Treat U, S.A.

PEGASEMP: Novel, versatile and profitable technological platform for cancer therapy

Spin-off
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UTEN – University Technology Enterprise Network
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Bluepharma Indústria Farmacêutica, S.A.
Cancer accounts for nearly one-quarter of deaths in the United States, exceeded only by heart diseases. In 2004, there were 553,888 cancer deaths in the US. Cancer is the second most common cause of death in these countries after cardiovascular diseases. There still remains a high unmet need for effective cancer therapies.
Surgery, chemotherapy and radiotherapy are likely to remain the cornerstones of cancer therapy in future, despite the fact that they often significantly impair the patient's quality of life.

Cancer cells share many common features with the normal host cells from which they derive. Consequently, the high levels of selective toxicity that can be achieved with bacterial or viral chemotherapeutics cannot be achieved with anticancer chemotherapeutics because of the lack of unique molecular targets on cancer cells. Most, if not all, cancer chemotherapeutics that are in common use at present — including doxorubicin, vincristine, cyclophosphamide, topotecan and paclitaxel — owe what little selectivity they have for cancer cells to their higher proliferation rates. This can lead to increased toxicities against normal tissues that also show enhanced proliferative rates, such as the bone marrow, gastrointestinal tract and hair follicles. Side effects that occur as a result of toxicities to normal tissues mean that anticancer chemotherapeutics are often given at suboptimal doses, resulting in the eventual failure of therapy; this is often accompanied by the development of drug resistance and metastatic disease. An example of this is the dose-limiting cardiotoxicity that accompanies ANTHRACYCLINE therapy, which results in upper limits being placed on the maximum drug exposure of these otherwise effective drugs.

The selective toxicity of an anticancer drug can be increased by either increasing the dose of the drug that reaches the diseased tissue or by decreasing the dose that reaches normal tissues, but, ideally, both will occur. Several approaches for improving the selective toxicity of anticancer therapeutics are being pursued at present.

Many current or potential anticancer drugs have nonideal properties (Table 1), which can lead to a number of adverse consequences, including lack of or sub-optimal therapeutic activity, dose-limiting side effects and poor patient quality of life. Nanoscale drug delivery systems (DDS), defined as DDS having particle diameters of approximately 100 nm or less, are attracting considerable attention as a means of overcoming some of the limitations of 'conventional' anticancer drug therapy. Nanoscale DDS include liposomes and lipid-based carriers, polymer-drug conjugates and other polymer-based DDS and site-directed DDS such as immunoliposomes, immunopolymers, immunoconjugates and immunotoxins.
PEGASEMP: the rationale

Specific targeting for 2 distinct populations in the tumor microenvironment

Ferrari M
Nat Rev. 66, 1, 2005.
How does it compare with competitor against breast cancer?

- 180-fold more active than competitor
- Tumor uptake is 18-fold higher than competitor

Suppression of tumor invasion into adjacent healthy tissues
Historical perspective

**Sept 2005**  
Project funded by FCT  
(Targeted drug delivery to tumor vasculature: a new therapy against human breast cancer)

**May 2008**  
Patent filed to the United States Patent Trademark Office

**June 2008**  
"Smart nanomedicines for cancer"  
Best presentation award at iLechpartner Forum - Medtech

**May 2009**  
International extension of the patent

**Nov 2009**  
"Smart Medicines" – InovCapital award  
(Arrisca Coimbra 2009)

**Jan 21st 2010**  
Treat U, Lda

**April 2010**  
"Smart Medicines" – 2nd place award  
(12P idea to product competition 2010 - COTEC)

**September 2011**  
Treat U, S.A.
"LIGAND-MEDIATED TARGETED DELIVERY OF NUCLEIC ACIDS TO ANGIOGENIC-DEPENDENT DISEASES"

"MULTI-TARGETING SYSTEM COMPRISING A NANOCARRIER, NUCLEIC ACID(S) AND NON-NUCLEIC ACID BASED DRUG(S)"

Exclusive license agreement between UC/CNC and the promoters
Teva Pharmaceutical Industries Ltd. is a top 20 global pharmaceutical company specializing in the development, production and marketing of generic and proprietary branded pharmaceuticals. Teva operates in more than 50 countries and employs approximately 28,000 people. We offer the widest range of cost-effective pharmaceuticals, both generic and branded, to patients and healthcare providers. Known as the leading generic company worldwide, Teva also is committed to developing proprietary (innovative) pharmaceutical products through in-licensing pre-clinical and clinical stage compounds.
We have performed a qualitative evaluation between our technology and the main competitors, including the ones in clinical trials.

This technological advantages translate into unique properties of the product that generate added value to the end users.
Once the development of the product is completed we estimate a market value of 1 billion dol for our product, which corresponds to 5% market share of targeted therapies within breast cancer.

Based on this estimates, and according to our business model we believe we can obtain an upfront payment of around 11-15 million dollars.
PEGASEMP: final remarks

- Breakthrough technological platform
- Unique product pipeline (nature of the delivered material or tumor targeted)
- Addresses unmet market needs

Business sustainability
Thank you

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