

Reason for report:

EARNINGS

KARYOPHARM THERAPEUTICS, INC.

4Q13 Recap - Selinexor Development Broadening; Increasing Price Target

• **Bottom Line:** KPTI reported 4Q13 results today and provided pipeline updates, including addl. details on the first two registration trials in hematological cancers, as well as 2014 plans for Phase II studies in gynecological cancers (1), squamous cell cancers (2) and hormone and/or chemotherapy refractory metastatic prostate cancer (3), glioblastoma (4), as well as Richter's syndrome (5). KPTI expects to end 2014 with \$100M cash. **Reiterate OP and increasing our PT to \$63 from \$26.**

• **Registration trials initiating in 2014 in AML and DLBCL; plans in MM evolving.** Trials in AML and DLBCL will enroll pts slightly earlier in disease progression than we had initially expected which translates into a longer Selinexor treatment duration vs. our model. The AML trial will randomize 150 pts in first relapse 2:1 to Selinexor or physicians' choice including HMAs. The trial is powered at 80% to show an OS of 5.4 vs. 3.0 months (HR=0.56). The second pivotal trial will randomize 300 DLBCL pts 2:1 to Selinexor vs. single agent physician's choice of chemotherapy after progression from 2+ lines of prior therapy. The trial is powered at 80% to show a PFS of 6.4 vs. 4.0 months respectively Selinexor and control arms. We see upside in Multiple Myeloma (MM), as KPTI's evolving plans for registration point to an earlier line of therapy in combination with an approved agent (e.g. Revlimid, Kyprolis).

• **Addl. Phase II studies planned in 2014 in prostate cancer and glioblastoma broaden Selinexor dev't.** Given the Phase II go-decision in prostate cancer and glioblastoma, we believe respective Phase I dose-expansion data to be presented at ASCO '14 should be positive. Two studies are planned in prostate cancer, a company sponsored trial will in post-chemotherapy CRPC and an investigator-sponsored trial in chemotherapy-naïve post-abiraterone pts. A Phase II trial will also be starting in relapsed glioblastoma as the mgmt noted that ~70% of Selinexor is able to cross the blood-brain barrier. A single arm registrational Phase II trial in 50 Richter's syndrome patients is also planned to commence with an ORR endpoint, based on promising responses seen in Phase I. In-line with our expectation a Phase II trial in 60 gynecological malignancy patients will initiate next month. A single-arm Phase II trial in advanced head and neck and lung cancer is also planned with an enrollment of 44 patients. We believe KPTI is on track to identify target markets and path to approval in solid tumor indications by 2015.

• **Our increased \$63 price target** is a result of the following changes to our model which was in our view conservative. (1) Increase of assumed Selinexor treatment duration in hematologic indications AML, DLBCL, and MM, (2) inclusion of Richter's Syndrome (20% probability-weighted) and (3) solid tumor indications (10% probability-weighted) in our Selinexor model.

Model	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	
2013A	--	--	0.0	0.0	0.4	--	--	(3.66)	(0.71)	(5.59)	NM
2014E - New	0.3	0.3	0.3	0.3	1.0	(0.46)	(0.50)	(0.53)	(0.56)	(2.05)	NM
2014E - Old	--	--	--	--	1.0	--	--	--	--	(1.33)	NM
2015E	--	--	--	--	1.0	--	--	--	--	(2.49)	NM

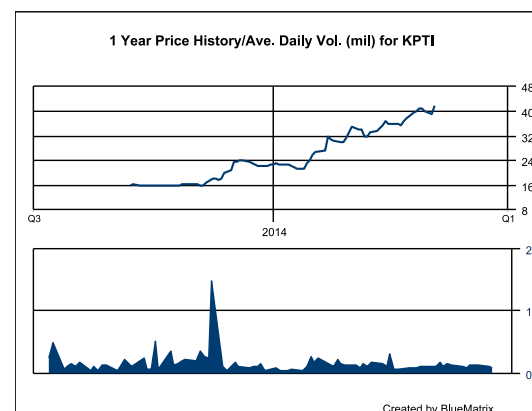
Source: Company Information and Leerink Partners LLC Research
Revenues in \$MM.
GAAP EPS.

Key Stats:

(NASDAQ:KPTI)

S&P 600 Health Care Index:	1,323.06
Price:	\$41.67
Price Target:	\$63.00 from \$26.00
Methodology:	DCF, 12% discount rate
52 Week High:	\$42.19
52 Week Low:	\$15.50
Shares Outstanding (mil):	29.7
Market Capitalization (mil):	\$1,237.6
Book Value/Share:	\$5.34
Cash Per Share:	\$5.29
Dividend (ann):	NA
Est LT EPS Growth:	NA

General: shares outstanding and cash/share account for IPO in 4Q13



INVESTMENT THESIS

Karyopharm Therapeutics (KPTI) is a biotech company focused on developing small molecule cancer drugs called "Selective Inhibitors of Nuclear Export" (SINE) which based on our checks with MEDACorp KOLs are an exciting new class of oral drugs. The company's clinical stage product Selinexor (KPT-330) is a orally bioavailable small molecule inhibitor of XPO1/CRM1 and was discovered by KPTI which has world-wide rights to the product. Selinexor is a first-in-class agent with a new mechanism of action: XPO1 mediates nuclear export of tumor suppressor proteins which then cannot promote cell death (apoptosis) in cancer cells anymore. Inhibition of XPO1 with KPT-330 restores tumor-suppressor activity in the nucleus which drives cancer cells into apoptosis. Selinexor has completed Phase I dose-escalation trials and based on our due diligence, we believe the drug is active in a broad range of cancers. We believe driven by positive data readouts KPTI shares will appreciate in value as the probability of success for Selinexor increases in currently tested indications or as activity in new indications becomes evident. We also believe KPTI could be a takeover target.

VALUATION

Our price target for KPTI is \$63/share. Our valuation is based on a discounted cash flow (DCF) analysis. We apply a 12% discount rate to 35% probability of success (POS) weighted Selinexor cash flows derived from three relapsed/refractory hematological cancer indications (AML, DLBCL, and MM), 20% POS-weighted sales in Richter's syndrome and 10% POS-weighted sales in solid tumor indications. Our valuation uses a terminal value derived by applying a 6x multiple to 2025E Selinexor revenue, discounted back by 11 periods. The 6x revenue multiple is in line with mid-cap biotech industry average. Based on our DCF analysis, we attribute \$60/share to Selinexor and the remainder to expected cash in one year.

RISKS TO VALUATION

Early stage biotech companies such as KPTI face significant clinical and regulatory development risk, as well as commercial risks. KPTI also faces execution risk and financial risk. We estimate that KPTI's current cash will be sufficient to fund into early 2016, and the company may have additional financing needs before turning cash flow positive. The vast majority of our KPTI valuation is based on Selinexor, the company's only clinical stage product candidate, so potential setbacks due to possible safety and/or efficacy related issues of Selinexor could have a significant impact to our valuation.

Event	Indication	Timing (old)
<u>Phase I program</u>		
Phase I dose expansion data	Heme Arm 1 (MM, WM, DLBCL)	mid-14
Phase I dose expansion data	Heme Arm 2 (AML)	mid-14
Phase I dose expansion data	Solid tumors	mid-14
Phase I dose expansion data	Heme Arm 3 (TCL)	2014
Phase I dose expansion data	Heme Arm 4, 5 (ALL, CML)	2014
Phase I data	Food effect study in soft tissue/bone sacromas	mid-14
<u>Hematological cancers</u>		
Initiation of pivotal Phase II/III (single agent)	elderly r/r AML	2Q14
Initiation of pivotal Phase II/III (single agent)	r/rDLBCL	3Q14
Initiation of pivotal Phase II	Richter's syndrome	1H14
Pivotal Phase II/III data	elderly r/r AML	2Q16
Pivotal Phase II/III data	DLBCL or MM	3Q16
Pivotal Phase II data	Richter's syndrome	1H16
<u>Solid tumors</u>		
Initiate Phase II	single agent solid tumor (gynecological)	1Q14
Initiate Phase II	single agent solid tumor (squamous cell cancers [lung, head and neck, esophageal])	1Q14
Initiate Phase II	single agent recurrent glioblastoma	1H14
Initiate Phase II	single agent CRPC	2014
Phase II data	single agent CRPC	2H15
Phase II data	single agent recurrent glioblastoma	2H15
Phase II data	single agent solid tumor (gynecological)	2H15
Phase II data	single agent solid tumor (squamous cell cancers [lung, head and neck, esophageal])	2H15

Source: Company filings and Leerink Partners estimates

KPTI P&L (in \$MM)	2011	2012	1H13	3Q13	4Q13A	2013A	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E
Contract and grant revenue	0.2	0.6	0.4	-	0.0	0.4	0.3	0.3	0.3	0.3	1.0	1.0
Selinexor US sales (p/w)	-	-	-	-	-	-	-	-	-	-	-	-
Selinexor EU royalty (p/w)	-	-	-	-	-	-	-	-	-	-	-	-
Total revenue	0.2	0.6	0.4	-	0.0	0.4	0.3	0.3	0.3	0.3	1.0	1.0
COGS	-	-	-	-	-	-	-	-	-	-	-	-
R&D expense	8.6	14.1	11.0	7.7	9.7	28.5	11.0	12.0	13.0	14.0	50.0	60.0
SG&A expense	1.8	2.4	1.8	1.6	2.5	5.9	3.0	3.0	3.0	3.0	12.0	15.0
Total operating expenses	10.5	16.5	12.8	9.3	12.2	34.3	14.0	15.0	16.0	17.0	62.0	75.0
Operating income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(12.1)	(34.0)	(13.8)	(14.8)	(15.8)	(16.8)	(61.0)	(74.0)
Total other income (expense)	-	0.0	0.0	-	0.0	0.0	-	-	-	-	-	-
Income Tax expense	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(12.1)	(33.9)	(13.8)	(14.8)	(15.8)	(16.8)	(61.0)	(74.0)
Common shares outstanding	1.1	1.8	2.3	2.5	17.2	6.1	29.7	29.7	29.7	29.7	29.7	29.7
EPS	(9.34)	(8.95)	(5.39)	(3.66)	(0.71)	(5.59)	(0.46)	(0.50)	(0.53)	(0.56)	(2.05)	(2.49)

KPTI BS & CFS (in \$MM)	2011	2012	1H13	3Q13	4Q13E	2013E	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E
Cash & equivalents	6.5	0.4	17.7	52.9	156.0	156.0	143.3	129.8	115.3	99.9	99.9	31.9
Debt	-	-	-	-	-	-	-	-	-	-	-	-

Change in Cash	3.1	(6.1)	17.3	35.3	102.0	154.5	(12.6)	(13.6)	(14.5)	(15.4)	(56.0)	(68.0)
Cash from operations	(8.5)	(15.5)	(11.3)	(8.9)	(11.7)	(31.9)	(12.6)	(13.6)	(14.5)	(15.4)	(56.0)	(68.0)
Net income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(12.1)	(33.9)	(13.8)	(14.8)	(15.8)	(16.8)	(61.0)	(74.0)
Share based comp	0.0	0.7	0.4	1.3	0.4	2.2	1.1	1.2	1.3	1.4	5.0	6.0
D&A	0.1	0.1	0.1	0.0	0.1	0.2	-	-	-	-	-	-
Other (Change in WC)	1.7	(0.4)	0.7	(0.9)	-	(0.3)	-	-	-	-	-	-
Cash from investing	(0.4)	(0.1)	-	(0.0)	-	(0.0)	-	-	-	-	-	-
CapEx	(0.4)	(0.1)	-	(0.0)	-	(0.0)	-	-	-	-	-	-
Acquisitions	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-
Cash from financing	12.0	9.5	28.6	44.2	113.7	186.5	-	-	-	-	-	-
Equity issue (buyback)	12.0	9.5	28.6	44.2	113.7	186.5	-	-	-	-	-	-
Debt issue (principal payment)	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-

Source: SEC Filings and Leerink Partners Estimates

KPTI Valuation, \$MM except per share

Year	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
P/W FCF	(61.0)	(74.0)	(86.0)	(81.2)	(51.5)	39.3	212.3	328.7	317.3	336.5	343.5	350.6
Periods	-	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	9.00	10.00	11.00
DR	12%	12%	12%	12%	12%	12%	12%	12%	12%	12%	12%	12%
PWFCF	(61.0)	(66.1)	(68.6)	(57.8)	(32.8)	22.3	107.5	148.7	128.1	121.4	110.6	100.8
NPV	1,783											
NPV/sh	60											
Cash/share	3											
Total	63											

POS AML	35%
POS DLBCL	35%
POS MM	35%
POS Richter's syndrome	20%
Solid tumors	10%
Discount Rate	12%
Terminal Year	2025
Terminal sales multiple	6

*POS = probability of success

Source: Leerink Partners estimates

Disclosures Appendix

Analyst Certification

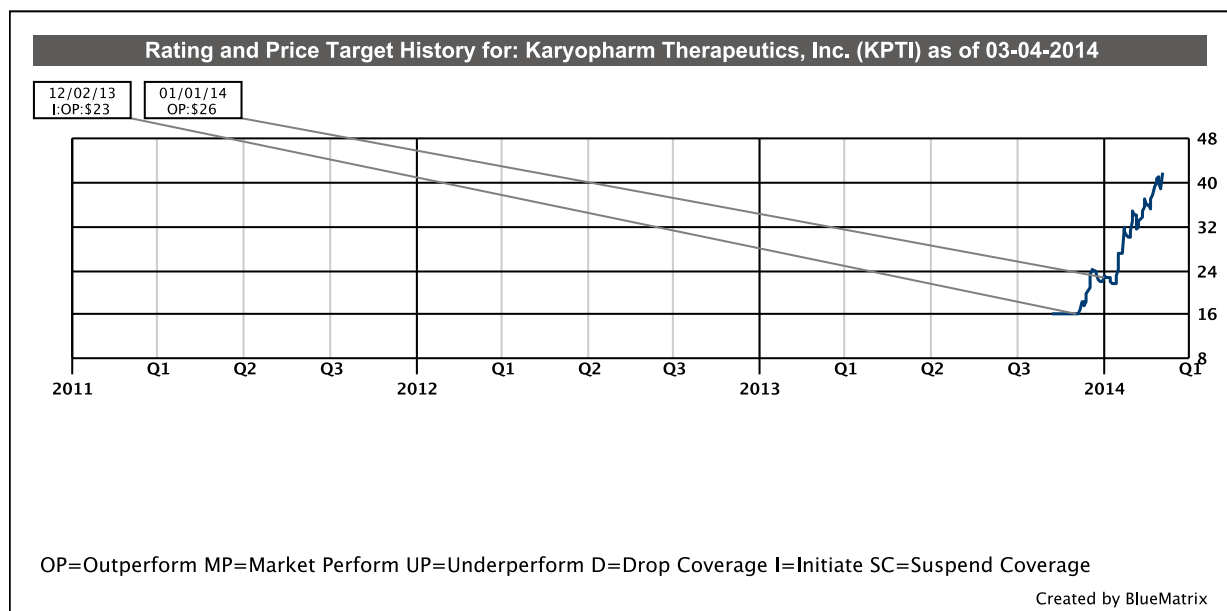
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

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Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	118	64.50	30	25.00
HOLD [MP]	65	35.50	2	3.00
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.

Important Disclosures

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

Leerink Partners LLC makes a market in Karyopharm Therapeutics, Inc.

Leerink Partners LLC has acted as the manager for a public offering of Karyopharm Therapeutics, Inc. in the past 12 months.

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