Acceleron Pharma Inc. (XLRN) — Overweight

Dalantercept Active In RCC & Head & Neck Cancer, Awaiting More Data At ASCO

CONCLUSION

Acceleron ended 1Q:14 with $214.1 million and sold 1.1 million shares to Celgene on May 5th bringing proforma cash to $261.2 million. The company will present two posters on wholly-owned dalantercept in renal cell carcinoma (RCC) in combination with axtinib as well as head & neck at ASCO at the end of the month. We also expect to see Phase II data on both sotatercept and ACE-536 at the European Hematology Association (EHA) meeting in Milan next month. We reiterate our Overweight rating and price target of $65.

- **Enough cash to carry through to 2017.** Acceleron ended 1Q:14 with cash of $214.1 million including net proceeds of $129 million received in a January follow-on offering. On May 5, the company sold 1.1 million shares to Celgene for $47.1 million, who now holds 14.6% of Acceleron, and brings proforma cash to $261.2 million. As Celgene covers the development costs of Acceleron's anemia franchise, the cash should last through 2017.

- **Dalantercept data at ASCO.** Acceleron provided a first look at data from the RCC and head & neck cancer Phase II studies of dalantercept that will be presented at the American Society of Oncology (ASCO) annual meeting being held May 30-June 3 in Chicago. 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 patients) 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 dose to be used in Part 2 of this study, which should begin shortly in 130 RCC patients. 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, head & neck cancer patients at similar staging would have a mean OS of ~5.9 months. Investigators will also report data on dalantercept in endometrial cancer (Abstract #5594), although the study showed limited monotherapy activity. Dalantercept will likely be further examined in this cancer as part of a combination therapy. The company expects to initiate a Phase Ib dalantercept trial in HCC in the near-term.

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$31.56
Note: Price as of the close May 15, 2014.

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$23.5	US$22.5
FY15E Rev (mil)	—	US$20.4
FY14E EPS	US$(1.58)	US$(1.44)
FY15E EPS	US$(2.01)	US$(1.92)

52-Week High / Low	US$57.89 / US$15.00
Shares Out (mil)	32.6
Incl. 1.1M shares sold to CELG on May 5th	
Market Cap. (mil)	US$1,028.9
Avg Daily Vol (000)	319
Book Value/Share	US$5.54
Net Cash Per Share	US$8.03
Pro forma cash incl. equity sale to CELG	
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	4.3	11.5	57.2	18.0x	0.08	0.62	(0.86)	(0.64)	(0.96)	NM
2014E	3.3A	3.0	3.1	13.1	22.5	45.7x	(0.30)A	(0.45)	(0.47)	(0.21)	(1.44)	NM
2015E	—	—	—	—	20.4	50.4x	—	—	—	—	(1.92)	NM

- **After ASCO, Focus Will Turn Back To Anemia Franchise.** At the European Hematology Association (EHA) meeting being held June 12th-15th, we expect to see updated Phase II data evaluating both Sotatercept and ACE-536 in beta-thalassemia. We also expect to see data evaluating ACE-536 in myelodysplastic syndrome (MDS). After recent data showing activity for sotatercept in anemia for end-stage renal disease (ESRD) patients, partner Celgene is initiating a Phase II ESRD study with a 60-patient dose-escalation and 230-patient randomized control study versus ESA. The companies also intend to present pre-clinical data for ACE-536 in sickle cell disease that point to some potential in this challenging indication. 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. We anticipate Celgene will begin a Phase III trial in Beta-thalassemia in late 2014 or early 2015.

- **ACE-083 Phase I To Start 2H:14.** The company is prepping to submit an IND for this compound as a therapy that encourages both increased physical strength in addition to muscle mass.

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

May 15, 2014

	1QA	2QA	3QA	4QA	2013A	1QA	2QE	3QE	4QE	2014E	2015E
Collaboration Revenue:											
Total Revenues	**$15,012**	**$26,428**	**$4,270**	**$11,521**	**$57,230**	**$3,307**	**$3,000**	**$3,100**	**$13,100**	**$22,507**	**$20,400**
Operating Expenses:											
Research and Development	8,780	8,911	8,143	10,216	36,050	11,765	12,500	13,000	14,000	51,265	60,000
General and Administrative	3,096	3,365	3,011	4,756	14,228	3,750	4,000	4,500	5,000	17,250	20,000
Total Operating Expenses	**$11,876**	**$12,276**	**$11,154**	**$14,972**	**$50,278**	**$15,515**	**$16,500**	**$17,500**	**$19,000**	**$68,515**	**$80,000**
Operating Income/(Loss)	**$3,136**	**$14,152**	**($6,884)**	**($3,451)**	**$6,952**	**($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]	(1,489)	(1,074)	(11,629)	(14,659)	(28,850)	3,088	(1,050)	(1,100)	(1,150)	(212)	(5,100)
Pretax Income/(Loss)	**$1,647**	**$13,078**	**($18,513)**	**($18,110)**	**($21,898)**	**($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)	**$1,647**	**$13,078**	**($18,513)**	**($18,110)**	**($21,898)**	**($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
Other Items	0	0	0	0	0	0	0	0	0	0	0
Net income applicable to common shareholders[1]	**$1,647**	**$13,078**	**($18,513)**	**($18,110)**	**($21,898)**	**($9,120)**	**($14,550)**	**($15,500)**	**($7,050)**	**($46,220)**	**($64,700)**
Net Income/(Loss) per Share	**$0.08**	**$0.62**	**($0.86)**	**($0.64)**	**($0.96)**	**($0.30)**	**($0.45)**	**($0.47)**	**($0.21)**	**($1.44)**	**($1.92)**
Basic Shares Outstanding[1]	20,954	20,954	21,500	28,123	**22,898**	30,321	32,250	32,750	33,000	**32,080**	**33,650**

Source: Company reports and Piper Jaffray & Co. analysis.
Note: Acceleron competed its IPO on September 18, 2013 and a follow-on offering on January 22, 2014
1. Net income applicable to common shareholders for 1Q:13A, 2Q:13A, 3Q:13A and 2013A assumes full conversion of convertible preferred stock.

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

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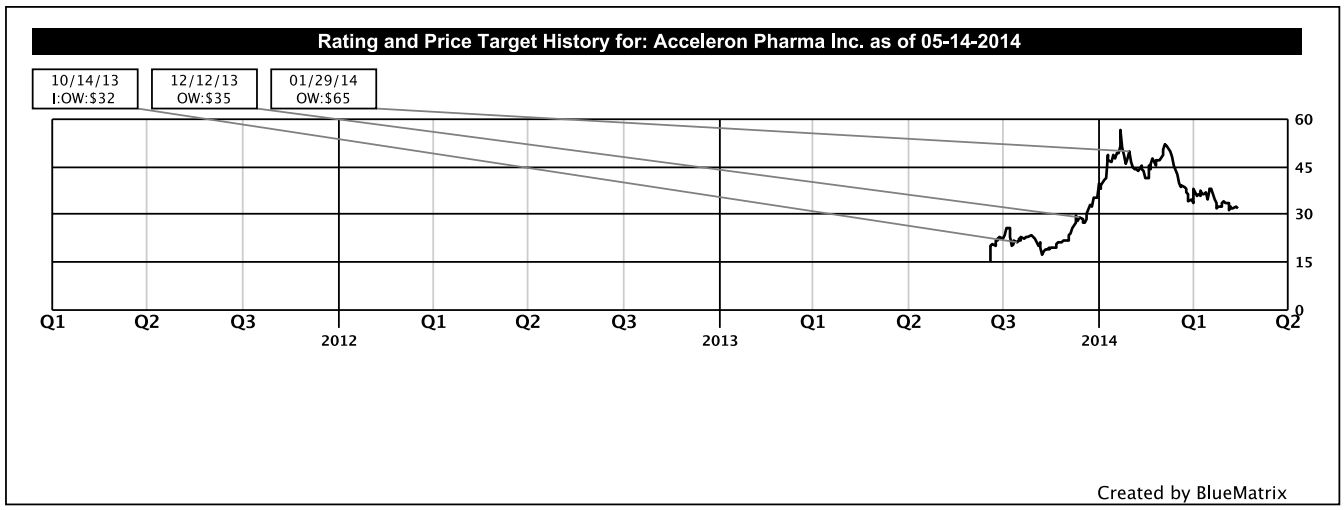

Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written 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]	355	61.74	88	24.79
HOLD [N]	203	35.30	20	9.85
SELL [UW]	17	2.96	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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- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

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