

Reason for report:

PROPRIETARY INSIGHTS

ACCELERON PHARMA

MEDACorp Feedback/Our Analysis of NEW SOT/ACE-536 Data at EHA; Reit OP & \$52 PT

• **Bottom Line:** We reviewed available EHA abstracts with MEDACorp KOLs and provide our analysis that follows XLRN's EHA press release (see our [XLRN 5.21.14 note](#)). KOL feedback and our analysis of new ACE-536 Myelodysplastic Syndrome (MDS) data reinforce that it is particularly impressive for Transfusion Dependent (TD) patients and key is that no SAEs were observed in the trial. For the new ACE-536 β -Thal., our analysis again reinforces that emerging data are impressive in both TD and non-TD (NTD) patients, again with favorable safety and no SAEs. Sotatercept (SOT) β -Thal data will be presented at the meeting (presentations 6.14.14; 8:15/8:45 CEST). Between ASCO Dalantercept data & EHA ACE/536/SOT updates, we believe shares will appreciate near term and we reiterate our Outperform rating & \$52 price target (PT).

• **Our analysis of new ACE-536 MDS data suggest it is particularly impressive for TD and it is key that no SAEs were observed in the trial (presentation 6.15.14; 8:45 CEST).** In terms of efficacy, 40% (4/10) experienced $\geq 50\%$ reduction in transfused units during an 8-week interval on ACE-536 vs. the 8 weeks prior to treatment with 1 previously non-responsive to ESA/Revlimid who became transfusion-free for ~22 weeks during the trial. We believe the TD are particularly strong given mean baseline units of RBCs in prior 8 weeks was 6.2 units suggesting this was a relatively heavily transfused cohort of patients. For the 60% (3/5) of NTD patients treated at 0.75mg/kg that were either ESA refractory or non-responders, we view the maximum Hgb increases of 1.6 and 1.9 meaningful while the 3.3g/dL increase is impressive. While the abstract describes transient increases in reticulocytes and/or neutrophils in some patients, we are hopeful the mature data at EHA for these dose groups and the 1mg/kg cohort will improve. More specifically, at EHA and then ASH, we hope to see reticulocyte counts that cycle with ACE-536 administration and that sustain hematocrit levels without tachyphylaxis. In terms of safety, it is very important that ACE#536 was generally well tolerated, there were no SAEs, and no patients discontinued treatment early due to an ACE-536 related AE.

• **Our analysis of new ACE-536 β -Thal. also suggest data are and will be impressive for both TD and NTD patients, again in the context of favorable safety with no SAEs (SOT β -Thal data will be presented at meeting) (presentations 6.14.14; 8:15/8:45 CEST).** Overall, encouraging abstract data were observed in β -Thal-intermedia patients (rather than major). For TD patients, 100% responded by experiencing transfusion reductions but by how much will be key at EHA. For NTD patients the trend in the first 3 dose escalation cohorts is encouraging, and we would look for the 0.8mg/kg dose to show Hgb increased $>1.4\text{g/dL}$ and ideally in the 1.8-2.0g/dL without a plateau effect. XLRN is currently enrolling 1 1mg/kg dose cohort that could further improve upon 0.8mg/kg data.

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	\$15.0	\$26.4	\$4.3	\$11.5	\$57.2	\$0.13	\$0.64	(\$0.66)	(\$0.64)	(\$4.15)	NM
2014E	\$3.3A	0.0	\$30.0	0.0	\$33.3	(\$0.30)A	(\$0.55)	\$0.39	(\$0.60)	(\$1.06)	NM
2015E	--	--	--	--	0.0	--	--	--	--	(\$2.29)	NM

Source: Company Information and Leerink Partners LLC Research
Revenue in MM.

GAAP EPS presented.

Key Stats:

(NASDAQ:XLRN)

S&P 600 Health Care Index:	1,251.15
Price:	\$31.23
Price Target:	\$52.00
Methodology:	DCF analysis; 10% discount rate; 1% terminal growth rate
52 Week High:	\$57.89
52 Week Low:	\$15.00
Shares Outstanding (mil):	30.3
Market Capitalization (mil):	\$946.3
Book Value/Share:	\$1.32
Cash Per Share:	\$7.06
Dividend (ann):	\$0.00
Dividend Yield:	0.0%

Cash Per Share:



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 2Q14; (2) top-line Sotatercept and ACE-536 Phase II MDS and β -Thal. (4 trials) data at EHA in 2Q14; (3) final Sotatercept and ACE-536 Phase II MDS and β -Thal. (4 trials) data at ASH in 4Q14; (4) 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 probability of success in the low 30s percent range for Sotatercept/ACE-536 in MDS, 40% for β -Thal., 20% in end-stage renal disease (ESRD) patients on hemodialysis, and low 30s percent range for Dalantercept in 2nd-line RCC.

ASCO 2014 Titles

Dalantercept:				
Abstract #	Title	Time/Session	Poster Board	Presenter
5594	Phase II evaluation of dalantercept, a soluble recombinant activin receptor-like kinase 1 (ALK1) receptor-fusion protein, for treatment of recurrent/persistent endometrial cancer: GOG-0229N.	5/31(Sat) 8-11:45am S Hall A2 General poster Track(s): Gynecologic Cancer	376	Vicky Makker, MD
6045	Phase 2 study of dalantercept in recurrent or metastatic squamous cell carcinoma of the head and neck	5/31 (Sat) 1:15-5pm S Hall A2 General poster Track(s): Head and Neck Cancer	80	Antonio Jimeno, MD, PhD
4566	A two-part phase 2 randomized study of dalantercept and axitinib versus placebo plus axitinib in advanced renal cell carcinoma: Results from the part 1 dose escalation cohorts.	6/2 (Mon) 1:15-5pm S Hall A2 General Poster Track(s): Genitourinary Cancer	134	Michael B. Atkins, MD

Source: ASCO.org, Leerink Partners estimates

Milestones

Product	Partner	Indication	Phase	Timing	Milestone		
ACE-536	CELG	MDS + β -Thal.	Ph. II	2Q14	Phase II dose escalation data for MDS + β -Thal. at EHA-2014		
				4Q14	Final Phase II in MDS and β -Thal. data		
				YE14/ early-15	Initiate Phase III trial for MDS and/or β -Thal.		
				2018	Approval and launch		
Sotatercept (ACE-011)			2Q14	Phase II dose escalation β -Thal. data at EHA-2014 + Preclinical data in sickle cell anemia			
				4Q14	Final Phase II in MDS + β -Thal. data		
				2018	Approval and launch		
				YE14/early-15	Initiate Phase III trial for MDS and/or β -Thal.		
			CELG	ESRD	Ph. II	11.11-16.14	Phase II Part-1 data (0.7mg/kg cohort+BMD/Vasc Calcificat)
Dalantercept (ACE-041)			Proprietary	Oncology	Ph. II	early-June -14	Interim Part-1 Phase II RCC combo data (ASCO-2014)
	2Q14	Initiate Phase II Dal-sorafenib combo trial in HCC					
	YE14	Preliminary Phase II Dal-sorafenib combo RR data in HCC					
	2H15	Phase II RCC Part-2 data					
	2014	Phase II data in SCCHN					
	2018	Approval and launch in RCC					
ACE-083		Muscle		PC	2H14	Advance ACE-083 into clinic for Muscle Loss	
New TGF- β Candidates		Fibrosis		PC	2015	Advance Fibrosis (i.e., PAH) candidate into clinic	

Source: Company reports, Leerink Partners estimates

VALUATION

Our \$52 12-month price target of XLRN shares reflects probability-adjusted royalty revenue at 20% from end-stage renal disease (ESRD) patients on hemodialysis. Our valuation is based on a discounted cash flow analysis. 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). 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 (myelodysplastic syndromes) 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, except per share data)																	
	2013A	1Q14A	2Q14E	3Q14E	4Q14E	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
Dalanterscept 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 Dalanterscept WW Revenue in 2nd-line RCC										\$21,780	\$42,127	\$67,304	\$95,637	\$127,415	\$162,954	\$202,592	\$246,694	\$280,916
Sotatercept US Revenue in ESRD Patients on Hemodialysis												\$301,866	\$819,107	\$1,418,781	\$1,915,379	\$2,064,199	\$2,220,226	\$2,383,756
Probability of Success												20%	20%	20%	20%	20%	20%	20%
Risk Adjusted Sotatercept US Revenue in ESRD Patients on Hemodialysis												\$60,373	\$163,821	\$283,756	\$383,076	\$412,840	\$444,045	\$476,751
Risk Adjusted Sotatercept US Royalties in ESRD Patients on Hemodialysis												\$12,075	\$36,041	\$65,264	\$91,938	\$103,210	\$111,011	\$119,188
Collaboration Revenue	\$57,231	\$3,307	-	\$30,000	-	\$33,307	-	\$40,000	\$30,000	\$22,400	\$8,000	-	\$8,000	-	\$8,000	\$8,000	\$8,000	\$8,000
Total Revenue	\$57,231	\$3,307	-	\$30,000	-	\$33,307	-	\$40,000	\$30,000	\$48,702	\$64,493	\$106,071	\$183,529	\$254,543	\$348,727	\$428,184	\$507,813	\$581,062
Costs and Expenses																		
Probability Adjusted Dalanterscept COGS	-					-	-	-	-	\$3,267	\$6,319	\$10,096	\$9,564	\$12,742	\$16,295	\$20,259	\$24,669	\$28,092
Research and Development	\$36,051	\$11,765	\$12,200	\$12,500	\$13,280	\$49,745	\$55,217	\$61,291	\$68,033	\$34,016	\$35,717	\$37,503	\$39,378	\$41,347	\$43,415	\$45,585	\$47,864	\$50,258
SG&A (Risk Adjusted from Time of Dalanterscept Launch)	\$14,227	\$3,750	\$4,500	\$5,100	\$5,200	\$18,550	\$20,405	\$22,446	\$24,690	\$31,090	\$34,199	\$36,935	\$38,782	\$40,721	\$42,757	\$44,895	\$47,139	\$49,496
Total Costs and Expenses	\$50,278	\$15,515	\$16,700	\$17,600	\$18,480	\$68,295	\$75,622	\$83,736	\$92,723	\$68,373	\$76,235	\$84,534	\$87,724	\$94,810	\$102,467	\$110,739	\$119,673	\$127,846
Operating Income (EBIT)	\$6,952	(\$12,208)	(\$16,700)	\$12,400	(\$18,480)	(\$34,988)	(\$75,622)	(\$43,736)	(\$62,723)	(\$19,671)	(\$11,742)	\$21,537	\$95,805	\$159,733	\$246,260	\$317,445	\$388,140	\$453,217
Y/Y growth																		
Other Income (Expenses)	(\$27,710)	\$3,088				-	-	-	-	-	-	-	-	-	-	-	-	-
Interest Income	\$20																	
Interest Expense	(\$1,161)		(\$378)	(\$298)	(\$219)	(\$895)	(\$521)	-	-	-	-	-	-	-	-	-	-	-
Income Before Taxes	(\$21,898)	(\$9,120)	(\$17,078)	\$12,102	(\$18,699)	(\$32,795)	(\$76,143)	(\$43,736)	(\$62,723)	(\$19,671)	(\$11,742)	\$21,537	\$95,805	\$159,733	\$246,260	\$317,445	\$388,140	\$453,217
Provision for Taxes						-	-	-	-	-	-	-	-	-	29,317	107,931	131,967	154,094
Tax Rate						0%	0%	0%	0%	0%	0%	0%	0%	0%	12%	34%	34%	34%
Net income	(\$21,898)	(\$9,120)	(\$17,078)	\$12,102	(\$18,699)	(\$32,795)	(\$76,143)	(\$43,736)	(\$62,723)	(\$19,671)	(\$11,742)	\$21,537	\$95,805	\$159,733	\$216,942	\$209,514	\$256,172	\$299,123
Net income (loss) applicable to common stockholders—diluted	(\$39,003)	(\$9,120)	(\$17,078)	\$12,102	(\$18,699)	(\$32,795)	(\$76,143)	(\$43,736)	(\$62,723)	(\$19,671)	(\$11,742)	\$21,537	\$95,805	\$159,733	\$216,942	\$209,514	\$256,172	\$299,123
Change in fair value of warrants	\$1,500																	
EPS (LPS) Basic	(\$4.15)	(\$0.30)	(\$0.55)	\$0.39	(\$0.60)	(\$1.06)	(\$2.29)	(\$1.30)	(\$1.85)	(\$0.57)	(\$0.34)	\$0.62	\$2.72	\$4.48	\$6.03	\$5.76	\$6.98	\$8.07
Basic Shares (000)	9,407	30,321	31,140	31,171	31,202	30,931	33,241	33,573	33,909	34,248	34,590	34,936	35,286	35,639	35,995	36,355	36,719	37,086

Source: Leerink Partners estimates and company reports.
NTD=non-transfusion dependent.

DCF Calculation	
Discount rate	10%
Terminal Growth Rate	1%
Valuation (\$M)	\$1,668
Valuation / Share	\$52

Source: Leerink Partners estimates.

XLRN DCF Valuation/Share Sensitivity Analysis						
		Discount Rate				
		8.0%	9.0%	10.0%	11.0%	12.0%
Terminal Growth Rate	0.0%	\$69	\$57	\$48	\$41	\$36
	1.0%	\$76	\$62	\$52	\$44	\$38
	2.0%	\$86	\$69	\$57	\$47	\$40
	3.0%	\$99	\$78	\$62	\$51	\$43
	4.0%	\$120	\$90	\$70	\$57	\$47

Source: Leerink Partners estimates.

Disclosures Appendix

Analyst Certification

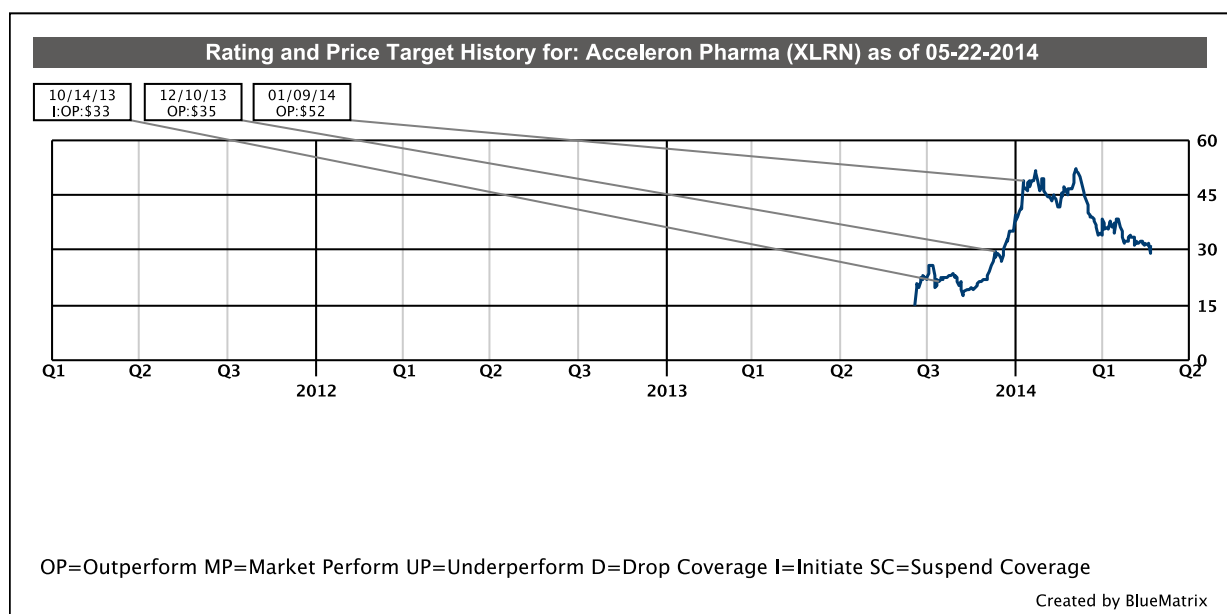
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

Our \$52 12-month price target of XLRN shares reflects probability-adjusted royalty revenue at 20% from end-stage renal disease (ESRD) patients on hemodialysis. Our valuation is based on a discounted cash flow analysis. 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). 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

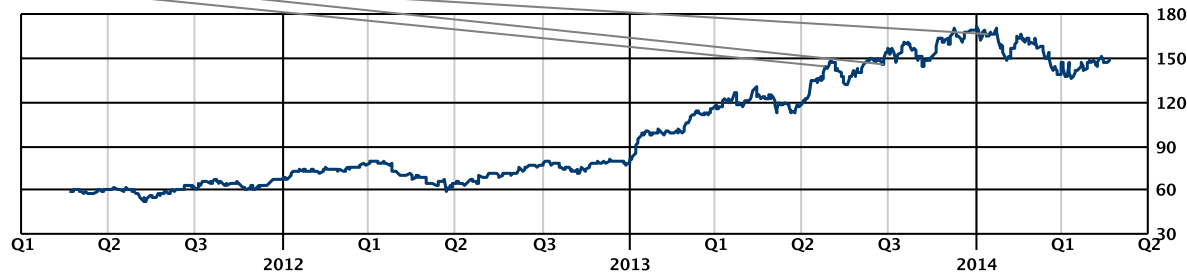
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Rating and Price Target History for: Celgene, Inc. (CELG) as of 05-22-2014

07/26/13
OP:\$165

09/25/13
OP:\$177

01/14/14
OP:\$197


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 03/31/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
SELL [UP]	0	0.00	0	0.00

Explanation of Ratings

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

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

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

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

Important Disclosures

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

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