Acceleron Pharma Inc. (XLRN) — Overweight

Dalantercept Benefits In RCC and Head & Neck, Anemia Data on Tap For EHA

CONCLUSION

Acceleron presented 3 posters on dalantercept at the American Society for Clinical Oncology (ASCO) meeting being held in Chicago. Two of the posters examined the dalantercept in renal cell carcinoma (RCC) and head & neck cancer, both of which show encouraging efficacy. A poster that evaluated the drug in endometrial cancer showed limited benefit and as such the company will discontinue investigating dalantercept in this cancer. Later this month, we expect to see Phase II data on both sotatercept and ACE-536 at the European Hematology Association (EHA) meeting. We reiterate our Overweight rating and price target of $65.

- **Dalantercet Active in RCC.** Part 1 of the Phase II RCC trial was a dose escalation study (Abstract #4566). In 20 evaluable RCC patients, dalantercept in combination with INLYTA (axtinib) achieved an objective response rate (ORR) of 25% (5/20) and 10 patients had stable disease equating to a disease control rate (DCR) of 75%. There were no dose-limiting toxicities. Based on these results, Acceleron has selected the dalantercept dose to be used in Part 2 of this study, which should begin shortly in 130 RCC patients and has progression free survival (PFS) as a primary endpoint.

- **Also Shows Benefit in Head & Neck Cancer.** In 40 evaluable heavily pre-treated squamous head & neck cancer patients (Abstract #6045), dalantercept achieved a DCR of 45%. The benefit from therapy was dose dependent with a 48.1% DCR in 1.2 mg/kg patients and 38.5% in the 0.6mg/kg group. Median overall survival (OS) was 9.5 months at the higher dose and 7.1 at the lower dose. Typically, H&N cancer patients at similar staging would have a mean OS of ~5.9 months. PFS was similar between both dose levels. Considering how heavily pretreated these patients were (median of 4 prior regimens), Acceleron will continue to investigate dalantercept in H&N cancer.

- **Focus Shifts Back to Anemia Franchise.** At EHA being held June 12th-15th, we expect to see updated Phase II data on both Sotatercept and ACE-536 in beta-thalassemia. We also expect to see data evaluating ACE-536 in myelodysplastic syndrome (MDS). The partners also intend to present pre-clinical data for ACE-536 in sickle cell disease. Celgene is conducting a Phase II end-stage renal disease (ESRD) study with a 60-patient dose-escalation and 230-patient randomized control study versus ESA. Celgene is responsible for all costs of these drugs going forward with Acceleron retaining co-promote rights in North America and low-to-mid 20% royalties. Celgene intends to begin a Phase III trial in Beta-thalassemia by early 2015.

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$30.00
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$945.0
Avg Daily Vol (000)	338
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	16.7x	0.13	0.64	(0.54)	(0.21)	(0.07)	NM
2014E	3.3A	3.0	3.1	13.1	22.5	42.0x	(0.30)A	(0.46)	(0.49)	(0.22)	(1.47)	NM
2015E	—	—	—	—	20.4	46.3x	—	—	—	—	(1.98)	NM

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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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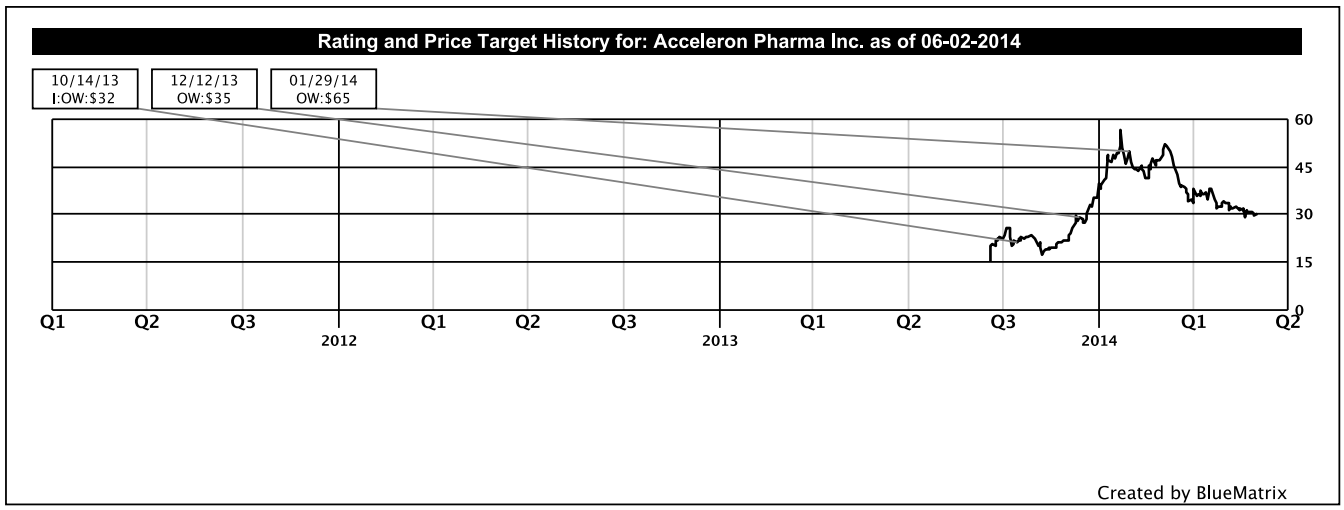

Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written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

Legend:
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]	354	61.78	90	25.42
HOLD [N]	203	35.43	21	10.34
SELL [UW]	16	2.79	0	0.00

Note: Distribution of Ratings/IB Services shows the number of companies currently in each rating category from which Piper Jaffray and its affiliates received compensation for investment banking services within the past 12 months. FINRA rules require disclosure of which ratings most closely correspond with "buy," "hold," and "sell" recommendations. Piper Jaffray ratings are not the equivalent of buy, hold or sell, but instead represent recommended relative weightings. Nevertheless, Overweight corresponds most closely with buy, Neutral with hold and Underweight with sell. See Stock Rating definitions below.

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