



White Paper

**New Breakthrough Anti-Cancer Treatment**

***SapC-DOPS Nanovesicles***

## Problem

Cancer is currently the leading cause of death worldwide according to the World Health Organization (1). In 2007, approximately 7.9 million people died of cancer, and over 12 million new cases are diagnosed each year. As a result, cancer is the world's most costly disease (2). In terms of disability and years of life lost, cancer's global economic toll was \$895 billion (1.5% of world GDP) in 2008. In the U.S., the National Institutes of Health estimates overall cancer costs in 2010 at \$264 billion, i.e. \$103 billion for direct medical costs and \$161 billion due to indirect costs associated with loss of productivity to illness and death (3).

Almost everyone has experienced the devastating heartache of cancer either as a patient or with the loss of loved family members or close friends.

## Solution

With much anticipation and hope, the long-awaited, silver bullet *cure for cancer* may have recently been discovered. Bexion Pharmaceuticals is developing a new breakthrough anti-cancer technology (SapC-DOPS nanovesicles) that has shown exceptional success in the treatment of many different types of cancer in laboratory animals with no side effects.

What are SapC-DOPS nanovesicles and how do they work? SapC-DOPS nanovesicles are tiny lipid micro-bubbles (dioleoylphosphatidylserine = DOPS) that contain a unique human protein (saposin C = SapC). In the body, SapC-DOPS nanovesicles stick only to cancer cells inside a cancer and increase *natural cell death* (apoptosis) in the cancer to kill it. Treatment with SapC-DOPS nanovesicles is very safe, because it uniquely causes natural cell death of cancer cells without toxic side effects and does not affect any healthy cells in the body.

The SapC-DOPS nanovesicles are also useful for delivering dyes into cancers that can help oncologists visualize and detect cancer very early. The ability to stick only to cancer cells also makes SapC-DOPS nanovesicles an exciting new tool for early diagnosis of cancer.

## Results

New research findings on SapC-DOPS nanovesicles for the diagnosis and treatment of cancer in laboratory animals are simply stunning and very promising. The anti-cancer activity of SapC-DOPS nanovesicles was discovered and first published by researchers at Cincinnati Children's Hospital Medical Center (4). These researchers showed that SapC-DOPS nanovesicles killed human brain, nerve, and breast cancer cells in test tubes. They also showed that SapC-DOPS nanovesicles stopped the growth of human brain and nerve cancers transplanted into mice with no adverse effects after 5 weeks of dosing.

Additional unpublished research sponsored by Bexion Pharmaceuticals has shown that SapC-DOPS nanovesicles kills over 50 different types of human cancer cells in test tubes. By adding a dye to SapC-DOPS nanovesicles, the Bexion scientists have been able to visualize penetration of the drug into several different types of cancers in rats and mice including pancreas, breast, and brain cancers, with no penetration into healthy tissues. Of importance, SapC-DOPS treatment significantly increased (doubled) survival times and completely cured many mice with either brain or pancreatic cancer. Sixty-seven (67%) of mice with pancreatic cancer survived with SapC-DOPS treatment. In total, SapC-DOPS nanovesicles have been shown to be highly effective for treating cancer in 7 different mouse cancer models. In these extensive pre-clinical (animal) studies, there have been no adverse effects on animal behavior, body weight, feed consumption, organ weights, clinical pathology blood tests, or tissue pathology.

## Future

As a result of these exciting pre-clinical findings, the National Cancer Institute (NCI) has selected Bexion Pharmaceuticals as a collaborative research partner to advance research on SapC-DOPS nanovesicle treatment of cancer. The purpose of this partnership is to speed the generation of scientific data needed for filing an investigative new drug (IND) application to the U.S. Food & Drug Administration (FDA). This NCI research will begin in Q3 of 2010. An IND submission to the FDA is planned for Q2 of 2011. A clinical research plan is being developed, and first-in-man (FIM) phase I research studies are planned for Q3 of 2011.

If similar results are found in human cancer patients, SapC-DOS nanovesicle treatment of cancer will have tremendous potential to increase survivability and reduce the extensive medical and societal costs associated with cancer world-wide. SapC-DOPS nanovesicles just may be the long-awaited silver bullet cure for cancer and *the answer to millions of prayers* by cancer patients and families around the world.

## References

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3. American Cancer Society. *Cancer facts and figures 2010*. ACS, Atlanta, GA, USA, 2010. <http://www.cancer.org/Research/CancerFactsFigures/CancerFactsFigures/cancer-facts-and-figures-2010>
4. Qi X., Chu Z., Mahller Y. , Stringer K., Witte D., Cripe T. Cancer-selective targeting and cytotoxicity by liposomal-coupled lysosomal saposin C protein. *Clin Cancer Res.* 15(18): 5840-5851, 2009.