June 3, 2014

OUTPERFORM

Marko Kozul, M.D.
(415) 905-7221
Marko.Kozul@Leerink.com

LEERINK

Reason for report:

COMPANY UPDATE

ACCELERON PHARMA

Encouraging Dal-Axitinib at ASCO & SOT β-Thal at EHA Could Surprise; Reit $52PT

• **Bottom Line:** Dalantercept (Dal)-Axitinib combination data at ASCO included incremental new information but were overall in line with our previous expectations (LINK). Key opinion leaders (KOLs) at the meeting and our Leerink-MEDACorp event described the Phase II Part-1 (N=20) poster as an encouraging proof of concept (POC) experience they hope will be replicated in the Part-2 randomized trial (N=130). Looking forward to the European Hematology Association (EHA, 6.12-15.14), we learned that the Sotatercept (SOT) β-Thal. abstract was never published online with the others because EHA has selected it for plenary presentation. Thus, we believe these SOT data could positively surprise the Street when they are released next Friday (6.13.14) along with additional details beyond the abstracts for ACE-536 in myelodysplastic syndrome (MDS) and β-Thal. (LINK). We reiterate our OP rating & $52 price target (PT).

• **ASCO data for the proprietary Dal program were essentially in line and demonstrate the program is maturing and advancing very solidly.** New for us in Poster-4566 for this Dal-Axitinib combo 2nd-line Renal Cell Carcinoma (RCC) trial were additional baseline patient characteristics and a trend demonstrating greater Partial Response (PR) rates in later line patients. More specifically, ~58% and ~42% of patients had failed sunitinib or pazopanib, respectively, with some also failing Evero-/temsirolimus, sorafenib, bevacizumab or nivolumab. In terms of the PRs, ~38% of those failing ≥2 prior therapies experienced a PR vs. ~17% for those failing just 1 prior therapy. While we view this as an interesting trend, we currently believe it is likely more correlated to the small sample size. It is clear why the 0.9mg/kg dose was selected as the recommended Phase II dose (RP2D) given no observation of common Grade-3 toxicities.

• **We were surprised to learn that the SOT β-Thal. EHA abstract was never published online with the others because EHA selected it for plenary presentation, and this may surprise the Street when SOT β-Thal. data are released next Friday (6.13.14).** Given this dynamic, we would anticipate the SOT β-Thal. data should be even stronger than the already encouraging ACE-536 β-Thal. data made recently available in the EHA abstracts on 5.22.14. As a reminder, these ACE-536 β-Thal. data were impressive for both transfusion dependent (TD) and non-transfusion dependent (NTD) patients, in the context of favorable safety with no serious adverse event (SAE).

• **SOT data at the European Renal Association and European Dialysis and Transplant Association (ERA-EDTA) built upon what was previously presented at the National Kidney Foundation (NKF) meeting back in April.** Interestingly, available SOT data may actually be more impressive than we currently appreciate given a washout of ESA effect (HBg decline before randomization) as the time of randomization was 3.5x slower for the placebo group vs. both SOT cohorts.

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,263.70**
Price:	**$30.00**
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):	$909.0
Book Value/Share:	$1.32
Cash Per Share:	$7.06
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	$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.

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.

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	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**
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**
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 Dalantercept 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 Dalantercept 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 Calcuation

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

Source: Leerink Partners estimates.

XLRN DCF Valuation/Share Sensitivity Analysis

Terminal Growth Rate \ Discount Rate	8.0%	9.0%	10.0%	11.0%	12.0%
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

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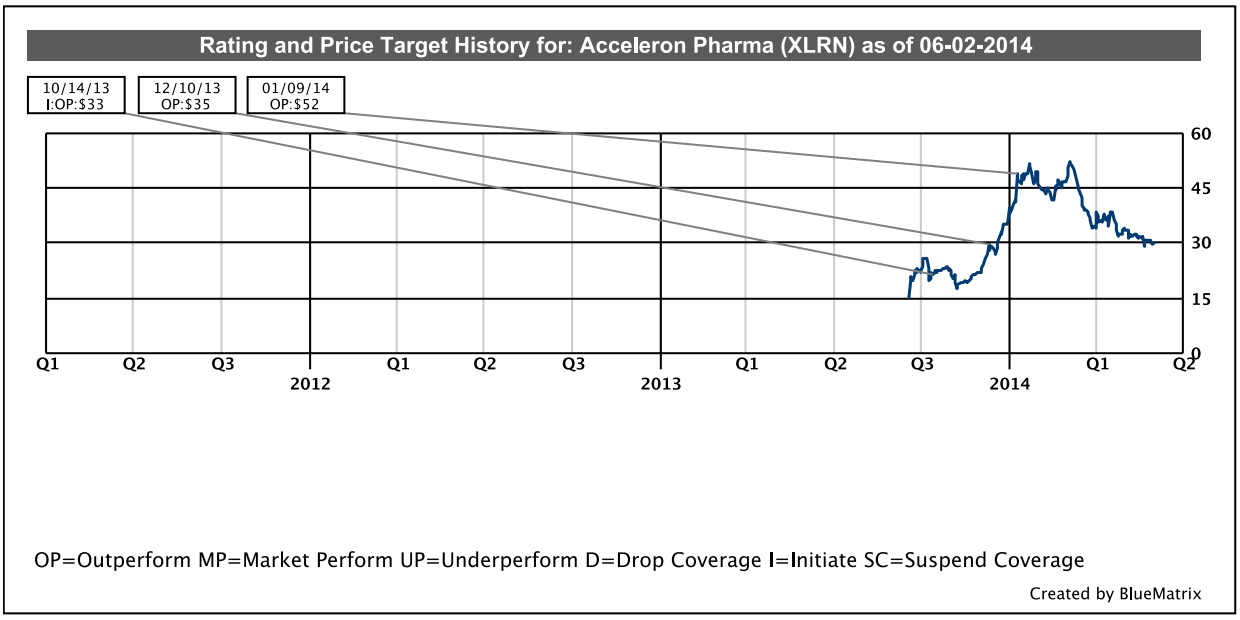

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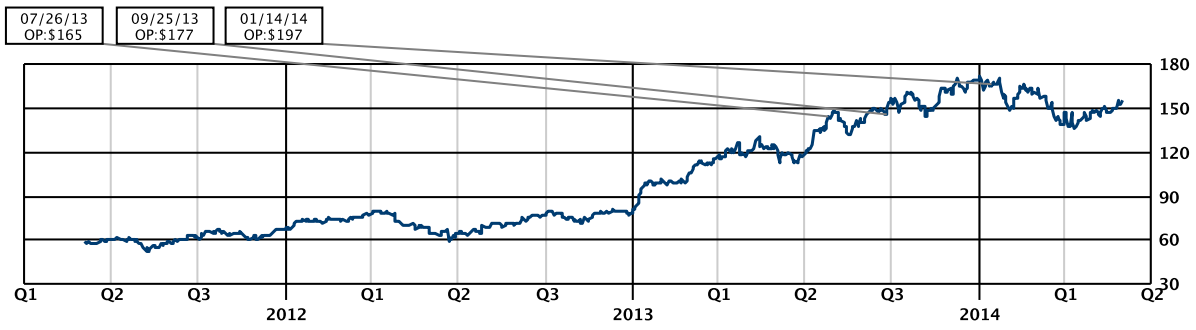

Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

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

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Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14			IB Serv./Past 12 Mos.	
Rating	Count	Percent	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.

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

Leerink Partners LLC Equity Research

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink
Associate Director of Research	**Alice C. Avanian, CFA**	(617) 918-4544	alice.avanian@leerink
Healthcare Strategy	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink
Biotechnology	**Howard Liang, Ph.D.**	(617) 918-4857	howard.liang@leerink
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink
	Paul Matteis	(617) 918-4585	paul.matteis@leerink
	Richard Goss	(617) 918-4059	richard.goss@leerink
Life Science Tools and Diagnostics	**Dan Leonard**	(212) 277-6116	dan.leonard@leerink
	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink
Pharmaceuticals/Major	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink
	Ario Arabi	(617) 918-4568	ario.arabi@leerink
	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink
Specialty Pharmaceuticals, Generics	**Jason M. Gerberry, JD**	(617) 918-4549	jason.gerberry@leerink
Medical Devices, Cardiology & Orthopedics	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink
	Richard Newitter	(212) 277-6088	richard.newitter@leerink
	Ravi Misra	(212) 277-6049	ravi.misra@leerink
Healthcare Services	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink
Healthcare Technology & Distribution	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink
Sr. Editor/Supervisory Analyst	**Mary Ellen Eagan, CFA**	(617) 918-4837	maryellen.eagan@leerink
Supervisory Analysts	Robert Egan		bob.egan@leerink
	Amy N. Sonne		amy.sonne@leerink
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink

New York
299 Park Avenue, 21st floor
New York, NY 10171
(888) 778-1653

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