ALN-GO1, an Investigational RNAi Therapeutic for the Treatment of Primary Hyperoxaluria Type 1 (PH1)

September 8, 2015
Agenda

Welcome
• Joshua Brodsky  
  Senior Manager, Investor Relations and Corporate Communications

Introduction
• Barry Greene  
  President, Chief Operating Officer

Overview of Primary Hyperoxaluria Type 1
• Sally-Anne Hulton, M.D., FRCPCH, MRCP, FCP, MBBCh, Consultant Paediatric Nephrologist and Clinical Lead, Birmingham Children’s Hospital NHS Trust

Patient Advocacy: Hyperoxaluria Patient Perspective
• Kim Hollander, Executive Director, Oxalosis & Hyperoxaluria Foundation

Q&A Session
• With Dr. Hulton and Kim Hollander

ALN-GO1 Program
• David Erbe, Ph.D., Director, Research

Q&A Session
Reminders

• Event will run for approximately 60 minutes
• Q&A Session at end of each presentation
  ◦ Submit questions at top of webcast screen
  ◦ Questions may be submitted at any time
• Replay, slides and audio available at www.alnylam.com
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.
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Q&A Session
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
# Development Pipeline

## Genetic Medicines

<table>
<thead>
<tr>
<th>Condition</th>
<th>Discovery</th>
<th>Development</th>
<th>Phase 1</th>
<th>Phase 2</th>
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## Cardio-Metabolic Diseases

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## Hepatic Infectious Diseases

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Q&A Session
Primary Hyperoxaluria

Sally-Anne Hulton

Birmingham Children's Hospital UK
Clinical presentation & diagnosis

asymptomatic → stones single or recurrent → nephrocalcinosis → renal impairment

Stone in urine

Stone to laboratory for analysis

Calcium Oxalate present

Specific blood and urine tests
Oxalate synthesis

**peroxisome**
- serine \( \xrightarrow{AGT} \) hydroxypyruvate
- pyruvate \( \xrightarrow{AGT} \) alanine
- glycine \( \xrightarrow{GO} \) glyoxylate

**cytosol**
- hydroxypyruvate \( \xrightarrow{GRHPR} \) D-glycerate
- NADPH \( \xrightarrow{GRHPR} \) NADP+

**mitochondrion**
- oxalate
- \( \xrightarrow{LDH} \) glycollate
- \( \xrightarrow{GRHPR} \) glyoxylate

\( \xrightarrow{HOGA} \) glycollate

- hydroxyproline \( \xrightarrow{？GRHPR} \) 4-OH-oxoglutarate
- \( \xrightarrow{？GRHPR} \) pyruvate + glyoxylate
PH mutations and diagnosis

- PH1  AGXT gene chr 2
  
  *PH1 incidence 6 to 7 per million population*

- PH2  GRHPR gene chr 9

- PH3  HOGA1 gene chr10

- PH1  $\rightarrow$ plasma oxalate + glycollate
  $\rightarrow$ urine oxalate

- PH2  $\rightarrow$ urine L-glyceric acid
  (may be absent)

- PH3  $\rightarrow$ urine oxalate + glycollate
  $\rightarrow$ urine Ca + uric acid

*Database of pathological mutations + polymorphic variants*
http://www.uclh.nhs.uk/phmd
Age of onset: comparative UK data: PH1, 2 and 3

% PH Patients

Age of onset in years

- <1y
- 1-4.9
- 5-9.9
- 10-14.9
- 15-19.9
- 20-39.9
- 40-80

PH1
PH2
PH3
Consequences of PH1

• Phenotypic variability

• Systemic deposition of Ox in all organs
  – bones
  – EPO resistant anaemia
  – retina
  – myocardium AV block

• Progressive renal impairment
Consequences of PH1

Female aged 9 years with calcification of kidneys, marked osteopenia. Pin in femoral neck following fracture.

Oxalate crystals on fundoscopy and in retina on post mortem
64 out of 449 patients (14%) died

J Harambat on behalf of OXIAI EUROPE
Early therapy

- affects long term outcome
- 20/27 PH1 children stable GFR over 20yrs
- document genetic data
- allows time to reflect on phenotypic variability

*Fargue S et al* *KI* 2009; 76: 767-73
Conservative treatment

1. Minimize absorption of oxalate
   – Avoid Vit C / high oxalate foods
   – *Oxalobacter formigines*

2. Reduce oxalate synthesis
   – Vit B6 pyridoxine
   – Gly170Arg or Phe1 52Ile mutation

3. Minimize oxalate deposition in kidney
   – $\uparrow$ Fluid intake 2.5 l/m²/day minimum
   – $\downarrow$ Salt Na⁺ intake
   – Potassium citrate to reduce urine acidity
On-going management

Annual GFR ml/min/1.73m²

• If > 60 → stable

• If 40-60 → consider isolated liver Tx
  review genotype Gly170Arg

• If < 40 → combined liver/kidney Tx
Dialysis in PH

• Unable to reduce oxalate load
• Weekly clearance = 6-9 mmol/wk/SA equivalent to 2 days of endogenous oxalate production
• Haemo clearance better at 120ml/min than PD at 7ml/min

Perit Dial Int 1994; 14: 81-84
NDT 2001; 16: 2407-11
KI 2006; 70: 1642-8
Isolated liver transplant

- ? Timing + genotype Gly170Arg
- Drugs compromise renal function
- Possible subsequent renal Tx

Patient survival in pre-emptive Liver Tx

- 82% at 10 yrs of age
- 72% at 20 yrs of age
- 5 out of 24 (21%) patients died

J Harambat on behalf of

Ped Transpl 2000; 4: 177-81
Perera T et al NDT 2010; Aug
Hepato-renal transplantation

- **Combined**
  - Total hepatectomy required
  - 80% patient 5yr survival with GFR of 40-60
  - Risk factors:
    - age < 5 yrs
    - dialysis > 2 yrs
    - poor renal graft function

- **Sequential**
  - liver 1\textsuperscript{st} then kidney
    - advantage of early liver replacement of AGT
    - can delay renal Tx
  - cadaveric or LRD or combination

*Ellis et al. NDT 2001; 16: 348-54*
*Heffron TG et al. Ped Transpl 2009;13:805-7*
*Harambat J et al. KI 2010; 77: 383-5*
*Brinkert F et al. Transplant 2009; 15: 1415-21*
Patient survival after combined hepatorenal transplant

- 82% at 5 yrs
- 74% at 10 yrs
- 66% at 20 yrs

24 out of 113 (21%) patients died
Current problems

• Management of recurrent stones

• Oxalate deposition in the transplanted kidney
  – sometimes immediate graft loss

• Management of recurrent bone fractures

• Coordinated care for multisystem disease
Current Research

• Gene therapy
  – In vivo (gene delivered by vector)
  – Ex vivo on induced pluripotential stem cells (iPSC) together with hepatocellular Tx

• Systems development
  – Mouse knockout – single or multiple:
    AGT / GRHRP / HOGA1 / GO
RNAi therapeutics

• New class of innovation
• Harnesses natural pathway mediated by small interfering RNA siRNA
• Therapeutic gene silencing
• Clinically validated in humans
• Current research
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Q&A Session

A Past with Uncertainty –
A Future with Hope
Primary Hyperoxaluria

“Of 330 patients, 50% had ESRD by 15 years of age and 80% by the third decade.”

Latta and Brodehl, Eur J. Pediatr 1990
Oxalosis & Hyperoxaluria Foundation

Connecting the Dots

- Advocate
- Empower
- Support
- Engage
- Hope
- Research
- Educate
- Awareness
Timeline

1986
- AGT deficiency in PH1 discovered
- First enzyme diagnosis of PH1
- First liver-kidney transplantation in PH1

1987
- First enzyme prenatal diagnosis of PH1

1988
- GR deficiency in PH2 discovered

1989
- OHF founded

1990
- Human AGXT gene cloned & mutations identified in PH1

1995
- First prenatal diagnosis of PH1 by DNA analysis

1997
- OHF funded first research grant
- OHF.org launched

1999
- Human GRHPR gene cloned & identification of first mutations in PH2

2000
- OHF Talk began
- SAB was formed

2003
- Crystal structure of AGT solved
- OHF opens Mayo Clinic Hyperoxaluria Center

2004
- First Patient Day at Mayo Clinic

2006
- OHF began globalization in Germany
- Crystal structure of GR solved
- AGXT knockout mouse generated
- SAC formed

2007
- GRHPR knockout mouse generated

2008
- PAB formed

2009
- Joined Facebook Causes
- Take the Challenge campaign

2010
- HOXA1 deficiency in PH3 discovered

2011
- Structure of HOXA1 solved

2012
- Mechanism of action of pyridoxine deciphered

2014
- FDA-approved drug counteracts effects of mutant AGT in cells
From a Patient’s Perspective

Become an Organ Donor.
DEDICATED TO FINDING THE CURE

IT TAKES A VILLAGE
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Glycolate Oxidase (GO) Knockdown to Starve Substrate for Oxalate Synthesis in PH1

- Human GO deficiency well tolerated → provides validation through increased glycolate excretion
  - 8-yr old boy with homozygous GO loss-of-function identified by Dr. Yaacov Frishberg
  - 20x increase in glycolate, normal oxalate, normal kidneys, no nephrocalcinosis
- GO deficient mice also validate therapeutic approach (Dr. Eduardo Salido)
  - Breeding with PH1 disease mice (AGT deficient) substantially resolves Uox levels

Urinary Oxalate (Uox)
ALN-GO1 in Normal and Diseased Mice
Potent mRNA Silencing, Substantial Efficacy with Durability

**Normal mice (single dose)**

- HAO1 mRNA (relative to PBS)
- Serum glycolate (μM)

**PH1 mice (single dose)**

- Urine Oxalate (mg/g Creatinine/24 hr)
- Urine Glycolate (mg/g Creatinine/24 hr)

In collaboration with University of Alabama, Birmingham
ALN-GO1 Substantially Lowered Urinary Oxalate in Rat PH1 Model

Oxalate decreased up to 98% following weekly dosing

Note: >95% HAO1 mRNA silencing at all doses
Single, Low Dose ALN-GO1 in PH1 Rats
1:1 Relationship of Oxalate Lowering to HAO1 mRNA Silencing

Demonstrates substantial potential for efficacy
ALN-GO1 in Non-human Primates (ongoing)
Potent mRNA Silencing, Expected Increases in Serum Glycolate

Up to 99% silencing of HAO1 mRNA in non-human primates

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<th>Group #</th>
<th>Dose Level (mg/kg)</th>
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ALN-GO1
Summary and Next Steps

• Pre-clinical data summary
  ◦ Potent, durable silencing of HAO1 mRNA across species
    – Translates into expected increases in serum glycolate in healthy animals
  ◦ Profound lowering of urinary oxalate in animal models of PH1
    – 1:1 relationship of oxalate lowering to mRNA silencing

• Next steps
  ◦ Plan to file CTA in late 2015 and start Phase 1 study in early 2016 to study safety, along with impact on glycolate and oxalate metabolism
    – Pre-clinical durability supports monthly, and potentially quarterly, subcutaneous dosing
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Q&A Session
Upcoming RNAi Roundtables

ALN-PCSsc for the treatment of Hypercholesterolemia

*Wednesday, September 16, 9:30 – 10:30 a.m. ET*

- Kevin Fitzgerald, Ph.D., Vice President, Research
- David Kallend, MBBS, Vice President and Global Medical Director, The Medicines Company
- Moderator: Barry Greene, President and Chief Operating Officer
- Guest Speaker: Marc S. Sabatine, M.D., M.P.H., Chairman, Thrombolysis in Myocardial Infarction (TIMI) Study Group at Brigham and Women’s Hospital, Lewis Dexter, MD Distinguished Chair in Cardiovascular Medicine, and Professor of Medicine, Harvard Medical School

ALN-AS1 for the treatment of Acute Hepatic Porphyrias

*Tuesday, September 24, 11:00 a.m. – 12:00 p.m. ET*

- Bill Querbes, Ph.D., Associate Director, Research
- Moderator: John Maraganore, Ph.D., Chief Executive Officer
- Guest Speaker: Robert J. Desnick, M.D., Ph.D., Dean for Genetics and Genomic Medicine, Professor and Chair Emeritus, Department of Genetics and Genomic Sciences, Icahn School of Medicine at Mount Sinai Hospital

Replays, presentations, transcripts of all RNAi Roundtables available at www.alnylam.com/capella
Speaker Biographies

Barry Greene
President and Chief Operating Officer, Alnylam

Barry Greene joined Alnylam in 2003, and brings over 25 years of experience in healthcare, pharmaceutical, and biotechnology industries. Prior to Alnylam, he was General Manager of Oncology at Millennium Pharmaceuticals, Inc., where he led the company’s global strategy and execution for its oncology business including strategic business direction and execution, culminating in the successful approval and launch of VELCADE™ (bortezomib) in mid 2003. Prior to joining Millennium in February 2001, Barry served as Executive Vice President and Chief Business Officer for Mediconsult.com. Prior to Mediconsult.com, his past experiences include Vice President of Marketing and Customer Services for AstraZeneca, formerly AstraMerck; Vice President Strategic Integration with responsibility for the AstraZeneca North American post merger integration; and Partner, Andersen Consulting responsible for the pharmaceutical/biotechnology marketing and sales practice. Barry received his B.S. in Industrial Engineering from University of Pittsburgh and served as Senior Scholar at Duke University, Fuqua School of Business. Barry also serves on the Boards of Acorda Therapeutics and Karyopharm Therapeutics.

Sally-Anne Hulton, M.D., FRCPCH, FRCP, MRCP, FCP, MBBCh
Consultant Paediatric Nephrologist and Clinical Lead, Birmingham Children’s Hospital NHS Trust

Dr. Hulton was appointed Consultant Paediatric Nephrologist at Birmingham Children’s Hospital NHS Foundation Trust and Honorary Senior Lecturer at University of Birmingham UK in 1995. In this position she has served as lead physician for both combined liver kidney transplantation and renal metabolic disease. She has developed particular expertise in the primary hyperoxalurias in this post and leads the national UK registry for this disorder. She has also served as Vice Chair of the hospital Ethics Committee and Chair of College Specialist Advisory Committee (CSAC) for Paediatric Nephrology and Specialty Training Advisor for the Royal College of Paediatrics and Child Health (RCPCH). Dr. Hulton received her degree in Medicine and Surgery (MBBCh) from the University of the Witwatersrand, South Africa, her doctoral thesis was awarded from the University of Birmingham, UK following research at the Institute of Child health in London. She conducted her fellowship in Paediatrics at the Colleges of Physicians of South Africa (FCP SA) and the Royal College of Paediatrics and Child Health (FRCPCH). Dr. Hulton is also a fellow of the Royal Colleges of Physicians for the United Kingdom (FRCP).

Kim Hollander
Executive Director, Oxalosis & Hyperoxaluria Foundation (OHF)

Kim Hollander has served as Executive Director of the Oxalosis & Hyperoxaluria Foundation (OHF) since December 2002 where she has overall strategic and operational responsibility for OHF programs and execution of its mission to improve the care and treatment for Oxalosis, Primary Hyperoxaluria and other hyperoxaluria related stone diseases. In addition to initiating and maintaining relationships within the Hyperoxaluria community, Kim manages expansion of OHF initiatives into international markets and creates programming to enhance development efforts. Ms. Hollander also serves on the Board of the National Organization for Rare Diseases (NORD), a non-profit organization focused on providing a unified voice for people living with rare diseases, a position she has held since January 2013. Prior to joining OHF, Kim held various teaching and coaching positions within the New Jersey public school system. Ms. Hollander is a graduate of West Virginia University.

David Erbe, Ph.D.
Director, Research, Alnylam

Dave Erbe joined Alnylam in September 2014 with over 20 years’ experience in drug discovery and development, and currently serves as the Project Leader for the ALN-GO1 program. Prior to Alnylam, he was most recently Head of Product Development for Pronutria Biosciences, and Director of Discovery Research at Eleven Biotherapeutics. Dave’s career began with postdoctoral training in protein engineering at Genentech before joining Genetics Institute (now a part of Pfizer) and serving in positions of increasing responsibility, translating novel therapeutics from discovery through clinical proof-of-concept across a range of diseases. Dave received a Ph.D. in Biochemistry from Dartmouth following a B.S. in Chemistry from Carnegie Mellon.
Thank You

www.alnylam.com