

## KARYOPHARM THERAPEUTICS, INC.

### 3Q13 Model Update

• **Bottom line:** We are updating our estimates to reflect 3Q13 financial results. KPTI's pipeline remains on track with Phase Ib Selinexor updates expected in 2014. KPTI reported EPS of (\$3.66) and ended the third quarter with \$52.9M in cash and equivalents excluding net proceeds of \$113.7M from the IPO in 4Q13. We are increasing our one-year price target to \$26/share (from \$23) based on our updated model. Reiterate Outperform rating.

• **KPTI reported EPS of \$(3.66) in 3Q13 and ended the quarter with \$52.9M in cash and equivalents.** KPTI reported no revenue in 3Q vs. contract and grant revenue of \$0.366M in 1H13 as work required under the grant was completed in 2Q13. KPTI received net proceeds of \$113.7M from the IPO in November and underwriters' option exercise in December. We now estimate KPTI will end 2013 with \$157M in cash which should be sufficient to finance Selinexor pivotal trials in two initial hematological indications and Phase II trials in solid tumors.

• **Updates from now five Phase Ib dose-expansion cohorts in multiple hematologic indications are expected throughout 2014.** KPTI recently added two additional dose-expansion trials in acute lymphocytic leukemia (ALL) and chronic myelogenous leukemia (CML) and continues enrolling existing Phase Ib trials in multiple myeloma (MM), diffuse large B-cell lymphoma (DLBCL), acute myeloid leukemia (AML), T-cell lymphoma (TCL), and solid tumors. Additionally, KPTI has now opened a first Phase II trial in recurrent glioblastoma. Mgmt continues to expect initiation of two initial pivotal heme trials in 2Q14, likely in elderly relapsed/refractory AML and DLBCL or MM, in our view. Following updated Phase I data recently presented at ASH, we continue to believe Selinexor has clear single agent activity across a broad range of indications and a manageable tolerability profile.

• **Increasing one-year price target to \$26 from \$23 previously.** We have updated our model to reflect the additional 1.02M shares purchased by the underwriters by December 10, which resulted in an additional \$15.2M in net proceeds to KPTI. We note that our current KPTI price target is conservative since it only accounts for probability-adjusted revenues from three initial last-line of therapy hematological indications and doesn't account for the Selinexor opportunity in solid tumor indications or additional heme indications where the drug has shown activity in Phase I.



LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

#### Key Stats:

(NASDAQ:KPTI)

S&P 600 Health Care Index: 1,287.72  
Price: \$22.92

Price Target: \$26.00 from \$23.00

Methodology: DCF, 12% discount rate, 6x terminal value

52 Week High: \$25.69

52 Week Low: \$15.50

Shares Outstanding (mil): 29.7

Market Capitalization (mil): \$680.7

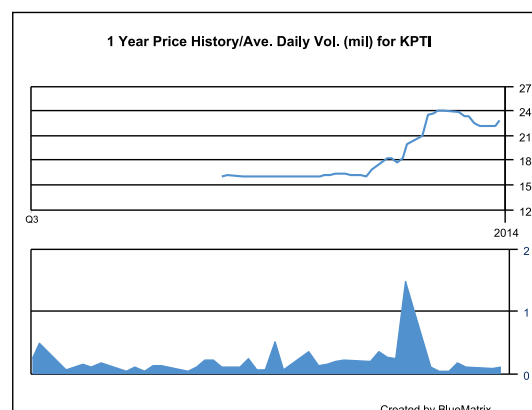
Book Value/Share: \$5.34

Cash Per Share: \$5.29

Dividend (ann): NA

Est LT EPS Growth: NA

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



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	
2012A	--	--	--	--	0.6	--	--	--	--	( 8.95)	NM
2013E - New	--	--	0.0A	0.2	0.6	--	--	( 3.66)A	( 0.33)	( 3.42)	NM
2013E - Old	--	--	--	--	0.8	--	--	--	--	( 3.22)	NM
2014E - New	--	--	--	--	1.0	--	--	--	--	( 1.33)	NM
2014E - Old	--	--	--	--	1.0	--	--	--	--	( 1.38)	NM

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



## 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. Karyopharm has treated 170 patients in three Phase I trials since May 2012, and the drug appears to have activity in patients with B-Cell cancers, such as Multiple Myeloma (MM), Non-Hodgkin’s Lymphoma (NHL), and Chronic Lymphocytic Leukemia (CLL), and also in Acute Myeloid Leukemia (AML), and in solid tumors including gynecological cancers and squamous cell cancers (lung, head and neck). Safety and tolerability appear to be manageable, with most side effects being mild-moderate gastrointestinal (GI) adverse events (AEs) and fatigue.

We believe several near-term data readouts could potentially validate Selinexor activity seen in Phase I dose-escalation. KPTI is currently conducting five Phase Ib fixed dose expansion trials in MM (10 pts), Diffused Large B-Cell Lymphoma (DLBCL) (15 pts), AML (25 pts), T-Cell Lymphoma (TCL) (12 pts), and in solid tumor indications (30 pts) which should have data available in mid-2014 and form the company’s path to registration. Karyopharm expects to initiate two pivotal trials in 2Q14 for the two initial hematological indications, likely one trial in relapsed/refractory AML and one trial in DLBCL or MM, in our view. KPTI plans to filed for accelerated approval in 2H16. Data from two Phase II trials in solid tumor indications in 2015 will form the path to market there, in our view.

KPTI management with track record of success implements a lean business model. Karyopharm is led by Michael Kauffman who was previously CMO at ONYX (previously Proteolix) where he led the development of Kyprolis (carfilzomib) which obtained accelerated approval in MM. KPTI was founded in 2008 and KPT-330 took only 17 months from lead identification into the clinic.

## VALUATION

We estimate a \$26 fair value for KPTI shares in 12 months, based on a discounted cash flow (DCF) analysis. We apply a 12% discount rate to 35% probability of success-weighted Selinexor cash flows derived from three relapsed/refractory hematological cancer indications (AML, DLBCL, and MM). Potential future Selinexor revenues derived from solid tumor indications as well as the preclinical and pet pipeline are upside to our valuation. 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 \$22/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 2017, 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.

<b>KPTI P&amp;L (in \$MM)</b>	<b>2011</b>	<b>2012</b>	<b>1H13</b>	<b>3Q13</b>	<b>4Q13E</b>	<b>2013E</b>	<b>2014E</b>	<b>2015E</b>
Contract and grant revenue	0.2	0.6	0.4	-	0.2	0.6	1.0	1.0
Selinexor US sales (p/w)	-	-	-	-	-	-	-	-
Selinexor EU royalty (p/w)	-	-	-	-	-	-	-	-
<b>Total revenue</b>	<b>0.2</b>	<b>0.6</b>	<b>0.4</b>	<b>-</b>	<b>0.2</b>	<b>0.6</b>	<b>1.0</b>	<b>1.0</b>
COGS	-	-	-	-	-	-	-	-
R&D expense	8.6	14.1	11.0	7.7	8.0	26.8	35.3	40.3
SG&A expense	1.8	2.4	1.8	1.6	2.0	5.4	5.3	5.8
Total operating expenses	10.5	16.5	12.8	9.3	10.0	32.2	40.6	46.1
Operating income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(9.8)	(31.6)	(39.6)	(45.1)
Total other income (expense)	-	0.0	0.0	-	-	0.0	-	-
Income Tax expense	-	-	-	-	-	-	-	-
<b>Net income (loss)</b>	<b>(10.3)</b>	<b>(15.9)</b>	<b>(12.5)</b>	<b>(9.3)</b>	<b>(9.8)</b>	<b>(31.6)</b>	<b>(39.6)</b>	<b>(45.1)</b>
Common shares outstanding	1.1	1.8	2.3	2.5	29.7	9.2	29.7	29.7
Common shares outstanding (Pro Forma)		5.8	9.8	21.7	29.7	17.8		
EPS	(9.34)	(8.95)	(5.39)	(3.66)	(0.33)	(3.42)	(1.33)	(1.52)
EPS (Pro Forma)		(2.74)	(1.27)	(0.43)	(0.33)	(1.78)		

<b>KPTI BS &amp; CFS (in \$MM)</b>	<b>2011</b>	<b>2012</b>	<b>1H13</b>	<b>3Q13</b>	<b>4Q13E</b>	<b>2013E</b>	<b>2014E</b>	<b>2015E</b>
Cash & equivalents	6.5	0.4	17.7	52.9	157.3	157.3	121.0	79.6
Debt	-	-	-	-	-	-	-	-

Change in Cash	3.1	(6.1)	17.3	35.3	104.4	156.9	(36.3)	(41.4)
<b>Cash from operations</b>	<b>(8.5)</b>	<b>(15.5)</b>	<b>(11.3)</b>	<b>(8.9)</b>	<b>(9.4)</b>	<b>(29.6)</b>	<b>(36.3)</b>	<b>(41.4)</b>
Net income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(9.8)	(31.6)	(39.6)	(45.1)
Share based comp	0.0	0.7	0.4	1.3	0.4	2.2	3.2	3.7
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)	-	-
<b>Cash from investing</b>	<b>(0.4)</b>	<b>(0.1)</b>	<b>-</b>	<b>(0.0)</b>	<b>-</b>	<b>(0.0)</b>	<b>-</b>	<b>-</b>
CapEx	(0.4)	(0.1)	-	(0.0)	-	(0.0)	-	-
Acquisitions	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-
<b>Cash from financing</b>	<b>12.0</b>	<b>9.5</b>	<b>28.6</b>	<b>44.2</b>	<b>113.7</b>	<b>186.5</b>	<b>-</b>	<b>-</b>
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 Swann Estimates

Event	Indication	Timing
<b><u>Phase I program</u></b>		
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 sarcomas	2014
<b><u>Hematological cancers</u></b>		
Initiation of pivotal Phase II/III (single agent)	elderly r/r AML	2Q14
Initiation of pivotal Phase II/III (single agent)	DLBCL or MM	2Q14
Pivotal Phase II/III data	elderly r/r AML	1H16
Pivotal Phase II/III data	DLBCL or MM	1H16
Launch	r/r AML	1H17
Launch	DLBCL or MM	1H17
<b><u>Solid tumors</u></b>		
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
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
<b><u>Investigator-Initiated Studies</u></b>		
Initiation Phase I/II	combination	2014

Source: Company filings and Leerink Swann estimates



## Disclosures Appendix

### Analyst Certification

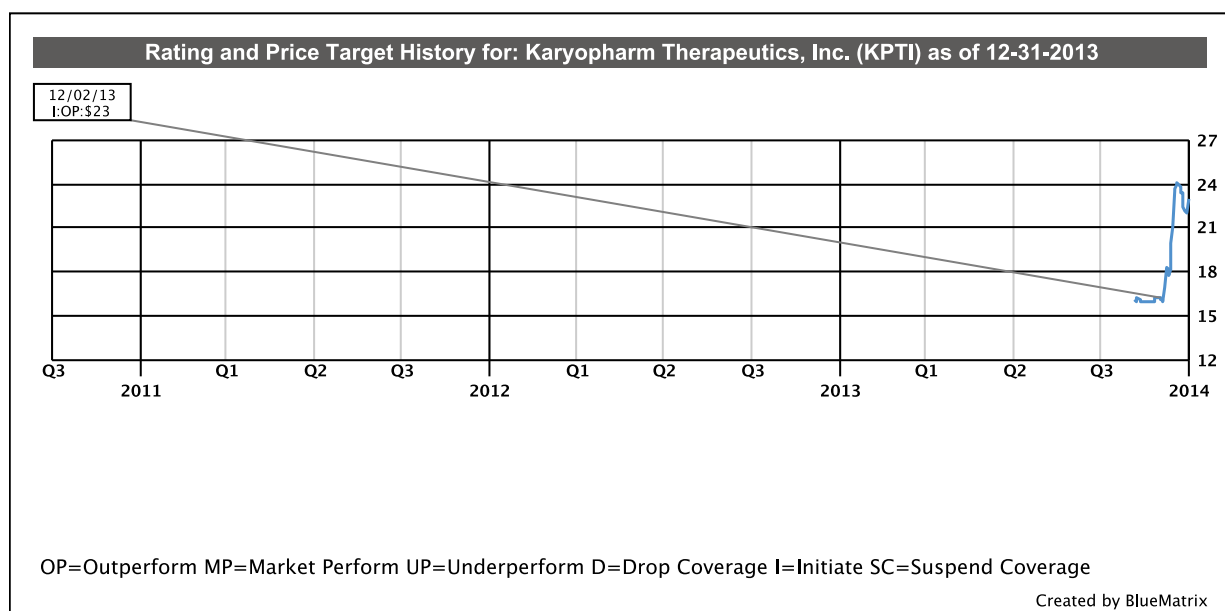
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

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Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	111	64.90	27	24.00
HOLD [MP]	60	35.10	0	0.00
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 Swann LLC makes a market in Karyopharm Therapeutics, Inc.

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

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