Acceleron Pharma Inc. (XLRN) — Overweight

Strengthens Balance Sheet Ahead of Clinical Readouts; Increasing Target to $65

CONCLUSION

Acceleron recently raised net proceeds of $129 million by issuing 2.76 million shares at $50.00 bring pro forma cash to $242 million. With Celgene covering all development expenses for sotatercept and ACE-536, Acceleron can focus these funds to advance wholly owned dalantercept. We anticipate a busy June with Phase I/II dalantercept renal cell carcinoma (RCC) data at ASCO and Phase II data on both sotatercept and ACE-536 at EHA. We reiterate our Overweight rating and are increasing our price target to $65 from $35 ahead of multiple clinical data read-outs.

- **Strengthened Balance Sheet.** Acceleron recently raised net proceeds of $129 million by issuing 2.76 million shares at $50.00 bring pro forma cash to $242 million, which we estimate will last into 2H:17. With Celgene covering all expenses for sotatercept and ACE-536, Acceleron can focus these funds to advance wholly owned dalantercept.

- **Dalantercept the Next Avastin?** Acceleron is conducting a Phase I/II trial of dalantercept in combination with axitinib in renal cell carcinoma (RCC) with preliminary data likely at ASCO in June. Acceleron intends to begin a Phase I/II trial of dalantercept in combination with Nexavar in liver cancer ahead of schedule in 1H:14. We anticipate dalantercept clinical progress will drive value with blockbuster potential in multiple cancers.

- **Potential Blockbuster Anemia Drugs.** Sotatercept is currently in Phase II trials for Beta-thalassemia and myelodysplastic syndromes (MDS). At ASH in December, partner Celgene updated Phase II sotatercept data in Beta-thalassemia showing the drug remained well tolerated with only 3/25 (12%) patients with Grade 2 or greater tox. The majority (84% each) of non-transfussion dependent (NTD) patients in the 0.3mg/kg and 0.5mg/kg cohorts achieved at least a 1g/dL increase in Hemoglobin (Hb) levels with 16% on 0.3mg/kg and 33% on 0.5mg/kg achieving at least a 2g/dL increases in Hb. These are impressive results with escalation continuing to 7.5mg/kg. We anticipate additional Phase II Beta-thalessemia and MDS data on both sotatercept and ACE-536 at the European Hematology Association (EHA) meeting in 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 will also report preliminary Phase II data on sotatercept in chronic kidney disease (CKD) in April. 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.

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$47.39
TARGET: US$65.00
Proj. EV of $1.9B + YE:14E net cash

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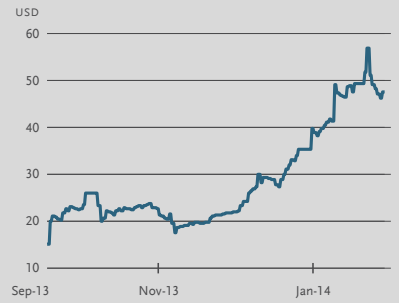
Changes	Previous	Current
Rating	—	Overweight
Price Tgt	US$35.00	US$65.00
FY13E Rev (mil)	56.8	57.0
FY14E Rev (mil)	—	23.5
FY13E EPS	(0.54)	(0.53)
FY14E EPS	(1.71)	(1.58)

52-Week High / Low	US$57.89 / US$15.00
Shares Out (mil)	31.1
Incl. impact of recent 2.76M share offering	
Market Cap. (mil)	US$1,473.8
Avg Daily Vol (000)	176
Book Value/Share	US$5.40
Net Cash Per Share	US$7.43
Proforma cash incl. recent offering less notes payable	
Debt to Total Capital	6%
$11M in notes payable less current portion	
Div (ann)	NA
Fiscal Year End	Dec

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	96.3x	—	—	—	—	(1.44)	NM
2013E	15.0A	26.4A	4.3A	11.3	57.0	25.9x	0.13A	0.64A	(1.11)A	(0.13)	(0.53)	NM
2014E	3.6	3.6	3.1	13.1	23.5	62.7x	(0.41)	(0.45)	(0.50)	(0.23)	(1.58)	NM

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UPCOMING CLINICAL DATA TO CONTINUE TO DRIVE VALUE AT ACCELERON

Acceleron is a biotechnology company that is 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**. In addition, Acceleron hopes to advance **ACE-083** into the clinic this 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 ~$137 million including $7 million last month for the initiation of a Phase II trial in end-stage renal disease (ESRD), and is eligible for up to $560 million in additional milestones. Importantly, Celgene is responsible for all development, manufacturing and commercialization expenses going forward. The partners will co-promote sotatercept and 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). Partner Celgene reported positive Phase II dose-escalation data on sotatercept in Beta-thalassemia at the American Society of Hematology (ASH) meeting in December. Sotatercept remains well tolerated with only 3/25 (12%) patients showing Grade 2 or higher toxicity including 1 bone pain, 1 superficial thromophlebitis and 1 ventricular extrasystoles. Importantly, the majority (84% each) of non-transfusion dependent (NTD) patients in the 0.3mg/kg and 0.5mg/kg doses achieved at least a 1g/dL increase in Hemoglobin (Hb) levels with 16% on the 0.3mg/kg and 33% on the 0.5mg/kg doses achieved at least a 2g/dL increase in Hb. These are impressive results and escalation continuing to 7.5mg/kg cohort. We anticipate Phase II Beta-thalassemia at additional dose levels, as well as MDS dose escalating data at the European Hematology Association (EHA) meeting in June. Complete Phase II Beta-thalassemia and MDS data is expected at ASH 2014 this coming December. We anticipate Celgene will begin Phase III trials of sotatercept and/or ACE-536 in either indication in late 2014 or early 2015.

We envision Celgene will develop sotatercept and/or ACE-536 broadly to treat anemia as evidenced by the Phase II trial in chronic kidney disease (CKD) with preliminary data at the National Kidney Foundation Spring Clinical Meeting in April. 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* are around $4 billion annually, 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 and highly lucrative royalty stream to Acceleron.

Acceleron is also developing wholly-owned dalantercept, 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 to treat cancer. Anti-angiogenic therapy has become a primary weapon in the armamentarium of the oncologist. Roche/Genentech's anti-VEGF antibody *Avastin* achieved global sales of $6.2 billion in 2012. 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 *INLYTA* (axitinib) in renal cell carcinoma is underway with preliminary data at the American Society of Clinical Oncology (ASCO) meeting in June. Acceleron intends to initiate a 2nd Phase I/II combo study of dalantercept with *Nexavar* (sorafenib) in the liver cancer in 1H:14. Acceleron is also conducting a Phase II monotherapy study of dalantercept in head & neck cancer and an investigator sponsored study in ovarian cancer, but the path forward is clearly in combination.

Upcoming Events and Expected Timing

- Celgene to report initial Phase II data on sotatercept in chronic kidney disease at the National Kidney Foundation Spring Clinical Meeting in April 2014
- Initiate Phase II dalantercept + Nexavar trial in hepatocellular carcinoma in 1H:14
- Acceleron to report full Phase II dalantercept data in Head & Neck cancer 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
- Investigators to report Phase II sotatercept data in Diamond-Blackfan anemia in 1H:14
- Celgene to report additional Phase II data on both sotatercept and ACE-536 in Beta-thalassemia and MDS at EHA in June
- Celgene to initiate Phase III trials of sotatercept and/or ACE-536 in either Beta-thalassemia and/or MDS in late 2014/early 2015
- Investigators to report Phase II sotatercept data in multiple myeloma in 2014
- Acceleron to file an IND for ACE-083 for muscle wasting disorders in 2014

INVESTMENT RECOMMENDATION

We reiterate our Overweight rating on Acceleron and are increasing our price target to $65 from $35. Our new $65 target is based on a projected enterprise value of $1.9 billion, up from $939 million. We now value dalantercept at $1.2 billion. Specifically we apply our industry standard 5x multiple to 2021 WW sales in RCC of $939 million, 2020 WW sales in HCC of $509 million and 2021 WW sales in a 3rd cancer indication of $806 million; all discounted back at 45% through YE:14. Prior we valued dalantercept at $588 million by applying a 5x multiple to 2021 WW sales in RCC of $882 million plus $705 million in sales in a 2nd indication; both discounted back at 45% through YE:14.

We now value Acceleron's portion of sotatercept/ACE-536 revenues at $701 million, up from $287 million, by applying a 10x multiple to 2020 Beta-thalassemia royalties of $221 million discounted back at 35% and 2019 MDS anemia royalties of $215 million discounted back at 45% through YE:14. We believe this multiple is appropriate for high margin royalty revenues and these discount rates are appropriate for Phase II assets following the positive Beta-Thalassemia data at ASH. Positive clinical data could result in a lower discount rate and an increase in our price target.

Acceleron completed a successful follow-on offering on January 22nd of 2.76 million shares including the over-allotment at $50.00 bringing in net proceeds of $129 million. Acceleron ended 2013 with cash of ~$113 million, bringing pro forma cash to $242 million, which should fund the company into 2H:17. Thus we add YE:14E cash of $200 million to arrive at a projected market capitalization of $2.06 billion, which we divide by an increased 32 million YE:14E shares outstanding to arrive at our $65 price target.

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 both the IPO and the recent follow-on will go to develop dalantercept. Secondly, as a wholly owned asset, we see more potential value creation coming from clinical validation and progress of this therapy.

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.

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 were observed. Thirteen (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.

In January 2013, the Gynecologic Oncology Group (GOG) with support from the National Cancer Institute (NCI) initiated a Phase II monotherapy study of dalantercept in 43 women with persistent or recurrent ovarian, fallopian tube or primary peritoneal cancer. The study measures PFS at 6 months and objective tumor response. Since this is a monotherapy trial, our expectations are low, however results could provide insight for future dalantercept combination trials in these indications.

In February 2013, 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%) patients achieved stable disease for a 100% disease control rate. While early data and a small number of patients, these results clearly point to an active combination. Acceleron has also now dosed 5 patients in the 1.2mg/kg dalantercept cohort and selected this dose for the expansion cohort, which is currently enrolling an additional 20 patients. We expect Acceleron to 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. Part 2 of the trial will be a randomized comparison of axitinib +/- dalantercept with PFS as the primary endpoint in up to 112 RCC patients.

Based on this time frame, we anticipate Acceleron could report Phase II RCC data 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 RCC in 2019. Priced at a discount to the U.S., especially with the potential for biogeneric bevacizumab, we forecast European sales of $17 million in 2019, $139 million in 2020 and $445 million in 2023. This would equate to global dalantercept RCC sales of $62 million in 2018, $331 million in 2019 and $1.13 billion in 2023.

Acceleron intends to begin a 2nd Phase I/II combo study of dalantercept + Nexavar (sorafenib) in hepatocellular carcinoma in 1H:14. The primary endpoint is the determination of safety & tolerability of the combination at a multiple dose levels: 0.9mg/kg dalantercept + 400mg QD sorafenib, 1.2mg/kg dalantercept + 400mg QD sorafenib and 1.2mg/kg dalantercept + 400mg BID sorafenib. The secondary endpoints include time-to-progression (TTP), PFS, disease control rate and overall survival (OS). Each cohort will enroll up to 6 patients with an expansion phase with an additional 20 patients. Based on our current expectations, we believe Acceleron could initiate pivotal liver cancer studies in 2017 and reach the U.S. market in 2019.

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 Acceleron and 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 $41.5 million in R&D funding and milestones, and is eligible for up to $360 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 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 additional Phase II dose-escalation data on sotatercept in both Beta-thalassemia and MDS at the European Hematology Association (EHA) meeting in June in Milan and full data at ASH 2014 this coming December. 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 (Hb) levels by ≥1g/dL. Celgene has completed the 0.1mg/kg, 0.3mg/kg and 0.5mg/kg cohorts. Preliminary results showed 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 Hb.

At ASH in December, Celgene reported positive Phase II sotatercept dose-escalation data on sotatercept in Beta-thalassemia. Sotatercept remains well tolerated with only 3/25 (12%) patients showing Grade 2 or higher toxicity including 1 bone pain, 1 superficial thromophlebitis and 1 ventricular extrasystoles. Importantly, the majority (84% each) of non-transfusion dependent (NTD) patients in the 0.3mg/kg and 0.5mg/kg doses achieved at least a 1g/dL increase in Hb levels while 16% on the 0.3mg/kg and 33% on the 0.5mg/kg doses achieved at least a 2g/dL increase in Hb. These are impressive results with escalation continuing to 7.5mg/kg cohort. Once an MTD is selected, Celgene will enroll a 10 patient expansion cohort. We anticipate additional Beta-thalassemia data to be presented at EHA in June in Milan with final Phase II data likely at ASH in December.

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, 1.0mg/kg and 2.0mg/kg subcu sotatercept every 3 weeks. Each cohort could contain up to 20 patients, with up to 115 patients overall. 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 likely presented at the ASH meeting in December of this year.

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 will randomize up to 56 patients (9:3) in 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 0.1-0.5mg/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 CKD in at the National Kidney Foundation Spring Clinical Meeting in April 2014.

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 this 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 DFA data 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 $28.3 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 this June in Milan with final Phase II data on ACE-536 in Beta-thalassemia by the ASH meeting in December 2014.

Phase II MDS Trial

In January, 2013, 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 the ASH meeting December 2014.

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

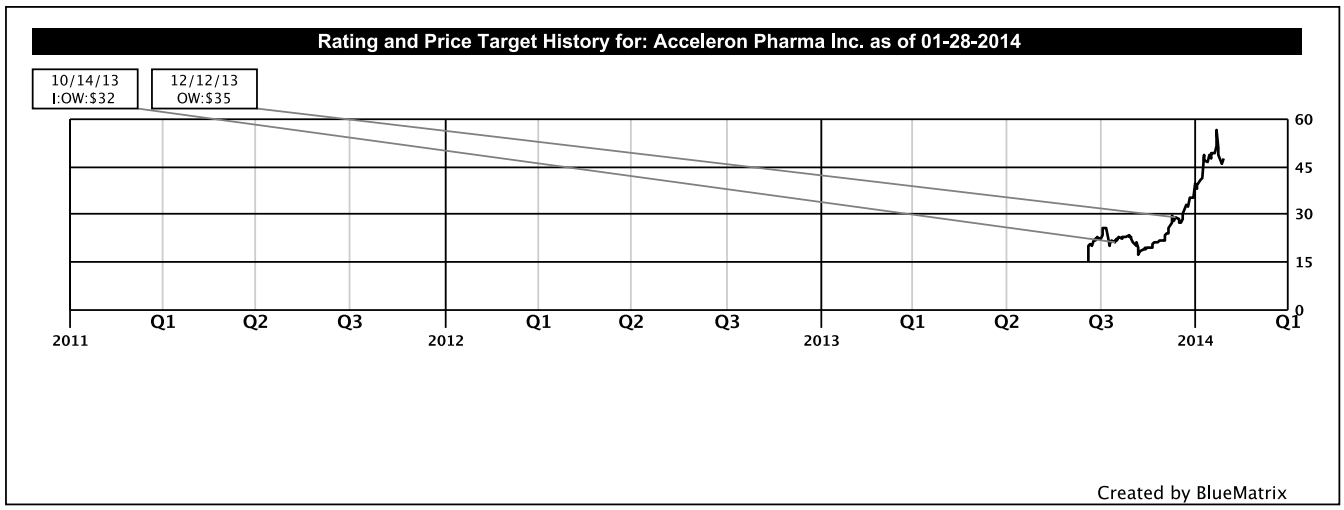
1/29/2014

	2012A	1QA	2QA	3QA	4QE	2013E	1QE	2QE	3QE	4QE	2014E
Collaboration Revenue:											
License and milestone	$9,696	$12,515	$22,891	$638	$7,638	$43,682	$638	$638	$100	$10,100	$11,476
Cost-sharing, net	5,558	2,497	3,537	3,632	3,632	13,298	3,000	3,000	3,000	3,000	12,000
Total Revenues	**$15,254**	**$15,012**	**$26,428**	**$4,270**	**$11,270**	**$56,980**	**$3,638**	**$3,638**	**$3,100**	**$13,100**	**$23,476**
Operating Expenses:											
Research and Development	35,319	8,780	8,911	8,143	10,000	35,834	11,000	12,000	13,000	14,000	50,000
General and Administrative	8,824	3,096	3,365	3,011	4,000	13,472	4,000	4,500	4,500	5,000	18,000
Total Operating Expenses	**$44,143**	**$11,876**	**$12,276**	**$11,154**	**$14,000**	**$49,306**	**$15,000**	**$16,500**	**$17,500**	**$19,000**	**$68,000**
Operating Income/(Loss)	**($28,889)**	**$3,136**	**$14,152**	**($6,884)**	**($2,730)**	**$7,674**	**($11,362)**	**($12,862)**	**($14,400)**	**($5,900)**	**($44,524)**
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Total Other Income/(Expense)	(3,693)	(1,489)	(1,074)	(11,629)	(1,000)	(15,192)	(1,050)	(1,100)	(1,150)	(1,200)	(4,500)
Pretax Income/(Loss)	**($32,582)**	**$1,647**	**$13,078**	**($18,513)**	**($3,730)**	**($7,518)**	**($12,412)**	**($13,962)**	**($15,550)**	**($7,100)**	**($49,024)**
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)	**($32,582)**	**$1,647**	**$13,078**	**($18,513)**	**($3,730)**	**($7,518)**	**($12,412)**	**($13,962)**	**($15,550)**	**($7,100)**	**($49,024)**
Net Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM
Other Items	2,258	1,067	433	(6,272)	0	(4,772)	0	0	0	0	0
Net income applicable to common shareholders	**($30,324)**	**$2,714**	**$13,511**	**($24,785)**	**($3,730)**	**($12,290)**	**($12,412)**	**($13,962)**	**($15,550)**	**($7,100)**	**($49,024)**
Net Income/(Loss) per Share	**($1.44)**	**$0.13**	**$0.64**	**($1.11)**	**($0.13)**	**($0.53)**	**($0.41)**	**($0.45)**	**($0.50)**	**($0.23)**	**($1.58)**
Basic Shares Outstanding[1]	**21,062**	20,954	20,954	22,250	28,250	**23,102**	30,100	31,100	31,250	31,500	**30,988**

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. Shares Outstanding for 2012A, 1Q:13A, 2Q:13A, and 3Q:13A are Piper Jaffray estimates.

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

IMPORTANT RESEARCH DISCLOSURES



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]	349	58.46	75	21.49
HOLD [N]	227	38.02	23	10.13
SELL [UW]	21	3.52	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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- **Neutral (N):** Anticipated to perform in line relative to the median of the group of stocks covered by the analyst.
- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

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