



EctoPharma Awarded Top SMART: SCOTLAND Grant

Green light for research into pioneering new cancer product

Selkirk, 5th December 2010. EctoPharma, the Borders-based emerging pharmaceutical development company, today announces that the Company has been awarded the maximum Feasibility Study grant of £70,000 under Scottish Enterprise's SMART: SCOTLAND Scheme which supports innovative research and development projects within small and medium sized enterprises in Scotland.

The award comes in response to EctoPharma's application for funding towards advanced research into the company's Ryboquin™ product which has the potential to significantly improve the efficacy of existing chemotherapy treatments against a range of common cancers.

Ryboquin™ uses pioneering new nano-technology to deliver gene-blocking RNA direct to cancer cells in the body, halting or significantly reducing growth in tumours and potentially saving or prolonging millions of lives throughout the world.

Initial research carried out this year by the London School of Pharmacy has produced extremely positive results in controlling pancreatic tumour growth. The next step is to carry out research into Ryboquin's effectiveness against refractory prostate cancer, the most difficult form of prostate cancer to treat. It is this important area for which the SMART award is being made. Further research into Ryboquin™ will now be carried out in Scotland by Dundee-based contract research organisation, CXR. EctoPharma hopes to initiate "in man" studies in 2012.

Commenting, EctoPharma CEO, **Alan Walker**, said: "We are delighted to receive this SMART: SCOTLAND award which recognises the potential of our Ryboquin™ product. There is enormous global demand for a product which can boost the effectiveness of chemotherapeutic agents in the fight against a number of common cancers. Results from our early research demonstrate that Ryboquin™ helps to significantly reduce tumour growth rates, offering real hope to terminally ill patients. Ryboquin™ has the potential to revolutionise cancer treatments across the globe. I believe the work we are carrying out could act as a major beacon for the vitally important Scottish life science sector as a whole."

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Dr Chris McNab, SMART: SCOTLAND Programme Executive at Scottish Enterprise, said:
“Companies like EctoPharma with a commitment to commercially focussed research and development are those best placed to compete and grow. EctoPharma is using major advances in biotechnology for the development of Ryboquin™ and this project has the potential to bring hope to cancer sufferers and their families worldwide as well as growing Scotland's reputation as a global centre of innovation in Life Sciences.”

NOTES TO EDITORS

EctoPharma

Based in the Scottish Borders, EctoPharma is a privately held, virtual pharmaceutical search and development company focused on commercialising patented technologies in the areas of human and veterinary medicine. The company takes forward research and development into under-developed intellectual property with the aim of producing new, effective medicines and therapies for which there is significant demand from patients and healthcare professionals around the world. It is one of very few Scottish life science companies to have taken a product (Kindaped for the treatment of headlice) from earliest research to a Phase 3 trial and then on to commercialisation.

Scottish Enterprise

Scottish Enterprise is Scotland's main economic development agency and aims to deliver a significant, lasting effect on the Scottish economy. Our role is to help identify and exploit the best opportunities for economic growth. We support ambitious Scottish companies to compete within the global marketplace and help build Scotland's globally competitive sectors. We also work with a range of partners in the public and private sectors to attract new investment to Scotland and to help create a world-class business environment. Grants from SMART: SCOTLAND support technical and commercial feasibility studies and R&D for innovative projects which represent significant technological advance with an industry.

Ryboquin™

Background:

- In over 50% of cancer cases, cancerous cells fail to respond to chemotherapy at some time in the course of the treatment.
- One of the great challenges in the cancer field is how to overcome current prevalence of chemo-resistant cancers.
- Majority of non-responsive patients will die within 5 years of diagnosis.
(Source: *Cancer Research UK*)
- Ryboquin™ offers the potential to make these patients responsive to chemotherapy.
- Ryboquin™ is a fusion of two Nobel prize winning technologies: Ubiquitin (2004) and siRNA (2007) resulting in a therapy which has been shown *in vitro* to be effective in treating drug resistant cancers.
- Ryboquin™ employs novel nano-technology to deliver agents with the potential to significantly improve receptiveness of cancer cells to chemotherapy.

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The Process: How RyboQuin™ works:

- In most cases, chemotherapy aims to promote the cell death (suicide or apoptosis) of cancer cells.
- Sufficient levels of a protein called p53 are required in the cancer cells to trigger cell death. p53 is a tumour suppressant.
- However, in over 50% of tumours, p53 is deficient in the cancer cells (p53 null or p53 mutated) and chemotherapy fails to have the desired effect.
- Another protein from the same family - p73 - can substitute act as the necessary trigger mechanism.
- But p73 exists only at low levels in cells, so it has to be boosted (up-regulated).
- This can be achieved by switching off an enzyme called Itch Ubiquitin Ligase (Itch for short) which controls p73 levels.
- Proteins within cells are broken up when they have 4 Ubiquitin molecules attached to themselves.
- Preventing the Ubiquitin molecules from performing – ie. degrading or down-regulating Itch – allows p73 to go on accumulating in cells.
- Higher levels of p73 mean the cancer cells are more susceptible to cell death as a result of sustaining DNA damage caused by chemotherapeutic agents. In other words, the cells are less likely to be chemotherapy resistant.
- The technology which down-regulates Itch is based on small interfering RNA (siRNA) which prevents messenger RNA (mRNA) from creating the Ubiquitin Ligase enzyme.
- The challenge is how to deliver the ribonucleic acids (ie. siRNA) direct to the cancer cells because they rapidly break down before reaching the cells.
- The London School of Pharmacy has discovered a way of creating nano-particles which are a combination of a dendrimer (a small polymer) and a DNA gene grown in bacteria (a plasmid gene).
- This nano-particle – the Ryboquin™ product – is delivered into the body intravenously. DNA separates when Ryboquin™ meets the cancer cells and siRNA is created which then blocks the Ubiquitin Ligase, so increasing the levels of the p73 protein.

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