August 19, 2013

OUTPERFORM

Reason for report:

INITIATION

Howard Liang, Ph.D.
(617) 918-4857
Howard.Liang@Leerink.com

Gena Wang, Ph.D.
(212) 277-6073
Gena.Wang@Leerink.com

LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

ONCONOVA THERAPEUTICS, INC.

A Late-Stage Story that Goes Beyond the Lead Indication; Initiate at OP

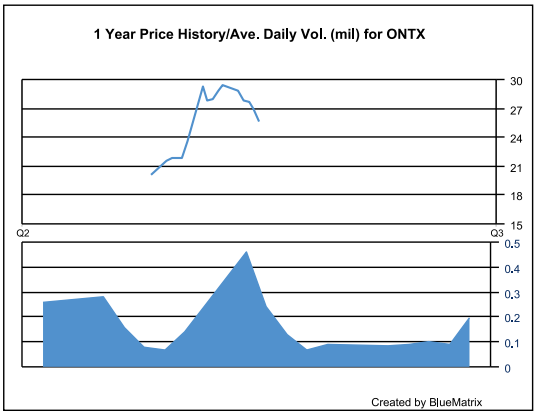
• **Bottom Line:** We are initiating coverage of ONTX with an Outperform rating and a $37 valuation based on DCF analysis. ONTX is a late-stage story with robust clinical news flow in the next 6-9 months including pivotal Phase III (ONTIME) data of IV rigosertib in higher-risk, second-line myelodysplastic syndrome (MDS).

• **Although the Phase III ONTIME readout is clearly a binary event and there is considerable risk, we believe the risk/reward remains favorable.** In Phase II trials of higher-risk MDS patients, rigosertib demonstrated good bone marrow (BM) response but more modest hematological improvements. Although we are not aware of data clearly showing a correlation of BM response and survival, we believe the clear correlation of BM blast percentage and survival in historical data is supportive. Another positive consideration is that rigosertib is being compared to best supportive care, and based on our analysis, we do not believe low-dose ara-C will have a meaningful contribution. Although the study is not large for a survival study (270 patients targeted, nearly 300 enrolled), the number of events used for analysis (at least 223) looks reasonable in comparison to the successful AZA-001 study in the first-line setting (195 events) even considering the 2:1 randomization. MEDACorp key opinion leaders (KOLs) generally peg the probability of success to be at least 50%, and as high as 80%.

• **We believe rigosertib's opportunity in lower-risk MDS should be viewed independent of the outcome of ONTIME.** In contrast to the higher-risk setting, more robust hematological response such as transfusion independence was seen. MEDACorp KOLs view rigosertib data in lower-risk patients to be even stronger than in higher-risk MDS. We believe upcoming ASH data (Dec. 7-10) could further solidify the profile. We believe there are limited expectations for the pancreatic cancer interim Phase II/III readout in 4Q:13/1Q:14, therefore the risk/reward is favorable. We find signals of single-agent activity in head and neck cancer intriguing.

• **ONTX is one of the minority of biotech companies that have been able to maintain full US rights to their lead compound near the finish line.** If the Phase III is positive, we believe rigosertib will be an attractive asset to potential acquirers due to retained economics as well as a pipeline of additional indications.

Key Stats:	**(OTC Un:ONTX)**
S&P 600 Health Care Index:	**1,087.73**
Price:	**$25.69**
Price Target:	$37.00
Methodology:	DCF analysis
52 Week High:	$30.00
52 Week Low:	$15.00
Shares Outstanding (mil):	21.4
Market Capitalization (mil):	$549.8
Book Value/Share:	$4.53
Cash Per Share:	$5.14
Dividend (ann):	$0.00
Dividend Yield:	0.0%



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2012A	--	--	--	--	$46.2	--	--	--	--	($2.67)	NM
2013E	$1.1A	$1.1	$1.1	$1.1	$4.5	($1.03)A	($0.79)	($0.71)	($0.72)	($3.18)	NM
2014E	--	--	--	--	$2.1	--	--	--	--	($3.34)	NM

Source: Company Information and Leerink Swann LLC Research
Revenues in millions; EPS are GAAP.

LEERINK SWANN

The Healthcare Investment Bank™

Onconova Therapeutics

Howard Liang, Ph.D.
Managing Director, Biotechnology
(617) 918-4857
howard.liang@leerink.com

Gena Wang, Ph.D.
Biotechnology Associate
(212) 277-6073
gena.wang@leerink.com

Onconova Overview

LEERINK SWANN

- Onconova's lead candidate, IV rigosertib, is in Phase III for second-line, higher-risk MDS with data expected 4Q:13/1Q:14.
  - Oral rigosertib has shown encouraging efficacy in Phase II of lower-risk MDS for which the company is preparing to start a Phase III.
  - Rigosertib is in a Phase II/III for first-line pancreatic cancer (IV formulation, in combination with Gemzar) and Phase II in refractory head and neck cancer (oral formulation as monotherapy).
  - PLK1/PI3K dual pathway inhibitor
  - US commercial rights retained by Onconova
  - Partnerships with Baxter (Europe), Symbio (Korea, Japan)
  - Composition of matter patents extend to 2026
- Key financials: 21.4M shares outstanding, 24.2M fully diluted shares; ~$110M cash ($5.14 per share).

Investment Thesis

LEERINK SWANN

- ONTX is a late-stage story with robust clinical news flow in the next 6-9 months including pivotal Phase III (ONTIME) data of IV rigosertib in higher-risk second-line myelodysplastic syndrome (MDS).

- Although the Phase III ONTIME readout is clearly a binary event and there is considerable risk, we believe risk/reward remains favorable. In Phase II trials of higher-risk MDS patients, rigosertib demonstrated good bone marrow (BM) response but more modest hematological improvements. Although we are not aware of data clearly showing a correlation of BM response and survival, we believe the clear correlation of BM blast percentage and survival in historical data is supportive. Another positive consideration is that rigosertib is being compared to best supportive care, and based on our analysis, we do not believe low-dose ara-C will have a meaningful contribution. Although the study is not large for a survival study (270 patients targeted, nearly 300 enrolled), the number of events used for analysis (at least 223) looks reasonable in comparison to the successful AZA-001 study in the first-line setting (195 events). MEDACorp key opinion leaders generally peg the probability of success to be at least 50%, and as high as 80%.

Investment Thesis

LEERINK SWANN

- We believe rigosertib's opportunity in lower-risk MDS should be viewed independent of the outcome of ONTIME. In contrast to the higher-risk setting, more robust hematological response such as transfusion independence was seen. MEDACorp KOLs view rigosertib data in lower-risk patients to be even stronger than in higher-risk MDS. We believe upcoming ASH data could further solidify the profile. We believe there is limited expectations for pancreatic cancer interim Phase II/III readout in 4Q:13/1Q:14, therefore risk/ reward is favorable. We find signals of single-agent activity in head and neck cancer intriguing.
- ONTX is one of the minority of biotech companies that have been able to maintain full US rights to their lead compound near the finish line. If the Phase III is positive, we believe rigosertib will be an attractive asset to potential acquirers due to retained economics as well as a pipeline of additional indications.

Valuation

LEERINK SWANN

- We value ONTX at $37 per share based on DCF methodology.
- We assume rigosertib launches in high-risk MDS in 2015 and in low-risk MDS in 2017. Our royalty assumption is 12-19% for ex-US sales.
- Our projection for peak penetration is 30% for high-risk MDS and 25% for low-risk MDS. Our projection for probability-weighted (60% for high-risk and 50% for low-risk MDS) sales of rigosertib reaches $394M for US and ex-US royalties reach $75M by 2029, three years after patent expiration.
- We use a discount rate of 10%, which we believe is appropriate given the probability-weighted sales projection.

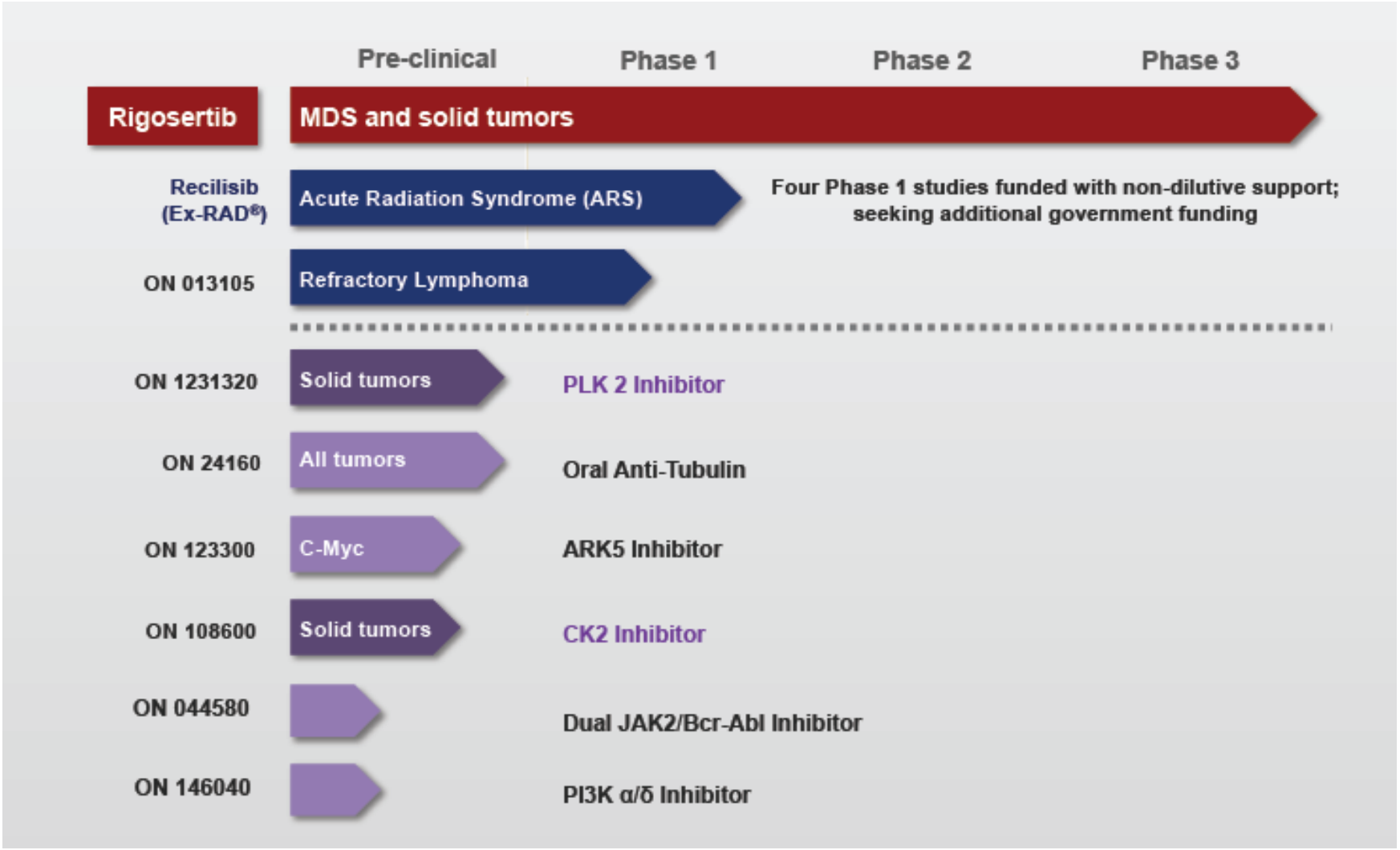
Risks to Valuation

LEERINK SWANN

- Binary clinical risk with Phase III readout of rigosertib in higher-risk MDS. Although Phase II demonstrated bone marrow response, full partial or complete responses by traditional definition were limited, and it is not clear that bone marrow response would predict survival benefit. In addition, although the survival observed in the Phase II compared favorably to historical control, such comparisons are difficult and have significant caveats.
- Commercial and execution risks as a small company. The current continuous infusion dosing regimen for the IV formulation may present a challenge.
- Financing risk – ONTX has estimated pro forma cash of ~$110M, which we estimate to be sufficient to fund operations through the end of 2014, and the company may have additional financing needs before turning cash flow positive.

Broad Clinical and Pre-Clinical Pipeline

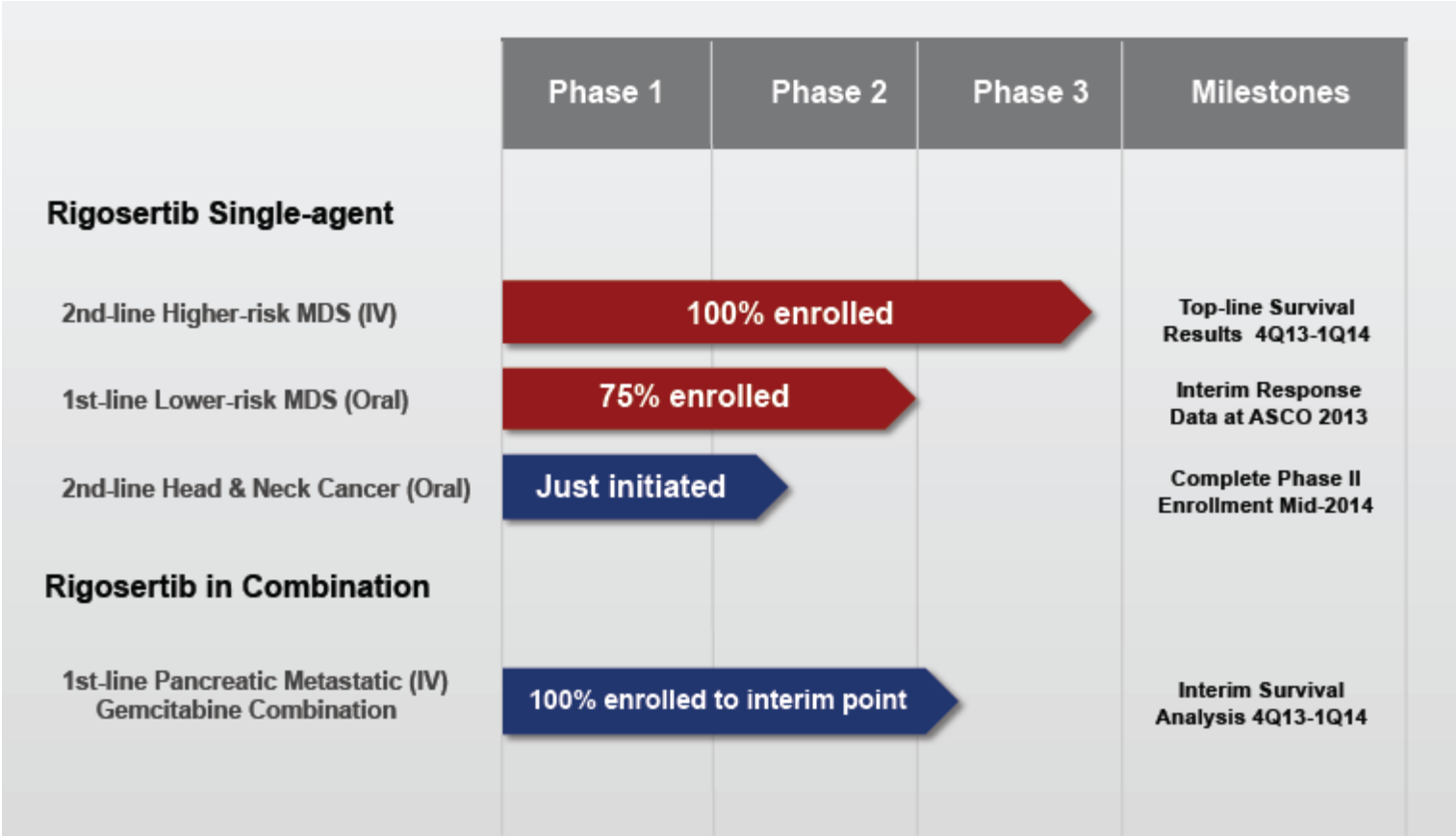
LEERINK SWANN



Source: Company Reports

Phase III Data Readout by Early 2014

LEERINK SWANN



Source: Company Reports

Key Expected Events – Data News Flow

LEERINK SWANN

Event	Time	Comment
Rigosertib		
2nd-line, higher-risk MDS Phase III top-line results	4Q:13 - 1Q:14	270+ patients completed enrollment during Dec. 2010 - May 2013. Data more likely in 1Q:14.
1st -line, lower-risk MDS Phase II update	ASH (Dec. 7-10, New Orleans)	Data on ~60 patients expected; data on 34 patients presented at ASCO 2013
1st-line pancreatic cancer Phase II/III interim futility/survival analysis	4Q:13 - 1Q:14	Data more likely in 4Q:13

Source: Company reports and Leerink Swann

LEERINK SWANN

KEY INVESTMENT CONSIDERATIONS

LEERINK SWANN

PHASE III READOUT IN HIGHER-RISK MDS LIKELY THE MOST IMPORTANT NEAR-TERM EVENT

ONTIME, Phase III of Rigosertib in Higher-risk MDS Patients

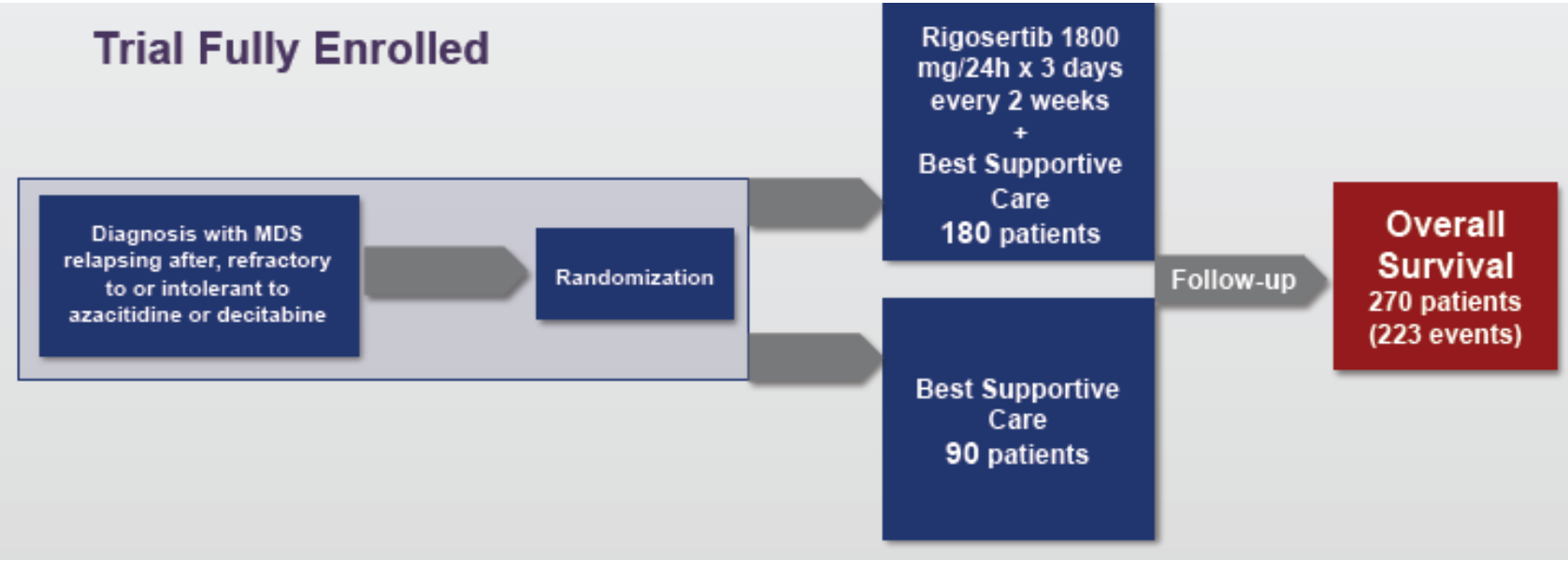
LEERINK SWANN

- Continuous infusion rigosertib for 3 days (wearable pump) every 2 weeks for 16 weeks (every 4 weeks thereafter) + BSC vs. BSC (low-dose ara-C allowed).
- Higher-risk MDS patients (RAEB-1 and RAEB-2 under WHO and RAEB-t and CMML under FAB classification) who relapsed after, were refractory to or intolerant to hypomethylating agents (Vidaza or Dacogen).
  - Designed to enroll 270 patients, 2:1 randomization – total # of patients likely approaching 300
  - Enrollment started in Dec. 2010 and 270th patient enrolled May 7, 2013
  - Majority (~2/3) of patients enrolled in the US; remainder in Western Europe
  - Intolerant patients <5%, relapsed or refractory patients balanced; ~10% failed both Vidaza and Dacogen
- Primary endpoint = overall survival (OS)
- One efficacy analysis after 223 events (83% of patients) has 90% power to detect a 50% improvement in OS (26 vs. 39 weeks).

MDS: myelodysplastic syndrome; BSC: best supportive care; RAEB: refractory anemia with excess blasts; RAEB-t: refractory anemia with excess blasts in transformation; CMML: chronic myelomonocytic leukemia; WHO: World Health Organization; FAB: French American British

Source: Company Reports; clinicaltrials.gov

Rigosertib Phase III Design Under SPA with the FDA



- Secondary efficacy outcomes:
  - Overall response, complete bone marrow response, hematological improvements (according to IWG 2006 criteria)
  - Quality of life (based on EORTC questionnaire)
  - Changes in aneuploidy
  - Transition time to AML

SPA: special protocol assessment; IWG: International Working Group; EORTC: European Organization for Research and Treatment of Cancer; AML: acute myeloid leukemia

Source: Company Reports

Control Arm in ONTIME: Low-dose Ara-C Is Allowed

LEERINK SWANN

- Trial description in clinicaltrials.gov states:
  - Crossover of BSC patients to ON 01910.Na after progression will not be allowed. However, **patients in the BSC-only group will be allowed, as medically justified, access to low-dose cytarabine 20 mg/m2 subcutaneously (SC) once daily for the first consecutive 14 days of each 28-day cycle, up to 4 cycles, until progression or unacceptable toxicity develops.** Low-dose cytarabine will be delayed as needed until recovery of blood counts. All study participants will be allowed, as medically justified, access to RBC and platelet transfusions and to growth factors (erythropoietin, Filgrastim [G-CSF]). Hydroxyurea will be allowed to manage blastic crisis with hyperleukocytosis when patients transition to leukemia.
- Based on MEDACorp key opinion leader feedback, low-dose ara-C is not commonly used in the US in MDS patients.
- Majority of trial population was enrolled in US (see next page).
- Management confirms that the percent of patients in the control arm receiving ara-C is very low. In addition, low-dose ara-C is allowed in the treatment arm as well.
- As we further discuss later, we do not believe low-dose ara-C use will meaningfully impact survival.

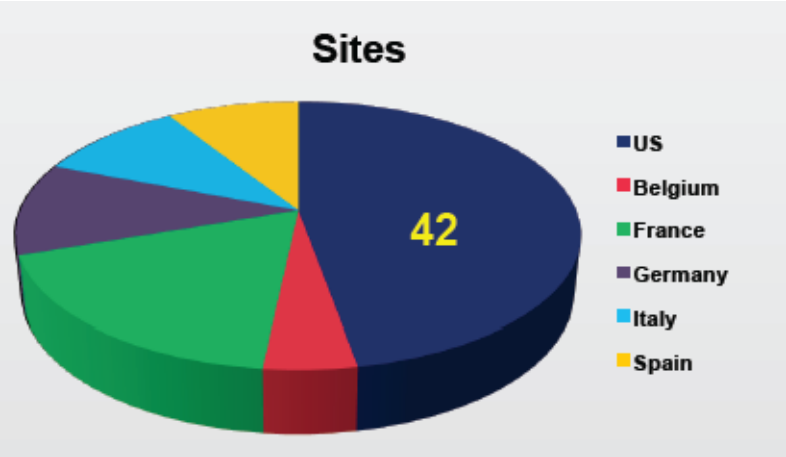
BSC: best supportive care; ON01910.Na = rigosertib

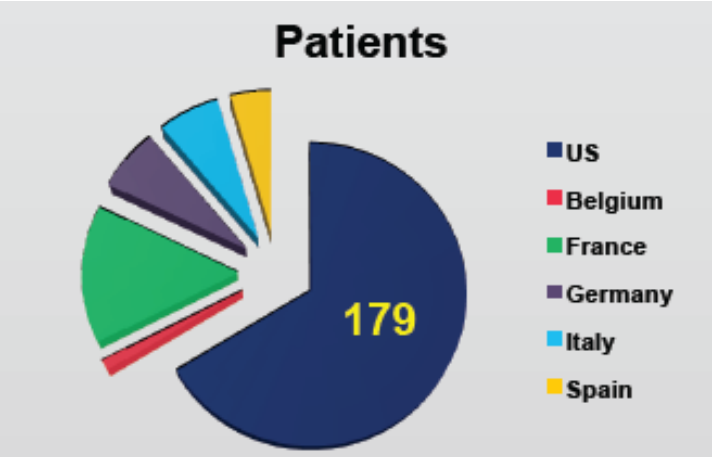
Source: Company Reports; clinicaltrials.gov

Approximately 2/3 of Phase III Population Was Enrolled in the US with the Remainder in Western Europe

LEERINK SWANN

- Geographic breakdown of clinical sites (89 total) and patients (270+) in ONTIME





Top Enrolling Sites for Pivotal MDS Trial

Country	Site	City	Investigator	Patients
USA	MD Anderson	Houston	Garcia-Manero	39
USA	Mayo Clinic	Rochester	Al-Kalli	14
France	Avicene	Paris	Fenaux	12
USA	Univ. Maryland	Baltimore	Bauer	10
USA	Cleveland Clinic	Cleveland	Sekeres	9

- Enrollment hurdles:
  - Slow approval in EU
  - Randomization to BSC

BSC: best supportive care;

Source: Company Reports

DSMB Review of Study Has Revealed No Safety Issues

LEERINK SWANN

Date of DSMC	Patients Analyzed	Patients Accrued	Decision
February 2012	63	73	Continue w/o modification
August 2012	142	153	Continue w/o modification
March 2013	228	244	Continue w/o modification
TBD	270+	270+	Final review analysis

Drug Safety Monitoring Committee comprised of independent experts

- Only safety analysis at interim looks

Source: Company Reports

LEERINK SWANN

MAIN RESPONSE SEEN WITH RIGOSERTIB IS BONE MARROW RESPONSE – CAN IT TRANSLATE INTO SURVIVAL BENEFIT?

Phase I/II Trials of IV Rigosertib in MDS

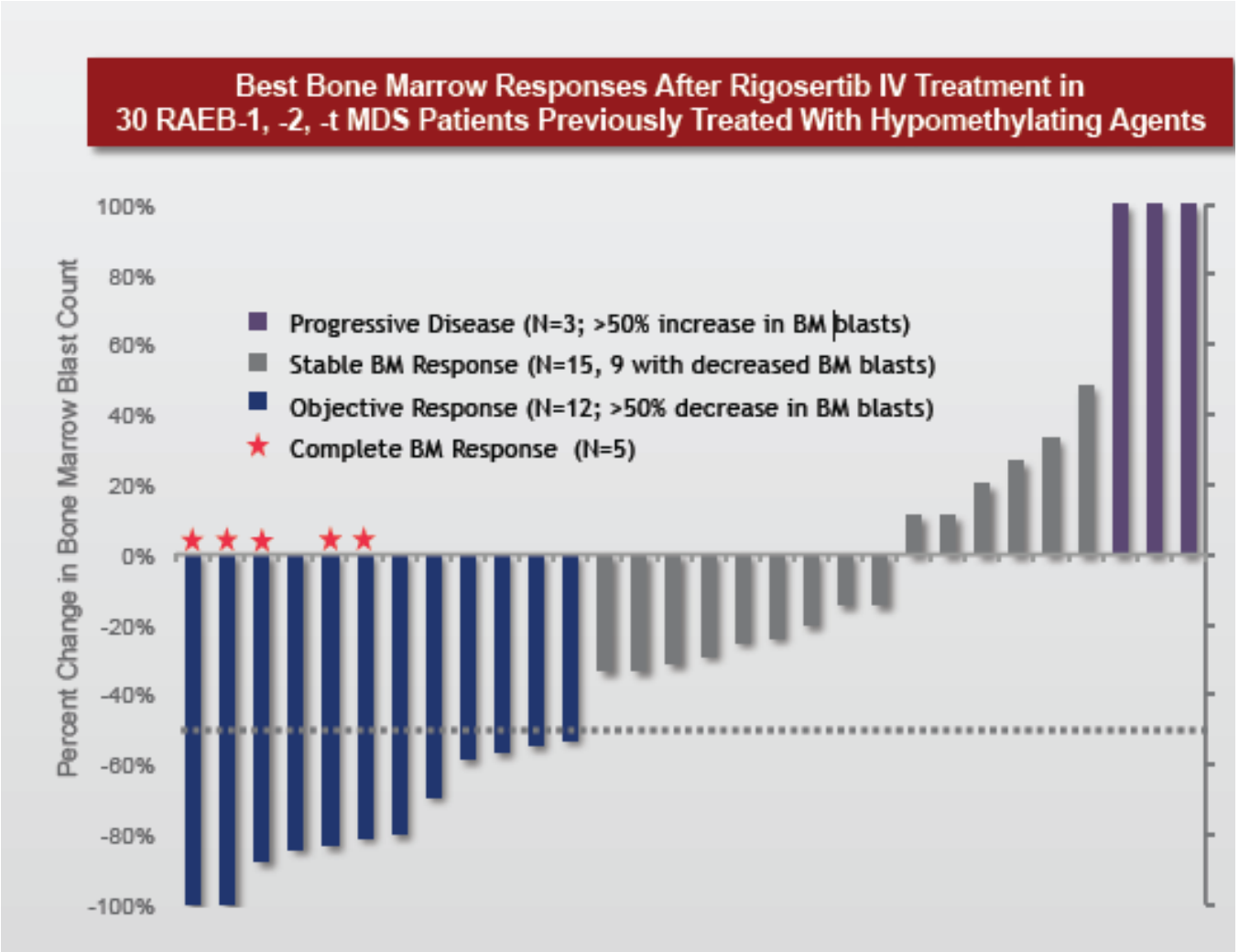
LEERINK SWANN

	Study 07-H-0225 (E Sloand/M Olnes)	Study 04-05 (L Silverman)	Study 04-15 (A Raza)	Study 04-17 (P Greenberg)
Population	**Trisomy 8 Refractory AML or MDS**	**MDS/AML Refractory to Azacytidine/ decitabine**	**All Comers MDS/AML**	**Trisomy 8 and Int-2/High Risk Refractory MDS**
Dosing Regimen	800 mg/m^2 given 3-5 days Q2W	650-1700 mg/m^2 given 3-6 days Q2W	800-1500 mg/m^2 given 2 days QW for 3 of 4 weeks; amended to 1800 mg given 3 days Q2W	800 mg/m^2 given 2 days QW for 3 of 4 weeks; amended to 1800 mg given 3 days Q2W
Total Patients (MDS &AML) /MDS patients	14/12	14/7	35/28	13/13
Q2W = every other week; QW = weekly.				

Source: Raza et al., ASH 2011 presentation

Rigosertib Demonstrated Bone Marrow Response in Patients Failing Vidaza/Dacogen

LEERINK SWANN



MDS: myelodysplastic syndrome; RAEB: refractory anemia with excess blasts; RAEB-t: refractory anemia with excess blasts in transformation; BM: bone marrow

Source: Company Reports

In the Pretreated Higher-risk MDS Patients, Rigosertib Led to More Bone Marrow Responses than Hematological Improvements

LEERINK SWANN

- In 39 RAEB-1,-2,-t patients previously treated with hypomethylating agents, rigosertib showed:
  - >= 50% bone marrow blast decrease from baseline in 33% (13/39) patients
  - 5 hematological improvements
  - Complete bone marrow response in 5 patients (including 1 with hematological improvement)
  - Of 15 patients previously treated with hypomethylating agents who received the dosing regimen used in the Phase III trial (1,800 mg/day for 3 days, every 2 weeks), 47% had >= 50% bone marrow response
- An IWG response requires both a bone marrow response as well as normalization of peripheral blood counts (see next slide).
- In this population of patients, rigosertib's impact appears mainly on the bone marrow, although there is some hematological improvement

MDS: myelodysplastic syndrome; RAEB: refractory anemia with excess blasts; RAEB-t: refractory anemia with excess blasts in transformation; IWG: International Working Group

Source: Raza et al., ASH 2011 presentation

International Working Group (IWG) Response Definition

LEERINK SWANN

Complete remission	Bone marrow: ≤ 5% myeloblasts with normal maturation of all cell lines*
	Persistent dysplasia will be noted*†
	Peripheral blood‡
	Hgb ≥ 11 g/dL
	Platelets ≥ 100 × 10^9/L
	Neutrophils ≥ 1.0 × 10^9/L†
	Blasts 0%
Partial remission	All CR criteria if abnormal before treatment except:
	Bone marrow blasts decreased by ≥ 50% over pretreatment but still > 5%
	Cellularity and morphology not relevant
Marrow CR†	Bone marrow: ≤ 5% myeloblasts and decrease by ≥ 50% over pretreatment†
	Peripheral blood: if HI responses, they will be noted in addition to marrow CR†
Stable disease	Failure to achieve at least PR, but no evidence of progression for > 8 wks
Failure	Death during treatment or disease progression characterized by worsening of cytopenias, increase in percentage of bone marrow blasts, or progression to a more advanced MDS FAB subtype than pretreatment
Relapse after CR or PR	At least 1 of the following:
	Return to pretreatment bone marrow blast percentage
	Decrement of ≥ 50% from maximum remission/response levels in granulocytes or platelets
	Reduction in Hgb concentration by ≥ 1.5 g/dL or transfusion dependence
Disease progression	For patients with:
	Less than 5% blasts: ≥ 50% increase in blasts to > 5% blasts
	5%-10% blasts: ≥ 50% increase to > 10% blasts
	10%-20% blasts: ≥ 50% increase to > 20% blasts
	20%-30% blasts: ≥ 50% increase to > 30% blasts
	Any of the following:
	At least 50% decrement from maximum remission/response in granulocytes or platelets
	Reduction in Hgb by ≥ 2 g/dL
	Transfusion dependence

Source: Cheson et al., 2006

A Key Question Is Whether Bone Marrow Response Will Lead to Survival Benefit

LEERINK SWANN

- We are not aware of published analyses on the association of bone response and survival from randomized studies
- AZA-001 (Vidaza vs. conventional care) is the only randomized Phase III trial in MDS that has demonstrated a survival benefit. An analysis of relationship between response and survival (Gore et al.) showed:
  - CR or PR are not obligate responses for improved OS with Vidaza.
  - Hematological improvement only also appears to be associated with OS benefit.
  - Interestingly, the analysis suggested that hematologic responses with Vidaza are qualitatively different from those with conventional care.
  - There was no analysis on the relationship between bone marrow response and OS.

MDS: myelodysplastic syndrome; CR: complete response; PR: partial response; OS: overall survival

Source: Gore et al., Haematologica, 2013

Bone Marrow Blast Level Is a Clear Prognostic Factor for Survival

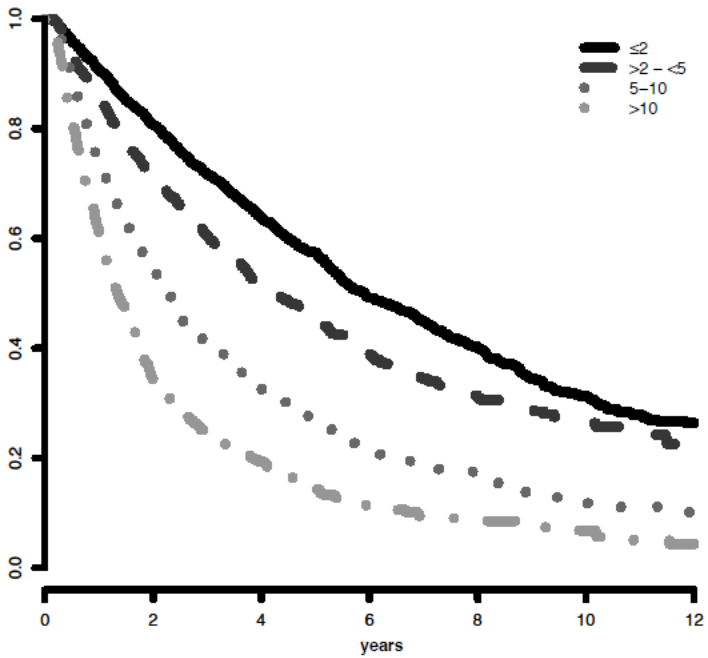
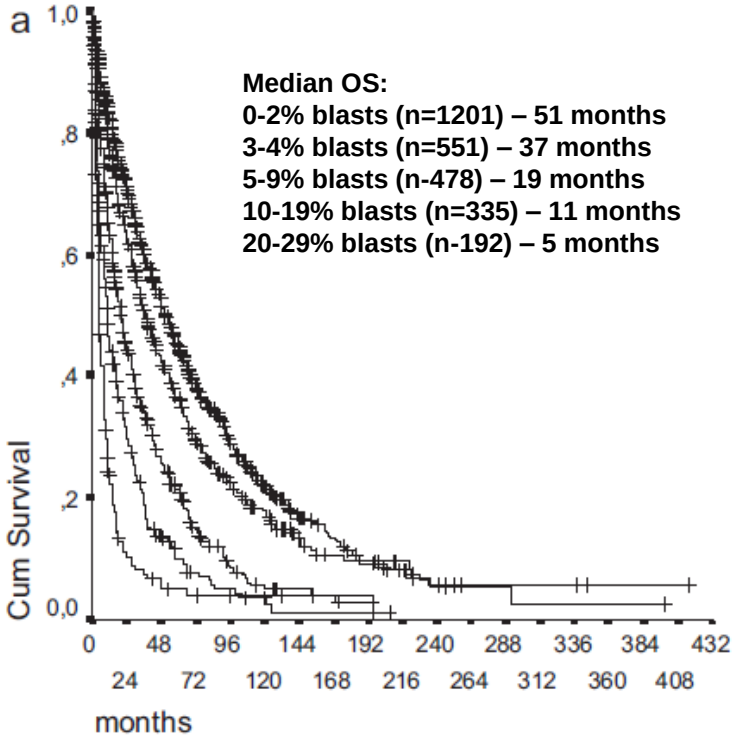


Figure 1. IWG-PM patients marrow blast subgroups. Impact on survival. Survival related to MDS patients' individual marrow blast percent categories (Kaplan-Meier curves, Dxy 0.3, $P < .001$). The number of patients in each category and their proportional representation are shown in Table 1.



Median OS:
0-2% blasts (n=1201) – 51 months
3-4% blasts (n=551) – 37 months
5-9% blasts (n-478) – 19 months
10-19% blasts (n=335) – 11 months
20-29% blasts (n-192) – 5 months

While this does not prove that inducing a bone marrow response will lead to improved OS, it does show that lower blast percentages at baseline correlate positively with a better prognosis.

Source: Greenberg et al., 2013; Germing et al., 2012

Bone Marrow Blast Level Also Correlates with Risk of AML Transformation

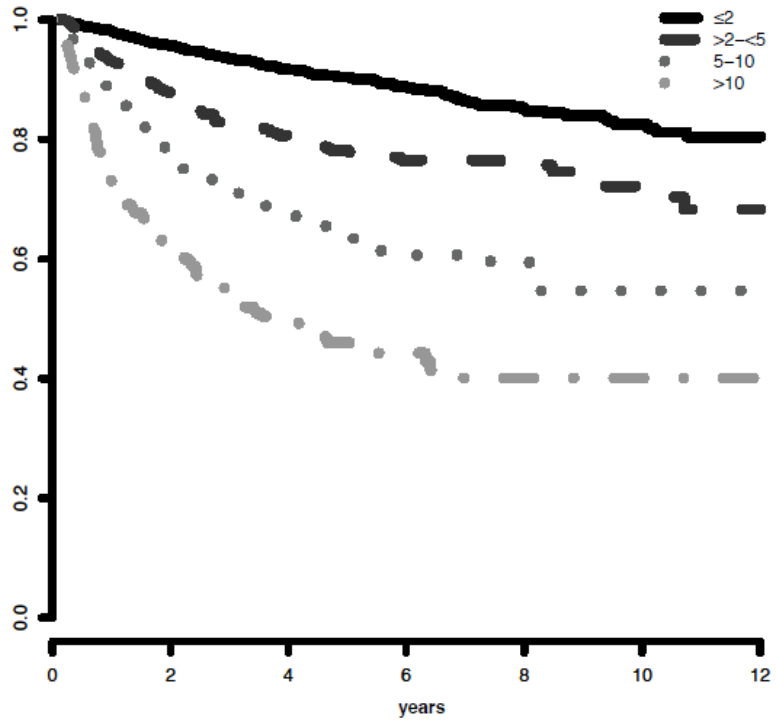
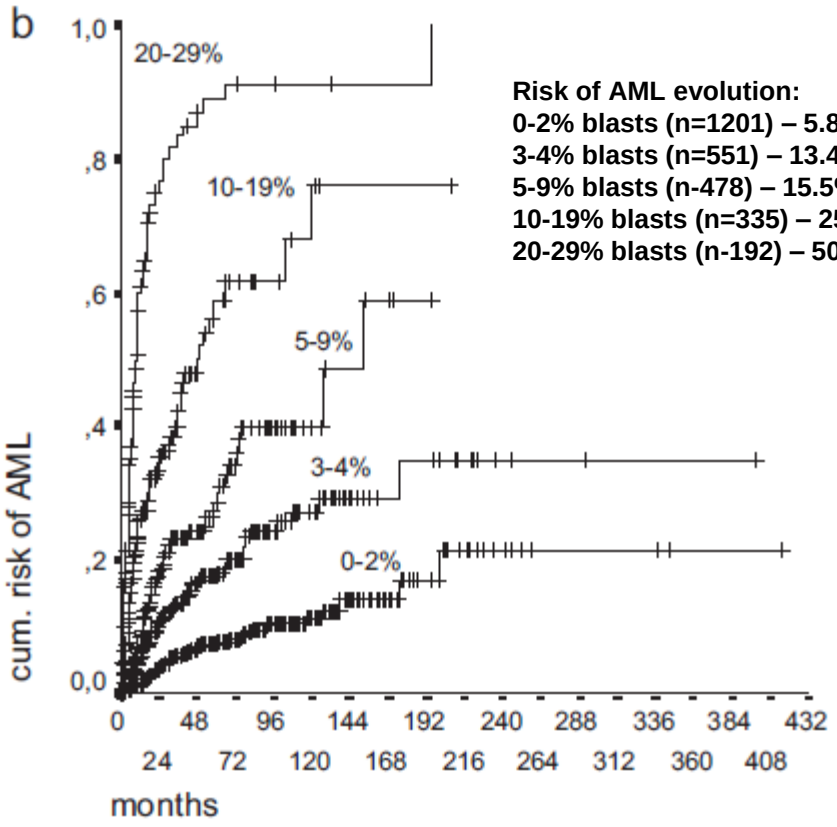


Figure 2. IWG-PM patients marrow blast subgroups: Impact on AML evolution. Progression to AML related to MDS patients' individual marrow blast percent categories (Kaplan-Meier curves, Dxy 0.47, $P < .001$). The number of patients in each category and their proportional representation are shown in Table 1.



Risk of AML evolution:
0-2% blasts (n=1201) – 5.8%
3-4% blasts (n=551) – 13.4%
5-9% blasts (n-478) – 15.5%
10-19% blasts (n=335) – 25.9%
20-29% blasts (n-192) – 50.1%

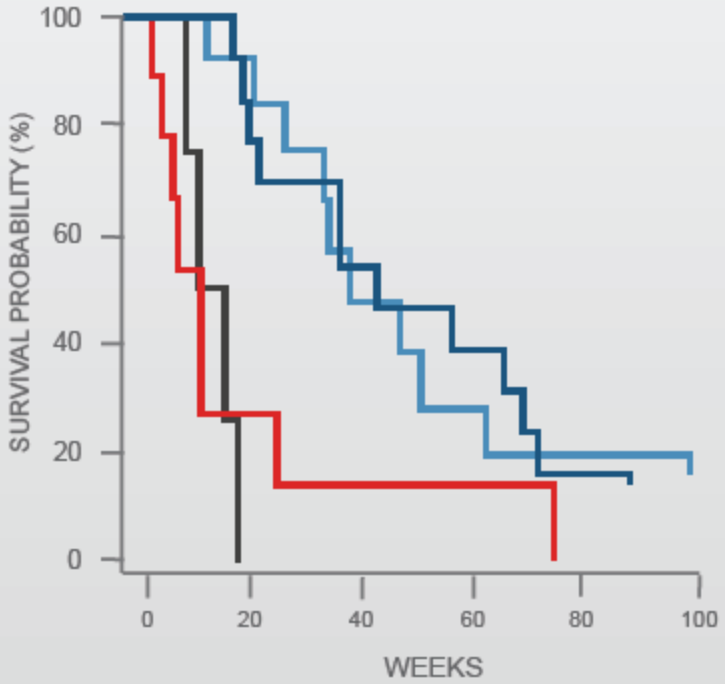
Bone marrow blast percentages correlate with a lower risk of AML transformation, which is a major cause of death of higher-risk MDS patients, according to MEDACorp KOLs

Source: Greenberg et al., 2013; Germing et al., 2012

In Rigosertib's Own Dataset, Bone Marrow Response Correlated with Survival

LEERINK SWANN

K-M Curves of Response-based Subsets



Relation Between BM Response and Survival

BM Blast Response	>50% Blast Decrease	Stable BM Response	Progressive Disease	Not Assessed (No Biopsy)	P value
Number of Patients	12	15	3	9	
Median Overall Survival (wks)	37	40	10	11	.003

Clearly the number of patients is small, although there is a sizable separation of the curves.

K-M: Kaplan Meier; BM: bone marrow

Source: Company Reports

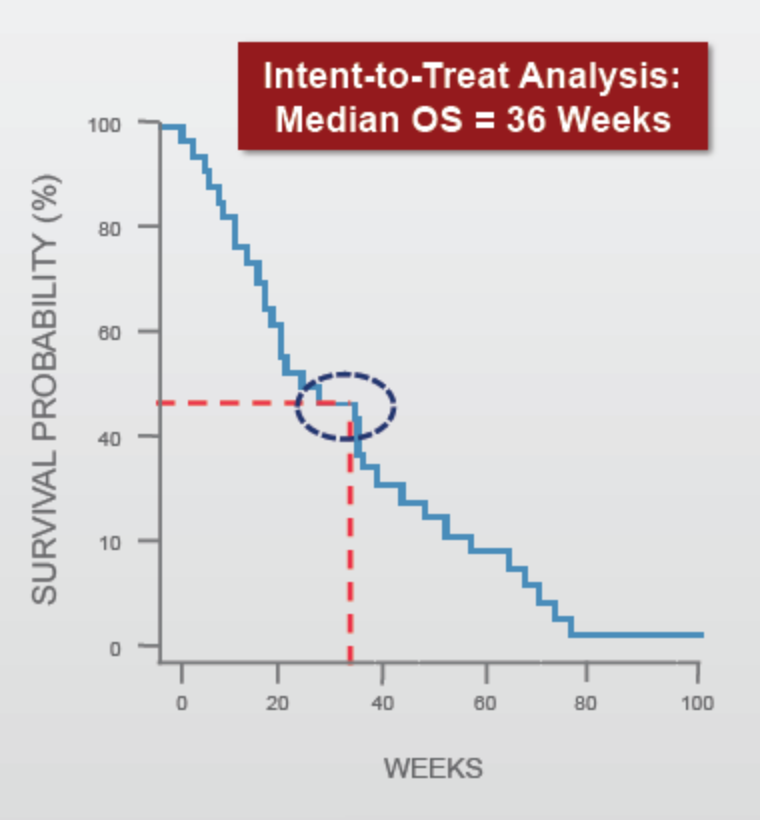
LEERINK SWANN

SURVIVAL DATA -- COULD THE USE OF LOW-DOSE ARA-C IN THE CONTROL ARM MAKE IT A MORE DIFFICULT COMPARISON?

Rigosertib Survival from Phase II Patients

LEERINK SWANN

- 39 RAEB-1, -2, -t patients previously treated with hypomethylating agent
- Median of 36 weeks (8.4 months).



RAEB: refractory anemia with excess blasts; RAEB-t: refractory anemia with excess blasts in transformation

Source: Company Reports, Seetharam, Leukemia Research 2012

Overall Survival after Vidaza/Dacogen Failure Is Short

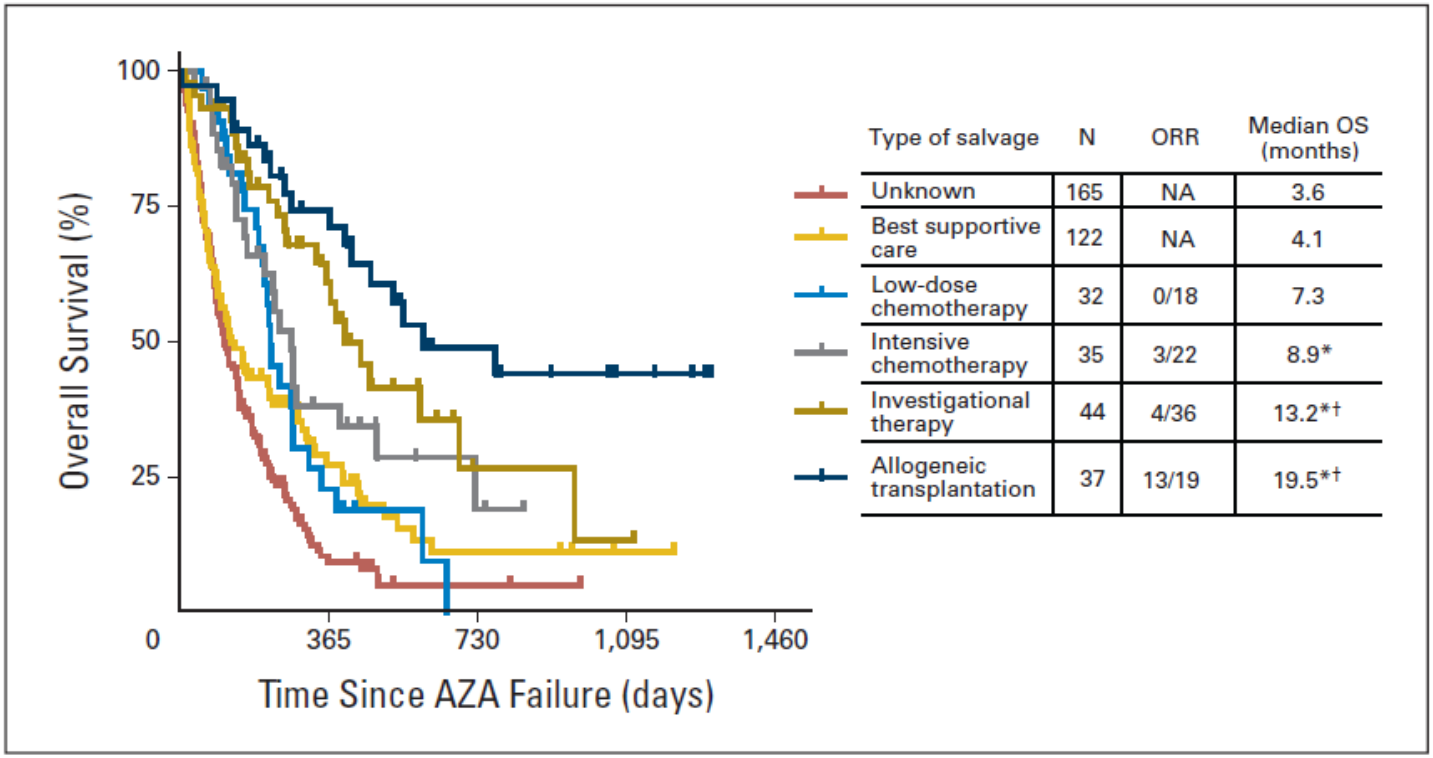
LEERINK SWANN

- Several studies analyzed survival after failure of hypomethylating agents
  - The most relevant could be a study by Prebet et al. on a cohort of patients from 4 studies and the French compassionate use program. Median survival in a 435-patient cohort of high-risk MDS patients failing azacitadine was 5.6 months (95% CI 5 to 7.2 months).
  - Analysis of data from MD Anderson Cancer Center showed a median survival of 4.3 months in 87 patients with MDS and chronic myelomonocytic leukemia after failure of Dacogen (Jabbour et al.).
  - Another single-institution analysis (Lin et al.) showed median survival post-Vidaza of 252 days (8.4 months) for 59 patients treated at the Moffitt Cancer Center. However, this is from the time of Vidaza failure and may not be comparable to survival data in the clinical trial setting, which would be from randomization.
    - Of 31 of these patients with known information on post-Vidaza therapy, 13 received induction chemotherapy, 6 Dacogen, 3 Revlimid, 2 hematopoietic stem cell transplant, 5 clinical trial, 2 other, and only 5 received supportive care.

CI, confidence interval

Source: Prebet, JCO 2011; Jabbour et al., Cancer 2010; Lin et al., 2010 ASH abstract

On First Look, It Would Appear that Post-Vidaza Survival Depends on the Second-line Treatment Received



Type of salvage	N	ORR	Median OS (months)
Unknown	165	NA	3.6
Best supportive care	122	NA	4.1
Low-dose chemotherapy	32	0/18	7.3
Intensive chemotherapy	35	3/22	8.9*
Investigational therapy	44	4/36	13.2*†
Allogeneic transplantation	37	13/19	19.5*†

- However, treatment selection based on patient fitness likely played a big role in the difference in survival. Patients who received more intensive treatment were likely healthier patients to begin with, therefore they had longer overall survival not necessarily because of the treatment selection. A clear example of this was shown in Vidaza's AZA-001 study, described on next page.

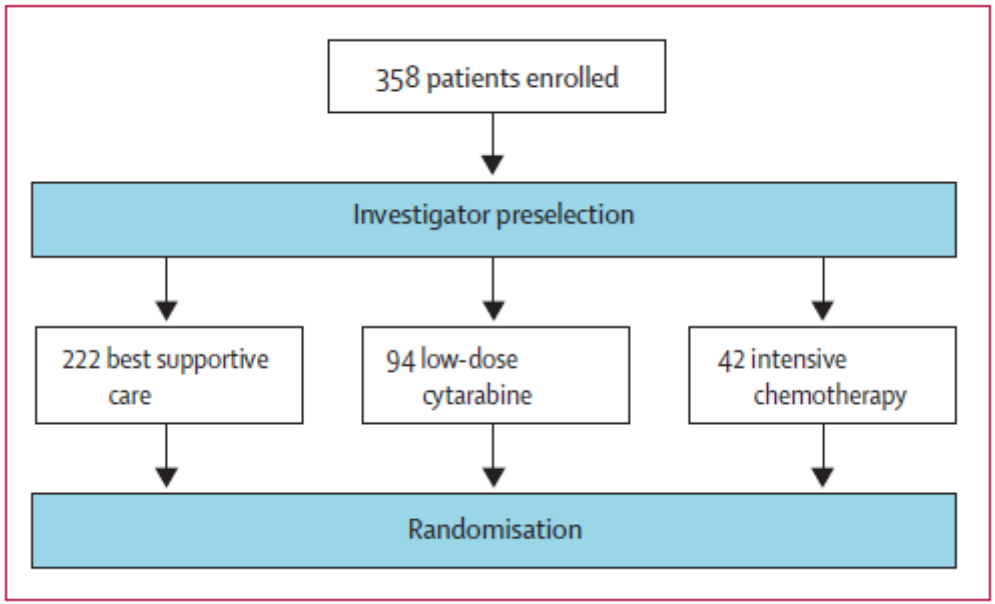
AZA: azacitidine (Vidaza); ORR, objective response rate; OS: overall survival

Source: Prebet, JCO 2011

AZA-001 Trial Assigned Patients First to Each of the Conventional Care Before Randomization

LEERINK SWANN

- In the AZA-001 study, patients were assigned to one of three groups (BSC, low-dose cytarabine [ara-C], or intensive chemotherapy) at the discretion of the prescribing physician.
- Patients were then randomized to receive either that treatment or Vizada:



Source: Fenaux et al. (2008), Lancet

Patient Selection Drove the Difference in Survival in Patients Receiving Various Regimens in the Control Arm of AZA-001

LEERINK SWANN

- While patients in the control arm who actually received low-dose cytarabine or intensive chemotherapy had a higher OS than those who received BSC (15.3/15.7 vs. 11.5 months), the patients in the Vidaza arm assigned to the cytarabine/intensive chemo cohort also had a higher OS than those assigned to the BSC cohort (24.5/25.1 vs. 21.5 months) even though these patients received exactly the same drug (Vidaza). The difference vs. BSC was relatively similar for the control (3.8/4.2 months) and Vidaza arms (3.0/3.6 months).
- This suggests that the differences in OS in patients receiving various treatment were mostly due to patient characteristics rather than treatment choice.

	BSC only (n=222)				Low-dose cytarabine (n=94)				Intensive chemotherapy (n=42)			
	Azacitidine (n=117)	BSC (n=105)	HR (95%CI)	p value	Azacitidine (n=45)	Low-dose cytarabine (n=49)	HR (95%CI)	p value	Azacitidine (n=17)	Intensive chemotherapy (n=25)	HR (95%CI)	p value
Overall survival (months)	21·1 (10·5–NR)	11·5 (5·7–NR)	0·58 (0·40–0·85)	0·0045	24·5 (8·4–34·7)	15·3 (4·9–25·8)	0·36 (0·20–0·65)	0·0006	25·1 (10·0–NR)	15·7 (8·2–24·1)	0·76 (0·33–1·74)	0·51
Time to transformation to AML (months)	15·0 (8·8–27·6)	10·1 (3·9–19·8)	0·41 (0·27–0·63)	<0·0001	15·0 (7·3–25·5)	14·5 (4·9–19·2)	0·55 (0·28–1·11)	0·097	23·1 (6·4–25·4)	10·7 (4·6–15·4)	0·48 (0·16–1·45)	0·19

Data are median (IQR). Hazard ratios calculated with stratified Cox proportional hazards model adjusted for treatment, subgroup, Eastern Cooperative Oncology Group performance status, lactate dehydrogenase, haemoglobin, number of previous red-blood-cell transfusions, and presence or absence of cytogenetic –7/del(7q) abnormality. No subgroup-by-treatment interactions were significant (p>0·20). BSC=best supportive care. NR=not reached. HR=hazard ratio. AML=acute myeloid leukaemia.

Table 2: **Overall survival and time to progression to acute myeloid leukaemia comparison for groups according to investigator preselection**

Source: Fenaux et al. (2008)

Low-dose Ara-C Use in BSC Arm in ONTIME Trial Not Likely to Significantly Affect Survival of the Control Arm

LEERINK SWANN

- Patients in the BSC arm of the ONTIME trial are allowed to receive low-dose ara-C (cytarabine), as medically justified.
- Although both the AZA-001 study and the Prebet JCO 2011 study appeared to show longer survival for low-dose ara-C vs. BSC, we believe our analysis in the preceding pages shows that this difference in OS is likely due to patient selection rather than treatment.
  - i.e., patients who could receive and tolerate ara-C would naturally be healthier (and presumably have a better prognosis) than those who could not.
- MEDACorp KOLs do not believe low-dose ara-C confers a survival benefit.
- Percent of control patients receiving ara-C is low, further mitigating the impact.

LEERINK SWANN

RIGOSERTIB HAS BEEN WELL TOLERATED, AND LACK OF MYELOSUPPRESSION COULD BE A SIGNIFICANT ADVANTAGE

Substantial Patient Exposure to Rigosertib

LEERINK SWANN

- Rigosertib clinical trial patients
  - 718 IV; 146
  - 642 monotherapy; 222 combination
  - 680 in US, 100 Europe and 84 in India

Source: Company Reports

Rigosertib Appears to Be Well Tolerated

LEERINK SWANN

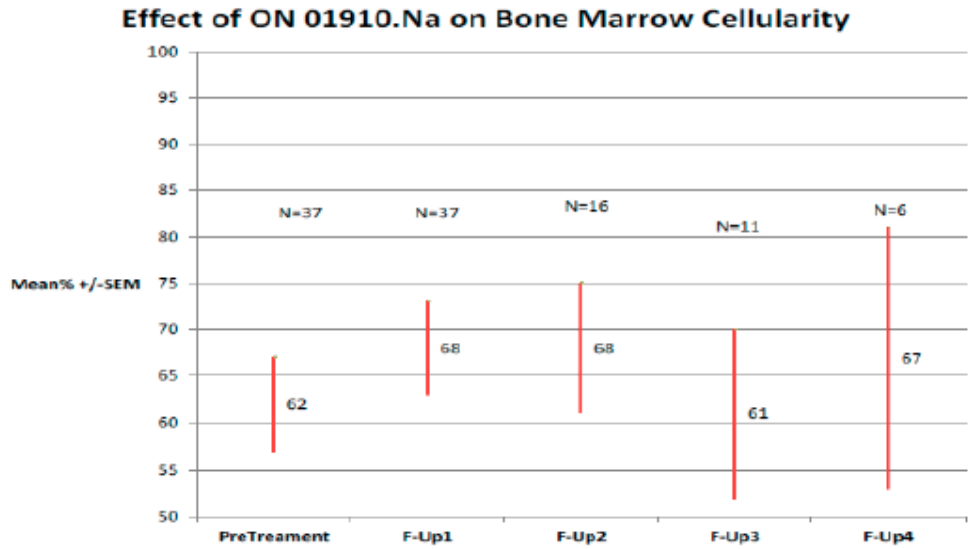
- Data from 79 MDS and AML patients treated in 4 Phase I or II trials show IV rigosertib to be well tolerated.
- Most frequent drug-related AEs (occurring in >10% of patients):
  - Nausea, diarrhea, fatigue, anemia, dysuria, and hematuria
- Grade 3 or 4 toxicities seen in at least two patients:
  - Anemia, thrombocytopenia, neutropenia, decreased white blood cells, urinary frequency, dysuria, decreased blood sodium, increased clotting time, fatigue, fever, and diarrhea.

Source: Company Reports

Lack of Myelosuppression Highlighted as a Significant Advantage for Rigosertib

LEERINK SWANN

Lack of Myelotoxicity



- MEDACorp KOLs commented that rigosertib has a very good safety/tolerability profile, and lack of myelosuppression is a significant advantage.
- Myelosuppresion can be especially problematic in MDS patients. The KOL noted that for Revlimid, which is approved for 5q-del MDS, 40% of patients cannot take a second dose due to myelosuppression. Revlimid label indicates that 80% of MDS patients required a dose delay or reduction.

Source: Raza et al., ASH 2011 presentation

LEERINK SWANN

ORAL RIGOSERTIB, DATA IN LOWER-RISK MDS

Phase I Results of Oral Rigosertib in MDS Patients (n=37)

LEERINK SWANN

Patient Demographics and Duration of Treatment

		Rigosertib BID Dosing (mg) for 2 out of 3 weeks					Total
		70	140	280	560	700	
	# Patients	3	2	2	24	6	37
	Sex	3F	1M/1F	2M	15M/9F	5M/1F	21M/14F
	Age (range)	64-82	74-84	73-76	56-89	53-82	53-89
	Duration of Treatment (wks)	4-18	9-34	11-31	1-31+	5-17	1-31+
IPSS	Low-Int-1 non Tx dependent	1	1	0	4	1	7
	Low-Int-1 Tx dependent	0	0	0	12	4	16
	Int-2 High risk	2	1	2	8	1	14
FAB/WHO	RCMD	1	1	0	15	5	22
	RAEB-1	2	1	0	3	0	6
	RAEB-2	0	0	2	6	1	9
Prior Azacitidine/Decitabine Treatment		3	2	2	14	5	26

Response Summary

IPSS	N Patients 560 mg bid	Transfusion Independence	HI-E	SD	BM CR	Overall Response
Low-Int-1	16	4 (including 2 HI-N and 1 HI-P)	1	6		69%
Int-2 High	8			1	1	25%
Total	24	4	1	7	1	54%

		560 mg bid			700 mg bid			
		Low risk	Int-1	All	Low risk	Int-1	All	Total
	Total Patients	4	8	12	1	3	4	16
Evaluable	Yes	4	6	10	0	2	2	12
	No	0	2	2	1	1	2	4
Tx response (TI+ER) in Evaluable pts	Yes	3	2	5	NE	0	0	5
	No	1	4	5	NE	2	2	7
% Tx response (ITT)		75%	25%	**42%**	NE	0%	**0%**	**31%**

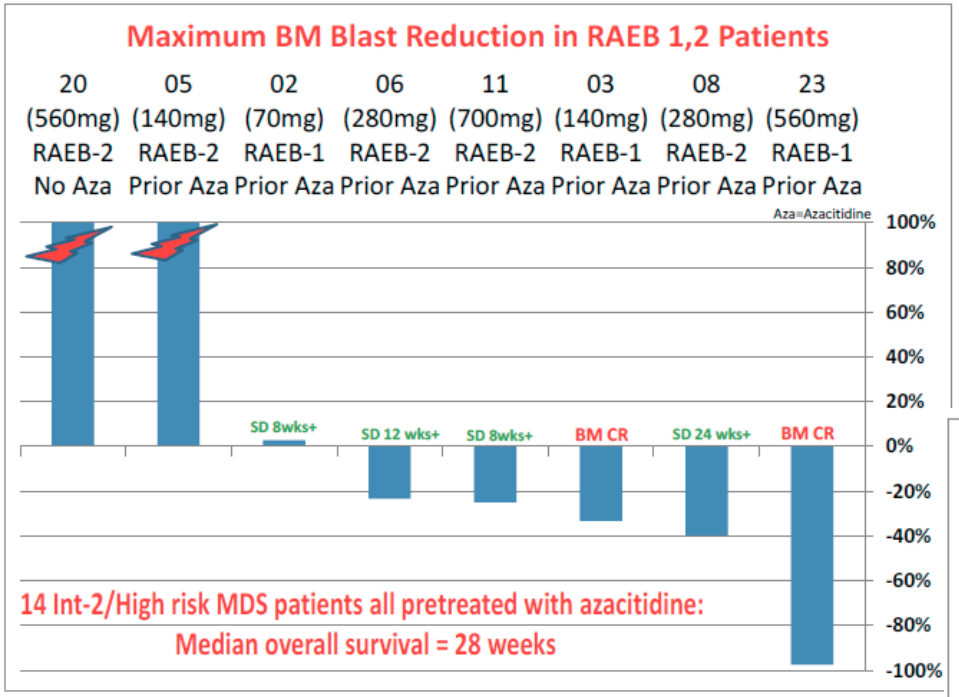
- Bioavailability ~35%
- 560 mg twice a day (BID) dosing recommended
- Responses include 2 bone marrow CR, 1 platelet and 1 ANC responses in high-risk patients, and 4 cases of transfusion independence and 1 erythroid response in low/int-1 transfusion dependent patients.

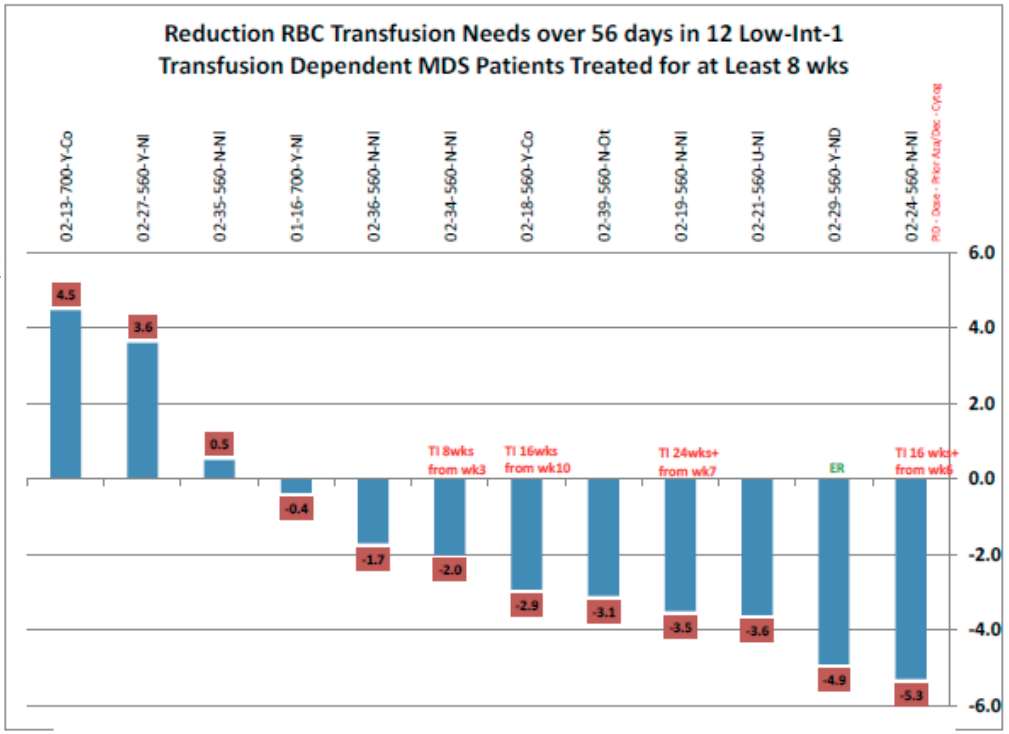
CR: complete response; ANC: absolute neutrophil count

Source: Komrokji et al., ASH 2011 presentation

Phase I Results of Oral Rigosertib in MDS: Bone Marrow and Transfusion Independence Responses

LEERINK SWANN





Source: Komrokji et al., ASH 2011 presentation

Phase I Safety Findings for Oral Rigosertib

LEERINK SWANN

Grade 2+ Drug Related Adverse Events (SAEs)

	560mg (N=24)		700mg (N=6)	
	Grade 2	Grade 3	Grade 2	Grade 3
Dysuria	4	1	3	1 (DLT)
Hematuria (onset 5 wks)	4 (1)	1	1	1
Urinary Frequency	0	1	0	0
Nocturia	0	0	1	0
Cystitis	0	0	1	0
Diarrhea	1	1	0	0
Abdominal pain (onset 8 wks)	0	1	0	0
Hypotension, syncope (onset 10 wks)	0	0	0	1
Shortness of breath	0	0	0	1 (DLT)
Fatigue	1	1	0	0

Gr 2+ Urinary Symptoms (dysuria, hematuria, nocturia, urinary frequency, cystitis)

Rigosertib BID Dosing	560 mg	700 mg
Incidence (%)	7/24 (29%)	5/6 (83%)
Onset week median [range]	5, [3,10]	3, [2,15]
Hold/Dose reduction	4	2
Dc'd Rigosertib	3	3

Additional Safety Review:

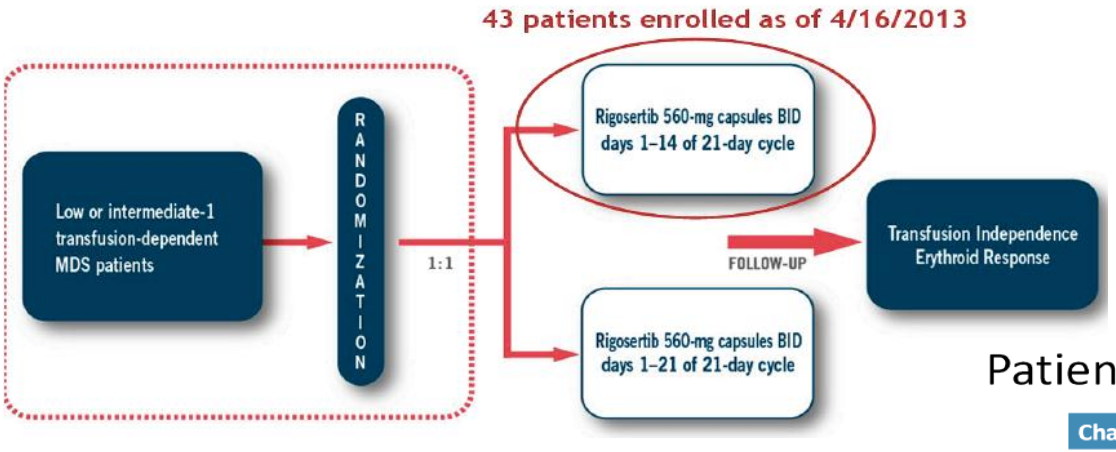
- No myelotoxicity (ANC, WBC, Platelet, Hb)
- No renal (creatinine) or liver (ALT, bilirubin toxicity
- No hypokalemia or hyponatremia
- No change of glucose, calcium/phosphorus
- No variation of urinary pH
- No change in vital signs (temperature, blood pressure, pulse)

Source: Komrokji et al., ASH 2011 presentation

Phase II Study in Lower-Risk Transfusion Dependent MDS Patients with Oral Rigosertib

LEERINK SWANN

Study Design of Oral Rigosertib in Lower Risk Transfusion Dependent MDS Patients



Patient Characteristics and Demographics

Characteristic	N=43
Median Age, years (range)	72 (54-84)
Male/Female	25/18
Median years from diagnosis (range)	2 (0-12)
Median prior MDS therapies (range)	1 (0-10)
Prior treatment with HM agents/Lenalidomide	12/10
Prior treatment with ESAs	22
IPSS Risk at Screen (Low/Int-1/Int-2)	7/34/2
ECOG PS (0/1/2)	35/3/5
FAB/WHO Classification	
Refractory Anemia	11
Refractory Cytopenia with Multiple Dysplasia	25
RAEB-1	6
RAEB-2	1
Cytogenetics (Normal/Tri8/del5q/Other)	20/4/2/26

Source: Raza et al., ASCO 2013 presentation,

Phase II Study in Lower-Risk Transfusion Dependent MDS Patients with Oral Rigosertib

LEERINK SWANN

Grade 2/3 Drug Related Adverse Events and SAEs

Symptom	Incidence in 34 Patients Dosed Intermittently		
Severity	*Grade 2*	*Grade 3*	*Total %*
Urinary urgency/frequency (1 SAE)	12	1	38
Dysuria	5	1	18
Hematuria/cystitis (4 SAES in 3 patients)	0	5	15
Fatigue	5	0	15
Nausea	3	0	9
Intermittent Neutropenia	0	1 gr 3; 1 gr 4	6
Hemolytic anemia	1	0	3
Diarrhea	1	0	3
Myalgia	1	0	3
Abdominal pain/discomfort	1	0	3
Insomnia	1	0	3

Symptom	Incidence in 9 Patients Dosed Continuously		
Severity	*Grade 2*	*Grade 3*	*Total %*
Hematuria/cystitis/bladder inflammation (1 SAE)	2	3	56
Urinary urgency/frequency	4	0	44
Dysuria	2	0	22

No other drug-related serious adverse event (SAE)

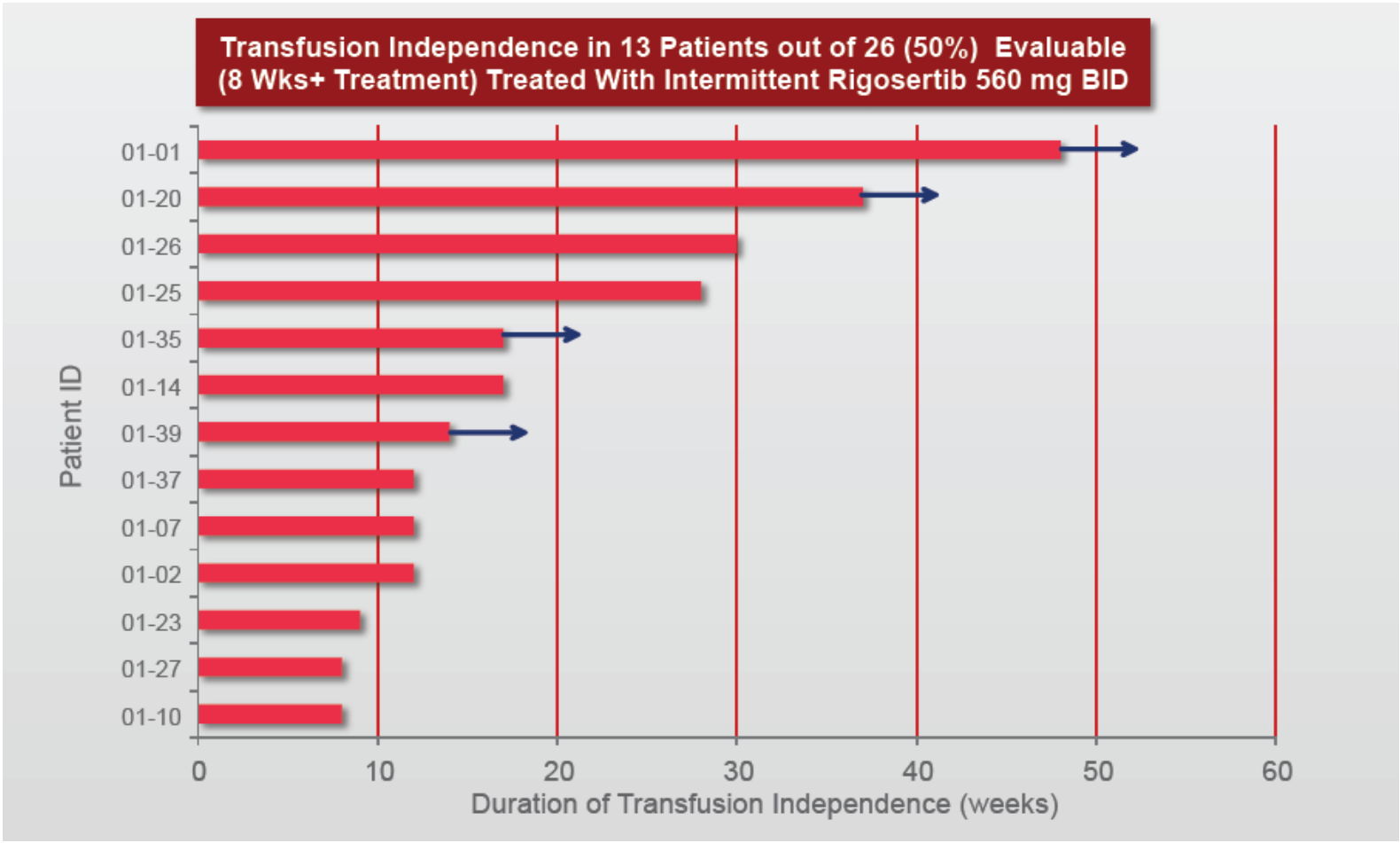
Overall Transfusion Response

Regimen	Responders	Evaluable Patients		ITT Patients	
		Total	*%*	*Total*	*%*
Continuous	2	8	25%	9	22%
Intermittent	13	26	50%	34	38%
Total	**15**	**34**	**44%**	**43**	**35%**

- Very encouraging signal in transfusion independence
- MEDACorp KOLs believe data in lower-risk MDS patients with oral rigosertib are even stronger than IV formulation in high-risk population.

Source: Raza et al, ASCO 2013 presentation,

50% of Patients Treated with Rigosertib Became Transfusion Independent



Source: Company Reports

Urinary Adverse Events and Mitigation Strategies

LEERINK SWANN

- Urinary symptoms include urinary frequency, dysuria, hematuria
- Working hypothesis is that extended residence time of excreted drug in the urinary bladder causes inflammation and cytotoxicity
- Mitigation strategy involves:
  - Dose reduction for grade 2+ urinary symptoms
  - Replace 560 BID dosing with 560 mg am (30 min+ before breakfast) and 280 mg 2 hours+ after lunch and 30 min+ before dinner
  - Dysuria questionnaire to all patients at baseline, 3 weeks and every 3 cycles thereafter
  - Recommend vigorous hydration and bicarbonate as needed

Source: Raza et al., ASCO 2013 presentation,

Path Forward in Lower-Risk MDS to Be Elucidated in 2014

LEERINK SWANN

- Updated Phase II data in lower-risk MDS patients treated with oral rigosertib are expected at ASH.
  - Data on a total of ~60 patients expected (ASCO data had 34 evaluable patients).
- Onconova will hold discussions with regulatory agencies in 4Q 2014 for the path forward to garner approval.
- Onconova is examining potential prognostic markers by correlating response with genomics.
- Two Phase II studies are expected to be completed in 3Q 2014.
  - The MDS consortium is also interested in initiating a 3rd randomized trial.

Transfusion Independence Likely to Be Sufficient for FDA Approval

LEERINK SWANN

- In 2005, the FDA approved CELG's Revlimid (lenalidomide) for MDS patients with a 5q deletion based on a single-arm, Phase II trial with a primary endpoint of transfusion independence
- Two of 3 other agents in Phase III trials for lower-risk MDS are using transfusion independence as a primary endpoint:
  - CELG's oral Vidaza – Phase III 003 trial is using transfusion independence as the primary endpoint.
  - CELG's Revlimid (in non-del(5q) patients) – Phase III MDS-005 trial is using transfusion independence as the primary endpoint (ex-US trial).
  - AMGN's Aranesp – Phase III trial is using IWG erythroid (HI-E) response as a primary endpoint (ex-US trial).

Source: Company reports and Leerink Swann research

LEERINK SWANN

RIGOSERTIB IN SOLID TUMORS

Rigosertib Showed Single-agent Activity in Phase I Trials in Solid Tumors

LEERINK SWANN

- In Phase I dose escalation study of IV formulation rigosertib in 20 refractory solid tumor patients (Jimeno et al, JCO 2012),
  - a refractory ovarian cancer patients had an objective response and remained progression-free for 24 months.
- In a Phase I dose escalation study of oral rigosertib involving 48 refractory solid tumor patients (Jimeno et al, AACR 2013),
  - 2 metastatic head and neck squamous cell carcinoma (H&NSCC) patients refractory to platinum-based therapy had confirmed CR (disappearance of mediastinal and lung disease) and PR (53% decrease of liver metastasis), lasting 101+ and 40 weeks respectively. There were 6 H&NSCC patients enrolled in the study.
  - Further details of the study are shown on the next pages.

CR: complete response; PR: partial response,

Source: Jimeno et al, J. Clin. Oncol. 2012; Jimeno et al, AACR 2013 presentation,

Patient Characteristics and Tumor Responses for Oral Rigosertib Phase I

LEERINK SWANN

Best Overall Response

Patients	48
Sex	M24 F24
Median age (range)	58 (20-79)
Colorectal carcinoma	10
HNSCC	**6**
Ovarian carcinoma	4
Esophageal adenocarcinoma	4
Renal cell carcinoma	3
Hepatocellular carcinoma	2
Breast carcinoma	2
Uterine adenocarcinoma	1
Uterine carcinosarcoma	1
Leiomyosarcoma	1
Cervical adenocarcinoma	1
Transitional cell carcinoma	1
Pancreatic neuroendocrine	1
Vulvovaginal melanoma	1
Carcinoid tumor	1
Salivary gland carcinoma	1
Gastrointestinal stromal tumor	1
Craniopharyngioma	1
Adrenocortical carcinoma	1
SCC lung	1
Osteosarcoma	1
Prostate carcinoma	1
SCC vulva	1
Adenoid cystic nasopharyngeal	1

- 1 HNSCC CR (101+ wks)
- 1 HNSCC PR (30% reduction; 40 wks)
- Clinical benefit rate (CR+PR+SD ≥12 wks): 10/46 (22%)

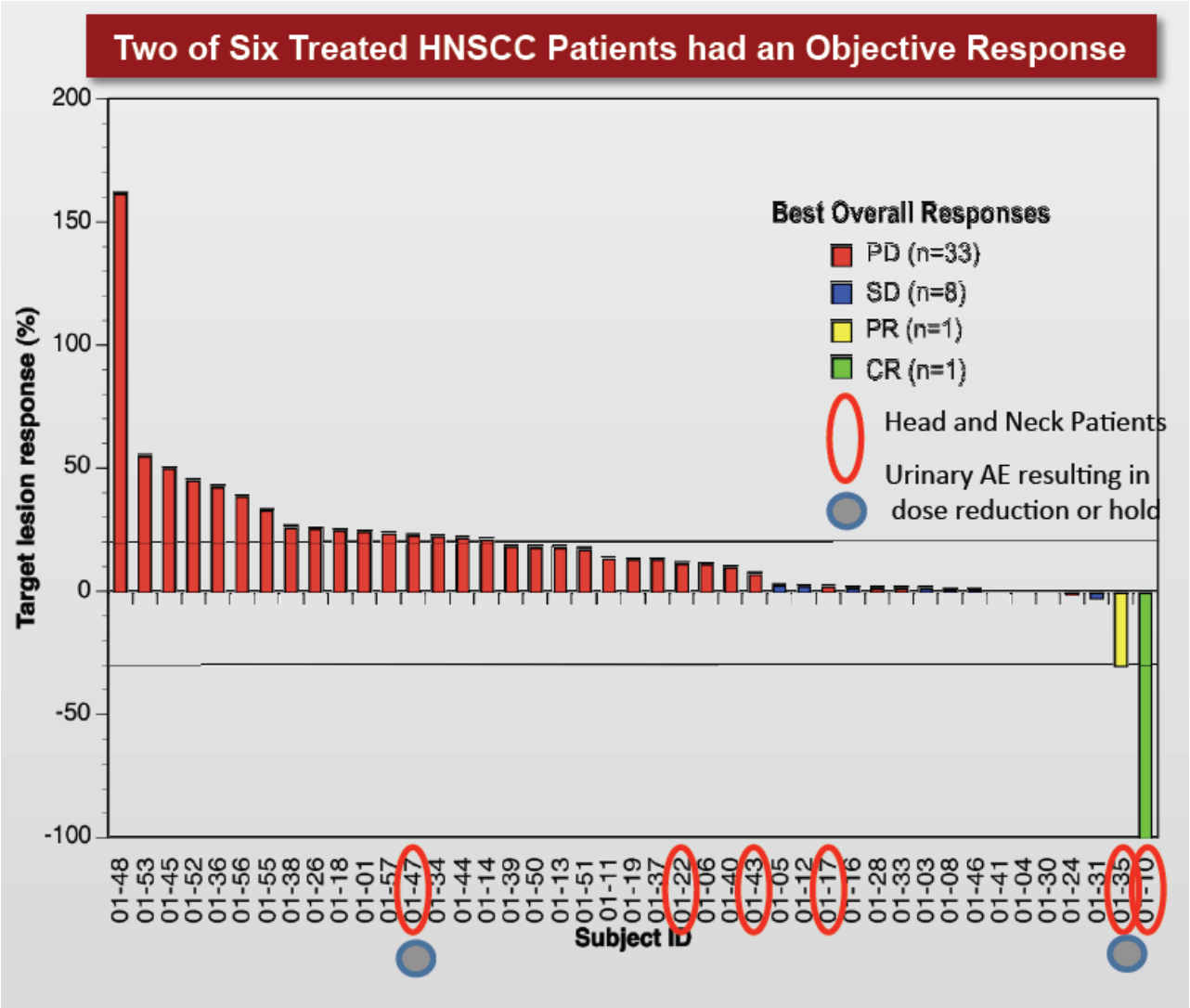
Dose	Site	Best overall response	Duration of Response or SD (weeks)
70	Ovarian carcinoma	SD	36
70	Ovarian carcinoma	SD	12
140	Pancreatic neuroendocrine	SD	24
280	Carcinoid tumor	SD	20
280	***HNSCC***	***CR***	***101+***
560	Adenoid cystic carcinoma	SD	22
560	Craniopharyngioma w/squamous differentiation	SD	12
560	***HNSCC***	***PR***	***40***
560	Hepatocellular carcinoma	SD	15
700	Renal cell carcinoma	SD	23

CR: complete response; PR: partial response; HNSCC: head and neck squamous cell caricinoma; SCC: squamous cell carcinoma

Source: Jimeno et al, AACR 2013 presentation,

Oral Rigosertib Phase I in Solid Tumors

LEERINK SWANN



Source: Company Reports

HNSCC – head and neck squamous cell carcinoma

Rigosertib/Gemzar Combination Data in Solid Tumors

LEERINK SWANN

- IV rigosertib was combined with Gemzar in a Phase I dose escalation trial that included an expansion cohort of pancreatic ductal adenocarcinoma patients,
  - Among 40 treated advanced solid tumor and lymphoma patients (32 evaluable), partial responses by RECIST were observed in 3 patients: 1 Gemzar-pretreated Hodgkin lymphoma, 1 thymic cancer, and 1 Gemzar-pretreated PDA. Another patient with Gemzar-naive PDA had an unconfirmed response. One patient with non–small cell lung cancer achieved stable disease for 48 weeks. Two heavily pretreated patients with ovarian cancer, including one previously treated with Gemzar, achieved more than 50% decrease in CA-125 level.
  - Of the 19 evaluable patients with PDA, one had a partial response (PR; 55% reduction) and 11 had stable disease (SD) as best response. Minor response was observed in 5 of 19 evaluable in patients with PDA including one unconfirmed PR (55% reduction). Among the subset of patients with PDA who had previously received gemcitabine, 4 of the 12 evaluable subjects had shrinkage including the confirmed PR. Decrease in CA19-9 (>=50%) was observed in 10 of 16 evaluable patients; 1 had PR, 4 SD/MR, 1 SD, and 4 PD. When analyzed according to the dose of rigosertib received, a trend was observed with the median survival of the 1,800-mg/m2 dose group being longer than 1,200 mg/m2 and 600 mg/m2 (42, 31,and 9.5 weeks, respectively), that was not significant statistically. The overall survival of patients with PDA at the recommended Phase II dose (1800 mg/m2 rigosertib / 1000 mg/m2 Gemzar) was 42 weeks.

CR: complete response; PR: partial response; MR: minor response; SD: stable disease; PD, progressive disease; PDA: pancreatic ductal adenocarcinoma

Source: Ma et al, Clinical Cancer Research 2012,

Rigosertib/Gemzar Combination in Pancreatic Cancer Is Supported by a Mechanistic Study

LEERINK SWANN

- Preclinical study identified Polo-Like Kinase 1 (PLK1, one of the targets of rigosertib) as a mediator of Gemzar resistance in pancreatic cancer
  - Gene expression in 11 tumors from freshly generated pancreatic cancer xenografts with known degrees of varying gemcitabine sensitivity was examined.,
  - The only gene that differentiated sensitive versus resistant cases was PLK1, showing >50% downregulation in sensitive cases and no change in the resistant cases.
  - Inhibition of PLK1 by either small interfering RNA gene knockdown or with rigosertib synergized with gemcitabine in gemcitabine-refractory in vitro models.
  - In vivo experiments in gemcitabine-resistant xenografts showed synergistic activity decreasing cell proliferation and tumor regressions.

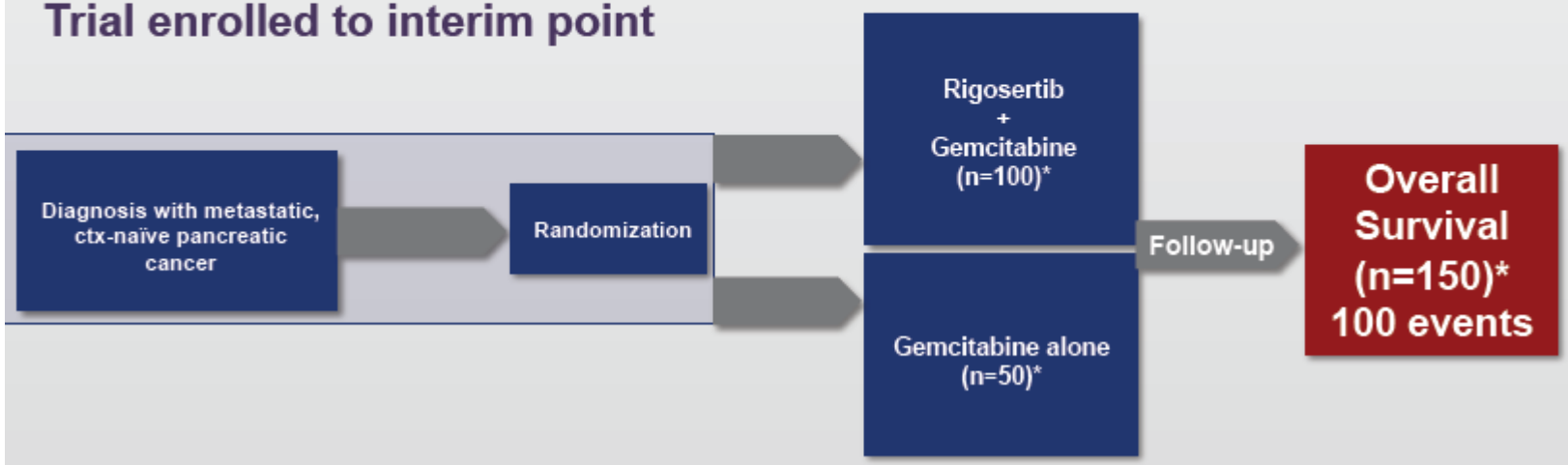
Source: Jimeno et al, Mol. Cancer Ther, 2010

Part 1 of Phase III in Pancreatic Cancer Is Scheduled to Read Out 4Q:13/1Q:14

LEERINK SWANN

- First-line chemotherapy naïve metastatic pancreatic patients
- Randomized Phase 3 trial based on survival endpoint
- Trial re-sizing after interim look and go/no-go decision in 2014

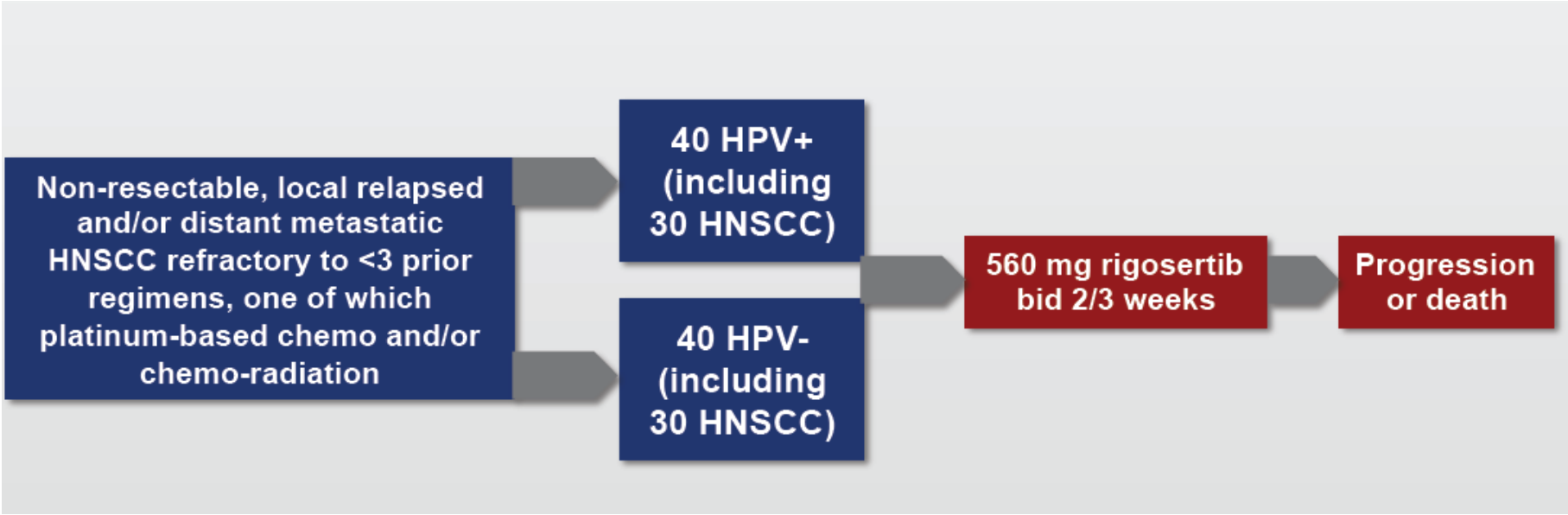
Trial enrolled to interim point



Source: Company Reports

A Phase II Is Ongoing To Test Oral Rigosertib in Head and Neck Cancer

LEERINK SWANN



Source: Company Reports

HNSCC – head and neck squamous cell carcinoma

LEERINK SWANN

MDS BACKGROUND, CURRENT TREATMENT AND COMPETITIVE LANDSCAPE

No Standard of Care for Patients Who Fail Vidaza/Dacogen

LEERINK SWANN

National Comprehensive Cancer Network®

NCCN Guidelines Version 2.2014
Myelodysplastic Syndromes

NCCN Guidelines Index
MDS Table of Contents
Discussion

PROGNOSTIC CATEGORY[aa] **TREATMENT**

IPSS: Intermediate-2, High[ee]
IPSS-R: Intermediate,[kk] **High, Very High**
WPSS: High, Very High

High-intensity therapy candidate[cc,ll] → **Transplant candidate and Donor available**

- **Yes** → **Allo-HSCT**[mm,nn] → **If relapse** → **Azacitidine/decitabine or Clinical trial**
- **No** → **Azacitidine (preferred) (category 1)/decitabine**[oo] **or High-intensity chemotherapy**[pp] **or Clinical trial**

Not high-intensity therapy candidate[cc,ll] → **Azacitidine (preferred) (category 1)/decitabine**[oo] **or Clinical trial**

Response[dd] → **Continue**

No response[dd] **or relapse** → **Clinical trial or Supportive care**[cc]

Source: NCCN

Current Market for MDS Drugs

LEERINK SWANN

- Higher- and lower-risk MDS are essentially treated as two distinct diseases:
  - Higher-risk: Goal of therapy is to improve survival and risk of progression to AML
    - Younger patients may be eligible for hematopoetic stem cell transplant (HSCT)
    - Most patients receive DNA methyltransferase inhibitors such as CELG's Vidaza (azacitidine) and/or Eisai/JNJ's Dacogen (decitabine)
    - Patients failing these have few good options – primarily best supportive care, low-dose cytarabine (ara-C), intensive chemotherapy, or clinical trials
  - Lower-risk: Goal of therapy is to improve anemia, thrombocytopenia, and neutropenia, along with attaining RBC transfusion independence
    - Patients with del(5q) MDS receive CELG's Revlimid (lenalidomide)
    - Patients may also receive agents such as ESAs (for anemia) or granulocyte colony-stimulating factor (G-CSF) analogs (for neutropenia)

Sales of Hypomethylating Agents

W/W sales ($M)	2009	2010	2011	2012	1H:13
Vidaza (reported)	387	534	705	823	415
Dacogen (estimated)	206	263	303	356	194
Total	593	797	1,008	1,179	609

Source: Company reports and Leerink Swann research

Agents in Clinical Development for Higher-Risk MDS

LEERINK SWANN

Drug	Company	Phase	Mechanism of action/molecule type	Route of Admin.
sapacitabine	CYCC/DSKYF	II/III	2'-deoxycytidine nucleoside analog	oral
CPI-613	Cornerstone Pharma	II	(part of Cornerstone's Altered Energy Metabolism Directed (AEMD) Platform)	oral
Pracinostat	MEIP/S*BIO	II	HDAC inhibitor	oral
Promacta (eltrombopag)	GSK/LGND	II	Thrombopoietin receptor agonist	oral
Temodar (temozolomid)	MRK	II	alkylating agent	oral
SGI-110	ASTX	I/II	DNA Methyltransferase inhibitor	SC
tosedostat	CTIC/Vernalis	I/II	Aminopeptidase inhibitor	oral
SL-401	STML	I/II	IL-3 recombinantly conjugated to diphtheria toxin	IV
belinostat (IV)	SPPI/TopoTarget	I	HDAC inhibitor	IV
ganetespib	SNTA	I	Heat Shock Protein 90 inhibitor	IV
BP-100-1.01	BPTH	I	antisense RNA against Grb-2	IV

Source: Company reports, clinicaltrials.gov and Leerink Swann research

Agents in Clinical Development for Lower-Risk MDS

LEERINK SWANN

Drug	Company	Phase	Mechanism of action/molecule type	Route of Admin.
Aranesp (darbepoetin alfa)	AMGN	III	ESA	IV
oral azacitidine	CELG	III	DNA Methyltransferase inhibitor	oral
Faridak (panobinostat)	NVS	II	HDAC inhibitor	oral
Telintra (ezatiostat)	TELK	II	Glutathione S-transferase inhibitor	oral
Promacta (eltrombopag)	GSK/LGND	II	Thrombopoietin receptor agonist	oral
sotatercept (ACE-011)	CELG	II	Activin receptor type IIA fusion protein	IV/SC
ACE-536	CELG	II	TGF-β inhibitor	SC
Apocept	Apogenix	I	CD95 receptor inhibitor	IV
ARRY-614	ARRY	I	p38/Tie2 inhibitor	oral
TXA127	Tarix Pharmaceuticals/UGNE	I	Angiotensin (1-7)	oral/SC

Source: Company reports, clinicaltrials.gov and Leerink Swann research

LEERINK SWANN

PARTNERSHIPS

Baxter Partnership in Europe

LEERINK SWANN

- Signed agreement in September 2012 with Baxter for all rigosertib indications in Europe. Baxter had purchased $50M of Series J convertible preferred stock in July 2012 previously.
- Upfront payment of $50M. Royalties on net sales ranging from the low-teens to the low-twenties
- Pre-commercial milestones of $512.5M, including:
  - $50M for positive Phase III results in higher risk MDS patients
  - $25M for each of the two joint decisions to proceed with development of rigosertib for certain indications specified in the arrangement with Baxter
  - $25M for each drug approval application filed for indications specified in the arrangement with Baxter

Source: Company reports

Symbio Pharmaceuticals Partnership in Japan and Korea

LEERINK SWANN

- Signed agreement in July 2011
- Upfront payment of $7.5M. Royalties based on net sales ranging from the mid-teens to 20% of rigosertib net sales in the licensed territories
- Milestone payments of up to $33M for development and regulatory milestones
- Tied milestone payments up to an aggregate of $30M based upon annual net sales of rigosertib by Symbio in the licensed territory

Source: Company reports

LEERINK SWANN

INTELLECTUAL PROPERTY

Intellectual Property

LEERINK SWANN

- Rigosertib patents
  - 64 issued patents
  - 18 pending patent applications
  - US composition of matter patent expires in 2026

Source: Company Reports

LEERINK SWANN

MANAGEMENT

Senior Management Has Experience Bring Drugs to Market

LEERINK SWANN

Ramesh Kumar
President & CEO
Dr. Kumar is a co-founder of Onconova, and is currently President and Chief Executive Officer, a position he has held since 1998. Prior to founding Onconova, he held senior positions at Princeton University, Bristol-Myers Squibb, DNX Corp., and Kimeragen, Inc. (later ValiGen Inc.), where he was President of the Genomics and Transgenics Division. Dr. Kumar received his Ph.D. in Molecular Biology from the University of Illinois, Chicago, and trained at the National Cancer Institute.

Tom McKearn
President, Research & Development
Dr. McKearn has served as President, Research & Development since September 2012. Prior to joining Onconova, he held senior positions at Agennix AG (formerly GPC Biotech GP), Bristol-Myers Squibb and Cytogen Corporation, which he founded and later served as its Chief Executive Officer. Dr. McKearn received his medical, graduate, and post-graduate training at the University of Chicago.

Ajay Bansal
CFO
Mr. Bansal has served as Chief Financial Officer since March 2013. Prior to joining Onconova, he served as Chief Financial Officer at Complete Genomics Incorporated, Lexicon Pharmaceuticals, Tercica Inc., and Nektar Therapeutics. Mr. Bansal received a B.S. from IIT, Delhi, an M.S. in Operations Research and an M.B.A. from Northwestern University.

Source: Company Reports

ONTX Income Statement ($000)	2011A	2012A	Mar-13A	Jun-13E	Sep-13E	Dec-13E	2013E	2014E	2015E	2016E	2017E	2018E
Collaboration agreements												
Royalties									2,681	6,914	21,868	32,739
Sales									44,678	79,013	168,213	242,512
Total revenue	1,487	46,190	1,116	1,116	1,116	1,116	4,464	2,116	47,359	85,927	190,080	275,251
COGS										6,874	15,206	19,401
% of revenue										*8%*	*8%*	*8%*
R&D	22,624	52,762	12,756	12,884	13,012	13,143	51,794	59,478	61,263	63,100	64,993	66,943
SG&A	6,436	15,707	3,346	3,379	3,413	3,447	13,586	30,000	67,017	79,013	75,696	84,879
% of revenue									*150%*	*100%*	*45%*	*35%*
Total operating expenses	29,060	68,469	16,102	16,263	16,426	16,590	65,381	75,080	128,279	148,988	155,895	171,223
Net income (loss) from operations	(27,573)	(22,279)	(14,986)	(15,147)	(15,310)	(15,474)	(60,917)	(72,964)	(80,921)	(63,061)	34,185	104,028
Change in fair value of warrant liability	1,287	367	14	0	0	0	14					
Interest expense	(19)	(8,608)	0	0	0	0	0					
Other income, net	11	608	127	0	0	0	127	0	0	0	0	0
Net income (loss) before income taxes	(26,294)	(29,912)	(14,845)	(15,147)	(15,310)	(15,474)	(60,776)	(72,964)	(80,921)	(63,061)	34,185	104,028
Provision (benefit) for income taxes	0	0	0	0	0	0	0	0	0			
Tax rate												
Net income (loss)	(26,294)	(29,912)	(14,845)	(15,147)	(15,310)	(15,474)	(60,776)	(72,964)	(80,921)	(63,061)	34,185	104,028
Accretion of preferred stock	(4,020)	(3,953)	(1,019)	(1,797)	0	0	(2,816)	0	0			
Net income (loss) to common stockholders	(30,314)	(33,865)	(15,864)	(16,944)	(15,310)	(15,474)	(63,592)	(72,964)	(80,921)	(63,061)	34,185	104,028
Net loss per share	**(14.18)**	**(2.67)**	**(1.03)**	**(0.79)**	**(0.71)**	**(0.72)**	**(3.18)**	**(3.34)**	**(3.52)**	**(2.61)**	**1.23**	**3.57**
Basic shares	2,137	12,669	15,446	21,389	21,496	21,604	19,984	21,875	22,969	24,117	25,323	26,589
Dilutive shares			15,446	24,186	24,428	24,672	22,183	24,919	25,168	26,426	27,748	29,135

Source: Company Reports and Leerink Swann

Disclosures Appendix

Analyst Certification

I, Howard Liang, 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.

Distribution of Ratings/Investment Banking Services (IB) as of 06/30/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	103	62.80	30	29.00
HOLD [MP]	61	37.20	2	3.00
SELL [UP]	0	0.00	0	0.00

Explanation of Ratings

Outperform (Buy): **We expect this stock to outperform its benchmark over the next 12 months.**

Market Perform (Hold/Neutral): **We expect this stock to perform in line with its benchmark over the next 12 months.**

Underperform (Sell): **We expect this stock to underperform its benchmark over the next 12 months.The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.**

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

Important Disclosures

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's asset management group and proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Publishing Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, the Private Client Division, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

Leerink Swann Consulting LLC, an affiliate of Leerink Swann LLC, is a provider of evidence-based strategy and consulting to the healthcare industry.

For price charts, statements of valuation and risk, as well as the specific disclosures for covered companies, client should refer to https://leerink2.bluematrix.com/bluematrix/Disclosure2 or send a request to Leerink Swann Publishing Department, One Federal Street, 37th Floor, Boston, MA 02110.

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
	Irene Lau	(415) 905-7256	irene.lau@leerink.com
	Gena Wang, Ph.D.	(212) 277-6073	gena.wang@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@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 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
Research Assistant	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	George Villarina	(212) 277-6012	george.villarina@leerink.com

New York
1251 Avenue of Americas, 22nd Floor
New York, NY 10020
(888) 347-2342

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