Acceleron Pharma Inc. (XLRN) — Overweight

Encouraging Anemia Data at EHA; Reiterate Overweight Rating and $65 Target

CONCLUSION

Over the weekend at the European Hematology Association (EHA) meeting, Acceleron and partner Celgene reported data on both Sotatercept and ACE-536 in 3 oral presentations. These data reinforce our view of the potential for Acceleron's anemia franchise in EPO resistant patients. Acceleron and Celgene remain on track to initiate Phase III beta-thalessimia trial likely of ACE-536 around year-end and new MDS trials in 2015. We suspect that the partners will focus sotatercept development in End-Stage Renal Disease (ESRD) patients. We believe XLRN shares are down today because of impressive competitive beta-thalessimia data from gene therapy play bluebird bio (BLUE). We reiterate our Overweight rating and $65 price target.

- **First ACE-536 MDS Data Promising.** Of the 50,000 MDS patients in the U.S., Acceleron is focusing on evaluating ACE-536 as a treatment in the low-to-intermediate-1 (low/int-1), non-del(5q), EPO resistant population (~16,000 patients). At EHA, Acceleron and partner Celgene presented data supporting safe every 3-weeks dosing of ACE-536. ACE-536 lowered transfusion burden by >50% in 6/16 (38%) transfusion-dependent MDS patients. In one case, the patient actually ceased requiring transfusions. Two of the 5 non-transfusion dependent patients had a hemoglobin increase >1.5g/dL for more than 2 weeks, including 1 patient for >8 weeks. The response to ACE-536 therapy (0.125-1.33mg/kg SC Q3W) was dose-dependent and escalation continues at 1.75mg/kg SC Q3W.

- **Both ACE-536 & Sotatercept Active In Beta-Thal.** In the sotatercept study, 32 patients (22 NTD, 10 TD) were administered 0.1-0.75mg/kg Q3W demonstrating dose dependent increase in hemoglobin. In NTD patients, 83% at 0.5mg/kg and 100% at 0.75mg/kg had increases of 1.0d/dL or greater. In TD patients, 3 of 5 patients doses at 0.5mg/kg and 0.75mg/kg saw a >20% reduction in the frequency of transfusions. In the ACE-536 trial, 24 patients received 0.2-0.8mg/kg. In NTD patients, there were increases of hemoglobin levels at all dose levels, including 11/14 patients at the highest doses (0.4, 0.6 and 0.8mg/kg) had hemoglobin increases >1g/dL. One TD patient received 0.6mg/kg and 3 TD patients received 0.8mg/kg, achieving reductions in transfusion burden of -78.5%, -66.7% -66.7% and -69.8%. Based on this data, we expect that Acceleron and Celgene will initiate a Phase III trial looking at ACE-536 in beta-thalessemia around year-end.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Sotatercept, ACE-536 and/or dalantercept may fail in the clinic or to gain regulatory approval. The Celgene partnership may falter. Acceleron may require additional capital or could face future unforeseen litigation.

COMPANY DESCRIPTION

Acceleron is developing novel drugs for hematology and cancer.

PRICE: US$33.88
TARGET: US$65.00
Proj. EV of $1.9B + YE:14E net cash

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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	—	US$65.00
FY14E Rev (mil)	—	US$22.5
FY15E Rev (mil)	—	US$20.4
FY14E EPS	—	US$(1.47)
FY15E EPS	—	US$(1.98)

52-Week High / Low	US$57.89 / US$15.00
Shares Out (mil)	31.5
Market Cap. (mil)	US$1,067.2
Avg Daily Vol (000)	361
Book Value/Share	US$5.74
Net Cash Per Share	US$6.81
Debt to Total Capital	0%
Div (ann)	NA
Fiscal Year End	Dec

Price Performance - 1 Year

Source: Bloomberg

YEAR	REVENUE (US$ m)						EARNINGS PER SHARE (US$)					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2013A	15.0	26.4	4.1	11.1	56.7	18.8x	0.13	0.64	(0.54)	(0.21)	(0.07)	NM
2014E	3.3A	3.0	3.1	13.1	22.5	47.4x	(0.30)A	(0.46)	(0.49)	(0.22)	(1.47)	NM
2015E	—	—	—	—	20.4	52.3x	—	—	—	—	(1.98)	NM

Murine ACE-536 Shows Benefit In Mice. Although investors will focus on data from the MDS and beta-thal studies, preclinical data in mice examining ACE-536 and its potential in sickle cell disease showed some beneficial activity. In the study, the murine version of ACE-536 showed benefit for a variety of endpoints including a 66.5% reduction in irreversibly sickled cells versus the control, a 20.5% decrease in spleen weight as well as decreases in multiple key biomarkers (Annexin V/PS, reactive oxygen species, total billirubin, cell free hemoglobin and blood urea nitrogen). The data also seemed to indicate a reduced stickiness in the murine ACE-536 arm. On the whole the data was quite interesting. We expect Acceleron and Celgene will continue to evaluate ACE-536 in this indication.

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Acceleron Pharma Inc.
Quarterly Earnings Estimates
($ in thousands, except per share data)

5/15/2014

	1QA	2QA	3QE	4QE	2013E	1QA	2QE	3QE	4QE	2014E	2015E
Collaboration Revenue:											
License and milestone	$12,515	$22,891	$625	$7,625	$43,656	$0	$0	$100	$10,100	$10,200	$10,400
Cost-sharing, net	2,497	3,537	3,500	3,500	13,034	3,307	3,000	3,000	3,000	12,307	10,000
Total Revenues	**$15,012**	**$26,428**	**$4,125**	**$11,125**	**$56,690**	**$3,307**	**$3,000**	**$3,100**	**$13,100**	**$22,507**	**$20,400**
Operating Expenses:											
Research and Development	8,780	8,911	9,500	10,000	37,191	11,765	12,500	13,000	14,000	51,265	60,000
General and Administrative	3,096	3,365	3,500	4,000	13,961	3,750	4,000	4,500	5,000	17,250	20,000
Total Operating Expenses	**$11,876**	**$12,276**	**$13,000**	**$14,000**	**$51,152**	**$15,515**	**$16,500**	**$17,500**	**$19,000**	**$68,515**	**$80,000**
Operating Income/(Loss)	**$3,136**	**$14,152**	**($8,875)**	**($2,875)**	**$5,538**	**($12,208)**	**($13,500)**	**($14,400)**	**($5,900)**	**($46,008)**	**($59,600)**
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Total Other Income/(Expense)[1]	(422)	(641)	(3,075)	(2,950)	(7,088)	3,088	(1,050)	(1,100)	(1,150)	(212)	(5,100)
Pretax Income/(Loss)	**$2,714**	**$13,511**	**($11,950)**	**($5,825)**	**($1,550)**	**($9,120)**	**($14,550)**	**($15,500)**	**($7,050)**	**($46,220)**	**($64,700)**
Pretax Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Income Tax	0	0	0	0	0	0	0	0	0	0	0
Tax Rate	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%
Net Income/(Loss)	**$2,714**	**$13,511**	**($11,950)**	**($5,825)**	**($1,550)**	**($9,120)**	**($14,550)**	**($15,500)**	**($7,050)**	**($46,220)**	**($64,700)**
Net Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net Income/(Loss) per Share	**$0.13**	**$0.64**	**($0.54)**	**($0.21)**	**($0.07)**	**($0.30)**	**($0.46)**	**($0.49)**	**($0.22)**	**($1.47)**	**($1.98)**
Basic Shares Outstanding	20,954	20,954	22,250	28,250	23,102	30,321	31,500	31,750	32,000	31,393	32,650

Source: Company reports and Piper Jaffray & Co. analysis.
Note: Acceleron competed its IPO on September 18, 2013 and has not yet provided fully quarterly results for 2012.
1. 2012, 1Q:13 and 2Q:13 Total Other Income/(Expense) line incl. extinguishment of convertible preferred stock and change in fair value of warrants.

Current disclosure information for this company can be found at http://www.piperjaffray.com/researchdisclosures.

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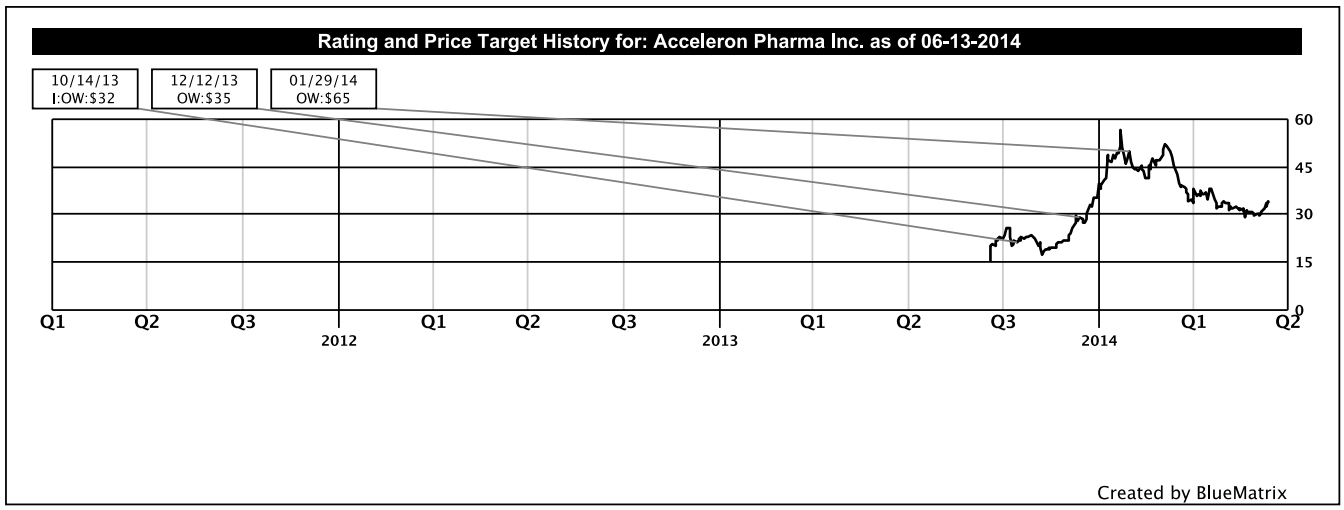

Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written during the past three years.

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I: Initiating Coverage
R: Resuming Coverage
T: Transferring Coverage
D: Discontinuing Coverage
S: Suspending Coverage
OW: Overweight
N: Neutral
UW: Underweight
NA: Not Available
UR: Under Review

Distribution of Ratings/IB Services
Piper Jaffray

			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OW]	355	61.95	87	24.51
HOLD [N]	203	35.43	21	10.34
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