

# Karyopharm Therapeutics Inc. (KPTI)

Increased Confidence in Selinexor Activity in DLBCL and Richter's at ASCO

## MARKET DATA

Price	\$26.25
52-Week Range:	\$15.50 - \$47.87
Shares Out. (M):	29.8
Market Cap (\$M):	\$782.3
Average Daily Vol. (000):	374.0
Cash (M):	\$156
Cash/Share:	\$5.24
Enterprise Value (M):	\$785
Float (M):	14.6
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$26.25 | Target Price: \$50.00

## INVESTMENT HIGHLIGHTS

**Several positive takeaways from updated Phase I selinexor data in NHL; we reiterate our Market Outperform rating on Karyopharm Therapeutics and our \$50 price target based on our DCF and SOTP valuation methodologies.** The updated Phase I data were presented during the Lymphoma Target Therapies Session today at ASCO. Most encouraging was the incremental increase in objective response rates seen in DLBCL patients, increasing from 21% per abstract (3/14 pts) to 29% (6/21 pts), including one complete response in 'double hit' patient. Of note, selinexor activity is non-subtype specific, given that responses in GCB-subtype disease resemble those of non-GCB disease (27% and 25%, respectively). In addition, duration of therapy among responders was  $\geq 4$  months, altogether, we have greater confidence in the broad clinical success in DLBCL. Similarly in Richter's, we view selinexor's ability to maintain a minimum of stable disease among all treated patients as a positive indicator for the drug's performance in an upcoming registration-directed study.

**Additional highlights from Phase I update safety profile in NHL.** In addition, the incremental improvement in response outcomes in DLBCL, we believe investors should draw greater comfort from selinexor's evolving safety profile. We note that higher adverse event rates observed within the first cycle of therapy, particularly nausea, fatigue, and anorexia, generally decreased in subsequent cycles. Furthermore, in the opinion of the session's discussant, Dr. Owen O'Conner, reported rates of Grade 3/4 hematologic adverse events were somewhat overstated by coincident thrombocytopenia and neutropenia in select patients.

FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	--	\$0.2A	\$0.0
	2Q	\$0.4	\$0.0	\$0.0
	3Q	\$0.0	\$0.0	\$0.0
	4Q	\$0.0	\$0.0	\$0.0
	FY	\$0.0	\$0.0	\$0.0
EPS	1Q	--	(\$0.46)A	--
	2Q	(\$5.39)	(\$0.44)	--
	3Q	(\$3.66)	(\$0.46)	--
	4Q	(\$0.47)	(\$0.53)	--
	FY	(\$5.59)	(\$1.89)	(\$5.18)

Source: Company reports and JMP Securities LLC

## STOCK PRICE PERFORMANCE



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**FIGURE 1. Best Responses with Selinexor Across NHL (May 13 cut-off)**

Best Responses in NHL/Richter's Syndrome Patients as of 13-May-2014								
Cancer	N*	DCR (%)	ORR(%)	CR (%)	PR (%)	SD (%)	PD (%)	WC (%)
DLBCL	21	15 (70%)	6 (29%)	1 (5%)	5 (25%)	9 (40%)	5 (25%)	1 (5%)
Follicular	7	6 (86%)	1 (14%)	--	1 (14%)	5 (71%)	--	1 (14%)
Mantle Cell	3	2 (67%)	1 (33%)	--	1 (33%)	1 (33%)	--	1 (33%)
Transformed	3	1 (33%)	1 (33%)	--	1 (33%)	--	2 (67%)	--
T-Cell	4	3 (75%)	1 (25%)	1 (25%)	--	2 (50%)	--	1 (25%)
Richter's Syndrome	5	5 (100%)	2 (40%)	--	2 (40%)	3 (60%)	--	--
<b>Total</b>	<b>43</b>	<b>32 (74%)</b>	<b>12 (28%)</b>	<b>2 (5%)</b>	<b>10 (23%)</b>	<b>20 (47%)</b>	<b>7 (16%)</b>	<b>4 (9%)</b>

DCR=Disease Control Rate (CR+PR+SD), ORR=Overall Response Rate, CR=Complete Response, PR=Partial Response, SD=Stable Disease, PD=Progressive Disease, WC=Withdrew Consent

Source: Gutierrez M, ASCO 2014

**FIGURE 2. DLBCL Response by GCB Subtype**

Responses in Diffuse Large B-Cell Patients as of 13-May-2014								
Type	N	DCR (%)	ORR (%)	CR (%)	PR (%)	SD (%)	PD (%)	WC (%)
GCB	11	8 (72%)	3 (27%)	1 (9%)	2 (18%)	5 (45%)	2 (18%)	1 (9%)
non-GCB	4	3 (75%)	1 (25%)	--	1 (25%)	2 (50%)	1 (25%)	--

Source: Gutierrez M, ASCO 2014

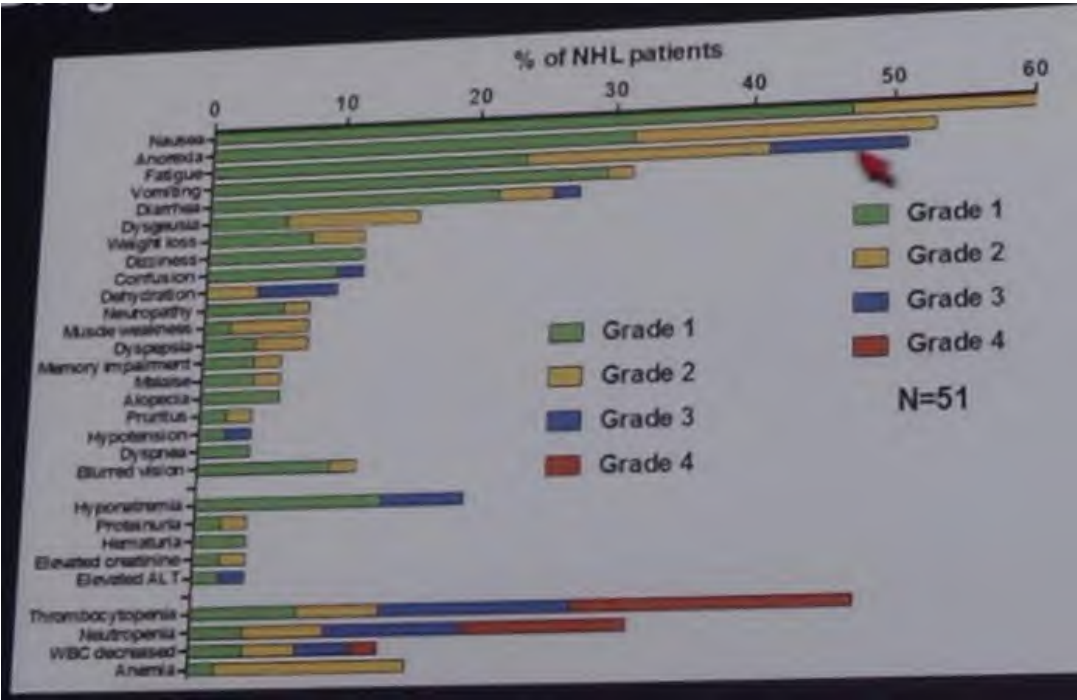
FIGURE 3. Duration of Response and Stable Disease in DLBCL

Patient	Disease	Best Response	Maximal Reduction	Days on Study	Off-Study
003	DLBCL	PR	-83%	632+	
046	DLBCL	CR	PET negative	239+	
050	DLBCL	PR	-91%	219+	
070	DLBCL	PR	-54%	119+	
067	DLBCL	PR	-63%	78	WC
083	DLBCL	PR	-52%	44	PD

Patient	Disease	Best Response	Maximal Reduction	Days on Study	Off-Study
028	DLBCL	SD	-12%	366+	
072	DLBCL	SD	-43%	114+	WC
001	DLBCL	SD	0%	86	PD
054	DLBCL	SD	-4%	84	PD
063	DLBCL	SD	-22%	80	PD
053	DLBCL	SD	+3%	56	PD
044	DLBCL	SD	+30%	55	PD
087	DLBCL	SD	PET -37%	49+	
086	DLBCL	SD	PET -40%	49+	
065	DLBCL	SD	+3%	51	PD
088	DLBCL	SD	PET -0%	49	PD

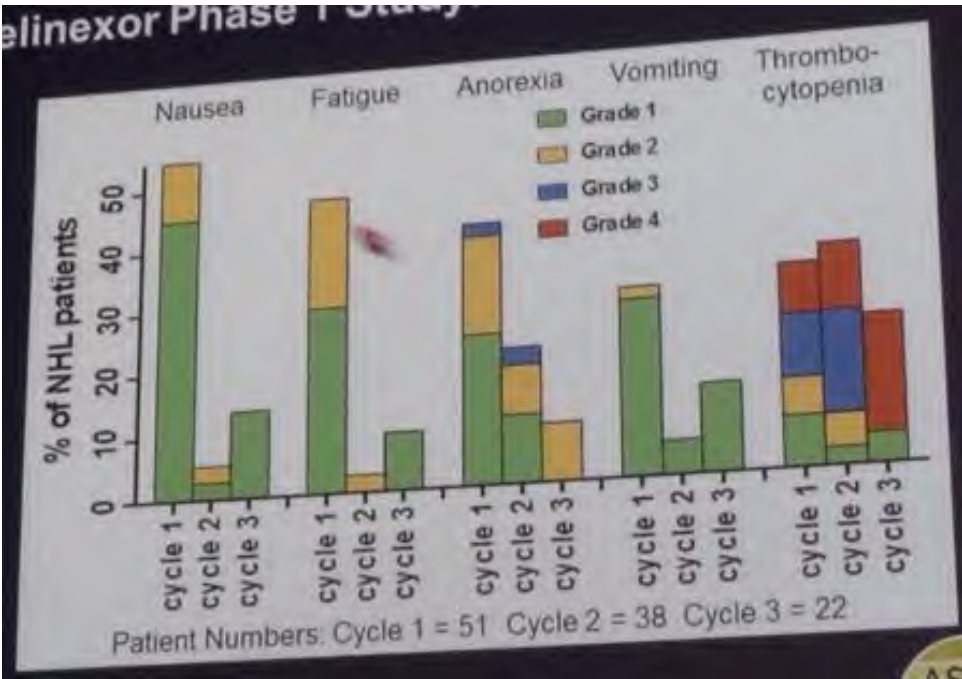
Source: Gutierrez M, ASCO 2014

FIGURE 4. Selinexor Adverse Events in NHL



Source: Gutierrez M, ASCO 2014

FIGURE 5. Common AEs by Selinexor Cycle of Therapy



Source: Gutierrez M, ASCO 2014

## Company Description

Karyopharm Therapeutics (KPTI) is a Natick, MA-based, clinical-stage biopharmaceutical company focused on the discovery and development of novel first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases. Karyopharm's Selective Inhibitors of Nuclear Export (SINE) compounds function by preventing the export of tumor suppressor proteins from the nucleus, driving accumulation and restoration of function. The company's lead pipeline candidate, selinexor (KPT-330), is a Phase I orally available small molecule inhibitor of XPO1, set to initiate pivotal Phase II/III evaluation in various hematologic malignancies in 2014. Karyopharm is also developing selinexor and SINE as potential therapies for autoimmune and inflammatory disease, viral infections, and wound healing.

## Investment Risks

**Clinical.** Drug development is an inherently risky business. Clinical trials always carry a risk of failure and Karyopharm's assets (Selinexor (KPT330), KPT-350, PAK4 inhibitor, verdinexor or future drug candidates) may fail to demonstrate meaningful enough levels of efficacy in current or future clinical trials.

**Regulatory and commercial.** The ability of Karyopharm to market its drugs depends upon the drugs obtaining approval from the FDA and foreign regulatory agencies. Failure to achieve approval or delays in the timelines to approval could negatively impact the company's share price.

**Competitive.** Hematologic malignancies including multiple myeloma, indolent non-Hodgkin lymphoma and acute myeloid leukemia represent increasingly competitive fields and Karyopharm faces competition from both commercial and development-stage companies with product(s) or product candidates addressing similar clinical indications. Some of these companies may possess substantially greater R&D and commercial resources than Karyopharm. As such, there is no assurance Karyopharm will be competitive or differentiated from other drug products.

**Financial.** Following its IPO, Karyopharm ended 1Q14 with approximately \$156MM in cash and cash equivalents. We anticipate the company is likely to seek additional equity financing in the form of a secondary offering in order to complete the development of its drug candidates, creating dilution risk for existing shareholders.

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JMP Securities was manager or co-manager of a public offering of securities for Karyopharm Therapeutics Inc. (KPTI) in the past 12 months, and received compensation for doing so.

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

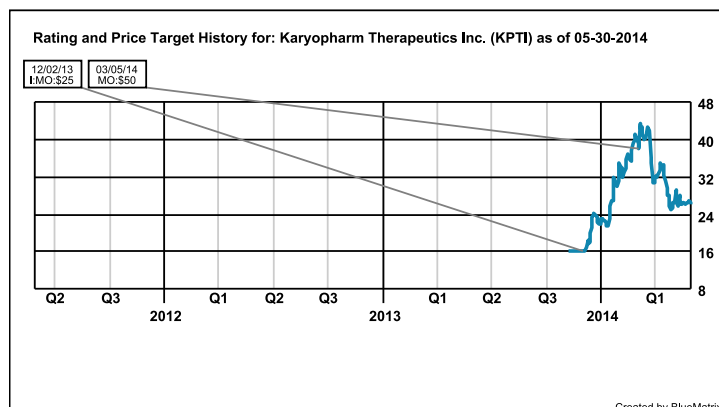
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

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JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	258	58.64%	Buy	258	58.64%	99	38.37%
MARKET PERFORM	Hold	134	30.45%	Hold	134	30.45%	16	11.94%
MARKET UNDERPERFORM	Sell	5	1.14%	Sell	5	1.14%	0	0%
COVERAGE IN TRANSITION		43	9.77%		43	9.77%	0	0%
TOTAL:		440	100%		440	100%	115	26.14%

### Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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