

## **Axelar reports good safety profile of AXL1717 in Phase I/II in cancer patients**

**STOCKHOLM, SWEDEN - September 26, 2011. Axelar AB, a Karolinska Development AB portfolio company, today releases results of the completed Phase I/II clinical safety study for its drug candidate AXL1717 at the European Multidisciplinary Cancer Congress (ESMO) meeting in Stockholm, Sweden. All major objectives were met in the study and signs that suggest clinical benefit were observed in some patients with non-small cell lung cancer.**

AXL1717 is the first targeted oral small-molecule Insulin-like Growth Factor 1 (IGF-1) receptor inhibitor clinically tested which does not affect the closely-related insulin receptor. The drug was administered to a total of 49 advanced-stage cancer patients with progressive solid tumors who had no remaining treatment options. The main purpose of the study was to investigate the safety profile of AXL1717. The open label study consisted of three parts: a single day twice-daily ascending dose part, a 7-28 days twice-daily dose-finding part, and an extension part at a fixed twice-daily dose for 28 days in two cycles 14 days apart.

All major objectives have been met in the clinical study and the results from the study show that:

- AXL1717 was well-tolerated with no unexpected drug-related dose-limiting adverse events even at exposures being 5-10 fold higher than those needed for obtaining anti-tumor activity in animals. No changes in blood levels of glucose, insulin or C-peptide were reported.
- Dose-related, easily-monitored and reversible neutropenia was the only dose-limiting event. Since neutropenia has been shown in toxicity studies in animals, this event was expected to occur also in man. The recommended Phase II dose was found to be 400 mg AXL1717 twice-daily as monotherapy in a continuous 28-day treatment. At this dose only few neutropenias were registered.

Fifteen non-small cell lung cancer (NSCLC) patients administered with AXL1717 as single agent treatment with a total duration of at least two weeks were reported. The patients were assessed with imaging at the start of the study and thereafter every two months.

In spite of the study design with repeated wash-out periods and frequent dose changes, signs that suggest clinical benefit were observed in these fifteen NSCLC patients. Partial response in one of the NSCLC patients, according to RECIST criteria, was reported following treatment with AXL1717 as monotherapy. At cut-off, five patients were still alive and two of these patients had not reported progression. The results may therefore improve further over time.

The next step will be to study clinical efficacy with continuous dosing of the AXL1717 drug candidate in a randomized Phase II study in NSCLC patients, a program which is fully financed.

**Dr. Johan Harmenberg, CEO, Axelar:**

“We are very satisfied with the results of the study that support Axelar’s strategy to direct the upcoming randomized Phase II program towards patients with non-small cell lung cancer. We believe that AXL1717 has the potential to become an effective treatment in patients with lung cancer.”

**Dr. Torbjörn Bjerke, CEO, Karolinska Development:**

“Axelar is one of Karolinska Development’s prioritized companies. The data presented today from the completed clinical study strengthens our belief that AXL1717 can become an important novel treatment for patients with non-small cell lung cancer, and potentially those with other tumors. We look forward to the next important milestone which is the initiation of the randomized Phase II study.”

A poster with more detailed data from the clinical study will be presented at ESMO at 2.00 p.m. today and thereafter it will also be available on Axelar's and Karolinska Development's websites.

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**TO THE EDITORS**

**About Axelar**

Axelar AB is a Swedish biotech company founded in 2003. The company is developing insulin-like growth factor-1 (IGF-1) receptor inhibitors for treatment of cancer and other diseases. Axelar is part of the Karolinska Development portfolio of companies. [www.axelar.se](http://www.axelar.se)

**About AXL1717**

Axelar's lead compound, AXL1717 provides a novel potential treatment regimen for a wide range of cancers. AXL1717 is the first targeted oral small-molecule Insulin-like Growth Factor 1 (IGF-1) receptor inhibitor with no observable effect on the closely-related insulin receptor. Most tumor cells are dependent on the IGF-1 receptor signal pathway and the IGF-1 receptor is therefore regarded as a promising target for cancer therapy. To date, there are no IGF-1 receptor inhibitor drugs on the market. AXL1717 has just completed its first-in-man phase I/II clinical trial and the compound has already demonstrated a superior preclinical efficacy against numerous tumors and an excellent tolerability profile.

**About Karolinska Development**

Karolinska Development aims to create value for investors, patients, and researchers by developing innovations from world class research into products that can be sold or out-licensed with high returns. The business model is to: SELECT the most commercially attractive medical innovations; DEVELOP these to the stage where the greatest return on investment can be achieved; and COMMERCIALIZE the innovations through the sale of companies or out licensing of products. This will result in upfront payments, milestone payments and royalties.

An exclusive deal flow agreement with Karolinska Institutet Innovations AB, along with other cooperation agreements with leading Nordic universities, delivers a continuous flow of innovations.

Karolinska Development's flexible exit strategy enables projects to be exited at whichever stage of development offers the greatest return on investment, usually after Phase II clinical trials have indicated the desired pharmaceutical effect on patients - this being an important value enhancing step.

Today, the portfolio consists of over 35 projects at various stages, from concept development to Phase II clinical trials, twelve projects are in clinical trials. The portfolio is particularly strong in the areas of cancer, dermatology, inflammation, cardiovascular disease, women's health and diseases that affect the central nervous system. For more information, see [www.karolinskadevelopment.com](http://www.karolinskadevelopment.com).