August 13, 2014

OUTPERFORM

Reason for report:

EARNINGS

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LEERINK

ACCELERON PHARMA

2Q:14 Update -- Focus on Additional Phase II Data and Phase III Start

• **Bottom Line:** On its 2Q call, XLRN announced plans to initiate Phase III for one of the CELG-partnered compounds, sotatercept or ACE-536 (the latter appears to be the front-runner) in beta-thalassemia by mid-2015. Although this is slightly behind prior projections of late 2014/1Q:15, management noted greater certainty due to regulatory discussions that have been initiated. Management provided additional color on upcoming data presentations -- ASH data in myelodysplastic syndrome (MDS) may be particularly interesting as it pertains to the next potential Phase III indication, although there is less visibility on sotatercept vascular calcification data at ASN. For dalantercept, the company announced the initiation of a combination study with Nexavar in liver cancer, which we believe could be an interesting opportunity. We are assuming coverage of XLRN and maintaining the OP rating and $57 price target.

• **Emphasis on broader benefit of ACE-536 in beta-thalassemia -- report of second patient with leg ulcer healing.** Management noted that these represented the known cases of patients with leg ulcers.

• **ASH data could focus on MDS.** Data on both higher doses of ACE-536 and sortatercept in beta-thalassemia and MDS trials are expected at ASH. We would be especially interested in MDS data regarding transfusion independence.

• **New dalantercept study in combination with Nexavar in first-line liver cancer patients could represent an interesting opportunity.** In addition to an ongoing randomized Phase II trial (axitinib +/- dalantercept) in kidney cancer, XLRN announced that it started a combination study with Nexavar as first-line treatment for liver cancer, which we see as an interesting opportunity with high unmet need. Although normally it would be difficult to decipher the contribution of a new agent from a single arm combination study, given that there is minimal activity for Nexavar in objective response, any significant levels of response could represent signs of activity for dalantercept.

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,293.21**
Price:	**$28.43**
Price Target:	$57.00
Methodology:	NPV analysis
52 Week High:	$57.89
52 Week Low:	$15.00
Shares Outstanding (mil):	31.6
Market Capitalization (mil):	$898.4
Book Value/Share:	$1.27
Cash Per Share:	$6.47
Dividend (ann):	$0.00
Dividend Yield:	0.0%
Cash Per Share:	

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 - New	**$3.3A**	**$4.1A**	**$30.0**	**0.0**	**$37.4**	**($0.30)A**	**($0.52)A**	**$0.38**	**($0.59)**	**($1.03)**	**NM**
2014E - Old	$3.3A	0.0	$30.0	0.0	$33.3	($0.30)A	($0.55)	$0.39	($0.60)	($1.06)	NM
2015E - New	**--**	**--**	**--**	**--**	**0.0**	**--**	**--**	**--**	**--**	**($2.26)**	**NM**
2015E - Old	--	--	--	--	0.0	--	--	--	--	($2.29)	NM

Source: Company Information and Leerink Partners LLC Research
Revenue in MM; GAAP EPS presented.

Please refer to pages 5 - 8 for Important Disclosures, Price Charts and Analyst Certification.

INVESTMENT THESIS

We rate XLRN Outperform. We believe XLRN's biology expertise has generated a rich pipeline consisting of a portfolio of novel biologics that provides multiple shots on goal in cancer, nephrology and orphan diseases. We see opportunities for share appreciation as the pipeline agents advance, e.g. with the anticipated Phase III advancement of CELG-partnered compounds ACE-536 or sotatercept in beta-thalassemia in mid-2015 and potentially MDS. Sotatercept could also be a highly differentiated agent for end-stage renal disease if preclinical signal in bone loss prevention and reduction of vascular calcification plays out. The wholly owned proprietary anti-angiogenic agent dalantercept (ACE-041) appears differentiated in its combinability, has shown an initial efficacy signal in kidney cancer and could have a role in liver cancer where we see a continued unmet need. Beyond the clinical candidates, XLRN has a preclinical pipeline that could soon deliver additional interesting clinical-stage candidates, such as ACE-083 for muscle loss.

Model update. XLRN reported $4.1M in 2Q:14 revenues and $17.3M in operating expenses vs. our estimates of $0.0M/$16.7M. Net income/EPS were ($16.6M/($0.52) vs. our estimates of ($17.1M/$0.55). The company ended the quarter with $204.3M in cash, which should be sufficient to fund operations into 2H:17. We are updating our model to reflect these changes. As a result, our estimate for 2014 revenue changes from $33.3M to $37.4M and EPS changes from ($1.06) to ($1.03).

XLRN – Upcoming Catalysts

Product	Partner	Indication	Phase	Timing	Milestone
ACE-536 / Sotatercept (ACE-011)	CELG	MDS/ β-Thal	Ph. II	4Q14	Phase II in MDS and β-Thal. data
				Mid-15	Initiate Phase III trial for β-Thal.
		ESRD	Ph. II	11.11-16.14	Phase II Part-1 data
Dalantercept (ACE-041)	Wholly Owned	Oncology	Ph. II	2015	Preliminary Phase II Dal-sorafenib combo RR data in HCC
				2H15	Phase II RCC Part-2 data
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 valuation for XLRN is $57 based on an NPV methodology. We assign a 35% probability of success for sotatercept/ACE-536 in MDS, 50% in beta-thalassemia and 20% in ESRD. For dalantercept, we assign a 35% probability of success in RCC. We apply a discount rate of 10% and a terminal multiple of 6x, which we believe reasonable given probability-weighted sales projections and large molecules.

RISKS TO VALUATION

Risks to our valuation include clinical, regulatory, commercial and competitive risks for pipeline products. Most of XLRN's clinical programs are relatively early stage, therefore significant uncertainties exist. As a development-stage company several years away from a commercial launch, there are also financing risks.

XLRN P&L ($000s, except per share data)	2013A	1Q14A	2Q14A	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Revenues										
Sotatercept/ACE-536 WW Revenue in MDS to CELG										$66,089
Probability of Success										*35%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue										$23,131
Risk Adjusted Sotatercept/ACE-536 WW Royalties in MDS										**$4,626**
Sotatercept/ACE-536 WW Revenue in NTD β-Thal. to CELG										$3,659
Probability of Success										*50%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue in NTD β-Thal.										$1,830
Risk Adjusted Sotatercept/ACE-536 WW Royalties in NTD β-Thal.										**$366**
Dalantercept WW Revenue in 2nd-line RCC										$68,061
Probability of Success										*35%*
Risk Adjusted Dalantercept WW Revenue in 2nd-line RCC										**$23,821**
Sotatercept US Revenue in ESRD Patients on Hemodialysis										
Probability of Success										
Risk Adjusted Sotatercept US Revenue in ESRD Patients on Hemodialysis										
Risk Adjusted Sotatercept US Royalties in ESRD Patients on Hemodialysis										
Collaboration Revenue	$57,231	$3,307	$4,078	$30,000	-	$37,385	-	$40,000	$30,000	$24,500
Total Revenue	**$57,231**	**$3,307**	**$4,078**	**$30,000**	**-**	**$37,385**	**-**	**$40,000**	**$30,000**	**$53,314**
Costs and Expenses										
Probability Adjusted Dalantercept COGS	-					-	-	-	-	$3,573
Research and Development	$36,051	$11,765	$12,677	$12,500	$13,280	$50,222	$55,746	$61,879	$68,685	$34,343
Litigation settlement			$5,000							
SG&A (Risk Adjusted from Time of Dalantercept Launch)	$14,227	$3,750	$3,712	$5,100	$5,200	$17,762	$19,538	$21,492	$23,641	$30,641
Total Costs and Expenses	**$50,278**	**$15,515**	**$21,389**	**$17,600**	**$18,480**	**$72,984**	**$75,285**	**$83,371**	**$92,326**	**$68,557**
Operating Income (EBIT)	**$6,952**	**($12,208)**	**($17,311)**	**$12,400**	**($18,480)**	**($35,599)**	**($75,285)**	**($43,371)**	**($62,326)**	**($15,243)**
Y/Y growth										
Other Income (Expenses)	($27,710)	$3,088				-	-	-	-	-
Interest Income	$20		$761							
Interest Expense	($1,161)			($298)	($219)	($517)	($521)	-	-	-
Income Before Taxes	**($21,898)**	**($9,120)**	**($16,550)**	**$12,102**	**($18,699)**	**($32,267)**	**($75,805)**	**($43,371)**	**($62,326)**	**($15,243)**
Provision for Taxes						-	-	-	-	-
Tax Rate						*0%*	*0%*	*0%*	*0%*	*0%*
Net income	**($21,898)**	**($9,120)**	**($16,550)**	**$12,102**	**($18,699)**	**($32,267)**	**($75,805)**	**($43,371)**	**($62,326)**	**($15,243)**
Net income (loss) applicable to common stockholders—diluted	**($39,003)**	**($9,120)**	**($16,550)**	**$12,102**	**($18,699)**	**($32,267)**	**($75,805)**	**($43,371)**	**($62,326)**	**($15,243)**
Change in fair value of warrants	$1,500									
EPS (LPS) Basic	**($4.15)**	**($0.30)**	**($0.52)**	**$0.38**	**($0.59)**	**($1.03)**	**($2.26)**	**($1.28)**	**($1.82)**	**($0.44)**
Basic Shares (000)	**9,407**	**30,321**	**31,552**	**31,584**	**31,615**	**31,218**	**33,530**	**33,866**	**34,204**	**34,546**

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

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.

Valuation

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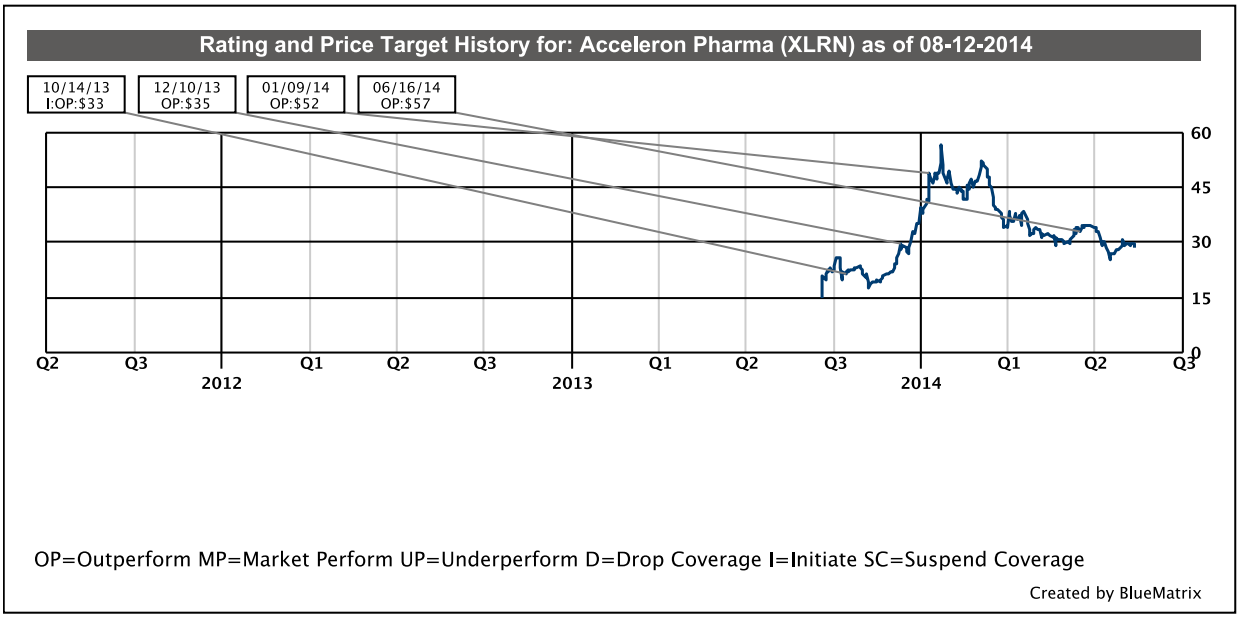
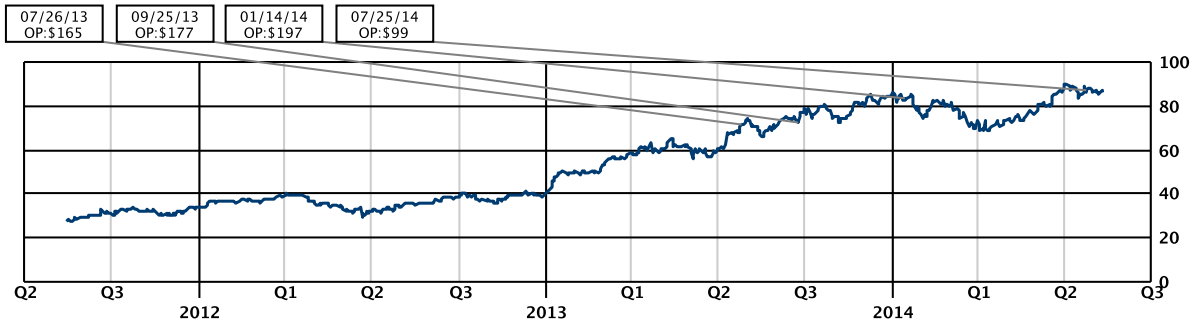


Rating and Price Target History for: Celgene, Inc. (CELG) as of 08-12-2014



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 06/30/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	138	69.00	50	36.20
HOLD [MP]	62	31.00	2	3.20
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 Pharma .

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

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

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