

## Acceleron Pharma Inc. (XLRN)

Acceleron Announces Interim Data to be Presented at the National Kidney Foundation Meeting

### MARKET DATA

Price	\$42.50
52-Week Range:	\$16.78 - \$57.89
Shares Out. (M):	26.5
Market Cap (\$M):	\$1,126.3
Average Daily Vol. (000):	254.0
Cash (M):	\$116
Cash/Share:	\$3.74
Enterprise Value (M):	\$1,360
Float (M):	27.0
LT Debt (M):	\$11

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$42.50 | Target Price: \$53.00

### INVESTMENT HIGHLIGHTS

**Dose-dependent increase in hemoglobin highlights budding success of sotatercept in ESRD; reiterate our Market Outperform rating on Acceleron Pharma and our \$53 price target based on our DCF and SOTP valuation methodologies.**

Acceleron announced that its collaboration partner Celgene (CELG, MO, \$205 PT) will present interim data from its Phase IIa study of sotatercept in patients with end-stage renal disease (ESRD) at the National Kidney Foundation meeting (NKF) taking place April 22 to 26 in Las Vegas, NV. Interim safety, pharmacologic, and hemoglobin data will be presented by Dr. William Smith of Celgene Corporation from the first 28-day dose cycle for two dose groups of sotatercept (0.3 mg/kg and 0.5 mg/kg) versus placebo. After a single dose in the placebo, 0.3 mg/kg, and 0.5 mg/kg sotatercept dose groups, mean peak hemoglobin in the first 28 days increased by 0.1, 0.5, and 0.8 g/dL, respectively. The company has reported that adverse events in the study were mostly mild, unrelated to study drug, and generally consistent with patients' medical histories. In addition, no dose-dependent changes in blood pressure were observed. These data will be further detailed in a poster presentation on April 23rd from 6-7:30 p.m. PDT.

**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. In our opinion, the company's first wave of product candidates is the tip of the iceberg that, over time, should create significant value for shareholders.

FY DEC	2012A	2013A	2014E
Revenue (\$M) 1Q	--	\$15.0	\$4.9
2Q	--	\$26.4	\$5.2
3Q	--	\$4.3	\$5.4
4Q	--	\$11.5	\$20.9
<b>FY</b>	<b>\$15.3</b>	<b>\$57.2</b>	<b>\$36.4</b>
EPS 1Q	--	\$0.12	(\$0.38)
2Q	--	\$0.44	(\$0.38)
3Q	--	(\$5.62)	(\$0.42)
4Q	--	(\$0.64)	\$0.04
<b>FY</b>	<b>(\$1.43)</b>	<b>(\$4.15)</b>	<b>(\$1.13)</b>

Source: Company reports and JMP Securities LLC

### STOCK PRICE PERFORMANCE



**FIGURE 1. Upcoming Milestones**

Timing	Drug	Milestones
1Q14	Sotatercept	Initiation of Phase II trial in patients with end-stage renal disease (ESRD)
1Q14	Dalantercept	Preliminary data from Phase II, GOG-sponsored trials in ovarian cancer
1Q14	Sotatercept & ACE-536	Initiation of RP2D expansion cohorts in ongoing $\beta$ -thalassemia Phase II trials
1Q14	Dalantercept	Preliminary data from dose-escalation stage of Phase II RCC trial in combination with axitinib; start of randomized stage versus axitinib alone
2Q14	Sotatercept & ACE-536	Presentation of dose escalation Phase II results in $\beta$ -thalassemia and MDS
3Q14	Dalantercept	Initiation of Phase II trial(s) in additional indication(s)
4Q14	Sotatercept & ACE-536	Final results from Phase II trials in $\beta$ -thalassemia and MDS
4Q14	Sotatercept & ACE-536	Initiation of Phase III trial in $\beta$ -thalassemia and/or MDS
4Q14	ACE-083	Initiation of Phase I trial in muscular dystrophy

Source: JMP Securities LLC and 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 $\beta$  signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to  $\beta$ -thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGF $\beta$ .

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  $\beta$ -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.

## JMP FACTS AND DISCLOSURES

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### JMP Securities Disclosures:

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, and received compensation for doing so, for Accelaron Pharma Inc. in the past 12 months.

### JMP Securities Investment Opinion Definitions:

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.

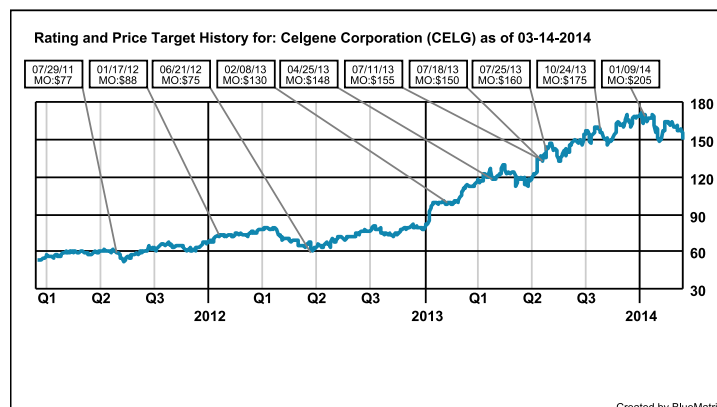
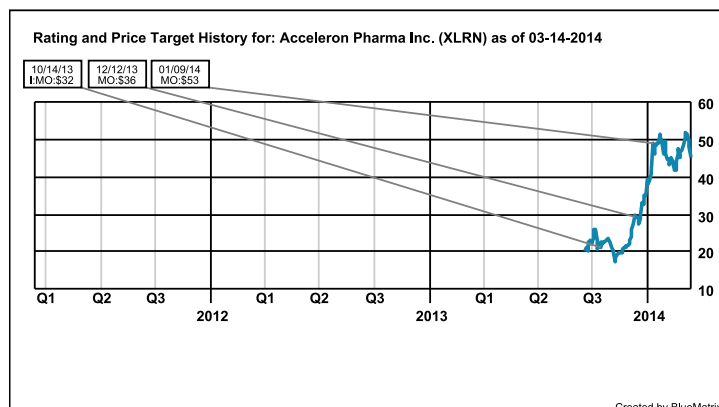
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

### JMP Securities Research Ratings and Investment Banking Services: (as of March 16, 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	248	56.88%	Buy	248	56.88%	96	38.71%
MARKET PERFORM	Hold	138	31.65%	Hold	138	31.65%	17	12.32%
MARKET UNDERPERFORM	Sell	7	1.61%	Sell	7	1.61%	0	0%
COVERAGE IN TRANSITION		43	9.86%		43	9.86%	0	0%
TOTAL:		436	100%		436	100%	113	25.92%

### 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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Jeffrey H. Spurr  
Director of Research  
(415) 835-3903

## RESEARCH PROFESSIONALS

### FINANCIAL SERVICES

#### Alternative Asset Managers

Devin Ryan	(212) 906-3578
Brian McKenna	(212) 906-3545

#### Commercial & Specialty Finance

Christopher York	(415) 835-8965
Hannah Kim, CFA	(415) 835-8962

#### Consumer Finance

David M. Scharf	(415) 835-8942
Jeremy Frazer	(312) 768-1796

#### Financial Processing & Outsourcing

David M. Scharf	(415) 835-8942
Jeremy Frazer	(312) 768-1796

#### Insurance

Matthew J. Carletti	(312) 768-1784
Christine Worley	(312) 768-1786

#### Investment Banks & Brokers

Devin Ryan	(212) 906-3578
Brian McKenna	(212) 906-3545

#### Mortgage Operating Companies

##### REITs: Agency, Hybrid, & Commercial Mortgage

Steven C. DeLaney	(404) 848-7773
Trevor Cranston, CFA	(415) 869-4431
Charter Robinson	(757) 613-8955
Benjamin Zucker	(212) 906-3529

### HEALTHCARE

#### Biotechnology

Liisa A. Bayko	(312) 768-1785
Heather Behanna, PhD	(312) 768-1795
Andrew Prigodich	(312) 768-1788
Jason N. Butler, PhD	(212) 906-3505
Christopher T. Radom, PhD	(212) 906-3519
Caroline Palomeque	(212) 906-3509
Michael G. King, Jr.	(212) 906-3520
Eric Joseph, PhD	(212) 906-3514
Joseph A. Knowles	(212) 906-3525

#### Healthcare Services & Facilities

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Arthur Kwok	(415) 835-8908

#### Life Science Tools & Diagnostics

J. T. Haresco, III, PhD	(415) 869-4477
Marie T. Casey, PhD	(415) 835-3955

#### Medical Devices

J. T. Haresco, III, PhD	(415) 869-4477
Marie T. Casey, PhD	(415) 835-3955

#### Medical Devices & Supplies

David Turkaly	(212) 906-3563
John Gillings	(212) 906-3564

### REAL ESTATE

#### Housing & Land Development

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Bharathwajan Iyengar	(415) 835-3902

#### Lodging & Leisure

Robert A. LaFleur	(212) 906-3510
Whitney Stevenson	(212) 906-3538

#### Property Services

Mitch Germain	(212) 906-3546
Peter Lunenburg	(212) 906-3537

#### REITs: Healthcare, Residential, & Specialty

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Arthur Kwok	(415) 835-8908

#### REITs: Office, Industrial, & Diversified

Mitch Germain	(212) 906-3546
Peter Lunenburg	(212) 906-3537

#### Residential Services

Peter L. Martin, CFA	(415) 835-8904
Aaron Hecht	(415) 835-3963
Bharathwajan Iyengar	(415) 835-3902

### TECHNOLOGY

#### Communications Equipment & Internet Security

Erik Suppiger	(415) 835-3918
John Lucia	(415) 835-3920

#### Internet & Digital Media

Ronald V. Josey III	(212) 906-3528
Andrew Boone	(415) 835-3957

#### Software

Patrick Walravens	(415) 835-8943
Peter Lowry	(415) 869-4418
Caitlin Schields	(415) 835-8960
Greg McDowell	(415) 835-3934

#### Wireless & Cloud Computing Technologies

Alex Gauna	(415) 835-8998
Michael Wu	(415) 835-8996

## ADDITIONAL CONTACTS

Thomas R. Wright  
Director of Equities  
(212) 906-3599

Dan Wychulis  
Director of Institutional Sales  
(617) 235-8530

600 Montgomery Street, Suite 1100  
San Francisco, CA 94111  
[www.jmpsecurities.com](http://www.jmpsecurities.com)