

Reason for report:

ESTIMATE CHANGE

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LEERINK SWANN

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## ACCELERON

Moving On Up; Meaningful 2g Hg Increase for Sotatercept in  $\beta$ -Thal; New \$35PT

• **Bottom Line:** ASH-2013 updates suggest Sotatercept is beginning to achieve meaningful hemoglobin (Hg) increases in  $\beta$ -Thalassemia ( $\beta$ -Thal.) patients with potential to improve with dose escalation and biomarker leverage. Sotatercept is beginning to demonstrate a clinically meaningful 2g Hg increase in  $\beta$ -Thal. patients. Responses and duration are likely to improve with continued dose escalation (+0.75mg/kg), and potential outliers identified through a ongoing efforts to prospectively use a biomarker and its correlation. Based on ASH updates, we are increasing our Sotatercept probability of success in  $\beta$ -Thal to ~40% (from low 30%<sup>s</sup>) and increasing out price target (PT) to \$35 (from \$33) and reiterating an Outperform (OP) rating.

• **Sotatercept begins to deliver on aim for clinically meaningful 2g Hg increase in  $\beta$ -Thal. Patients.** Phase II interim data from the ongoing trial demonstrate dose dependent increases in Hg in non-transfusion dependent (NTD)  $\beta$ -thal. patients including new interim data from the 0.5mg/kg dose and longer term data on the 0.1mg/kg and 0.3mg/kg dose levels. The trial is currently dosing patients at 0.75mg/kg. In terms of efficacy, dose dependent increases in Hg were observed across 3 dose levels. Within the first 2 months of dosing: 1) 84% of NTD in 0.5 and 0.3mg/kg dose levels achieved  $\geq 1$ g/dL Hg increase; 2) no non-transfusion dependent (NTD) patients at 0.1-0.3mg/kg achieved this threshold; 3) 33%, 16% and 0% of NTD patients achieved a Hg increase of  $\geq 2$ g/dL in the 0.5, 0.3, and 0.1mg/kg dose levels, respectively; 4) 5/6 NTD patients treated at 0.3mg/kg experienced sustained increase in Hg through  $\geq 10$  treatment cycles (3 weeks/cycle); 5) a statistically significant relationship ( $p < 0.001$ ) was observed between drug exposure and maximum increase in Hg during the first 3 cycles across all 3 dose levels tested. In terms of safety, Sotatercept was generally safe and well tolerated in patients at all dose levels evaluated.

• **Next Steps include showing more 2g response with dose escalation (+0.75mg/kg), prolonged duration of response with dose escalated patients and eventual biomarker identification and correlation.** With 33% (2/6) of 0.5mg/kg treated patients achieving a 2g/dl Hg increase, we anticipate this efficacy benefit will increase further at the 0.75 and potential 1g/kg cohorts. Safety appears fairly unremarkable except 2 patients that experience too much benefit and achieved  $>11$ g/dl Hg increases leading to a 1 and 3 week subsequent dose cycle prolongation before continuing the normal protocol. Despite these patients quickly overshooting their target responses and needing a small temporary dose frequency adjustment, we do not foresee this as a dose escalation obstacle. Furthermore, ongoing interest in correlating a potential predictive biomarker could quickly neutralize this hypothetical issue. Biomarker data is likely going to be available ~YE14 at ASH.

## Key Stats:

(NASDAQ:XLRN)

S&P 600 Health Care Index: 1,277.21  
Price: \$27.24

Price Target: \$35.00 from \$33.00

Methodology: DCF with 10% discount rate & 1% terminal growth rate

52 Week High: \$28.90

52 Week Low: \$15.00

Shares Outstanding (mil): 2.4

Market Capitalization (mil): \$65.4

Book Value/Share: \$16.68

Cash Per Share: \$16.29

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	\$15.0A	\$26.4A	\$4.3A	\$18.0	\$59.4	\$0.13A	\$0.64A	(\$0.66)A	\$0.13	\$0.25	NM
2014E	--	--	--	--	\$40.0	--	--	--	--	(\$0.52)	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) Preliminary Dalantercept Phase II RCC data in 1Q14; 2) Top-line Sotatercept and ACE-536 Phase II MDS and  $\beta$ -Thal. (4 trials) data at EHA in 2Q14; 3) Final Sotatercept and ACE-536 Phase II MDS and  $\beta$ -Thal. (4 trials) data at ASH in 4Q14; 4) Initiate pivotal MDS and/or  $\beta$ -Thal. trials by YE14. MEDACorp KOLs are very bullish and encouraged by emerging pipeline data and science. We assume probability of success in the low 30% for Sotatercept/ACE-536 in MDS, 40% for  $\beta$ -Thal. and low 30% for Dalantercept in 2nd-line RCC.

### Change in Estimates

We modified our model based on incrementally positive Phase II interim results of Sotatercept in  $\beta$ -Thal. released on 12.9.13. Our probability of success for Sotatercept in  $\beta$ -Thal. increased from low 30% to 40%.

### MILESTONES

Product	Partner	Indication	Timing	Milestone
Sotatercept (ACE-011)	CELG	MDS + $\beta$ -Thal.	YE13	Initiate Phase II Expansion Cohort for Sotatercept $\beta$ -Thal.
			1Q14	Initiate Phase II in ESRD CKD
			2Q14	Phase II dose escalation Sotatercept MDS + $\beta$ -Thal. data at EHA-2014
			4Q14	Final Phase II Sotatercept in MDS + $\beta$ -Thal. data
			2018	Approval and launch
ACE-536			YE14	Initiate Phase III trial for MDS and/or $\beta$ -Thal.
			1Q14	Initiate Phase II Expansion Cohort for II ACE-536 $\beta$ -Thal.
			2Q14	Phase II dose escalation data for ACE-536 MDS and $\beta$ -Thal. at EHA-2014
			4Q14	Final Phase II ACE-536 in MDS and $\beta$ -Thal. data
			YE14	Initiate Phase III trial for MDS and/or $\beta$ -Thal.
Dalantercept (ACE-041)	Proprietary	Oncology	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)
				GOG Ovarain Cancer single agent trial Go-No-Go to Part-2 of trial
			1Q14	Initiate Phase II (Part-2, N=112) RCC randomized trial (PFS endpoint)
			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- $\beta$ Candidates		Muscle	2014	Advance Muscle Loss candidate into clinic (ACE-083)
		Fibrosis	2015	Advance Fibrosis (i.e., PAH) candidate into clinic

Source: Company Reports, Leerink Swann LLC estimates



## β-Thalassemia: Sotatercept & ACE-536

<b>Phase IIa Sotatercept (ACE-011) Safety/Tolerability Trial in Adults With β- Thalassemia (ACE-011-B-Thal-001):</b>	
<b>Purpose:</b>	Dose finding study to determine safety/tolerability of ACE-011 in β-Thalassemia adults
<b># Pts:</b>	<b>N=45</b>
<b>Design:</b>	Interventional Non-Randomized, Safety/Efficacy Study, Single Group Assignment, Open Label, treatment trial
<b>Trial Arms:</b>	<b>Arm-1:</b> 0.1mg/kg <b>Arm-2:</b> 0.3mg/kg <b>Arm-3:</b> 0.5mg/kg <b>Arm-4:</b> 0.75mg/kg <b>Note:</b> All administered as a subcutaneous injection once every 21 days during treatment period <b>As of 12.9.13, enrolling patients in 4<sup>th</sup> cohort at 0.75mg</b>
<b>Primary End Point:</b>	<b>Potential Recommended Dose (PRD)</b> [Up to 27 months] [Designated as safety issue] <ul style="list-style-type: none"> <li>PRD determined following assessment of efficacy/safety parameters based on 1<sup>st</sup> of 3 doses of Sotatercept administered, up to at least 21 days following 1<sup>st</sup> dose, for all doses evaluated</li> <li>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. <b>Efficacy defined as:</b> <ul style="list-style-type: none"> <li><b>Transfusion Dependent (TD) Patients:</b> Reduction of transfusion burden by ≥20% vs. calculated baseline transfusion burden to each subject</li> <li><b>Non-Transfusion Dependent (NTD) Patients:</b> Increase in Hgb ≥1g/dl vs. baseline Hgb, sustained for 12 weeks</li> </ul> </li> </ul> <b>Actual Recommended Dose (RD)</b> [Up to 27 months] [Designated as safety issue] <ul style="list-style-type: none"> <li>RD defined based on review of efficacy/safety parameters + dose modification data. <b>Efficacy defined as:</b> <ul style="list-style-type: none"> <li><b>Transfusion Dependent Patients:</b> Reduction of transfusion burden by ≥20% vs. calculated baseline transfusion burden to each subject</li> <li><b>Non-Transfusion Dependent Patients:</b> Increase in Hgb ≥1 g/dl vs. baseline Hgb, sustained for 12 weeks</li> </ul> </li> </ul>
<b>Secondary End Points:</b>	<ul style="list-style-type: none"> <li>RBC Transfusion Burden [Up to 27 months] [Designated as safety issue]</li> <li>Reduction of transfusion burden by ≥20% vs. calculated baseline transfusion burden for transfusion dependent Beta Thalassemia major and Beta Thalassemia Intermedia subjects</li> <li># participants with AEs [Up to 27 months] [Designated as safety issue]</li> <li># participants with AEs</li> <li>PK-Serum Concentration of Sotatercept, PK-Observed Maximum Concentration (C<sub>max</sub>), PK-Time to Maximum Concentration (T<sub>max</sub>), PK-Area Under Concentration-Time Curve (AUC), PK-Concentration of Anti-Sotatercept Antibody in Serum</li> <li>Hgb Level Increase [Up to 27 months]</li> <li>Hgb level increase during study treatment vs. baseline Hgb level in non-transfusion dependent Beta Thalassemia intermedia subjects</li> </ul>
<b>Start:</b>	March 2012
<b>Data:</b>	<b>January 2014</b> (Final data collection date for primary outcome measure) then June 2014
<b>Status:</b>	May 16, 2013
<b>Sponsors:</b>	CELG
<b>Clin. Trials:</b>	NCT01571635, <b>ACE-011-B-THAL-001, 2011-005659-15</b>

Source: Clinicaltrials.gov and Leerink Swann LLC estimates

<b>Phase II Trial to evaluate Effects of ACE-536 in Patients with β-Thalassemia Intermedia (A536-04):</b>	
<b>Purpose:</b>	Evaluate effects of ACE-536 in patients with β-Thalassemia intermedia
<b># Pts:</b>	<b>N=50</b>
<b>Design:</b>	Interventional, Non-Randomized, Safety/Efficacy Study, Single Group Assignment, Open Label, treatment
<b>Trial Arms:</b>	<b>Arm-1:</b> ACE-536 - 1 of 5 possible dose levels (0.2, 0.4, 0.6, 0.8, ?) <b>**Note:</b> 2 SC every 3 weeks for up to 5 cycles
<b>Primary End Point:</b>	<b>% patients with erythroid response, defined as hemoglobin increase of ≥1.5g/dL from baseline for ≥ 14 days (in absence of transfusion) [at ~24 weeks from patient screening]</b>
<b>Secondary End Points:</b>	<ul style="list-style-type: none"> <li>Number of patients with AEs [From treatment initiation to End-of-Study visit (~24 weeks later)]</li> <li>Change in hemoglobin [Baseline to ~24 weeks]</li> <li>Changes in biomarkers of erythropoiesis, hemolysis, iron metabolism, bone metabolism [Baseline to ~24 weeks]</li> <li>ACE-536 pharmacokinetics [Measured multiple time points of treatment, from study day 1 to ~24 weeks]</li> </ul>
<b>Start:</b>	January 2013
<b>Data:</b>	<b>November 2014</b> (Final data collection date for primary outcome measure) then November 2014
<b>Status:</b>	Recruiting March 14, 2013
<b>Sponsors:</b>	<b>Acceleron</b>
<b>Clin. Trials:</b>	NCT01749540, <b>A536-04</b>

Source: Clinicaltrials.gov and Leerink Swann LLC estimates



## MDS: Sotatercept & ACE-536

<b>ACE-011 Phase II Anemia in low-interm-1 Risk Myelodysplastic Syndromes (MDS) Trial (ACE-011-MDS-001):</b>	
<b>Purpose:</b>	Determine safe, tolerable and effective dose of Sotatercept to treat anemia in patients with low/intermediate-1 risk MDS or non-proliferative chronic myelomonocytic leukemia (CMML)
<b># Pts:</b>	<b>N=100</b> (low-Int-1 patients who are transfusion dependent or hemoglobin <10g/dL)
<b>Design:</b>	Randomized, safety/efficacy, parallel, open label, treatment trial
<b>Trial Arms:</b>	<b>Sotatercept Doses:</b> All arms administered SC once every 3 weeks (q3W) for 5 cycles <b>Arm-1:</b> ACE-011 0.1mg/kg <b>Arm-2:</b> ACE-011 0.3mg/kg <b>Arm-3:</b> ACE-011 0.5mg/kg <b>Arm-4:</b> ACE-011 0.75mg/kg (enrolling as of 11.6.13)
<b>Primary End Point:</b>	<b>Erythroid Hematological Improvement (HI-E) [Up to 24 weeks]</b> <ul style="list-style-type: none"> <li><b>Rare transfused patients (requiring transfusion &lt;4 units RBCs):</b> % patients with increase in Hgb <math>\geq 1.5</math>g/dL over 8 weeks in absence of RBC transfusion</li> <li><b>Heavily transfused patients (requiring transfusion <math>\geq 4</math> RBCs units):</b> % patients with 50% decrease in units transfused over 8 weeks</li> </ul>
<b>Secondary End Points:</b>	<ul style="list-style-type: none"> <li>Adverse Event (AEs) up to 3 years, Number of participants with AEs</li> <li>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</li> <li>Duration to HI-E [Up to 24 weeks] Length of time between first and last assessment of HI-E</li> <li>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]</li> <li>Time from baseline until progression to AML, until progression to events of higher risk MD</li> <li>Number of participants who survive without progressing</li> <li>Pharmacokinetics-Cmax, Tmax, AUC [Up to 24 weeks]</li> <li>Max concentration in serum</li> <li>Time to maximum observed concentration serum</li> <li>Area under the plasma concentration-time curve</li> </ul>
<b>Start:</b>	November 2012
<b>Data:</b>	March 2014 (Final data collection date for primary outcome measure) then September 2016
<b>Status:</b>	May 13, 2013
<b>Sponsors:</b>	CELG
<b>Clin.Trials:</b>	NCT01736683, <b>ACE-011-MDS-001</b>

Source: Clinicaltrials.gov and Leerink Swann LLC estimates

<b>ACE-536 Phase II trial for Treatment of Anemia in Patients with Myelodysplastic Syndromes (MDS) (A536-03)</b>	
<b>Purpose:</b>	Evaluate effects of ACE-536 on anemia in patients with low-intermediate-1 risk MDS
<b># Pts:</b>	<b>N=60</b>
<b>Design:</b>	Interventional, non-randomized, safety/efficacy, single group assignment, open label, treatment trial
<b>Trial Arms:</b>	<b>ACE-536 Doses:</b> Note: 2 SC every 3 weeks for up to 5 cycles <b>Arm-1:</b> 0.125mg/kg <b>Arm-2:</b> 0.25mg/kg <b>Arm-3:</b> 0.5mg/kg <b>Arm-4:</b> 0.75mg/kg <b>Arm-5:</b> 1mg/kg (enrolling as of 11.6.13)
<b>Primary End Point:</b>	<b>% patients with modified erythroid response (mHI-E) [at ~28 weeks from screening]</b> mHI-E defined as: <ul style="list-style-type: none"> <li><b>Non-transfusion Dependent:</b> Hgb increase <math>\geq 1.5</math>g/dL vs. baseline for <math>\geq 14</math> days (in absence of RBC transfusions)</li> <li><b>Transfusion Dependent Patients:</b> <math>\geq 50\%</math> reduction in RBC units transfused vs. pretreatment</li> </ul>
<b>Secondary End Points:</b>	<ul style="list-style-type: none"> <li># patients with AEs [from treatment initiation to end-of-study visit ~28 weeks later]</li> <li>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]</li> <li>Time to mHI-E response [measured over course of study, up to ~24 weeks from initiation of dosing on study day 1]</li> <li>Frequency of RBC transfusions in transfusion-dependent patients [~28 weeks from patient screening]</li> <li>ACE-536 pharmacokinetics (serum half-life, peak serum concentration, time to peak concentration, etc.) [measured at multiple time points over course of treatment, from study day 1 to ~24 weeks]</li> </ul>
<b>Start:</b>	January 2013
<b>Data:</b>	<b>July 2014</b> (Final data collection date for primary outcome measure) then November 2014
<b>Status:</b>	Recruiting as of early 3Q13
<b>Sponsors:</b>	Acceleron
<b>Clin.Trials:</b>	NCT01749514, <b>A536-03</b>

Source: Clinicaltrials.gov and Leerink Swann LLC estimates



## VALUATION

We arrive at a new 12-month price target of XLRN shares of \$35 a share (previously \$33) based on incrementally positive Phase II interim results of Sotatercept in  $\beta$ -Thal. released on 12.9.13. As a result, we increase our probability of success for Sotatercept in  $\beta$ -Thal. from Our valuation is based on a discounted cash flow analysis. 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 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  $\beta$ -Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS,  $\beta$ -Thal. and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.

	XLRN P&L (\$000s, except per share data)																		
	2012A	1Q13A	2Q13A	3Q13A	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E
Revenues																			
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											40%	40%	40%	40%	40%	40%	40%	40%	40%
Risk Adjusted Sotatercept/ACE-536 WW Revenue in NTD β-Thal.											\$1,464	\$24,257	\$50,837	\$90,619	\$135,639	\$186,439	\$243,608	\$306,889	\$361,856
Risk Adjusted Sotatercept/ACE-536 WW Royalties in NTD β-Thal.											\$293	\$4,851	\$10,676	\$19,936	\$29,841	\$42,881	\$58,466	\$73,653	\$90,464
Dalantcept 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 Dalantcept 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	\$48,702	\$62,893	\$93,996	\$145,888	\$189,279	\$255,188	\$323,374	\$395,202	\$460,275
Costs and Expenses																			
Probability Adjusted Dalantcept 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 Dalantcept 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)	(\$6,482)	\$758	\$24,434	\$73,885	\$110,976	\$170,053	\$230,834	\$294,636	\$352,492
Y/Y growth																			
Other Income (Expenses)	(\$2,255)	(\$1,066)	(\$356)	(\$11,629)	-	(\$13,051)	-	-	-	-	-	-	-	-	-	-	-	-	-
Interest Income	\$91	\$12	\$8																
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)	(\$6,482)	\$758	\$24,434	\$73,885	\$110,976	\$170,053	\$230,834	\$294,636	\$352,492
Provision for Taxes																			
Tax Rate						0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	12%	34%	34%	34%
Net income	(\$32,582)	\$1,647	\$13,078	(\$18,513)	\$3,824	\$36	(\$14,909)	(\$59,515)	(\$24,894)	(\$46,384)	(\$6,482)	\$758	\$24,434	\$73,885	\$110,976	\$149,804	\$152,350	\$194,460	\$232,645
Change in fair value of warrants	\$2,258	\$1,067	\$433			\$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)	\$0.02	\$0.73	\$2.19	\$3.25	\$4.35	\$4.38	\$5.53	\$6.56
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)	\$0.02	\$0.73	\$2.19	\$3.25	\$4.35	\$4.38	\$5.53	\$6.56
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 Diluted shares outstanding are pro forma for IPO priced 9/18/13.

NTD=non-transfusion dependent.

## DCF Calculation

Discount rate	10%
Terminal Growth Rate	1%
Valuation (\$M)	\$1,121
<b>Valuation / Share</b>	<b>\$35</b>

Source: Leerink Swann estimates.

## XLRN DCF Valuation/Share Sensitivity Analysis

		Discount Rate				
		8.0%	9.0%	10.0%	11.0%	12.0%
Terminal Growth Rate	0.0%	\$47	\$39	\$32	\$27	\$23
	1.0%	\$52	\$42	<b>\$35</b>	\$29	\$25
	2.0%	\$59	\$47	\$38	\$32	\$27
	3.0%	\$68	\$53	\$42	\$34	\$29
	4.0%	\$83	\$61	\$48	\$38	\$31

Source: Leerink Swann estimates.



## Disclosures Appendix

### Analyst Certification

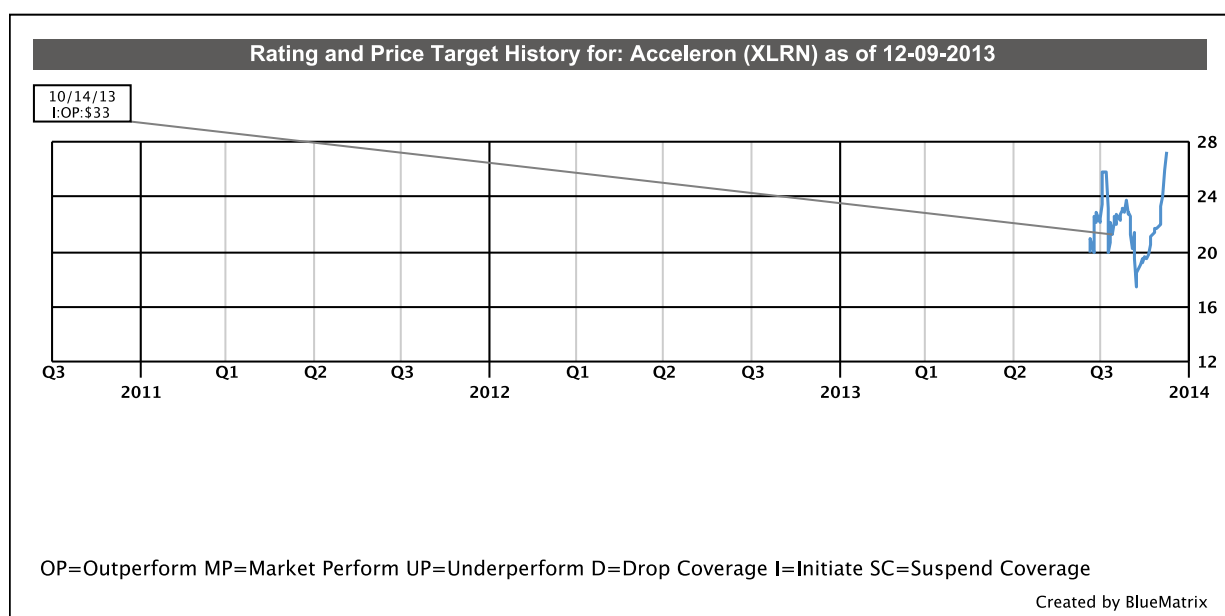
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

We arrive at a new 12-month price target of XLRN shares of \$35 a share (previously \$33) based on incrementally positive Phase II interim results of Sotatercept in  $\beta$ -Thal. released on 12.9.13. As a result, we increase our probability of success for Sotatercept in  $\beta$ -Thal. from Our valuation is based on a discounted cash flow analysis. 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 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  $\beta$ -Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS,  $\beta$ -Thal. and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.

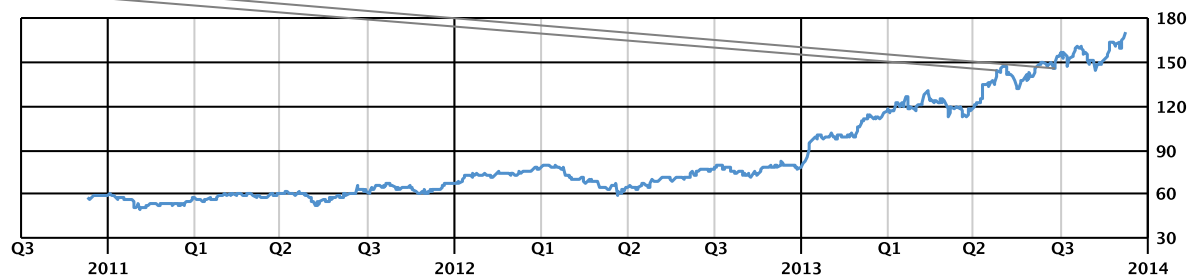




### Rating and Price Target History for: Celgene, Inc. (CELG) as of 12-09-2013

07/26/13  
OP:\$165

09/25/13  
OP:\$177



Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix





Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	111	64.90	27	24.00
HOLD [MP]	60	35.10	0	0.00
SELL [UP]	0	0.00	0	0.00

## Explanation of Ratings

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

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

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

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



## Important Disclosures

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In the past 12 months, the Firm has received compensation for providing investment banking services to Acceleron.

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

In the past 12 months, an affiliate of the Firm, Leerink Swann Consulting LLC, has received compensation for providing non-securities services to: Celgene, Inc.

Leerink Swann LLC has acted as the manager for a public offering of Acceleron in the past 12 months.

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