OUTPERFORM

Reason for report: **EARNINGS**

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ACCELERON

3Q13 CC: Solid Execution, & Multiple Catalysts Ahead; Reit. OP Rating & \$33 PT

- **Bottom Line:** We believe 3Q13 EPS updates demonstrate that XLRN in on track and management executing very well straight out of the IPO gate. Additionally, we see that any potential pipeline dose escalation related sell-off concerns create an usually attractive buying opportunity given MEDACorp key opinion leaders (KOLs) suggest we are only now beginning to advance to therapeutic dose levels, and they predicted meaningful efficacy would be observed at still higher doses. For example, the KOLs predicted (LINK) Sotatercept at 0.5mg could be somewhat effective but they had robust expectations for a future 1g cohort. Nonetheless, we anticipate that the 12/9 ASH-13 abstract for β-Thalassemia will have dose escalating Hemoglobin (Hgb) increases. Early to late 2014 appears full of catalysts that could significantly drive the shares. We reiterate our Outperform rating & \$33 price target (PT).
- MEDACorp KOLs previously set our expectations to consider the Sotatercept 0.5mg dose as minimally effective, and they have robust expectations for a future 1g cohort. The 3Q13 update indicated that dose escalation in the β -Thalassemia (β -Thal.) and myelodysplastic syndromes (MDS) trials continues with current enrollment at 0.75mg (1g could be next). As a reminder, previous β -Thal. data demonstrated dose dependent Hgb increases in the 0.1 and 0.3mg cohorts that could be raised during the first month of therapy but also matured further over time. As a result, we are optimistic the upcoming ASH update for the 0.5mg cohort will demonstrate further meaningful improvements and serve as an incremental driver. ACE-536 dose escalation also appears to be advancing well.
- 2014 should be thick with catalysts for the entire pipeline. The Dalantercept recommended dose level (RDL) will be 1.2g, which should build upon the dose dependent efficacy observed at the primary and metastatic lesions observed in early cohort in the Phase II renal cell carcinoma (RCC) Axitinib combo trial. The expansion phase with 20 patients is now starting, and the randomized Phase II PFS (progression free survival) primary endpoint portion will start by end of 1Q14. Also during 1Q14, we continue to expect top-line data from the dose escalation phase of the trial. For Sotatercept and ACE-536, full Phase II dose escalation β -Thal and MDS data should be ready for presentation in 2Q14 at EHA-2014 and expansion trial data in 4Q14 at ASH-2014.
- XLRN reported a net loss of ~\$18.5M for 3Q13 vs. our estimate of ~\$6M with the difference primarily due to expenses associated with the increase in common warrant valuation of \$10.1M in 3Q13. Revenue was \$4.3M vs. our ~\$8M and R&D was \$8.1M vs. our \$9.2M estimates. XLRN ended 3Q13 with \$116.5M in cash (~\$4.15 cash/share).

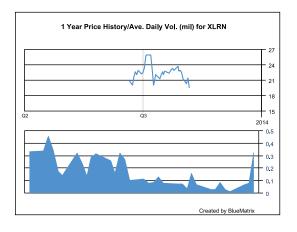
HEALTHCARE EQUITY RESEARCH

(NASDAQ:XLRN)

Kev Stats:

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S&P 600 Health Care Index:	1,197.55
Price:	\$19.53
Price Target:	\$33.00
Methodology:	DCF analysis
52 Week High:	\$26.73
52 Week Low:	\$15.00
Shares Outstanding (mil):	28.1
Market Capitalization (mil):	\$548.8
Book Value/Share:	\$1.42
Cash Per Share:	\$4.15
Dividend (ann):	\$0.00
Dividend Yield:	0.0%

Cash Per Share: Cash per share is based on pro forma shares outstanding of 28.1M at end of 3Q13.



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2012A					\$15.3					(\$1.44)	NM
2013E - New	\$15.0A	\$26.4A	\$4.3A	\$18.0	\$59.4	\$0.13A	\$0.64A	(\$0.66)A	\$0.13	\$0.25	78.1x
2013E - Old	\$15.0A	\$26.4A	\$8.0	\$18.0	\$67.4	\$0.13A	\$0.64A	(\$0.21)	\$0.12	\$0.68	NM
2014E - New					\$40.0					(\$0.52)	NM
2014E - Old					\$40.0					(\$0.59)	NM

Source: Company Information and Leerink Swann LLC Research

Revenue in MM, GAAP EPS presented



INVESTMENT THESIS

We rate XLRN Outperform. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). XLRN has multiple significant data read-out catalysts during almost every quarter until YE14. Pivotal Catalysts through 2014: 1) Interim Sotatercept Phase II (ongoing) β-Thal. 0.5mg/kg cohort data at ASH in 4Q13; 2) Preliminary Dalantercept Phase II RCC data in 1Q14; 3) Top-line Sotatercept and ACE-536 Phase II MDS and β-Thal. (4 trials) data at EHA in 2Q14; 4) Final Sotatercept and ACE-536 Phase II MDS and β-Thal. (4 trials) data at ASH in 4Q14; 5) Initiate pivotal MDS and/or β-Thal. trials by YE14. MEDACorp KOLs are very bullish and encouraged by emerging pipeline data and science. We assume 32% probability of approval for Sotatercept/ACE-536 in MDS, β-Thal. and Dalantercept in 2nd-line RCC.

Change in Estimates

We modified our model based on earnings released on 11.6.13. As a result, our 2013E revenue changed from \$67.4M to \$59.4M. Our 2013E EPS changed from \$0.68 to \$0.25 and 2014E EPS from (\$0.59) to (\$0.52).

MILESTONES

Product	Partner	Indication	Timing	Milestone
			Dec-2013	Interim Phase II ACE-011 β-Thal. (incl 0.5mg/kg dose) at ASH-2013
			YE13	Initiate Phase II Expansion Cohort for Sotatercept β-Thal.
			1Q14	Initiate Phase II in ESRD CKD
Sotatercept (ACE-011)			2Q14	Phase II dose escalation Sotatercept MDS + β-Thal. data at EHA-2014
(AGE 011)			4Q14	Final Phase II Sotatercept in MDS + β-Thal. data
	CELG	MDS +	2018	Approval and launch
	CLLG	β-Thal.	YE14	Initiate Phase III trial for MDS and/or β-Thal.
			1Q14	Initiate Phase II Expansion Cohort for II ACE-536 β-Thal.
ACE-536			2Q14	Phase II dose escalation data for ACE-536 MDS and β-Thal. at EHA-2014
			4Q14	Final Phase II ACE-536 in MDS and β-Thal. data
			YE14	Initiate Phase III trial for MDS and/or β-Thal.
			2018	Approval and launch
			4Q13	Initiate expansion Phase II in RCC combo trial (20 patients)
			4Q13	GOG Endometrial single agent trial Go-No-Go to Part-2 of trial
			1Q14	Dose escalation Phase II data in RCC combo trial (full at ASCO-2014)
Dalantercept		Oncology	10/14	GOG Ovarain Cancer single agent trial Go-No-Go to Part-2 of trial
(ACE-041)	Dransiator	Oncology	1Q14	Initiate Phase II (Part-2, N=112) RCC randomized trial (PFS endpoint)
	Proprietary		3Q14	Initiate Phase II combo trials in other indications (i.e., HCC, CRC, NSCLC)
			2014	Phase II data in SCCHN
			2018	Approval and launch
New TGF-β		Muscle	2014	Advance Muscle Loss candidate into clinic (ACE-083)
Candidates		Fibrosis	2015	Advance Fibrosis (i.e., PAH) candidate into clinic

Source: Company Reports, Leerink Swann LLC estimates



β-Thalassemia: Sotatercept & ACE-536

Phase IIa So	tatercept (ACE-011) Safety/Tolerability Trial in Adults With β- Thalassemia (ACE-011-B-Thal-001):
Purpose:	Dose finding study to determine safety/tTolerability of ACE-011 in β-Thalassemia adults
# Pts:	N=45
Design:	Interventional Non-Randomized, Safety/Efficacy Study, Single Group Assignment, Open Label, treatment trial
Trial	Arm-1 : 0.1mg/kg
Arms:	Arm-2 : 0.3mg/kg
	Arm-3: 0.5mg/kg
	Arm-4 : 0.75mg/kg
	• Note: All administered as a subcutaneous injection once every 21 days during treatment period
	As of 3Q13 (11.6.13) enrolling patients in 4 th cohort at 0.75mg
Primary End Point:	 Potential Recommended Dose (PRD) [Up to 27 months] [Designated as safety issue] PRD determined following assessment of efficacy/safety parameters based on 1st of 3 doses of Sotatercept
Liid i Oilit.	administered, up to at least 21 days following 1 st dose, for all doses evaluated
	PRD defined as highest dose level at which no more than 1/6 subjects experiences DLT. Recommended dose of
	Sotatercept defined based on review of efficacy/safety parameters + dose modification data. Efficacy defined as:
	• Transfusion Dependent (TD) Patients: Reduction of transfusion burden by ≥20% vs. calculated baseline
	transfusion burden to each subject
	 Non-Transfusion Dependent (NTD) Patients: Increase in Hgb ≥1g/dl vs. baseline Hgb, sustained for 12 weeks
	Actual Recommended Dose (RD) [Up to 27 months] [Designated as safety issue] DD defined based on review of efficient (Safety representation) does modified the data. This could be added to the country of
	 RD defined based on review of efficacy/Safety parameters + dose modification data. Efficacy defined as: Transfusion Dependent Patients: Reduction of transfusion burden by ≥20% vs. calculated baseline transfusion
	burden to each subject
	 Non-Transfusion Dependent Patients: Increase in Hgb ≥1 g/dl vs. baseline Hgb, sustained for 12 weeks
Secondary	■ RBC Transfusion Burden [Up to 27 months] [Designated as safety issue]
End	 Reduction of transfusion burden by ≥20% vs. calculated baseline transfusion burden for transfusion dependent Beta
Points:	Thalassemia major and Beta Thalassemia Intermedia subjects
	# participants with AEs [Up to 27 months] [Designated as safety issue]
	# participants with AEs
	■ PK-Serum Concentration of Sotatercept, PK-Observed Maximum Concentration (Cmax), PK-Time to Maximum
	Concentration (Tmax), PK-Area Under Concentration-Time Curve (AUC), PK-Concentration of Anti-Sotatercept
	Antibody in Serum
	Hgb Level Increase [Up to 27 months]
	■ Hgb level increase during study treatment vs. baseline Hgb level in non-transfusion dependent Beta Thalassemia
	intermedia subjects
Start:	March 2012
Data:	January 2014 (Final data collection date for primary outcome measure) then June 2014
Status:	May 16, 2013
Sponsors:	CELG
Clin. Trials:	NCT01571635, ACE-011-B-THAL-001, 2011-005659-15

Source: Clinicaltrials.gov and Leerink Swann LLC estimates

Phase II Trial	to evaluate Effects of ACE-536 in Patients with β-Thalassemia Intermedia (A536-04):
Purpose:	Evaluate effects of ACE-536 in patients with β-Thalassemia intermedia
# Pts:	N=50
Design:	Interventional, Non-Randomized, Safety/Efficacy Study, Single Group Assignment, Open Label, treatment
Trial Arms:	Arm-1: ACE-536 - 1 of 5 possible dose levels (0.2, 0.4, 0.6, 0.8, ?)
	**Note: 2 SC every 3 weeks for up to 5 cycles
Primary End	% patients with erythroid response, defined as hemoglobin increase of ≥1.5g/dL from baseline for ≥ 14 days (in
Point:	absence of transfusion) [at ~24 weeks from patient screening]
Secondary	■Number of patients with AEs [From treatment initiation to End-of-Study visit (~24 weeks later)
End Points:	■ Change in hemoglobin [Baseline to ~24 weeks]
	■ Changes in biomarkers of erythropoiesis, hemolysis, iron metabolism, bone metabolism [Baseline to ~24 weeks]
	 ACE-536 pharmacokinetics [Measured multiple time points of treatment, from study day 1 to ~24 weeks]
Start:	January 2013
Data:	November 2014 (Final data collection date for primary outcome measure) then November 2014
Status:	Recruiting March 14, 2013
Sponsors:	Acceleron
Clin.Trials:	NCT01749540, A536-04

Source: Clinicaltrials.gov and Leerink Swann LLC estimates



MDS: Sotatercept & ACE-536

or non-proliferative chronic myelomonocytic leukemia (CMML) #Pts: N=100 (low-Int-1 patients who are transfusion dependent or hemoglobin <10g/dL) Randomized, safety/efficacy, parallel, open label, treatment trial Trial Arms: Sotatercept Doses: All arms administered SC once every 3 weeks (q3W) for 5 cycles Arm-1: ACE-011 0.1mg/kg Arm-2: ACE-011 0.3mg/kg Arm-3: ACE-011 0.5mg/kg Arm-4: ACE-011 0.75mg/kg (enrolling as of 11.6.13) Primary End Point: Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary End Points: RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E		1010100 01 01 710 = 000
ronon-proliferative chronic myelomonocytic leukemia (CMML) # Pts: N=100 (low-Int-1 patients who are transfusion dependent or hemoglobin <10g/dL) Randomized, safety/efficacy, parallel, open label, treatment trial Trial Arms: Sotatercept Doses: All arms administered SC once every 3 weeks (q3W) for 5 cycles Arm-1: ACE-011 0.1mg/kg Arm-2: ACE-011 0.5mg/kg Arm-3: ACE-011 0.5mg/kg Arm-3: ACE-011 0.75mg/kg Arm-4: ACE-011 0.75mg/kg (enrolling as of 11.6.13) Frimary End Point: Frythroid Hematological Improvement (HI-E) [Up to 24 weeks] Fare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary Arderse Event (AEs) up to 3 years, Number of participants with AEs RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to e	ACE-011 Phas	se II Anemia in Iow-interm-1 Risk Myelodysplastic Syndromes (MDS) Trial (ACE-011-MDS-001):
# Pts: N=100 (low-Int-1 patients who are transfusion dependent or hemoglobin <10g/dL)	Purpose:	Determine safe, tolerable and effective dose of Sotatercept to treat anemia in patients with low/intermediate-1 risk MDS
Design: Randomized, safety/efficacy, parallel, open label, treatment trial Trial Arms: Sotatercept Doses: All arms administered SC once every 3 weeks (q3W) for 5 cycles Arm-1: ACE-011 0.1mg/kg Arm-2: ACE-011 0.5mg/kg Arm-3: ACE-011 0.5mg/kg Arm-4: ACE-011 0.5mg/kg Arm-4: ACE-011 0.5mg/kg Arm-4: ACE-011 0.5mg/kg (enrolling as of 11.6.13) Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] • Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Secondary • Adverse Event (AEs) up to 3 years, Number of participants with AEs End Points: • RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E • Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E • Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] • Time from baseline until progression to AML, until progression to events of higher risk MD • Number of participants who survive without progressing • Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] • Max concentration in serum • Time to maximum observed concentration serum • Area under the plasma concentration-time curve Start: Movember 20		or non-proliferative chronic myelomonocytic leukemia (CMML)
Trial Arms: Sotatercept Doses: All arms administered SC once every 3 weeks (q3W) for 5 cycles Arm-1: ACE-011 0.1mg/kg Arm-2: ACE-011 0.5mg/kg Arm-3: ACE-011 0.5mg/kg Arm-3: ACE-011 0.75mg/kg (enrolling as of 11.6.13) Primary End Point: Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary End Points: REC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progressing Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] Max concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve November 2012 Data: May 13, 2013		N=100 (low-Int-1 patients who are transfusion dependent or hemoglobin <10g/dL)
Arm-1: ACE-011 0.1mg/kg Arm-2: ACE-011 0.5mg/kg Arm-3: ACE-011 0.5mg/kg Arm-4: ACE-011 0.5mg/kg Arm-4: ACE-011 0.5mg/kg Arm-4: ACE-011 0.75mg/kg (enrolling as of 11.6.13) Primary End Point: Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary End Points: Adverse Event (AEs) up to 3 years, Number of participants with AEs RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progressing Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] Max concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve November 2012 March 2014 (Final data collection date for primary outcome measure) then September 2016	Design:	Randomized, safety/efficacy, parallel, open label, treatment trial
Arm-2: ACE-011 0.3mg/kg	Trial Arms:	Sotatercept Doses: All arms administered SC once every 3 weeks (q3W) for 5 cycles
Arm-3: ACE-011 0.5mg/kg		Arm-1: ACE-011 0.1mg/kg
Primary End Point: Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary End Points: Patients Adverse Event (AEs) up to 3 years, Number of participants with AEs RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Number of participants who survive without progression to events of higher risk MD Nax concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016		Arm-2: ACE-011 0.3mg/kg
Erythroid Hematological Improvement (HI-E) [Up to 24 weeks] • Rare transfused patients (requiring transfusion <4 units RBCs): % patients with increase in Hgb ≥1.5g/dL over 8 weeks in absence of RBC transfusion • Heavily transfused patients (requiring transfusion ≥4 RBCs units): % patients with 50% decrease in units transfused over 8 weeks Secondary End Points: • Adverse Event (AEs) up to 3 years, Number of participants with AEs • RBC Transfusion Independence [up to 24 weeks] Time between randomization (for Part 1)/start of therapy (for Part 2) and date the start of HI-E • Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E • Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] • Time from baseline until progression to AML, until progression to events of higher risk MD • Number of participants who survive without progressing • Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] • Max concentration in serum • Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		
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 Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progressing Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] Max concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve Start: November 2012 March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013 	End Points:	
Progression-free survival (PFS), Overall survival (OS) [2-year] Time from baseline until progression to AML, until progression to events of higher risk MD Number of participants who survive without progressing Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] Max concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		 Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E
 Number of participants who survive without progressing Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks] Max concentration in serum Time to maximum observed concentration serum Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		 Time to progression to Acute Myeloid Leukemia (AML), Time to progression to events of higher risk MDS, Progression-free survival (PFS), Overall survival (OS) [2-year]
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Time to maximum observed concentration serum Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		
 Area under the plasma concentration-time curve Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013 		
Start: November 2012 Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		
Data: March 2014 (Final data collection date for primary outcome measure) then September 2016 Status: May 13, 2013		
Status : May 13, 2013		***************************************
Sponsors: CELG		
	_	CELG
Clin.Trials: NCT01736683, ACE-011-MDS-001	Clin.Trials:	NCT01736683, ACE-011-MDS-001

Source: Clinicaltrials.gov and Leerink Swann LLC estimates

ACE-536 Phas	e II trial for Treatment of Anemia in Patients with Myelodysplastic Syndromes (MDS) (A536-03)
Purpose:	Evaluate effects of ACE-536 on anemia in patients with low-intermediate-1 risk MDS
# Pts:	N=60
Design:	Interventional, non-randomized, safety/efficacy, single group assignment, open label, treatment trial
Trial Arms:	ACE-536 Doses: Note: 2 SC every 3 weeks for up to 5 cycles
	Arm-1: 0.125mg/kg
	Arm-2 : 0.25mg/kg
	Arm-3: 0.5mg/kg
	Arm-4 : 0.75mg/kg
	Arm-5: 1mg/kg (enrolling as of 11.6.13)
Primary End	% patients with modified erythroid response (mHI-E) [at ~28 weeks from screening] mHI-E defined as:
Point:	 Non-transfusion Dependent: Hgb increase ≥1.5g/dL vs. baseline for ≥14 days (in absence of RBC transfusions)
	 Transfusion Dependent Patients: ≥50% reduction in RBC units transfused vs. pretreatment
Secondary	# patients with AEs [from treatment initiation to end-of-study visit ~28 weeks later]
End Points:	 Rates of erythroid, neutrophil and platelet (HI-E, HI-N and HI-P) responses [measured during any 8 week period on
	study, up to 28 weeks from patient screening, vs. the 8-week period prior to study day 1]
	■ Time to mHI-E response [measured over course of study, up to ~24 weeks from initiation of dosing on study day 1]
	• Frequency of RBC transfusions in transfusion-dependent patients [~28 weeks from patient screening]
	 ACE-536 pharmacokinetics (serum half-life, peak serum concentration, time to peak concentration, etc.) [measured
011	at multiple time points over course of treatment, from study day 1 to ~24 weeks]
Start:	January 2013
Data:	July 2014 (Final data collection date for primary outcome measure) then November 2014
Status:	Recruiting as of early 3Q13
Sponsors:	Acceleron
Clin.Trials:	NCT01749514, A536-03

Source: Clinicaltrials.gov and Leerink Swann LLC estimates



VALUATION

XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). We arrive at a 12-month price target of XLRN shares of ~\$33 a share based on a discounted cash flow analysis. We apply a discount rate of 10% and a terminal growth rate of 1%, which translates to an 11x terminal multiple, which we believe is comparable to biotechnology companies in a similar development stage.

RISKS TO VALUATION

An investment in XLRN is fundamentally a high-risk, high-reward investment, in our opinion. XLRN may face significant clinical, regulatory, and commercial risks for pipeline products. Most important is clinical risk for Phase II Sotatercept and ACE-536 trials in MDS and β -Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS, β -Thal. and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.

						XLRN P&L (\$	000s excent	per share d	ata)										
	2012A	1Q13A	2Q13A	3Q13A	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E
Revenues	ZUIZA	IQIOA	ZQIOA	JULION	4013L	20132	20142	20132	2010L	20172	2010L	20132	ZUZUL	20212	ZUZZE	ZUZUL	2024L	ZUZUL	2020L
Sotatercept/ACE-536 WW Revenue in MDS to CELG											\$66,089	\$141.589	\$227.505	\$324.937	\$435.091	\$559,283	\$698,955	\$855,680	\$1,031,180
											,	. ,	. ,	* - ,	,			,	
Probability of Success											32%	32%	32%	32%	32%	32%	32%	32%	32%
Risk Adjusted Sotatercept/ACE-536 WW Revenue											\$21,148	\$45,308	\$72,801	\$103,980	\$139,229	\$178,971	\$223,666	\$273,818	\$329,978
Risk Adjusted Sotatercept/ACE-536 WW Royalties in MDS											\$4,230	\$9,515	\$16,016	\$23,915	\$32,023	\$42,953	\$55,916	\$68,454	\$82,494
Sotatercept/ACE-536 WW Revenue in NTD β-Thal. to CELG											\$3,659	\$60,642	\$127,092	\$226,546	\$339,098	\$466,097	\$609,020	\$767,222	\$904,639
Probability of Success											32%	32%	32%	32%	32%	32%	32%	32%	32%
Risk Adjusted Sotatercept/ACE-536 WW Revenue in NTD β-Thal.											\$1,171	\$19,405	\$40,669	\$72,495	\$108,511	\$149,151	\$194,886	\$245,511	\$289,485
Risk Adjusted Sotatercept/ACE-536 WW Royalties in NTD β-Thal.											\$234	\$3,881	\$8,541	\$15,949	\$23,873	\$34,305	\$46,773	\$58,923	\$72,371
Dalantercept WW Revenue in 2nd-line RCC											\$68.061	\$131.647	\$210.325	\$298,864	\$398,173	\$509,233	\$633,101	\$770.918	\$877.863
											,		,					,	** /***
Probability of Success											32%	32%	32%	32%	32%	32%	32%	32%	32%
Risk Adjusted Dalantercept WW Revenue in 2nd-line RCC											\$21,780	\$42,127	\$67,304	\$95,637	\$127,415	\$162,954	\$202,592	\$246,694	\$280,916
Collaboration Revenue						-	-												
License and milestone (Risk Adjusted beyond approval)	\$9,696	\$12,515	\$22,891	-	\$15,000	\$50,406	\$40,000	-	\$40,000	\$25,000	\$22,400	\$6,400	-	\$6,400	-	\$6,400	\$6,400	\$6,400	\$6,400
Cost-Sharing, Net	\$5,558	\$2,497	\$3,537	-	\$3,000	\$9,034													
Contract Manufacturing	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Collaboration Revenue				\$4,270															
Total Revenue	\$15,254	\$15,012	\$26,428	\$4,270	\$18,000	\$59,440	\$40,000	-	\$40,000	\$25,000	\$48,643	\$61,923	\$91,861	\$141,901	\$183,311	\$246,612	\$311,681	\$380,471	\$442,182
Costs and Expenses																			
Probability Adjusted Dalantercept COGS	-	-	-	-	-	-	-	-	-	-	\$3,267	\$6,319	\$10,096	\$9,564	\$12,742	\$16,295	\$20,259	\$24,669	\$28,092
Research and Development	\$35,319	\$8,780	\$8,911	\$8,143	\$9,500	\$35,334	\$38,867	\$42,754	\$47,030	\$51,733	\$25,866	\$27,160	\$28,518	\$29,943	\$31,441	\$33,013	\$34,663	\$36,396	\$38,216
SG&A (Risk Adjusted from Time of Dalantercept Launch)	\$8,824	\$3,096	\$3,365	\$3,011	\$3,950	\$13,422	\$14,764	\$16,241	\$17,865	\$19,651	\$26,051	\$28,656	\$30,949	\$32,496	\$34,121	\$35,827	\$37,618	\$39,499	\$41,474
Total Costs and Expenses	\$44,143	\$11,876	\$12,276	\$11,154	\$13,450	\$48,756	\$53,632	\$58,995	\$64,894	\$71,384	\$55,184	\$62,135	\$69,562	\$72,003	\$78,303	\$85,135	\$92,541	\$100,565	\$107,782
Operating Income (EBIT)	(\$28,889)	\$3,136	\$14,152	(\$6,884)	\$4,550	\$14.954	(\$13,632)	(\$58.995)	(\$24.894)	(\$46,384)	(\$6.541)	(\$212)	\$22,299	\$69.898	\$105.008	\$161,477	\$219,140	\$279.906	\$334,400
Y/Y growth	(4_5,555)	,	,	(**,****)	,		(***)	(,,,,,,,	(,_ ,,,,,	(+15,557)	(,,,,,,,	(*/	, , , , , , , , , , , , , , , , , , , ,		, , , , , , , , , , , , , , , , , , , ,				,
Other Income (Expenses)	(\$2,255)	(\$1,066)	(\$356)	(\$11,629)	_	(\$13,051)	_	_	_		_	_			_	_	_	_	_
Interest Income	(\$2,233) \$91	\$12	(\$336) \$8	(φ11,029)	-	(\$13,031)	-	-	-	-	-	-		-	-	-	-	-	-
Interest Expense	(\$1,529)	(\$435)	(\$726)	_	(\$726)	(\$1,887)	(\$1,278)	(\$521)	_		_	_			_	_	_	_	_
Income Before Taxes	(\$32,582)	\$1,647	\$13,078	(\$18,513)	\$3,824	\$36	(\$14,909)	(\$59,515)	(\$24,894)	(\$46,384)	(\$6,541)	(\$212)	\$22,299	\$69,898	\$105,008	\$161,477	\$219,140	\$279,906	\$334,400
Provision for Taxes	(\$32,362)	\$1,047	\$13,076	(\$10,313)	\$3,024	\$30	(\$14,505)	(\$35,313)	(\$24,054)	(\$40,364)	(\$0,541)	(\$212)	\$22,233	\$05,050	\$105,000	12.873	74,508	95.168	113.696
Tax Rate							0%	0%	0%	0%	0%	0%	0%	0%	0%	12,073	34%	34%	34%
Net income	(\$32.582)	\$1.647	\$13.078	(\$18.513)	\$3.824	\$36	(\$14.909)	(\$59.515)	(\$24.894)		(\$6.541)	(\$212)	\$22,299	\$69.898	\$105.008	\$148,604	\$144.633	\$184.738	\$220.704
Change in fair value of warrants	\$2,258	\$1,047	\$433	(\$10,313)	\$3,024	\$1,500	(\$14,505)	(403,010)	(\$24,034)	(\$40,364)	(\$0,541)	(\$212)	\$ZZ,Z33	\$05,050	\$105,000	\$140,004	\$144,033	\$104,730	\$220,704
Change in fall value of warrants	\$2,236	\$1,007	φ433			\$1,500													
EPS (LPS) Basic	(\$1.44)	\$0.13	\$0.64	(\$0.66)	\$0.13	\$0.25	(\$0.52)	(\$1.87)	(\$0.77)	(\$1.43)	(\$0.20)	(\$0.01)	\$0.67	\$2.07	\$3.08	\$4.31	\$4.16	\$5.26	\$6.22
EPS (LPS) Diluted	(\$1.44)	\$0.12	\$0.59	(\$0.66)	\$0.13	\$0.18	(\$0.52)	(\$1.87)	(\$0.77)	(\$1.43)	(\$0.20)	(\$0.01)	\$0.67	\$2.07	\$3.08	\$4.31	\$4.16	\$5.26	\$6.22
Basic Shares (000)	21,062	20,954	20,954	28,100	28,381	6,138	28,665	31,809	32,127	32,448	32,772	33,100	33,431	33,765	34,103	34,444	34,789	35,136	35,488
Diluted Shares (000)	21.062	22.971	22,971	28,100	28.381	8,429	28.665	31.809	32,127	32,448	32,772	33.100	33,431	33,765	34,103	34,444	34,789	35.136	35.488

Source: Leerink Swann estimates and company reports.

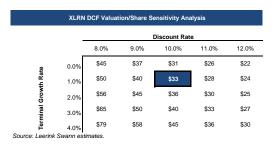
Note: Basic and Diluated shares outstanding are pro forma for IPO priced 9/18/13.

NTD=non-transfusion dependent.

DCF Calcuation

Discount rate	10%
Terminal Growth Rate	1%
Valuation (\$M)	\$1,067
Valuation / Share	\$33

Source: Leerink Swann estimates.





Disclosures Appendix Analyst Certification

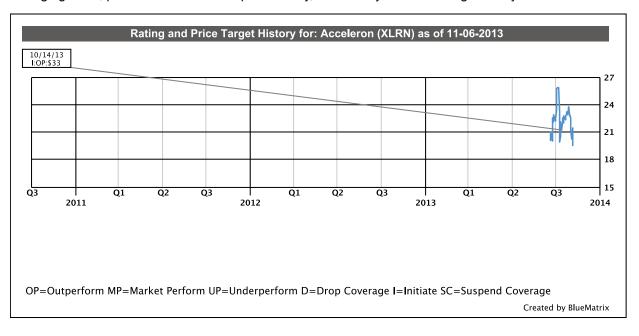
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

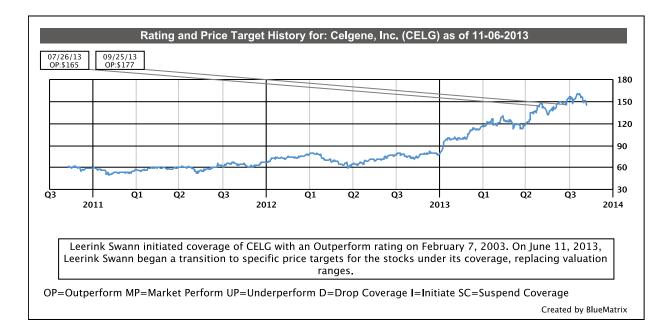
XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). We arrive at a 12-month price target of XLRN shares of ~\$33 a share based on a discounted cash flow analysis. We apply a discount rate of 10% and a terminal growth rate of 1%, which translates to an 11x terminal multiple, which we believe is comparable to biotechnology companies in a similar development stage.

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			Mos.
nt	Percent	Count	Percent
	64.90 35.10	27 0	24.00 0.00 0.00
	int 111 60 0	11 64.90	11 64.90 27 60 35.10 0

Explanation of Ratings

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

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

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

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

Important Disclosures

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Leerink Swann LLC makes a market in Acceleron and Celgene, Inc.

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