KAMADA INVESTOR PRESENTATION

NASDAQ & TASE: KMDA

September 2016





Forward Looking Statement

This presentation is not intended to provide investment or medical advice. It should be noted that some products under development described herein have not been found safe or effective by any regulatory agency and are not approved for any use outside of clinical trials.

This presentation contains forward-looking statements, which express the current beliefs and expectations of Kamada's management. Such statements involve a number of known and unknown risks and uncertainties that could cause Kamada's future results, performance or achievements to differ significantly from the results, performance or achievements expressed or implied by such forwardlooking statements. Important factors that could cause or contribute to such differences include risks relating to Kamada's ability to successfully develop and commercialize its pharmaceutical products, the progress and results of any clinical trials, the introduction of competing products, the impact of any changes in regulation and legislation that could affect the pharmaceutical industry, the difficulty of predicting U.S. Food and Drug Administration, European Medicines Agency and other regulatory authority approvals, the regulatory environment and changes in the health policies and structures of various countries, environmental risks, changes in the worldwide pharmaceutical industry and other factors that are discussed in Kamada's prospectus related to this offering.

This presentation includes certain non-GAAP financial information, which is not intended to be considered in isolation or as a substitute for, or superior to, the financial information prepared and presented in accordance with GAAP. The non-GAAP financial measures may be calculated differently from, and therefore may not be comparable to, similarly titled measures used by other companies. A reconciliation of these non-GAAP financial measures to the comparable GAAP measures is included in an appendix to this presentation. Management uses these non-GAAP financial measures for financial and operational decision-making and as a means to evaluate period-toperiod comparisons. Management believes that these non-GAAP financial measures provide meaningful supplemental information regarding Kamada's performance and liquidity.

Forward-looking statements speak only as of the date they are made, and Kamada undertakes no obligation to update any forward-looking statement to reflect the impact of circumstances or events that arise after the date the forward-looking statement was made. You should not place undue reliance on any forwardlooking statement and should consider the uncertainties and risks noted above, as well as the risks and uncertainties more fully discussed under the heading "Risk Factors" of Kamada's 2015 Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission on February 25, 2016.



Kamada - Company Profile (KMDA)

Commercial Stage Biotech

- Growing Biopharma company generating revenue and profits from 10 proprietary products
- Leader in Alpha-1 Antitrypsin ("AAT") products globally, both commercialized and in development, and specific immunoglobulins
- Glassia®, for AAT deficiency is the first and only liquid, ready-to-use intravenous AAT product approved by FDA. Marketing by Baxalta/ Shire in US and by a network of distributors in 7 other countries
- Fully Integrated Manufacturing and Distribution

Rich Product	•	Inhaled AAT submitted MAA in EU and Phase 2 US study topline result released				
Pipeline	•	Attractive pipeline of intravenous AAT is being developed in 3 Orphan Indications				
	1	KamRAB for rabies prophylaxis (BLA submitted Sep 16) to be launched in U.S. through collaboration with Kedrion				
	•	Market cap: \$176 M ⁽¹⁾				
	•	2015 revenues = \$70 M				
Financial	1.1	Guidance: 2016 revenues \$75-80 M and 2017 revenues \$100 M				
Summary	1.1	Cash: \$29.5 M, no debt ⁽²⁾				
		Founded in 1991. Public on TASE in 2005; IPO on Nasdaq in 2013.				
		Shares Outstanding = 36.4 million. Employees = $340^{(3)}$				

Notes: 1. Market data as of September 7, 2016. 2. As of June 30, 2016 3. As of December 31, 2015



Kamada Investment Highlights















- Globally Positioned Biopharmaceutical Company focused on Orphan Diseases and Plasma-Derived Protein Therapeutics
- \$100M of revenues expected by 2017
- Flagship Product Glassia[®] Approved for Alpha-1 Antitrypsin (AAT) Deficiency Disease
 - Unique and Differentiated Product Profile Represents an Exciting Growth Opportunity
- Advanced R&D Pipeline Focused on Various Orphan Indications
- Significant Opportunity for Novel Inhaled AAT for AAT
 Deficiency and Intravenous AAT Pipeline in Graft vs. Host
 Disease, Lung Transplant Rejection, Type-1 Diabetes
- Strategic Partnerships with Industry Leaders, Validating Kamada's Portfolio → Baxalta/Shire, Chiesi, Kedrion and Pari
- Integrated, Efficient and Scalable Best-in-class Patented Platform Technology
 - Patents and know-how act as substantial barrier to entry
 - Facility FDA approved
- Strong Financial Profile with Increasing Profitability
 - Expect to generate positive cash flow in 2017



Diversified Product Portfolio with Extended Global Reach

Diverse Portfolio of Predominantly Plasma-Derived Protein Therapeutics

	Respiratory	Glassia®	Alpha-1 Antitrypsin (human)		
Proprietary Products Segment 2015	Immunoglo -bulin	KamRAB™ KamRho (D) IM KamRho (D) IV Snake Antiserum	Anti-rabies immunoglobulin (human) Rho(D) immunoglobulin (human) Rho(D) immunoglobulin (human) Anti-snake venom		
Revenue: \$43M	Other Products	Heparin Lock Flush Kamacaine 0.5% Human Transferrin	Heparin sodium Bupivacaine HCl Transferrin (Diagnostic grade)		
	Respiratory	Bramitob Foster	Tobramycin Beclomethasone+Formoterol		
Distribution Segment*	Immunoglo- bulins	IVIG 5% Varitect Hepatect CP Megalotect Zutectra	Gamma globulins (IgG) (human) Varicella zoster immunoglobulin (human) Hepatitis B immunoglobulin (human) CMV immunoglobulin (human) Hepatitis B Immunoglobulins S.C		
2015 Revenue: \$27M	Critical Care	Heparin sodium Injection Albumin	Heparin sodium Human serum Albumin		
	Other	Factor VIII Factor IX Ixiaro	Coagulation Factor VIII (human) Coagulation Factor IX (human) Japanese encephalitis		

Global Presence with Exposure to Emerging Markets





*Kamada distributes products directly in Israel through its own sales force

Growing Proprietary Products Segment Through Glassia®



AAT Deficiency: Relatively Common, Potentially Lethal, Often Undiagnosed



Genetic/Hereditary condition causing decreased levels of AAT in blood and tissues



Affects at least 100,000 people in the US and a similar number in Europe



Predisposes to lung, liver, other disease

AAT deficiency-associated lung disease is characterized by airway obstruction and destructive changes in the lungs (Emphysema)

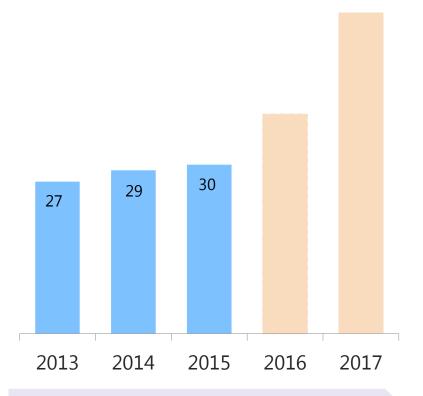


Glassia® is a Differentiated Product

Key Product Advantages

- Glassia[®] is the first and only liquid, ready-touse, IV plasma-derived AAT product
 - ✓ No reconstitution required
 - ✓ Reduces treatment time
 - ✓ Reduces risk of contamination and infection
- Kamada's highly purified liquid product is manufactured through a proprietary process
- Glassia[®] is sold in the U.S. by Baxalta, a leading plasma therapeutics company (now part of Shire)
- Patient count on Glassia has increased 25%/yr. in years 2014 and 2015, growing our market share
- Significantly faster infusion rate was approved by the U.S. FDA (2014)
- Self-infusion approved May 2016

AATD (IV) Product Sales w/o Milestone Revenues (in \$M)



Glassia[®] is sold in 8 countries, with majority of sales in the U.S.



Growth of Glassia[®] Driven by Strategic Partnership with Baxalta (part of Shire)

- Commencement: Sales to Baxalta started in September 2010
- Agreements: Distribution, technology license, and supply of fraction IV
- Product: AAT IV (Glassia®), including all future AAT IV indications in the territories below
- Territories: U.S., Canada, Australia and New Zealand
- Agreement recently extended in October 2015:
 - Baxalta to distribute Glassia® produced by Kamada through 2018 and thereafter Glassia® produced by Baxalta
 - Minimum revenues of \$240 M between 2010 through 2018 (remaining minimum commitment for 2016-2018 = \$97 MM
 - o Starting in 2019 Baxalta will pay royalties on sales of Glassia ® produced by Baxalta

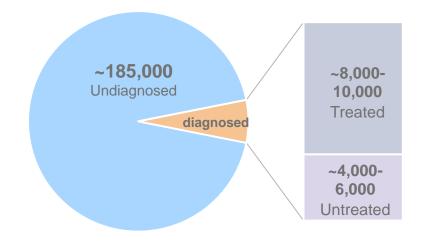




Significant Opportunity to Expand the AATD Market

- Patients suffering from AAT Deficiency ("AATD") remain under-identified and undertreated
- Only ~6% of cases treated in the U.S. and ~2% in EU
- Current market estimated at \$800M WW
- US Market is growing by 10% annually, mainly through expanding diagnostics sponsored by the drug companies
- Simple blood test for diagnosis expected to continue to impact demand
- Greater AAT use in Europe and other geographies could further accelerate market growth
- Chronic therapy creates sustainable product revenue opportunity

AATD Prevalence: ~200,000 Yet <u>Fewer than 5%</u> of Potential Patients in the U.S. and Europe are Treated



Average annual cost of treatment estimated at ~\$80-\$100K per patient

Source : Alpha 1 Foundation, MRB and Company estimates



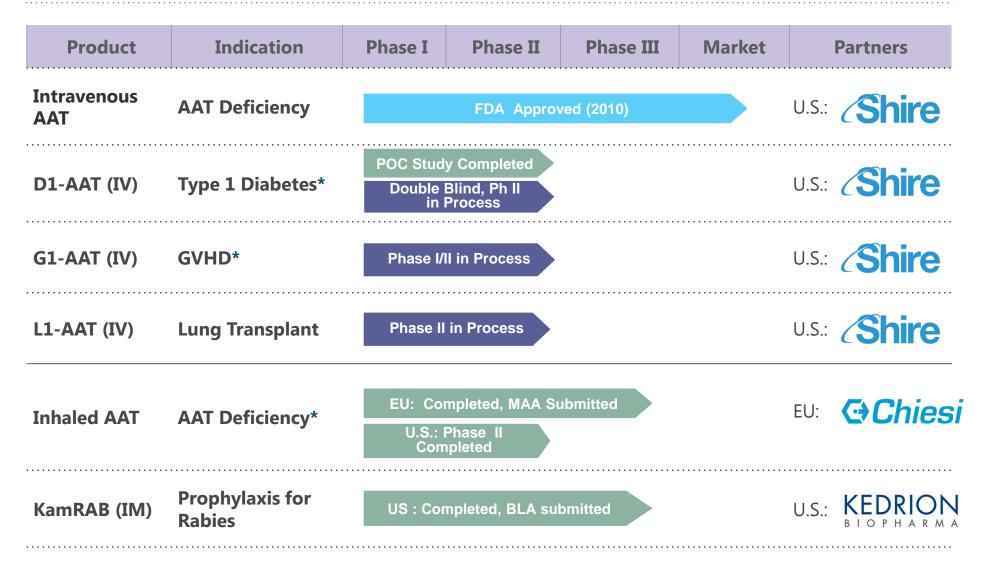
AAT Serves as An Exciting Potential Therapy for Multiple Indications

AAT is a safe plasma-derived protein with known & newly discovered therapeutic roles





High Value Pipeline Focused on Orphan Indications

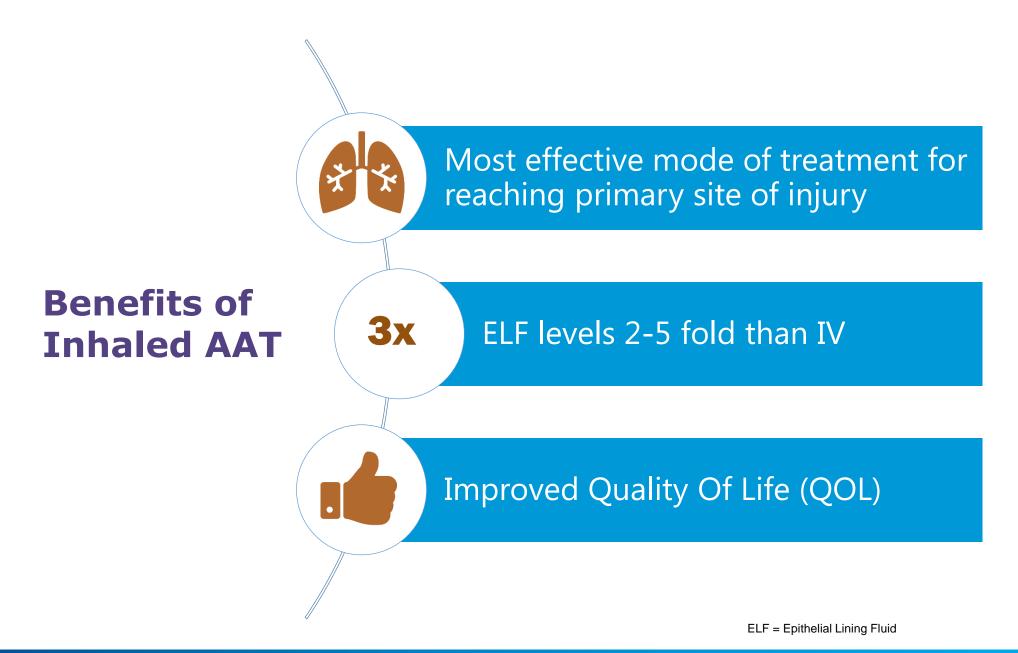


* Orphan Drug Designation



Inhaled AAT to Treat Alpha-1 Antitrypsin Deficiency (AATD)

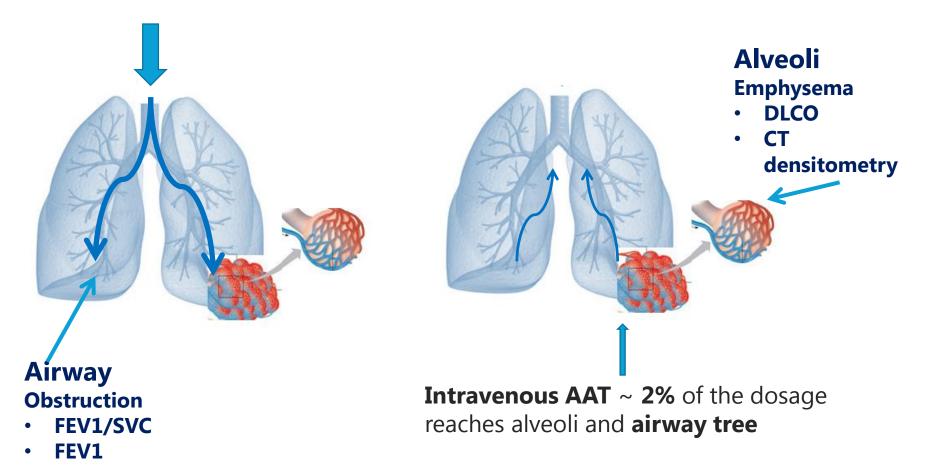




KAMADA

Inhalation Enables Delivery of AAT 3x Higher Than Intravenous

Inhaled AAT ~ 50% of the dosage reaches airway tree and alveoli





Inhaled AAT for AATD: Completed Pivotal Phase II/III Trials in Europe and ongoing Phase II in the U.S.

US

Phase II

EU Phase II / III trial - Completed

Description	 Over 160 AATD subjects, majority are treatment- naïve Double blind, placebo controlled, randomized Multi-center international study: Western EU (in 7 countries) and Canada 80% power to detect a difference between the two groups at 1 year Powered for 20% difference between the two groups Power is based on number of events collected during the study 	 Randomized; Sample size of 36 subjects Double blind, placebo controlled, randomized
Route & Dosage Form	 Inhalation of human AAT, 160mg total, twice daily, ~10-15 minutes using eFlow[®] device 	 Inhalation of human AAT; two dosage groups (80mg and 160mg daily); eFlow[®] device
Clinical Endpoints	• Exacerbation events (Primary: time to first moderate/severe, Secondary (among others): rate, severity of first event; Safety: Lung function	 Primary: Concentration of AAT in ELF Secondary: safety and tolerability, Concentration AAT in serum, ELF inflammatory analytes
Duration	 50 week treatment in DB period; daily treatment 50 week open label extension ; daily treatment Study completed 	 12 weeks double blind 12 weeks open label extension Topline released August 16



Inhaled AAT Phase II/III Trial: Summary of Results

Results demonstrate:

- 1. Primary and secondary endpoints did not demonstrate statistical significant difference.
- 2. Efficacy in lung function (statistically significant)
- **3.** Change in the nature of exacerbations Reduction in number of Type 1exacerbations (trend) and reduction in dyspnea score (statistically significant) for first exacerbation
- 4. Safe and tolerable drug

Kamada submitted MAA in March 2016 on the basis of:

- 1. Orphan designated drug
- 2. Demonstrated efficacy in lung function
- **3. Unmet patient need** Clinical primacy in efficacy data for Inhaled AAT and AATD in general
- 4. EMA confirmed review of **post-hoc analysis** and **"totality of the data"**, irrespective of not meeting primary endpoint
- 5. Precedence for approved drugs of similar nature (ODD, post-hoc analyses and existing patient unmet medical need)



Inhaled AAT Phase II/III Trial Results*: Spirometry Measures (MMRM**)

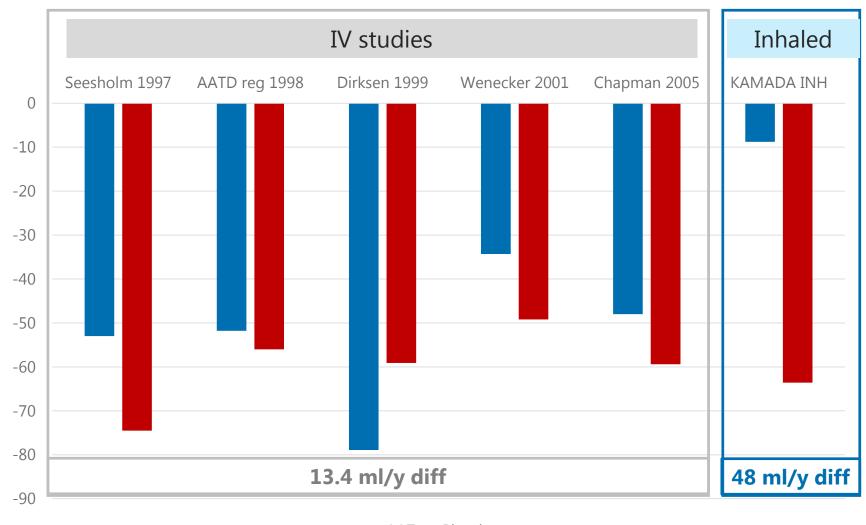
Lung Function	(Changes at V Base	east Squares Means (SEM) Changes at Week 50 from Baseline)		(SEM) ו Overall trea	ares Means method: tment effect	P-Value** (Overall Effect)
	AAT (N= 84)	Placebo (N= 81)		AAT (N= 84)	Placebo (N= 81)	
FEV ₁ (L)	-12mL	-62mL	0.0956	+15mL	-27mL	0.0268
FEV ₁ (% of predicted)	-0.1323	-1.6205	0.1032	0.5404	-0.6273	0.0658
FEV₁/SVC (%)	0.6183	-1.0723	0.0132	0.6230	-0.8715	0.0074

*Safety population

- ** MMRM = Mixed Model Repeated Measure
- FEV = Forced Expiratory Volume. SVC = Slow Vital Capacity.



Inhaled AAT slowed FEV1 deterioration better than former IV trials

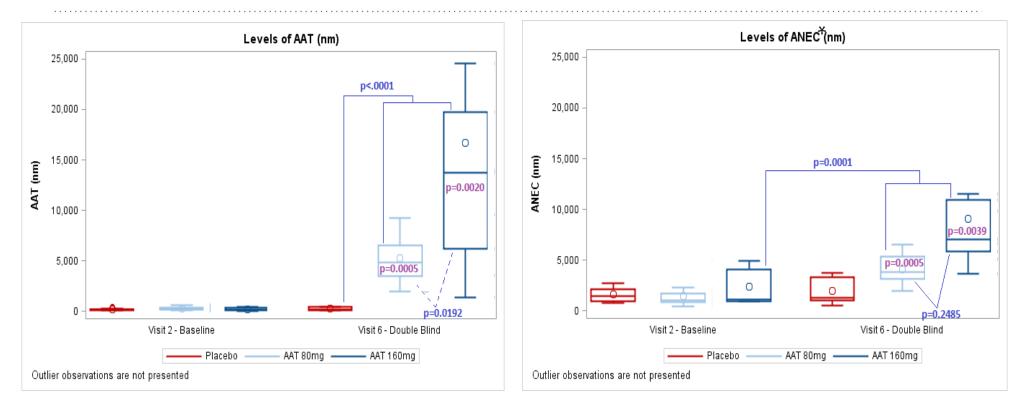


Chapman KR, Stockley RA, COPD:6:177–184

■ AAT ■ Placebo



Inhaled AAT Phase II Topline Result: ELF AAT Antigenic Level & Inhibitory Capacity Increased Significantly



- Inhaled AAT is the most effective mode of treatment for reaching the primary sites of potential lung injury, and restoring AAT inhibitory capacity
- Trial demonstrated strong safety profile in AATD patients

*ANEC- Anti-Neutrophil Elastase inhibitory capacity



Inhaled AAT: Moving Forward

EMA: EU Front

- MAA submitted (centralized procedure) March 2016
- Day 120 comments received, response expected early 2017
- Expecting mid-2017 approval



FDA: U.S. Front

- Approach FDA with results in 2016 to obtain guidance on the clinical/ regulatory pathway for licensing the IH AAT by Kamada in the U.S.
- Expecting guidance from FDA H1/2017 and approved IND for registration trial H2/2017



- Alpha-1 Foundation Survey Confirms Inhaled-AAT as a Preferred Treatment Approach⁽²⁾
- Inhaled AAT opportunity is estimated by Kamada at \$1-2 billion (1.5-2.0x larger than current IV AAT augmentation market of \$800 MM)

Notes: 1. http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm435242.htm

2. <u>http://www.ncbi.nlm.nih.gov/pubmed/23537112</u>

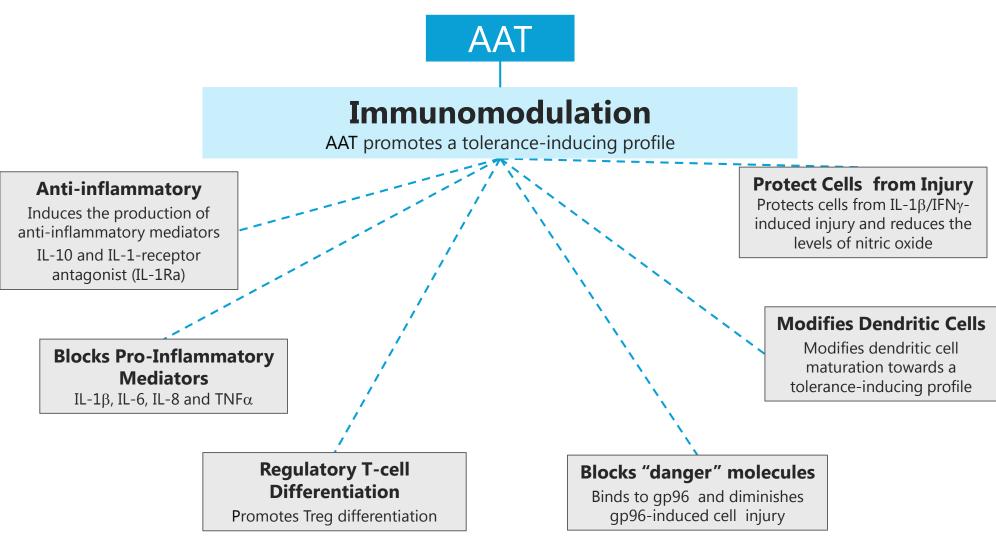


Immune-Modulatory Indications





Mechanistic Evidence - Alpha1-Antitrypsin, a Therapeutic Approach





AAT to Treat Graft Versus Host Disease



Graft Versus Host Disease (GVHD): A Major Complication in Stem Cell Transplantation

Deadly side effects



Searching for an effective treatment

- Standard of care prophylaxis exhibits poor efficacy/severe AE's
- No FDA-approved specific drug for GVHD indication An Unmet Medical Need

Estimated market size: ~ \$700 million



Proof-of-Concept Study with AAT (IV) for Graft-Versus-Host Disease

Phase I/II study open label of 24 patients with steroid-resistant GVHD following allogeneic bonemarrow stem cell transplant **Dose:** 4 dose groups - 15 day regimen. Doses given on days: 1, 3, 5, 7, 9, 11, 13 and 15

Primary End Points: % of patients at each dosing cohort who experience no toxicity and in whom GVHD is stable or improved

Secondary End Points - AAT levels, cytokine levels, infection rate, progression of GVHD, SAEs

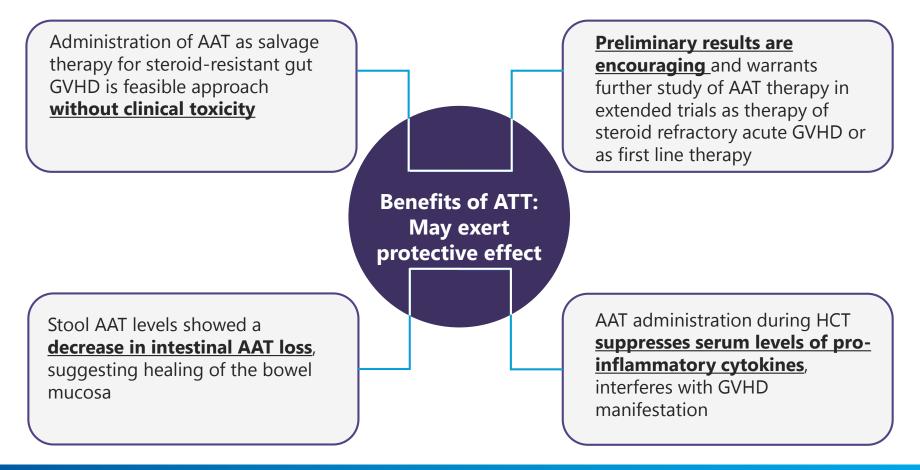
In cooperation with Baxalta/Shire; conducted at the Fred Hutchinson Cancer Research Center in Seattle, Washington

Study may serve as a potential platform, to expand the use of AAT beyond GVHD to other transplantations, based on a similar mechanism of action



AAT May Exert a Protective Effect on the Bowel Mucosa in Gut GVHD

Study results have indicated that AAT may exert healing of the bowel mucosa in gut GVHD slowing/stopping the disease progression and re-modulation of the immune attack





Images from Phase I/II Clinical Study Interim Report

Before

Duodenits Suspect severe upper and lower GVHD

After 8 doses of AAT

Moderate mucosal denudement and edema noted throughout the duodenum







AAT to Treat Lung Transplant Rejection



Lung Transplant Rejection - Attractive Opportunity To Deliver A Differentiated Therapy

Lungs have the highest rate of rejection among transplanted solid organs

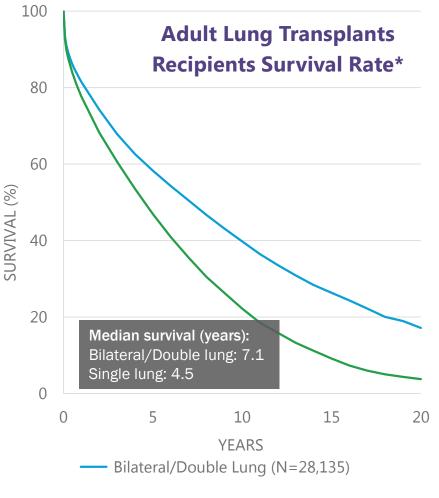
- ~33% will experience acute rejection within the first year
- ~50% will develop chronic rejection within the first 5 years

No new treatment options have been made available for years

 Physician feedback indicates strong need for improved post-transplant therapies over existing options (toxicity, immunosuppressive)

Kamada initiated the first clinical trial designed to prevent lung transplant rejection

Potential market size: ~ \$400-500 million



*JHLT. 2015 Oct; 34(10): 1264-1277



Initiation Of Phase II Study With AAT IV For Prevention Of Lung Transplant Rejection

Phase II: Prospective, open label, standard of care (SOC) controlled, randomized, parallel group single center study

In collaboration with Baxalta/Shire, Led by Prof. Mordechai Kramer, Rabin Medical Center **Study objective:** To assess the safety of AAT IV administration and the effect on rate and severity of acute and chronic lung rejection as well as pulmonary infections, in subjects undergoing first lung transplantation

Design: 30 lung transplant recipients randomized 2:1 to receive AAT IV on top of standard-of-care (SOC) or SOC alone, for 48 weeks plus 12 months of follow-up period

Primary Endpoints:
Safety - Related adverse events (AEs)
Efficacy - Changes in FEV1 from baseline and overall effect, incidence and rate of acute lung rejection



"Preclinical data published in Blood suggest that IV AAT has an immunomodulatory and anti- inflammatory mechanism of action that would support its efficacy in the prevention of lung transplant rejection" (Prof. Mordechai Kramer)



AAT to Treat Newly Diagnosed Type-1 Diabetes



AAT (IV) is a Promising Potential Treatment for Newly Diagnosed Type -1 Diabetes Patients

Market Opportunity

Type-1 Diabetes occurs when the immune system attacks and destroys beta cells in the pancreas

- More than 10 million suffer from Type 1 diabetes globally
- 100,000 new patients/year diagnosed globally
- In the U.S. alone: 3 million patients, with 30,000 new patients diagnosed annually

Drug Impact

Studies have shown that AAT protects beta cell islets

- Delays the onset of autoimmune diabetes
- Reduces the incidence of diabetes
- Inhibits insulitis and beta-cell apoptosis
- Decreases beta-cell inflammation

Benefits

Preservation of beta cells correlates with reduced risk of long-term complications

- DCCT* indicated that patients with C-peptide on MMTT ≥0.2 pmol/mL were less likely to develop retinopathy and hypoglycemia complications (Greenbaum et al 2012)
- Higher / sustained levels of Cpeptide correlate with reduced incidences of the microvascular complications (Steffes et al 2013)



Newly Diagnosed Type-1 Diabetes Clinical Trial Ongoing

Phase II: Double-Blind, Randomized, Placebo-Controlled, Multicenter Study



Study objective: To evaluate the efficacy and safety of human, Alpha-1 Antitrypsin (AAT) in the treatment of new onset Type 1 Diabetes

Design: Two doses, placebo controlled, randomized with ~70 pediatric and young adult patients

Expected duration: One year, enrollment complete,

Last Patient Out expected January 2017 → Topline results expected mid-2017

Endpoints: In accordance with FDA / EMA guidance for clinical trials evaluating beta-cell preservation (C-peptide parameters, HbA1C, hypoglycemic events and insulin daily dose)

Planned Extension: Patients that completed the study will be eligible to enter into an Investigator Initiated study for an additional one year treatment



KamRAB: Human Rabies Immune Globulin

Kamada's human rabies immune globulin is a post-exposure prophylaxis (PEP) for rabies

U.S. Opportunity: Strategic agreement with Kedrion S.p.A for the clinical development and marketing of KamRAB in U.S.

- U.S. pivotal Phase II/III clinical trial met primary endpoint of non-inferiority when measured against an IgG reference product
- Biological License Application submitted to FDA in August 2016
- U.S. launch expected by 2017
- In the U.S., there are ~40,000 post-exposure prophylaxis treatments administered each year, representing ~\$100 million market opportunity
- Currently, only one significant provider of anti-rabies immunoglobulin exists

Product marketed by Kamada in 10 countries currently

- Our product has been marketed since 2003, over 1 million vials sold to date
- WHO estimates ~10 million people worldwide require medical treatment against rabies each year after being exposed to an animal suspected of rabies infection







Financials





Compelling Investment Driven by Multiple Pillars of Growth

Existing Anchor Products Profitable unit

- Sales in 15 countries
- Predictable, stable business

(\$0.5B)*

Glassia® (AAT-IV)	
in U.S.&ROW	
 Estimated only ~5% of cases treated in U.S. 	
 Annual therapy costs ~\$80K – \$100K per patient 	
 Partnered with Baxalta solely for IV products in the U.S. (agreement also covers Canada, Australia and New Zealand) 	
 Key geographies retained 	
(100K pts., \$0.75-1B)*	
ntential •	

Inhaled AAT for AATD in Europe & US • Estimated only ~2% of cases treated in Europe

- Estimated only ~5% of cases treated in US
- Orphan drug designation in US and FU
- Partnered with Chiesi for Inhaled AAT for AATD in Europe only
 Distribution (no technology outlicensed in Europe)
- Unencumbered asset in U.S.
- (200K pts.,\$1-2B)*

Geographies Potential to sell existing and new products into new geographies Rabies Ig to U.S. and additional territories

 Capital-efficient strategy minimizes outlay required by Kamada

New

(\$0.5B)*

ell • **G1-AAT (IV):** GVHD phase I/II in process (\$0.5-1B)*

 L1-AAT (IV): Lung transplant rejection entering phase I/II (\$0.5B)*

Additional

Unencumbered

Pipeline Products

 D1-AAT (IV): Type-1 diabetes in Phase I/II (100K pts.,\$3.5-5B)*

(All AAT (IV) are unencumbered outside of U.S., Canada, Australia and New Zealand)

The Kamada Pillars

Existing Anchor Products

+

Glassia[®] (AAT-IV) in U.S.

+

Inhaled AAT for AATD in Europe & U.S.

+

New Geographies

+

Additional Unencumbered Pipeline Products



* Estimated market potential

Strong Financial Profile with Revenue Growth and Expanding Profitability

- Stable, profit generating revenue stream from marketed products
- Revenue Guidance: ~\$75 to \$80 M in 2016, \$100 M in 2017
- Strategic partnership model results in efficient operating expenses
 - Baxalta/Shire purchase obligations provide predictable revenue through 2018 and royalties thereafter
 - Kedrion partnership for Rabies Ig expected to increase revenues and profitability from 2018 and beyond
- Better product mix expected to improve gross margin
- Pipeline products expected to accelerate revenue growth
 - o Profits from marketed products to partly fund clinical development programs
- Low capital expenditures to support infrastructure investments in order to meet future demand
- Preferred tax treatment under Israeli law
- Cash of \$29.5 M (as of June 30, 2016) with modest cash use



Sustained Revenues and Gross Profits are Funding R&D

\$ M	FY2014	FY2015	1H 2015	1H 2016	% change
Proprietary Products	44	43	16	23	
Distribution	27	27	12	11	
Total Revenues	71	70	28	34	21%
Gross Profit	15	16	4	10	150%
R&D	(16)	(17)	(7)	(8)	
S&M and G&A	(10)	(11)	(5)	(5)	
Net Loss	(13)	(11)	(8)	(4)	(50%)
Adjusted EBITDA ¹	(5)	(6)	(6)	(0)	

Guidance: 2016 revenues \$75-80 M and 2017 revenues \$100 M

Note

1. See 20F for a reconciliation of Adjusted EBITDA to IFRS Net Profit (Loss)



Future Milestones and Value Creation

	Milestone Date
Initiation of pivotal Phase II/III GVHD trial	2016
Strategic agreements	2016 2016
FDA guidance for Inhaled AAT for AATD regulatory path	1H-2017
Response EMA comments for Inhaled AAT for AATD	1H-2017
Approved IND for registration trial of Inhaled AAT for AATD	2H-2017
Final report for Phase II for type-1 diabetes trial	Mid-2017
Rabies product launch in the U.S. (if approved)	2H-2017
Inhaled AAT for AATD launch (EU) (if approved)	2H-2017
Achieve \$100 million in annual revenues	₂₀₁₇ 2018
Double* the number of Glassia patients WW	2018

* Compared to number of patients in 2014



Kamada Investment Highlights

Globally Positioned Biopharmaceutical Company focused on Orphan Diseases and Plasma-Derived Protein Therapeutics











- \$100 M of revenues expected by 2017
- Flagship Product Glassia[®] Approved for Alpha-1 Antitrypsin (AAT) Deficiency Disorder
 - Unique and Differentiated Product Profile and Represents an Exciting Growth Opportunity
- Advanced R&D Pipeline Focused on Various Orphan Indications
- Significant Opportunity for Novel Inhaled AAT for AAT Deficiency and Intravenous AAT Pipeline in Graft vs. Host Disease, Lung Transplant Rejection, Type-1 Diabetes
- Strategic Partnerships with Industry Leaders, Validating Kamada's Portfolio → Baxalta/Shire, Chiesi, Kedrion and Pari
- Integrated, Efficient and Scalable Best-in-class Patented Platform Technology
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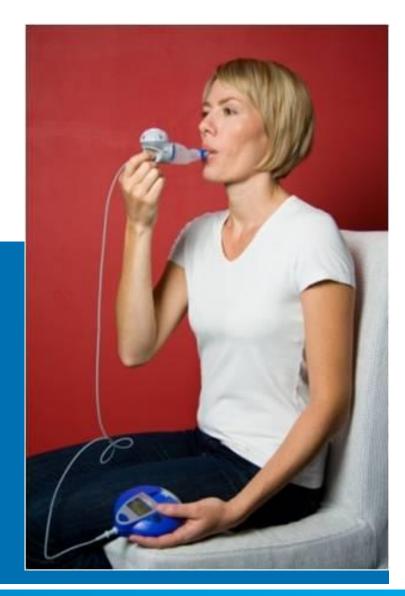


Next Generation \rightarrow **AAT Inhaled**

- Inhaled directly to the Lungs
- Clinical trial in Europe completed
- Registration file submitted to EMA
- Expecting mid 2017 approval

"The study results demonstrated primarily that the overall treatment effect on lung functions, is of significant clinical value. This study is the first study ever that is indicative of inhaled AAT's ability to potentially reduce lung inflammation as expressed by its preservation of lung function and the changes shown in symptoms."

Prof. Jan Stolk, MD, Department of Pulmonology, Leiden Medical Center, Principal Investigator of the Phase 2/3 clinical trial and Chairman of the Alpha 1 International Registry (AIR)





Fewer symptoms in first EX - AAT vs. Placebo Less Type I (3 symptoms) and more type II (2 symptoms)

Turne (Octogram)	AAT	Placebo	D Value
Type/Category	N=85	N=83	P Value
Туре І	16 (18.8%)	26 (31.3%)	0.0614
Туре II	23 (27.1%)	12 (14.5%)	0.0444
Type III	34 (40.0%)	33 (39.8%)	0.9746
None	12 (14.1%)	12 (14.5%)	0.9498

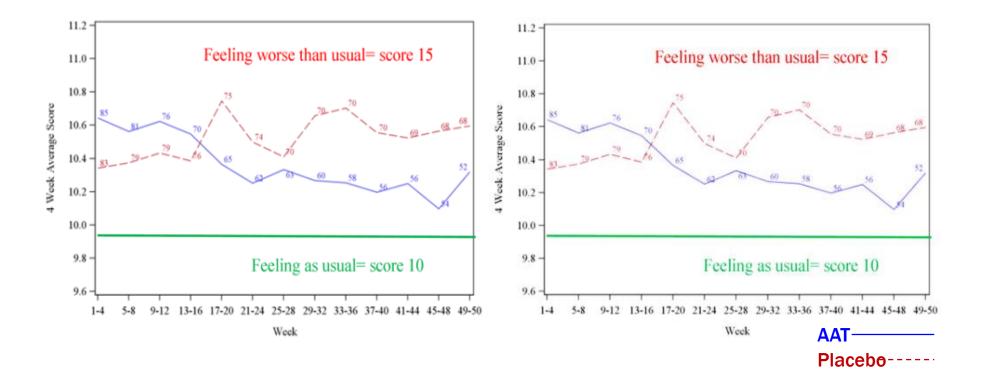


AAT patients tended to have better Dyspnea score

Dyspnea 4 Week Moving Average Graphs

AAT patients tended to have better Well-Being score

Well Being 4 Week Moving Average Graphs





In the Words of the Key Opinion Leaders

"The study results demonstrated primarily that the overall treatment effect on lung functions, is of significant clinical value. This study is the first study ever that is indicative of inhaled AAT's ability to potentially reduce lung inflammation as expressed by its preservation of lung function and the changes shown in symptoms."

Prof. Jan Stolk, MD, Department of Pulmonology, Leiden University Medical Center, Principal Investigator of the Phase 2/3 clinical trial and acting Chairman of the Alpha 1 International Registry (AIR)

"These new analyses confirm the clinically-meaningful lung function improvement seen with inhaled AAT patients in this study. These results are impressive and underscore the initial findings from this study. In my opinion, inhaled AAT has shown to be an efficacious treatment for this orphan disease."

Prof. Kenneth Chapman, M.D., Director of the Canadian Registry for the Alpha-1 Antitrypsin Deficiency (Asthma and Airway Centre in Toronto Western Hospital, University of Toronto) and an investigator in the Phase 2/3 clinical trial.

"The study analysis suggests exciting results that may lead to wider acceptance of the inhaled route of administration of alpha-1 antitrypsin augmentation therapy, which could be a real breakthrough for AATD patients."

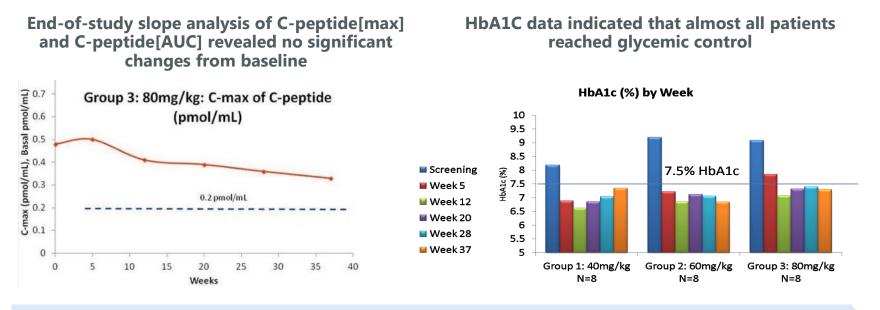
Robert A. Sandhaus, Ph.D., M.D., FCCP, Founder and Director of the Alpha1-Antitrypsin Deficiency Program at National Jewish Health in Denver, Colorado, and the Clinical Director of the Alpha-1 Foundation





Clinical Development for Newly Diagnosed Type-1 Diabetes: New Exciting Prospects

Phase I/II Open Label Study to evaluate the safety, tolerability and efficacy of AAT on beta cell preservation and glycemic control on newly diagnosed T1D pediatric patients (N=24)



- AUC% for C-peptide decreased 23% from baseline vs. ~40-50% expected decrease after 12-15 months from diagnosis¹
- Specific diabetes antibody levels decreased in all groups from baseline to study completion, a decrease that may indicate an immune modulatory effect
- At end-of-study, 38% of patients decreased insulin dose
- All subjects completed the study. No Serious AEs occurred. AEs were mild and mostly infusion-related (fatigue, headache)

1. Greenbaum et al 2012

