Acceleron Pharma Inc. (XLRN)

Sotatercept Poised for Success in ESRD with Top-line Data Possible for National Kidney Foundation Meeting

MARKET OUTPERFORM | Price: $45.28 | Target Price: $53.00

MARKET DATA

Price	$45.28
52-Week Range:	$16.78 - $57.89
Shares Out. (M):	26.5
Market Cap ($M):	$1,199.9
Average Daily Vol. (000):	291.0
Cash (M):	$116
Cash/Share:	$4.13
Enterprise Value (M):	$678
Float (M):	24.2
LT Debt (M):	$11

Source: Thomson Reuters and JMP Securities LLC

FY DEC		2012A	2013E	2014E
Revenue ($M)	1Q	--	$15.0A	$4.9
	2Q	--	$26.4A	$5.2
	3Q	--	$4.3A	$5.4
	4Q	--	$11.7	$20.9
	FY	**$15.3**	**$57.4**	**$36.4**
EPS	1Q	--	$0.12A	($0.33)
	2Q	--	$0.44A	($0.33)
	3Q	--	($5.62)A	($0.34)
	4Q	--	($0.07)	$0.13
	FY	**($1.43)**	**($0.55)**	**($0.81)**
	P/E	NM	NM	NM

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE

INVESTMENT HIGHLIGHTS

Recent weakness in share price represents a buying opportunity ahead of the NKF meeting; reiterate our Market Outperform rating on Acceleron Pharma and price target of $53 based on our DCF and SOTP valuation methodologies. Recently, Acceleron Pharma updated its S-1 filing to reflect "encouraging early data" for subcutaneously administered sotatercept in patients with end-stage renal disease (ESRD). The data were characterized by "dose-dependent increases in hemoglobin", indicating the potential for a presentation of positive top-line data at the National Kidney Foundation meeting (NKF) taking place April 22 to 26 in Las Vegas, NV. While XLRN shares have more than doubled over the past three months, they are well off from their 52-week high of $57 reached in late January, and down from the $50 price at which the company closed a $129MM follow-on offering on January 28. With the company also providing positive updates to Phase II results for ACE-536 in beta-thalassemia, preliminary read-outs from dalantercept Phase II trials in ovarian cancer, and the filing of an IND for muscle growth stimulant, ACE-083, we believe investors may benefit from adding shares before the upcoming deluge of positive news reports. As a reminder, XLRN is scheduled to report financial results before market open on Wednesday, February 26.

Review of anemia in ESRD highlights significant unmet medical need. Over the past decade, the market for anemia in ESRD has been dominated by Amgen's (AMGN, Not Covered) erythropoiesis stimulating agent (ESA) Epogen. While annual U.S. sales of Epogen are ~$2 billion, heavy ESA use in ESRD patients on hemodialysis (HD) is a known risk factor for cardiovascular complications, hypertension, malignancy, and death. In a NKF sponsored study of almost 100K patients, high doses of Epogen were less effective in raising hematocrit levels indicating a reduced responsiveness to the drug (Zhang Y, et al. Am J Kidney Disease, 2004) and resulted in more patient deaths. Physicians could, therefore, reduce the relative risk of death in ESRD patients being dosed with high-dose Epogen by switching to a drug like sotatercept that stimulates the production of red blood cells (RBCs), but does not target the EPO-receptor. Sotatercept functions by blocking negative regulators of red blood cell differentiation, a process wholly independent of the EPO pathway. In addition, sotatercept also inhibits activin A, a TGF-beta ligand whose inhibition has been shown reduce bone loss and vascular calcification associated with renal osteodystrophy. Thus, there is significant upside to treating patients with sotatercept as it can positively impact two chronic complications in ESRD: anemia and loss of bone mass.

Positive data from current sotatercept Phase II trials in ESRD are critical for success. Currently, XLRN is partnered with Celgene (CELG, MO, $205 PT) in two Phase II trials in ESRD. The first is a Phase IIa dose escalation trial (NCT01146574)

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seeking to evaluate pharmacodynamics as primary outcomes, and safety, efficacy, and tolerability as secondary outcomes. The second Phase II trial (NCT01999582) is a randomized, open-label study of intravenous and subcutaneous administration of sotatercept in subjects with ESRD on hemodialysis switched from ESAs. Primary outcomes focus on pharmacodynamics and adverse events, but secondary outcomes will measure both efficacy and bone turnover. Both trials are critical for sotatercept's success, as demonstrating safety with similar or better efficacy in treating anemia compared to ESAs will allow sotatercept to gain market share from Epogen. We expect XLRN to present data for one or both of these trials at the spring NKF meeting discussed above. The company will also be presenting data on the use of sotatercept and ACE-536 at ASCO (May 30th – June 3rd) and EHA (June 12-15th) in the MDS and beta-thalassemia patient setting.

ESRD patient population for sotatercept drives upside potential in our XLRN model. The ESRD population currently taking ESAs for anemia is over 300,000 in the U.S. and close to one million worldwide. Based on the increased risk of death associated with high-dose Epogen discussed above, we believe 27.5% of this patient population would be eligible for switching to an anemia drug without an EPO-receptor dependent mechanism of action. A conservative market penetration of 15-18% in these high-dose EPO patients is sufficient for a $53 price target, based on our SOTP ($50) and DCF ($56) valuation methodologies.

Acceleron represents a compelling opportunity in the biotech space over the course of the next several years. Our view is drawn from the company's focus and understanding of TGF beta biology, as well as developmental and commercialization advantages offered through its strategic partnership with Celgene (CELG, MO, $205 PT). In our opinion, the company's first wave of product candidates is the tip of the iceberg that, over time, should create significant value for shareholders.

FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
1Q14	Sotatercept & ACE-536	Initiation of RP2D expansion cohorts in ongoing β-thalassemia Phase II trials
1Q14	Dalantercept	Preliminary data from dose-escalation stage of Phase II RCC trial in combination with axitinib; start of randomized stage versus axitinib alone
2Q14	Sotatercept	Presentation of data in ESRD at National Kidney Foundation meeting (April 22nd – 26th, 2014 - Las Vegas, NV)
2Q14	Sotatercept & ACE-536	Presentation of dose escalation Phase II results in β-thalassemia and MDS at ASCO (May 30th – June 3rd) and EHA (June 12th-15th)
3Q14	Dalantercept	Initiation of Phase II trial(s) in additional indication(s)
4Q14	Sotatercept & ACE-536	Final results from Phase II trials in β-thalassemia and MDS
4Q14	Sotatercept & ACE-536	Initiation of Phase III trial in β-thalassemia and/or MDS
4Q14	ACE-083	Initiation of Phase I trial in muscular dystrophy

Source: JMP Securities LLC and Company Reports

Company Description

Acceleron Pharma (XLRN) is a Cambridge, MA biotechnology company focused on the discovery, development, and commercialization of its ligand trap fusion proteins directed against components of TGFβ signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to β-thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGFβ.

Since 2008, the company has benefited by robust strategic collaboration with Celgene related to its development lead programs, sotatercept and ACE-536, entitling the company to full reimbursement on both programs and eligibility for up to $567MM in development, regulatory, and commercial milestones, and a ≥20% royalty on worldwide sales, by our estimates. Sotatercept and ACE-536 are currently in Phase II trials for the treatment of β-thalassemia and low/intermediate-1 MDS with pivotal Phase III trials expected to initiate in the first half of 2014.

Dalantercept, the company's wholly owned, clinical-stage fusion protein, is directed against ALK1, a key mediator of tumor angiogenesis that functions independently from the VEGF axis. Dalantercept is currently in Phase II evaluation for the treatment of second-line RCC in combination with TKI therapy.

Investment Risks

Clinical. Drug development is an inherently risky business. Clinical trials always carry a risk of failure and Acceleron's assets (sotatercept, ACE-536, Dalantercept, or future drug candidates) may fail to demonstrate meaningful enough levels of efficacy in current or future clinical trials.

Regulatory and commercial. The ability of Acceleron or its partners to market its drugs depends on those drugs obtaining approval from the FDA and foreign regulatory agencies. Failure to achieve approval or delays in the timelines to approval could negatively impact the company's share price.

Competitive. Hereditary anemic disorders represent an increasingly competitive field and Acceleron faces competition from companies with development-stage drug candidates addressing similar biologic mechanisms, and from companies attempting to broaden the applicable indications for products already approved for use. Some of these companies may possess substantially greater R&D and commercial resources than Acceleron or its partners. As such, there is no assurance Acceleron will be competitive or differentiated from other drug products.

Partners. Acceleron has formed development and commercial partnerships with Celgene and is highly dependent on these partnerships for non-dilutive sources of capital, and for the potential commercialization of sotatercept and/or ACE-536. Changes to these partnership arrangements could have a substantially negative impact on the company's share price.

Financial. Following its IPO we estimated that Acceleron would end 4Q13 with approximately $87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron's financial guidance. We anticipate that Acceleron is likely to seek additional equity financing in the form of a secondary offering in order to complete the development of its drug candidates, creating dilution risk for existing shareholders.

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JMP Securities Disclosures:

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

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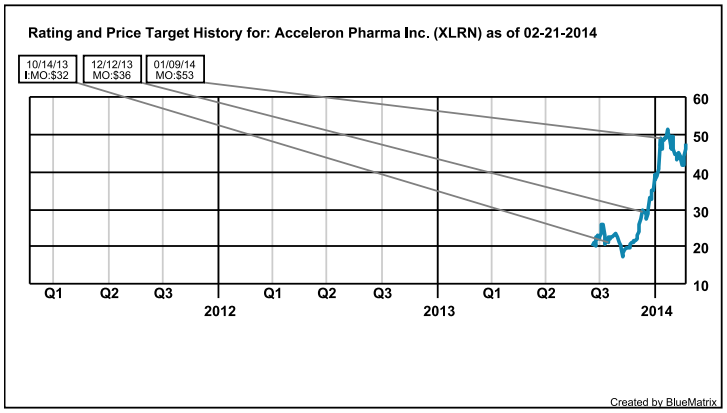
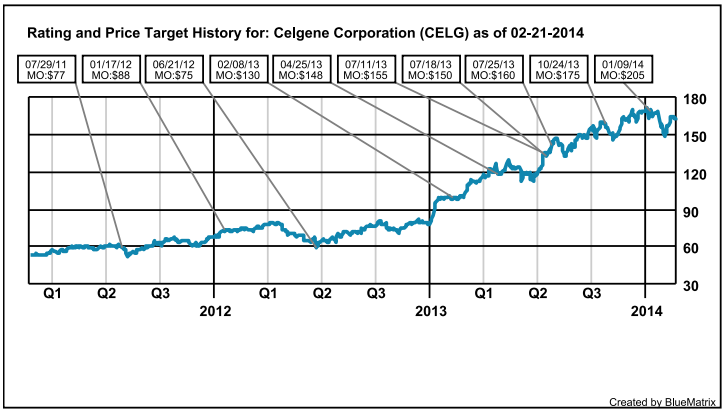
JMP Rating	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	Regulatory Equivalent	# Co's Under Coverage	*% of Total*	# Co's Receiving IB Services in Past 12 Months	*% of Co's With This Rating*
MARKET OUTPERFORM	Buy	241	*56.18%*	Buy	241	*56.18%*	89	*36.93%*
MARKET PERFORM	Hold	137	*31.93%*	Hold	137	*31.93%*	21	*15.33%*
MARKET UNDERPERFORM	Sell	8	*1.86%*	Sell	8	*1.86%*	0	*0%*
COVERAGE IN TRANSITION		43	*10.02%*		43	*10.02%*	0	*0%*
TOTAL:		429	*100%*		429	*100%*	110	*25.64%*

Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.





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