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OUTPERFORM

Reason for report:

FLASH NOTE

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ACCELERON PHARMA

NKF Data Interpretation & Multiple 2014 Catalysts Create Buying Opportunity

• **Bottom Line:** We see XLRN weakness as unwarranted and would be taking advantage of it this morning. We believe the disconnect stems from investors comparing National Kidney Foundation (NKF) abstract (released late yesterday) data for Sotatercept's magnitude of hemoglobin increases with historical ESA numbers. The main point of this Phase II trial is not to demonstrate Sotatercept's ability to compete directly with ESA but rather to demonstrate the ability to ameliorate bone mineral density issues that can plague End-Stage Renal Disease (ESRD) patients on hemodialysis. The secondary goal is to demonstrate Sotatercept's ability to induce a hemoglobin increase in these patients that is relatively similar to ESAs. We believe early data in the abstract demonstrate this secondary goal is being accomplished. Bone biomarker data was not scheduled to be presented at NKF but will be part of the Part-2 presentation in 1H15.

• **NKF Abstract demonstrate early dose escalation Sotatercept hemoglobin increases relatively on par with Erythrocyte Stimulating Agents (ESAs) even if this is not the main goal of the Phase II trial.** While the Sotatercept 0.3 and 0.5mg/kg cohorts demonstrate 0.5g/dL and 0.8g/dL hemoglobin increases, we should appreciate from Myelodysplastic Syndrome (MDS) and β-Thalasemia studies that the drug can achieve higher levels and that 0.7mg/kg data when available will be in the 1-1.5g range.

• **The main point of this study is not to demonstrate Sotatercept's superiority to ESAs but rather to capitalize on its ability to positively impact ESRD hemodialysis patients experiencing bone mineral density (BMD) abnormalities or vascular calcifications.** Any expectation that meaningful BMD data from this dose escalation trial would be presented in April at NKF is unrealistic given the small patient numbers and dose exploration. This data will be a key feature of the Part-2 randomized portion of the trial that we estimate will yield data by 1H15.

• **Nearer term, We look forward to main pipeline 2Q14 catalysts and initiation of a Dalantercept-Sorafenib combo Phase II trial in Hepatocellular Carcinoma (HCC) that should yield preliminary data ~4Q14 and could drive further upside to our current valuation**. In 2Q14 and at ASCO, we anticipate Phase II Dalantercept-Axitinib 2nd-line Renal Cell Carcinoma (RCC) and data. A week later, 4 Phase II trials of Sotatercept and ACE-536 will have topline data at the European Hematologic Association (EHA) meeting. Finally, building upon the experience in renal cell carcinoma (RCC), very near term XLRN plans to initiate this 1st-line combo trial in HCC. Sorafenib was approved based on a second interim analysis showing a statistically significant 2.8 month overall survival (OS) benefit (10.7mos) vs. placebo (7.9mos) and 2.7 month Time to Progression benefit (5.5mos) vs. placebo (2.8mos). Preliminary response data from the Dalancercept combo trial will be available by YE14 with maturing PFS data in 2015. We believe HCC

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,296.10**
Price:	**$42.50**
52 Week High:	$57.89
52 Week Low:	$15.00
Shares Outstanding (mil):	31.1
Market Capitalization (mil):	$1,321.8

could provide a further large market opportunity currently not in our model with foreseeable incremental and de-risking milestones.

Phase II Sotatercept Intravenous (IV) or 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 erythropoiesis stimulating agent (ESA) to maintain hemoglobin levels and will then be 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 [Time Frame: 211 days] ▪ **Adverse Events:** [Time Frame: 211 days] [Designated as safety issue], TEAEs and number of subjects with 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.Trials. Gov ID:	NCT01999582 **ACE-011-REN-002, 2012-003788-23**

Source: Clinicaltrials.gov and Leerink Partner estimates

Disclosures Appendix

Analyst Certification

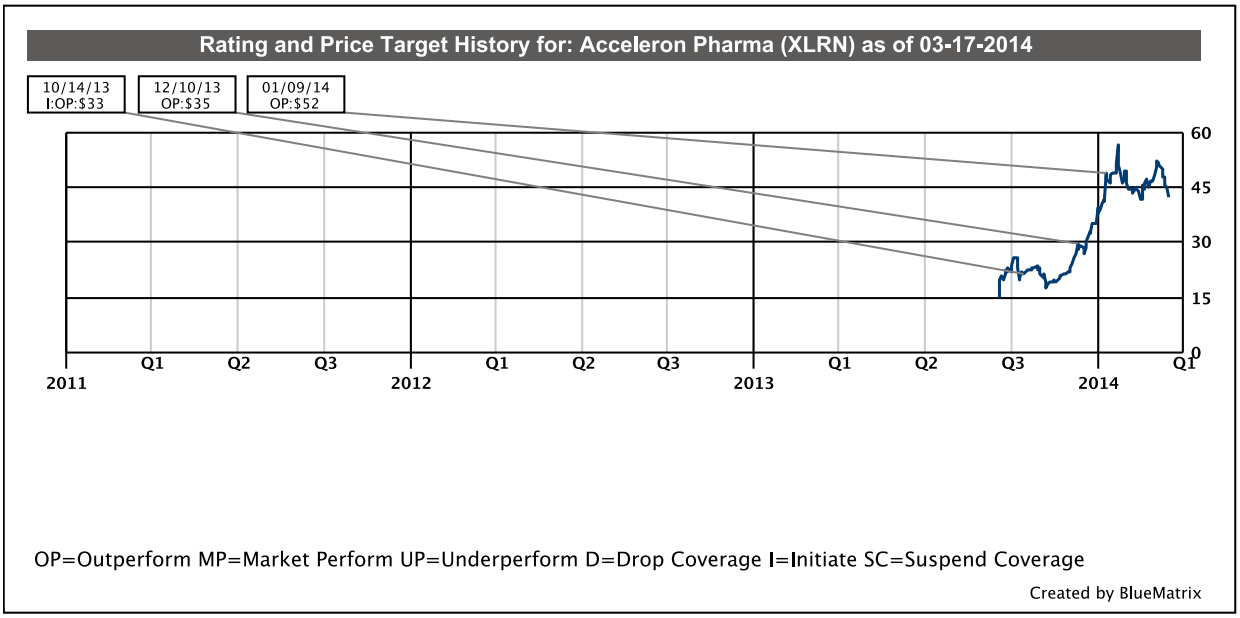
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

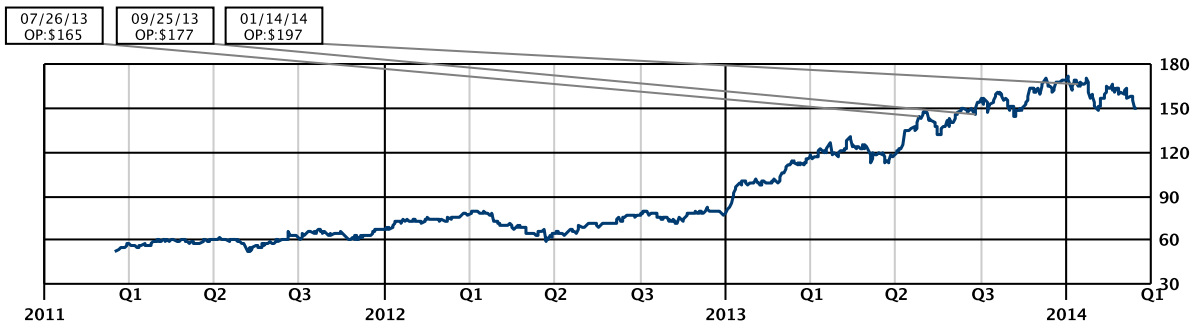
Valuation

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.





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 12/31/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	118	64.50	30	25.00
HOLD [MP]	65	35.50	2	3.00
SELL [UP]	0	0.00	0	0.00

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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.

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

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

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