

Cancer Remains a Serious Health Issue

Deaths in 2015 (United States, CDC)

1. Heart disease: 614,348

2. Cancer: 591,699

1. Now the leading cause of death in 22 States

1. One in three women will develop cancer

2. One in two men will develop cancer

3. 14.5 million are diagnosed with cancer

3. Chronic lower respiratory diseases: 147,101

4. Accidents (unintentional injuries): 136,053

5. Stroke (cerebrovascular diseases): 133,103

6. Alzheimer's disease: 93,541

7. Diabetes: 76,488



Finding a cure to cancer would be worth about \$47 trillion to the U.S. Economy alone.

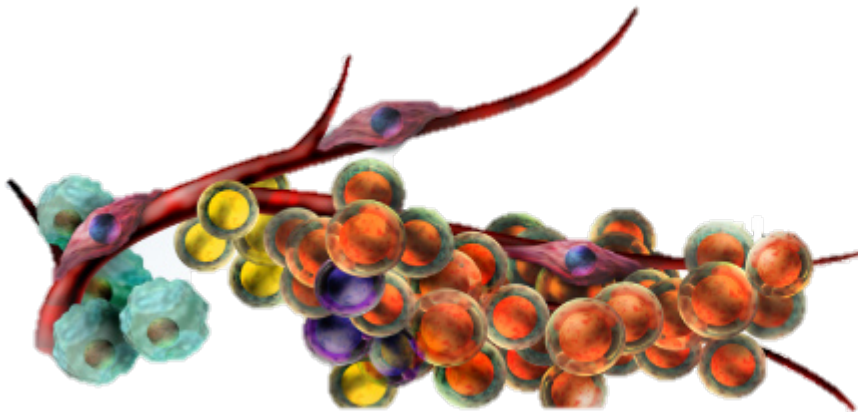
(Wu, "Cure for Cancer". 2006)

So why is finding a cure so hard?

Cancer has many faces:

National Cancer Institute (NCI) – “Cancer” consists of more than 100 diseases...

Even a specific kind of cancer has many faces:



Tumor cells are heterogeneous AND they create their own microenvironments



And yet, this battle has raged for decades



Screenshot of Opdivo commercial

“The next step—the complete cure - is almost sure to follow.”

—Kenneth Endicott, NCI director, 1963

IT CAN KILL CANCER!

REALLY!?!?



**10,000 Times Stronger against
CANCER than the leading
chemotherapy treatments**

Read more at <http://livelovefruit.com/2012/09/soursop-the-cancer-killer-we-never-knew-about/>



What if there was a “universal marker” and what if we could exploit it to defeat cancer?

There is a universal marker...it is a fat molecule (lipid) called phosphatidylserine.

In fact, these “blebs” on this cancer cell are rich in that lipid and they help the tumor grow and metastasize

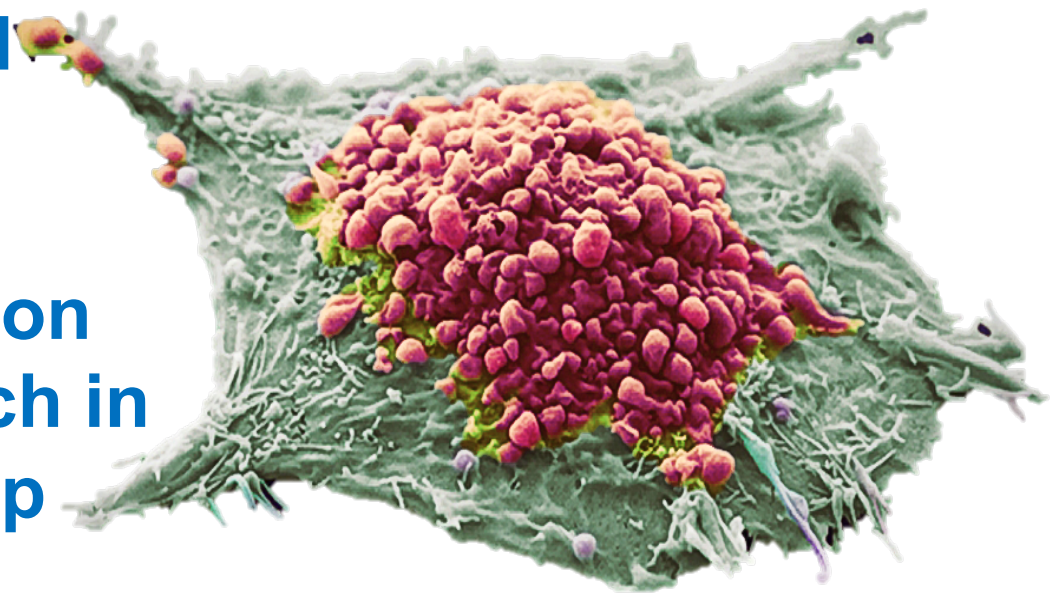


Photo by Anne Weston, Cancer Research UK

Image: Cells from culture were imaged with a JEOL 6700 Field Emission Scanning Electron Microscope, and then false colored with Adobe Photoshop



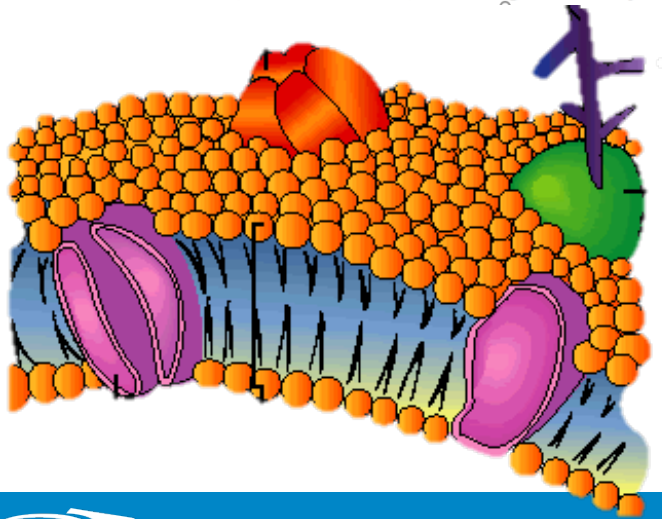
Phosphatidylserine (PS) is one of cancer's first offensive maneuvers against our immune system

PubMed
Central

Sponsored document from
Biochimica et Biophysica Acta

In search of a novel target — Phosphatidylserine exposed by non-apoptotic tumor cells and metastases of malignancies with poor treatment efficacy

Sabrina Riedl^{a,1}, Beate Rinner^{b,1}, Martin Asslaber^c, Helmut Schaidler^{b,e}, Sonja Walzer^{d,2}, Alexandra Novak^b, Karl Lohner^a, and Dagmar Zweytick^{a,*}



PS “shields” cancer from our immune system

 The Journal of
Immunology

Interaction between Phosphatidylserine and the Phosphatidylserine Receptor Inhibits Immune Responses In Vivo¹

Peter R. Hoffmann,^{2,*} Jennifer A. Kench,^{*} Andrea Vondracek,[†] Ellen Kruk,^{*} David L. Daleke,[‡] Michael Jordan,[†] Philippa Marrack,[†] Peter M. Henson,^{*} and Valerie A. Fadok^{*†}

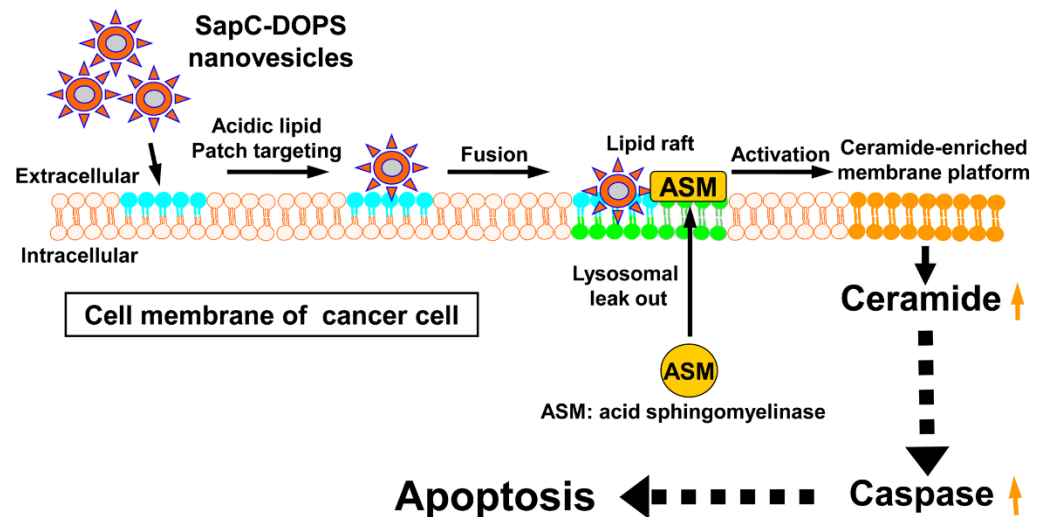
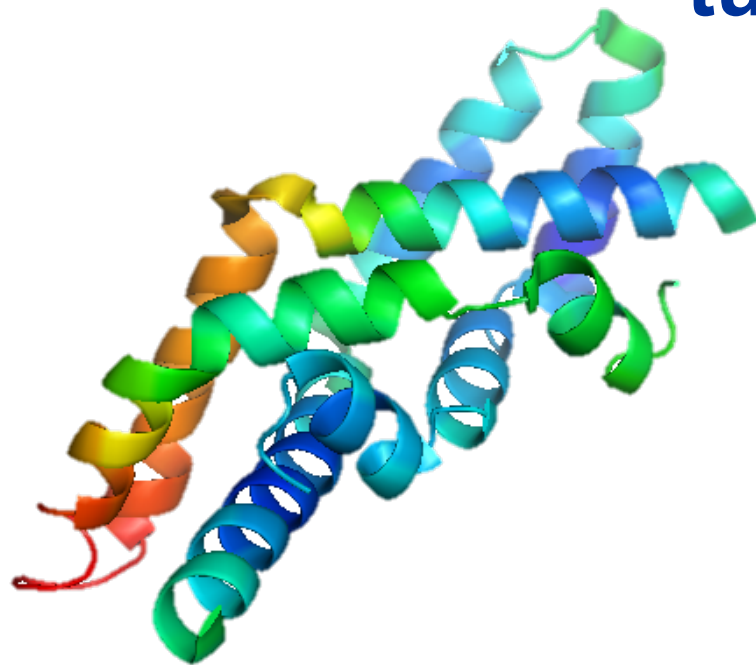


Bexion Pharmaceuticals

5

Developing Cures for Cancer

One of our body's own molecules, called Saposin C (SapC) not only binds PS; it can, under the right circumstances, also kill tumor cells



PLOS ONE

Targeting and Cytotoxicity of SapC-DOPS Nanovesicles in Pancreatic Cancer

Zhengtao Chu¹, Shadi Abu-Baker¹, Mary B. Palascak¹, Syed A. Ahmad², Robert S. Franco¹, Xiaoyang Qi^{1,3*}

Image download from Wikipedia, Katherine Lamb

SapC attaches to cancer cells and catalyzes reactions that cause tumor cell death



The Approach Was an Accidental Discovery

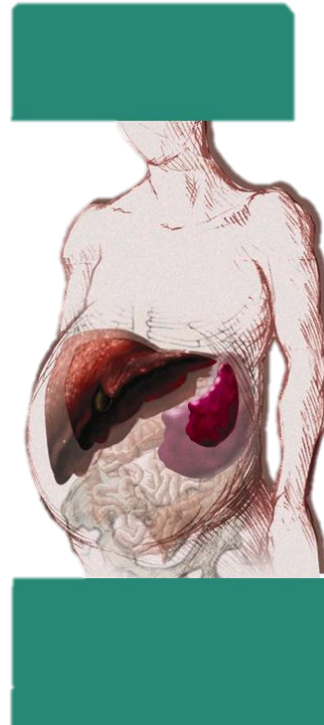
Gaucher's Disease ... and Cancer

Rare, genetic disorder

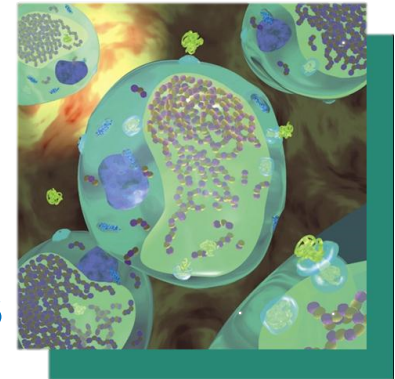
Debilitating, progressive and can be fatal

Classified as a “lysosomal storage disorder”

The lysosome is the “stomach of the cell”



One critical component of lysosomes, **Saposin C** goes awry in Gaucher's disease



Saposin C is essential in normal lysosomes AND can kill cancer cells...



The Bexion Story

National Recognition and Achievements

Series A investment		Bexion wins partnership with Nanotechnology Characterization Lab of NCI (NCL), and \$1.5MM grant for GBM		<i>NCI \$2.9MM Award for GBM</i> <i>(NCI Director Harold Varmis, MD)</i>		IND and Phase 1 clinical trial at four sites in the United States		
				<i>Developed clinically suitable formulation of SapC, called BXQ-350</i>				
2009	2010	2011	2012	2013	2014	2015	2016	2017
NCI grants for: <ul style="list-style-type: none"> • Prostate Cancer • Pancreatic Cancer • Glioblastoma Multiforme (GBM) 		Pre-IND Meeting with FDA		<i>Activity across multiple solid tumor types, including pancreatic cancer and GBM</i>		Tibbet's Award FDA granted Orphan Drug Status for GBM		

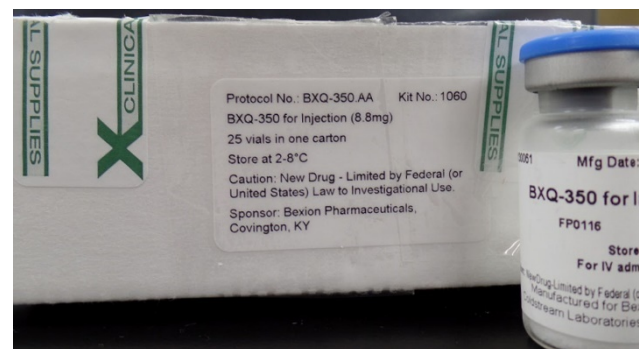


Bexion is Dosing Cancer Patients in a Phase I trial at National Cancer Institute Designated Centers

Phase I has two parts: A rising-dose safety program and two trials to dimension efficacy

To date - 10 patients with various solid tumors, including glioblastoma multiforme (GBM), pancreatic cancer and others have been dosed with BXQ-350

- Patients have exhausted standard-of-care options
- The first patient (lowest dose) has GBM and has been on BXQ-350 for 9 months (and counting)
- On track to start the second part of our Phase I in the Fall of this year



Summary - BXQ-350

- Novel drug that capitalizes on a unique vulnerability of cancer cells
- Completing Phase I safety trials with (so far) a very strong safety profile
- Potential opportunities against a range of solid tumors

Bexion Pharmaceuticals

- Awarded \$6MM in grants from the NCI and the Commonwealth of Kentucky
- Series A and other investments are \$22MM
- Pediatric Phase I trial in early 2018
- Phase II trials in late 2018
- Seeking Series B round of \$25MM

