

Acceleron Pharma Inc. (XLRN)

Sotatercept Moves Forward in ESRD

MARKET DATA

Price	\$28.23
52-Week Range:	\$16.78 - \$31.43
Shares Out. (M):	26.5
Market Cap (\$M):	\$748.1
Average Daily Vol. (000):	193.0
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

MARKET OUTPERFORM | Price: \$28.23 | Target Price: \$36.00

INVESTMENT HIGHLIGHTS

Celgene initiates a Phase II trial of sotatercept in ESRD patients on hemodialysis, delivering a \$7MM milestone payment to Acceleron Pharma as anticipated; reiterate Market Outperform rating and \$36 price target. The two-stage Phase II trial, which will be led by Celgene, will determine the safety and efficacy of IV and sub-cutaneous (SQ) administrations of sotatercept in the management of anemia and other clinical manifestations of chronic kidney disease-mineral and bone disorder (CKD-MBD). After establishing an appropriate dosing regimen in the initial, 60-patient, dose-escalation portion of the study, approximately 230 patients will be randomized to receive either sotatercept or ESAs and monitored for primary endpoints of mean change in hemoglobin levels from baseline and bone turnover. The ESRD hemodialysis patient population is considerable (~400,000 patients by our estimates), representing an appreciable market opportunity for sotatercept at even modest penetration rates. Given that our sotatercept model anticipates its use in the treatment of beta-thalassemia and MDS, ESRD represents an upside to our current valuation of the program. Reflecting on the receipt of the \$7MM milestone payment slightly ahead of expectations, we have reduced our net loss estimate for 4Q13 EPS from (\$0.32) to (\$0.07), and our 2Q14 net loss estimates have been increased from (\$0.09) to (\$0.33), with negligible impact to our valuation. Our \$36 price target is derived through a synthesis of DCF, comparable and CAGR model 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, \$175 PT). In our opinion, the company's first wave of product candidates, validated by Celgene's significant investment, is but the tip of the iceberg that, over time, should create significant value for shareholders.

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
	Previous FY	NC	(\$0.82)	(\$0.57)

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



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FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
4Q13	Sotatercept	Update from Sotatercept Phase II trial in β -thalassemia at ASH (Dec 9)
4Q13/1Q14	Dalantercept	Preliminary data from Phase II, GOG-sponsored trials in ovarian cancer
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 & ACE-536	Presentation of dose escalation Phase II results in β -thalassemia and MDS
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 TGF β 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 TGF β .

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 $\geq 20\%$ 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 to the company's share price.

Financial. Following its IPO, we estimate that Acceleron will end 4Q13 with approximately \$87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron 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.

JMP FACTS AND DISCLOSURES

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JMP Securities Disclosures:

JMP Securities currently makes a market in the securities of Accelaron Pharma Inc. and Celgene Corporation

JMP Securities was manager or co-manager of a public offering, and received compensation for doing so, for Accelaron Pharma Inc. in the past 12 months.

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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.

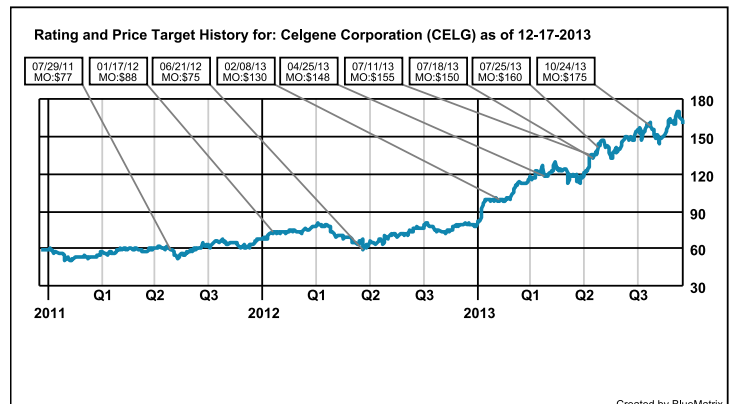
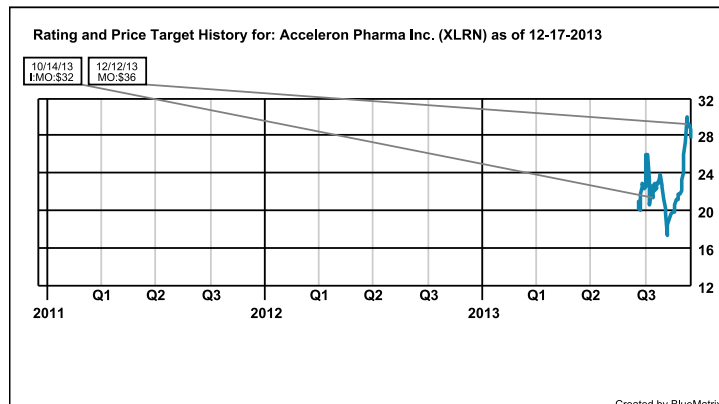
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JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	231	55.00%	Buy	231	55.00%	89	38.53%
MARKET PERFORM	Hold	140	33.33%	Hold	140	33.33%	24	17.14%
MARKET UNDERPERFORM	Sell	5	1.19%	Sell	5	1.19%	0	0%
COVERAGE IN TRANSITION		44	10.48%		44	10.48%	0	0%
TOTAL:		420	100%		420	100%	113	26.90%

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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