



Stifel Nicolaus Healthcare Conference

September 2012

Anylam Forward Looking Statements

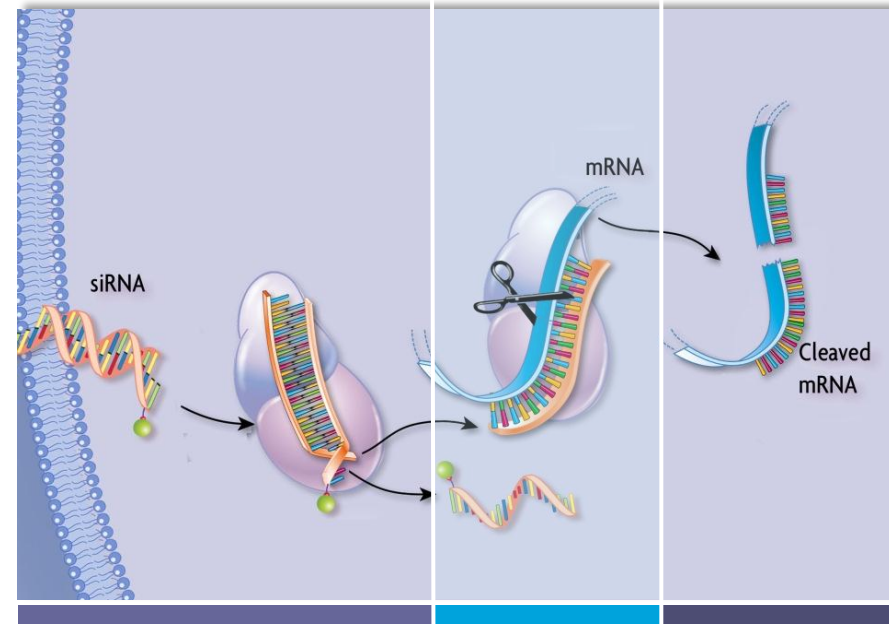
This presentation contains forward-looking statements, within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. There are a number of important factors that could cause actual results to differ materially from the results anticipated by these 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.

RNA Interference (RNAi)

A New Class of Innovative Medicines

RNAi Therapeutics

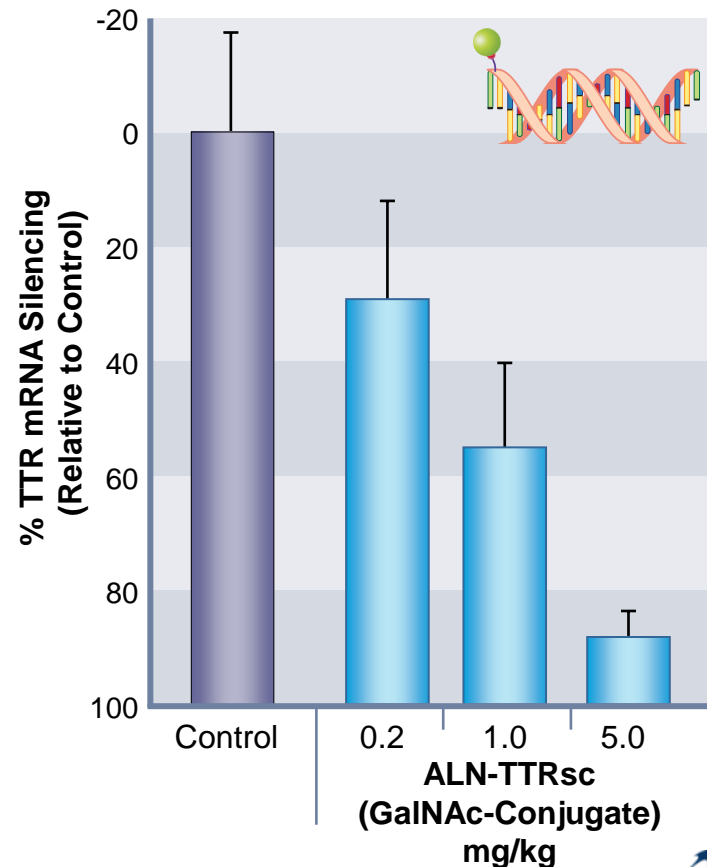
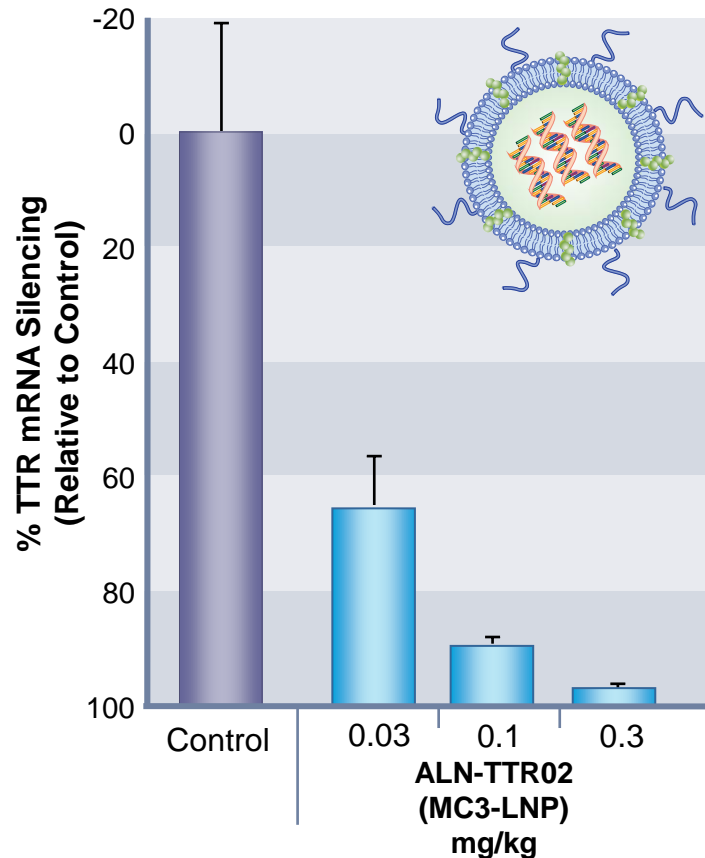
- Harness natural pathway
 - » Catalytic mechanism
 - » Mediated by small interfering RNAs or “siRNAs”
- Treat disease with therapeutic gene silencing
 - » Any gene in genome
 - » Creates unique opportunities for innovative medicines



RNAi Delivery to Liver Solved IV and SC Platforms

Enables advancement of innovative medicines to patients

- Potent, rapid, and durable target gene silencing with proprietary lipid nanoparticle (LNP) technology and IV dosing
- Advancement of conjugate platform for clinical translation and SC dosing



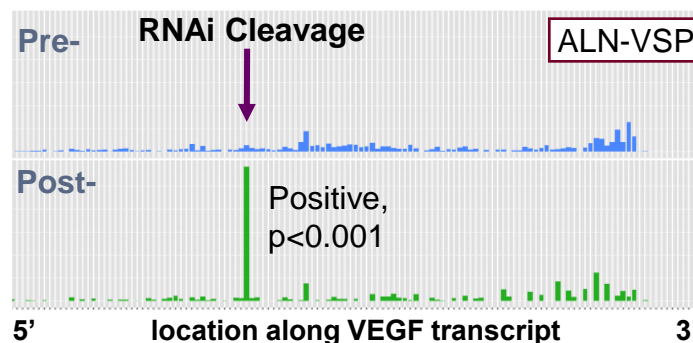
RNAi Human Translation Achieved

Multiple Alnylam Therapeutic Programs

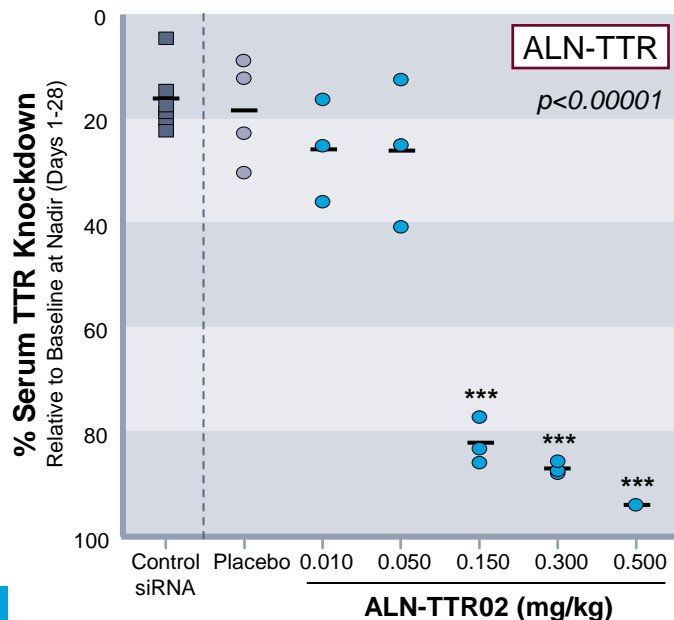
1. Human Safety and PK

- >575 Subjects/patients enrolled overall
- Systemic delivery in human trials
 - » ~100 Patients dosed
 - » >325 Doses administered
 - » >26 months of dosing
- RNAi therapeutics generally well tolerated
- Pharmacologically relevant human tissue levels achieved

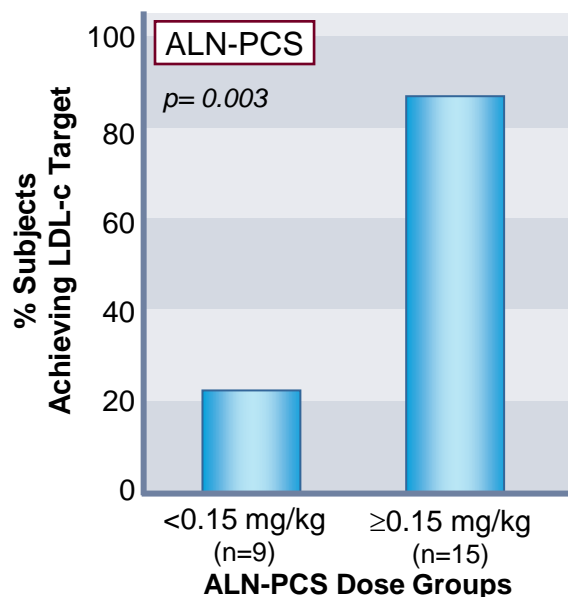
2. Human Proof of Mechanism



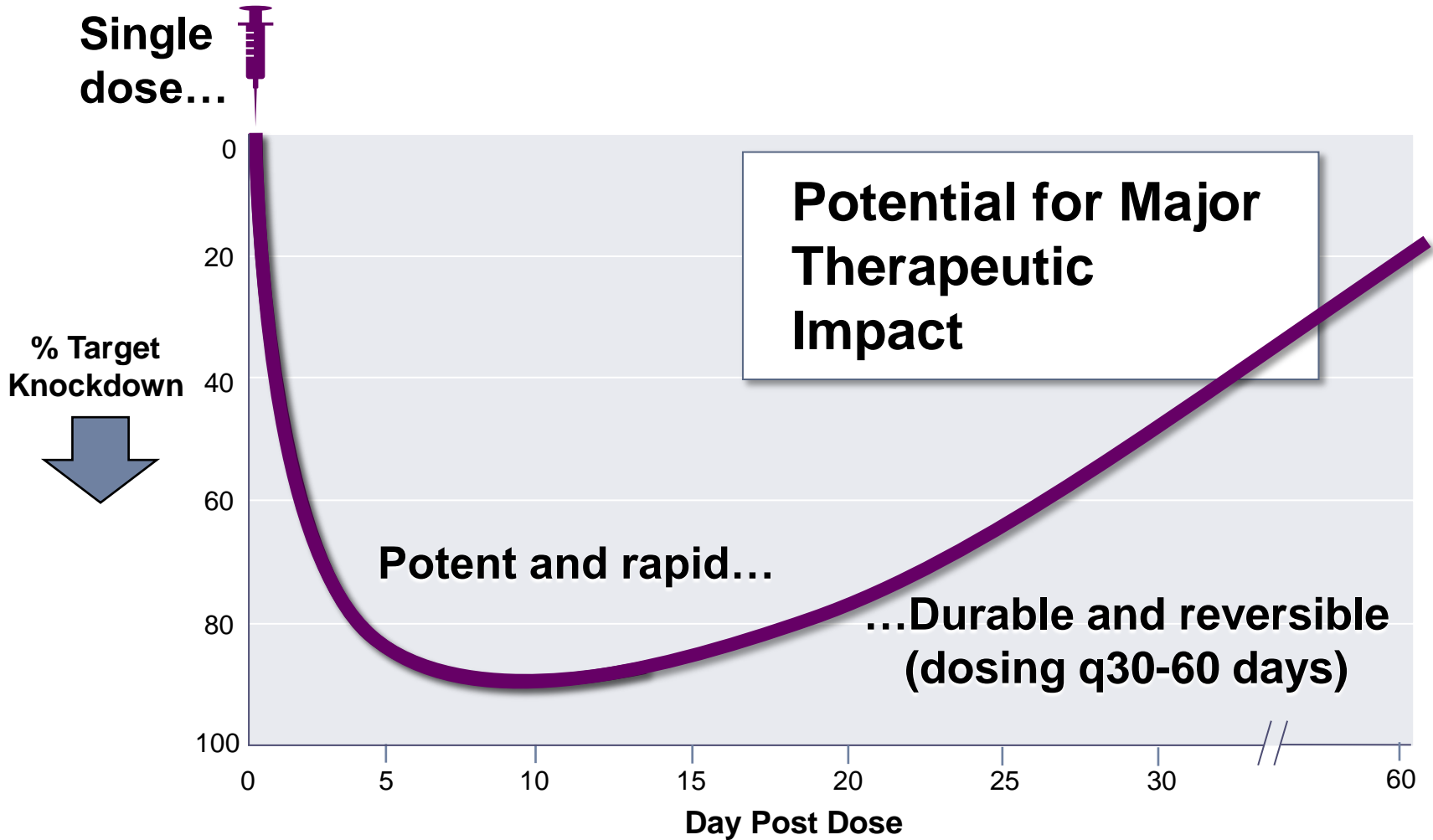
3. Human Proof of Concept



4. Human Clinical Efficacy



RNAi Therapeutics Profile



Focused Product Development Pipeline for Innovative Medicines

RNAi for genetically defined disease as core strategy

- 5 Key products in clinical development through 2015
- Near term focus on TTR and AT3 programs

Product characteristics

- ✓ Genetically defined target/disease
- ✓ Existing Alnylam delivery platform
- ✓ Early biomarkers in Phase I
- ✓ Clear and rapid development
- ✓ Significant commercial opportunity



Alnylam Goals

Pipeline Goals 2012-2013

		IND/Phase I	Clinical Data	Phase II	Pivotal
Alnylam 5x15 Programs	ALN-TTR02	✓	✓	✓	2013
	ALN-TTRsc	H2 '12	H1 '13		
	ALN-AT3	2013			
	ALN-PCS	✓	✓	Partner	
	ALN-HPN	Partner			
	ALN-TMP	Partner			
Partner Programs	ALN-RSV01	✓	✓	✓	
	ALN-VSP	✓	✓	✓/Partner	

Additional Corporate Goals 2012

- Additional partnerships
- Year-end Cash >\$250M



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Thank You

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