December 2, 2013

OUTPERFORM

Reason for report:

INITIATION

Michael Schmidt, Ph.D.
(617) 918-4588
Michael.Schmidt@Leerink.com

Jonathan Chang, Ph.D.
(617) 918-4015
Jonathan.Chang@Leerink.com

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HEALTHCARE EQUITY RESEARCH

KARYOPHARM THERAPEUTICS, INC.

Selinexor - Unique Cancer Drug with Broad Activity; Initiate with OP

• **Bottom Line:** Karyopharm Therapeutics is an early stage biopharmaceutical company focused on developing small molecule cancer drugs called "Selective Inhibitors of Nuclear Export" (SINE). Its lead product candidate Selinexor (KPT-330) is a first-in-class orally bioavailable inhibitor of XPO1 and was discovered by KPTI. Based on feedback from MEDACorp KOLs, we believe Selinexor has promising single-agent activity in shrinking tumors or delaying progression in highly refractory patients across a broad range of solid and hematological cancers. Dose-expansion data in 2014 could validate Selinexor activity observed during Phase I dose-escalation trials. KPTI's experienced management team is pursuing a fast-to-market strategy for Selinexor with data from two initial pivotal trials likely available in 1H16. Our $23 price target is based on 35% probability-weighted Selinexor cash flows, and we think KPTI could be an attractive takeover candidate.

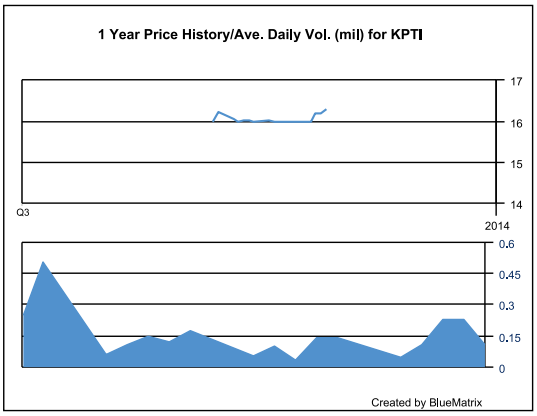
• **Selinexor (KPT-330) a first-in-class oral inhibitor of XPO1 is KPTI's key value driver**. Selinexor selectively induces apoptosis in cancer cells by restoring tumor-suppressor protein activity in the nucleus. Cancer cells over express XPO1 as a survival mechanism, causing increased export of tumor suppressor proteins from the nucleus. KPTI discovered Selinexor using a structure-based drug design that allowed its scientists to develop an inhibitor with improved molecular properties relative to naturally occurring XPO1 inhibitors such as Leptomycin B.

• **Based on our due diligence with MEDACorp KOLs we believe Selinexor has promising single agent activity in shrinking tumors or delaying progression in highly refractory patients across a broad range of solid and hematological cancers.** We believe the drug has a good safety profile based on MEDACorp KOL comments ,and drug-related toxicities including anorexia and fatigue have been largely manageable through supportive care or in few cases through temporary dose-reductions or interruptions. Selinexor has a differentiated mechanism of action from any other drugs approved, and given its safety/ tolerability profile, it is potentially combinable with a wide range of agents which could produce a synergistic effect.

• **Dose-expansion data in 2014 could validate Selinexor activity observed in Phase I.** KPTI is currently enrolling patients in dose-expansion cohorts in Multiple Myeloma (MM), Diffuse Large B-Cell Lymphoma (DLBCL), acute myeloid leukemia (AML), T-Cell Lymphoma (TCL) and solid tumors where activity during dose-escalation has been most promising. To date, anti-cancer activity has been observed in 74% of patients with rrB-cell cancers, 47% of patients with rrAML and 45% of patients with heavily pretreated solid tumor malignancies. We believe dose expansion data could provide even more impressive data as patients are treated with an optimized dosing regimen and tolerability is managed with supportive care.

Key Stats:	**(NASDAQ:KPTI)**
S&P 600 Health Care Index:	**1,273.08**
Price:	**$16.18**
Price Target:	$23.00
Methodology:	DCF, 12% discount rate, 5x terminal value
52 Week High:	$19.09
52 Week Low:	$15.50
Shares Outstanding (mil):	28.7
Market Capitalization (mil):	$464.4
Book Value/Share:	$5.53
Cash Per Share:	$5.03
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	--	--	--	--	0.8	--	--	--	--	(3.22)	NM
2014E	--	--	--	--	1.0	--	--	--	--	(1.38)	NM

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

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The Healthcare Investment Bank™

Karyopharm Therapeutics (NASDAQ: KPTI)
Initiation of Coverage
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Michael Schmidt, Ph.D. | Research Analyst | Leerink Swann LLC | (617) 918-4588 | michael.schmidt@leerink.com

Jonathan Chang, Ph.D. | Research Associate| Leerink Swann LLC | (617) 918-4015 | jonathan.chang@leerink.com

KPTI Investment Thesis

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- **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 lean business model.** Karyopharm is led by Michael Kauffman who was previously CMO at ONYX (previously Proteolyx) 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 and Risks

- **Valuation:** We estimate a $23 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 in three relapsed/refractory hematological cancer indications (AML, DLBCL, and MM). Potential 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 5x multiple to 2032 Selinexor revenue, discounted back by 18.25 periods. We believe use of a 5x revenue multiple is conservative compared to that of the average (6x) for its biotech industry peer group, which we define as public companies with market caps between $1B to $10B and current year sales. Based on our DCF analysis, we attribute $20/share to Selinexor and the rest to the preclinical pipeline and platform and the remainder to expected cash in one year.
- **Risks:** 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 operations 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. Additionally 47% of the shares are controlled by one entity and are subject to a 180-day lock-up from the effective date of the registration statement. The two senior officers of the company control 14% of the stock.

KPTI Management with Track Record of Success Implements Lean Business Model

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- CEO/CMO: Michael Kauffman
  - CEO/CMO since January 2011
  - 2008-2010: CMO at ONYX (previously Proteolyx); led the clinical development of Kyprolis (carfilzomib)
  - 2006-2008: Bessemer Venture Partners
  - 2002-2008: CEO Epix/Perdix Pharmaceuticals; oversaw the discovery and development of four new clinical candidates and led collaboration transactions with AMGN and GSK
  - 2000-2002: Vice President, Clinical at Millennium Pharmaceuticals; led the Velcade development program
- CSO: Sharon Shacham
  - Founded Karyopharm in 2008; CEO 2010-2011
  - 2000-2009: SVP Drug Development at Epix Pharmaceuticals, Inc.,
- SVP Finance: Paul Brannelly
  - 2011-2013: VP Finance/CFO at Verastem
  - Previously: Longwood Fund, Sirtris
- Lean Business Model
  - KPT-330: 17 months from lead identification into clinic
  - IPO proceeds potentially sufficient, in our view, to get KPT-330 to market in early 2017
  - Significant upside potential: additional indications (Richter's syndrome, solid tumors)

Selinexor (KPT-330) an XPO1 Inhibitor Is KPTI's Main Value Driver

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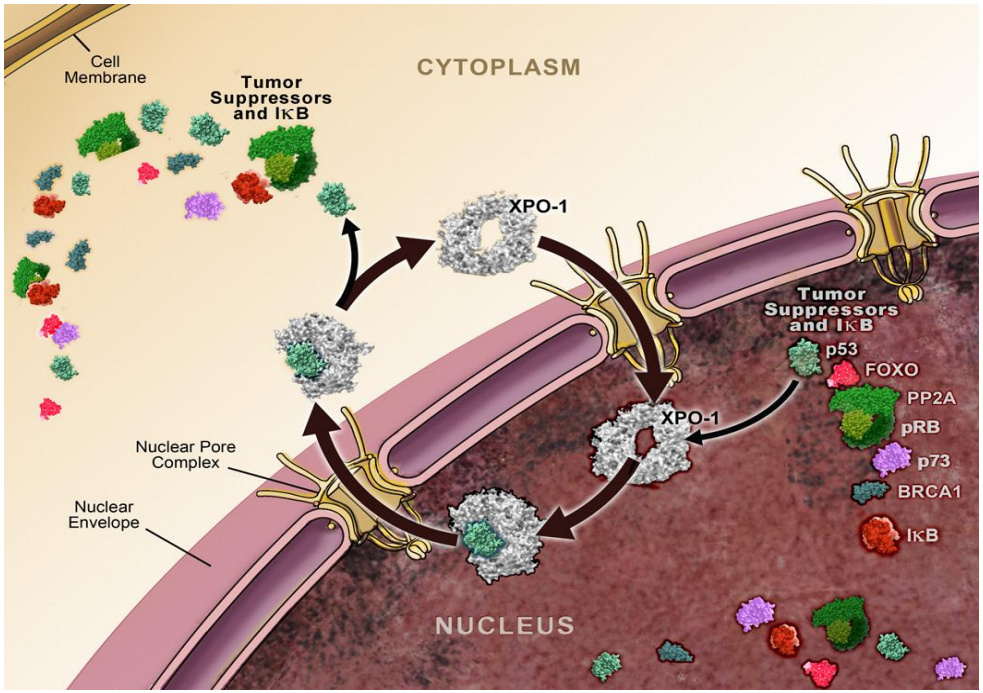
- Selinexor is a highly specific orally bioavailable small molecule inhibitor of XPO1 (CRM1), currently in Phase I clinical trials
- XPO1 is a major export protein that facilitates the transport of many proteins, including important tumor suppressors across the nuclear membrane to the cytoplasm.
- Selinexor is thought to selectively induce apoptosis in cancer cells by restoring tumor-suppressor protein activity in the nucleus, their natural location of activity. Cancer cells over express XPO1 as a survival mechanism causing increased export of tumor suppressor proteins from the nucleus.
- Based on broad anti-tumor activity and minimal toxicity against normal hematopoietic cells seen in preclinical models, KPTI filed two investigational new drug applications (INDs), one in solid tumors and one in hematological malignancies, respectively, in May 2012.
- Selinexor is to our knowledge the first and only XPO1 inhibitor in currently clinical trials. By using structure-based design, KPTI has been able to discover a series of molecules which irreversibly bind to the XPO1 cargo-binding site tighter and with improved positioning relative to previously investigated XPO1 inhibitors.
- Leptomycin B (LMB) is a widely known inhibitor of XPO1 with significant in-vitro potency but limited in-vivo efficacy due to significant toxicity. Other approaches e.g. by Kosan Biosciences (acquired by BMY [OP]) using LMB analogues have had only limited success given that the therapeutic index remained too narrow.



Source: Karyopharm illustration

Early Stage Pipeline Candidates are Upside, In Our View

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- KPT-335 (Verdinexor)
  - In Phase IIb for pet dog Lymphoma to support regulatory approval
  - SINE compound related to Selinexor (KPT-330)
  - KPTI plans to submit a New Animal Drug Application to the FDA in early 2014 and outlicense the product post-approval
- KPT-350
  - Preclinical
  - SINE compound related to Selinexor (KPT-330)
  - Potential therapeutic benefit in autoimmune and inflammatory diseases/wound healing based on preclinical data
- Pak4 inhibitors
  - Preclincial; KPTI is currently conducting IND-enabling research
  - Pak4 is an XPO1 cargo protein (P21-activated kinase 4) identified by KPTI
  - PAK4 inhibitors have shown evidence of anti-cancer activity against hematological and solid tumor malignancies cells in vitro and evidence of anti-cancer activity in mouse xenografts

For Selinexor, KPTI Pursues an Accelerated Path to Approval with Potential Label Expansions Thereafter

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- KPTI is currently enrolling dose-expansion cohorts in Selinexor Phase Ib trials across a wide range of solid tumor indications and hematological cancers.
- KPTI expects initiation of two pivotal Phase II/III trials in 2Q14 in first hematological indications
  - Based on feedback from management, we believe the 1st pivotal trial is likely to target elderly relapsed/refractory (r/r) AML after progression from Vidaza and/or Dacogen based regimen
    - 200pts randomized/controlled trial vs. best supportive care; survival (overall survival [OS]) endpoint
    - We believe KPTI could power the study for 2.5 months OS in KPT-330 arm vs. 1 month for placebo
  - 2nd pivotal trial likely to target relapsed or refractory (r/r) DLBCL in patients failing at least 2 lines of therapy, in our view
    - Potential trial design could include 250pts in a randomized/controlled trial of KPT-330 + chemo vs. chemo (physician's choice); progression-free survival (PFS) endpoint (2-3 months in the control arm)
  - We believe Multiple Myeloma could be a potential 3rd hematological indication due to Selinexor activity there in Phase I and high physician enthusiasm. Selinexor could be tested as single agent in last line of therapy after Kyprolis or in combination with Kyprolis
- KPTI expects initiation two Phase II trials in solid tumor indications in early 2014
  - 40-60 pts in each study to assess progression free survival (PFS) and response rates (RR)
  - Goal is to identify market and path to approval in solid tumor indications by 2015
- In addition, KPTI expects near-term initiation of over 20 Phase I/II single agent or combination investigator-initiated trials which we believe could provide evidence of activity in additional cancers or specific regimen
  - Solid tumors with chemotherapies, radiation, tyrosine kinase inhibitors (TKIs)
  - Hematologic cancers in combinations with steroids, alkylators, proteasome inhibitors

Selinexor Upcoming Events

Event	Indication	Timing
Phase I program		
Phase I data update	Hematological cancers	ASH Dec. 12, 2013
End of Phase I FDA/EMA meeting	Pivotal trial protocol review	4Q13
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 data	Heme Arm 3 (TCL)	2014
Phase I data	Food effect study in soft tissue/bone sacromas	2014
Hematological cancers		
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
Solid tumors		
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 solid tumor (gynecological)	2H15
Phase II data	single agent solid tumor (squamous cell cancers [lung, head and neck, esophageal)	2H15
Investigator-Initiated Studies		
Initiation Phase I/II	combination	2013/14

Source: Company filings and Leerink Swann estimates

Takeaways from Selinexor Phase I Trials

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- **Selinexor has been tested in a broad Phase I program with over170 patients enrolled through September 20, 2013.** KPTI are currently conducting three open-label Phase I clinical trials of Selinexor, the first in patients with heavily pretreated relapsed and/or refractory hematological malignancies, the second in patients with heavily pretreated relapsed and/or refractory solid tumor malignancies, and the third, a food effect study, in patients with metastatic, locally advanced or locally recurrent soft tissue or bone sarcomas. The dose-escalation phase has been completed without reaching a maximum tolerated dose [MTD]. KPTI is currently enrolling patients in five dose expansion cohorts.
- **MEDACorp specialists we spoke to are positive on Selinexor's safety/efficacy profile, based on Phase I data.** Based on MEDACorp KOL feedback, we believe Selinexor has strong single agent activity in shrinking tumors or delaying progression in very refractory cancer patients across a broad range of solid and hematological cancers. Overall the drug has a good safety profile, based on MEDACorp KOL comments and drug-related toxicities have been largely manageable through supportive care or in few cases through dose-reductions or interruptions. Selinexor has a differentiated mechanism of action from any other drugs approved or in clinical trials and given the safety/tolerability profile it is potentially combinable with a wide rage of agents which could produce a synergistic effect.
- **Selinexor Phase I dose-escalation data suggests the drug has broad activity in hematological cancers and solid tumors.** Anti-cancer activity was observed in 29 of 39 patients (74%) evaluated as of September 20 with B-cell cancers progressing from prior therapy (PR (15%), MR (15%) or SD (44%). Preliminary evidence of anti-cancer activity was also seen in relapsed/refractory AML. 15 of 32 patients (47%) evaluated as of September 20 experienced a CR, CR(i) or SD (6%, 3%, and 38% respectively). Selinexor also showed signs of efficacy in patients with heavily pretreated relapsed/refractory solid tumor malignancies. 35 of the 77 patients (45%) evaluated as of September 20 in this arm experienced a PR or SD, including a PR in a patient with CRC and a PR in a patient with melanoma. 33 patients have experienced SD, based on RECIST criteria.
- **Selinexor fixed-dose expansion data in 2014 from ongoing studies could provide even more impressive efficacy data as patients are treated with an optimized dosing regimen.** KPTI expects to see fewer and milder gastrointestinal events and reduced fatigue in dose expansion cohorts as a result of the initiation of supportive care and medications prior to beginning Selinexor therapy. We believe optimized dosing could result in higher response rates in expansion cohorts.

170 Patients Treated Through Sept 20 in Ongoing Selinexor Phase I Trials Since July 2012

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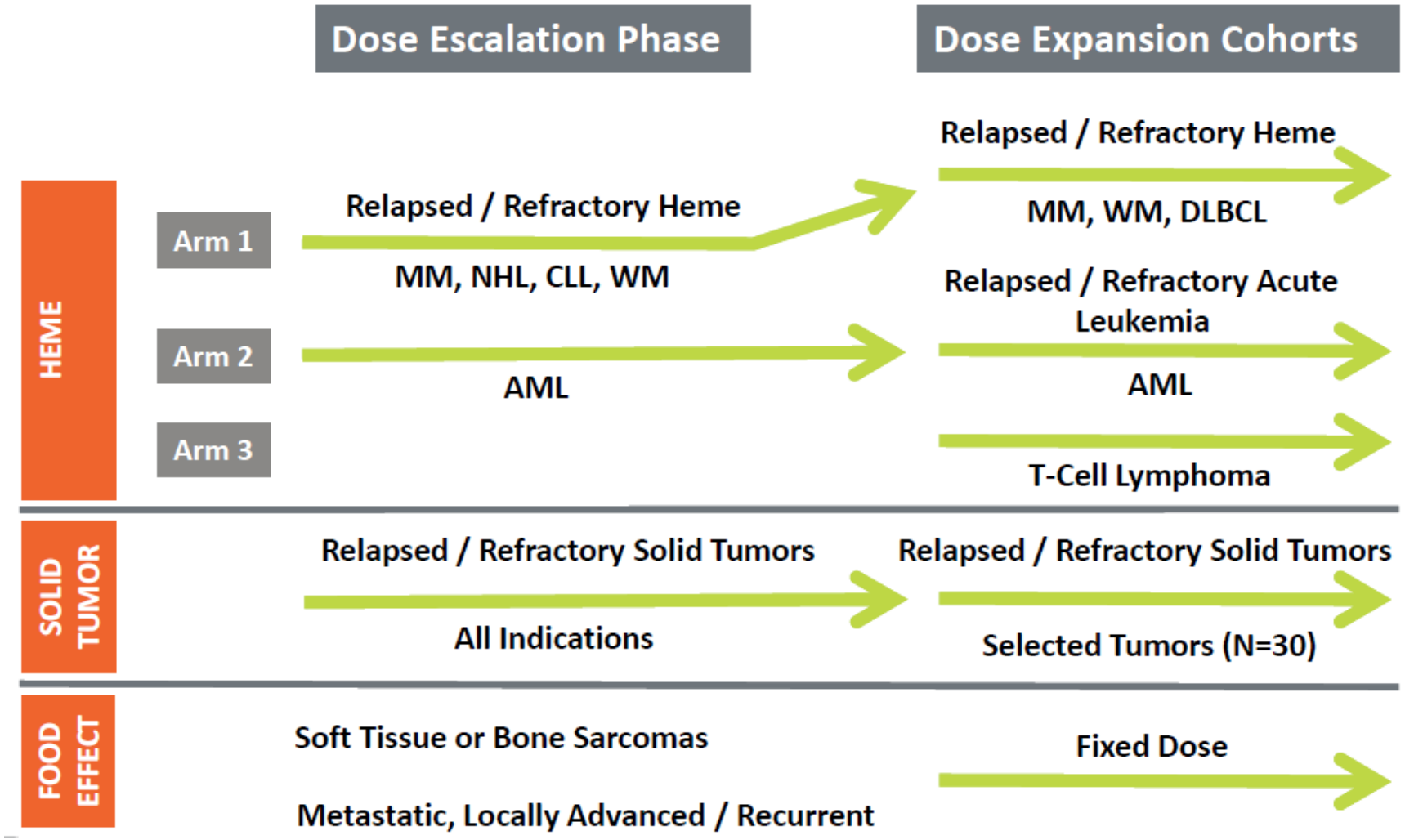

Source: Karyopharm

MEDACorp Specialists Positive on Selinexor Profile

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- **Feedback from MEDACorp Specialists and opinion leaders supports our thesis that Selinexor has strong activity in shrinking tumors or delaying progression in very refractory cancer patients across a broad range of indications**. Overall the drug has a god safety profile, based on MEDACorp KOL comments and drug-related toxicities have been manageable, either through supportive care or in few cases through dose-reductions or interruptions. Specialists we spoke to noted a clear dose-response in terms of the pharmokinetics (PK), but the exact minimally effective dose and maximum tolerated dose (MTD) still remain unclear. It is still mechanistically unclear how exactly XPO1 inhibition exhibits anticancer activity, but Selinexor has a differentiated mechanism from any other approved or tried drugs and a good safety profile, which makes it potentially combinable with a wide rage of agents. Preclinical data suggests potential synergistic activity of Selinexor with proteasome inhibitors, topoisomerase I inhibitors, and hypomethylating agents. Future discovery of a biomarker which could predict which patients have a high likelihood for a response would be highly attractive. according to KOLs.

- **MEDACorp KOLs view Selinexor's differentiated mechanism of action is a big positive, since the drug could be combined with other therapies and because there is an unmet medical need for new anti-cancer drugs.** KPTI rationally designed Selinexor, a small molecule inhibitor based on the recent determination of the XPO1 crystal structure. Leptomycin B is a naturally occurring XPO1 inhibitor that has been tested in the clinic in the late '90s, but its narrow therapeutic window limited further development beyond Phase I. KOLs view Selinexor data from animal models of AML "remarkable" since the drug can be dosed very aggressively and still spare normal bone marrow cells. Still a subject of investigation is how the drug induces cell death and in which patients it is most likely to have the biggest effects. One hypothesis is that Selinexor-induced nuclear localization of tumor suppressor proteins induces the observed apoptosis in cancer cells. Selinexor's activity is not limited to one type of tumor and is thus being investigated across a large variety of different tumor types. All the MEDACorp physicians with whom we spoke are excited to test a drug with a new mechanism.

Enthusiasm Among MEDACorp Specialists Regarding Clinical Efficacy of Selinexor

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- **Enthusiasm about clinical efficacy seen to date among MEDACorp specialists**. Consensus among physicians we spoke to was that any type of response is important in the type of patients treated to date, especially patients that have minimal responses with other agents. In AML for instance, no good therapy is currently available for refractory patients according to specialists. Thus, the general expectation is that one "doesn't see anything" in AML with new agents in the clinic. Here some patients had resolution of bone marrow lesions, which is a significant positive. In addition, specialists felt that Selinexor produced an "incredible signal" in refractory MM, but the clinical bar is a bit higher here than in AML where the threshold to show success is very low, especial in the elderly. Overall, Selinexor seems to be a very good disease stabilizer in hematology and even stable disease is good in these patients ("50% of pts having stable disease is great"). MEDACorp specialists also viewed the observed refractory solid tumor activity as "highly interesting" and believed Selinexor may be likely used in combination with cytotoxic therapy. One specialist stated that the drug has activity at levels that are well tolerated by human patients. Main drawback noted by most of the physicians we spoke to has been that fact that some cancers may be more responsive to Selinexor therapy than others. And within the same type of cancer, it is still unclear which patients respond. We believe a potential future biomarker to guide drug use could improve the profile of Selinexor even more. Most physicians believed that the next step would be to combine the drug with one that could cause synergies, such as Kyprolis in MM, Vidaza or Dacogen in AML, or topotecan in solid tumors.
- **Safety profile encouraging according to MEDACorp specialists**. Consensus among physicians we spoke to was that Selinexor's safety and tolerability profile is acceptable. Most physicians considered AEs seem as very mild, "mainly anorexia and a bit of diarrhea". Toxicity has been largely limited to GI, with some patients having thrombocytopenia which has not been an issue. Others noted that sometimes it is tough to distinguish a blunting of the drug's effect vs. real toxicity in late stage hematological indications. MEDACorp specialists noted that the initial occurrence of discontinuations was likely due to inadequate experience with managing the drug's AE profile early which is now done fairly successfully e.g., in treating anorexia and GI AEs. MEDACorp specialists believe that platelet drops seen at higher doses in some patients has not been an issue, especially since bleeding has not been seen at tested doses. There has been no cumulative or organ toxicity.

Selinexor Is Active in B-Cell Cancers, Based on Phase I (Arm 1) Dose-Escalation Data

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- Dose-escalation 3 - 45mg/m2 2/3 x weekly for 28 days/cycle
- Population: Late stage, relapsed/refractory B-cell cancers
- Stable disease (no tumor growth) for >4-8 weeks is an indicator of anti-cancer activity according to MEDACorp specialists
- 5 patients were at >9 months response duration (September 20th, 2013)
- Currently (since June) enrolling dose-expansion MM, WM, DLBCL 8 doses/cycle @35mg/m2

Dose-escalation (3-45mg/m2 2-3x weekly)												
Arm1 B-cell cancers (14 pts still on study)	**Enrolled**	**Evaluated**	**PR**		**MR**		**SD**		**PD**		**WC**		**NE**	
MM	20	17	1	6%	4	24%	7	41%	4	24%	1	6%		
WM	2	2			2	100%								
CLL	8	5	3	60%			2	40%						
NHL	19	15	2	13%			8	53%						
DLBCL		5	1	20%			2	40%	2	40%				
MCL		3	1	33%			1	33%					1	33%
FL		5					5	100%						
Transformed		2							2	100%				
Total	49	39	6	15%	6	15%	17	44%	8	21%	1	3%	1	3%
Dose expansion (n=10)	8 doses/cycle @ 35mg/m2													
MM, WM	10 Fully enrolled													
DLBCL	15													

Source: Karyopharm and Leerink Swann Research

Abbreviations:
WM: Waldenström's Macroglobulinemia
MCL: Mantle Cell Lymphoma
FL: follicular lymphoma
NHL: Non Hodgkin's lymphoma
DlBCl: Diffuse large B cell lymphoma

PR: Partial Response
MR: Minimal Response
SD: Stable Disease
PD: Progressive Disease

WC: Withdrew Consent
NE: Not Evaluated

Selinexor Phase I (Arm 2) Dose-Escalation Data Suggests Drug Is Active in AML

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- Dose-escalation 16 - 40mg/m2 2/3 x weekly for 28 days/cycle
- Population: heavily pretreated relapsed/refractory AML with progressive disease
- Planning dose-expansion 8 doses @40mg/m2 in 4Q13
- 4 patients at >3months response duration at last update (September 20, 2013); typical overall survival (OS) is ~1 month in these patients according to MEDACorp KOLs
- In 4Q13, KPTI plans to enroll 25 patients with heavily pretreated AML in a fixed dose expansion cohort of 40 mg/m2.

Arm2 AML (8 pts still on study)	**Enrolled**	**Evaluated**	**CR**		**CRi**		**SD**		**PD**		**WC**		**NE**	
AML	34	32	2	6%	1	3%	12	38%	11	34%	4	13%	2	6%
Dose expansion (n=25)	8 doses/cycle @ 40mg/m2 begin enrolling in 4Q13													

Source: Karyopharm and Leerink Swann Research

Selinexor Phase I Dose-Escalation Data Suggest Drug Is Active in Solid Tumors

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- Dose-escalation 3 - 50mg/m2 2/3 x weekly for 28 days/cycle
- Population: 85 patients with heavily pretreated relapsed/refractory solid tumor malignancies with progressive disease
- 7 patients at >6 months response duration (September 20th, 2013)
- Currently (since May) enrolling dose-expansion in 30 patients with colorectal (CRC), prostate, ovarian, squamous cancers or malignant gliomas; 8 doses/cycle @35mg/m2

Solid tumors (n=85 treated in P1a; 20pts still on study)												
Dose-escalation (n=77 evaluable)	**Evaluated**	**PR**		**SD**		**PD**		**WC**		**NE**	
CRC	29	1	3%	10	34%	16	55%			2	7%
SCCHN	9			4	44%	4	44%			1	11%
lung cancer	6			4	67%	2	33%				
ovarian cancer	7			3	43%	2	29%	1	14%	1	14%
cervical cancer	2			2	100%						
endometrial stromal sarcome	2			2	100%						
melanoma	2	1	50%	1	50%						
pancreatic cancer	5			1	20%	1	20%	2	40%	1	20%
prostate cancer	4			3	75%			1	25%		
glioblastoma	1					1	100%				
other	10			3	30%	6	60%			1	10%
Total	77	2	3%	33	43%	32	42%	4	5%	6	8%
Dose expansion (n=30)	8 doses/cycle @ 35mg/m2										

SCCHN: squamous cell carcinoma of the head and neck

Source: Karyopharm and Leerink Swann Research

Good Selinexor Safety Profile, Tolerability Manageable

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- B-Cell lymphomas (49 patients treated as of September 20th)
  - Grade 1/2 AEs: Gastrointestinal AEs (nausea [61%], anorexia [49%], vomiting [31%], diarrhea [31%]) and fatigue (39%) are the most common types of Grade 1/2 AEs seen in Arm 1. GI AEs are generally responsive to standard supportive care.
  - Grade 3/4 AEs: Fatigue (2%), thrombocytopenia (24%), neutropenia (22%). KPTI believes that thrombocytopenia and neutropenia are primarily a result of patients entering this arm with marked bone marrow suppression due to both disease and prior therapies.
  - KPTI now instructs physicians to initiate supportive care and medications prior to patients beginning on Selinexor therapy (maintaining caloric and fluid intake as well as the introduction of anti-nausea medication).
  - 49 serious adverse events (SAEs) reported in 21 patients in Arm 1. One SAE was deemed related to Selinexor (Grade 2 blurred vision).
- AML (34 patients treated as of September 20th)
  - Grade 1/2 (93%) AEs: Primarily GI related and generally responsive to standard supportive care. Nausea (53%), anorexia (50%), vomiting (35%) and weight loss in 7 patients (21%).
  - Fatigue was observed in 47%, including Grade 3 fatigue (6%) and Grade 1/2 fatigue (41%).
  - Grade 4 thrombocytopenia (9%) and Grade 4 neutropenia (6%) likely due to patients entering this arm with marked bone marrow suppression due to both disease and prior therapies.
  - Of the 49 SAEs reported, one SAE was deemed to be related to Selinexor (Grade 3 nausea with Grade 1 vomiting). Pt recovered later.
- Solid tumors (85 patients treated as of September 20th)
  - GI AEs Grade 1/2 (94%) nausea (65%), anorexia (59%), vomiting (48%), dysgeusia (35%), weight loss (32%) diarrhea (25%).
  - Fatigue was observed in 64%, including Grade 3 fatigue in 12% and Grade 1/2 fatigue in 52%
  - Anemia (24%), including Grade 3 anemia (7%) and Grade 1 or Grade 2 anemia in 14 patients (16%).
  - 61 SAEs reported, 4 were deemed related to Selinexor: 1 Grade 3 nausea, 1 Grade 3 nausea and vomiting, 1 Grade 3 weight loss and severe malnutrition, and 1 Grade 3 fatigue. All four patients who experienced these SAEs have recovered from these SAEs following supportive care.

Two Additional Phase I Studies are Ongoing

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- Food Effect Study (ongoing)
  - Depending on the results of the food effect study, KPTI may consider using the tablet formulation (vs. capsules) in future clinical trials of Selinexor.
  - Enrolling since July 2013 (3/20 pts enrolled as of September)
  - Phase Ib open-label food effect study in heavily pretreated patients who have metastatic, locally advanced or locally recurrent soft tissue or bone sarcomas
  - The trial is primarily designed to evaluate the effects of food and formulation (capsules and tablets) on the absorption of oral Selinexor.
  - KPTI also expects to gather additional safety and efficacy data regarding Selinexor in this trial.
  - KPTI is currently using the capsule formulation in Phase I clinical trials.
  - As of September 20, 2013, three patients have been enrolled in this clinical trial.
- Selinexor Phase I (Arm 3) Dose-Expansion Phase in T-Cell Lymphoma ongoing
  - KPTI is evaluating Selinexor in patients with heavily pretreated relapsed and/or refractory T-cell lymphoma
  - Up to 12 patients with relapsed / refractory Peripheral (PTCL) and Cutaneous (CTCL) T Cell Lymphoma will be enrolled in this cohort at a dose of 30mg/m2 twice weekly
  - KPTI began enrollment in August 2013 and has administered Selinexor to one patient in Arm 3 who had not yet been evaluated as of September 20, 2013.

Dose Expansion Data in 2014 Should Provide More Insight Into Selinexor's Promising Anti-Cancer Activity

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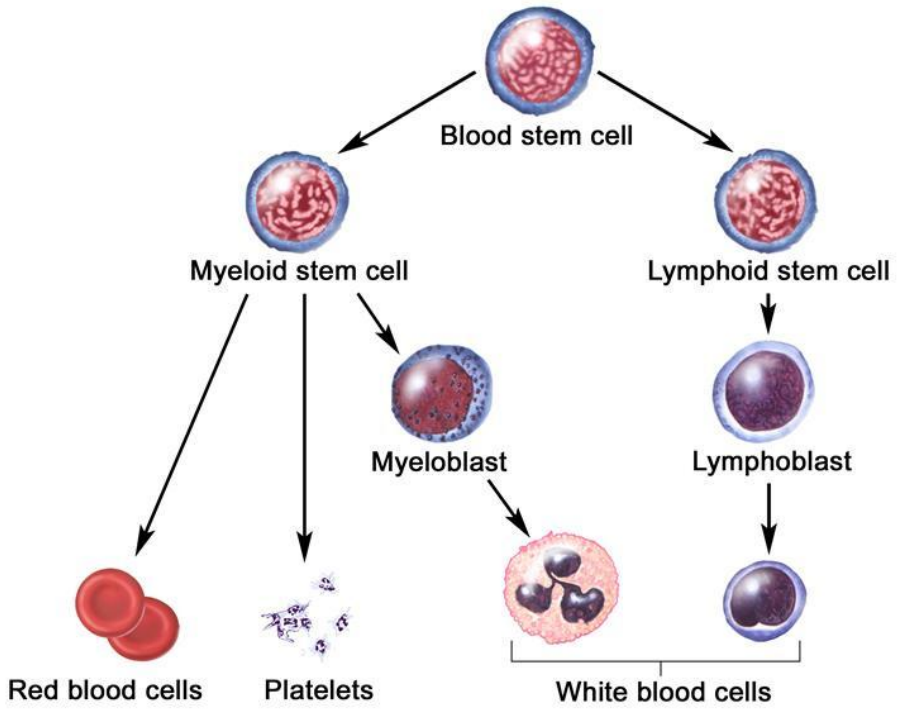
- KPTI is currently enrolling four dose-expansion cohorts:
  - 10 patients with MM/WM (35mg/m2 twice weekly)
  - 15 patients with DLBCL (35mg/m2 twice weekly)
  - 25 patients with AML (40mg/m2 twice weekly)
  - 30 patients with solid tumors (squamous cell and gynecological cancers, gliomas): 35mg/m2 twice weekly
- We believe data from expansion phase patients in mid-2014 will inform selection of the two initial indications.
- In addition, KPTI will get a better picture of the efficacy/safety profile of Selinexor in more coherent patient cohorts, all treated at that same dose, and including consistent management of AEs.
- We expect response rates and duration of responses to increase over those seen for the dose-escalation phase.

Acute Myeloid Leukemia (AML) Background



Source: cancer.gov

- Adult acute myeloid leukemia (AML) is a cancer of the blood and bone marrow
- Normally, the bone marrow makes blood stem cells (immature cells) that become mature blood cells over time. A blood stem cell may become a myeloid stem cell or a lymphoid stem cell. A lymphoid stem cell becomes a white blood cell.
- A myeloid stem cell becomes one of three types of mature blood cells:
  - Red blood cells that carry oxygen and other substances to all tissues of the body.
  - White blood cells that fight infection and disease.
  - Platelets that form blood clots to stop bleeding.
- In AML, the myeloid stem cells usually become a type of immature white blood cell called myeloblasts and sometimes abnormal red blood cells, or platelets which are called leukemia cells or blasts.
- Leukemia cells can build up in the bone marrow and blood so there is less room for healthy white blood cells, red blood cells, and platelets. When this happens, infection, anemia, or easy bleeding may occur.

Significant Unmet Medical Need in Elderly AML

- AML (Acute Myeloid Leukemia) is described as untreated, in remission, or recurrent.
- The two treatment phases of adult AML are:
  - Remission induction therapy: The goal is to kill the leukemia cells in the blood and bone marrow which puts the leukemia into remission. Standard treatment depends on the subtype of AML and may include the following: Combination chemotherapy, high-dose chemotherapy, with or without radiation therapy, and stem cell transplant
  - Post-remission therapy: It begins after the leukemia is in remission. The goal of post-remission therapy is to kill any remaining leukemia cells that may not be active but could begin to regrow and cause a relapse.
- Recurrent AML: no standard of care
- AML presents at all ages, but is mainly a disease of the elderly, with a median age of 69 years in the white US population
- Decisions regarding the aggressiveness and timeliness of therapy are challenging in older adults, as the disease biology predicts for chemotherapy resistance, and intensive therapy is accompanied by high treatment-related mortality. In older patients, complete remission rates to standard remission induction therapy range from 40–60%, with limited long-term survival.
- Younger adults with AML who receive standard remission induction therapy experience complete remission (CR) rates of 65–85%, 25% higher than all older adults, and at least 35% higher than the very old (patients 70 years or more). As expected with lower CR rates, 5-year overall survival (OS) rates, which approach 30% in younger adults, are cut by half for older adults, and range from 5–15%. This low chance of durable remission comes at a price of a high treatment-related mortality that approaches 25%, compared to less than 10% in the younger population. Morbidity may also be extreme, with many older adults without advanced directives requiring stays in intensive care units.

Targeted Agents in Late Stage Development for AML

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Drug	Company	MoA	Status	Patients	Comments
Dacogen	Eisai/JNJ	DNA Methyltransferase inhibitor	CRL	Newly diagnosed elderly patients (age >65)	Negative Phase III data, but trend to improved OS
Midostaurin	NVS	PKC inhibitor	Phase III	Newly diagnised FLT3 mutated younger patients (age <65)	Top-line data expected in 4Q13
SC azacitidine (Vidaza)	CELG	DNA Methyltransferase inhibitor	Phase III	Newly diagnosed elderly patients (age >65)	Top-line data expected in 4Q13/1Q14
Oral azacitidine (Vidaza)	CELG	DNA Methyltransferase inhibitor	Phase III	Newly diagnosed, age >55, maintenance after CR/CRi	Trial completes in 2018
Volasertib	Boeringer Ingelheim	Polo-like kinase 1 (Plk1) inhibitor	Phase III	Newly diagnosed elderly patients (age >65); unfit for remission	Trial completes in 2016
Quizartinib	AMBI	FLT-3 inhibitor	Phase IIb	Relapsed/Refractory pts; FLT3 +/-	Phase III planned in FLT3-ITD positive patients with r/r AML in early 2014
Tosedostat	CTIC	Aminopeptidase inhibitor	Phase IIb	Relapsed/Refractory elderly pts (age >60)	Phase III clinical trial design under review

Source: clinical trials.gov, BioMedTracker
Selected agents shown

Significant Unmet Medical Need in Diffuse Large B-Cell lymphoma (DLBCL)

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- Adult non-Hodgkin lymphoma (NHL) is a disease in which cancer cells form in the lymph system.
- The lymph system is part of the immune system and is made up of: Lymph, Lymph vessels, Lymph nodes, Spleen, Thymus, Tonsils and Bone marrow. Because lymph tissue is found throughout the body, adult NHL can begin in almost any part of the body.
- Diffuse large B-cell lymphoma (DLBCL) is the most common form of NHL, accounting for up to 30% of newly diagnosed cases in the United States. DLBCL is an aggressive (fast-growing) lymphoma. The body has two main types of lymphocytes that can develop into lymphomas: B-lymphocytes (B-cells) and T-lymphocytes (T-cells). DLBCL affects B-lymphocytes. Almost all lymphocytes begin growing in the bone marrow or lymph nodes. B-cells develop and mature in the bone marrow and lymph nodes. In DLBCL, the abnormal B-cell lymphocytes are larger than normal, and they have stopped responding to signals that usually limit the growth and reproduction of cells.
- Because DLBCL advances very quickly, it usually requires immediate treatment. A combination of chemotherapy and rituximab (Rituxan) with or without radiation therapy can lead to a cure in two-thirds of people with DLBCL, based on registry data. The most widely used treatment for DLBCL is R-CHOP (rituximab + cyclophosphamide, doxorubicin, vincristine, and prednisone). When treating patients with DLBCL, etoposide (Vepesid) may also be added resulting in a combination called R-EPOCH.
- One-third of DLBCL patients develop relapsed or refractory (r/r) disease. R/r DLBCL patients are evaluated to determine whether or not they are candidates for high-dose therapy based on risk factors.
  - Patients who are candidates for high-dose therapy who receive a second-line chemotherapy regimen and respond, may undergo a stem cell transplant. If these patients relapse again, they may be enrolled in a clinical trial
  - When patients are not candidates for high-dose therapy, they are considered for clinical trials, additional second-line therapies, or palliative radiation therapies

Targeted Agents in Development for DLBCL Focused on B-Cell Surface Antigens

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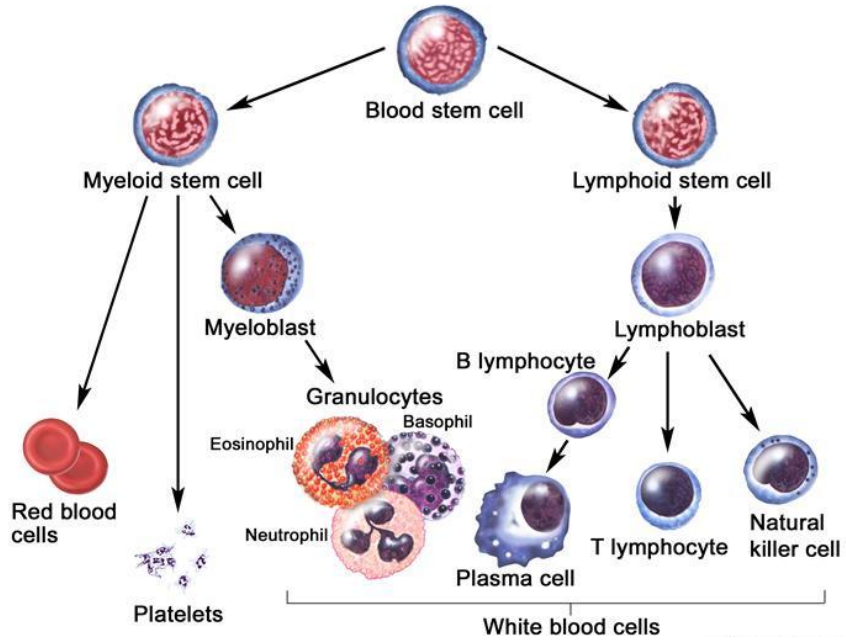
Drug	Company	MoA	Status	Patients
Everolimus (Afinitor)	NVS	mTOR inhibitor	Phase III	Second line maintenance
Ofatumumab (Arzerra)	Genmab/JNJ	CD20 mAb	Phase III	r/r DLBCL
GA101 (Gazyva)	Roche	CD20 mAb	Phase III	First line w/ CHOP
Ibrutinib (Imbruvica)	PCYC/JNJ	BTK inhibitor	Phase III	First line w/ RCHOP
Lenalidomide (Revlimid)	CEGE	Angiogenesis inhibitor	Phase III	Second line maintenance
Zevalin	SPPI/BIIB	CD20 mAb radio ADC	Phase III	Second line maintenance
Adcetris	SGEN	CD30 mAb ADC	Phase II	First line w/ RCHOP
Blinatumomab	AMGN	CD19 BiTE	Phase II	r/r DLBCL
Epratuzumab	IMMU	CD22 mAb	Phase II	n/a
ISF35	Memgen	CD40 vaccine	Phase II	n/a
MEDI-551	AZN	CD19 mAb	Phase II	r/r DLBCL
Pidilizumab	Curetech	PD1 mAb	Phase II	after transplant
RG7593	Roche/SGEN	CD22 ADC	Phase II	r/r DLBCL
RG7596	Roche/SGEN	CD79b ADC	Phase II	r/r DLBCL
SAR3419	SNY/IMGN	CD19ADC	Phase II	r/r DLBCL
Bortezomib (Velcade)	Takeda/JNJ	Proteasome inhibitor	Phase II	First line w/ RCHOP
Vorinostat (Zolinza)	MRK	HDAC inhibitor	Phase II	Second line maintenance

Source: clinical trials.gov, BioMedTracker

Selected agents shown

MoA=mechanism of action

Multiple Myeloma (MM) Background



Source: cancer.gov

- Plasma cells develop from B-lymphocytes (B-cells), a type of white blood cell that is made in the bone marrow.
- Normally, when bacteria or viruses enter the body, some of the B-cells will change into plasma cells which make antibodies to fight bacteria and viruses, to stop infection and disease.
- Plasma cell neoplasms are diseases in which abnormal plasma cells or myeloma cells form tumors in the bones or soft tissues of the body. The plasma cells also make an antibody protein, called M protein, that is not needed by the body and does not help fight infection.
- There are several types of plasma cell neoplasms. In multiple myeloma, abnormal plasma cells (myeloma cells) build up in the bone marrow and form tumors in many bones of the body. These tumors may keep the bone marrow from making enough healthy blood cells.
- As the number of myeloma cells increases, fewer red blood cells, white blood cells, and platelets are made. The myeloma cells also damage and weaken the bone.

Significant Unmet Medical Need in Multiple Myeloma (MM) Despite Recent New Drug Approvals

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- Patients without symptoms may not need treatment. When symptoms appear, the treatment of multiple myeloma is typically done in phases, including induction therapy, consolidation chemotherapy, maintenance therapy, and treatment of refractory Multiple Myeloma.
- Despite the increased effectiveness of the first line agents, the majority of patients will eventually relapse and become drug-resistant
- The following targeted agents are approved for MM:
  - Bortezomib (Velcade)
  - Thalidomide (Thalomid)
  - Lenalidomide (Revlimid)
  - Carfilzomib (Kyprolis):
    - ORR: 22.9% in patients refractory to two or more prior therapies
    - Median response duration: 7.8 months
  - Pomalidomide (Pomylast):
    - ORR: 34% in low dose dexamethasone combination in patients relapsed from two or more prior therapies, including Revlimid and Velcade
    - Median response duration: 8.3 months (PFS: 4.6 months)

Late Stage MM Pipeline

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Drug	Company	MoA	Status	Patients
Aplidin	PharmaMar	marine sponge toxin	Phase III	rrMM (>3 lines) Dex combo
Elotuzumab	BMY	CS1 mAb	Phase III	rrMM (>1-3 lines) Lenalidomide/Dex combo
Faridak (panobinostat)	NVS	HDAC inhibitor	Phase III	rrMM (>1 line) Velcade/Dex combo
Masitinib	AB Science S.A.	multi-TKI (PDGFR, FGFR, Kit)	Phase III	rrMM (>1 line) Velcade/Dex combo
MLN9708	Takeda	Proteasome inhibitor	Phase III	1st line MM and rrMM Lenalidomide/Dex combo
ARRY-520	ARRY	KSP inhibitor	Phase III	rrMM (>1 line) Kyprolis combo
Tabalumab	LLY	BAFF mAb	Phase II/III	rrMM (>1-3 lines) Velcade/Dex combo

Source: clinical trials.gov, BioMedTracker

Selected agents shown

KPTI Owns World-Wide Rights to Selinexor

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- We expect solid IP protection for KPTI's drug candidates, if patients are issued. KPTI filed patent applications directed to the composition of matter and methods of use and manufacture for its drug candidates.
  - KPTI has 19 pending patent applications in the US and 4 pending international applications filed under the Patent Cooperation Treaty (PCT) and 15 pending patent applications in foreign jurisdictions
- Selinexor (KPT-330):
  - Composition of matter, methods of use, methods of making Selinexor patent applications
  - Four pending foreign patent applications and one Patent Cooperation Treaty (PCT) application that provides the opportunity for seeking protection in all PCT member states, including the U.S.
  - Expiration in 2032 w/o Hatch-Waxman extension (HWE)
  - Composition of matter and methods of use applications
  - One pending U.S. provisional patent application, one pending non-provisional U.S. patent application and one PCT application that provides the opportunity for seeking protection in all PCT member states
  - Expiration in 2033 w/o HWE
- PAK4 Inhibitors:
  - Composition of matter and methods of use applications
  - Three patent families with eight pending U.S. provisional patent applications. KPTI expects to file non-provisional patent applications claiming the benefit of these provisional applications in late 2013 for one family and the second half of 2014 for the other two families
  - Expiration in 2033 w/o HWE
- One issued U.S. Patent # 8,513,230 expiring March 5, 2031, w/o HWE relating to other XPO1 inhibitors

Acute Myeloid Leukemia (AML) Market Model

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AML (US)	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
AML incidence	15,485	15,718	15,953	16,193	16,436	16,682	16,932	17,186	17,444	17,706	17,971	18,241	18,514	18,792	19,074	19,360	19,651
% growth	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*	*1.5%*
% elderly	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*	*70%*
AML incidence elderly	10,840	11,002	11,167	11,335	11,505	11,677	11,853	12,030	12,211	12,394	12,580	12,769	12,960	13,155	13,352	13,552	13,755
% elderly, refractory to induction	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Elderly, refractory pts	5,420	5,501	5,584	5,667	5,752	5,839	5,926	6,015	6,105	6,197	6,290	6,384	6,480	6,577	6,676	6,776	6,878
% KPT-330 penetration	*5%*	*15%*	*25%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*5%*
Patients treated with KPT-330	271	825	1,396	2,834	2,876	2,919	2,963	3,008	3,053	3,099	3,145	3,192	3,240	3,289	3,338	3,388	344
Cost per course ($)	30,000	30,600	31,212	31,836	32,473	33,122	33,785	34,461	35,150	35,853	36,570	37,301	38,047	38,808	39,584	40,376	41,184
US sales ($MM)	**8**	**25**	**44**	**90**	**93**	**97**	**100**	**104**	**107**	**111**	**115**	**119**	**123**	**128**	**132**	**137**	**14**
EU sales ($MM)	**4**	**19**	**33**	**77**	**93**	**97**	**100**	**104**	**107**	**111**	**115**	**119**	**123**	**128**	**132**	**137**	**14**
US+EU Sales ($MM)	**12**	**44**	**76**	**167**	**187**	**193**	**200**	**207**	**215**	**222**	**230**	**238**	**247**	**255**	**264**	**274**	**28**
POS	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
P/W US+EU sales	**4**	**15**	**27**	**58**	**65**	**68**	**70**	**73**	**75**	**78**	**81**	**83**	**86**	**89**	**92**	**96**	**10**

Source: Leerink Swann Estimates

- Assumptions:
  - 70% patients are elderly (age > 60)
  - 50% of those are refractory to remission induction
  - 50% of those are treated with Selinexor
  - Price: $10k/cycle (similar to Kyprolis, Kinase inhibitors)
  - Assume 3 months of therapy
  - 35% probability of success

Diffuse Large B-Cell Lymphoma (DLBCL) Model

DLBCL	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
NHL incidence	72,572	73,297	74,030	74,771	75,518	76,274	77,036	77,807	78,585	79,371	80,164	80,966	81,776	82,593	83,419	84,254	85,096
% growth	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*
% DLBCL	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*
DLBCL incidence	21,772	21,989	22,209	22,431	22,656	22,882	23,111	23,342	23,575	23,811	24,049	24,290	24,533	24,778	25,026	25,276	25,529
% cured	*50%*	*51%*	*52%*	*53%*	*54%*	*55%*	*56%*	*57%*	*58%*	*59%*	*60%*	*61%*	*62%*	*63%*	*64%*	*65%*	*66%*
Pts with r/r DLBCL treated	10,886	10,775	10,660	10,543	10,422	10,297	10,169	10,037	9,902	9,763	9,620	9,473	9,322	9,168	9,009	8,847	8,680
% penetration	*5%*	*15%*	*25%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*5%*
Patients treated with KPT-330	544	1,616	2,665	5,271	5,211	5,148	5,084	5,019	4,951	4,881	4,810	4,737	4,661	4,584	4,505	4,423	434
Cost per course ($)	50,000	51,000	52,020	53,060	54,122	55,204	56,308	57,434	58,583	59,755	60,950	62,169	63,412	64,680	65,974	67,293	68,639
US sales ($MM)	**27**	**82**	**139**	**280**	**282**	**284**	**286**	**288**	**290**	**292**	**293**	**294**	**296**	**296**	**297**	**298**	**30**
EU sales ($MM)	**14**	**62**	**104**	**238**	**282**	**284**	**286**	**288**	**290**	**292**	**293**	**294**	**296**	**296**	**297**	**298**	**30**
US+EU Sales ($MM)	**41**	**144**	**243**	**517**	**564**	**568**	**573**	**576**	**580**	**583**	**586**	**589**	**591**	**593**	**594**	**595**	**60**
%POS	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
P/W US+EU sales	**14**	**50**	**85**	**181**	**197**	**199**	**200**	**202**	**203**	**204**	**205**	**206**	**207**	**208**	**208**	**208**	**21**

Source: Leerink Swann Estimates

- Assumptions:
  - r/rDLBCL annual incidence of ~11,000 (>10,000 deaths) in 2017;
  - Assume improving cure rate in front-line therapy
  - No cures in first line of chemotherapy for r/rDLBCL
  - Assume 50% Selinexor penetration in 2nd or later line r/rDLBCL
  - Price: $10k/cycle (similar to Kyprolis, kinase inhibitors)
  - Assume 5 months of Selinexor therapy
  - 35% probability of success

Multiple Myeloma (MM) Market Model

MM	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
MM incidence	23,257	23,490	23,725	23,962	24,202	24,444	24,688	24,935	25,185	25,436	25,691	25,948	26,207	26,469	26,734	27,001	27,271
Growth	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*
% progress to rr	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*	*49%*
rr/MM incidence	11,447	11,561	11,677	11,793	11,911	12,031	12,151	12,272	12,395	12,519	12,644	12,771	12,898	13,027	13,158	13,289	13,422
% penetration		*3%*	*15%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*30%*	*3%*
Patients treated with KPT-330		289	1,752	3,538	3,573	3,609	3,645	3,682	3,719	3,756	3,793	3,831	3,870	3,908	3,947	3,987	403
Cost per course ($)		51,000	52,020	53,060	54,122	55,204	56,308	57,434	58,583	59,755	60,950	62,169	63,412	64,680	65,974	67,293	68,639
US sales ($MM)		**15**	**91**	**188**	**193**	**199**	**205**	**211**	**218**	**224**	**231**	**238**	**245**	**253**	**260**	**268**	**28**
EU sales ($MM)		**11**	**68**	**160**	**193**	**199**	**205**	**211**	**218**	**224**	**231**	**238**	**245**	**253**	**260**	**268**	**28**
US+EU Sales ($MM)		**26**	**159**	**347**	**387**	**398**	**411**	**423**	**436**	**449**	**462**	**476**	**491**	**506**	**521**	**537**	**55**
%POS		*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
P/W US+EU sales		**9**	**56**	**122**	**135**	**139**	**144**	**148**	**152**	**157**	**162**	**167**	**172**	**177**	**182**	**188**	**19**

Source: Leerink Swann Estimates

- Assumptions:
  - r/rMM annual incidence of ~11,000 (>10,000 deaths) in 2017
  - All patients fail 1-2 lines of prior therapy for r/rMM
  - Assume 30% Selinexor patient share in >3rd line r/rMM
  - Price: $10k/cycle (similar to Kyprolis, kinase inhibitors)
  - Assume average duration of therapy of 5 months (single agent and/or combination)
  - 35% probability of success

P&L and BS

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KPTI P&L (in $MM)	2011	2012	1H13	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Contract and grant revenue	0.2	0.6	0.4	0.2	0.2	0.8	1.0	1.0	1.0	-	-	-	-
Selinexor US sales (p/w)	-	-	-	-	-	-	-	-	-	12.4	42.8	95.7	195.2
Selinexor EU royalty (p/w)	-	-	-	-	-	-	-	-	-	1.5	8.0	17.9	41.5
Total revenue	**0.2**	**0.6**	**0.4**	**0.2**	**0.2**	**0.8**	**1.0**	**1.0**	**1.0**	**13.9**	**50.9**	**113.6**	**236.7**
COGS	-	-	-	-	-	-	-	-	-	1.0	3.4	7.7	15.6
R&D expense	8.6	14.1	11.0	6.0	8.0	25.0	35.3	40.3	47.0	52.0	40.0	4.0	35.5
SG&A expense	1.8	2.4	1.8	1.0	2.0	4.8	5.3	5.8	6.4	21.0	23.1	24.3	39.0
Total operating expenses	10.5	16.5	12.8	7.0	10.0	29.8	40.6	46.1	53.4	74.0	66.5	35.9	90.1
Operating income (loss)	(10.3)	(15.9)	(12.5)	(6.8)	(9.8)	(29.1)	(39.6)	(45.1)	(52.4)	(60.1)	(15.6)	77.7	146.5
Total other income (expense)	-	0.0	0.0	-	-	0.0	-	-	-	-	-	-	-
Income Tax expense	-	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss)	**(10.3)**	**(15.9)**	**(12.5)**	**(6.8)**	**(9.8)**	**(29.1)**	**(39.6)**	**(45.1)**	**(52.4)**	**(60.1)**	**(15.6)**	**77.7**	**146.5**
Common shares outstanding (basic)	1.1	1.8	2.3	2.8	28.7	9.0	28.7	28.7	28.7	28.7	28.7	28.7	28.7
Common shares outstanding (Pro Forma)		5.8	9.8	21.9	28.7	17.6							
EPS (basic)	(9.34)	(8.95)	(5.39)	(2.42)	(0.34)	(3.22)	(1.38)	(1.57)	(1.82)	(2.09)	(0.54)	2.70	5.10
EPS (Pro Forma)		(2.74)	(1.27)	(0.31)	(0.34)	(1.66)							

KPTI BS & CFS (in $MM)	2011	2012	1H13	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Cash & equivalents	6.5	0.4	17.7	55.2	144.5	144.5	108.2	66.8	18.6	164.4	153.8	233.7	386.2
Debt	-	-	-	-	-	-	-	-	-	-	-	-	-

Change in Cash	3.1	(6.1)	17.3	37.6	89.3	144.1	(36.3)	(41.4)	(48.1)	145.8	(10.6)	80.0	152.5
Cash from operations	**(8.5)**	**(15.5)**	**(11.3)**	**(6.5)**	**(9.4)**	**(27.1)**	**(36.3)**	**(41.4)**	**(48.1)**	**(54.2)**	**(10.6)**	**80.0**	**152.5**
Net income (loss)	(10.3)	(15.9)	(12.5)	(6.8)	(9.8)	(29.1)	(39.6)	(45.1)	(52.4)	(60.1)	(15.6)	77.7	146.5
Share based comp	0.0	0.7	0.4	0.3	0.4	1.1	3.2	3.7	4.3	5.8	5.0	2.3	6.0
D&A	0.1	0.1	0.1	0.1	0.1	0.2	-	-	-	-	-	-	-
Other (Change in WC)	1.7	(0.4)	0.7	-	-	0.7	-	-	-	-	-	-	-
Cash from investing	**(0.4)**	**(0.1)**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**
CapEx	(0.4)	(0.1)	-	-	-	-	-	-	-	-	-	-	-
Acquisitions	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-	-
Cash from financing	**12.0**	**9.5**	**28.6**	**44.0**	**98.6**	**171.2**	**-**	**-**	**-**	**200.0**	**-**	**-**	**-**
Equity issue (buyback)	12.0	9.5	28.6	44.0	98.6	171.2	-	-	-	200.0	-	-	-
Debt issue (principal payment)	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-	-

Source: SEC 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.

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			IB Serv./Past 12 Mos.	
Rating	Count	Percent	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

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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.

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Leerink Swann LLC Equity Research

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	**Alice C. Avanian, CFA**	(617) 918-4544	alice.avanian@leerink.com
Healthcare Strategy	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Biotechnology	**Howard Liang, Ph.D.**	(617) 918-4857	howard.liang@leerink.com
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink.com
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Jonathan Chang, Ph.D	(617) 918-4015	jonathan.chang@leerink.com
	Irene Lau	(415) 905-7256	irene.lau@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink.com
Life Science Tools and Diagnostics	**Dan Leonard**	(212) 277-6116	dan.leonard@leerink.com
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink.com
Pharmaceuticals/Major	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink.com
	Ario Arabi	(617) 918-4568	ario.arabi@leerink.com
Specialty Pharmaceuticals, Generics	**Jason M. Gerberry, JD**	(617) 918-4549	jason.gerberry@leerink.com
	Christopher W. Kuehnle, JD	(617) 918-4851	chris.kuehnle@leerink.com
Medical Devices, Cardiology & Orthopedics	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink.com
	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Robert Marcus, CFA	(212) 277-6084	robert.marcus@leerink.com
	Ravi Misra	(212) 277-6049	ravi.misra@leerink.com
Healthcare Services	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink.com
Healthcare Technology & Distribution	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink.com
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Sr. Editor/Supervisory Analyst	**Mary Ellen Eagan, CFA**	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com

New York
299 Park Avenue, 21st floor
New York, NY 10171
(888) 778-1653

Boston
One Federal Street, 37th Floor
Boston, MA 02110
(800) 808-7525

San Francisco
201 Spear Street, 16th Floor
San Francisco, CA 94105
(800) 778-1164