KAMADA INVESTOR PRESENTATION

NASDAQ & TASE: KMDA

July 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.

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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 7 other countries
- Fully Integrated Manufacturing and Distribution

Rich Product Pipeline

- Inhaled AAT submitted application in EU and Phase 2 US study topline result expected Q3 2016
- Attractive pipeline of intravenous AAT being developed in 3 Orphan Indications
- KamRAB for rabies prophylaxis (BLA submission in mid-2016) to be launched in U.S. through collaboration with Kedrion
- Market cap: \$137 M ⁽¹⁾

Financial Summary

- 2015 revenues = \$70 M;
- Guidance: 2016 revenues \$75-80 M and 2017 revenues \$100 M
- Cash: \$35.5 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 June 5, 2016. 2. As of March 31, 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 and Represents an Exciting Growth Opportunity
- Valuable 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
- Validating Strategic Partnerships with Industry Leaders
 - → Baxalta/Shire, Chiesi, Kedrion and Pari Pharma
- Integrated, Efficient and Scalable Best-in-class Patented Platform Technology
 - Patents and know-how act as substantial barrier to entry
- 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: \$43MM	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* 2015 Revenue: \$27MM	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
	Critical Care	Heparin sodium Injection Albumin	Heparin sodium Human serum Albumin
	Other	Factor VIII Factor IX	Coagulation Factor VIII (human) Coagulation Factor IX (human)

Global Presence with Exposure to Emerging Markets



Countries where Kamada operates

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

Growing Proprietary Products Segment Through Glassia®

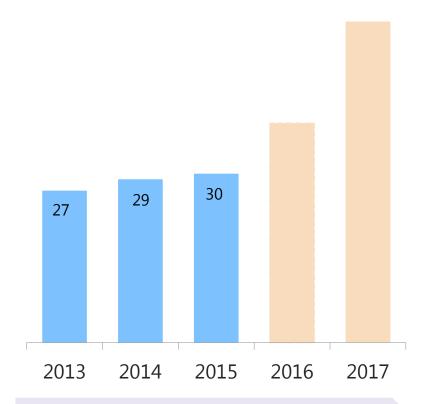


Glassia® is a Differentiated Product

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 delivered through a proprietary manufacturing 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 2014-2015, 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.



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)

AAT Serves as An Exciting Potential Therapy for Multiple Indications

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



Anti-Inflammatory



Immunemodulatory



Tissue Protective



Antimicrobial

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
 - Minimum revenues of \$240 MM between 2010 through 2018 (remaining minimum commitment for 2016-2018 = \$97 MM)
 - Royalties from sales of Glassia® produced by Baxalta expected starting in 2019

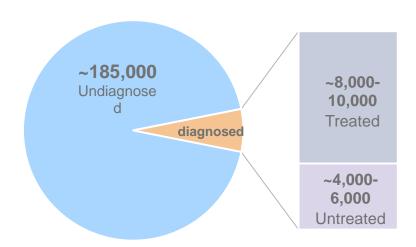




Significant Opportunity to Expand the AATD Market

- Patients suffering from AAT Deficiency ("AATD") remain under-identified and undertreated
- Only ~6% of cases treated in the U.S. and ~2% in FU
- US Market is growing by 10% annually, mainly through expanding diagnostic testing sponsored by 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 Fewer than 5% 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



High Value Pipeline Focused on Orphan Indications

Product	Indication	Phase I	Phase II	Phase III	Market		Partners
Intravenous AAT	AAT Deficiency		FDA Appro	ved (2010)		U.S.:	Shire
D1-AAT (IV)	Type 1 Diabetes*	Double I	y Completed Blind, Ph II Process			U.S.:	⊘Shire
G1-AAT (IV)	GVHD*	Phase I/I	II in Process			U.S.:	Shire
L1-AAT (IV)	Lung Transplant	Phase II	in Process			U.S.:	Shire
Inhaled AAT	AAT Deficiency*	U.S.: I	mpleted, MAA So Phase II rocess	ubmitted		EU:	⇔ Chiesi
KamRAB (IM)	Prophylaxis for Rabies		US : Completed			U.S.:	KEDRION BIOPHARMA



^{*} Orphan Drug Designation







Reach the target organ – Airway tree

Benefits of Inhaled AAT



ELF levels 3 fold than IV



Improved Quality Of Life (QOL)

ELF = Epithelial Lining Fluid



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

	EU Phase II / III trial - Completed	US Phase II
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 ~36-40 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 blind12 weeks open label extensionLast Patient Out in Q2 2016



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 1-exacerbations (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
- 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	Least Squares Means (SEM) (Changes at Week 50 from Baseline)		P-Value** (Changes at	Least Squares Means (SEM) method: Overall treatment effect		P-Value** (Overall
runction	AAT (N= 84)	Placebo (N= 81)	Week 50)	AAT (N= 84)	Placebo (N= 81)	Effect)
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
FEV ₁ /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: Moving Forward

EMA: EU Front

- MAA submitted (centralized procedure) March 2016
- 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.
- AATD is among 16 diseases targeted for "patient-focused drug development" in FY2013-15 (1)



- 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

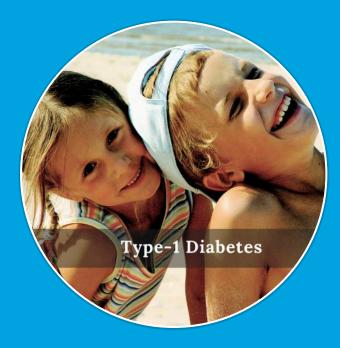


^{2. &}lt;a href="http://www.ncbi.nlm.nih.gov/pubmed/23537112">http://www.ncbi.nlm.nih.gov/pubmed/23537112

Immune-Modulatory Indications

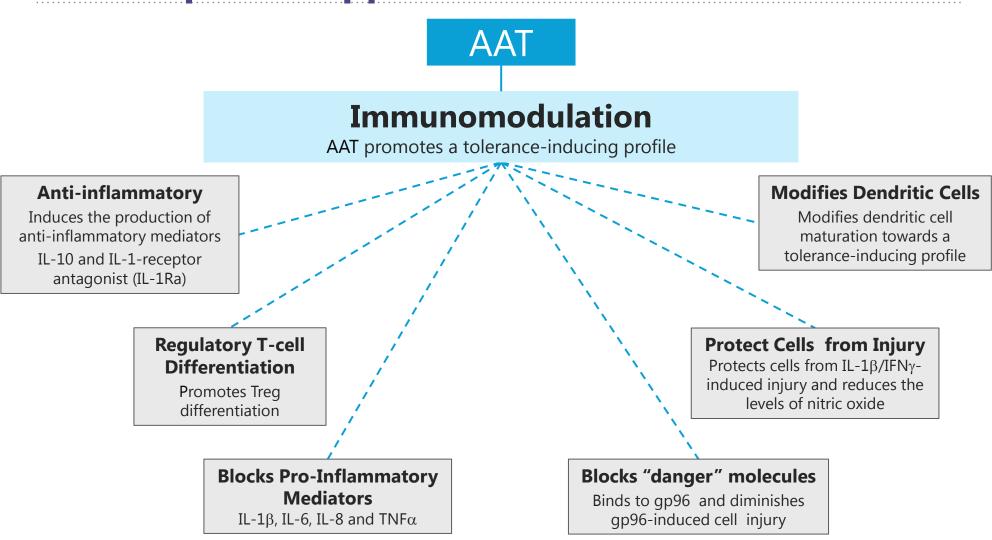








Mechanistic Evidence - Alpha1-Antitrypsin, a Therapeutic Approach

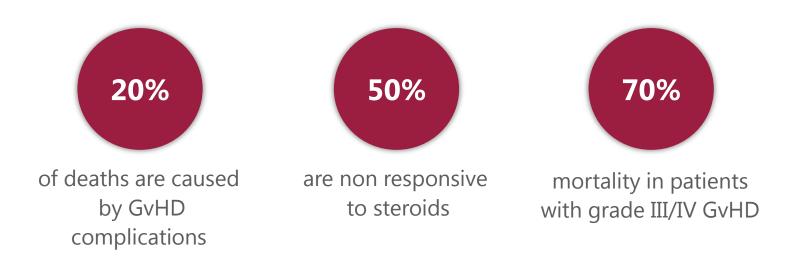






Graft versus Host Disease (GvHD): A Major Complication in Stem Cell Transplantation

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; 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

Administration of AAT as salvage therapy for steroid-resistant gut GVHD is feasible approach without clinical toxicity

Preliminary results are
encouraging and warrants
further study of AAT therapy in
extended trials as therapy of
steroid refractory acute GVHD or
as first line therapy

Benefits of ATT:
May exert
protective effect

Stool AAT levels showed a **decrease in intestinal AAT loss**, suggesting healing of the bowel mucosa

AAT administration during HCT suppresses serum levels of proinflammatory cytokines, interferes with GVHD manifestation



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







Lung Transplant Rejection - Attractive Opportunity To Deliver A Differentiated Therapy

Lungs have the highest rate of rejection among transplanted solid organs

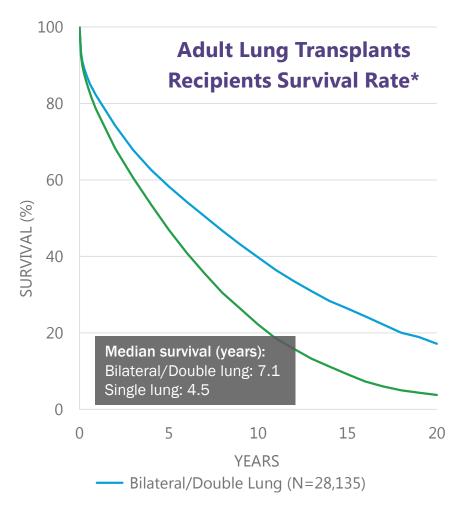
- ~33% will experience acute rejection within the first year
- ~50% will develop chronic rejection within the first 5 years

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

Benefits Market Opportunity Drug Impact Type-1 Diabetes Studies have shown Preservation of beta cells occurs when the immune correlates with reduced risk that AAT protects beta cell islets system attacks and destroys of long-term complications beta cells in the pancreas DCCT* indicated that patients More than 10 million suffer Delays the onset of autoimmune with C-peptide on MMTT ≥0.2 from Type 1 diabetes globally diabetes pmol/mL were less likely to Reduces the incidence of 100,000 new patients/year develop retinopathy and diagnosed globally diabetes hypoglycemia complications • In the U.S. alone: 3 million Inhibits insulitis and beta-cell (Greenbaum et al 2012) patients, with 30,000 new apoptosis • Higher / sustained levels of Cpatients diagnosed annually peptide correlate with reduced Decreases beta-cell incidences of the microvascular inflammation complications (Steffes et al 2013)



^{*}Diabetes Control and Complications Trial

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 December 2016

→ 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
- Expect to file Biological License Application with FDA by mid-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









Compelling Investment Driven by Multiple Pillars of Growth

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)*

* Estimated market potential

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
- 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)*

New **Geographies**

- Potential to sell existing and new products into new geographies
- Rabies Iq to U.S. and additional territories '
- Capital-efficient strategy minimizes outlay required by Kamada

(\$0.5B)*

Additional Unencumbered **Pipeline Products**

- **G1-AAT (IV):** GVHD phase I/II in process (\$0.5-1B)*
- L1-AAT (IV): Lung transplant rejection entering phase I/II (\$0.5B)*
- 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**



Existing

Anchor

Products

Profitable unit

Sales in 15

countries

Predictable.

(\$0.5B)*

stable business

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
 - o Baxalta 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
 - 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 \$35.5 M (as of March 31, 2016) with modest cash use



Sustained Revenues and Gross Profits are Funding R&D

\$ M	FY2014	FY2015	Q1 2016	Q1 2015	% change
Proprietary Products	44	43	11.1	3.2	
Distribution	27	27	3.7	5.8	
Total Revenues	71	70	14.8	8.9	66%
Gross Profit	15	16	4.8	0.4	
R&D	(16)	(17)	(4.1)	(3.6)	
S&M and G&A	(10)	(11)	(2.6)	(2.5)	
Net Loss	(13)	(11)	(2.3)	(5.3)	
Adjusted EBITDA ¹	(5)	(6)	(0.8)	(4.4)	

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
Report results from Phase II for Inhaled AAT for AATD trial (U.S.)	3Q16
Approach FDA to obtain guidance on the pathway for Inhaled AAT in the U.S.	2016
Strategic agreements	2016
BLA submission for the Rabies Ig in the U.S.	2016
Initiation of pivotal Phase II or III GVHD trial	2016
Final report for Phase II for type-1 diabetes trial	2017
Rabies product launch in the U.S. (if approved)	2017
Inhaled AAT for AATD launch (EU) (if approved)	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













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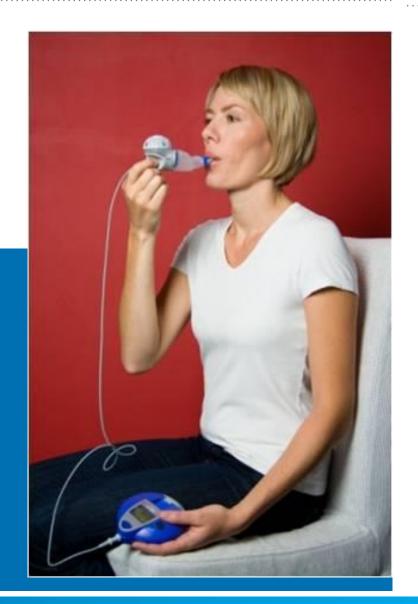


Next Generation → **AAT Inhaled**

- 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)



Consistent Track Record of Execution

U.S. FDA approval for Glassia®

Strategic agreement with Baxalta & First Glassia® sale in the U.S.

Strategic agreement for Rabies in the U.S. with Kedrion

Anti-Snake Venom launch

Strategic agreement with Chiesi for Inhaled AAT for AATD in EU

Newly diagnosed type-1 diabetes Phase II trial completed

Initiation of Phase II for type-1 diabetes

Initiation of U.S. Phase II for Inhaled AAT for AATD

Initiation of U.S. Phase I/II study of AAT IV in GVHD

Completion of EU Phase II/III Inhaled AAT for AATD trial

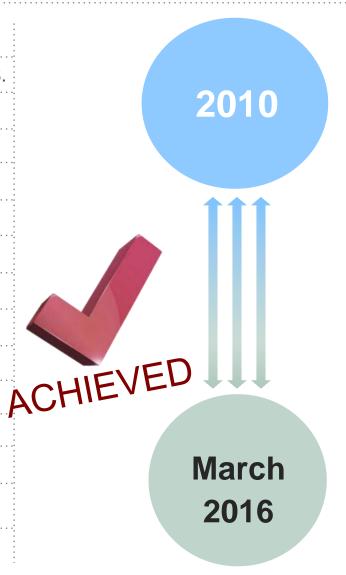
Completion of U.S. Phase III Rabies Ig

U.S. & EU Orphan Drug Designation for Glassia to treat GVHD

MAA submission for Inhaled AAT for AATD

Initiation of Phase II trial of AAT IV to Prevent Lung Transplant Rejection

Increased sales, profitability and production capacity





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."

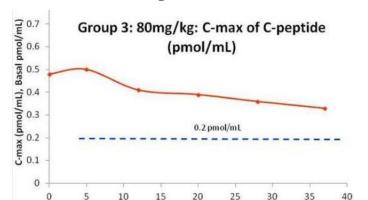
Robert A. Sandhaus, Ph.D., M.D., FCCP, Founder and Director of the Alpha1-Antitrypsin Deficiency Program at National Jewish Health in Denver, Colorado, and the Clinical Director of the Alpha-1 Foundation



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

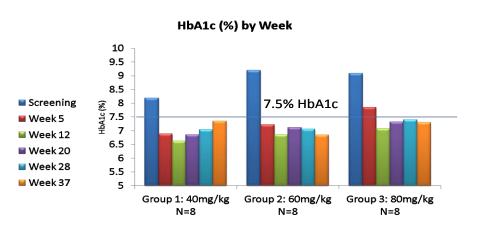
Phase I/II Open Label Study to evaluate the safety, tolerability and efficacy of AAT on beta cell preservation and glycemic control on newly diagnosed T1D pediatric patients (N=24)

End-of-study slope analysis of C-peptide[max] and C-peptide[AUC] revealed no significant changes from baseline



Weeks

HbA1C data indicated that almost all patients reached glycemic control



- 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

