

## **FDA Grants Kamada Orphan Drug Designation for the Treatment of Graft versus Host Disease**

**NESS ZIONA, Israel (October 29, 2014) – Kamada Ltd. (Nasdaq and TASE: KMDA)**, a plasma-derived protein therapeutics company focused on orphan indications, announces that the U.S. Food and Drug Administration's (FDA) Office of Orphan Products Development has granted orphan drug designation for Glassia®, the Company's proprietary human Alpha-1 Antitrypsin (AAT), to treat Graft-versus-host-disease (GVHD). Orphan drug designation carries multiple benefits, including the availability of grant money, certain tax credits and seven years of market exclusivity, as well as the possibility of an expedited regulatory process.

Preliminary human and animal studies indicate that Glassia may be able to treat and reduce the severity of GVHD, which is one of the key, life threatening complications of allogeneic stem cell transplantation. GVHD is an immunologically-based disease that may result in significant damage to the recipients' health including damage to multiple organs and tissues such as the liver, gastrointestinal tract, skin and mucosal membranes. Tissue destruction also leads to increased inflammatory signals, perpetuating and augmenting the disease process by contributing to the cytokine storm that fuels GVHD even further and, thereby, the damage continues and its intensity is increased.

In recent years, AAT has been investigated extensively and found to have anti-inflammatory, tissue protective, immune-modulatory and anti-apoptotic properties in direct or indirect consequence of its underlying anti-protease capabilities. These properties may attenuate inflammation by lowering levels of pro-inflammatory mediators such as cytokines, chemokines and proteases that are associated with this severe disease.

Currently, Glassia is being used in a Phase 1/2 clinical study that is being conducted by the Fred Hutchinson Cancer Research Center in Seattle, Washington in cooperation with Baxter International Inc. and Kamada. The Phase 1/2 study is evaluating 24 GVHD patients with inadequate response to steroid treatment following allogeneic bone-marrow stem cell transplant. The patients are enrolled into 4 dose cohorts, in which they receive up to 8 doses of Glassia. Interim data from this study is expected by the end of this year.

"We are pleased with the receipt of orphan drug designation for Glassia to treat GVHD as it is a key milestone that supports our broader regulatory and development strategy. Results from this Phase 1/2 study in GVHD may support global clinical development activities and may serve as a platform to apply for an expansion of the AAT indications to include general organ transplantation, based on a similar mechanism of action," stated David Tsur, co-Founder and Chief Executive Officer of Kamada. "GVHD is a disease of significant unmet medical need and both the disease and current therapy options carry considerable side effects."

"Given the favorable safety profile of Glassia, there is a strong rationale to support the development of this new indication and an increased likelihood of it becoming an effective therapy for this potentially life threatening disease. We will pursue discussion with the U.S. and European regulators with regard to our development pathway and with an aim to move forward with a more advanced study of Glassia to treat GVHD."

### About Graph-Versus-Host-Disease

Graft-versus-host-disease is a common complication following an allogeneic tissue transplant. It is commonly associated with stem cell transplant, but the term also applies to other forms of tissue graft. Immune cells (white blood cells) in the tissue (the graft) recognize the recipient (the host) as "foreign". The transplanted immune cells then attack the host's body cells.

GVHD occurs in 30-70% of patients who undergo a medical procedure of allogeneic hematopoietic stem cell transplantation (HSCT), usually as a treatment to leukemia or other blood cancer or blood conditions. HSCT is a stem cell transplantation that is usually derived from an external (allogeneic) bone marrow donor. One of the most common and dangerous complications of HSCT is GVHD. GVHD is expressed in damage to the recipients' tissues including damage to the liver, gastrointestinal system, skin and mucosal tissues, and is a major cause of morbidity and mortality in these patients.

Intravenously administered glucocorticoids, such as prednisone, are the standard of care in acute GVHD<sup>1</sup> and chronic GVHD.<sup>2</sup> The use of these glucocorticoids is designed to suppress the T-cell-mediated immune onslaught on the host tissues; however, in high doses, this immune-suppression raises the risk of infections and cancer relapse. In addition, more than 50% of patients do not respond well to steroids, and consequently have very low survival rates.

### About Glassia

Glassia is the first available ready-to-infuse liquid alpha1-proteinase inhibitor (Alpha1-PI) and is indicated as a chronic augmentation and maintenance therapy in adults with clinically evident emphysema due to severe congenital AAT deficiency. Glassia is administered intravenously once a week to augment the levels of AAT in the blood. AAT is a protein derived from human plasma with known and newly discovered therapeutic roles given its immunomodulatory, anti-inflammatory, tissue protective and antimicrobial properties. Glassia is approved by the U.S. Food and Drug Administration and is marketed through a strategic partnership with Baxter International Inc. in the United States. **Please see the full prescribing information for Glassia at:**

[http://www.baxter.com/downloads/healthcare\\_professionals/products/Glassia\\_PI.pdf](http://www.baxter.com/downloads/healthcare_professionals/products/Glassia_PI.pdf)

### About Kamada

Kamada Ltd. is focused on plasma-derived protein therapeutics for orphan indications, and has a commercial product portfolio and a robust late-stage product pipeline. The Company uses its proprietary platform technology and know-how for the extraction and purification of proteins from human plasma to produce Alpha-1 Antitrypsin (AAT) in a highly-purified, liquid form, as well as other plasma-derived proteins. AAT is a protein derived from human plasma with known and newly-discovered therapeutic roles given its immunomodulatory, anti-inflammatory, tissue-protective and antimicrobial properties. The Company's flagship product is Glassia®, the first and only liquid, ready-to-use, intravenous plasma-derived AAT product approved by the U.S. Food and Drug Administration. Kamada markets Glassia in the U.S. through a strategic partnership with Baxter International. In addition to Glassia, Kamada has a product line of nine other pharmaceutical products that are marketed through distributors in more than 15 countries, including Israel, Russia, Brazil, India and other countries in Latin America, Eastern Europe and Asia. Kamada has five late-stage plasma-derived protein products in development, including an inhaled formulation of AAT for the treatment of AAT deficiency that completed a pivotal Phase 2/3 clinical trials in Europe and has initiated Phase II clinical trials in the U.S. Kamada also leverages its expertise and presence in the plasma-derived protein therapeutics market by distributing 10 complementary products in Israel that are manufactured by third parties.

### Cautionary Note Regarding Forward-Looking Statements

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<sup>1</sup> Goker, H; Haznedaroglu, IC; Chao, NJ (2001). "Acute graft-vs-host disease Pathobiology and management". *Experimental Hematology* **29** (3): 259–77. doi:10.1016/S0301-472X(00)00677-9. PMID

<sup>2</sup> Menillo, S A; Goldberg, S L; McKiernan, P; Pecora, A L (2001). "Intraoral psoralen ultraviolet a irradiation (PUVA) treatment of refractory oral chronic graft-versus-host disease following allogeneic stem cell transplantation". *Bone Marrow Transplantation* **28** (8): 807–8. doi:10.1038/sj.bmt.1703231. PMID 11781637.

This release includes forward-looking statements within the meaning of Section 27A of the U.S. Securities Act of 1933, as amended, Section 21E of the US Securities Exchange Act of 1934, as amended, and the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical facts, such as statements regarding assumptions and results related to financial results forecast, commercial results, clinical trials, Intellectual Property, the EMA and U.S. FDA filings and authorizations and timing of clinical trials. Forward-looking statements are based on Kamada's current knowledge and its present beliefs and expectations regarding possible future events and are subject to risks, uncertainties and assumptions. Actual results and the timing of events could differ materially from those anticipated in these forward-looking statements as a result of several factors including, but not limited to, unexpected results of clinical trials, delays or denial in the U.S. FDA or the EMA approval process, additional competition in the AATD market or further regulatory delays. The forward-looking statements made herein speak only as of the date of this announcement and Kamada undertakes no obligation to update publicly such forward-looking statements to reflect subsequent events or circumstances, except as otherwise required by law.

**Contacts:**

Gil Efron

CFO

[ir@kamada.com](mailto:ir@kamada.com)

Anne Marie Fields

LHA

212-838-3777

[afields@lhai.com](mailto:afields@lhai.com)

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