Acceleron Pharma Inc. (XLRN) — Overweight

Promising Anemia and Cancer Play; Initiating Overweight with $32 Price Target

CONCLUSION

Acceleron is a biotechnology company focused on developing biotherapeutic drugs that target the TGF-β super family of proteins. The company's anemia drugs, sotatercept and ACE-536, are partnered with Celgene and currently in Phase II Beta-thalassemia and Myelodysplastic Syndromes (MDS) trials. We are most interested in Acceleron's wholly-owned dalantercept, a novel anti-angiogenic agent currently in a Phase I/II trial in renal cell carcinoma (RCC) in combination with axitinib. Acceleron recently completed a successful IPO and Celgene invested $10 million in a concurrent private placement, bringing pro forma cash of ~$126 million. We are initiating coverage of Acceleron with an Overweight rating and $32 price target.

- **Dalantercept the Next Avastin?** While most investors are focused on the Celgene deal and anemia drugs, we are more interested in wholly owned dalantercept. Acceleron is conducting a Phase I/II trial of dalantercept in combination with axtiinib in RCC with data likely at ASCO next June. Acceleron intends to begin another Phase I/II combo trial in either lung, liver or colon cancer in 2H:14. We anticipate dalantercept clinical progress will drive value with blockbuster potential in multiple cancers.

- **Validating Partnerships with Celgene.** In February 2008, Acceleron signed a global alliance with Celgene to develop sotatercept and later signed a 2nd deal in August 2011 for ACE-536. Acceleron has received ~$130 million from Celgene to date and is eligible for up to $567 million in milestones. Celgene is now responsible for all costs going forward. Acceleron retains co-promote rights for both sotatercept and ACE-536 in North America and is due low-to-mid 20% royalties.

- **Potential Blockbuster Anemia Drugs.** Sotatercept is currently in Phase II trials in Beta-thalassemia with dose-escalation data at the American Society of Hematology (ASH) meeting in December, as well as MDS. ACE-536 acts via a unique mechanism and is also in Phase II trials in Beta-Thalassemia and MDS with substantive data at the European Hematology Association (EHA) meeting next June. We anticipate Celgene will begin Phase III trials of sotatercept and/or ACE-536 in either indication in late 2014 or early 2015. Celgene intends to develop these drugs broadly to treat anemia with blockbuster sales potential.

RISKS TO ACHIEVEMENT OF PRICE TARGET

Sotatercept, ACE-536 and/or dalantercept may fail in the clinical 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$22.12
TARGET: US$32.00
Proj. EV of $875M + YE:14E net cash

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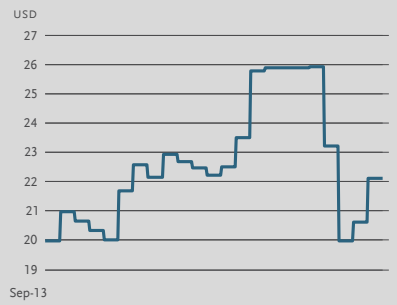
Changes	Previous	Current
Rating		Overweight
Price Tgt		US$32.00
FY13E Rev (mil)	—	56.7
FY14E Rev (mil)	—	23.5
FY13E EPS	—	(0.07)
FY14E EPS	—	(1.98)

52-Week High / Low	US$26.73 / US$15.00
Shares Out (mil)	28.0
Incl. impact of IPO, green shoe & CELG private placement	
Market Cap. (mil)	US$620.4
Book Value/Share	US$2.85
Net Cash Per Share	US$4.03
Debt to Total Capital	10%
Div (ann)	NA
Fiscal Year End	Dec

Pro forma cash incl. IPO, , green shoe & CELG private placement less SVB debt

Price Performance - 1 Year



Source: Bloomberg

YEAR	REVENUE (m)						EARNINGS PER SHARE ( )					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2012A	—	—	—	—	15.3	40.6x	—	—	—	—	(1.44)	NM
2013E	15.0A	26.4A	4.1	11.1	56.7	10.9x	0.13A	0.64A	(0.54)	(0.21)	(0.07)	NM
2014E	3.6	3.6	3.1	13.1	23.5	26.4x	(0.50)	(0.56)	(0.61)	(0.31)	(1.98)	NM

IMPRESSIVE PIPELINE PROVIDES SEVERAL UPCOMING VALUE DRIVERS

Acceleron is a biotechnology company focused on developing biotherapeutic drugs that target the TGF-β (transforming growth factor-beta) superfamily of proteins. TGF-β proteins are key regulators of tissue repair and growth. The TGF-β superfamily is comprised of 30 ligands that are classified as Activins, Growth and Differentiation Factors (GDFs) and Bone Morphogenic Proteins (BMPs). These ligands bind to 12 known receptors. Acceleron has licensed intellectual property on nine of these receptors, providing a rich source of novel drugs to treat a variety of diseases. The company has already advanced 3 fusion proteins into Phase II trials including **sotatercept** and **ACE-526**, both partnered with Celgene (CELG), as well as wholly owned **dalantercept**. (Please see Exhibit 1 below.) In addition, Acceleron hopes to advance **ACE-083** into the clinic next year for the treatment of muscle wasting disorders.

In February 2008, Acceleron partnered sotatercept (ACE-011) with Celgene. In August 2011, the partners signed a second collaboration to include ACE-536. Both sotatercept and ACE-536 stimulate red blood cell (RBC) count to treat anemia. Importantly, the agents appear to work downstream of erythropoietin (EPO) with potential safety advantages. To date, Acceleron has received ~$130 million and stands to receive up to $567 million in milestones. Importantly Celgene is responsible for all development, manufacturing and commercialization expenses going forward. The partners will co-promote sotatercept and/or ACE-536 in the U.S., with Celgene paying for Acceleron's sales force; and Acceleron is eligible for low-to-mid 20% royalties.

Both sotatercept and ACE-536 are currently in 2 Phase II trials to treat anemia caused by Beta-Thalassemia and Myelodysplastic Syndrome (MDS). Celgene will report Phase II dose-escalation data on sotatercept at the American Society of Hematology (ASH) meeting in December in New Orleans and substantively more data at the European Hematology Association (EHA) meeting next June in Milan. Depending upon the Phase II data, we anticipate Celgene could initiate Phase III trials of sotatercept and/or ACE-536 in MDS and/or Beta-thalassemia in late 2014 and early 2015.

It is difficult to envision a better hematology partner than Celgene or a better deal for Acceleron considering the company has to invest nothing going forward and retains such rich economics. (Celgene invested $10 million along with the IPO and now owns ~11% of Acceleron.) We envision Celgene will develop sotatercept and/or ACE-536 broadly to treat anemia as evidenced by the recently initiated Phase II trial in chronic kidney disease (CKD), triggering a $7 million milestone to Acceleron in 4Q:13. Investigator sponsored studies are also underway in multiple myeloma, Diamond-Blackfan anemia and myelofibrosis. Sales of Amgen's *Epogen* and *Aranesp* plus Johnson & Johnson's *Procrit* were almost $4 billion over the last 12 months, however have been on the decline despite steady price increases due to safety issues and labeling restrictions. We see the opportunity for a safe alternative to EPO products as a potential blockbuster for Celgene.

While most investors are focused on the Celgene deal and anemia drugs, we are more interested in dalantercept. First, the majority of Acceleron's proprietary R&D investment and IPO funds will go to dalantercept. Second, as a wholly owned asset, we see more potential value creation coming from clinical validation and progress of dalantercept.

Dalantercept is a fusion protein of activin receptor-like kinase 1 (ALK1), which promotes blood vessel formation and maturation. By inhibiting this pathway, dalantercept is an anti-angiogenic therapy being developed for cancer. Anti-angiogenic therapy has become a primary weapon in the armamentarium of the oncologist. Genentech's anti-VEGF antibody *Avastin* achieved global sales of $6.2 billion last year! Dalantercept could be used in Avastin-failure patients and in combination with oral anti-VEGF inhibitors to boost efficacy. To this point, a Phase I/II combo study of dalantercept with axitinib in renal cell carcinoma is underway with preliminary data at the American Society of Clinical Oncology (ASCO) next June. Acceleron intends to initiate a 2nd Phase I/II combo study in either liver, lung or colon cancer in 2H:14. Acceleron is also conducting a Phase II monotherapy study of dalantercept in head & neck cancer and investigator sponsored studies in endometrial and ovarian cancer, but the path forward will likely be combination studies.

Upcoming Events and Expected Timing

- Investigators to report initial Phase II dalantercept data in endometrial cancer in 4Q:13
- Celgene to report Phase II dose-escalation data on sotatercept at ASH in December
- Acceleron to report full Phase II dalantercept data in Head & Neck cancer in 1H:14
- Investigators to report Phase II sotatercept data in Diamond-Blackfan anemia in 1H:14
- Investigators to report Phase II sotatercept data in multiple myeloma in 2014
- Celgene to report initial Phase II data on sotatercept in chronic kidney disease in 1H:14
- Investigators to report initial Phase II dalantercept data in ovarian cancer in 1H:14
- Acceleron to report initial Phase I/II dalantercept data in RCC at ASCO in June '14
- Celgene to report additional Phase II data on both sotatercept and ACE-536 in MDS and Beta-thalassemia at EHA in June 2014
- Celgene to initiate Phase III trials of sotatercept and/or ACE-536 in either MDS and/or Beta-thalassemia in late 2014/early 2015
- Acceleron to file an IND for ACE-083 for muscle wasting disorders in 2014

Exhibit 1

ACCELERON'S PRODUCT PIPELINE

	Preclinical	Phase I	Phase II	Phase III	Market	Comments
Sotatercept						
β-Thalessemia						
Myelodysplastic Syndromes (MDS)						
Chronic Kidney Disease						Partnered with Celgene.
Muliple Myeloma						
Diamond-Blackfan Anemia						
Myelofibrosis						
ACE-536						
β-Thalessemia						Partnered with Celgene.
Myelodysplastic Syndromes (MDS)						
Dalantercept						
Head & Neck Cancer						
Renal Cell Carcinoma						Acceleron maintains worldwide rights at this time.
Endometrial Cancer						
Ovarian Cancer						

Source: Company Reports

INVESTMENT RECOMMENDATION

We are initiating coverage of Acceleron with an Overweight rating and a $32 price target based on a projected enterprise value of $875 million. We value dalantercept at $588 million by applying our industry standard 5x multiple to 2021 WW sales in RCC of $882 million plus $705 million in sales in a 2nd indication; both are discounted back at 45% through YE:14. We value Acceleron's portion of sotatercept/ACE-536 revenues at $287 million by applying a 5x multiple to 2019 MDS anemia royalties of $215 million and 2020 Beta-thalassemia royalties of $221 million; both are discounted back at 45% through YE:14. We believe these discount rates are appropriate for Phase II assets. Positive clinical data could result in a lower discount rate and an increase in our price target.

Acceleron completed a successful IPO on September 18, issuing 6.5 million primary XLRN shares (including a 937,000 green shoe) at $15.00 per share. In addition, Celgene invested $10 million at the IPO price in a concurrent private placement. We estimate Acceleron now holds pro forma cash of ~$126 million. Thus we add YE:14E cash of $51 million in our valuation methodology to arrive at a projected market capitalization of $926 million. We divide this by 29 million YE:14E shares outstanding to arrive at our $32 price target.

Acceleron is currently trading at a market cap of $620 million with estimated pro forma cash following the IPO of ~$126 million, equating to an enterprise value of $507 million. Acceleron's comp group of biotherapeutic and oncology companies is trading at an average market cap of $1.14 billion and enterprise value of $1.01 billion. (Please see Exhibit 2 below.) We look for data presentations and clinical progress to create value at Acceleron and close this valuation gap.

Exhibit 2

ACCELERON COMPARABLE COMPANY ANALYSIS

Company	Ticker	Price 10/11/13	Market Cap.	Cash	LTD	Ent. Value
Seattle Genetics	**SGEN**	**$39.72**	**$4,840**	**$338**	**$0**	**$4,502**
ImmunoGen	IMGN	$16.45	$1,391	$195	$0	$1,196
Epizyme	EPZM	$35.85	$1,019	$149	$0	$870
Array	**ARRY**	**$5.53**	**$647**	**$108**	**$99**	**$638**
Agios	AGIO	$24.76	$769	$210	$0	$559
NewLink Genetics	NLNK	$19.26	$495	$59	$0	$436
Curis	**CRIS**	**$4.29**	**$350**	**$57**	**$29**	**$322**
Merrimack	MACK	$3.61	$369	$235	$162	$297
OncoMed	**OMED**	**$14.15**	**$394**	**$144**	**$0**	**$250**
Average			**$1,142**	**$166**	**$32**	**$1,008**
TOTAL			**$10,274**	**$1,495**	**$291**	**$9,069**
Acceleron	**XLRN**	**$22.12**	**$620**	**$126**	**$13**	**$507**

Source: Company Reports and Piper Jaffray estimates

Bold = Covered companies

DALANTERCEPT (ACE-041)

While most investors are focused on Acceleron's Celgene deal and anemia drugs, we are more interested in dalantercept. Firstly, the majority of Acceleron's proprietary R&D investment and funds raised in the IPO will go to develop dalantercept. Secondly, as a wholly owned asset, we see more potential value creation coming from clinical validation and progress of dalantercept.

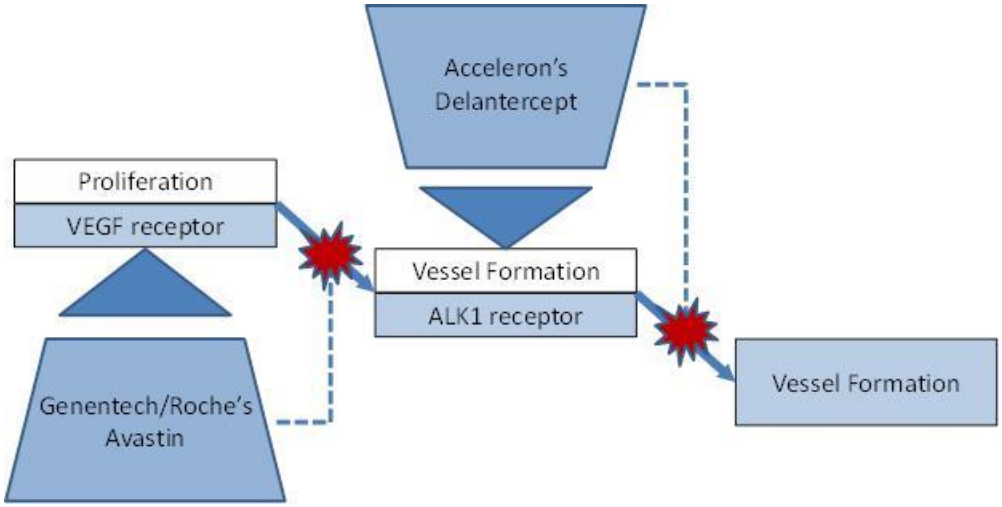
Tumors need significant quantities of oxygen and nutrients to grow. One survival adaption is that cancers high-jack angiogenic pathways to grow new blood vessels in order to feed the tumor. As the father of anti-angiogenesis therapy, Dr. Judah Folkman preached that denying a tumor of its sustenance would "starve" the tumor and inhibit its ability to grow. There are several targets involved in the angiogenesis pathway, most notably vascular endothelial growth factor (VEGF). VEGF is the target of several approved cancer therapies including Genentech's anti-VEGF antibody *Avastin* (bevacizumab), as well as small molecule receptor tyrosine kinase inhibitors (RTKs) including Pfizer's *Inlyta* (axitinib) and *Sutent* (suntinib), Amgen's *Nexavar* (sorafenib), as well as GlaxoSmithKline's *Votrient* (pazopanib) and *Tykerb* (lapatinib). In total, this class achieved >$8 billion in sales last year. Unfortunately, when VEGF is blocked, the tumors mutate and over-express other angiogenic factors resulting in resistance and recurrence. It is for this reason, much like other areas in oncology, that the future of anti-angiogenesis will be combination therapy.

That's where dalantercept comes in. Activin receptor-like kinase (ALK1) is one of the 12 TGF-β superfamily receptors found selectively on the surface of proliferating vascular endothelial cells. ALK1 plays a key role in the formation of blood vessels, in particular in vessel maturation. Dalantercept is fusion protein that combines the ALK1 receptor to the Fc domain of IgG. In so doing, dalantercept is a ligand trap that selectively binds the proteins in circulation that activate ALK1 -- in this case BMP09, BMP10 and TGF-β. (Cunha and Pietras, *Blood*, 2011, Vol. 117, #26). By mopping up the BMP09 and BMP10, there are fewer ligands to signal ALK1 on the surface of endothelial cells and thus less blood vessel formation.

In pre-clinical models, Acceleron has demonstrated that dalantercept inhibits ALK1 signaling and has potent anti-angiogenic, anti-tumor and anti-metastatic activity. Importantly, dalantercept maintained activity in models that were resistant to VEGF inhibitors. This indicates that not only could dalantercept be used in Avastin failure patients who have limited or no alternative therapy, but also in combination with oral anti-VEGF inhibitors to boost efficacy. (Please see Exhibit 3 below.)

Exhibit 3

ACCELERON COMPARABLE COMPANY ANALYSIS



Source: Company Reports and Piper Jaffray estimates

Acceleron conducted a Phase I dose-escalation study of subcutaneous dalantercept administered once every 3 weeks in 37 advanced solid tumor patients. Final results were presented at the American Association of Cancer Research (AACR) meeting in April 2011. Dalantercept was well tolerated with most common AEs of mild or moderate peripheral edema, fatigue, anemia, nausea, dyspnea, anorexia and headache. A single case of Grade 3 congestive heart failure (CHF) was observed, although none of the toxicities common with VEGF inhibition such as hypertension, proteinuria or bleeding was observed. 13 (35%) patients demonstrated >20% reduction in tumor metabolic activity as measured by FDG-PET imaging. One (3%) patient with refractory head & neck cancer had a partial response (PR) and 8 (22%) patients achieved prolonged stable disease of >12 weeks.

Based on the PR seen in the Phase I study, Acceleron initiated an open-label Phase II monotherapy study of dalantercept in recurrent or metastatic squamous cell Head & Neck cancer. Acceleron completed enrollment in July of 46 patients including 2 at 80mg, 13 at 0.6mg/kg and 31 at 1.2mg/kg subcu dalantercept once every 3 weeks. As of August 22nd, 36 patients were evaluable including 1 (3%) PR and 11 (31%) patients with stable disease. These monotherapy results are comparable to what was seen in the Phase I supporting our view that future dalantercept development will be as combination therapy. The study is also evaluating safety and measuring PK, ALK1 expression in tissue and blood, progression free survival (PFS), overall survival, time to tumor progression (TTP), duration of response and disease control rate (DCR) with data likely reported sometime in 1H:14.

The Gynecologic Oncology Group (GOG) with support from the National Cancer Institute (NCI) has initiated 2 monotherapy studies of dalantercept in endometrial and ovarian cancer. The GOG initiated an open-label study in up to 52 women with endometrial cancer in November 2012. In February 2013, the GOG initiated a Phase II study of dalantercept in 43 women with persistent or recurrent ovarian, fallopian tube or primary peritoneal cancer. Both studies are measuring PFS at 6 months and objective tumor response. Since the endometrial study started earlier, we anticipate we could get data as early as by year-end with data from the ovarian cancer study likely in 1H:14. Again since these are monotherapy studies, our expectations are low, however results could provide insight for future dalantercept combination trials in these indications.

In January, Acceleron initiated a Phase I/II combo study of dalantercept with *Inlyta* (axitinib) in renal cell carcinoma (RCC). Inlyta is an oral RTK inhibitor of VEGF receptors 1, 2, 3; platelet derived growth factor receptor (PDGFR) and cKIT that was approved by the FDA in January 2012 for refractory RCC. The initial dose-escalation phase of the study is designed to determine the maximum tolerated dose (MTD) of the combination. The study has already dosed 4 patients each at 0.6mg/kg and 0.9mg/kg subcu dalantercept every 3 weeks + axitinib. Of the 4 patients at 0.6mg/kg dalantercept + axitinib, 1 (25%) patient achieved a PR and 1 (25%) patient stable disease, while 2 (50%) patients progressed. Of the 4 patients at 0.9mg/kg dalantercept + axitinib, 1 (25%) patient achieved a PR and 3 (75%) patient stable disease for a 100% disease control rate! While early data in a small number of patients, these results clearly point to an active combination.

Acceleron is now enrolling the 1.2mg/kg dalantercept cohort and could further escalate to 1.5mg/kg dalantercept before enrolling an additional 20 patients in an expansion cohort at the MTD. We anticipate Acceleron will report preliminary data from the Phase I portion of the study at ASCO in June 2014. Based on the safety and tolerability observed to date, the company is attempting to amend the trial protocol to potentially include other VEGF inhibitors. We expect Acceleron will begin the Phase II portion of the study comparing dalantercept + axitinib vs. axitinib alone in up to 112 RCC patients in early 2014.

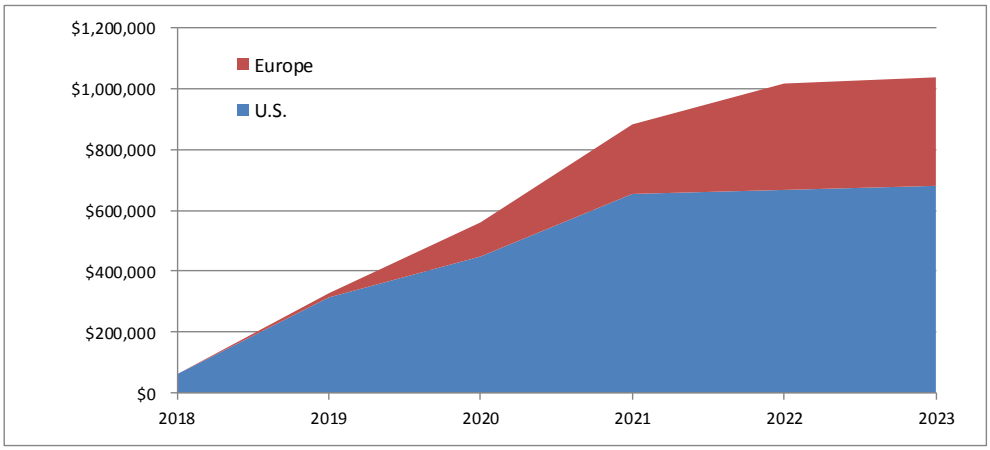
Based on this time frame, we anticipate Acceleron could report Phase II RCC data in 2015 and initiate pivotal combination trials in 2016 with axitinib or other oral VEGF inhibitors. If successful, Acceleron could file a BLA in 2H:17 and gain FDA approval and launch dalantercept in the U.S. in 2018. We assume comparable pricing to Avastin in the U.S. resulting in sales of $62 million in 2018, $314 million in 2019 and $680 million in 2023.

At present, Acceleron retains global rights to dalantercept. We envision the company will either partner dalantercept overseas or potentially market the drug in Europe and partner for Asia and the rest of the world. We presently anticipate EMA approval and European launch in 2019. Priced at a discount to the U.S., especially with the potential for biogeneric bevacizumab, we forecast European sales of $14 million in 2019, $112 million in 2020 and $356 million in 2023. This would equate to global dalantercept RCC sales of $62 million in 2018, $328 million in 2019 and $1.04 billion in 2023. (Please see Exhibit 4 below.)

Acceleron intends to begin a 2nd Phase I/II combo study of dalantercept + a VEGF inhibitor in either liver, lung or colon cancer in 2H:14. Based on the broad range of approvals of Avastin, we are confident that dalantercept combination therapy will also be successful and approved in additional cancers.

Exhibit 4

GLOBAL DALANTERCEPT RENAL CELL CARCINOMA SALES FORECAST



	2018	2019	2020	2021	2022	2023
Newly Diagnosed RCC Patients in U.S.	51,415	51,930	52,449	52,973	53,503	54,038
Clear Cell RCC	35,991	36,351	36,714	37,081	37,452	37,827
% Patients with Clear Cell RCC	*70.0%*	*70.0%*	*70.0%*	*70.0%*	*70.0%*	*70.0%*
Recurrent RCC	11,517	11,632	11,749	11,866	11,985	12,105
% Patients Recurrent RCC	*32.0%*	*32.0%*	*32.0%*	*32.0%*	*32.0%*	*32.0%*
dalantercept Treated Patients	1,152	2,908	4,112	5,933	5,992	6,052
% Treated	***10.0%***	***25.0%***	***35.0%***	***50.0%***	***50.0%***	***50.0%***
Price per 3-week cycle	$54,000	$108,000	$109,080	$110,171	$111,273	$112,385
U.S. dalantercept RCC Sales ($000)	**$62,192**	**$314,070**	**$448,536**	**$653,645**	**$666,783**	**$680,186**
Newly Diagnosed RCC Patients in Europe	67,265	67,937	68,617	69,303	69,996	70,696
Clear Cell RCC	47,085	47,556	48,032	48,512	48,997	49,487
% Patients with Clear Cell RCC	*70.0%*	*70.0%*	*70.0%*	*70.0%*	*70.0%*	*70.0%*
Recurrent RCC	15,067	15,218	15,370	15,524	15,679	15,836
% Patients Recurrent RCC	*32.0%*	*32.0%*	*32.0%*	*32.0%*	*32.0%*	*32.0%*
dalantercept Treated Patients		380	1,537	3,105	4,704	4,704
% Treated		***2.5%***	***10.0%***	***20.0%***	***30.0%***	***29.7%***
Price per 3-week cycle		$36,000	$72,720	$73,447	$74,182	$74,923
EU dalantercept RCC Sales ($000)		**$13,696**	**$111,772**	**$228,036**	**$348,930**	**$355,943**
WW dalantercept RCC Sales ($000)	**$62,192**	**$327,766**	**$560,308**	**$881,681**	**$1,015,713**	**$1,036,129**

Source: American Cancer Society, Company Reports and Piper Jaffray estimates

ANEMIA

Anemia is a term that is broadly defined by a low number of red blood cells (RBCs). As RBCs primarily transport oxygen to the cells throughout the body, too few RBCs can result in fatigue, weakness, as well as long-term tissue necrosis and organ damage. Anemia can be caused for several reasons including end stage renal disease (ESRD), side-effects of chemotherapy or other drugs, or blood disorders such as sickle cell disease (SCD), Beta-thalassemia or myelodysplastic syndromes (MDS).

Erythropoiesis is the process by which hematopoietic precursor cells mature into red blood cells (RBCs) in the bone marrow. Erythropoietin (EPO) is a hormone that initiates erythropoiesis and is effective at stimulating production of early stage RBC precursors. EPO sales including Amgen's *Epogen* and *Aranesp* plus Johnson & Johnson's *Procrit* were almost $4 billion over the last 12 months. Despite steady price increases, EPO sales have been declining due to safety issues and labeling restrictions. Acceleron is partnered with Celgene to develop sotatercept and ACE-536 to broadly treat anemia. We see the opportunity for a safe alternative to EPO products as a potential blockbuster for Celgene.

Beta-thalassemia

Hemoglobin (Hb) is the metalloprotein present in RBCs. Hb is comprised of two alpha and two beta chains, which contain the iron-rich **heme** groups that bind oxygen. Beta-thalassemia, also known as Cooley 's anemia, is a rare blood disorder caused by genetic mutations that result in inappropriate or insufficient synthesis of the Hb beta chains. There are two distinct types of Beta-thalassemia: minor and major. Individuals with Beta-thalassemia minor are heterozygous, meaning they have one good copy of the HB beta gene and do produce some beta chains. These individuals have few symptoms and generally need minimal or no therapy.

Cases of Beta-thalassemia major are more severe. These patients are homozygous meaning they have mutations in both copies of the HB beta gene and thus make no beta chains. Beta-thalassemia becomes evident within months of birth and these children have severe anemia, skeletal abnormalities and exhibit poor growth. Such children need frequent blood transfusions, as well as iron chelation therapy. There are no approved drugs to treat this population, although allogeneic bone marrow transplant is often considered. There are only ~1,000 Beta-thalassemia major patients in the U.S., representing an ultra-orphan indication. Since the disease is most prevalent in Mediterranean populations, there are close to 20,000 patients in Europe and as many as 300,000 patients worldwide.

Myelodysplastic Syndromes (MDS)

Myelodysplastic syndromes (MDS) are a collection of hematologic disorders in which the stem cells in the marrow do not mature properly. This leads to an increase in the number of blasts (immature) and dysblasts (malformed) hematopoietic cells. In turn, this means that the bone marrow will fail to produce enough mature blood cells. As such, many of these patients will eventually have at least one form of cytopenia: whether it be anemia (low RBC count), neutropenia (low white blood cell count) and/or thrombocytopenia (low platelet count). MDS is a debilitating disorder that often progresses into acute myeloid leukemia (AML), a form of blood cancer.

SOTATERCEPT (ACE-011)

In contrast to EPO, certain ligands in the TGF-β superfamily down-regulate erythropoiesis. Sotatercept is soluble receptor consisting of activin receptor type IIA (ActRIIA) combined with the Fc domain of IgG. Sotatercept is a ligand trap that selectively binds the TGF-β proteins that suppress erythropoiesis, and thereby stimulate RBC production. Importantly this represents a unique mechanism of action from EPO.

In February 2008, Acceleron partnered sotatercept with Celgene. Celgene paid $45 million upfront and invested $5 million in Acceleron equity. To date, Acceleron has received $34.2 million in R&D funding and milestones, and is eligible for up to $367 million in additional milestones. Acceleron retains co-promote rights in North America, for which Celgene will pay all costs, and will receive tiered royalties in the low-to-mid 20% range on net sales of sotatercept. As of January 1, 2013, Celgene became responsible for 100% of future clinical, manufacturing and commercial costs associated with sotatercept. Celgene is conducting Phase II trials in Beta-thalassemia, MDS and chronic kidney disease. In addition, investigator sponsored trials are underway in multiple myeloma, Diamond-Blackfan anemia and myelofibrosis.

Celgene will report Phase II dose-escalation data on sotatercept in Beta-thalassemia at the American Society of Hematology (ASH) meeting in December in New Orleans and substantively more data in both Beta-thalassemia and MDS at the European Hematology Association (EHA) meeting next June in Milan. Depending upon the Phase II data, we anticipate Celgene could initiate Phase III trials of sotatercept and/or ACE-536 in MDS and/or Beta-thalassemia in late 2014 and early 2015.

Phase II Beta-thalassemia Trial

Celgene initiated a Phase II dose-escalation trial of sotatercept in Beta-thalassemia in November 2012. The trial is designed to evaluate doses of 0.1mg/kg, 0.3mg/kg and 0.5mg/kg subcu sotatercept every 3 weeks in up to 28 patients in the United Kingdom, Italy and France. The primary endpoint of the trial in transfusion dependent patients is a ≥20% reduction in transfusion burden and in transfusion independent patients is an increase in hemoglobin levels by ≥1g/dL. Celgene has completed the 0.1mg/kg and 0.3mg/kg cohorts and preliminary results have shown dose dependent increases in hemoglobin. Specifically, 1/5 (20%) of the 0.1mg/kg patients and all 5 (100%) of the 0.3mg/kg patients gained at least 1g/dL in hemoglobin. No significant safety issues have been observed in the study to date. Celgene is now enrolling the 0.5mg/kg sotatercept cohort and will report data at the ASH meeting in December down in New Orleans. The company is also attempting to modify the protocol to evaluate higher doses of sotatercept. Once an MTD is selected, Celgene will enroll a 10 patient expansion cohort of 10 patients with additional data likely presented at EHA meeting next June in Milan. We would anticipate final Phase II data on sotatercept in Beta-thalassemia by year-end 2014.

Phase II MDS Trial

In December 2012, Celgene initiated a Phase II study of sotatercept in low or intermediate-1 risk MDS. Similar to the Beta-thalassemia study, this trial is designed to evaluate doses of 0.1mg/kg, 0.3mg/kg and 0.5mg/kg subcu sotatercept every 3 weeks. This study is larger in up to 75 MDS patients. The primary endpoint is erythroid hematologic improvement (HI-E), but the study will also evaluate the drug impact on iron overload and bone metabolism. Celgene is attempting to modify the protocol to evaluate higher doses. We expect Celgene to present Phase II sotatercept dose-escalation data at EHA in June with final Phase II MDS data by year-end 2014.

Phase II CKD Trial

Celgene is conducting a placebo-controlled Phase II study of sotatercept in End-stage Renal Disease (ESRD) patients on hemodialysis. The study may randomize up to 56 patients (9:3) to escalating cohorts of 0.1mg/kg, 0.3mg/kg, 0.5mg/kg and 0.7mg/kg subcu sotatercept every 4 weeks for up to 8 cycles. Celgene has completed enrollment in the 0.1-.05mg/kg cohorts and is now enrolling the 0.7mg/kg cohort. Celgene intends to modify the protocol to evaluate higher doses of sotatercept. The primary endpoints are safety and PK, but the study will also measure effects on hemoglobin and markers of bone metabolism. We expect Celgene to report initial Phase II data on sotatercept in chronic kidney disease in 1H:14.

Phase II Multiple Myeloma Trial

Celgene is supporting an investigator sponsored Phase II study of sotatercept in up to 34 multiple myeloma patients at Massachusetts General Hospital. Multiple myeloma is a cancer of plasma cells, a type of white blood cells. Many multiple myeloma patients suffer from anemia. The study is designed to evaluate the anti-tumor effect of *Revlimid* + dexamethasone + sotatercept, as well as the effect on anemia and bone lesions. Investigators may report Phase II sotatercept data in multiple myeloma sometime next year.

Phase II Diamond-Blackfan Anemia Trial

Diamond-Blackfan is a rare and severe form of anemia. The disease is characterized by low birth weight, delayed growth and sometimes congenital abnormalities such as cardiac defects and cleft palate. The Phase II study will enroll up to 20 transfusion dependent adults with DFA at North Shore Long Island Jewish Health System. The goal of the study is to determine a safe and effective dose of sotatercept that reduces transfusion dependence. We anticipate investigators may report Phase II sotatercept data in DFA in 1H:14.

Phase II Myelofibrosis Trial

Myelofibrosis is a fibrosis of the bone marrow in which patients cannot produce mature blood cells. A common symptom of the disease is anemia due to insufficient RBC count. An investigator sponsored Phase II study of sotatercept is underway in up to 40 patients with myeloproliferative neoplasm-associated Myelofibrosis and Anemia at M.D. Anderson Cancer Center. The primary endpoint is anemia response with duration of response an important secondary measure.

ACE-536

ACE-536 is soluble receptor consisting of activin receptor type IIB (ActRIIB) combined with the Fc domain of IgG. Similar to sotatercept, ACE-536 is a ligand trap that selectively binds the TGF-β proteins that suppress erythropoiesis, and thereby stimulate RBC production. While a subtle difference, ACE-536 is a distinct soluble receptor that has unique characteristics from sotatercept. It is for this reason that the partners are running Phase II studies of both sotatercept and ACE-536 in Beta-thalassemia and MDS, and will decide which drug to advance into Phase II trials in which indication in late 2014/early 2015.

In August 2011, Acceleron and Celgene entered into a second partnership for ACE-536. Celgene paid $25 million upfront and has paid Acceleron $25.2 million in R&D funding and milestones to date. Acceleron is eligible for up to $200 million in additional milestones for ACE-536. Acceleron retains co-promote rights in North America, for which Celgene will pay all costs, and will receive tiered royalties in the low-to-mid 20% range. Similar with sotatercept, Celgene is responsible for 100% of future ACE-536 clinical, manufacturing and commercial costs.

Phase II Beta-thalassemia Trial

In March, Acceleron initiated a Phase II dose-escalation trial of ACE-536 in Beta-thalassemia. The trial is designed to evaluate doses of 0.2mg/kg, 0.4mg/kg, 0.6mg/kg, 0.8mg/kg and 1.0mg/kg subcu ACE-536 every 3 weeks for up to 85 days. The primary endpoint in transfusion dependent patients is a ≥20% reduction in transfusion burden and in transfusion independent patients is an increase in hemoglobin levels by ≥1.5g/dL from baseline for at least 14 days. The study will also evaluate ACE-536's impact on iron overload by measuring serum iron and hemolysis. Acceleron has completed the 0.2mg/kg and 0.4mg/kg cohorts and is now enrolling the 0.6mg/kg cohort. We expect preliminary data to be presented at the EHA meeting next June in Milan with final Phase II data on ACE-536 in Beta-thalassemia by year-end 2014.

Phase II MDS Trial

In January, Acceleron initiated a Phase II study of ACE-536 in up to 60 low or intermediate-1 risk MDS patients in Germany. The MDS trial is designed to evaluate doses of 0.125mg/kg, 0.25mg/kg, 0.5mg/kg, 0.75mg/kg and 1.0mg/kg subcu ACE-536 every 3 weeks for up to 85 days. The primary endpoint in transfusion dependent patients is a ≥50% reduction in transfusion burden within 8 weeks and in transfusion independent patients is an increase in hemoglobin levels by ≥1.5g/dL from baseline for at least 14 days. Acceleron has completed the 0.125mg/kg, 0.25mg/kg and 0.5mg/kg cohorts and is now enrolling the 0.75mg/kg cohort. We expect preliminary data to be presented at the EHA meeting next June in Milan with final Phase II data on ACE-536 in MDS by year-end 2014.

MANAGEMENT TEAM

John Knopf, PhD; Founder, President & CEO

Dr. Knopf co-founded Acceleron in 2003. Today he serves on the company's board of directors and as President and CEO. Prior to founding Acceleron, Dr. Knopf served as Site Head of the Wyeth Research facilities in Cambridge, MA, and Vice President of Metabolic and Respiratory Disease. Dr. Knopf was an early scientist at Genetics Institute from 1982 to 1998. Dr. Knopf received a BS in Biology from SUNY Stonybrook and a Ph.D. in Biology at SUNY Buffalo.

Kevin McLaughlin, CFO

Mr. McLaughlin joined Acceleron in November 2010 as SVP, Chief Financial Officer and Treasurer. He most recently served as SVP and CFO of Qteros, a cellulosic biofuels company. He was a co-founder of Aptius Education and the Chief Operating Officer and a Director from 2007 through 2009. From 1996 through 2007, Mr. McLaughlin held several executive positions with PRAECIS Pharmaceuticals including CFO, COO, and then President and CEO, and member of the board. In this capacity, he was responsible for negotiating the sale of PRAECIS to GlaxoSmithKline. He began his career in senior financial roles at Prime Computer and Computervision Corporation. Mr. McLaughlin received a BS in Business from Northeastern University and an MBA from Babson College.

Matthew L. Sherman, M.D., CMO

Dr. Sherman joined Acceleron in May 2006 as SVP and Chief Medical Officer. Previously, he served as SVP and CMO at Synta Pharmaceuticals. Prior to that, Dr. Sherman worked at the Genetics Institute and Wyeth Pharmaceuticals in various capacities including Therapeutic Area Director for Oncology. Dr. Sherman is board certified in Medical Oncology and Internal Medicine and held various clinical positions at Harvard Medical School with corresponding hospital appointments at the Dana-Farber Cancer Institute and Brigham and Women's Hospital. Dr. Sherman received a BS in Chemistry from the Massachusetts Institute of Technology and an MD from Dartmouth Medical School.

Steven Ertel, CBO

Mr. Ertel joined Acceleron in January 2006 as SVP and Chief Business Officer. Mr. Ertel has over 20 years of experience in the biotechnology industry at Vivus, Genentech, Biogen Idec and Synta. Mr. Ertel began his career in the venture capital industry at Oxford Bioscience Partners. Mr. Ertel received a BSE in Biomedical Engineering from Duke University and an MBA from the Wharton School at the University of Pennsylvania.

FINANCIALS

Revenues

Acceleron primarily receives revenues from its partners. Total revenues were $15.3 million in 2012.Total revenues increased to $26.4 million in 2Q:13 due to accelerated recognition of $20.5 million of the amortized Shire upfront due to termination of the partnership. We forecast revenues of $4.1 million in 3Q:13 and $11.1 million in 4Q:13, due to a $7 million sotatercept CKD milestone, totaling $56.7 million for the full year 2013.

Operating Expenses

R&D investment was $35.3 million in 2012. Celgene is now responsible for 100% of sotatercept and ACE-536 development expenses, although we anticipate Acceleron's proprietary R&D investment will grow on dalantercept. R&D investment was $8.9 million in 2Q:13 and we forecast $9.5 million in 3Q:13, totaling $37.2 million for the full year 2013.

G&A expense was $8.8 million in 2012 and $3.4 million in 2Q:13. We budget G&A expense of $3.5 million in 3Q:13 totaling $14 million for the full year 2013.

Net Loss

Acceleron lost $30.3 million or ($1.44) per pro forma share in 2012. Due to the recognition of $20.5 million of the amortized Shire upfront, Acceleron reported net income $13.5 million or $0.64 per pro forma share in 2Q:13. We forecast net loss of $12 million or ($0.54) per share in 3Q:13, totaling $1.6 million or ($0.07) per primary share for the full year 2013.

Balance Sheet

Acceleron ended 2Q:13 with cash of $28.6 million. The company completed a successful IPO on September 18th issuing 6.5 million primary XLRN shares at $15.00 per share. In addition, Celgene invested $10 million at the IPO price in a concurrent private placement. We estimate Acceleron now holds pro forma cash of ~$126 million. Acceleron currently owes long-term notes payable to Silicon Valley Bank of $12.9 million.

INVESTMENT RISKS

Risks associated with Acceleron are common to other drug discovery and development companies including clinical, regulatory and commercial. Sotatercept, ACE-536 and/or dalantercept may fail in the clinical or to gain regulatory approval. If approved, these drugs will face competition, may not achieve premium pricing, and therefore not meet our sales forecast. Acceleron's partnership with Celgene may falter, thereby affecting funding requirements. Acceleron may be unable to sign new partnerships or file an IND for ACE-083. Acceleron may need to raise additional capital or could face future unforeseen litigation.

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Piper Jaffray Co.

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

10/14/2013

	2012A	1QA	2QA	3QE	4QE	2013E	1QE	2QE	3QE	4QE	2014E
Collaboration Revenue:											
License and milestone	$9,696	$12,515	$22,891	$625	$7,625	$43,656	$625	$625	$100	$10,100	$11,450
Cost-sharing, net	5,558	2,497	3,537	3,500	3,500	13,034	3,000	3,000	3,000	3,000	12,000
Total Revenues	**$15,254**	**$15,012**	**$26,428**	**$4,125**	**$11,125**	**$56,690**	**$3,625**	**$3,625**	**$3,100**	**$13,100**	**$23,450**
Operating Expenses:											
Research and Development	35,319	8,780	8,911	9,500	10,000	37,191	11,000	12,000	13,000	14,000	50,000
General and Administrative	8,824	3,096	3,365	3,500	4,000	13,961	4,000	4,500	4,500	5,000	18,000
Total Operating Expenses	**$44,143**	**$11,876**	**$12,276**	**$13,000**	**$14,000**	**$51,152**	**$15,000**	**$16,500**	**$17,500**	**$19,000**	**$68,000**
Operating Income/(Loss)	**($28,889)**	**$3,136**	**$14,152**	**($8,875)**	**($2,875)**	**$5,538**	**($11,375)**	**($12,875)**	**($14,400)**	**($5,900)**	**($44,550)**
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Total Other Income/(Expense)[1]	(1,435)	(422)	(641)	(3,075)	(2,950)	(7,088)	(3,000)	(3,050)	(3,100)	(3,150)	(12,300)
Pretax Income/(Loss)	**($30,324)**	**$2,714**	**$13,511**	**($11,950)**	**($5,825)**	**($1,550)**	**($14,375)**	**($15,925)**	**($17,500)**	**($9,050)**	**($56,850)**
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)	**($30,324)**	**$2,714**	**$13,511**	**($11,950)**	**($5,825)**	**($1,550)**	**($14,375)**	**($15,925)**	**($17,500)**	**($9,050)**	**($56,850)**
Net Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Net Income/(Loss) per Share	**($1.44)**	**$0.13**	**$0.64**	**($0.54)**	**($0.21)**	**($0.07)**	**($0.50)**	**($0.56)**	**($0.61)**	**($0.31)**	**($1.98)**
Basic Shares Outstanding	21,062	20,954	20,954	22,250	28,250	23,102	28,500	28,600	28,800	29,000	28,725

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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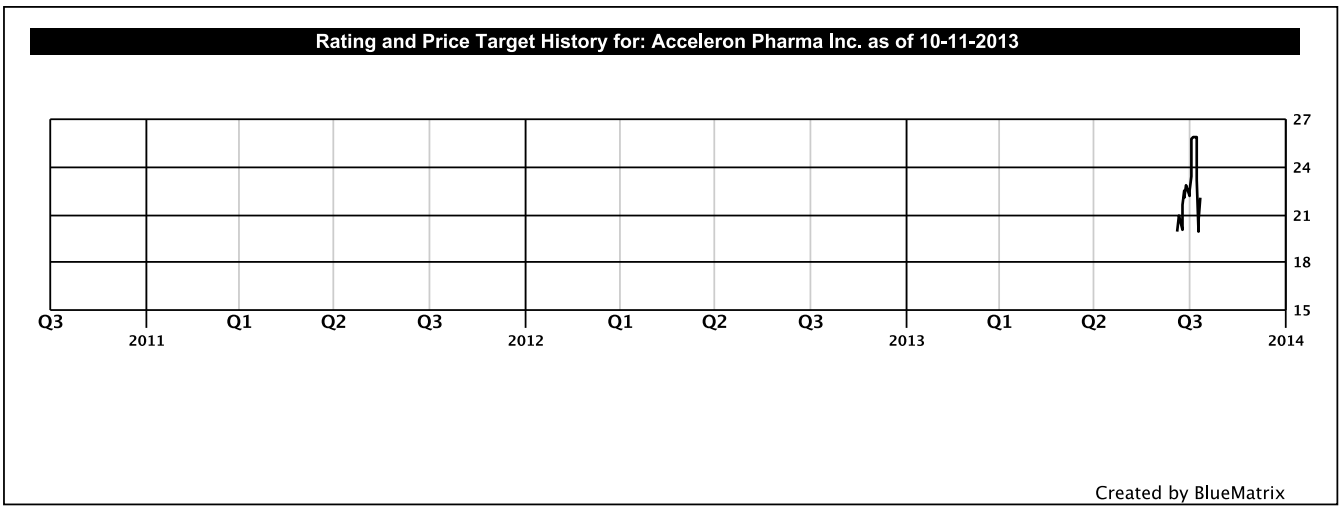

Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written 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]	334	56.80	67	20.06
HOLD [N]	228	38.78	13	5.70
SELL [UW]	26	4.42	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.

Analyst Certification — Edward A. Tenthoff, Sr Research Analyst

The views expressed in this report accurately reflect my personal views about the subject company and the subject security. In addition, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendations or views contained in this report.

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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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