April 21, 2014

OUTPERFORM

Reason for report:

COMPANY UPDATE

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LEERINK

ACCELERON PHARMA

3 ASCO Pipeline Presentations & Other Important 2Q14 Catalysts; Reit OP & $52PT

• **Bottom Line:** 2Q14 is catalyst rich for XLRN with pipeline data presentations at the National Kidney Foundation (NKF) meeting (4/22-26), ASCO (5/30-6/3) and European Hematology Association (EHA [6/12-15]) that we believe should drive shares higher. Recently released ASCO abstract titles demonstrate there will be 3 presentations made for proprietary compound Dalantercept. Our main focus will be on results from the 2nd-line Renal Cell Carcinoma (RCC) Phase II Dalantercept-Axitinib combo trial (abstract 4566) that will detail both dose-escalation and cohort expansion data. We view potentially positive data in Phase II trials for endometrial cancer (abstract 5594) and squamous cell carcinoma of head and neck (SCCHN) (abstract 6045) as free call options given these indications are not accounted for in our model. Investors should also anticipate some potential volatility related to additional XLRN shares (~10.7M) coming off lock-up today. We reiterate an Outperform (OP) rating and $52 price target (PT).

• **NKF Sotatercept presentation this week shows early dose escalation hemoglobin increases in ESRD hemodialysis patients experiencing bone mineral density (BMD) abnormalities or vascular calcifications.** The BMD and vascular calcification impact will become clear when Part-2 of this trial reads out in 1H15

• **Our main focus at ASCO-2014 will be on data from the 2nd-line RCC Phase II Dalantercept-Axitinib combo trial (abstract 4566).** This presentation will include both dose-escalation and cohort expansion data. Beating the 19% response rate (RR) seen with Axitinib monotherapy and emerging duration of response in a growing sample size should de-risk the potential of this program. Previously, in the early dose escalation experience representing small patient numbers, the combination showed trends for a higher RR with the combo and encouraging tumor reductions at metastatic sites (liver and lung) after each cycle.

• **Other ASCO Dalantercept updates from 2 Phase II trials in endometrial cancer (abstract 5594) and SCCHN (abstract 6045) could serve as meaningful free call options given we do not account for them in our current model.** Given Dalantercept appears more effective in combination with VEGF-inhibitors our expectations are modest for these monotherapy trials but they could provide a strong rationale to initiate future combo trials.

• **EHA-2014 will provide 5 key Sotatercept & ACE-536 Presentations**. The most important will be topline Phase II Sotatercept and ACE-536 data from 4 different trials in MDS and β-Thalassemia (β-Thal.). The meeting also should provide the first preclinical data demonstrating Sotatercept/ACE-536 potential in sickle cell anemia**.** This should bolster the rationale for utility in this indication and reinforce these drugs' emerging potential in larger indications vs. MDS and β-Thal.

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,243.11**
Price:	**$36.37**
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):	31.1
Market Capitalization (mil):	$1,131.1
Book Value/Share:	$1.29
Cash Per Share:	$7.81
Dividend (ann):	$0.00
Dividend Yield:	0.0%

General: intra day price used.
Cash Per Share: Cash per share is pro forma for Jan-2014 financing (~$129M in net proceeds).

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	0.0	0.0	$30.0	0.0	$30.0	($0.50)	($0.53)	$0.40	($0.58)	($1.22)	NM
2015E	--	--	--	--	0.0	--	--	--	--	($2.20)	NM

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

Please refer to pages 6 - 9 for Important Disclosures, Price Charts and Analyst Certification.

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 30%s for Sotatercept/ACE-536 in MDS, 40% for β-Thal., 20% in end-stage renal disease (ESRD) patients on hemodialysis, and low 30%s 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 and β-Thal. at EHA-2014**
				4Q14	**Final Phase II in MDS and β-Thal. data**
				YE14 or Beg-15	Initiate Phase III trial for MDS and/or β-Thal.
				2018	Approval and launch
Sotatercept (ACE-011)				**2Q14**	**Phase II dose escalation MDS + 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 or Beg-15	Initiate Phase III trial for MDS and/or β-Thal.
	CELG	ESRD	Ph. II	**4.22.26-14**	**Additional Data on Dose dependent Hg Increase from Ongoing Phase IIa Study at National Kidney Foundation (NKF)**
				1H15	**Part-2 top-line data**
Dalantercept (ACE-041)	Proprietary	Oncology	Ph. II	**2Q14**	**Interim data from dose escalation Phase II RCC combo data trial (full at ASCO-2014)**
					GOG Ovarian Cancer single agent trial Go-No-Go to Part-2 of trial
				1H14	Initiate Phase II combo (sorafenib) trial in HCC
				YE14	Preliminary Phase II combo (sorafenib) data in HCC
				2014	Phase II data in SCCHN
				2018	Approval and launch in RCC
New TGF-β Candidates		Muscle	PC	2014	Advance Muscle Loss candidate into clinic (ACE-083)
		Fibrosis	PC	2015	Advance Fibrosis (i.e., PAH) candidate into clinic

Source: Company reports, Leerink Partners estimates.

Phase II Sotatercept Intravenous (IV)/Subcutaneous (SC) End-Stage Kidney Disease Patients on Hemodialysis	
Purpose:	Determine optimal administration route, dose level, and safety of IV or SC sotatercept for maintaining hemoglobin levels in ESRD hemodialysis subjects
# Pts:	**Part-1:** N=60 **Part-2:** N=230 19 international sites (as of 1.8.14)
Design:	Interventional, 2x Part, randomized, open label, treatment trial
Trial Arms:	**Note:** Patients in both parts of study must first be on stable dose of ESA to maintain Hg levels and switched to treatment with sotatercept after an ESA treatment free period of ~5 days **Part-1:** Staggered dose group escalation ▪ **Arm-1 (IV):** ACE-011 IV starting at **0.1mg/kg (gp-1)**, then **0.2mg/kg (gp-2)** and **0.3mg/kg (gp-3)** every 14 days for total of 8 doses and followed for 4 months after last dose ▪ **Arm-2 (SC):** ACE-011 SC starting at **0.13mg/kg (gp-1)**, then **0.26mg/kg (gp-2)**, and **0.4mg/kg (gp-3)** every14 days for total of 8 doses and followed for 4 months after last dose **Part-2:** Parallel group, randomized vs. active control (ESA)
Primary Endpoint:	**Part-1:** ▪ **Pharmacokinetics:** C-max, T-max, AUC 28days [Time Frame: 28 days] and T-1/2,z [211 days] ▪ **Adverse Events:** [Time Frame: 211 days] [Designated as safety issue], TEAEs **Part-2:** ▪ **Change in mean hemoglobin concentration from baseline** ▪ **Ability of sotatercept to maintain hemoglobin levels within target range after switching from ESA to sotatercept**
Secondary Endpoints:	▪ Efficacy [Time Frame: 113 days] ▪ Change in mean hemoglobin (Hg) concentration between baseline and day-113 ▪ Bone Turnover biomarkers for remodeling and mineral metabolism for 211 days ▪ Change in serum bone biomarker concentrations between baseline and end of study (day-211)
Start:	October-2013
Data:	October-2015
Status:	Recruiting (as of 1.8.14)
Sponsors:	**CELG**
Clin.Trial.ID:	NCT01999582, **ACE-011-REN-002, 2012-003788-23**

Source: Company reports, Leerink Partners estimates.

VALUATION

Our $52 12-month price target of XLRN shares reflects probability adjusted royalty revenue from ESRD patients on hemodialysis at 20%. 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 (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	1Q14E	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	-	-	$30,000	-	$30,000	-	$40,000	$30,000	$22,400	$8,000	-	$8,000	-	$8,000	$8,000	$8,000	$8,000
Total Revenue	**$57,231**	**-**	**-**	**$30,000**	**-**	**$30,000**	**-**	**$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	$10,350	$11,200	$12,100	$12,780	$46,430	$51,073	$56,180	$61,798	$27,809	$29,200	$30,660	$32,193	$33,802	$35,492	$37,267	$39,130	$41,087
SG&A (Risk Adjusted from Time of Dalantercept Launch)	$14,227	$4,800	$5,000	$5,100	$5,200	$20,100	$22,110	$24,321	$26,753	$33,153	$36,468	$39,386	$41,355	$43,423	$45,594	$47,874	$50,267	$52,781
Total Costs and Expenses	**$50,278**	**$15,150**	**$16,200**	**$17,200**	**$17,980**	**$66,530**	**$73,183**	**$80,501**	**$88,551**	**$64,229**	**$71,987**	**$80,141**	**$83,112**	**$89,967**	**$97,382**	**$105,400**	**$114,067**	**$121,959**
Operating Income (EBIT)	**$6,952**	**($15,150)**	**($16,200)**	**$12,800**	**($17,980)**	**($36,530)**	**($73,183)**	**($40,501)**	**($58,551)**	**($15,527)**	**($7,494)**	**$25,930**	**$100,417**	**$164,576**	**$251,345**	**$322,784**	**$393,746**	**$459,103**
Y/Y growth																		
Other Income (Expenses)	($27,710)					-	-	-	-	-	-	-	-	-	-	-	-	-
Interest Income	$20																	
Interest Expense	($1,161)	($457)	($378)	($298)	($219)	($1,352)	($521)	-	-	-	-	-	-	-	-	-	-	-
Income Before Taxes	**($21,898)**	**($15,607)**	**($16,578)**	**$12,502**	**($18,199)**	**($37,882)**	**($73,704)**	**($40,501)**	**($58,551)**	**($15,527)**	**($7,494)**	**$25,930**	**$100,417**	**$164,576**	**$251,345**	**$322,784**	**$393,746**	**$459,103**
Provision for Taxes						-	-	-	-	-	-	-	-	-	40,225	109,747	133,873	156,095
Tax Rate						0%	0%	0%	0%	0%	0%	0%	0%	0%	16%	34%	34%	34%
Net income	**($21,898)**	**($15,607)**	**($16,578)**	**$12,502**	**($18,199)**	**($37,882)**	**($73,704)**	**($40,501)**	**($58,551)**	**($15,527)**	**($7,494)**	**$25,930**	**$100,417**	**$164,576**	**$211,119**	**$213,038**	**$259,872**	**$303,008**
Net income (loss) applicable to common stockholders—diluted	**($39,003)**	**($15,607)**	**($16,578)**	**$12,502**	**($18,199)**	**($37,882)**	**($73,704)**	**($40,501)**	**($58,551)**	**($15,527)**	**($7,494)**	**$25,930**	**$100,417**	**$164,576**	**$211,119**	**$213,038**	**$259,872**	**$303,008**
Change in fair value of warrants	$1,500																	
EPS (LPS) Basic	**($4.15)**	**($0.50)**	**($0.53)**	**$0.40**	**($0.58)**	**($1.22)**	**($2.20)**	**($1.20)**	**($1.72)**	**($0.45)**	**($0.22)**	**$0.74**	**$2.83**	**$4.59**	**$5.83**	**$5.82**	**$7.03**	**$8.12**
Basic Shares (000)	**9,407**	**31,109**	**31,140**	**31,171**	**31,202**	**31,147**	**33,458**	**33,793**	**34,130**	**34,472**	**34,817**	**35,165**	**35,516**	**35,871**	**36,230**	**36,593**	**36,958**	**37,328**

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

DCF Calcuation

Discount rate	*10%*
Terminal Growth Rate	*1%*
Valuation ($M)	$1,681
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

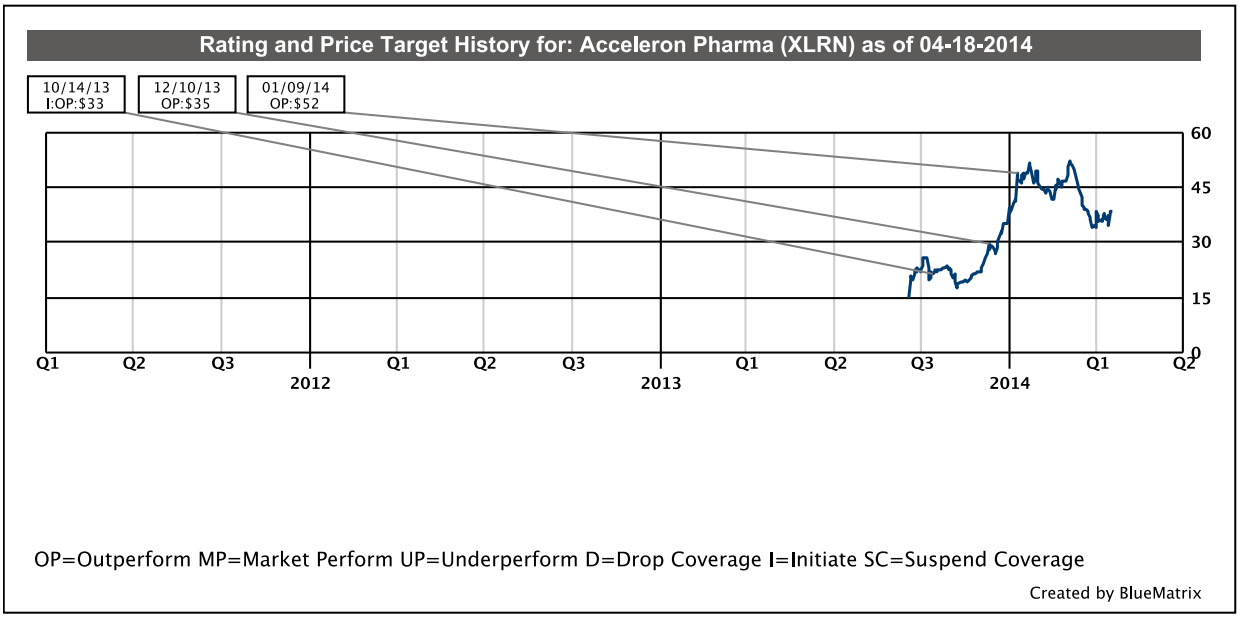
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

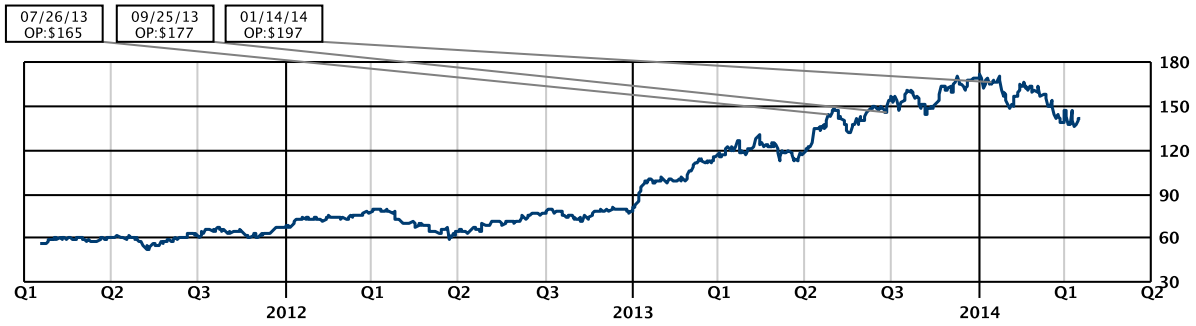
Our $52 12-month price target of XLRN shares reflects probability adjusted royalty revenue from ESRD patients on hemodialysis at 20%. Our valuation is based on a discounted cash flow analysis. XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 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 (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.



Rating and Price Target History for: Celgene, Inc. (CELG) as of 04-18-2014



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 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

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

LEERINK

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.

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