

Accelaron Pharma Inc. (XLRN)

Unfounded Weakness Creates Buying Opportunity into EHA

MARKET DATA

Price	\$29.43
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$779.9
Average Daily Vol. (000):	327.0
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: \$29.43 | Target Price: \$53.00

INVESTMENT HIGHLIGHTS

The Accelaron Pharma share sell-off, in reaction to the EHA abstract release, is unfounded by the data, in our view; reiterate our Market Outperform rating and \$53 price target based on our DCF and SOTP valuation methodologies. In a press release this morning, Accelaron summarized three oral abstracts with ACE-536 and sotatercept being presented at the upcoming EHA Congress (in Milan, Italy, June 12-15; EHA abstracts to be formally released May 22 BMO). Net-net, the abstracts foretell meaningful updates from both drug candidates in beta-thalassemia, including an additional dose cohort data, longer follow-up from previously disclosed dose cohorts, as well as preliminary data from transfusion-dependent (TD) patients. As presented, none of the data contained within the PR give us reason to moderate our positive outlook on the ACE-536/sotatercept chance of success in beta-thal. To the contrary, we view interim data showing reduced transfusion burden by ACE-536 in MDS as a positive read-through to forthcoming TD data in beta-thal.

EHA abstracts for ACE-536/sotatercept in beta-thalassemia more a 'table of contents' than actual 'story'. On their own, the EHA abstracts offer little read-through to either beta-thal program over prior disclosures at ASH in December or company filings in January, with new data being withheld until oral presentation at the congress. With ACE-536 (Abstract #S664, June 14, 8:45 CEST) investors can expect efficacy data from an additional 0.8mg/kg dose cohort in non-transfusion dependent (NTD) patients, longer follow-up from existing cohorts (0.2, 0.4, and 0.6mg/kg), and details related to a reported reduction in transfusion burden among four TD patients treated at 0.6 and 0.8mg/kg. Similarly with sotatercept (#S662, June 15, 8:45 CEST), we can expect data from the higher dose 0.75mg/kg, added follow-up from existing cohorts up to 0.5mg/kg (shown at ASH), and impact on transfusion burden in TD patients across all doses.

First look at ACE-536 data in MDS is impressive, despite small patient numbers. These data include Hgb increases $\geq 1.5\text{g/dL}$ (ranging 1.6 to 3.3 g/dL) in 3 of 5 total NTD patients treated at 0.75mg/kg that were either refractory or unresponsive to prior ESA therapy. Four of 10 TD patients experienced $\geq 50\%$ reductions in transfusion burden over an 8-week period - a potentially understated data point given that some of these patients were likely treated at sub-effective doses. Data from these patients as well as a fifth cohort of 1.0mg/kg will be presented at the upcoming meeting.

Accelaron 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	(\$0.37)
	2Q	\$0.44	(\$0.37)	(\$0.41)
	3Q	(\$5.62)	(\$0.41)	\$0.05
	4Q	(\$0.64)	\$0.05	(\$1.01)
	FY	(\$4.15)	(\$1.01)	(\$1.12)

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
2Q14	Dalantercept	Preliminary data from dose-escalation stage of Phase II RCC trial in combination with axitinib; start of randomized stage versus axitinib alone (ASCO)
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 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: 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 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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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.

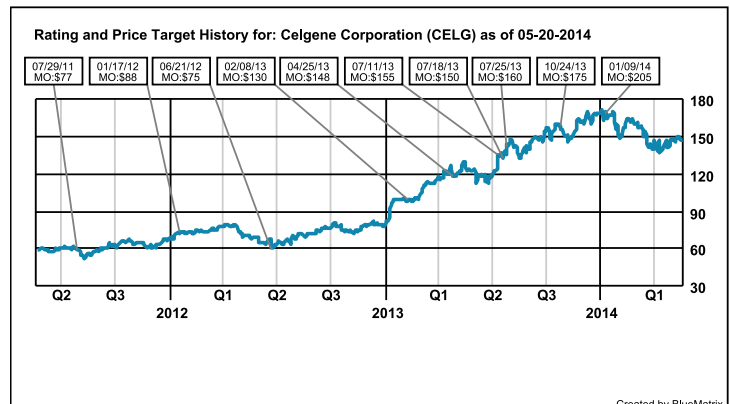
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MARKET OUTPERFORM	Buy	255	57.95%	Buy	255	57.95%	98	38.43%
MARKET PERFORM	Hold	137	31.14%	Hold	137	31.14%	16	11.68%
MARKET UNDERPERFORM	Sell	5	1.14%	Sell	5	1.14%	0	0%
COVERAGE IN TRANSITION		43	9.77%		43	9.77%	0	0%
TOTAL:		440	100%		440	100%	114	25.91%

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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