

**EACH
LIFE IS
UNIQUE**



KAMADA

KAMADA INVESTOR PRESENTATION

NASDAQ & TASE: KMDA

February 2019

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 prospected results, performances or achievements expressed or implied by such forward-looking statements. Important factors that could cause or contribute to such differences include, but are not limited to, risks relating to Kamada's ability to successfully develop and commercialize its products and product candidates, 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, obtaining or maintaining U.S. Food and Drug Administration, European Medicines Agency and other regulatory authority approvals, the regulatory environment, restraints related to third parties' IP rights 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 under the heading "Risk Factors" of Kamada's 2017 Annual Report on Form 20-F as well as in Kamada's recent Forms 6-K filed with the U.S. Securities and Exchange Commission.

This presentation includes certain non-IFRS 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 IFRS. The non-IFRS financial measures may be calculated differently from, and therefore may not be comparable to, similarly titled measures used by other companies. In accordance with the requirement of the SEC regulations a reconciliation of these non-IFRS financial measures to the comparable IFRS measures is included in an appendix to this presentation. Management uses these non-IFRS financial measures for financial and operational decision-making and as a means to evaluate period-to-period comparisons. Management believes that these non-IFRS 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, except as required by applicable securities laws. You should not place undue reliance on any forward-looking 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 2017 Annual Report on Form 20-F as well as in Kamada's recent Forms 6-K filed with the U.S. Securities and Exchange Commission.

KAMADA AT A SNAPSHOT



COMMERCIAL STAGE BIOPHARMA

2
FDA Approved
Products

Takeda / Shire
KEDRION
BIOPHARMA
**US Commercial
Partners**



4
**Additional
Products
Marketed WW**

ADVANCED CLINICAL PIPELINE

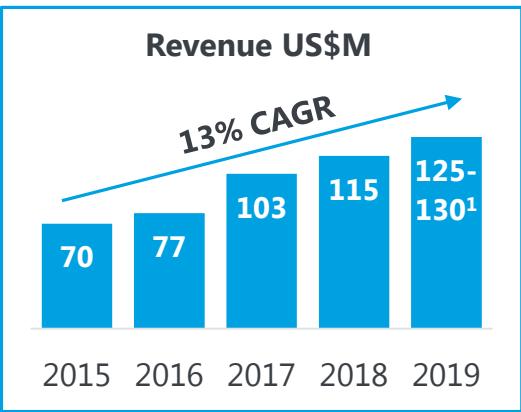
***Inhaled AAT
For AATD
Lead Product
Candidate***



3
**AAT IV Clinical
Programs
GvHD; Lung
Transplant; T1D**

2
**Early Stage R&D
Programs
rAAT;
Organ Preservation**

FINANCIAL GROWTH & PROFITABILITY



\$50.6MM
Cash (12/31/18)
No debt




DISTRIBUTION SEGMENT

20
**products exclusively
distributed in Israel**

**Focused on Rx and
orphan indications**

HIGH VALUE PRODUCT PORTFOLIO AND PIPELINE



Product	Indication	Phase I	Phase II	Phase III	Market
Glassia® (IV AAT)	AAT Deficiency	FDA approved (2010)	----->		U.S. distribution through  / 
KamRab®/KedRab® (IM Anti-Rabies)	Prophylaxis for Rabies	FDA approved (2017)	----->		U.S. distribution through 

Clinical Development

Inhaled AAT	AAT Deficiency ¹	EU Phase 2/3 (completed) MAA withdrawn (June 2017) EMA accepted new Ph3 design US Phase 2 (completed) FDA review of path forward	----->		May seek partner upon IND/CTA approval
G1-AAT (IV)	Graft vs Host Disease (GvHD) ¹	Phase 1/2 (completed) Phase 2 (ongoing)	----->		Ph2 in collaboration with MAGIC ³
L1-AAT (IV)	Lung Transplant	Phase 2 (ongoing)	----->		In collaboration with Shire
D1-AAT (IV)	Type 1 Diabetes ²	Phase 2 (completed)	----->		Seeking partner for further development

Early Stage Development

Recombinant AAT	AAT Deficiency	Early development			
AAT (liquid)	Organ preservation	Ex-Vivo study			Massachusetts General Hospital

1. Orphan drug designation (US & EU); 2. Orphan drug designation (US only);
3. Mount Sinai Acute GVHD International Consortium

EXPERIENCED MANAGEMENT TEAM



Amir London	CEO
Chaime Orlev	CFO
Eitan Kyiet	VP Business Development
Eran Nir	VP Operations
Yael Brenner	VP Quality
Michal Ayalon, PhD	VP Research and Development
Orit Pinchuk	VP Regulatory Affairs & PVG
Naveh Tov, MD, PhD	VP Clinical Development & Medical Director (Pulmonary)
Michal Stein, MD	VP Medical Director (Immunology)
Ariella Raban	VP Human Resources



INCREASING REVENUE GROWTH AND PROFITABILITY



US \$ MM	FY 2015 Audited	FY 2016 Audited	% Change 2016/2015	FY 2017 Audited	% Change 2017/2016	FY 2018 Unaudited	% Change 2018/2017
Proprietary Products	43	56	30%	80	42%	91	14%
Distribution Products	27	21	-22%	23	8%	24	2%
Total Revenues	70	77	10%	103	33%	115	11%
Gross Profit	15	21	39%	32	50%	42	30%
Gross Profit (%)	22%	28%		31%		36%	
R&D	(17)	(16)		(12)		(10)	
S&M and G&A	(10)	(11)		(13)		(12)	
Operating Profit (Loss)	(11)	(5)		7		19	
Net Profit (Loss)	(11)	(7)	40%	7	204%	22	223%
Adjusted EBITDA¹	(6)	(1)	83%	11	1200%	24	108%

2019 Revenue Guidance of \$125-130M;
 An anticipated 9% to 13% Increase Over Full-Year 2018
 Continued Profitability and Positive Cash Flow

1. See Appendix A for Adjusted EBITDA reconciliation

Alpha-1 Antitrypsin Deficiency (AATD)



AAT DEFICIENCY

Potentially Lethal and Often Undiagnosed



AAT
Level



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

U.S. ●



● EU



**Affects more than
100,000 people in the
U.S. and slightly lower
number in Europe**

AAT
Deficiency



**Causes predisposition
to lung and liver
diseases**

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

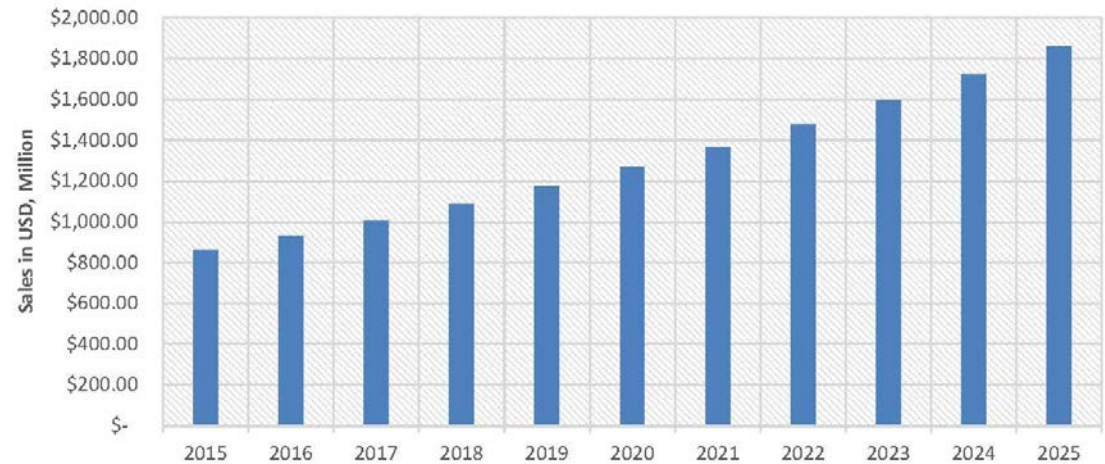
AAT DEFICIENCY (AATD) IS A \$1B MARKET

Significant expansion opportunity



- Majority of AATD patients are **undiagnosed & untreated**
- Better disease awareness and expanded diagnostics is contributing to increased demand
- Growing U.S. market – expect 6-8% annual growth¹
- Chronic therapy creates sustainable product revenue opportunity
- Average annual reimbursement (U.S.) ~**\$80-\$100K per patient**
- Greater AAT use in Europe and other regions could further accelerate market growth

Global Market Size(2015-2025) ¹



Source: DelveInsight

- **AATD prevalence ¹: Approx. 115,000 (U.S.) & 72,000 (EU5)** but only approx. 7,300 (U.S.) and 1,800 (EU5) patients treated ¹
- **Expected to reach \$1.8B by 2025**

**GLASSIA[®]:
Liquid AAT
for the
Treatment
of AAT
Deficiency**



GLASSIA® IS A DIFFERENTIATED PRODUCT



- Glassia® was the first liquid, FDA-approved ready-to-use, 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 Shire plc
- Self-infusion approved by FDA in 2016

GROWTH OF GLASSIA DRIVEN BY STRATEGIC PARTNERSHIP WITH SHIRE (NOW PART OF TAKEDA)

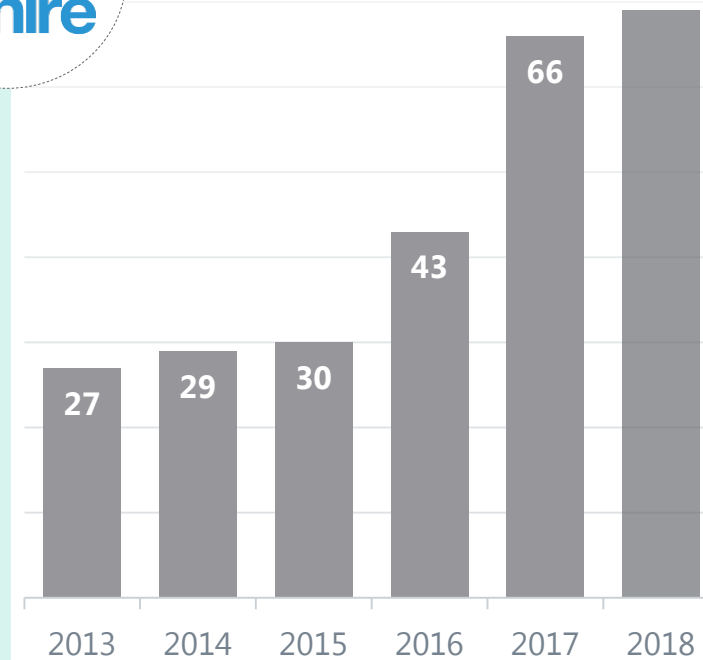


Significant Revenues to Kamada through 2020, followed by 20 Years of Royalties

- Cumulative minimum/maximum revenues of \$180MM/\$220MM to Kamada expected for 2018-2020
- Kamada is the BLA holder; Kamada manufactures and supplies Glassia to Shire/Takeda at least through 2020
- Commencing 2021, Shire/Takeda has option to manufacture Glassia and pay royalties (low DD%¹ through 2025 & SD%² thereafter) to Kamada through 2040
- Territories – U.S., Canada, Australia, and New Zealand
- Agreement covers all future AAT-IV indications in the territories



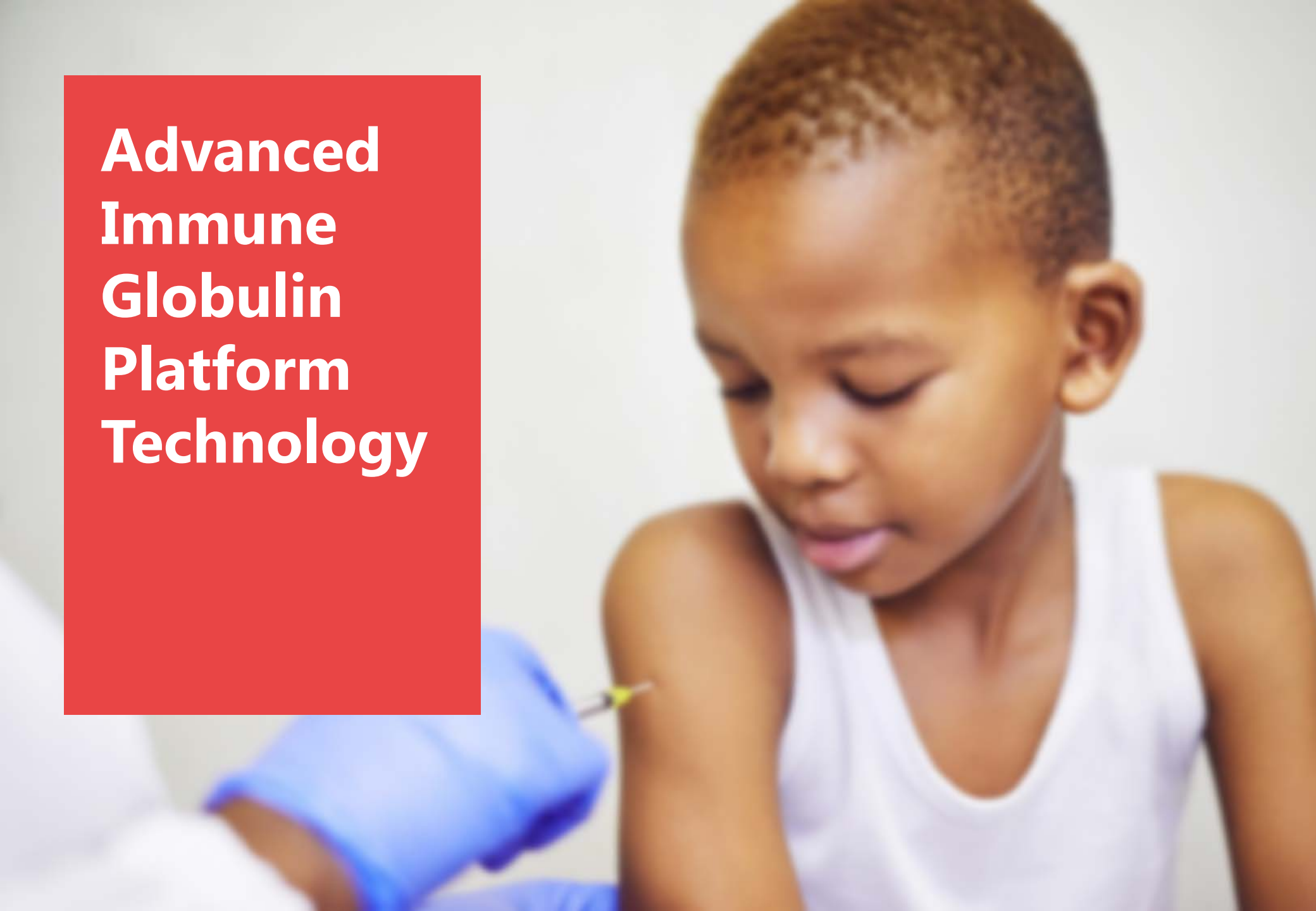
Glassia is sold in 5 countries majority of sales in the U.S.



Glassia World Wide Revenues
(in \$M)

1. Double Digits
2. Single Digits

**Advanced
Immune
Globulin
Platform
Technology**





KamRAB



Post-exposure prevention of rabies infection, FDA approved (as KEDRAB)



KamRho D IV



Second-line therapy for pediatric and adult patients with ITP¹



KamRho D IM



Prevention of Rh-D immunization in Rh-negative pregnant women



Anti- Snake Venom



Strategic partnership with the Israeli MOH, supplying all national immunization requirements



KamRAB/KedRAB

Human Rabies Immune Globulin



U.S. Market:

Strategic agreement with Kedrion for the clinical development and marketing of KedRAB in U.S.

KEDRION
BIOPHARMA

Substantial WW Market (WHO estimates)

~10 million people worldwide require medical treatment against rabies each year after being exposed to an animal suspected of rabies infection

U.S.

U.S. Market

- FDA Approved - August 2017
- Successful product launch: Q1/2018 in collaboration with Kedrion
- **~40,000 post-exposure prophylaxis treatments** administered each year, representing **~\$150 million market opportunity**¹

Worldwide

- More than 1.5MM vials sold by Kamada to date = **~ 300,000 people treated WW**
- Major markets: India, Thailand, Israel, Russia
- Approved Supplier of the WHO
- Health Canada Approved – November 2018
- November 2017: Signed a new \$13 MM supply agreement with an international organization for 2018-2020



1. The plasma protein market in the United States, 2017, The Marketing Research Bureau Inc

Distribution Product Segment



DISTRIBUTION PRODUCT SEGMENT

Exclusive distributor in Israel for leading biopharmaceutical companies



- Marketing and distribution of specialty pharmaceutical products of leading international pharmaceutical companies in the Israeli market through a dedicated salesforce
- Expanding product portfolio through new agreements

Main therapeutic fields	
Immunology	Hematology & Hemophilia
Hospital & Critical Care	Respiratory
Infectious Diseases	Transplantation



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



ANTICIPATED BENEFITS OF INHALED AAT



Inhaled AAT targeting a world wide market of over \$1B

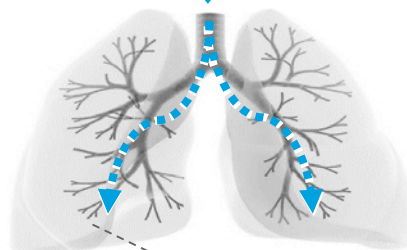
Alpha-1 Foundation survey¹ confirms high level of patients' interest in Inhaled-AAT

Improved Quality Of Life (QOL)

ELF² levels 2-3 fold greater than IV

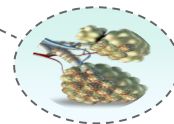
Most effective mode of treatment for reaching primary site of injury³

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

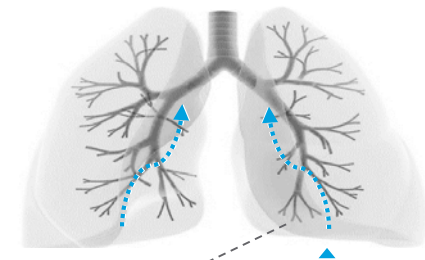


Airway Obstruction

- FEV1/SVC⁴
- FEV1

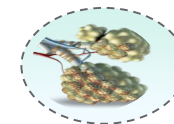


Intravenous AAT ~ 2% of the dosage reaches alveoli and airway tree



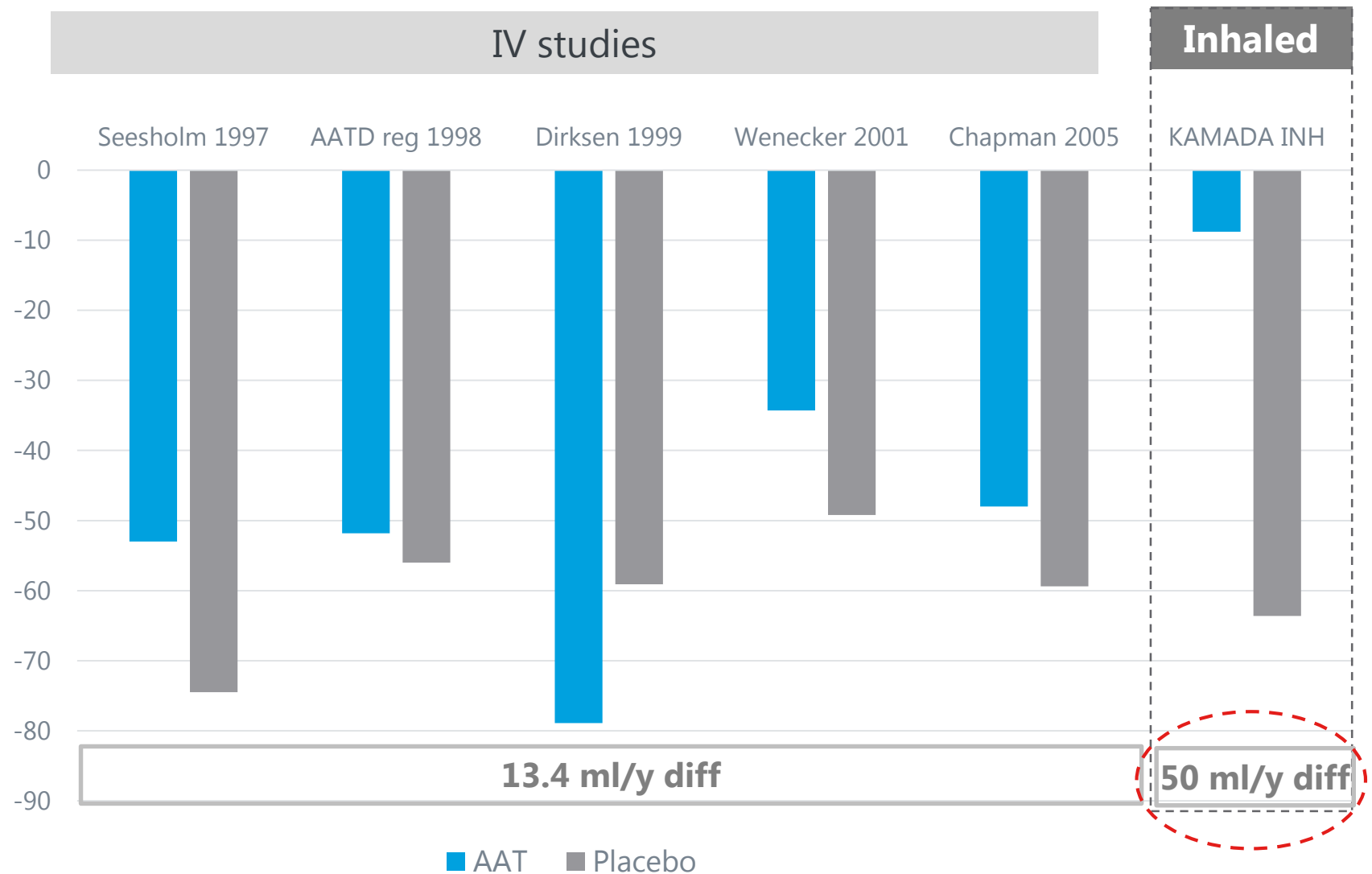
Alveoli Emphysema

- DLCO⁵
- CT densitometry



1. COPD: Journal of Chronic Obstructive Pulmonary Disease, Volume 10, 2013 - Issue 4; 2. ELF = Epithelial Lining Fluid
3. Kamada's clinical data; 4. Forced Expiratory Volume/Slow Vital Capacity; 5. Diffusing Capacity of the Lung for Carbon Monoxide

INHALED AAT DEMONSTRATED REDUCED FEV1¹ DETERIORATION COMPARED TO AAT-IV



1. FEV = Forced Expiratory Volume



U.S.



- Ongoing discussions with the FDA addressing concerns and questions regarding the safety and efficacy of Inhaled AAT for the treatment of AATD
- Revised proposed Ph3 protocol and additional information provided to the FDA during Q3 and Q4 2018
- Continued clinical development pending IND approval

EU



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

- Phase 2/3 completed; Study endpoints were not met; statistical significant lung function improvement was observed
- MAA submitted based on data showing Lung Function Improvements; MAA withdrawn (June 2017) EMA viewed data as insufficient for approval
- Proposed new Ph3 protocol accepted by EMA in a Scientific Advice meeting held July 2018

Considering all strategic options for Inhaled-AAT, including marketing partner

AAT REPRESENTS AN EXCITING POTENTIAL THERAPY FOR MULTIPLE INDICATIONS



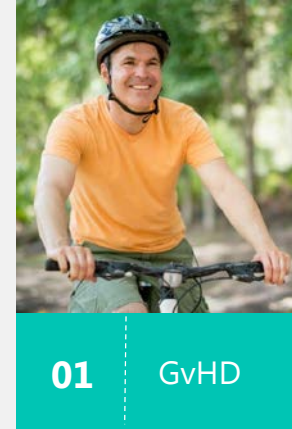
AAT
is a safe plasma-derived protein with known and newly discovered therapeutic roles

+ Anti-Inflammatory

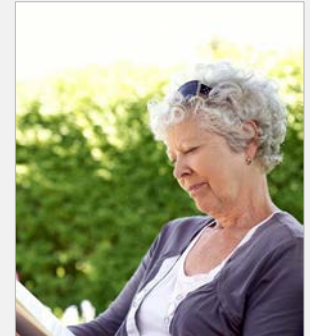
Y Immune Modulatory

Shield Tissue Protective

••• Antimicrobial



01 GvHD



02 Lung Transplant



03 Type-1 Diabetes

Excellent safety profile, encouraging clinical and pre-clinical experience coupled with biochemical rationale may position AAT as a high-potential future treatment in various indications.

01 GRAFT VERSUS HOST DISEASE (GVHD): A Major Complication in Hematopoietic Cell Transplantation



DEADLY SIDE EFFECTS

30-40%

of bone marrow transplantations will develop acute GvHD

40-50%

of acute GvHD will not respond to steroid treatment (SR-aGvHD)

~70%

mortality rate of patients with SR-aGvHD



SEARCHING FOR AN EFFECTIVE TREATMENT

No established prophylactic treatment

Existing prophylaxis may be associated with severe AEs

No established treatment for GvHD – An Unmet Medical Need

Estimated Market Size¹: ~ **\$500 MILLION**

01 COLLABORATION WITH MAGIC¹ TO EVALUATE AAT FOR PREEMPTION OF GVHD



Proof-of-Concept Study:

- Open label single arm multicenter study conducted in 5 US centers which are members of Mount Sinai Acute GVHD International Consortium (MAGIC)¹
- Study is co-funded by Mount Sinai and Kamada, and is sponsored by the Icahn School of Medicine at Mount Sinai (ISMMS) and Led by Prof James L.M. Ferrara, MD, and Prof. John Levine, MD, MS
- interim results expected to be available by the end of 2019

Kamada has exclusive rights to develop and commercialize AAT for preemption of GvHD using the MAGIC Biomarkers

Innovative approach

- Utilization of novel blood biomarker algorithms may identify patients at high risk of SR-GvHD and non-relapse mortality (NRM)
- Early intervention could prevent patients from further disease deterioration

Study objective

- To assess the safety and preliminary efficacy of IV AAT as preemptive therapy in patients at high risk for the development of SR-GvHD after BMT

Design

- 30 patients treated with IV AAT for 2 months with a follow-up period of 1 year after BMT

Endpoints

- Proportion of High Risk patients who develop SR-GvHD by day 100 post BMT, as well as safety, severity of GvHD, mortality, etc.

1. A consortium of 23 BMT centers in the USA, Europe and Asia that conducts clinical trials to prevent and treat acute GVHD (aGvHD).



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

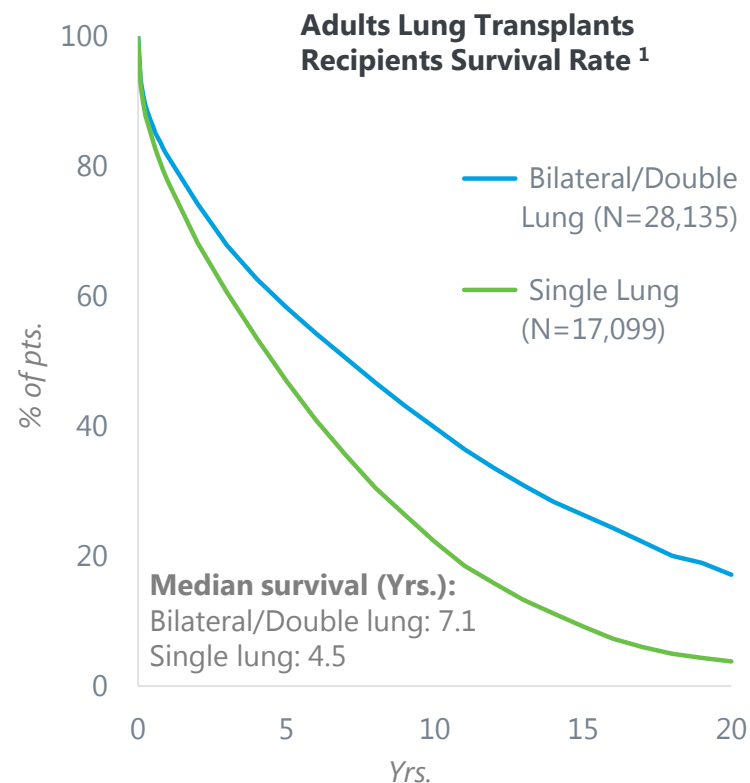


No new treatment options have been made available for years

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



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



Potential Market Size² ~ \$400-500 MILLION

02 ON-GOING 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 Shire led by Prof. Mordechai Kramer, Rabin Medical Center, Israel
- Next interim report will include data from one-year of treatment for all patients, and is expected during H1 '19
- Top-line data from the Phase 2 trial anticipated by the end of 2019.

Study objective

- To assess the safety of AAT IV 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

Endpoints

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

Interim results (29 Pts; 1 year)

- IV AAT demonstrated a trend towards improvements in multiple key clinical outcomes, including days on mechanical ventilation post-transplant, pulmonary function at week 4 and week 48 post-transplant and the six-minute walk test.

03 AAT (IV) AS POTENTIAL TREATMENT FOR NEWLY DIAGNOSED TYPE-1 DIABETES (T1D) PATIENTS



MARKET OPPORTUNITY	AAT IMPACT	EXPECTED BENEFITS
<p>Type-1 Diabetes Occurs when the immune system attacks and destroys beta cells in the pancreas</p>	<p>Studies have shown that AAT may protect beta cell islets</p>	<p>Preservation of beta cells correlates with reduced risk of long-term complications</p>

- 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 annually¹

- Preclinical models have shown that administration of AAT:
- Delays the progression of autoimmune diabetes
 - Inhibits insulinitis and beta-cell apoptosis
 - Decreases beta-cell inflammation

- DCCT² indicated that patients with C-peptide on MMTT ≥ 0.2 pmol/mL were less likely to develop retinopathy and hypoglycemia complications³
- Higher / sustained levels of C-peptide correlate with reduced incidences of the microvascular complications³

1. JDRF publication; 2. The Diabetes Control and Complications Trial (DCCT)
3. Greenbaum et al, 2012; 3. Steffes et al, 2013



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



Study objective

- To evaluate efficacy and safety of AAT) in treatment of newly diagnosed Type 1 Diabetes patients

Design

- Two doses, placebo controlled, randomized with 70 pediatric and young adult patients. One year study

Endpoints

- Beta cell preservation (C-peptide AUC), HbA1C, hypoglycemic events and insulin daily dose

Results

- In overall study population, no significant treatment effect was observed.
- In the pre-determined subgroup of patients between the ages of 12-18 years old, a trend toward better efficacy was demonstrated in the high dose arm of AAT (120mg/kg)
- Presented results in an oral session at 78th Scientific Sessions of the American Diabetes Association (ADA)

KEY VALUE CREATING 2019 MILESTONES



Inhaled AAT for AATD - Continued FDA discussion targeting approved IND	1H/2019
Inhaled AAT for AATD phase III pivotal study – First patient in (pending IND/CTA approval)	2H/2019
Organ preservation program – Define regulatory and commercial path	1H/2019
Lung transplant phase II study – Top line data	End of 2019
GvHD phase II study – Last patient in and interim report	End of 2019
Secure strategic collaborations through co-development agreements for IgG and /or AAT for new indications	2019
Achieve \$125-130 million in annual revenues, profitable, cash flow positive	2019



INVESTMENT HIGHLIGHTS



COMMERCIAL STAGE BIOPHARMA

- Leader in plasma-derived protein therapeutics, focused on Alpha-1 Antitrypsin (AAT) and specific hyper-immune IgGs
- 2 FDA approved products
 - *Glassia® for AAT Deficiency (AATD); first FDA-approved liquid, ready-to-use IV AAT. Commercialized in the U.S. through Shire plc. Estimated revenues: \$180-\$220 MM (2018-2020 cumulative); followed by 20 years of royalties.*
 - *KedRAB® for anti-rabies prophylaxis treatment. Commercialized in the U.S. through Kedrion Biopharma. Launched in Q1/2018.*

BROAD PIPELINE/ IP

- Focused on global leadership in AATD
- Inhaled AAT for AATD
 - *Completed Ph2 (U.S.) and Ph2/3 (EU), MAA withdrawn June 2017;*
 - *EMA accepted new pivotal Phase 3 design;*
 - *FDA discussions ongoing re development path forward;*
 - *New pivotal Phase 3 pending IND/CTA approval*
- AAT IV for other indications developed through strategic collaborations
- Fully integrated propriety manufacturing technology for protein purification from human plasma
- Distributed biopharmaceutical products segment in Israel

COMPELLING FINANCIAL PROFILE

- 2017 Revenue: \$102.8 MM (33% YoY)
- 2018 Revenue: \$114.5MM (11% YoY); profitable; cash flow positive
- 2019 Revenue guidance: \$125-\$130MM; profitable; cash flow positive
- Cash: \$50.6 MM (December 31, 2018); No Debt
- Strong balance sheet allows execution on pipeline and business development initiatives
- Listed on TASE (2005) & Nasdaq (2013)



THANK YOU
www.kamada.com



Appendix A: Reconciliation of Non-IFRS Measures

Adjusted EBITDA is defined as net income (loss), plus income tax expense, plus financial expense, net, plus depreciation and amortization expense, plus non-cash share-based compensation expenses, plus or minus income or expense in respect of exchange and translation differences and derivatives instruments not designated as hedging, and plus one-time management compensation payment.

We present adjusted EBITDA because we use this non-IFRS financial measure to assess our operational performance, for financial and operational decision-making, and as a means to evaluate period-to-period comparisons on a consistent basis. Management believes this non-IFRS financial measure is useful to investors because: (1) they allow for greater transparency with respect to key metrics used by management in its financial and operational decision-making; and (2) they exclude the impact of non-cash items that are not directly attributable to our core operating performance and that may obscure trends in the core operating performance of the business.

Non-IFRS financial measures have limitations as an analytical tool and should not be considered in isolation from, or as a substitute for, our IFRS results. We expect to continue reporting non-IFRS financial measures, adjusting for the items described below, and we expect to continue to incur expenses similar to certain of the non-cash, non-IFRS adjustments described below. Accordingly, unless otherwise stated, the exclusion of these and other similar items in the presentation of non-IFRS financial measures should not be construed as an inference that these items are unusual, infrequent or non-recurring. Adjusted EBITDA is not a recognized term under IFRS and does not purport to be an alternative to any other IFRS measure. Moreover, because not all companies use identical measures and calculations, the presentation of adjusted EBITDA may not be comparable to other similarly titled measures of other companies.

	(US\$K, Unaudited)	YE2015	YE2016	YE2017	YE2018
Net Income (Loss)		(11,270)	(6,733)	6,901	22,296
Taxes on income		0	1,722	269	(1,955)
Financial expenses (income), net		471	(343)	(338)	(480)
Depreciation and amortization expense		3,227	3,501	3,523	3,703
Share-based compensation charges		1,907	1,071	483	948
Expense (income) in respect of currency exchange and translation differences and derivatives instruments, net		(625)	(127)	612	(602)
Adjusted EBITDA		(6,290)	(909)	11,450	23,910

INHALED AAT – IN THE WORDS OF THE KEY OPINION LEADERS



EU Phase 2/3:

“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)

“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

“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.

US Phase 2:

“The results of this study are extremely compelling. Based on the results of this study, it is clear that inhaled AAT is the most effective mode of treatment for reaching the primary sites of potential lung injury, and restoring AAT inhibitory capacity. I look forward to the start of a pivotal study in the U.S. to confirm these results.”

Professor Mark Brantly, MD, the
Primary Investigator in this study
who serves as a Vice Chair of
Research, Department of Medicine,
Chief Division of Pulmonary, Critical
Care and Sleep Medicine, Professor
of Medicine, Molecular Genetics
and Microbiology at the University
of Florida College of Medicine and
Alpha One Foundation Research
Professor.

“AAT COULD BE AN EFFECTIVE TREATMENT OPTION FOR NEWLY DIAGNOSED 12-18 YEARS OLD T1D PATIENTS”



Peter Gottlieb, M.D.,
Professor of Pediatric and Medicine,
Barbara Davis Center for Diabetes,
University of Colorado School of
Medicine and a leading member in
TrialNet, an NIH-sponsored network of
institutions and researchers dedicated
to the prevention of type-1 diabetes.

“Given this study was not powered to show efficacy, the results are very encouraging.

These findings suggest that **administration of AAT could be an effective treatment option for newly diagnosed T1D patients who are 12-18 years old.** The results of this subgroup are intriguing and warrant further studies in a larger population.

Subgroup segmentation by age is common in this complicated disease, and the fact that we see the same positive trend in this age group for all three measures – C-peptide, daily insulin requirement, and HbA1C – suggests that the **results are consistent and could be promising.**”