Helsinn Group to present at ASCO pooled follow up efficacy data analysis from two Phase III studies for anamorelin in cachectic patients with non-small cell lung cancer (NSCLC)

Pooled, post-hoc efficacy data analysis from the ROMANA 1 and ROMANA 2 Phase III studies demonstrates that in NSCLC patients with cachexia and a low BMI, anamorelin significantly improves fatigue in addition to symptom burden (including appetite) and increases lean body mass and fat

Lugano, Switzerland, June 1, 2016 – Helsinn, the Swiss pharmaceutical Group focused on building quality cancer care, today announces that it will present the results of a pooled efficacy analysis of the pivotal ROMANA Phase III trials in a poster presentation at the American Society of Clinical Oncology (ASCO) meeting on June 6th, in Chicago, U.S.A.

The studies assessed the efficacy and safety of the ghrelin receptor agonist anamorelin in patients with Cancer Anorexia-Cachexia and non-small cell lung cancer (NSCLC). It was previously shown that in cachectic NSCLC patients, anamorelin significantly increased the lean and fat mass, and improved concerns related to anorexia-cachexia.

The analysis showed in the ASCO paper, *"Anamorelin in cachectic patients with non-small cell lung cancer (NSCLC) and low BMI (< 20 kg/m^2), a pooled efficacy data analysis of two phase III studies"*, assesses the response to anamorelin specifically in malnourished patients with a BMI below 20 kg/m^2. This post-hoc pooled analysis of efficacy data from two trials was conducted in patients with BMI < 20 kg/m^2 (N=182) and with BMI ≥ 20 kg/m^2 (N=647). Endpoints included changes in lean body mass (LBM), fat mass (FM), HGS, and changes in self-reported anorexia/cachexia concerns and fatigue.

Authors of the study were David Currow, Jennifer Temel, Amy Abernethy, Ruben Giorgino, John Friend and Ken Fearon. The poster #107 will be presented in Hall A from 1.00 pm to 4.30 pm.

David Currow, first-author of the paper, commented: *"This study suggests that malnourished patients with a low BMI respond well to anamorelin, in particular in terms of fatigue.*

This follow up data bolsters the previous strong data from the landmark Phase III ROMANA 1 and ROMANA 2 studies, showing that anamorelin increases lean body mass, fat and improves patient symptoms related to anorexia and cachexia."

Riccardo Braglia, Helsinn Group Vice Chairman and CEO, commented: *"At present there is no standard of treatment care for the management of Cancer Anorexia-Cachexia. Although some currently available drugs can improve patient's appetite or increase body weight, none can substantially affect lean body mass nor fatigue. The data from the ROMANA studies has already shown anamorelin to increase lean body mass, fat and alleviate patient concerns related to anorexia-cachexia, including appetite. This new analysis suggest that in those patients with a low BMI anamorelin has a particularly significant impact on the lean and fat mass, fatigue and patient's concerns related to anorexia-cachexia..*

"Helsinn is committed to helping provide options for people with cancer and these latest new data are supporting the potential for anamorelin."

Study design and methods

Stage III/IV NSCLC patients with cachexia (≥ 5% weight loss during prior 6 months or BMI < 20 kg/m^2) were randomly assigned (2:1) to daily oral 100 mg anamorelin or placebo for 12 weeks. A post-hoc pooled analysis of efficacy data from two phase 3 trials was conducted in patients with BMI < 20 kg/m^2 (N=182) and with BMI ≥ 20 kg/m^2 (N=647). Endpoints included changes in lean body mass (LBM), fat mass (FM), HGS, and changes in self-reported anorexia/cachexia concerns and fatigue.

Results

Compared with placebo, anamorelin significantly increased LBM both in patients with low BMI (treatment difference: 1.71 kg [95% CI 0.88 – 2.54]) and in those with normal/high BMI (1.47 kg [1.00 - 1.94]) (p<0.001). Greater increases in FM were observed in the BMI < 20 kg/m^2 (1.66 kg [0.86 – 2.46], p<0.001) than in the BMI ≥ 20 kg/m^2 subgroup (0.79 kg [0.30 - 1.28], p=0.002). There were no treatment differences in HGS. In patients with low BMI, anamorelin significantly improved vs placebo anorexia/cachexia symptoms (5.27 [2.11 - 8.43], p=0.001) and fatigue (3.94 [0.56 – 7-32], p=0.023), while these were not significant in patients with BMI ≥ 20 kg/m^2

(anorexia/cachexia symptoms: 0.91 [(-0.56) - 2.37], p=0.224; fatigue: (-0.42) [(-2.07) – 1.23], p=0.616).

ROMANA 1 and ROMANA 2 Phase III studies

The ROMANA 1 and ROMANA 2 phase III studies, which have previously been reported, clinically demonstrated that anamorelin significantly improved, in respect to placebo, lean body mass, fat and body weight, in addition to symptom burden, including appetite, in NSCLC patients with Cachexia. No differences between patients treated with anamorelin or placebo were observed for handgrip strength, one of the co-primary endpoint of the study.

Notes for editors:

Cancer Anorexia-Cachexia

Cancer Anorexia-Cachexia is a frequent condition in patients with advanced cancer, in particular in those with lung cancer. A combination of reduced food intake and altered metabolism leads to loss of muscle/lean body mass and body weight in patients affected by this condition.

NSCLC

Non-small cell lung cancer accounts for roughly 85% of all lung cancer cases. Lung cancer has some of the poorest survival rates comparing to other types of cancer, based on epidemiological data, and is the most common form of cancer globally.

About anamorelin and ghrelin

Anamorelin is an investigational agent that has not yet been approved by any regulatory authority. The marketing authorization application is under review by the European Medicines Agency

Anamorelin HCl is an investigational selective, novel, orally active ghrelin receptor agonist that is under development for the treatment of Anorexia, Cachexia, and Unintended Weight Loss in NSCLC patients. Ghrelin is an endogenous peptide primarily secreted by the stomach. Upon binding to its receptor, ghrelin stimulates multiple pathways in the positive regulation of body weight, lean body mass, appetite and metabolism.

The information discussed in this release is not intended to convey conclusions about its efficacy and safety.

About the Helsinn Group

Helsinn is a privately owned cancer supportive care pharmaceutical group with an extensive portfolio of marketed products and a broad development pipeline. Since 1976, Helsinn has been improving the everyday lives of patients, guided by core family values of respect, integrity and quality, through a unique integrated licensing business model working with long standing partners in pharmaceuticals, medical devices and nutritional supplement products. Helsinn is headquartered in Lugano, Switzerland, with operating subsidiaries in Ireland and the US, a representative office in China, as well as a product presence in about 90 countries globally.

In 2016, our 40th anniversary year, you can meet representatives from Helsinn at:

- ASCO Annual Meeting (Chicago, USA, 3-7 June)
- MASCC Annual Meeting (Adelaide, Australia, 23-25 June)
- ChemOutsourcing Conference (Parsippany, New Jersey, 19-21 September)
- CPhI Worldwide (Barcelona, Spain, 4-6 October)
- ESMO Congress (Copenhagen, Denmark, 7-11 October)
- BioEurope (Köln, Germany, 4-6 November)

For more information, please visit www.helsinn.com.

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As part of its patient-centered mission and support of the oncology community, Helsinn works closely with Patient Advocacy Groups. These key stakeholders protect the interests of cancer patients by helping them to receive appropriate and timely care, education, support and financial assistance, when needed.