

Reason for report:

**EARNINGS**

## KARYOPHARM THERAPEUTICS, INC.

### 1Q14 Recap: In-Line Quarter; Broad Selinexor Program On Track

• **Bottom Line:** KPTI reported in-line 1Q14 results yesterday and maintained previous cash guidance of ending 2014 with ~\$100M, which should be sufficient to fund the company into early 2016. We are updating our estimates to reflect 1Q14 results. Expansion of Selinexor trials across a wide range of indications via both company-sponsored trials and investigator-sponsored trials (ISTs) is underway. We believe this strategy is an efficient way to explore Selinexor's therapeutic potential in a wide range combinations and indications. Next up are five presentations at ASCO, including one oral presentation on the Phase I study in non-Hodgkin lymphoma. Reiterate OP. Our price target remains \$63/share.

• **Selinexor development on track with multiple trials launching this year, in addition to now nine ongoing clinical trials.** Karyopharm continues to expand Selinexor across a wide range of indications via both company-sponsored trials and investigator-sponsored trials. We believe this strategy is an efficient way to explore Selinexor's potential across various combinations and indications. KPTI has recently announced initiation of ISTs in acute myeloid leukemia (AML) and pediatric leukemia patients (please see enclosed table). KPTI has also initiated a Phase II study (SIGN) in advanced gynecologic malignancies and a Phase II study (KING) in recurrent glioblastoma. KPTI continues to expect initiation of a registration-directed study in AML in 2Q14, a registration-directed study in diffuse large B-cell lymphoma (DLBCL) in 2H14, a registration-directed study in Richter's syndrome in 2Q14, a Phase II study in squamous cell cancers of the head and neck, lung, or esophagus in 2Q14, and a Phase II study in metastatic castration resistant prostate cancer in 2H14. KPTI anticipates an additional ~20 ISTs that could initiate in 2014.

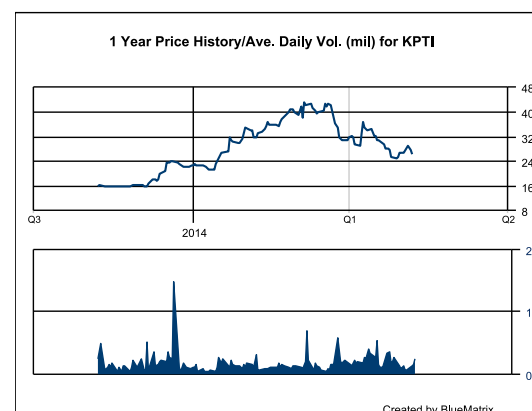
• **KPTI has one oral presentation and 4 posters at ASCO.** The oral presentation will pertain to the Phase I trial of Selinexor in non-Hodgkin lymphoma (NHL). Four Phase I Selinexor posters will provide updates in advanced solid tumors, relapsed/refractory AML, early clinical (and preclinical) data on platinum-resistant/refractory ovarian cancer, and a food effect study in sarcoma.

• **KPTI maintains cash guidance.** KPTI ended 1Q14 with \$144.9M in cash and equivalents, and continues to expect to end 2014 with ~\$100M in cash and equivalents. Management expects to have sufficient cash to fund the company into early 2016. For 1Q14, KPTI reported revenues of \$0.2M (vs. our estimate of \$0.3M), a net loss of \$13.7M (vs. our net loss estimate of \$13.8M), and a loss per share of (\$0.46) vs. our loss per share estimate of (\$0.46).

#### Key Stats:

(NASDAQ:KPTI)

<b>S&amp;P 600 Health Care Index:</b>	<b>1,214.38</b>
<b>Price:</b>	<b>\$26.36</b>
Price Target:	\$63.00
Methodology:	DCF, 12% discount rate
52 Week High:	\$47.87
52 Week Low:	\$15.50
Shares Outstanding (mil):	29.7
Market Capitalization (mil):	\$782.9
Book Value/Share:	\$5.34
Cash Per Share:	\$4.88
Dividend (ann):	\$0.00
Dividend Yield:	0.0%
Est LT EPS Growth:	NA



Dec Yr	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	\$0.2A	\$0.2	\$0.2	\$0.2	\$0.7	(\$0.46)A	(\$0.50)	(\$0.53)	(\$0.57)	(\$2.06)	NM
2014E - Old	\$0.3	\$0.3	\$0.3	\$0.3	\$1.0	(\$0.46)A	(\$0.50)	(\$0.53)	(\$0.56)	(\$2.05)	NM
2015E	--	--	--	--	\$1.0	--	--	--	--	(\$2.49)	NM

Source: Company Information and Leerink Partners 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 worldwide 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 that, 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 the 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 on our valuation.

Upcoming Events		
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 sacromas	mid-14
<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)	r/rDLBCL	2H14
Initiation of pivotal Phase II	Richter's syndrome	2Q14
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
<b><u>Solid tumors</u></b>		
Initiate Phase II	single agent solid tumor (squamous cell cancers [lung, head and neck, esophageal])	2Q14
Initiate Phase II	single agent CRPC	2H14
Phase II data	single agent CRPC	2H15
Phase II data	single agent recurrent glioblastoma -KING	2H15
Phase II data	single agent solid tumor (gynecological) - SIGN	2H15
Phase II data	single agent solid tumor (squamous cell cancers [lung, head and neck, esophageal])	2H15

Source: Company filings and Leerink Partners estimates

Selinexor Clinical Trials Overview				
Phase	Indication	Sponsor	ID	Status
II/III	Relapsed AML	KPTI	NCT02088541	Ongoing
II/III	DLBCL	KPTI	KCP-330-009	Planned
II/III	Richter's Syndrome	KPTI	KCP-330-010	Planned
II	Ovarian, endometrial, cervical carcinoma (SIGN)	KPTI	NCT02025985	Ongoing
II	Recurrent glioblastoma after radiation/TMZ (KING)	KPTI	NCT01986348	Ongoing
II	SCC of head and neck, lung, or esophagus	KPTI	KCP-330-006	Planned
II	mCRPC	KPTI	KCP-330-007	Planned
I	Effects of food and formulation in sarcoma	KPTI	NCT01896505	Ongoing
I	Solid tumors	KPTI	NCT01607905	Ongoing
I	Hematological malignancies	KPTI	NCT01607892	Ongoing
I	Melanoma	IST	NCT02120222	Ongoing
I	Relapsed childhood ALL and AML	IST	NCT02091245	Ongoing
I	Solid tumors (Asian patients)	IST	NCT02078349	Ongoing
I	Relapsed/refractory AML	IST	NCT02093403	Ongoing
I	Salivary gland cancers	IST	NCT02069730	Planned

Source: [clinicaltrials.gov](http://clinicaltrials.gov), KPTI

KPTI P&L (in \$MM)	2011	2012	1H13	3Q13	4Q13	2013	1Q14	2Q14E	3Q14E	4Q14E	2014E	2015E
Contract and grant revenue	0.2	0.6	0.4	-	0.0	0.4	0.2	0.2	0.2	0.2	0.7	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>0.0</b>	<b>0.4</b>	<b>0.2</b>	<b>0.2</b>	<b>0.2</b>	<b>0.2</b>	<b>0.7</b>	<b>1.0</b>
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	2.9	3.0	3.0	3.0	11.9	15.0
Total operating expenses	10.5	16.5	12.8	9.3	12.2	34.3	13.9	15.0	16.0	17.0	61.9	75.0
Operating income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(12.1)	(34.0)	(13.7)	(14.8)	(15.8)	(16.8)	(61.2)	(74.0)
Total other income (expense)	-	0.0	0.0	-	0.0	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>(12.1)</b>	<b>(33.9)</b>	<b>(13.7)</b>	<b>(14.8)</b>	<b>(15.8)</b>	<b>(16.8)</b>	<b>(61.2)</b>	<b>(74.0)</b>
Common shares outstanding	1.1	1.8	2.3	2.5	17.2	6.1	29.6	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)	(0.57)	(2.06)	(2.49)

KPTI BS & CFS (in \$MM)	2011	2012	1H13	3Q13	4Q13	2013	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E
Cash & equivalents	6.5	0.4	17.7	52.9	156.0	156.0	144.9	131.3	116.7	101.2	101.2	33.2
Debt	-	-	-	-	-	-	-	-	-	-	-	-

Change in Cash	3.1	(6.1)	17.3	35.3	103.0	155.6	(12.6)	(13.6)	(14.5)	(15.5)	(56.2)	(68.0)
<b>Cash from operations</b>	<b>(8.5)</b>	<b>(15.5)</b>	<b>(11.3)</b>	<b>(8.9)</b>	<b>(10.1)</b>	<b>(30.3)</b>	<b>(12.6)</b>	<b>(13.6)</b>	<b>(14.5)</b>	<b>(15.5)</b>	<b>(56.2)</b>	<b>(68.0)</b>
Net income (loss)	(10.3)	(15.9)	(12.5)	(9.3)	(12.1)	(33.9)	(13.7)	(14.8)	(15.8)	(16.8)	(61.2)	(74.0)
Share based comp	0.0	0.7	0.4	1.3	2.0	3.8	1.1	1.2	1.3	1.4	5.0	6.0
D&A	0.1	0.1	0.1	0.0	0.0	0.1	-	-	-	-	-	-
Other (Change in WC)	1.7	(0.4)	0.7	(0.9)	0.0	(0.3)	-	-	-	-	-	-
<b>Cash from investing</b>	<b>(0.4)</b>	<b>(0.1)</b>	<b>-</b>	<b>(0.0)</b>	<b>(0.0)</b>	<b>(0.1)</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>
CapEx	(0.4)	(0.1)	-	(0.0)	(0.0)	(0.1)	-	-	-	-	-	-
Acquisitions	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-
<b>Cash from financing</b>	<b>12.0</b>	<b>9.5</b>	<b>28.6</b>	<b>44.2</b>	<b>113.2</b>	<b>185.9</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>
Equity issue (buyback)	12.0	9.5	28.6	44.2	40.4	113.2	-	-	-	-	-	-
Debt issue (principal payment)	-	-	-	-	0.1	0.1	-	-	-	-	-	-
Other	-	-	-	-	72.7	72.7	-	-	-	-	-	-

Source: SEC Filings and Leerink Partners Estimates

1Q14 Results					
1Q14 (\$M, except EPS)	Consensus 1Q14	Leerink 1Q14E	1Q14A	Difference (Consensus)	Difference (Leerink)
Contract and grant revenue	0.16	0.25	0.17	0.01	(0.08)
<b>Total revenue</b>	<b>0.16</b>	<b>0.25</b>	<b>0.17</b>	<b>0.01</b>	<b>(0.08)</b>
R&D expense	10	11	11	1	(0)
SG&A expense	3	3	3	(0)	(0)
Total operating expenses	13	14	14	0	(0)
Operating income (loss)	(13.5)	(13.8)	(13.7)	(0.2)	0.0
Total other income (expense)	0	0	0.0	0.0	0.0
EBT	(12.5)	(13.8)	(13.7)	(1.2)	0.1
Income Tax expense	0	0	0	0	0
<b>Net income (loss)</b>	<b>(13.5)</b>	<b>(13.8)</b>	<b>(13.7)</b>	<b>(0.2)</b>	<b>0.1</b>
EPS	(0.47)	(0.46)	(0.46)	0.0	(0.00)
Common shares outstanding	29	30	30	0.8	0
BS & CF	Consensus 1Q14	Leerink 1Q14E	1Q14A	Difference (Consensus)	Difference (Leerink)
Cash & equivalents		143	145		2
Debt		0	0		0

Source: Leerink Partners Estimates and Company Filings

**KPTI Valuation**

Year	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
P/W FCF	(61.2)	(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	(61.2)	(66.1)	(68.6)	(57.8)	(32.8)	22.3	107.5	148.7	128.1	121.4	110.6	100.8
<b>NPV</b>	<b>1,783</b>											
NPV/sh	60											
Cash/share	3.41											
<b>Total</b>	<b>63</b>											

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

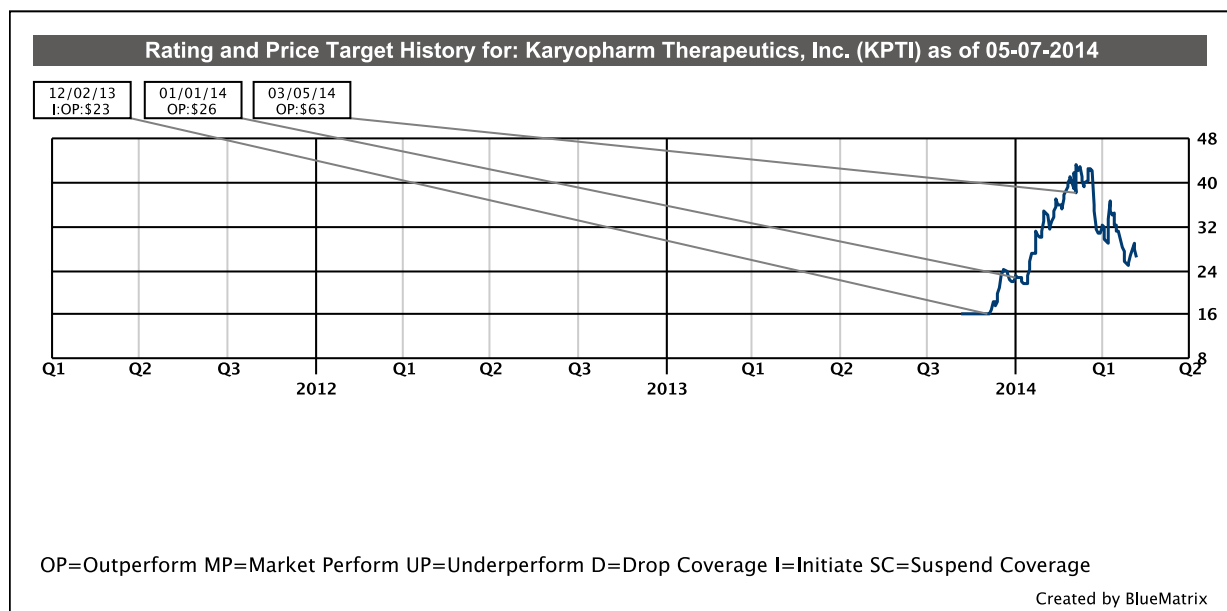
I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

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### 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 on our valuation.





Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
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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Leerink Partners LLC Equity Research			
<b>Director of Equity Research</b>	<b>John L. Sullivan, CFA</b>	(617) 918-4875	john.sullivan@leerink
<b>Associate Director of Research</b>	<b>Alice C. Avanian, CFA</b>	(617) 918-4544	alice.avanian@leerink
<b>Healthcare Strategy</b>	<b>John L. Sullivan, CFA</b>	(617) 918-4875	john.sullivan@leerink
	<b>Alice C. Avanian, CFA</b>	(617) 918-4544	alice.avanian@leerink
<b>Biotechnology</b>	<b>Howard Liang, Ph.D.</b>	(617) 918-4857	howard.liang@leerink
	<b>Joseph P. Schwartz</b>	(617) 918-4575	joseph.schwartz@leerink
	<b>Marko Kozul, M.D.</b>	(415) 905-7221	marko.kozul@leerink
	<b>Michael Schmidt, Ph.D.</b>	(617) 918-4588	michael.schmidt@leerink
	<b>Gena Wang, Ph.D., CFA</b>	(212) 277-6073	gena.wang@leerink
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink
	Paul Matteis	(617) 918-4585	paul.matteis@leerink
	Richard Goss	(617) 918-4059	richard.goss@leerink
<b>Life Science Tools and Diagnostics</b>	<b>Dan Leonard</b>	(212) 277-6116	dan.leonard@leerink
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink
<b>Pharmaceuticals/Major</b>	<b>Seamus Fernandez</b>	(617) 918-4011	seamus.fernandez@leerink
	Ario Arabi	(617) 918-4568	ario.arabi@leerink
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink
<b>Specialty Pharmaceuticals, Generics</b>	<b>Jason M. Gerberry, JD</b>	(617) 918-4549	jason.gerberry@leerink
	Christopher W. Kuehnle, JD	(617) 918-4851	chris.kuehnle@leerink
<b>Medical Devices, Cardiology &amp; Orthopedics</b>	<b>Danielle Antalffy</b>	(212) 277-6044	danielle.antalffy@leerink
	<b>Richard Newitter</b>	(212) 277-6088	richard.newitter@leerink
	Ravi Misra	(212) 277-6049	ravi.misra@leerink
<b>Healthcare Services</b>	<b>Ana Gupte, Ph.D.</b>	(212) 277-6040	ana.gupte@leerink
<b>Healthcare Technology &amp; Distribution</b>	<b>David Larsen, CFA</b>	(617) 918-4502	david.larsen@leerink
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink
<b>Sr. Editor/Supervisory Analyst</b>	<b>Mary Ellen Eagan, CFA</b>	(617) 918-4837	maryellen.eagan@leerink
Supervisory Analysts	Robert Egan		bob.egan@leerink
	Amy N. Sonne		amy.sonne@leerink
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink

**New York**  
299 Park Avenue, 21<sup>st</sup> floor  
New York, NY 10171  
(888) 778-1653

**Boston**  
**One Federal Street, 37<sup>th</sup> Floor**  
**Boston, MA 02110**  
**(800) 808-7525**

**San Francisco**  
201 Spear Street, 16<sup>th</sup> Floor  
San Francisco, CA 94105  
(800) 778-1164