

Acceleron Pharma Inc. (XLRN)

Encouraging Data but More to be Seen with ACE-536 in MDS

MARKET DATA

Price	\$33.88
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$897.8
Average Daily Vol. (000):	383.0
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: \$33.88 | Target Price: \$53.00

INVESTMENT HIGHLIGHTS

We reiterate our Market Outperform rating and \$53 price target on Acceleron Pharma following the presentation of preliminary ACE-536 Phase II dose-escalation results in lower risk MDS. While data reflect favorable PK, dose response, and safety profiles consistent with the experience in beta-thalassemia, efficacy (durable hemoglobin increases and reductions in transfusion burden) remains short of being clinically impactful. However, given the safety margin that allows for further exploration at higher doses, we remain optimistic about the chances for ACE-536 to ultimately demonstrate clinical benefit in MDS. We derive our \$53 price target through our DCF and SOTP valuation methodologies.

ACE-536 delivers consistent dose response in NTD patients, but durability remains a question. As shown in Figure 2, NTD patients treated in the highest dosing cohort (0.75 mpk) saw impressive max changes in hemoglobin levels (1.5-3.5 g/dL). However, but for at least one patient (Figure 3), the increases thus far appear to fall short of the durability required to satisfy the primary endpoint (≥ 1.5 g/dL for at least 14 days). We believe these data could improve at higher doses (1.33 and 1.75 mpk cohorts currently enrolling).

Outcomes in transfusion-dependent patients, while limited in number, are impressive given the refractory nature of these patients at baseline. As shown in Figure 4, 4/16 transfusion-dependent (TD) patients saw transfusion reductions of ≥ 4 units, while 5/16 patients saw reduction $\geq 50\%$ in transfusion burden over an 8-week period (co-primary endpoints for TD MDS). We note that several of these patents were refractory to prior therapy with lenalidomide and ESAs, which, in our view, speaks to ACE-536's potential to satisfy the unmet need among such patients.

We believe 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).

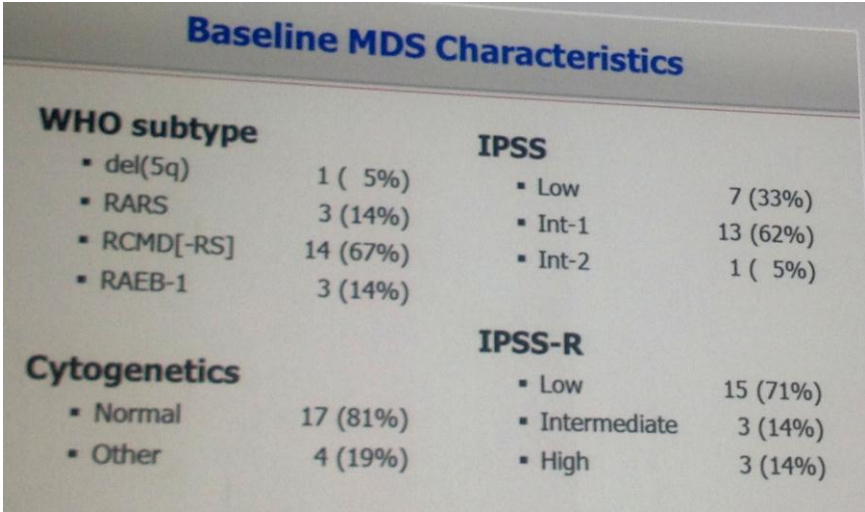
FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	\$15.0	\$3.3A	\$5.2
	2Q	\$26.4	\$5.2	\$5.4
	3Q	\$4.3	\$5.4	\$20.9
	4Q	\$11.5	\$20.9	\$34.8
	FY	\$57.2	\$34.8	\$42.9
EPS	1Q	\$0.12	(\$0.30)A	--
	2Q	\$0.44	(\$0.37)	--
	3Q	(\$5.62)	(\$0.41)	--
	4Q	(\$0.64)	\$0.05	--
	FY	(\$4.15)	(\$1.01)	(\$1.12)

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE

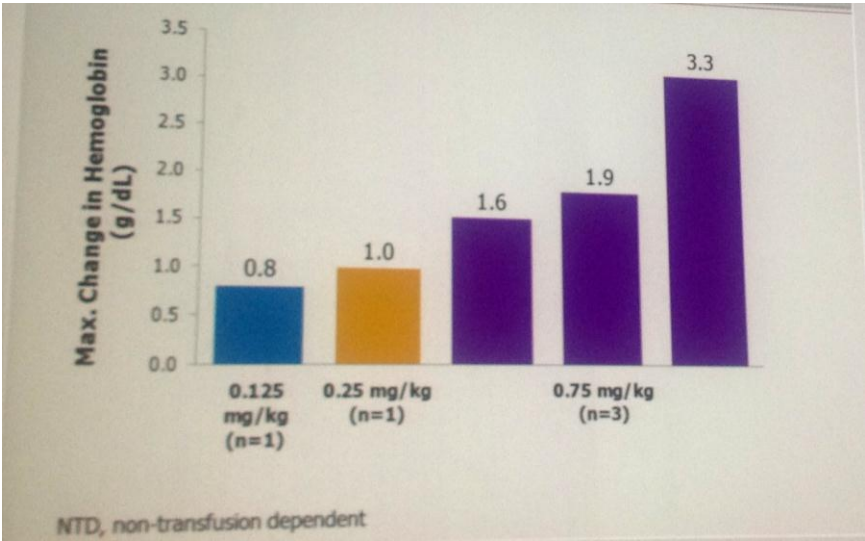


FIGURE 1. ACE-536 MDS Baseline Characteristics



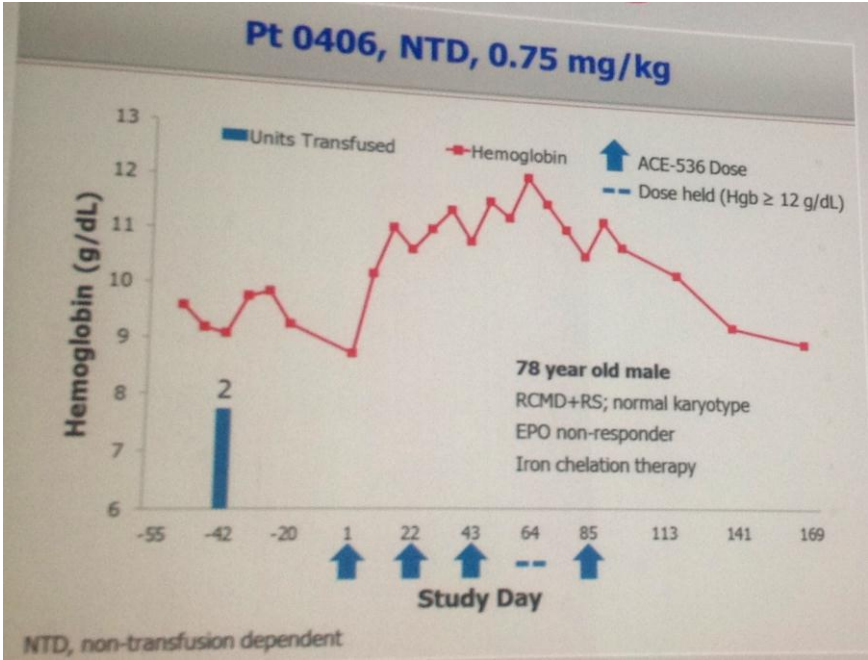
Source: EHA 2014

FIGURE 2. Max Hb Increase in NTD Patients



Source: EHA 2014

FIGURE 3. Individual Patient Showing Durable ≥ 1.5 g/dL Hb increase with ACE-536



Source: EHA 2014

FIGURE 4. ACE-536 Effect on Transfusion Burden in TD Patients

TD Patients: Transfusion Response				
Patient ID	Dose Group (mg/kg)	Prior MDS Therapy	% Change RBC Units	≥ 4 Unit Decrease / 8 Weeks [IWG]
0101	0.125	Len	0	No
0102	0.125	ESA	-50% (4→2)	No
0202	0.25	ESA	-25%	No
0203	0.25	ESA	-50% (4→2)	No
0301	0.5	Len	-50% (8→4)	Yes
0302	0.5	Len	+25%	No
0303	0.5	ESA	0	No
0401	0.75	ESA, Len	-100% (4→0)	Yes
0402	0.75	ESA	0	No
0403	0.75	ESA	+50%	No
0501	1.00	ESA, Len	-33%	No
0502	1.00	ESA, Len	-39% (13→8)	Yes
0503	1.00	ESA, Len	0	No
0601	1.33	ESA	0	No
0602	1.33	ESA	0	No
0603	1.33	ESA	-67% (6→2)	Yes

TD, transfusion dependent; Len, lenalidomide; ESA, erythropoiesis-stimulating agent

Source: EHA 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 upon 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.

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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.

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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.

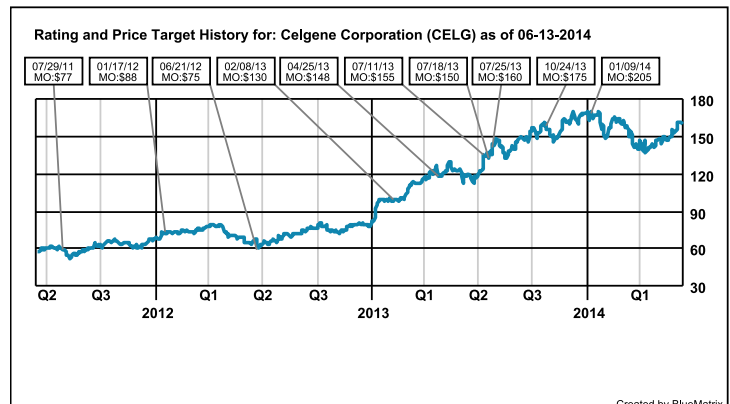
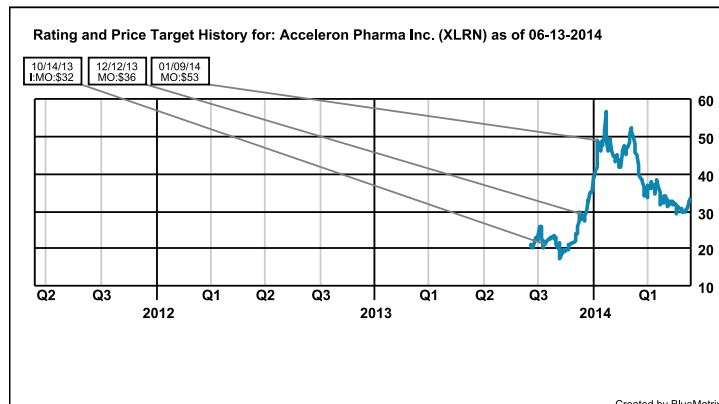
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

JMP Securities Research Ratings and Investment Banking Services: (as of June 14, 2014)

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	261	58.92%	Buy	261	58.92%	98	37.55%
MARKET PERFORM	Hold	135	30.47%	Hold	135	30.47%	19	14.07%
MARKET UNDERPERFORM	Sell	4	0.90%	Sell	4	0.90%	0	0%
COVERAGE IN TRANSITION		43	9.71%		43	9.71%	0	0%
TOTAL:		443	100%		443	100%	117	26.41%

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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