

Reason for report:

COMPANY UPDATE

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LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

VERASTEM, INC.

Management Meeting Highlights Aggressive Development Plan

• **Bottom Line:** We recently met with VSTM management including newly appointed Chief Medical Officer Dr. Joanna Horobin. The meeting highlighted management's plan to accelerate the development of its inhibitors of focal adhesion kinase (FAK), which VSTM has identified as a key target in cancer stem cell survival. Proof of principle data were recently reported by GSK from its Phase I data showing improved efficacy of FAK inhibitor in Merlin negative mesothelioma. VSTM plans to initiate a registration study in mesothelioma in mid-2013. We remain Outperform rated on VSTM with a valuation of \$15.

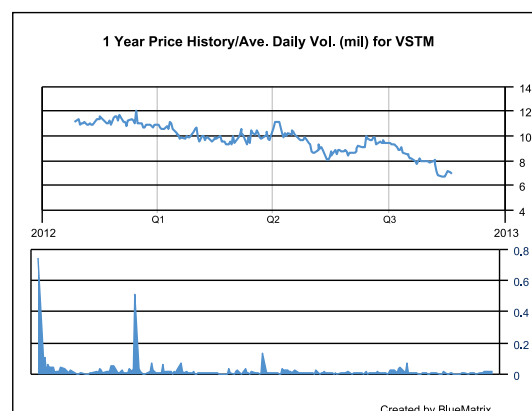
• **First clinical data suggest Merlin status may predict activity of FAK inhibitor.** At the 2012 EORTC Symposium on Molecular Targets and Cancer Therapeutics (Nov. 6-9), competitor GSK (MP) presented Phase I data for its FAK inhibitor GSK2256098. In the study, 29 patients with recurrent mesothelioma were treated with GSK2256098 ranging from 300-1500 BID. The maximum tolerant dose (MTD) was defined at 1000mg and the majority of patients were treated at the MTD. There were NO complete or partial responses (CR, PR) with 14 stable disease (SD) and 10 progressive disease (PD) reported in patients taking GSK2256098. The overall median PFS was 17.7 weeks, with 24.1 weeks in Merlin negative patients, 11.4 weeks in Merlin positive patients, and 10.4 weeks with unknown condition. Data presented at ASCO 2012 did not include Merlin biomarker data, and the EORTC data represent the first clinical data suggesting a link between Merlin-negative status (41% of the patients in the GSK trial) and improved efficacy. Lack of objective responses likely precludes a single-arm registration study, and VSTM is pursuing a randomized study that could potentially serve registrational purposes.

• **VSTM's FAK inhibitor VS-6063 appears to have a differentiated safety profile.** In the GSK2256098 Phase I study, the most common adverse events (over 25%) were nausea (83%), diarrhea (66%), decreased appetite (55%), vomiting (52%), asthenia (28%) and proteinuria (28%). Grade 3 drug-related SAEs include hypertriglyceridemia (n=2), hyperlipasemia, abdominal pain, lymphopenia and increased amylase (n=1 for each). In comparison, the most common Grade 1/2 AEs from the VS-6063 Phase I trial were nausea (37%), unconjugated hyperbilirubinemia (37%), fatigue (33%) and vomiting (28%) while diarrhea only occurred in 22% of patients. As discussed in our 7/13/12 [note](#), unconjugated hyperbilirubinemia may not be a real toxicity and is likely the result of biochemical competition of VS-6063 with bilirubin conjugation and disposition. Although small samples, initial data thus far suggest a better safety/tolerability profile for VS-6063.

Key Stats:

(NASDAQ:VSTM)

S&P 600 Health Care Index:	802.55
Price:	\$6.91
52 Week High:	\$12.24
52 Week Low:	\$6.53
Shares Outstanding (mil):	20.2
Market Capitalization (mil):	\$139.6
Book Value/Share:	\$2.86
Cash Per Share:	\$4.83
Dividend (ann):	NA
Valuation:	\$15



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2011A	--	--	--	0.0	0.0	--	--	(\$1.33)	(\$0.39)	(\$10.59)	NM
2012E - New	0.0A	0.0A	0.0A	0.0	0.0	(\$0.47)A	(\$0.34)A	(\$0.51)A	(\$0.54)	(\$1.87)	NM
2012E - Old	0.0A	0.0A	0.0A	0.0	0.0	(\$0.47)A	(\$0.35)	(\$0.37)	(\$0.39)	(\$1.54)	NM
2013E - New	--	--	--	--	0.0	--	--	--	--	(\$2.34)	NM
2013E - Old	--	--	--	--	0.0	--	--	--	--	(\$1.81)	NM

Source: Company Information and Leerink Swann LLC Research

EPS are GAAP. 3Q:11 loss is cumulative for 2011's first nine months. Quarterly figures may not sum to annual total due to change in shares out.



INVESTMENT THESIS

VSTM is a biotechnology company focused on novel cancer therapies targeting cancer stem cells. With few exceptions, current cancer treatments are generally unsatisfactory and new approaches to cancer therapy are needed. One issue encountered by most of the current agents has been the recurrence of cancer, despite intense chemotherapy and radiation therapy. Recent studies show a small subset of cancer cells, known as cancer stem cells (CSCs), are resistant to traditional chemotherapy but are capable of initiating tumors. While experimental and some controversies remain, targeting cancer stem cells (CSCs) represents a novel, potentially ground-breaking approach to cancer treatment, in our view. We believe our interest in CSCs is shared by major pharmaceutical companies including some that have designated CSC as a key area of focus for business development. This is evidenced by the recent announcement of the acquisition of CSC-focused biotechnology company Boston Biomedical Inc. by Daiichi Sankyo. VSTM recently licensed from PFE (MP) VS-6063, a Phase II ready FAK inhibitor targeting Merlin-negative mesothelioma. Clinical data on another FAK inhibitor GSK2256098 support VSTM's development plan in mesothelioma. In our view, VSTM has an exceptionally strong management team. The scientific founders of VSTM include some of the world's most renowned cancer biologists who developed technologies to recreate CSCs and screen compounds that target CSCs. Its business founders have proven track records in multiple prior biotech start-ups. With \$97M in cash as of 3Q12, we believe VSTM has sufficient cash resources to support operation and completion of the VS-6063 registration trial through the end of 2015.

A brief overview on mesothelioma. Mesothelioma is a rare form of cancer that develops from transformed mesothelium cells. Mesothelioma is usually caused by exposure to asbestos. There are 2,500-3,000 new cases in the US each year with ~12 month median survival. Approximately 40-50% of mesothelioma patients lack Merlin, a tumor suppressor encoded by the neurofibromatosis type 2 gene (NF2). Alimta (pemetrexed) from LLY (MP) is the only approved first-line therapy (on a 2.8 months OS benefit vs. cisplatin) for mesothelioma, and there are no approved drugs for the second-line therapy.

Potential registration trial to be started in mid 2013 could position VS-6063 as the most advanced FAK inhibitor. VSTM plans to initiate a randomized controlled study stratified for Merlin expression. The primary endpoint is PFS and the secondary endpoint is overall survival and biomarkers.

Preclinical data further support FAK as a target in cancer stem cells. At the 2012 EORTC Symposium, VSTM presented preclinical data of VS-4718 and VS-5095, both of which are FAK inhibitors, in breast cancer mouse models that are lacking the tumor suppressor Merlin. In an in vitro assay, VS-4718 and VS-5095 showed EC50 of 4 nM and 21 nM, respectively, against FAK activity. Further analysis showed that VS-4718 repressed a range of cancer stem cells. In a xenograft mouse model, VS-5095 showed comparable inhibition at 100mg/kg BID vs. paclitaxel at 15mg/mk QD IP. These data showed initial proof-of-concept for both VS-4718 and VS-5095 and further support the underlying science targeting FAK inhibition in Merlin-negative mesothelioma.

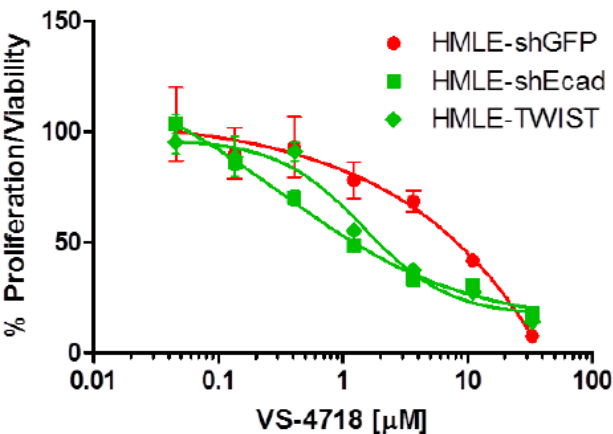


Recent publication highlights the technology strength for VSTM. Recent publication (J. Biomol Screen, 2012, 17:E1-E3) by VSTM cofounders highlighted cancer stem cells directed screening to identify potent compounds for potential cancer therapy. Using a high throughput screening on over 200K compounds, ~2K compounds showed initial promise. Three chemical series in the study are exclusively licensed to VSTM, forming a technology platform for a broad range of cancer indication.

Model update: We are updating our VSTM model to reflect its 3Q12 report. We are also adjusting our 2012 and 2013 outlook to incorporate higher R&D expenses for the upcoming clinical trials. Our 2012E EPS go from (\$1.54) to (\$1.87), and 2013E EPS go from (\$1.81) to (\$2.34).



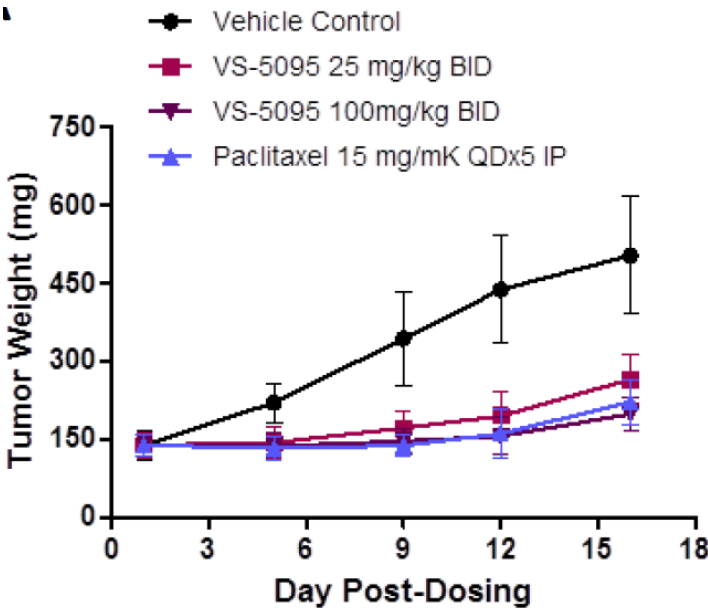
VS-4718 Inhibits Cancer Stem Cells



	HMLE-shGFP	HMLE-shECad	HMLE-TWIST
EC ₅₀ (μM)	~ 10	0.31	1.5

Source: Company Reports; Note: HMLE – human mammary epithelial cells

VS-5095 Antitumor Activity in Xenograft Mouse Model



Source: Company Reports



VALUATION

We rate Verastem Outperform with a 12-month valuation on VSTM shares of \$15. We reach this \$15 valuation by using a multiple-of-sales analysis based on a ~5x multiple applied to 2022E probability-weighted US sales of VS-6063 and VS-4718 totaling \$370M, and a ~12x multiple applied to 2022E royalties for ex-US probability-weighted sales of VS-6063 and VS-4718 of \$55M, each discounted back at 20%. We believe the multiples used are consistent with other biotechnology companies.

RISKS TO VALUATION

The field of cancer stem cells is a relatively new scientific arena. The overall concept of cancer stem cells, while continuing to build supportive scientific evidence, remains controversial within the academic community. There is also no clinical validation that targeting cancer stem cells will yield therapies that have improved outcomes relative to currently available agents. In addition, the clinical and registration paths for developing agents that target cancer stem cells remain to be paved, and appropriate clinical endpoints for this unique class of agents may need to be worked out.

Some competition has a longer presence in the field. While the technology behind Verastem's platform has been built on many years of scientific research, the company itself is very young, having been established in August 2010. Other companies with a focus on cancer stem cells have more advanced clinical candidates, and several have established collaborations with large biopharma companies.

Early stage of development, high risk for failure, and likely need for further financing.

Verastem's lead candidates are still in preclinical development, therefore have high risks of failure due to unproven safety and efficacy. As of the end of 3Q:12, the company had \$97M in cash and management previously guided that it would be sufficient for supporting operations into 2016, assuming Phase I programs on two compounds and a Phase II program on one compound. A broader, more aggressive development program could potentially shorten this cash runway and it seems likely that additional financing would be required prior to profitability.



VSTM Expected Events

Timing	Event
VS 6063 (FAK inhibitor)	
4Q:12 - 1Q:13	Manufacture clinical trial material
mid'13	Initiate registration trial in mesothelioma
Early 2014	Initiate Phase Ib combination studies
VS 4718 (FAK inhibitor)	
YE:12	Substantially complete pre-IND studies
1Q:13	Initiate Phase I trial in healthy volunteers
1H:13	Initiate Phase I in solid tumor patients
VS 5584 (PI3K/mTOR inhibitor)	
4Q:12	Start IND-enabling toxicology studies
Mid 2013	Initiate Phase I in solid tumor patients

Source: Company reports and Leerink Swann LLC

VSTM Product Pipeline

Program	Indication	Status
VS6063 (Focal adhesion kinase (FAK) inhibitor)	Mesothelioma and others	Phase I completed
VS4718 (Focal adhesion kinase (FAK) inhibitor)	Breast cancer and other solid tumors	Pre-clinical
VS-5584 (pan PI3 kinase and mTOR1/2 inhibitor)	Cancer	Pre-clinical
VS-507 (Salinomycin- Wnt inhibitor)	Cancer	Pre-clinical (deferred)
Companion diagnostic product	Oncology	Pre-clinical
NCE (New undisclosed target)	Cancer	Pre-clinical

Source: Company reports and Leerink Swann LLC

VSTM	2010A	2011A	Mar-12A	Jun-12A	Sep-12A	Dec-12E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
(Figures in \$000, except EPS)																	
VS-6063 US sales	0									0	0	0	50,000	100,000	150,000	200,000	250,000
20%)										0	0	0	0	4,858	7,287	21,521	27,534
VS-4718 sales										0	0	0	0	0	25,000	70,000	120,000
20%)										0	0	0	0	0	5,005	19,763	26,997
Total Product Sales	0	-								0	0	0	50,000	100,000	175,000	270,000	370,000
Total Royalties	0									0	0	0	0	4,858	12,293	41,283	54,531
Total Revenue	0	-	-	-						0	0	0	50,000	104,858	187,293	311,283	424,531
COGS	0	-					-	-	-	-	-	-	5,500	11,000	19,250	29,700	40,700
Research and Development	400	9,883	4,803	4,683	8,132	8,783	26,401	38,643	40,575	48,691	54,664	57,944	62,000	66,340	70,321	73,134	76,059
General and Administrative	384	3,815	2,125	2,213	2,298	2,367	9,003	9,941	10,538	11,064	11,618	34,853	69,706	91,033	97,405	106,172	111,480
Total Operating Expense	\$784	\$13,698	6,928	6,896	10,430	11,150	\$35,404	\$48,584	\$51,113	\$59,755	\$66,282	\$92,797	\$137,207	\$168,373	\$186,976	\$209,005	\$228,239
Interest Income/Expense Net	0	15	57	71	63	60	251	241	231	221	191	161	131	101	(3,642)	(8,845)	(15,372)
Collaborative revenues	(2)	(32)	(6)														
Net Income Before Taxes	(\$786)	(\$13,715)	(\$6,877)	(\$6,825)	(\$10,367)	(\$11,090)	(\$35,153)	(\$48,343)	(\$50,882)	(\$59,534)	(\$66,091)	(\$92,636)	(\$87,076)	(\$63,414)	(\$3,325)	\$93,433	\$180,919
Income Taxes	0	0					0	0	0	0	0	0	0	0	0	32,702	63,322
Tax rate	0%	0%					0%	0%	0%	0%	0%	0%	0%	0%	0%	35%	35%
Net Income After Taxes	(\$786)	(\$13,715)	(\$6,877)	(\$6,825)	(\$10,367)	(\$11,090)	(\$35,153)	(\$48,343)	(\$50,882)	(\$59,534)	(\$66,091)	(\$92,636)	(\$87,076)	(\$63,414)	(\$3,325)	\$60,732	\$117,598
GAAP EPS	(\$0.59)	(\$10.59)	(\$0.47)	(\$0.34)	(\$0.51)	(\$0.54)	(\$1.87)	(\$2.34)	(\$2.43)	(\$2.46)	(\$2.69)	(\$3.10)	(\$2.87)	(\$2.05)	(\$0.11)	\$1.72	\$3.27
Stock option expense	\$0	\$0					\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Basic Weighted Average Shares	1,325	1,295	14,693	19,863	20,160	20,362	18,769	20,667	20,977	24,174	24,537	29,905	30,354	30,961	31,580	32,211	32,856
Diluted Weighted Average Shares	1,325	1,295	17,047	22,099	23,229	23,431	21,451	23,736	24,046	27,243	27,606	32,974	33,423	34,030	34,649	35,280	35,925

Source: Company documents and Leerink Swann LLC estimates



Disclosures Appendix

Analyst Certification

I, Howard Liang, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

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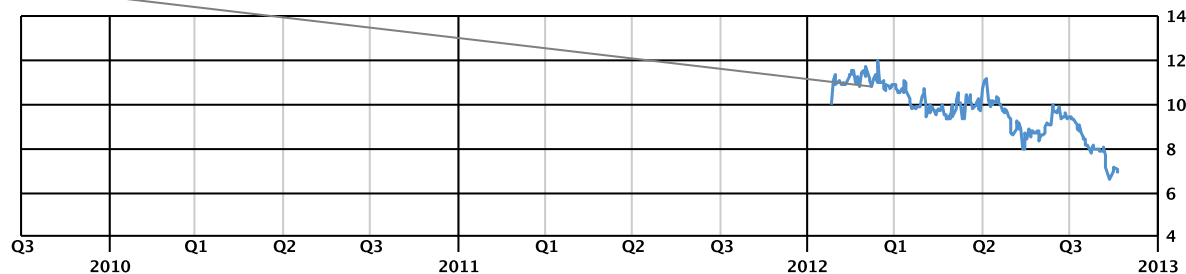
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Early stage of development, high risk for failure, and likely need for further financing. Verastem's lead candidates are still in preclinical development, therefore have high risks of failure due to unproven safety and efficacy. As of the end of 3Q:12, the company had \$97M in cash and management previously guided that it would be sufficient for supporting operations into 2016, assuming Phase I programs on two compounds and a Phase II program on one compound. A broader, more aggressive development program could potentially shorten this cash runway and it seems likely that additional financing would be required prior to profitability.



Rating and Price Target History for: Verastem, Inc. (VSTM) as of 11-20-2012

03/07/12
I:OP



OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

Rating and Price Target History for: GlaxoSmithKline plc (GSK LN) as of 11-20-2012

11/24/09
I:MP

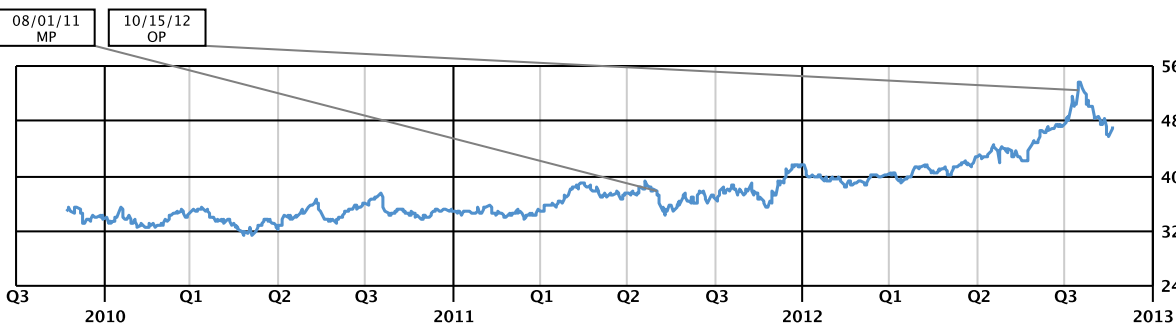


OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix



Rating and Price Target History for: Eli Lilly & Company (LLY) as of 11-20-2012



Leerink Swann placed an Underperform rating on LLY on April 7, 2009.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

Rating and Price Target History for: Pfizer Inc. (PFE) as of 11-20-2012



Leerink Swann placed an Outperform rating on PFE on Feb. 12, 2009.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix



Distribution of Ratings/Investment Banking Services (IB) as of 09/30/12				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	102	58.30	29	28.40
HOLD [MP]	73	41.70	3	4.10
SELL [UP]	0	0.00	0	0.00

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months. The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

From October 1, 2006 through January 8, 2009, the relevant benchmarks for the above definitions were the Russell 2000® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

Definitions of Leerink Swann Ratings prior to October 1, 2006 are shown below:

Outperform (Buy): We expect this stock to outperform its benchmark by more than 10 percentage points over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform within a range of plus or minus 10 percentage points of its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark by more than 10 percentage points over the next 12 months.

For the purposes of these definitions, the relevant benchmark were the Russell 2000® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Index for issuers with a market capitalization over \$2 billion.



Important Disclosures

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Leerink Swann Consulting LLC, an affiliate of Leerink Swann LLC, is a provider of evidence-based strategy and consulting to the healthcare industry.

In the past 12 months, the Firm has received compensation for providing investment banking services to Verastem, Inc.

Leerink Swann LLC makes a market in Verastem, Inc.

Leerink Swann LLC is willing to sell to, or buy from, clients the common stock of GlaxoSmithKline plc, Eli Lilly & Company and Pfizer Inc. on a principal basis.

In the past 12 months, an affiliate of the Firm, Leerink Swann Consulting LLC, has received compensation for providing non-securities services to: GlaxoSmithKline plc and Pfizer Inc.

Leerink Swann LLC has acted as a co-manager for a public offering of Verastem, Inc. in the past 12 months.

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