or IND equivalent*

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40
## Select Scientific and Clinical Meetings
### Mid-’15

<table>
<thead>
<tr>
<th>Code</th>
<th>Meeting Name</th>
<th>Date/Location</th>
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<tbody>
<tr>
<td>AAT</td>
<td>Digestive Disease Week (DDW)</td>
<td>May 16-19 (Washington DC)</td>
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<tr>
<td>CC5</td>
<td>European Hematology Association (EHA)</td>
<td>June 11-14 (Vienna)</td>
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<tr>
<td>AT3</td>
<td>International Society of Thrombosis and Haemostasis (ISTH)</td>
<td>June 20-25 (Toronto)</td>
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<tr>
<td>PCSsc</td>
<td>European Society of Cardiology (ESC)</td>
<td>August 29-September 2 (London)</td>
</tr>
<tr>
<td>GO1</td>
<td>European Society for Paediatric Nephrology (ESPN)</td>
<td>September 3-5 (Brussels)</td>
</tr>
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</table>
Financial Summary and Guidance

2015 Q1 Financial Results

• Cash ~$1.45B
• GAAP Revenues $18.5M
• Total GAAP Operating Expenses $70.7M
  ◦ Research and Development Expense $58.0M
  ◦ General and Administrative Expense $12.7M
• GAAP Net Loss of $50.8M
• Shares Outstanding ~84.2M

2015 Guidance

• Year-end cash >$1.2B
Thank You

www.alnylam.com