Cantor Fitzgerald Global Healthcare Conference
October 2, 2018

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Safe Harbor Statement

This presentation contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These statements are based upon our current expectations and speak only as of the date hereof. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of various factors and uncertainties, including, without limitation, our developmental stage and limited operating history, our ability to successfully and timely develop products, enter into collaborations and achieve other projected milestones, rapid technological change in our markets, demand for our future products, legislative, regulatory and competitive developments and general economic conditions. Our Annual Report on Form 10-K, recent and forthcoming Quarterly Reports on Form 10-Q, recent Current Reports on Forms 8-K, and other SEC filings discuss some of the important risk factors that may affect our ability to achieve the anticipated results, as well as our business, results of operations and financial condition. Readers are cautioned not to place undue reliance on these forward-looking statements. Additionally, Arrowhead disclaims any intent to update these forward-looking statements to reflect subsequent developments.

ARWR Profile

Arrowhead is a **small cap RNAi therapeutics platform company** with a **broad pipeline** of **wholly owned and partnered** product candidates targeting both **mass market and rare diseases**

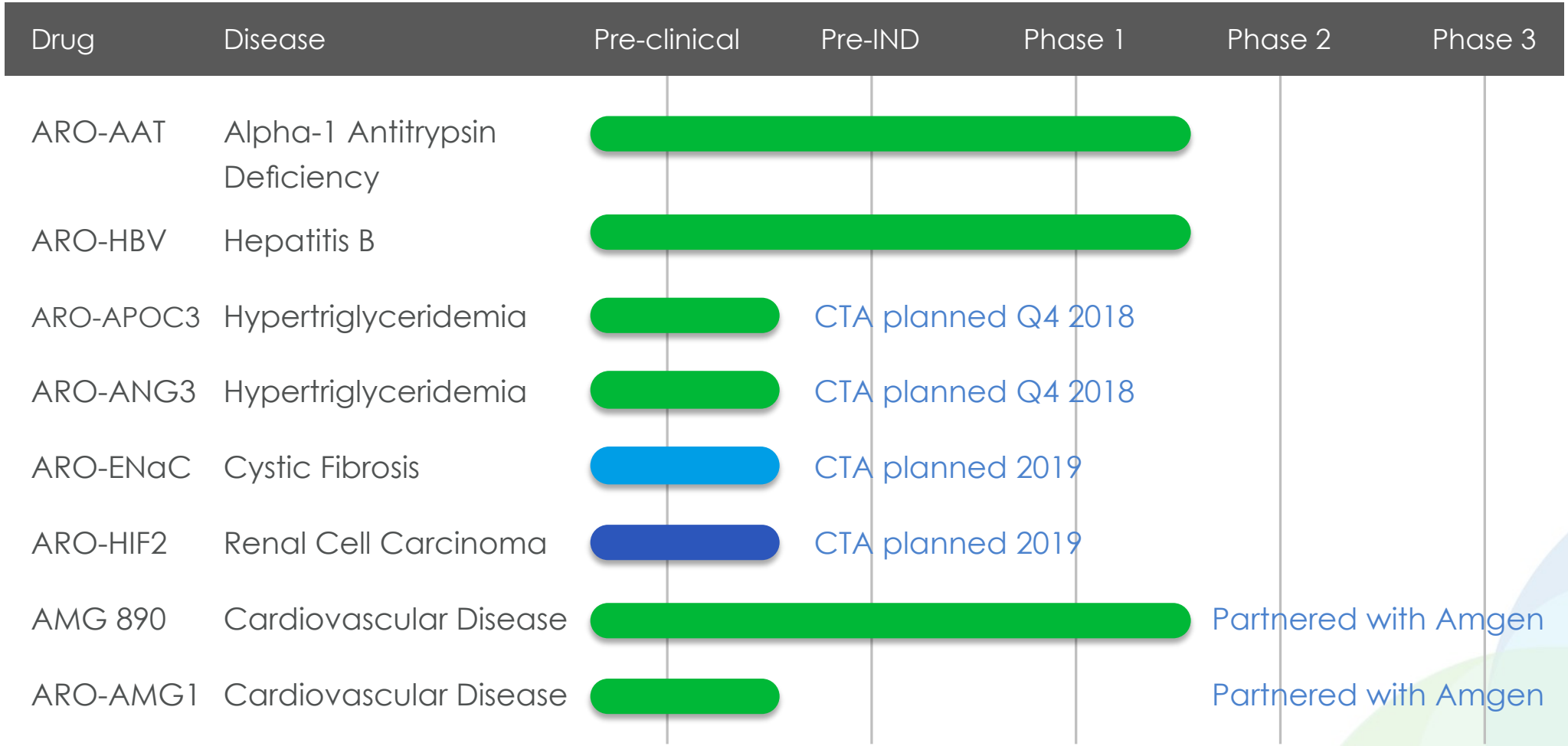
Our mission is to treat intractable medical conditions by **silencing the genes** that cause them

Broad Pipeline:	• Lead clinical programs in **Phase 1/2** against **hepatitis b** virus infection and **alpha-1 antitrypsin deficiency**-related liver disease • Potential to **grow** pipeline from **0 to 5** clinical programs in 2018
Proprietary Platform:	• **Targeted RNAi Molecules** platform **(TRiM™)** • Potential to be **best in class** for liver diseases and to **fulfill the promise** of bringing RNAi therapeutics to **diseases outside of the liver**
Partnership Opportunities:	• Two product cardiovascular deal with **Amgen** • Potential for **additional** product and/or platform **deals**

Financial Highlights

ARWR - NASDAQ Global Select	
Stock Price (a/o 9/28/18)	$19.17
Common Shares Outstanding	~87.9m
Market Capitalization (a/o 9/28/18)	~$1.7b
Cash (a/o 10Q 6/30/18)	~$78m (not including $10m milestone earned from Amgen)
Quarterly Cash Burn (range for last 4 quarters)	~$11-15m

Pipeline


Drug
Disease
Pre-clinical
Pre-IND
Phase 1
Phase 2
Phase 3
ARO-AAT
Alpha-1 Antitrypsin Deficiency
ARO-HBV
Hepatitis B
ARO-APOC3
Hypertriglyceridemia
CTA planned Q4 2018
ARO-ANG3
Hypertriglyceridemia
CTA planned Q4 2018
ARO-ENaC
Cystic Fibrosis
CTA planned 2019
ARO-HIF2
Renal Cell Carcinoma
CTA planned 2019
AMG 890
Cardiovascular Disease
Partnered with Amgen
ARO-AMG1
Cardiovascular Disease
Partnered with Amgen
Liver
Lung
Tumor

Targeted RNAi Molecules
TRiM™ Platform

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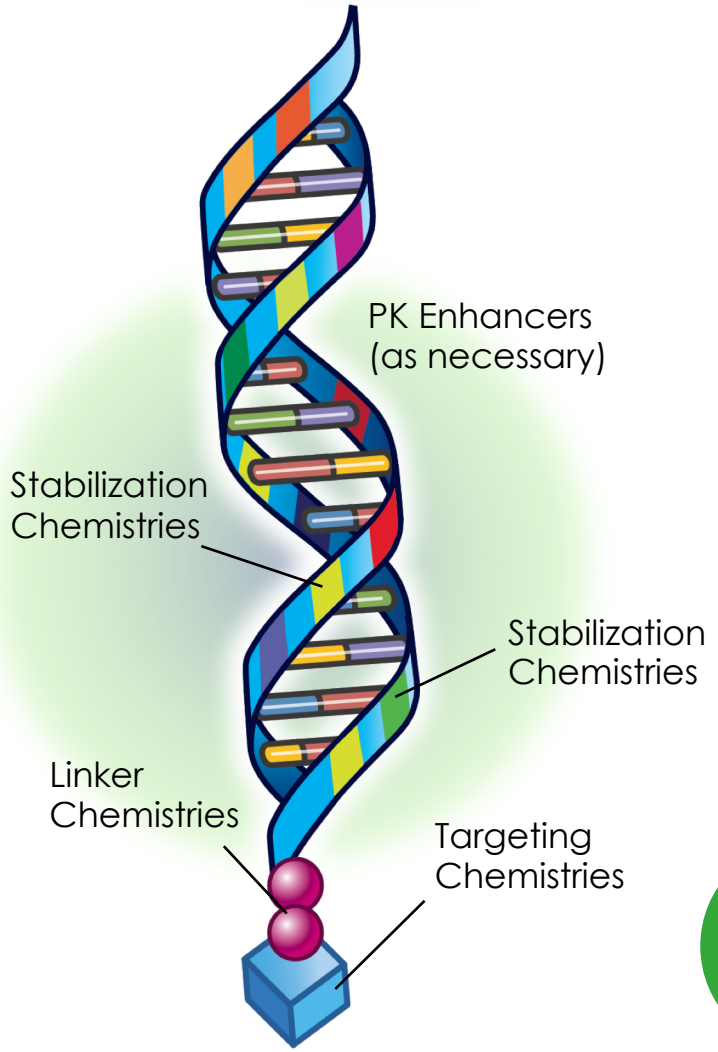
Targeted RNAi Molecules - TRiM™ Platform Simplicity, Specificity, and Activity



- Targeting ligands
  - Targeting has always been a core philosophy
- Linker chemistries
- Proprietary RNAi trigger selection technologies
  - Designed to maximize activity and innate stability
- Stabilization chemistries
- pk enhancers as necessary

Deep KD in diverse tissues using subQ, iv, and inhaled administration routes

Chronic Hepatitis B Virus Infection
ARO-HBV

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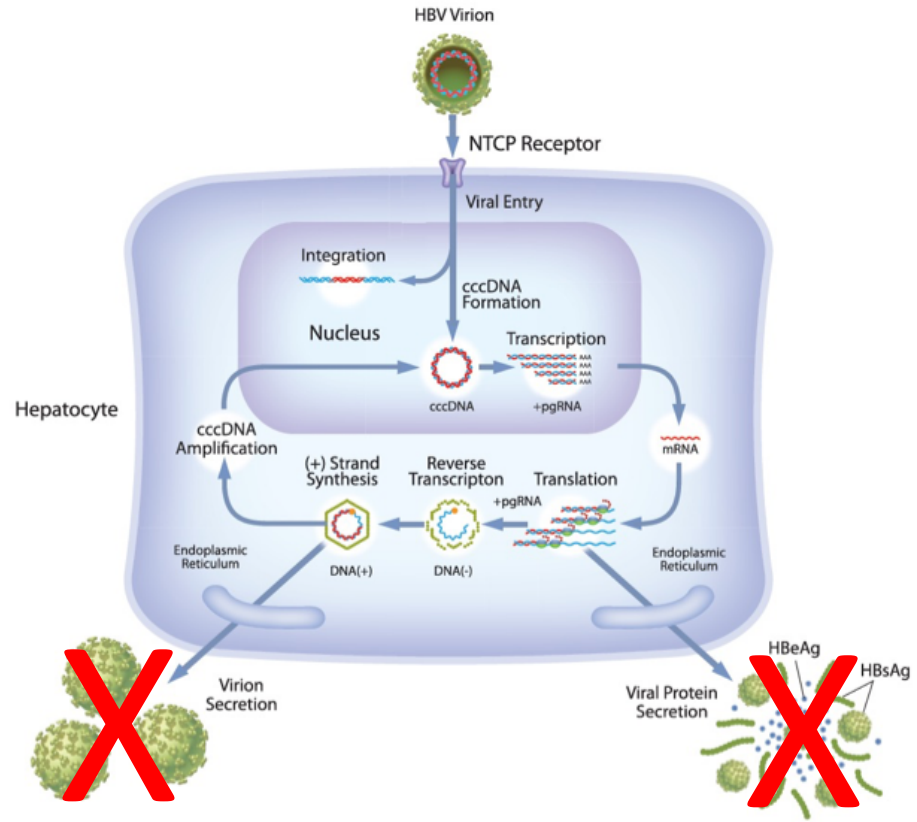
ARO-HBV

ARO-HBV

- For treatment of chronic hepatitis B infection
- CDC estimates up to 350m chronic infections worldwide
- Restart of clinical program that used prior DPC platform

Initial data released September 6, 2018
Additional data possible at AASLD in November 2018

ARO-HBV and the HBV Life Cycle



- Contagion
- Reinfection

- Immune suppression

Designed to silence the entire transcriptome

- Everything from cccDNA
- HBsAg from integrated DNA

Achieve functional cure after finite therapy by:

- Silencing immunosuppressive proteins
- Disrupting HBV life cycle
- Enabling natural immune control

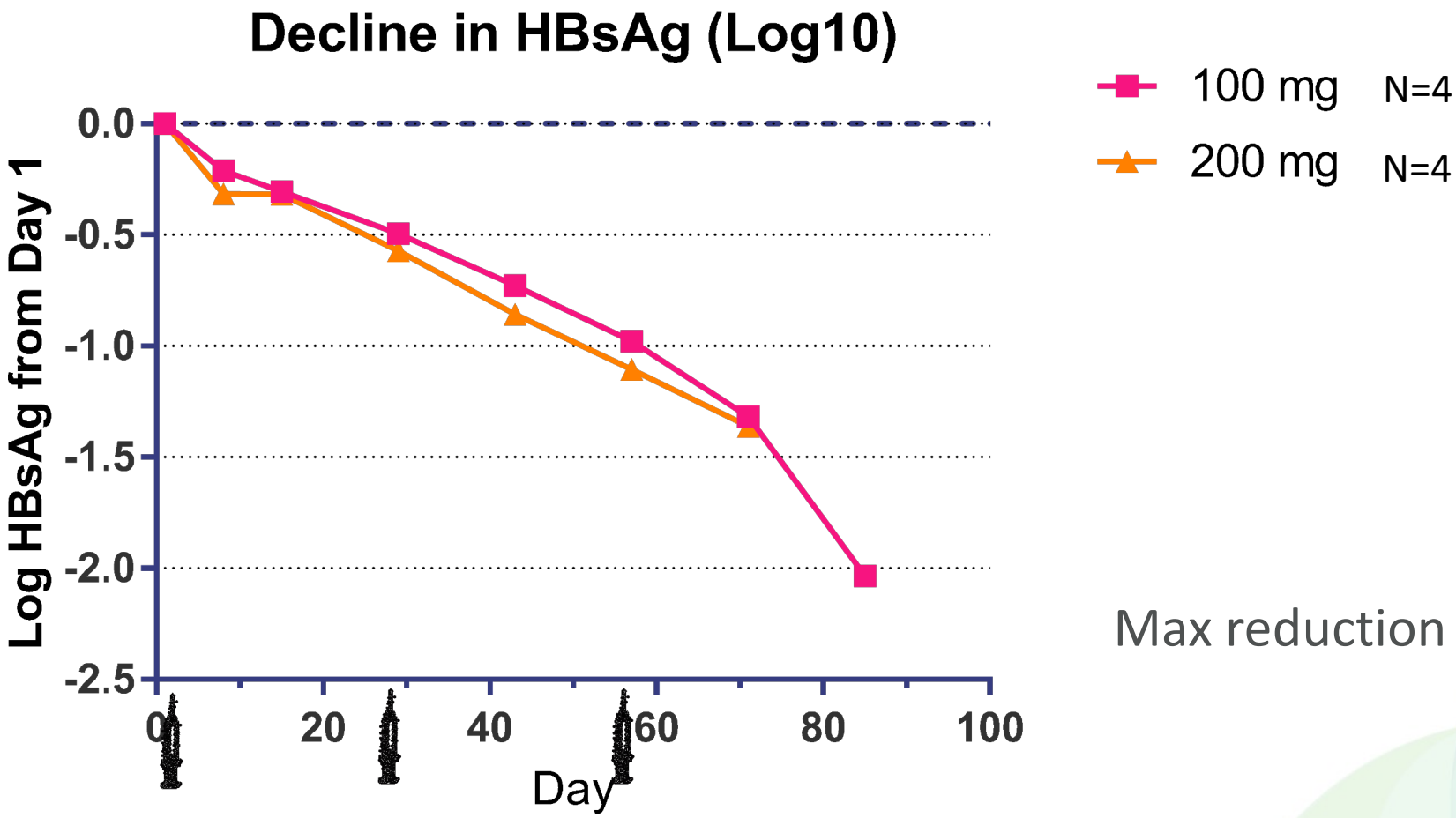
ARC-520 data suggest that immune recovery and control in humans is possible

Monthly (or less frequent) SQ dosing expected

Lessons from 9 clinical studies of ARC-520/521 inform development path of ARO-HBV

First Clinical Data with ARO-HBV After 3 monthly Doses

Includes cohorts with complete data through 14 days after 3rd dose



Max reduction 4 logs

CHB patient AE Table

AEs in >1 subject (data cut 8/24/2018)

AROHBV1001 HBV Patients	**Cohort 2b, 100mg X3 Q28 days**	**Cohort 3b, 200mg X3 Q28 days**	**Cohort 4b, 300mg X3 Q28 days**	**Cohort 5b, 400mg X3 Q28 days**	**Cohort 6, 100mg X3, Q2 wk**	**Cohort 7, 100mg X3 weekly**	**Cohort 8, e+ 300mg X3 Q28 day**	**Cohort 9, e+ 300mg X3 Q28 day**	**Cohort 10, 200mg X3 Q28 day**	**Cohort 11, 300mg X3 Q28 day**	**Total AEs**
AE Reported Terms	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	Open Label n = 4	
Insect bites ankles, Flea bites on neck	1		1								2
Upper respiratory tract infection, Sore throat, Laryngitis, Dry cough	1		1		3	1			1		7
Erythema around injection sites, Injection site redness, Haematoma at injection site, Injection Site Bruise			1	2		2	1			1	7
Facial acne, acne							2				2
Headache, headache – intermittent			1			2					3
Raised Creatine kinase			1				1				2
TOTALS	**2**	**0**	**5**	**2**	**3**	**5**	**4**	**0**	**1**	**1**	**23**

Interim AROHBV1001 Findings

- Mean reduction of HBsAg was 2.0 log10 on day 85 in cohort 2b (100 mg) and 1.4 log10 on day 71 in cohort 3b (200 mg)
  - This may not be the nadir
- Maximum reduction of HBsAg was 4.0 log10
- Activity demonstrated in all patient types (HBeAg pos/neg, NUC naïve/treated)
- Response appeared to be independent of starting HBsAg levels
- ARO-HBV appeared to be generally well-tolerated as of the data cutoff (August 24, 2018)
  - Injection site reactions were observed in approximately 10% of injections

Alpha-1 Antitrypsin Deficiency
ARO-AAT

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ARO-AAT

ARO-AAT

- For liver disease associated with alpha-1 antitrypsin deficiency
- Alpha-1 Foundation estimates >100k people with the ZZ AAT gene mutation in US
- Restart of clinical program that used prior DPC platform

Initial data released June 29, 2018
Additional data possible at AASLD in November 2018

Alpha-1 Antitrypsin Deficiency

Mutation in AAT gene (Z-AAT) leads to mis-folding of the protein and poor export from hepatocytes: low levels in circulation and accumulation in liver

Pathophysiology

Lung

Tissues susceptible to damage by neutrophil proteases: COPD

↓

Treated with AAT enzyme replacement therapy today

Liver

Accumulation of mutant Z protein causes clinical liver disease

↓

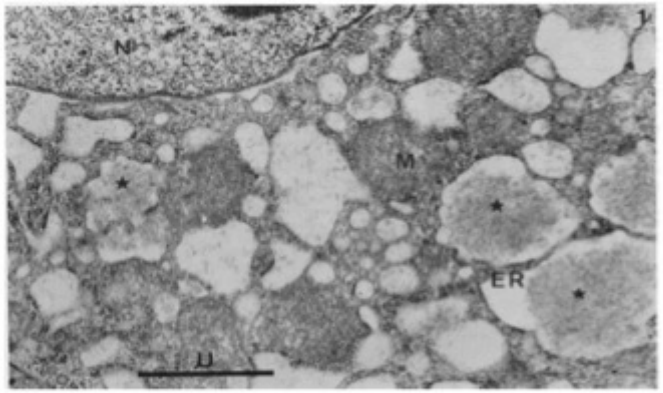
No current treatment

ARO-AAT Mechanism of Action

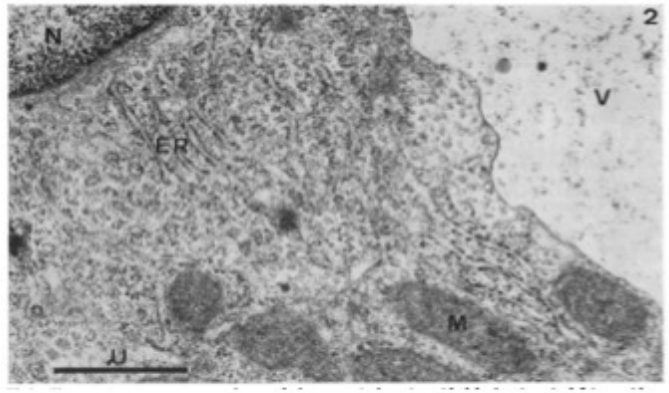
ARO-AAT designed to stop Z-AAT production by silencing AAT gene to:

- Prevent liver accumulation
- Allow clearance of accumulated protein
- Prevent cycles of cellular damage
- Prevent/Reverse progression of liver fibrosis

PiZZ phenotype (diseased)



Pi null phenotype (normal)



Feldmann G et al., *Gut* 1975

Phase 1 Study (AROAAT1001) Design

Multiple Ascending Dose (Blinded)

- 4 treatment arms
  - 35, 100, 200 and 300 mg
  - 100, 200, 300 mg receive ***3 monthly doses***
  - 4 active, 4 placebo
- Assessments of safety, tolerability, plasma levels of ARO-AAT, plasma AAT changes

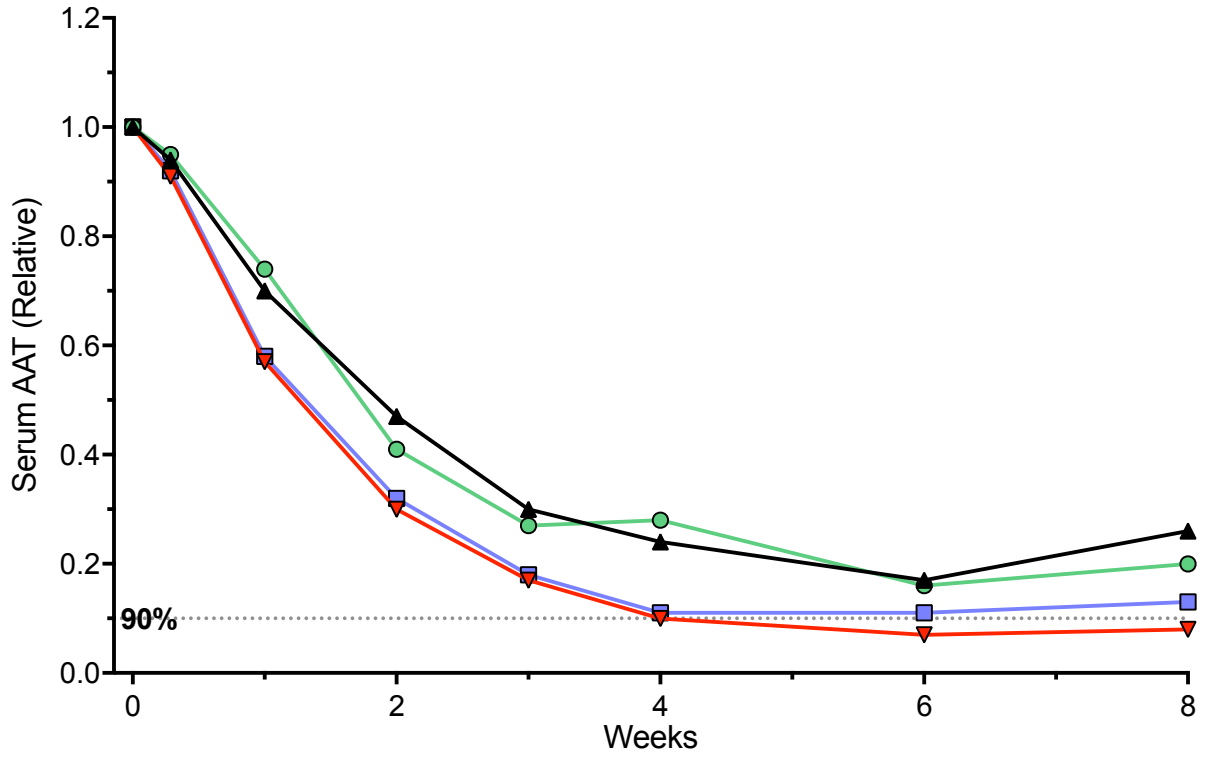
Single Ascending Dose (Open Label)

- No placebo
- 3 groups
  - ***Single doses*** of 100, 200 and 300 mg of ARO-AAT
  - 4 subjects per cohort
- Assessments of safety, tolerability, depth and duration of AAT reductions after a single dose

AROAAT1001 - Results to Date

- Enrollment complete
- 44 total subjects enrolled (28 active, 16 placebo) and have received at least one dose
- Multi-dose cohorts – dosing ongoing and still blinded
- Single-dose cohorts to be followed until plasma AAT normal or within 20% of baseline

Open Label AAT Plasma Data at 100 mg:
Single Dose, Healthy Volunteers



93%: Maximum Serum AAT Reduction achieved 6-weeks following a single dose
87%: Mean maximum serum AAT reduction achieved 6-weeks following a single dose

AROAAT1001 Safety Summary

- Safety data cutoff as of 6/11/18
- No SAEs, No Severe AEs
- Most AEs reported as mild (one moderate gastroenteritis)
- 2 cases of mild injection site erythema at 100 mg after 1st dose.
  - Both mild, both resolved within 48 hours
- No clinically meaningful adverse changes in BUN, creatinine, ALT, AST or total bilirubin
  - No pattern of adverse laboratory changes seen

Cardiometabolic Diseases
AMG 890, ARO-AMG1, ARO-APOC3, ARO-ANG3

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Amgen Collaboration – AMG 890 and ARO-AMG1

- Cardiovascular collaboration for two RNAi therapeutics
- Amgen received:
  1. Exclusive license to ARO-LPA (now called AMG-890)
  2. Option for an additional candidate against an undisclosed target
- Total deal value of up to $673.5 million
- Arrowhead received $56.5 million upfront
  - $35 million in upfront payments, $21.5 million equity investment
- Up to low double digit royalties for ARO-LPA and single digit royalties for the undisclosed target
- Amgen is wholly responsible for funding and conducting clinical development and commercialization

Building Cardiometabolic Pipeline with TRiM™

ARO-APOC3

- Targeting Apolipoprotein C-III (apoC-III) for treatment of hypertriglyceridemia
- Up to 90% KD observed in rodent models (intestines also a source of production)
- SubQ administration

CTA Planned Q4 2018

ARO-ANG3

- Targeting angiopoietin-like protein 3 (ANGPTL3) for treatment of hypertriglyceridemia
- NASH target
- >90% KD observed in rodent models
- SubQ administration

CTA Planned Q4 2018

Analyst R&D Day on October 16, 2018 to discuss emerging pipeline

Extra-hepatic Progams
ARO-ENaC, ARO-HIF2

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Targeting New Tissues Using TRiM™ Platform

ARO-ENaC

- Targeting the epithelial sodium channel (ENaC) alpha subunit for treatment of cystic fibrosis
- Inhaled administration targeting pulmonary epithelium
- Large animal studies and disease models underway

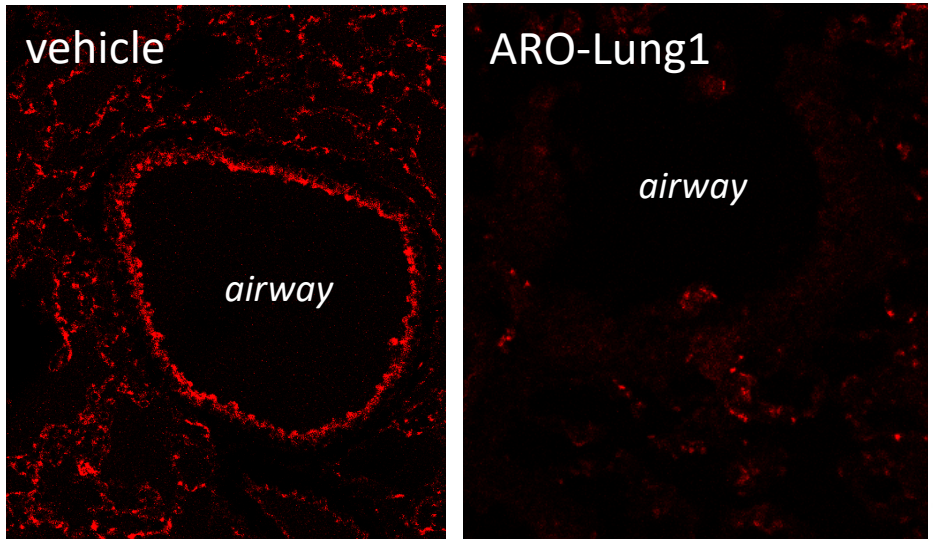
CTA Planned 2019

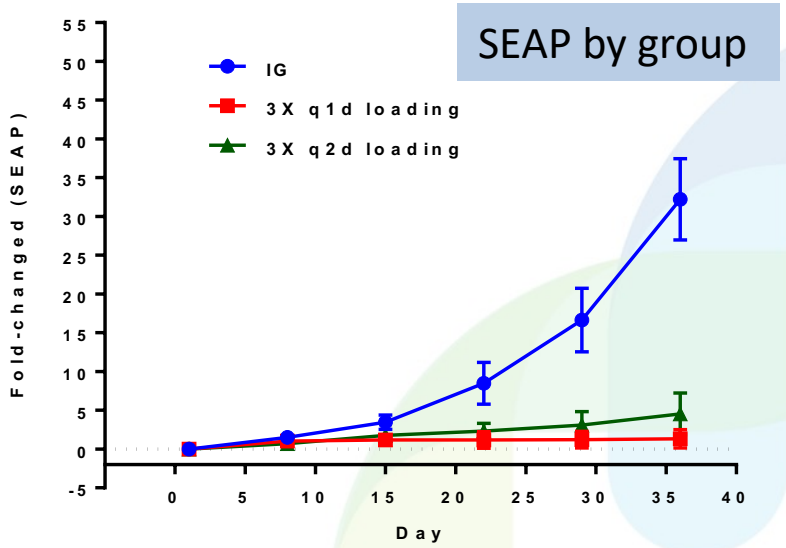
ARO-HIF2

- Targeting HIF2 alpha for treatment of clear cell renal cell carcinoma
- Up to 85% KD observed in rodent tumor model
- IV and subQ administration targeting solid tumors

CTA Planned 2019

Red: lung target protein expression by IHC





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Value Proposition

All built on the TRiM™ Platform

- Modular
- Scalable
- Structurally simple
- Widely targetable

- **ARO-AAT and ARO-HBV in Phase 1/2**
  - Potent in animal models
  - Validation from prior clinical programs
  - Impressive Initial clinical activity
  - Potential data at AASLD Nov 2018
- **ARO-APOC3 and ARO-ANG3 are exciting additions to CV portfolio with CTAs by YE18**
  - Orphan and large market opportunities
- **Business development progress**
  - Amgen partnered programs ARO-LPA (AMG 890) and ARO-AMG1 progressing
  - Opportunities for more partnering
- **Using RNAi outside the liver is real now**
  - Tumor targeting has matured
  - Inhaled administration for lung delivery opens interesting new opportunities