

Acceleron Pharma Inc. (XLRN)

Incremental Update to RCC Response Data at ASCO

MARKET DATA

Price	\$29.68
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$786.5
Average Daily Vol. (000):	351.0
Cash (M):	\$214
Cash/Share:	\$6.81
Enterprise Value (M):	\$933
Float (M):	27.0
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

MARKET OUTPERFORM | Price: \$29.68 | Target Price: \$53.00

INVESTMENT HIGHLIGHTS

Poster presentation of Phase I dalantercept combination in RCC to be presented at ASCO largely in line with abstract, breaking out responses by line of therapy; we reiterate our Market Outperform rating and \$53 price target on Acceleron Pharma based on our DCF and SOTP valuation methodologies. Recall that the study design was a single-arm, dose-escalation, Phase I/II trial that examined the use of dalantercept in combination with axitinib (Inlyta) in patients who have received between one and three prior lines of therapy. An overall ORR of 25% (5/20 pts), as previously reported, breaks down as 17% (2/12 pts) in 2L and 38% (3/8 pts) in 3L+, comparing favorably to Inlyta monotherapy. Of note, partial responses were observed in patients progressing from all classes of RCC therapy, including prior TKI, mTOR inhibitors, and immunotherapy (nivolumab)(Figure 2). In our view, these data bode well for the randomized Phase II study comparing dalantercept (0.9mg/kg) plus Inlyta versus Inlyta alone in 130 patients with TKI-refractory RCC and we gain greater comfort with our valuation attributable to dalantercept (NPV of \$4.68 by SOTP).

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).

FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	\$15.0	\$3.3A	\$5.2
	2Q	\$26.4	\$5.2	\$5.4
	3Q	\$4.3	\$5.4	\$20.9
	4Q	\$11.5	\$20.9	\$34.8
	FY	\$57.2	\$34.8	\$42.9
EPS	1Q	\$0.12	(\$0.30)A	--
	2Q	\$0.44	(\$0.37)	--
	3Q	(\$5.62)	(\$0.41)	--
	4Q	(\$0.64)	\$0.05	--
	FY	(\$4.15)	(\$1.01)	(\$1.12)

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



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FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
2Q14	Dalantercept	Initiation of Phase IIa study in HCC in combination with Nexavar
2Q14	Sotatercept & ACE-536	Presentation of dose escalation Phase II results in β -thalassemia and MDS at ASCO (May 30 – June 3) and EHA (June 12-15)
3Q14	Dalantercept	Initiation of Phase II trial plus Avastin in GBM
4Q14	Sotatercept & ACE-536	Final results from Phase II trials in β -thalassemia and MDS
4Q14/1Q15	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

FIGURE 2. Response Rate by Line of Therapy

Response Rate Analyses Based on Number of Prior Therapies*		
Endpoint	1 Prior Therapy (N = 12)	≥ 2 Prior Therapies (N = 8)
Partial Response, n (%)	2 (16.7)	3 (37.5)
Stable Disease, n (%)	7 (58.3)	3 (37.5)
Disease Control Rate ≥ 6 cycles, n (%)	6 (50.0)	5 (62.5)
Progressive Disease, n (%)	3 (25.0)	2 (25.0)

Source: Company presentation, ASCO 2014

FIGURE 3. Treatment History for Responding Patients

Subject	Dalantercept Dose Level (mg/kg)	Prior Therapies	Best Response to Last Therapy	Duration on Last Therapy (months)	Duration on Dalantercept + Axitinib (months)
0101	0.6	• sunitinib	SD	9.0	8.1
0102	0.6	• interleukin-2 • sunitinib • everolimus	SD	4.9	7.0
0201	0.9	• sunitinib • temsirolimus • bevacizumab	PD	1.0	10.4 +
0805	1.2	• sunitinib • nivolumab	SD	7.0	6.2 +
0806	1.2	• pazopanib	SD	23.5	6.7 +

Source: Company presentation, ASCO 2014

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 from 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 upon 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 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.

JMP FACTS AND DISCLOSURES

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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 of securities for Accelaron Pharma Inc. (XLRN) in the past 12 months, and received compensation for doing so.

JMP Securities Investment Opinion Definitions:

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.

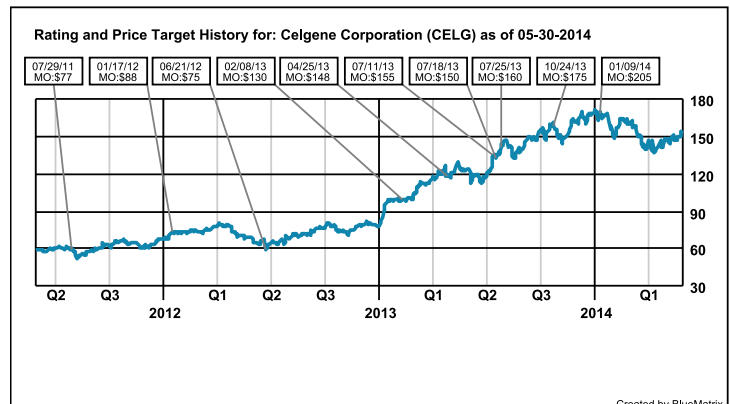
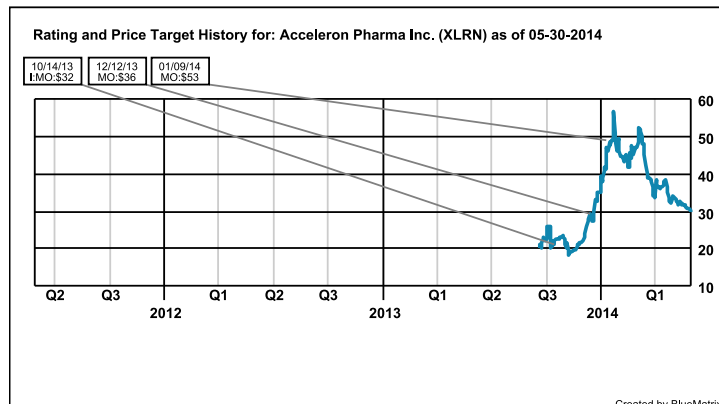
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

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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	258	58.64%	Buy	258	58.64%	99	38.37%
MARKET PERFORM	Hold	134	30.45%	Hold	134	30.45%	16	11.94%
MARKET UNDERPERFORM	Sell	5	1.14%	Sell	5	1.14%	0	0%
COVERAGE IN TRANSITION		43	9.77%		43	9.77%	0	0%
TOTAL:		440	100%		440	100%	115	26.14%

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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