Acceleron Pharma Inc. (XLRN)

Initiating Coverage at Market Outperform; Superstar of the TGF-Beta Superfamily

MARKET OUTPERFORM | Price: $22.12 | Target Price: $32.00

MARKET DATA

Price	$22.12
52-Week Range:	$18.50 - $26.54
Shares Out. (M):	26.5
Market Cap ($M):	$586.2
Average Daily Vol. (000):	271.0
Cash (M):	$95
Cash/Share:	$3.38
Enterprise Value (M):	$852
Float (M):	24.3
LT Debt (M):	$14

Source: Thomson Reuters and JMP Securities LLC

FY DEC		2012A	2013E	2014E
Revenue ($M)	1Q	--	$15.0	$4.9
	2Q	--	$26.4	$12.2
	3Q	--	$4.3	$5.4
	4Q	--	$4.7	$20.9
	FY	**$15.3**	**$50.5**	**$43.4**
EPS	1Q	--	$0.12	($0.33)
	2Q	--	$0.44	($0.07)
	3Q	--	($0.36)	($0.34)
	4Q	--	($0.32)	$0.14
	FY	**($1.43)**	**($0.17)**	**($0.53)**
	P/E	NM	NM	NM

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE

INVESTMENT HIGHLIGHTS

Initiating coverage of Acceleron Pharma with a Market Outperform rating and year-end 2014 price target of $32 based on a synthesis of DCF, standardized CAGR, and comparable company valuation methodologies. In our view, Acceleron has all the ingredients for a hugely successful investment opportunity in the biotech space: a powerful technology platform capable of generating unique fusion protein-based biologic drug candidates (ligand traps) with substantive intellectual property protection; molecules that are targeted at uncommon hematologic and cancerous conditions; a lucrative partnership with Celgene (CELG, MO, $160 PT); an experienced and credible management team with deep scientific expertise; and positive near-term pipeline value drivers.

Acceler-ating its way to greatness. After completing a successful IPO in September, we believe Acceleron has the ingredients to become a "big biology" company, much in the same way as the Regeneron (REGN, MO, $290 PT, Newman) of today or Medarex or Abgenix of the past. The complex biology of the TGFβ superfamily of targets provides Acceleron with a significant competitive advantage, as it is both difficult to target and to understand the precise role of the target (ligand or receptor) in the context of both the disease and the tissue. However, the TGFβ superfamily provides rewarding targets to those that can exploit its biology, and Acceleron has done this admirably in the creation of its pipeline of product candidates.

First foray into anemia, with Celgene as a partner by its side. Sotatercept (ACE-011) and ACE-536 represent Acceleron's lead clinical programs, each of which is in Phase II clinical development for various anemias (including the orphan indication, beta-thalessemia), in conjunction with Celgene. The economics of the agreement with Celgene are some of the most attractive of any partnership in the industry, essentially providing Acceleron with profit split-like returns and a near-zero investment outlay of its own. In addition to the $70MM already received, Acceleron is eligible for more than $567MM in development and commercial milestones. Celgene has also acquired a further $15MM of equity in the IPO. We believe that one or both of these candidates should enter Phase III trials by late 2014 or early 2015. Updated data on the sotatercept program will be featured at ASH 2013, providing a meaningful near-term value driver for the shares, in our view.

Dalantercept-targeting a different kind of ALK. While most investors are aware of the ALK (anaplastic lymphoma kinase) translocation prevalent in approximately 5% of patients with non-small cell lung cancer (NSCLC), the ALK1 ligand trap fashioned by XLRN targets the activin receptor-like kinase found on blood vessels, which plays a key role in the maturation stage of angiogenesis (in some ways similar to platelet-derived growth factor, PDGF).

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Dalantercept is currently in clinical trials in tumors known to be sensitive to treatment with angiogenesis inhibitors such as Avastin as a “partner of choice” to improve efficacy with little in the way of incremental toxicity.

Moving forward at warp speed, Acceleron lays claim to future greatness. It bears repeating that opportunities of the sort represented by Acceleron only come along in rare instances. Those that recognized the aforementioned companies early in their development stage were well-rewarded for the risks they took, despite the seemingly high-risk ventures they appeared to be at the time. We see many parallels between these once and future greats, and recommend that investors build significant positions in Acceleron as a core holding in the smid-cap biotech space.

INVESTMENT THESIS

We are initiating coverage of Acceleron with a Market Outperform rating and $32 price target. We view Acceleron as a well-differentiated, biology platform play, addressing meaningful and underserved market opportunities in both hematologic disorders and oncology. Acceleron has relative free rein from competition in the TGFβ therapeutics space, while the target indications for its leading candidates ACE-536 and Sotatercept offer attractive risk-adjusted, commercial opportunities in well-defined patient populations.

While certainly not the only route to outsized gains in the biotechnology sector, one of the most "tried and true" ways has been to invest in those companies that are deep in biologic expertise and whose drugs are biologic products, such as proteins and antibodies. Examples of this principle can be found in every era of the biotechnology industry, from the first leaders of the biotechnology sector to come of age in the 1980s, such as Amgen (AMGN, NC), Biogen (BIIB, MO, $270 PT, Newman), Centocor, Chiron, Genentech, and Genetics Institute, to the 1990s with Alexion, Medimmune, and Regeneron, to the oughts, with Human Genome Sciences and Seattle Genetics (SGEN, NC), to cite a few examples.

In our opinion, Acceleron represents a similar opportunity for investors to earn a return on investment over the course of the next several years on the basis of the company's focus on the area of TGF-beta biology, an area it clearly dominates and for which it has few competitors. Management's pedigree is mainly from Genetics Institute (now part of Pfizer (PFE, NC) through its acquisition of Wyeth), one of the legendary companies of the biotechnology industry's first wave of companies, and brings with it a unique appreciation of TGF-beta biology that has begotten, and we believe will further beget, differentiated therapeutic properties. In our opinion, the company's first wave of product candidates, validated by Celgene's significant investment, is but the tip of the iceberg that, over time, should create significant value for shareholders.

FIGURE 1. Upcoming Milestones

Timing	Drug	Milestones
4Q13	Sotatercept	Update from Sotatercept Phase II trial in β-thalassemia at ASH (Dec 7-10)
1Q14	Sotatercept & ACE-536	Initiation of RP2D expansion cohorts in ongoing β-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 β-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 β-thalassemia and MDS
4Q14	Sotatercept & ACE-536	Initiation of Phase III trial in β-thalassemia and/or MDS
4Q14	ACE-083	Initiation of Phase I trial in muscular dystrophy

Source: Acceleron presentations and JMP Securities LLC

VALUATION

We arrive at our year-end 2014 price target of $32 based on the synthesis of a discounted cash flow (DCF) analysis, our standardized CAGR methodology, and a relative valuation against a set of comparable stage biotechnology platform companies (Figure 2).

FIGURE 2. Price Target Synthesis

Synthesis of Price Target	
Approach	Valuation
DCF Analysis	$29.85
CAGR	41.92
Comparables	39.41
Price Target	**$32.00**

Source: JMP Securities LLC and Company Reports

Our DCF valuation projects milestones and royalty revenue from Celgene related to the development and worldwide commercialization of Sotatercept and ACE-536, plus dalantercept-related sales and royalty income, while subtracting cost of goods sold, projected operating expenses, and tax. Net cash flows to the company are discounted back to present values by 32.9%, representing a blended, risk-adjusted, discount rate that takes into account the stage of development of each drug candidate, its likelihood of success, and its relative contribution to peak revenue estimates. A terminal value for the company, calculated by applying a 5% long-term growth rate, was similarly discounted to present day. Present value of free cash flows, together with the terminal value, were added to arrive at a residual value for the company, to which estimated cash and long-term debt were added and subtracted, respectively. Thereby, we arrived at an equity valuation of $794MM. When we divide this amount by our estimated 2014 year-end outstanding share count, we derive a per share valuation of $29.85. Our DCF assumptions are detailed further in Figure 3.

FIGURE 3. Discounted Cash Flow Valuation

Discounted Cash Flow Model	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Product Revenues													
Sotatercept / ACE-536 Royalty Revenue	-	-	-	3.3	34.7	123.9	252.5	429.8	583.0	743.4	856.9	953.2	1,010.7
Dalantercept	-	-	-	-	-	41.9	90.1	213.8	424.3	688.9	924.9	1,147.1	1,351.1
Collaboration Revenue	50.5	43.4	42.9	88.0	80.8	133.8	87.2	40.9	135.0	49.5	94.5	59.9	65.9
Total Revenues	**50.5**	**43.4**	**42.9**	**91.2**	**115.4**	**299.6**	**429.8**	**684.5**	**1,142.3**	**1,481.8**	**1,876.3**	**2,160.1**	**2,427.7**
Cost of product sales				0.0	0.0	4.2	8.1	19.2	37.4	59.7	78.0	94.2	107.3
COGS as a % of Dalantercept Revenue						*10%*	*9%*	*9%*	*9%*	*9%*	*8%*	*8%*	*8%*
Gross Profit	50.5	43.4	42.9	91.2	115.4	295.4	421.7	665.2	1,104.9	1,422.1	1,798.3	2,065.9	2,320.5
R&D expense	36.1	39.7	57.5	69.0	79.3	87.3	95.1	103.7	113.0	123.2	131.8	138.4	145.3
R&D as a % of revenue	*72%*	*91%*	*134%*	*76%*	*69%*	*29%*	*22%*	*15%*	*10%*	*8%*	*7%*	*6%*	*6%*
SG&A expense	14.2	17.9	19.7	21.7	30.4	34.9	38.8	41.9	44.8	47.7	50.4	51.9	53.4
SG&A as a % of revenue	*28%*	*41%*	*46%*	*24%*	*26%*	*12%*	*9%*	*6%*	*4%*	*3%*	*3%*	*2%*	*2%*
Total operating expenses	50.3	57.6	77.2	90.7	109.7	122.2	133.9	145.6	157.9	170.9	182.2	190.3	198.8
% Margin	*100%*	*133%*	*180%*	*99%*	*95%*	*41%*	*31%*	*21%*	*14%*	*12%*	*10%*	*9%*	*8%*
Operating income (EBIT)	**0.2**	**(14.2)**	**(34.3)**	**0.5**	**5.7**	**173.2**	**287.7**	**519.6**	**947.0**	**1,251.2**	**1,616.1**	**1,875.6**	**2,121.7**
Taxes	0.0	(2.1)	(8.6)	0.2	2.0	60.6	100.7	181.9	331.5	437.9	565.6	656.5	742.6
Tax rate	*15%*	*15%*	*25%*	*30%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
After tax operating income	0.2	(12.0)	(25.7)	0.4	3.7	112.6	187.0	337.8	615.6	813.3	1,050.5	1,219.1	1,379.1
Discount year		0.25	1.25	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25
Discount factor	1.0	1.1	1.4	1.9	2.5	3.3	4.4	5.9	7.8	10.4	13.8	18.4	24.4
PV	0.2	(11.2)	(18.0)	0.2	1.5	33.6	42.1	57.2	78.5	78.0	75.9	66.3	56.4

Residual value of cash flow	$ 707.31
+ Cash and Cash equivalents	86.35
Company value	$ 793.65
- Long-term debt on 12/31/14	-
Value of equity	$ 793.65
Value per share	**$ 29.85**
Fully diluted shares outstanding on 12/31/14	26.58
Blended discount rate	32.9%
Longer-term growth rate	10%

Terminal Value	246.8

Source: JMP Securities LLC, Company filings

We also arrived at a valuation based on our standardized CAGR methodology. We began by calculating the profitable biotech PEG ratio (1.01), based on the mean 2014 P/E (25.6) and a mean forward CAGR of 25.4%. Based on projected EPS in the year 2020 and a discount rate of 32.9%, we arrived at a valuation of $41.92 per share. Our assumptions, together with a sensitivity analysis, are detailed in Figure 4.

FIGURE 4. CAGR Valuation Model and Sensitivity Analysis

CAGR Valuation		
Comparables		
Biotech Group P/E (2013)		25.6
Biotech Group Forward CAGR ('14- '15)		25.4%
Valued Company		
Year used for discounting		2020
Price Target Year		2014
5-year EPS CAGR		27.8%
EPS in the discounting year	$	8.22
Discount Rate		32.9%
# Years for Discounting		6
Target Price		**$41.92**

Sensitivity Analysis					
	Discount Rate				
CAGR	29.9%	31.4%	32.9%	34.4%	35.9%
12.8%	$22.10	$20.63	$19.27	$18.02	$16.86
17.8%	$30.76	$28.71	$26.82	$25.08	$23.46
22.8%	$39.42	$36.80	$34.37	$32.13	$30.06
27.8%	$48.08	$44.88	$41.92	$39.19	$36.67
32.8%	$56.74	$52.96	$49.48	$46.25	$43.27
37.8%	$65.40	$61.05	$57.03	$53.31	$49.88
42.8%	$74.06	$69.13	$64.58	$60.37	$56.48

Source: Thomson One and JMP Securities LLC

Finally, by taking the mean market cap valuation from a peer group of platform and oncology/hematology-focused biotechnology companies, we derive a comparable valuation for XLRN of $39.41 (Figure 5).

FIGURE 5. Comparable Company Valuation

Valuation of Comparable Companeis						
Comparable	Ticker	Price	Market Cap	Cash	Debt	EV
Agios Pharmaceuticals Inc	AGIO	$33.18	$1,031	$91	$0	$940
Alnylam Pharmaceuticals Inc	ALNY	$64.63	$4,069	$51	$0	$4,018
Array Biopharma Inc	ARRY	$6.49	$760	$61	$99	$799
Astex Pharmaceuticals Inc	ASTX	$8.55	$812	$15	$0	$796
Cell Therapeutics Inc	CTIC	$1.59	$182	$50	$5	$136
Celldex Therapeutics Inc	CLDX	$32.55	$2,636	$25	$6	$2,617
Clovis Oncology Inc	CLVS	$56.99	$1,719	$144	$0	$1,575
Curis Incorporated	CRIS	$4.32	$353	$13	$30	$370
Endocyte Inc	ECYT	$14.37	$518	$34	$0	$484
Epizyme Inc	EPZM	$39.28	$1,116	$98	$0	$1,018
Exelixis	EXEL	$5.77	$1,062	$170	$323	$1,215
Immunomedics Incorporated	IMMU	$6.75	$560	$41	$0	$518
Infinity Pharmaceuticals Inc	INFI	$16.78	$805	$176	$0	$629
Isis Pharmaceuticals Inc	ISIS	$36.07	$4,159	$124	$222	$4,257
Merrimack Pharmaceuticals Inc	MACK	$3.97	$406	$38	$37	$406
Oncogenex Pharmaceutical Inc	OGXI	$8.95	$131	$16	$4	$120
Regulus Therapeutics Inc	RGLS	$9.51	$393	$41	$10	$363
Sunesis Pharmaceuticals Inc	SNSS	$5.08	$263	$15	$18	$265
Synta Pharmaceuticals Corp	SNTA	$6.49	$448	$82	$4	$371
Threshold Pharmaceuticals Inc	THLD	$5.00	$290	$11	$0	$279
Intrexon Corp	XON	$23.59	$2,246	$10	$0	$2,236
Ziopharm Oncology Inc	ZIOP	$4.68	$391	$73	$0	$318
Average			$1,107			$1,079
Acceleron Pharma Inc	XLRN	$25.89	$727	$95	$0	$632

Comparable Valuation	$39.41

Source: Thompson One and JMP Securities LLC

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 to the company's share price.

Financial. Following its IPO, we estimate that Acceleron will end 4Q13 with approximately $87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron 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.

COMPANY OVERVIEW

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 the TGFβ signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to β-thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGFβ.

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 ≥20% royalty on worldwide sales, by our estimates. Sotatercept and ACE-536 are currently in Phase II trials for the treatment of β-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.

SOTATERCEPT AND ACE-536 – LOCKING DOWN TGFB IN ANEMIA

As highlighted above, Acceleron's biologic development platform leverages its pioneering work characterizing the role and regulation of the TGFβ signaling pathway in human disease along with its proprietary technology to engineer chimeric ligand traps consisting of the extracellular domain of specific TGFβ receptors and the Fc region of the human IgG1 antibody. The underlying technology behind the TGFβ trap platform was in-licensed from the laboratory of Dr. Tom Maniatis, then at Harvard University.

Sotatercept and ACE-536 - The Biotechnology Equivalent of the Barber Twins

With both sotatercept and ACE-536 (fusion traps of Activin receptor Type II A and B, respectively), Acceleron has set its sights first on improving the lives of children and young adults suffering from complications related to hereditary β-thalassemia and, secondly, on patients suffering from anemia-related, low-risk myelodysplasia. In both settings, there is an insufficient production of red blood cells (erythropoiesis) and patients become increasingly unresponsive to standard interventions including blood transfusions and erythropoiesis stimulation agents (EPO or ESAs). As we elaborate further below, the biology of TGFβ signaling in hematopoiesis suggests that ACE-536 and sotatercept are able to stimulate mature red blood cell production by removing the "log jam" that arises in the red blood cell pathway, as the originator (blast) cell makes its way to a fully mature red blood cell (RBC), whereas other cytokine therapy, principally with EPO, "turns up the volume" on blast production. However, because the kink in the hose is not alleviated, the resulting output of RBCs is inherently compromised.

With Celgene's backing, Acceleron has been able to advance both sotatercept and ACE-536 into β-thalessemia and MDS. While the end result of the 'pick-a-winner' competition between the two drugs is unclear at this time, it is possible that one or both of the molecules will be successfully developed. As we explain below, both have shown an impressive ability to raise hemoglobin, while ACE-536 has a differentiated clinical feature in its ability to also improve bone health. This latter property may make ACE-536 a superior candidate in the hematologic malignancy space, as well as in beta thalessemia, while sotatercept may be directed to more benign forms of anemia. At the end of the day, this is all speculation; however, we expect the clinical data from the respective molecules to help inform the respective clinical pathways. The good news for Acceleron and its shareholders is, because economics are identical on both, the ultimate outcome makes no difference.

Briefly, we value the combined anemia market opportunity for sotatercept and ACE-536 at over ~$1.7 billion in revenue by 2020 and up to $4 billion in 2025.

Overview of β-Thalassemia

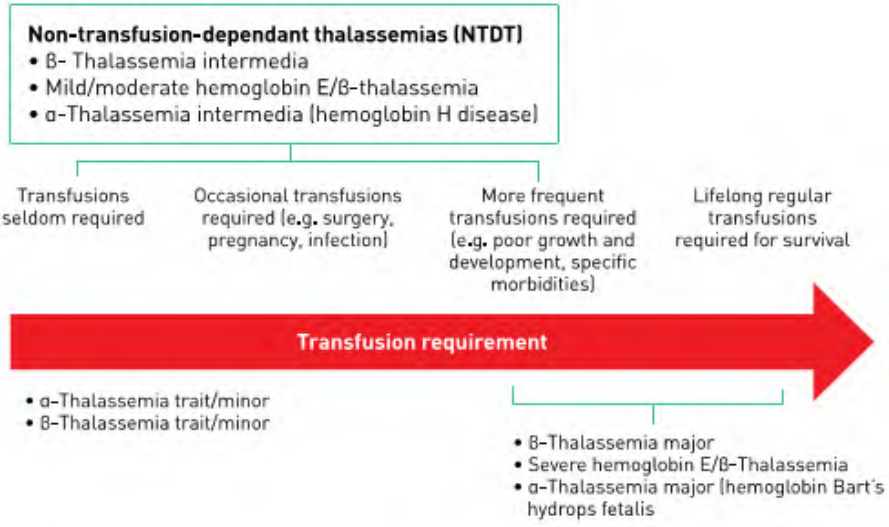
Thalassemia describes a group of inherited hematologic disorders resulting from defects in the production of hemoglobin. In broad terms, these genetic defects can result in an underproduction of alpha-like globin chain (α-globin), leading to α-thalassemia, or an underproduction of beta-like globin chains (β-globin), resulting in β- thalassemia. Gene exons encoding β-like globin chains are clustered on chromosome 11 and are susceptible to nearly 200 different disease-related point mutations resulting in the reduction or blockage of β-globin chain synthesis.

By and large, only one functional β-globin allele is required for sufficient β-globin production and thalassemia trait individuals (carriers of a single-mutated copy of β-globin) tend to experience mild or no anemic symptoms. Patients with two mutant β-globin alleles, one of which being mild in nature, generally experience β-thalassemia intermedia, characterized by mild-to-moderate anemia, requiring infrequent blood transfusions, prominent splenomegaly (enlargement of the spleen) and bone deformities. Depending on the severity of the anemia and the frequency of blood transfusions, iron overload is also common among β-thalassemia intermedia patients. If not properly managed, iron overload can lead to organ damage and ultimately, organ failure. While β-thalassemia intermedia comprises ~40-60% of β-thalassemia overall, marked variability in symptoms is observed within the class for reasons that are not fully understood.

For the remaining proportion of β-thalassemia patients, both β-globin alleles harbor several mutations (effective gene deletions from nonsense or frame shift mutations), resulting in β-thalassemia major, characterized by chronic severe anemia, a life-long dependence on blood transfusion from the time of infancy, splenomegaly, bone disease, and severe iron overload (Figure 6).

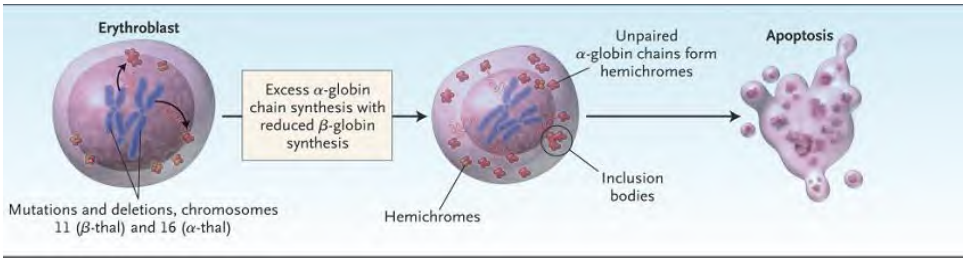
Regardless of the severity of disease, the primary culprit in the pathogenesis of β-thalassemia is a relative excess of free α-globin. In the absence of partnering β-globin, unpaired α-globin chains in the maturing erythroid precursor cell form insoluble hemichromes (denatured globin protein due to iron oxidation), which induce cell death (apoptosis) through a destabilization of heme and a spike in the level of reactive oxygen species (Figure 7). The resulting decline in erythropoiesis causes an erythroid marrow expansion of up to five or six times that of healthy controls and contributes to characteristic bone deformities of the skull and face, osteopenia (lower than normal bone mineral density), defects in both mineralization, as well as progressive splenomegaly.

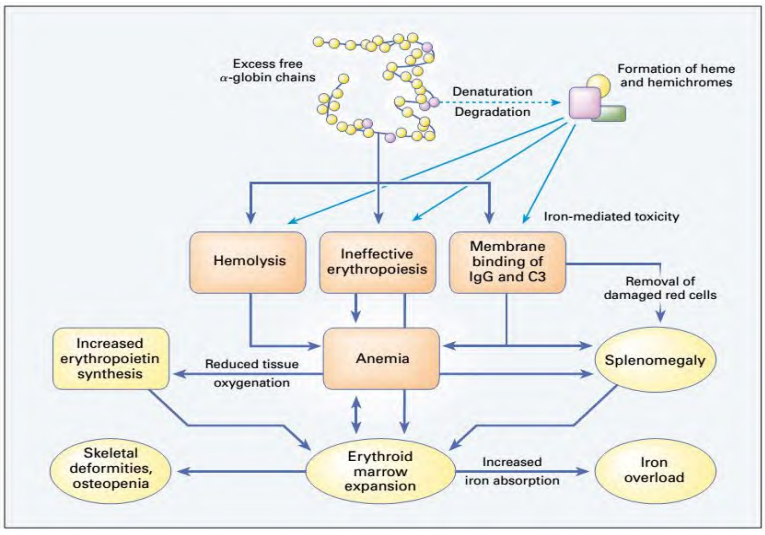
FIGURE 6. Transfusion Requirements in Sub-types of Thalassemia



Source: Guidelines for the Treatment of Non-Transfusion Dependent Thalassemia, TIF, 2013

FIGURE 7. Effects of Excess Production of Free α-Globin Chain

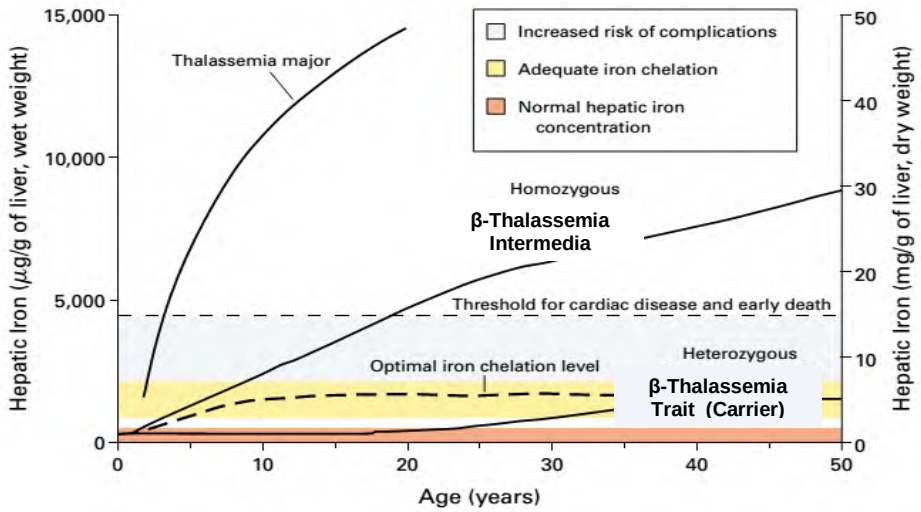




Source: Rund and Rachemilewitz, NEJM, 2005; Olivieri NF, NEJM, 1999.

The most significant contributor to β-thalassemia-related co-morbidity is organ iron overload, resulting from a combination of chronic hemolysis (heme degradation during erythropoiesis leading to higher concentrations of free iron) and repeated blood transfusions. The accumulation of iron deposits to the visceral organs – mainly the heart, liver, and endocrine glands – causes tissue damage, dysfunction, and ultimately organ failure. Cardiac dysfunction due to iron overload remains the primary cause of death among β-thalassemia patients. Patients with non-transfusion dependent β-thalassemia will generally experience an increase in iron burden in the range of 2g to 5g per year, while the rate of iron accumulation can be nearly doubled for transfusion-dependent patients. Iron overload for many patients can be moderated and well-managed with iron-chelating therapy, the most common of which is subcutaneous deferoxamine (Desferal, Novartis; NVS, NC). Notably, early intervention with deferoxamine can prevent the development of maturation defects in thalassemia children.

FIGURE 8. Liver Iron Burden and Health Consequence Among β-Thalassemia Patients



Source: Olivieri NF, NEJM, 1999.

Epidemiology of β-Thalassemia

Global epidemiology data for thalassemia disorders are limited. Extrapolating from World Health Organization estimates published in 2008, ~46,600 children are born with some form of β-thalassemia, 63% of whom are transfusion-dependent and the remainder are non-transfusion dependent. The vast majority of β-thalassemia births originate in regions with tropical climates such as South and South-east Asia, Eastern Mediterranean countries, regions along the Western Pacific, and sub-Saharan Africa (Figure 9). Global prevalence of β-thalassemia is estimated to be ~112,500 patients, again with the majority of that population residing in Eastern-Mediterranean and Southeast Asian countries. β-thalassemia prevalence in Europe is estimated to be ~18,000-19,500 patients, and much lower in the U.S. at ~1,250–1,900 patients (40-60% of a prevalence population of 3,150 for North, South, and Central Americas combined). Importantly, patients with transfusion-dependent disease are grossly underserved, with only 11.7% of β-thalassemia children overall receiving access to transfusion therapy and only 40% of the prevalence population receiving adequate iron chelation therapy.

FIGURE 9. Global WHO Estimates of β-Thalassemia Incidence and Prevalence

WHO region	Estimated annual births β thalassaemias		Transfusion			No. of known patients	Adequate iron chelation		Inadequate or no iron chelation	
	Total	Transfusion-dependent	Annual no. starting transfusion	% of transfusion-dependent patients transfused	Annual deaths because not transfused		% with chelation	No. with chelation	No. of patients	Annual deaths due to iron overload
African	1 386	1 278	35	2.7	1 243	–	–	–	–	–
American	341	255	134	52.4	121	2 750	58	1 604	1 146	57
Eastern Mediter-ranean	9 914	9 053	1 610	17.8	7 443	39 700	27	10 818	28 882	1 444
European	1 019	920	140	15.5	780	16 230	91	14 754	1 476	74
South-east Asian	20 420	9 983	962	9.6	9 021	35 500	19	6 621	28 879	1 444
Western Pacific	7 538	4 022	108	2.7	3 914	3 450	44	1 504	1 946	97
World	**40 618**	**25 511**	**2 989**	**11.7**	**22 522**	**97 630**	**39**	**37 866**	**59 764**	**2 988**

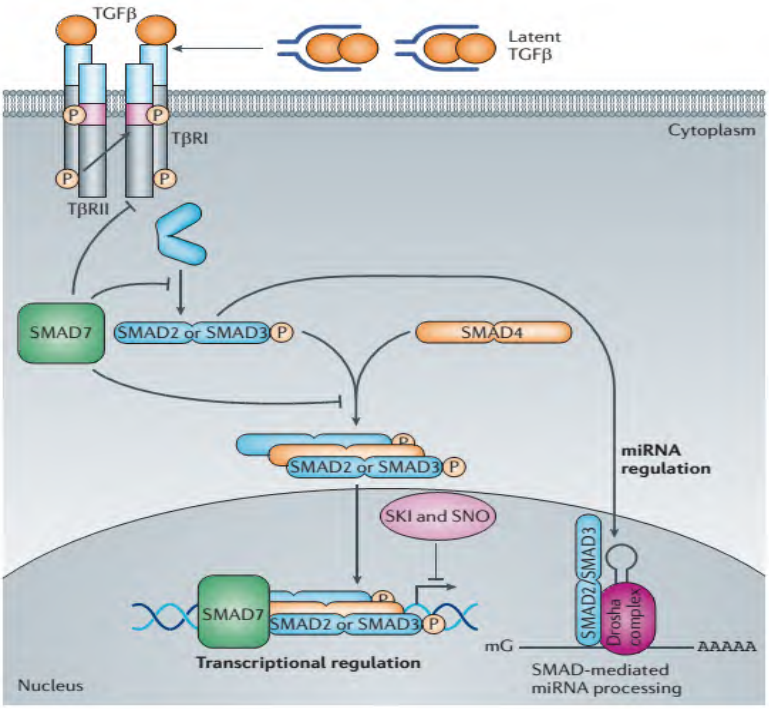
Source: Modell and Darlison, Bull World Health Organ. 2008 Jun;86(6):480-7

Anemia and the TGFβ Signaling Pathway

Erythropoiesis has been shown to be regulated, in part, by components of the transforming growth factor-beta (TGFβ) superfamily. The TGFβ pathway is a highly conserved, signaling network regulating a diversity of cellular functions during development and in the adult organism. Briefly, canonical pathway signaling begins with binding of a TGFβ superfamily cytokine to a Type II receptor, which subsequently recruits and phosphorylates a Type I receptor within a heterodimeric complex. An activated Type I receptor sequentially activates, via phosphorylation, members of the SMAD (an acronym combining the nomenclature for drosophila gene MAD (mother against decapentaplegic) and C. elegans gene SMA (for small body size) family of co-transcription factors, which translocate and accumulate in the nucleus to activate, or in some cases suppress, gene expression (Figure 10).

Several members of the TGFβ superfamily, including TGFβ, bone morphogenic proteins (BMPs) and activins, have been implicated in the determination of hematopoietic stem cell (HSC) and hematopoietic progenitor cell (HSC) fate. Pre-clinical studies have shown both TGFβ and BMP proteins to be negative regulators of HSC differentiation through the activation of SMAD1 and SMAD5 transcription factors (Bhatia et al., Jour of Exp Med, 1999, 189:1139-1148). Similarly, SMAD5 inhibition by RNAi has been shown to partially rescue HPC proliferation suppressed by an exposure to TGFβ (Bruno et al., Blood, 1998, 91: 1917-1923).

FIGURE 10. Overview of the Canonical TGF-B Signaling Pathway



Source: Akhurst and Hata, Nature Review Drug Discover, 2012.

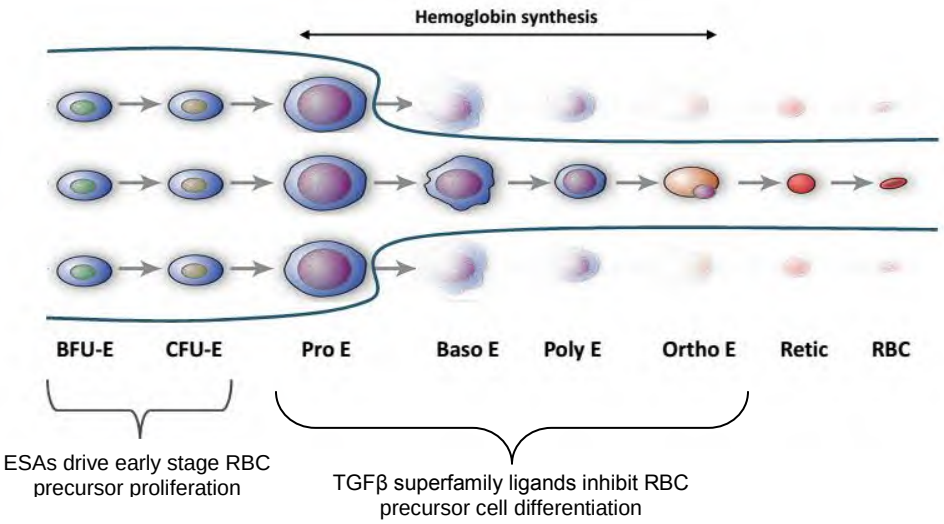
Sotatercept and ACE-536 Pre-clinical Data in β-Thalassemia

Based on these data, other work performed in myelodysplastic disorders (described below), and internal research at Acceleron, it has been recognized that hyper-activated TGFβ signaling prevents the maturation of precursor red blood cells downstream of erythropoiesis growth factors such as granulocyte-colony stimulating factor (G-CSF) and erythropoietin (EPO) (Figure 11). By extension, it is thought that by inhibiting TGFβ signaling, erythropoiesis can be restored, thereby ameliorating the anemia and related comorbidities resulting from β-thalassemia.

To that end, Acceleron has developed two distinct ligand trap therapeutic candidates directed at the TGFβ pathway. Sotatercept (ACE-011) is a soluble receptor fusion protein consisting of the extracellular domain Activin Type IIA receptor (ActRIIA) and the Fc domain of human IgG1. By mimicking ActRIIA, sotatercept binds and sequesters the TGFβ superfamily ligands that might otherwise stimulate pathway signaling. Similarly, Acceleron's second candidate molecule, ACE-536, is a fusion of a modified form of the extracellular domain of Activin Type IIB (ActRIIB) linked to human Fc IgG1.

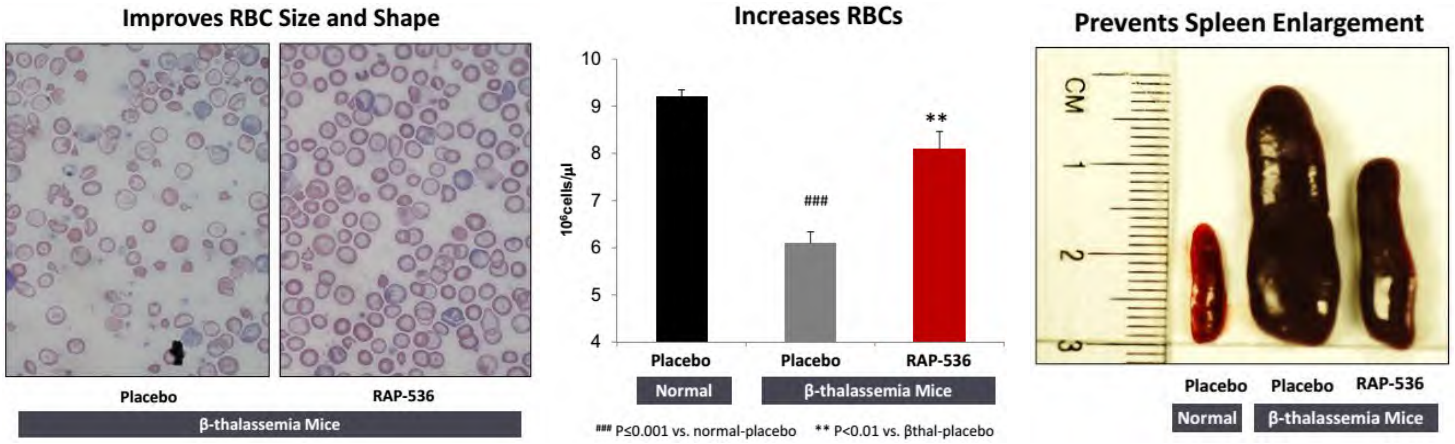
Pre-clinical data generated using mouse models of β-thalassemia have shown that sotatercept and ACE-536 can prevent or reverse several hallmarks of the disease. Mice in these experiments harbor similar deletion mutations to the genes coding for β-globin as those observed in humans, resulting in β-globin protein deficiency, anemia, and several of the related complications that characterize human β-thalassemia major. As shown in Figure 12, treating these mice with the mouse equivalent of ACE-536 (RAP-536) restores the number and health of mature red blood cells, while delaying the onset of related comorbidities such as spleen enlargement, iron deposition in liver and kidneys (not shown), and bone loss (also not shown).

FIGURE 11. Role of TGFβ Signaling During Hematopoiesis



Source: Acceleron company presentation

FIGURE 12. ACE-536 Reverses Several Hallmarks of β-Thalassemia in Mice



RAP-536: the mouse equivalent of ACE-536

Source: Acceleron company presentation

Pathogenesis and Epidemiology of Myelodysplastic Syndrome-Related Anemia

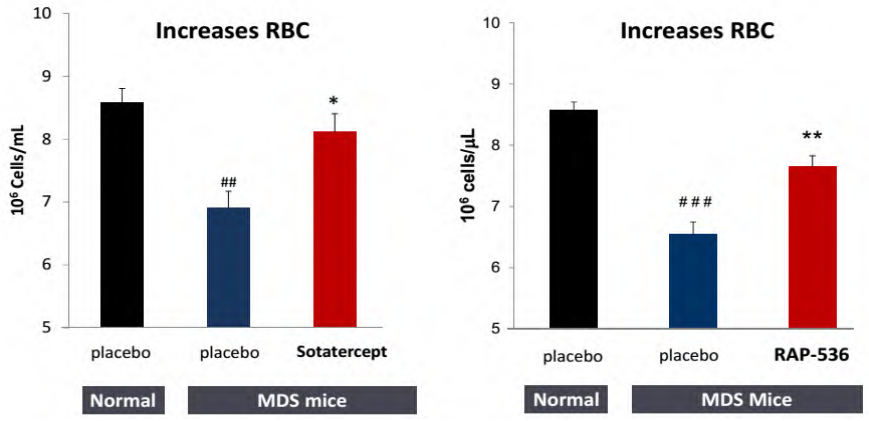
Myelodysplastic Syndrome (MDS) describes a heterogeneous group of blood disorders characterized by an ineffective production of normal blood cells due to a failure of precursor blood cells, or blasts, to differentiate into mature cell types. For a minority of MDS sufferers, blast counts progressively escalate and transform the disease into acute leukemia. Co-morbidity for the majority of MDS patients with low-grade disease, however, is characterized by chronically low peripheral blood counts, generally developing as a consequence of the apoptosis of progenitor stem cells in the bone marrow.

Current treatment guidelines for patients with low-risk/intermediate-1 disease call for the use of cytokine therapy such as G-CSF and ESAs in order to stimulate the proliferation and early differentiation of myeloid and erythroid progenitor cells. However, these agents often prove ineffective with prolonged exposure. As described above, the suppressive role played by TGFβ signaling in progenitor erythroid differentiation is well documented and recent work has shown the TGFβ pathway to be hyperactivated in MDS bone marrow precursors. Specifically, elevated levels of surface bound TGFβ and constitutively activated SMAD2 co-transcription factor have been detected in hematopoietic progenitor cells from MDS bone marrow. In addition, SMAD7 expression, a negative regulator of TGFβ activation, has been shown to be substantially decreased in MDS bone marrow. The suppressive effect of SMAD7 loss on progenitor cell proliferation has been shown to be reversible upon small molecule inhibition of Type I receptor kinase activity. As such, inhibitors of TGFβ signaling represent rational therapy for low grade MDS, either alone, or in combination with current supportive care.

Sotatercept and ACE-536 Pre-clinical Data Support Development in MDS-related Anemia

Pre-clinical studies using non-disease C57BL/6 mice have shown that subcutaneous delivery of RAP-536 can significantly increase hematocrit, hemoglobin, and total red blood cell count compared to mice treated with vehicle alone. Going a step further, subsequent analysis using a mouse model carrying a common translocation found in MDS patients (the NUP98-NOXD13 mouse model) shows that treatment with RAP-536 can slow the progression of anemia (Figure 13), preserving hematocrit (-8% versus -22% with vehicle alone) and RBC count (-13% versus -30%).

FIGURE 13. Sotatercept and ACE-536 Ameliorate Anemia in Pre-clinical Mouse Models



*p≤0.05; **p≤0.01 vs. MDS-placebo, ### P<0.001, ## P<0.01 vs. Normal-placebo, N=6/group

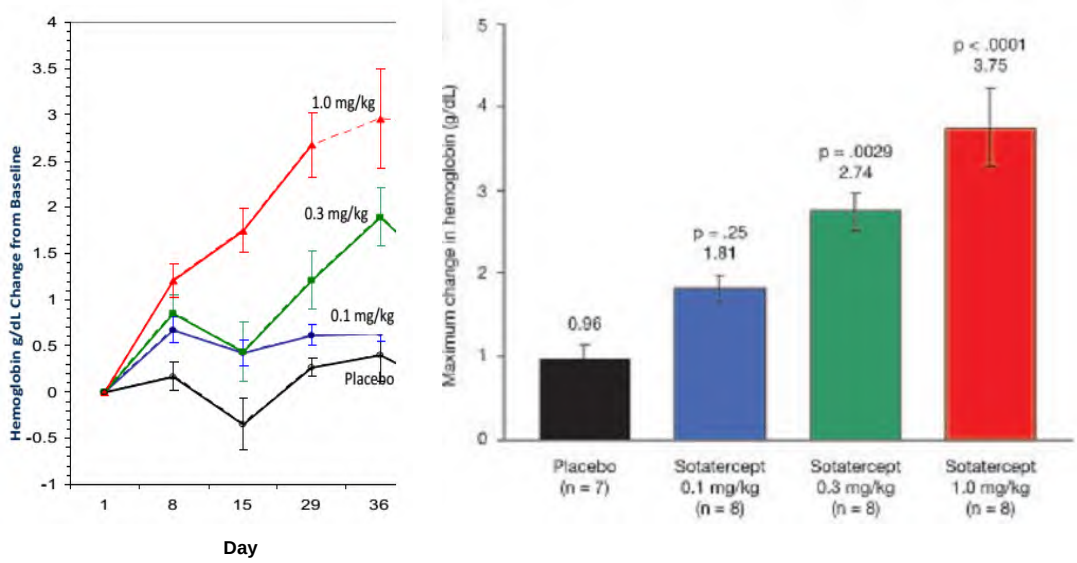
Source: Acceleron Company presentation

Sotatercept and ACE-536 Safety and POC Phase I Data in Healthy Subjects

Safety and initial proof-of-concept (POC) data with sotatercept was established in a multiple-ascending dose Phase I study in healthy post-menopausal women, reflecting an initial intent to develop sotatercept as an osteoporosis drug to restore bone mineral density. Study results, published in the Journal of Clinical Pharmacology in August 2013, show sotatercept to be generally safe, well-tolerated, and capable of eliciting dose-dependent increases in hemoglobin and hematocrit. Specifically, the study evaluated sotatercept at doses of 0.1, 0.3 and 0.5mg/kg administered subcutaneously on a Q4W (once every four weeks) schedule. Across all dosing cohorts, sotatercept treatment increased hemoglobin levels ≥1.5mg/dL from baseline in 22 of 24 (92%) of evaluable patients (Figure 14).

With respect to safety, frequently observed adverse events included headache, along with an increase in hemoglobin and hematocrit, which, in some cases, was associated with increased blood pressure. The majority of cases of treatment-related AEs were mild to moderate and did not require medical intervention; however, one severe case of persistent and progressive hypertension was reported at 1mg/kg, and was subsequently established as the dose-limiting toxicity.

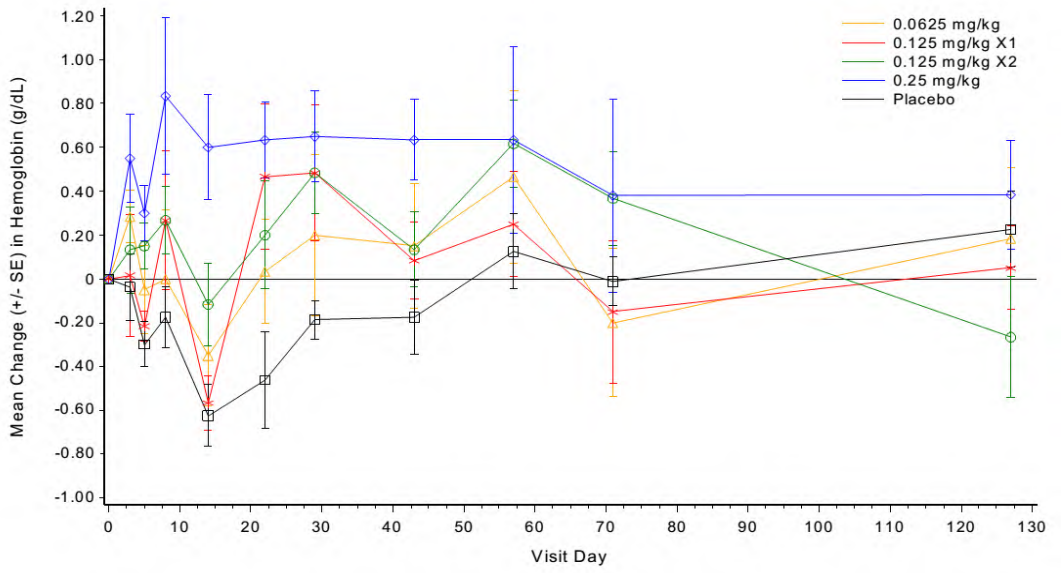
FIGURE 14. Mean Change in Hemoglobin from Baseline in Sotatercept Phase I Trial



Source: Adapted from Sherman et al, Journal of Clinical Pharmacology, 2013.

Initial clinical evaluation of ACE-536 was performed in a similar Phase I trial and patient population, the results of which were first presented at ASH 2012. In a slight variation from the sotatercept Phase I study, the ACE-536 trial tested ascending doses of drug (0.0625mg/kg, 0.125, and 0.25mg/kg) administered subcutaneously on a Q2W regimen (once every two weeks) until hemoglobin responses of ≥1g/dL from baseline were observed. Mean hemoglobin levels were durably increased by at least 0.6g/dL from Day 8 to Day 57 among patients treated with a single 0.25mg/kg dose of ACE-536 (Figure 15). With respect to safety, ACE-536 was observed to be well tolerated at all tested doses without the emergence of serious treatment-related adverse events, anti-drug antibody reactions, or study discontinuations. The most common adverse event was low-grade bleeding at the injection site (9% across all dose cohorts).

FIGURE 15. Mean Change in Hemoglobin from Baseline in ACE-536 Phase I Trial



Source: Attie et al, ASH, 2012

Phase II Trials with Sotatercept and ACE-536 in Patients with β-thalassemia

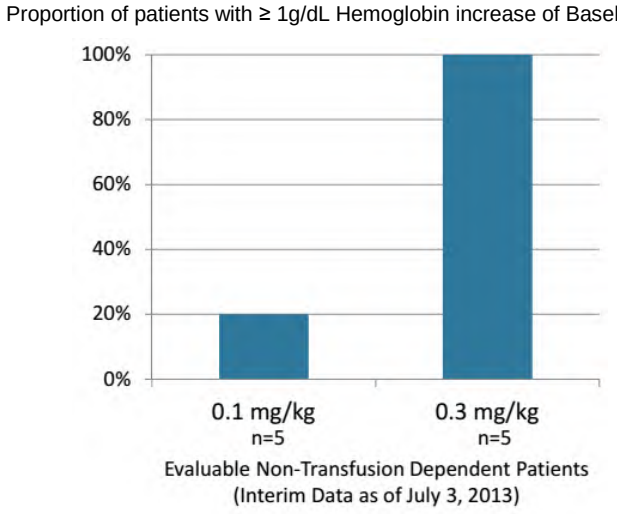
Based on the safety profile and initial efficacy signal observed in Phase I, a Phase II trial of sotatercept was initiated in November 2012, in collaboration with Celgene, to treat anemia in patients with β-thalassemia. The trial was designed as a multi-dose, ascending study evaluating 0.1, 0.3, and 0.5mg/kg subcutaneous doses administered Q3W for six cycles (with continued treatment at the discretion of clinical investigators). In addition to safety and establishing the appropriate dose for future study, primary endpoints of the trial include a percentage reduction of transfusion burden by ≥20% among transfusion-dependent patients, and the percentage increase in hemoglobin by ≥1g/d lasting at least twelve weeks among non–transfusion dependent patients. As noted above, the trial is currently enrolling a third cohort of patients at 0.5mg/kg. Interim results from the first two dosing cohorts among non-transfusion dependent patients were disclosed by Acceleron in July, wherein all five patients treated at 0.3mg/kg achieved a ≥1g/dL increase in hemoglobin from baseline, albeit with varying duration on therapy in achieving this threshold (Figure 17). We regard these data as particularly encouraging, in that the 1g/dl threshold is typically considered the most clinically relevant for patient benefit.

Updated interim results, including responses from the third-dose cohort are expected to be presented at ASH 2013 in December, at which point the trial is expected to transition to enrolling an expansion Phase II cohort at the established recommended Phase II dose (Figure 18).

A similar Phase II trial of ACE-536 in β-thalassemia was initiated in March 2013, again in collaboration with Celgene, evaluating subcutaneous doses of 0.2, 0.4, 0.6, 0.8, and 1.0mg/kg administered Q3W for up to four cycles. As of the latest update in July, patients were being enrolled into the 0.6mg/kg cohort. Interim data from the dose escalation portion of the trial is anticipated to be presented at ASH in December, while complete data from the dose escalation study and from the RP2D dose expansion cohort are expected during 2Q14 and 4Q14, respectively.

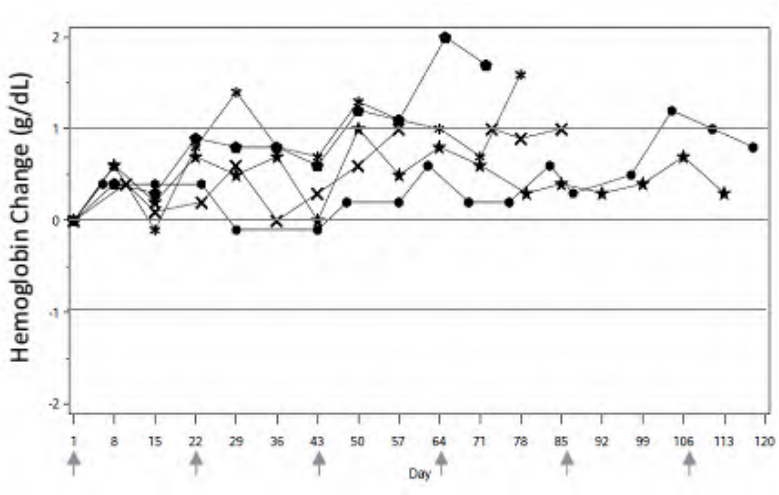
The fact that sotatercept and ACE-536 are both being pursued in the same indications has driven Acceleron/Celgene to adopt a pick-a-winner strategy across the ActRII franchise. While sotatercept will grant investors the greater near-term dataset with which to assess the clinical prospects of the program, Acceleron has stated that, all things being equal, it is likely to pursue ACE-536 in β-thalassemia, based on a slightly more specific ligand-binding profile, potentially contributing a wider therapeutic index. The molecule's benefit on bone parameters is also one of the deciding factors behind this strategy.

FIGURE 16. Increasing Hemoglobin Response in Non-Transfusion Dependent β-Thalassemia with Sotatercept



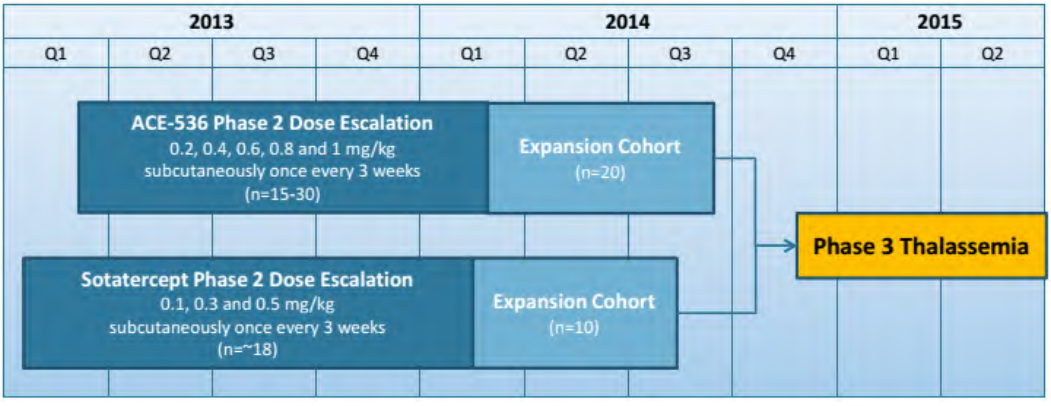
Source: Acceleron company presentation

FIGURE 17. Hemoglobin Change from Baseline in Non-Transfusion Dependent β-Thalassemia with 0.3 mg/kg of Sotatercept



Source: Acceleron company presentation

FIGURE 18. Protocols and Development Timelines for ACE-536 and Sotatercept in β-Thalassemia



Source: Acceleron company presentation

Phase II Trials of Sotatercept and ACE-536 in Patients with MDS and Myeloproliferative Neoplasias

Acceleron, in collaboration with Celgene, is also conducting separate Phase II clinical trials of sotatercept and ACE-536 in patients with low- or intermediate-1 risk MDS. The sotatercept trial, initiated in December 2012, is a multiple-ascending study evaluating 0.1, 0.3, 0.5 mg/kg and potentially higher doses of drug administered subcutaneously, Q3W. The trial plans to enroll up to 75 patients (60 patients in the dose escalation portion and 15 patients in an expansion phase) and will assess

sotatercept activity using the primary endpoint of hematological improvement (HI-E), defined as an increase in hemoglobin patients requiring <4 units red blood cell transfusions in the eight weeks prior to enrollment, or a decrease in number of red blood cell transfusions for patients requiring ≥4 transfusions prior to enrollment. A similar Phase II trial with ACE-536, initiated in January 2013, is evaluating multiple ascending doses of drug (0.125, 0.25, 0.5, 0.75, and 1mg/kg) administered subcutaneously Q3W. The trial will assess hematologic improvement in up to 60 patients with either transfusion dependent non-transfusion MDS. Data from the dose escalation portion of both trials are expected to be disclosed in 2Q14, while final data including results from the expansion phases are expected in 4Q14.

Competitive Landscape

The competitive landscape for sotatercept and ACE-536 can be viewed primarily from two perspectives: first, in relation to developing therapies targeting the TGFβ pathway, and secondly, in relation to other developing and commercially available hematology products with potential applicability to β-thalassemia-related anemia.

Key competitors in the TGFβ inhibition landscape include the small molecule Type I receptor kinase inhibitor LY2157299 and Type II neutralizing antibody IMC-TR1 (LY3022859), both by Eli Lily (LLY, NC). Pre-clinical studies have shown that LY2157299 can stimulate hematopoiesis in *in vitro* and *in vivo* models of MDS, while early Phase I testing showed the drug to be well tolerated. Development efforts with LY2157299, have been focused on advanced, difficult-to-treat, solid tumor indications including glioma/glioblastoma, pancreatic cancer, and HCC. Pre-clinical reporting with IMC-TR1, by contrast, is much harder to come by. Nevertheless, the antibody is currently being evaluated in a Phase I trial in patients with advanced refractory solid tumors.

Outside of the TGFβ pathway, Array Biopharma's (ARRY, NC) oral small molecule p38/Tie2 ARRY-612 presents as a compelling approach to MDS-related anemia. Pre-clinical evidence shows that p38 MAPK is activated in a large proportion of bone marrow cells in patients with low-grade MDS, while Phase I results with ARRY-614 have yielded signs of hematologic improvement, including erythrocyte, platelet, and neutrophil recovery, in heavily pre-treated patients with low/intermediate-1 MDS.

Taking yet another approach to ameliorating disease-associated anemia, HemaQuest's (private) oral small molecule, short chain fatty acid (SCFA) derivative compound, HQK-1001, has been shown to induce fetal hemoglobin expression. Phase I/II testing in patients with β-thalassemia intermedia showed HQK-1001 to be well-tolerated and capable of inducing fetal hemoglobin levels by 6.6% above baseline at the highest dose levels tested. For the time being, however, Phase II development with HQK1001 is taking place in the context of sickle cell disease, widening the competitive landscape for sotatercept/ACE-536.

Partnership with Celgene

Acceleron's partnership with Celgene is structured, such that CELG holds worldwide commercial rights to sotatercept and ACE-536, while being 100% responsible for costs related to the development and commercialization of both product candidates. Under this arrangement, Acceleron received an upfront payment of $70MM. In addition, the company is entitled to a royalty on worldwide net sales in the low-to-mid 20% range and is eligible to earn an aggregated $567MM in development, regulatory, and commercial milestones.

Commercial Opportunity

In modeling the commercial opportunity for ACE-536 in β-thalassemia, we developed a prevalence-driven model, further segmented by geography (U.S., EU, and Japan) and severity of disease (either transfusion-dependent or non-transfusion dependent). In the U.S., we estimate an addressable β-thalassemia population of ~2,250 patients at the time of projected launch in 2016, equally split across transfusion-dependent subtypes. In Europe, where the β-thalassemia prevalence rate is markedly greater than that of the U.S., we model a prevalence of ~28,000 at the time of the projected launch in 2017, similarly divided across severity of disease. Our model predicts chronic, long-term duration of therapy that approaches 15 cycles per year, while expecting higher rates of discontinuation among transfusion-dependent patients (15% versus 5% among non-transfusion dependent settings). We model peak market penetration rates of 45% and 70% in transfusion-dependent and non-transfusion dependent segments, respectively.

From a pricing perspective, we model a per cycle initial list price of $9,500 in the U.S.–a conservative assumption, in our view, when juxtaposed with other orphan indication products such as Soliris (ALXN, NC) and Naglazyme (BMRN, NC) that are priced at an annual cost in excess of $350,000. EU pricing is modeled at a 20% discount to U.S. pricing to reflect a stricter reimbursement environment. Carrying these assumptions forward, as well as a launch in Japan beginning in 2018, we forecast worldwide ACE-536 net sales of $1.4B in 2020 and $2.7B in 2025. We summarize ACE-526 revenue forecast by geography in Figure 19 and delineate our segmented U.S. and EU market model in Figures 20 and 21.

Separately, we have modeled the market opportunity for sotatercept in MDS, similarly segmented by geography. In the U.S., we begin with an addressable prevalence of a low-risk/intermediate-1 MDS population of ~10,200 at the anticipated time of market launch in 2017. We model a steady duration of therapy of ~six cycles and an initial list price of $9,500 U.S. (noting a 20% discount in the EU). Modeling peak penetration of 72% market share six years from launch, we forecast worldwide sotatercept sales of $~1.0B ($465MM in the U.S.) in 2023 and ~$1.3B by 2025 ($571MM in the U.S.). Our sotatercept revenue forecast is described in further detail in Figures 22 - 23.

FIGURE 19. Summary of Combined ACE-536/Sotatercept Revenues, by Geography and Disease

Sotatercept / ACE-536 Revenue Summary	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Sotatercept / ACE-536 Sales													
WW Sales	**$ -**	**$ -**	**$ -**	**$ 16.3**	**$ 173.3**	**$ 578.6**	**$ 1,086.5**	**$ 1,768.4**	**$ 2,357.7**	**$ 2,974.5**	**$ 3,411.2**	**$ 3,781.4**	**$ 4,002.8**
US Sales	-	-	-	16.3	50.4	141.8	253.5	391.7	530.7	665.6	775.5	868.8	948.6
Ex-US Sales	-	-	-	0.0	122.9	436.8	833.0	1,376.6	1,827.0	2,308.8	2,635.7	2,912.6	3,054.2
Effective royalty rate				*20%*	*20%*	*21%*	*23%*	*24%*	*25%*	*25%*	*25%*	*25%*	*25%*
Royalty revenue to XLRN	**$ -**	**$ -**	**$ -**	**$ 3.3**	**$ 34.7**	**$ 123.9**	**$ 252.5**	**$ 429.8**	**$ 583.0**	**$ 743.4**	**$ 856.9**	**$ 953.2**	**$ 1,010.7**
Sales milestones													
ACE-536						$ 50.0	$ 50.0		$ 50.0				
Sotatercept									$ 40.0		$ 40.0		
Breakdown by Indication and Geography													
US	**$ -**	**$ -**	**$ -**	**$ 16.3**	**$ 50.4**	**$ 141.8**	**$ 253.5**	**$ 391.7**	**$ 530.7**	**$ 665.6**	**$ 775.5**	**$ 868.8**	**$ 948.6**
B-thalassemia	-	-	-	16.3	27.4	81.1	131.5	185.8	237.1	274.4	310.9	345.7	377.5
MDS	-	-	-	-	23.0	60.6	122.0	206.0	293.6	391.3	464.6	523.1	571.2
EU	**$ -**	**$ -**	**$ -**	**$ -**	**$ 122.9**	**$ 424.1**	**$ 792.9**	**$ 1,267.7**	**$ 1,643.6**	**$ 2,057.0**	**$ 2,301.4**	**$ 2,531.8**	**$ 2,646.3**
B-thalassemia	-	-	-	-	122.9	424.1	758.4	1,159.3	1,454.7	1,766.8	1,915.3	2,071.2	2,133.3
MDS	-	-	-	-	-	-	34.5	108.4	188.9	290.2	386.1	460.6	513.0
JPN	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 12.7**	**$ 40.1**	**$ 108.9**	**$ 183.4**	**$ 251.8**	**$ 334.3**	**$ 380.8**	**$ 407.9**
B-thalassemia	-	-	-	-	-	12.7	40.1	73.1	119.2	155.0	190.3	209.9	220.5
MDS	-	-	-	-	-	-	-	35.7	64.2	96.8	144.0	170.9	187.3

Source: JMP Securities LLC and Company Reports

FIGURE 20. Summary ACE-536 Revenue, by Geography

ACE-536 Revenue Build	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
β-thalassemia - US				$ 16	$ 27	$ 81	$ 131	$ 186	$ 237	$ 274	$ 311	$ 346	$ 377
β-thalassemia - EU					123	424	758	1,159	1,455	1,767	1,915	2,071	2,133
β-thalassemia - JPN						13	40	73	119	155	190	210	221
Total ACE-536 Sales WW				**$ 16**	**$ 150**	**$ 518**	**$ 930**	**$ 1,418**	**$ 1,811**	**$ 2,196**	**$ 2,417**	**$ 2,627**	**$ 2,731**

Source: JMP Securities LLC and Company Reports

FIGURE 21. ACE-536 Revenue Model in β-thalassemia, U.S. and Europe

US

ACE-536 in β-thalassemia ($MM)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
β-thalassemia prevalence population, US	2,000	2,080	2,163	2,250	2,340	2,433	2,531	2,632	2,737	2,847	2,960	3,079	3,202
%Growth		*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*	*4.0%*
% transfusion-dependent	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Addressable transfusion-dependent population	1,000	1,040	1,082	1,125	1,170	1,217	1,265	1,316	1,369	1,423	1,480	1,539	1,601
% non-transfusion dependent population	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Addressable non-transfusion dependent population	1,000	1,040	1,082	1,125	1,170	1,217	1,265	1,316	1,369	1,423	1,480	1,539	1,601
Transfusion-dependent β-thalassemia													
# transfusion-dependent pts	1,000	1,040	1,082	1,125	1,170	1,217	1,265	1,316	1,369	1,423	1,480	1,539	1,601
Market penetration				***3%***	***5%***	***22%***	***35%***	***40%***	***45%***	***45%***	***45%***	***45%***	***45%***
Cycles of Therapy				15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0
Less % discontinuation				*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*
Transfusion-dependent patient months on Tx				430	746	3,413	5,646	6,711	7,852	8,166	8,493	8,833	9,186
Non-transfusion dependent β-thalassemia													
# Non- transfusion dependent pts	1,000	1,040	1,082	1,125	1,170	1,217	1,265	1,316	1,369	1,423	1,480	1,539	1,601
Market penetration				***8%***	***12%***	***25%***	***35%***	***50%***	***60%***	***66%***	***70%***	***72%***	***72%***
Cycles of Therapy				15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0
Less % discontinuation				*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*
Non-transfusion dependent pt months on Tx				1,282	2,000	4,334	6,311	9,376	11,701	13,386	14,765	15,795	16,427
Total patients cycles on therapy				**1,713**	**2,746**	**7,747**	**11,957**	**16,087**	**19,553**	**21,552**	**23,258**	**24,627**	**25,613**
Cost of per cycle of therapy				$9,500	$9,975	$10,474	$10,997	$11,547	$12,125	$12,731	$13,367	$14,036	$14,738
% price increase					*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*
US sales of ACE-536				**$16.3**	**$27.4**	**$81.1**	**$131.5**	**$185.8**	**$237.1**	**$274.4**	**$310.9**	**$345.7**	**$377.5**
% Growth					*68%*	*196%*	*62%*	*41%*	*28%*	*16%*	*13%*	*11%*	*9%*

EU

ACE-536 in β-thalassemia ($MM)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
β-thalassemia prevalence population, EU	25,000	25,750	26,523	27,318	28,138	28,982	29,851	30,747	31,669	32,619	33,598	34,606	35,644
%Growth		*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*	*3.0%*
% transfusion-dependent	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Addressable transfusion-dependent population	12,500	12,875	13,261	13,659	14,069	14,491	14,926	15,373	15,835	16,310	16,799	17,303	17,822
% non-transfusion dependent population	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Addressable non-transfusion dependent population	12,500	12,875	13,261	13,659	14,069	14,491	14,926	15,373	15,835	16,310	16,799	17,303	17,822
Transfusion-dependent β-thalassemia													
# transfusion-dependent pts	12,500	12,875	13,261	13,659	14,069	14,491	14,926	15,373	15,835	16,310	16,799	17,303	17,822
Market penetration					***3%***	***12%***	***22%***	***35%***	***40%***	***45%***	***45%***	***45%***	***45%***
Cycles of Therapy					15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0
Less % discontinuation					*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*	*15%*
Transfusion dependent patient months on Tx					5,381	22,171	41,866	68,604	80,757	93,577	96,384	99,276	102,254
Non-transfusion dependent β-thalassemia													
# Non- transfusion dependent pts	12,500	12,875	13,261	13,659	14,069	14,491	14,926	15,373	15,835	16,310	16,799	17,303	17,822
Market penetration					***5%***	***15%***	***25%***	***35%***	***45%***	***55%***	***60%***	***65%***	***65%***
Cycles of Therapy					15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0	15.0
Less % discontinuation					*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*
Non-transfusion dependent pt months on Tx					10,024	30,974	53,173	76,675	101,540	127,827	143,631	160,268	165,076
Total patients cycles on therapy					**15,405**	**53,145**	**95,039**	**145,279**	**182,296**	**221,404**	**240,015**	**259,544**	**267,330**
Cost of per cycle of therapy					$7,980	$7,980	$7,980	$7,980	$7,980	$7,980	$7,980	$7,980	$7,980
% price increase						*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
EU sales of ACE-536					**$122.9**	**$424.1**	**$758.4**	**$1,159.3**	**$1,454.7**	**$1,766.8**	**$1,915.3**	**$2,071.2**	**$2,133.3**
% Growth						*245%*	*79%*	*53%*	*25%*	*21%*	*8%*	*8%*	*3%*

Source: JMP Securities LLC and Company Reports

FIGURE 22. Summary of Sotatercept Revenues in MDS by Geography

Sotatercept Revenue Build	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
MDS - US					$ 23	$ 61	$ 122	$ 206	$ 294	$ 391	$ 465	$ 523	$ 571
MDS - EU							35	108	189	290	386	461	513
MDS - JPN								36	64	97	144	171	187
Total Sotatercept Sales WW					**$ 23**	**$ 61**	**$ 156**	**$ 350**	**$ 547**	**$ 778**	**$ 995**	**$ 1,155**	**$ 1,272**

Source: JMP Securities LLC and Company Reports

FIGURE 23. Segmented Sotatercept Revenue Model in MDS, U.S.

US													
Sotatercept of Low Risk/Int-1 MDS ($MM)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Low Risk/Int-1 MDS													
MDS prevalence population, US	30,000	30,330	30,664	31,001	31,342	31,687	32,035	32,388	32,744	33,104	33,468	33,836	34,209
% growth	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*
% with low risk/int-1 disease	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*	*77%*
% non-del(5q)	*85%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*	*67%*
% non-responsive to ESA therapy	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
Addressable patient population	9,818	7,824	7,910	7,997	8,085	8,174	8,263	8,354	8,446	8,539	8,633	8,728	8,824
Market Penetration					***5%***	***12%***	***22%***	***35%***	***47%***	***59%***	***66%***	***70%***	***72%***
Low risk/Int-1 MDS pts on Sotatercept					404	981	1,818	2,924	3,970	5,038	5,698	6,110	6,353
Cycles on therapy					5.7	5.9	6.1	6.1	6.1	6.1	6.1	6.1	6.1
Total patient cycles on therapy					2,304	5,787	11,090	17,837	24,216	30,733	34,757	37,269	38,755
Cost of therapy (per cycle)					$9,975	$10,474	$10,997	$11,547	$12,125	$12,731	$13,367	$14,036	$14,738
% price increase						*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*
US sales of Sotatercept					**$23.0**	**$60.6**	**$122.0**	**$206.0**	**$293.6**	**$391.3**	**$464.6**	**$523.1**	**$571.2**

Source: JMP Securities LLC and Company Reports

Additional opportunities for Sotatercept

In addition to β-thalassemia and MDS, sotatercept is concurrently being evaluated for its ability to ameliorate anemia-related complications due to multiple myeloma and chronic kidney disease. Investigators at Mass General have initiated a Phase I investigator-sponsored trial in patients with relapsed/refractory multiple myeloma in combination with Revlimid (lenalidomide, CELG) and dexamethasone. Multiple myeloma represents a large and attractive market opportunity, evidenced not only by Revlimid sales of $3.7B in 2012, but by the successful launch of new market entrants, Kyprolis (Onyx, now Amgen, AMGN, NC) and Pomalyst (Celgene).

A second IST sponsored by the North Shore Long Island Jewish Health System is evaluating sotatercept for the treatment of Diamond Blackfan Anemia (DBA) – a congenital, pure, red cell anemia that can manifest severe clinical abnormalities including craniofacial, kidney, and heart malformations, as well as growth retardation. Occurring in approximately seven per million live births, and driven by a similar underlying biology to that described above, DBA presents a compelling market opportunity for the sotatercept/ACE-536-one that rivals that of β-thalassemia in the U.S.

Finally, Acceleron/Celgene is conducting a Phase II trial evaluating sotatercept in patients with end-stage renal disease (ESRD) and on hemodialysis. Recognizing that there is likely a high threshold for demonstrating meaningful activity in this setting, given its size (an estimated prevalence population of ~875,000 in the U.S., supporting $2B in Epogen sales in FY2012), sotatercept adoption in ESRD could deliver appreciable revenue, with only moderate penetration rates. Taken together, there are multiple ongoing development avenues with the potential to create additional value for Acceleron over the medium to long term.

DALANTERCEPT – PUSHING ANTI-ANGIOGENESIS BEYOND VEGF

Acceleron's third clinical-stage program, dalantercept, takes advantage of the blood vessel specific expression of a particular TGFβ family receptor called ALK1 and the prominent role it plays in the maturation of blood vessels, both in normal tissue and during tumorigenesis. Like sotatercept/ACE-536, dalantercept is a soluble ALK1-Fc fusion protein functioning as a ligand trap. Unlike sotatercept/ACE-536, however, Acceleron retains worldwide development and commercialization rights to the program. On the basis of compelling pre-clinical activity in kidney cancer models, Acceleron's leading dalantercept program in second-line renal cell carcinoma RCC in combination with the VEGF TKI therapy, axitinib (Inlyta, PFE, NC). As a whole, the VEGF therapy space represents upwards of $8 billion in global annual sales; capturing a modest fraction of that market as a differentiated product with a complementary mechanism of action, dalantercept represents a significant portion of Acceleron value from this one indication alone. Our model forecasts ~$800MM in dalantercept sales ($663MM in the U.S.) in 2022.

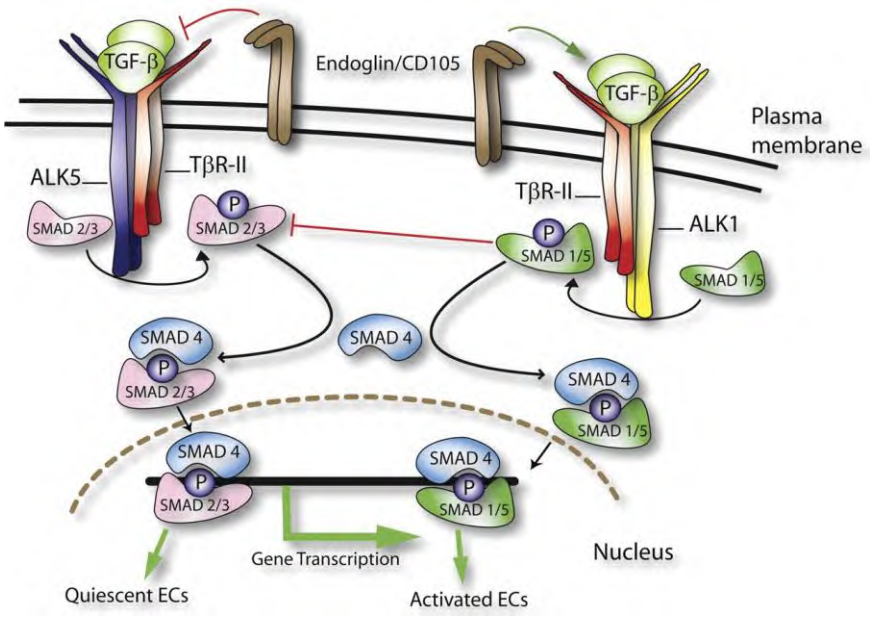
Biology of Activin-like Protein Kinase 1 (ALK1) and Its Role During Angiogenesis

As described above, TGFβ signaling can exert pleiotropic control over a diversity of cell types, notably in epithelial cells, fibroblasts, and immune cells, but also in endothelial and perivascular cells. TGFβ signaling in endothelial cells proceeds predominantly through the activation of Type I activin-receptor-like kinases (ALK) 5 and 1, following the unregulated secretion of bone morphogenic proteins BMP9, BMP10, and TGFβ (Figure 24). Whereas ALK5 is ubiquitously expressed, ALK1 expression is primarily restricted to endothelial cell types. In addition, growing genetic and histopathologic evidence supports ALK1 as playing a critical role in the development and maintenance of new blood vessel formation or angiogenesis. *Acvrll* (ALK1) knockout mice are embryonic lethal and die mid-gestation from severe vascular abnormalities. In humans, germ-line mutations of ALK1 have been observed in association with hereditary hemorrhage telangiectasia (HHT), characterized by spider veins, increasingly severe nosebleeds, and gastrointestinal bleeding. It is the genetic human proof of concept that, in our view, provides strong validity to ALK1 as a target for therapeutic intervention.

As such, ALK1 has been suspected to play a role in tumor-related angiogenesis. Tumors rely on new blood vessel formation as a source of nutrients and oxygen for continued growth. It has been widely accepted that initial steps in this process are driven by vascular endothelial growth factor (VEGF) signaling; pharmacologic inhibition of the pathway has been shown to be an effective means of cancer treatment, while the multiple indications and $5.98 billion in Avastin sales in 2012 have validated the commercial opportunity of anti-angiogenic therapy.

ALK1 has also been shown to be overexpressed in the vasculature of developing tumors, relative to tumor cells and normal tissue. Down-regulation of ALK1 either via genetic silencing or pharmacologic inhibition has been shown to slow the growth of nascent tumors in xenograft mice. On the basis of these data, ALK1 is regarded as an attractive therapeutic target in solid tumors that is both differentiated, and perhaps complementary, to the VEGF pathway, which is of particular importance in the significant proportion of patients underserved by VEGF-targeted agents.

FIGURE 24. ALK1 Signaling in Endothelial Cells



Source: Fonsatti et al, Cardiovascular Research, 86,12-19, 2010

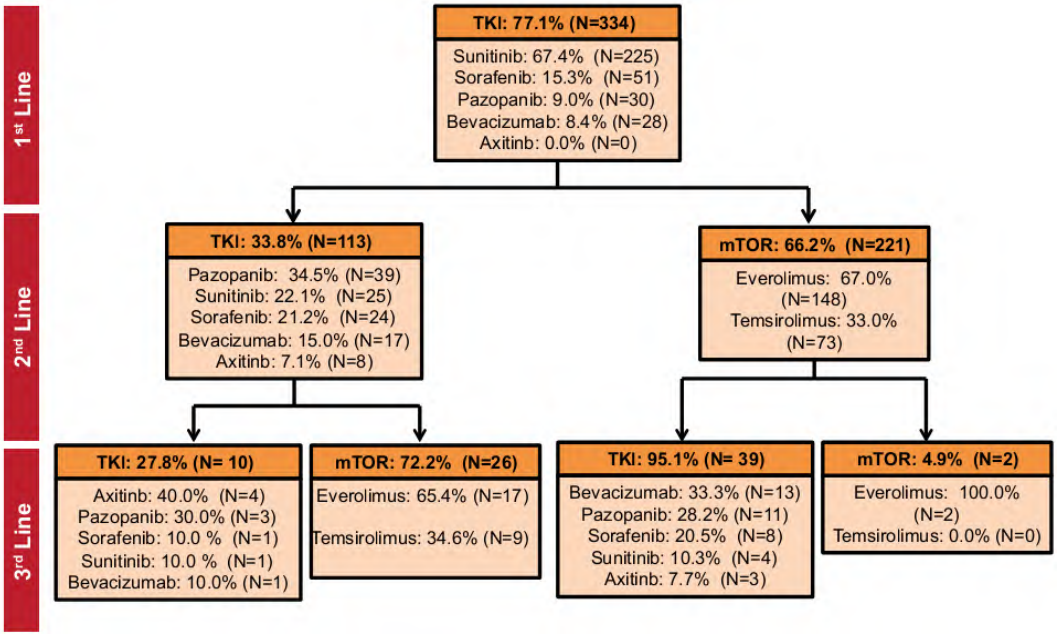
Current Treatment Paradigm for Advanced Renal Cell Carcinoma

In the U.S., kidney cancer accounts for 2-3% of adult cancer diagnoses per year. In 2012, the American Cancer Society estimated that nearly 65,000 new cases of renal cell carcinoma were diagnosed, and approximately 13,700 patients died as a result of the disease. Extrapolating Globocan 2008 estimates, roughly 52,000 patients were diagnosed with RCC among the EU5 in 2012, resulting in nearly 21,000 patient deaths. It is also estimated that at the time of diagnosis, about 18% of patients present with stage IV metastatic disease – a stage at which the cancer is no longer operable and system therapy becomes standard of care.

Several targeted and multi-targeted tyrosine kinase inhibitors (TKIs) have been approved and adopted in the treatment of metastatic RCC, creating an arguably crowded and highly competitive landscape (Figure 25). Currently, the first-line treatment setting is dominated by the multi-TKI Sutent (sunitinib; Pfizer), followed by the VEGF-inhibitor therapies, Avastin (bevacizumab, Roche; RHHBY, NC) and Votrient (pazopanib; GSK). Torisel (temsirolimus; Pfizer), the rapamycin analog inhibitor of mTOR, is seeing some use in the front-line setting, particularly in patients with non-clear cell histology (according to NCCN guidelines and market estimates from BioMed Tracker). The second-line treatment setting is divided among VEGF inhibitors Inlyta (axitinib, Pfizer) and Caprelsa (vandetanib, AstraZeneca; AZN, NC), the multi-kinase inhibitor Nexavar (sorafenib, Bayer; BAYRY, NC; Onyx, MO), and the mTOR inhibitors Afinitor (everolimus, Novartis; NVS, NC) and Torisel.

FIGURE 25. Current Treatment Landscape in Advanced RCC

Treatment preferences across the therapeutic landscape based on responses from surveyed community-oncologists (out of a total 433 patients with suitable survey criteria)

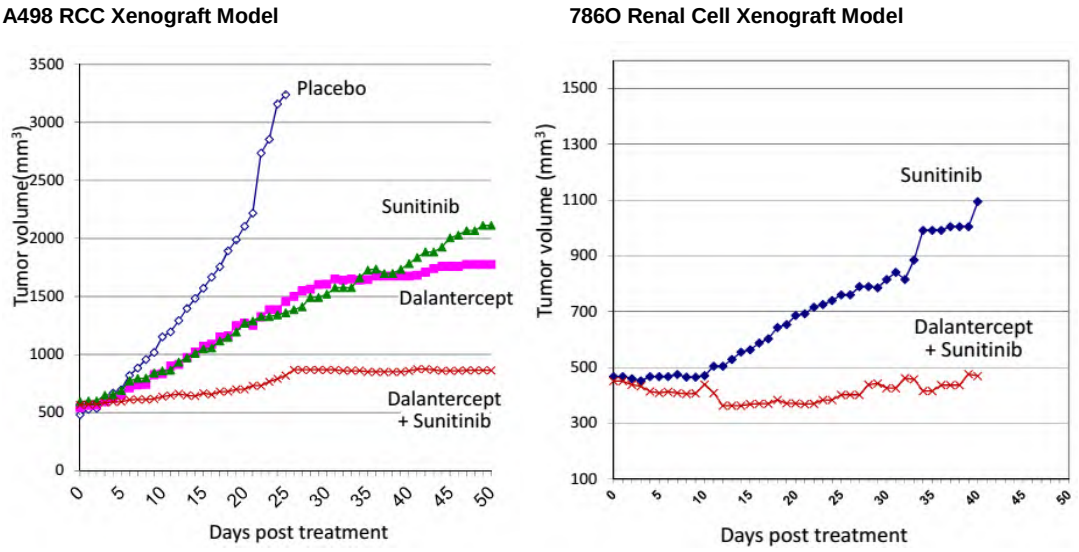


Source: Vogelzang, NJ et al., ASCO GU Abstract #418, US Oncology Research, and Novartis Pharmaceuticals.

Pre-clinical data with Dalantercept

Dalantercept (ACE-041) is an ALK1-Fc fusion protein that acts as a ligand trap that binds to BMP9 and BMP10, thereby neutralizing the ability to activate ALK1. Early *in vivo* pre-clinical studies using the chick choriallantoic membrane (CAM) assay established dalantercept as an antiangiogenic, able to reverse both BMP and VEGF-induced blood vessel formation. Treatment with dalantercept was shown to slow tumor growth in an orthotropic, breast cancer mouse model. More recently, Acceleron investigators have shown that dalantercept has an additive effect on the level of tumor growth inhibition achievable by TKI therapy (sunitinib) in RCC xenograft models (Figure 26).

FIGURE 26. Pre-clinical Data Demonstrated Dalantercept Synergies with TKI Therapy in RCC



Source: Acceleron company presentation

Clinical Results and Development Plans

Initial dalantercept safety and pharmacodynamic activity was assessed in an advanced solid tumor Phase I study, which was presented at ASCO in 2011. In the initial dose escalation portion of the trial, 25 patients were enrolled according to a standard 3+3 design, evaluating a dose range of 0.1 to 4.8mg/kg administered subcutaneously on a Q3W schedule. Frequent drug-related adverse events included fatigue, peripheral edema, anemia, nausea, and headache. Adverse events Grade≥3 observed at higher-dose cohorts included anemia, congestive heart failure, and thrombocytopenia (Figure 27).

Moderate tumor response was noted in the Phase I study, including one partial response in a patient with head and neck squamous cell carcinoma lasting ten cycles. Durable stable disease was noted in eight patients of varying origin (three NSCLC, colorectal, small bowel, HNSCC, ovarian, and carcinoid) (Figure 28).

Coupling the encouraging results from the Phase I study, with the pre-clinical data indicating potential synergism with VEGF TKI therapy, Acceleron is conducting a two-part combination Phase II study in second-line renal cell carcinoma (RCC) in combination with Inlyta (axitinib). The first portion will establish the safety and tolerability of combining Inlyta with escalating doses of dalantercept (0.6, 0.9, 1.2, and 1.5mg/kg subcutaneously Q3W), with the second portion of the trial evaluating the efficacy of the combination, at the recommended Phase II dose of dalantercept in single-arm expansion cohort in up to 20 additional patients. If the results of Part 1 merit further study, the trial will be expanded into a randomized, placebo-controlled study of dalantercept plus Inlyta, versus Inlyta plus placebo, in up to 112 patients.

FIGURE 27. Dalantercept Phase I Safety and Adverse Event Profile

Table 2. ACE-041 Related Treatment-emergent Adverse Events (TEAEs)
Any ACE-041 Related Event in ≥ 3 Patients [n (%)]

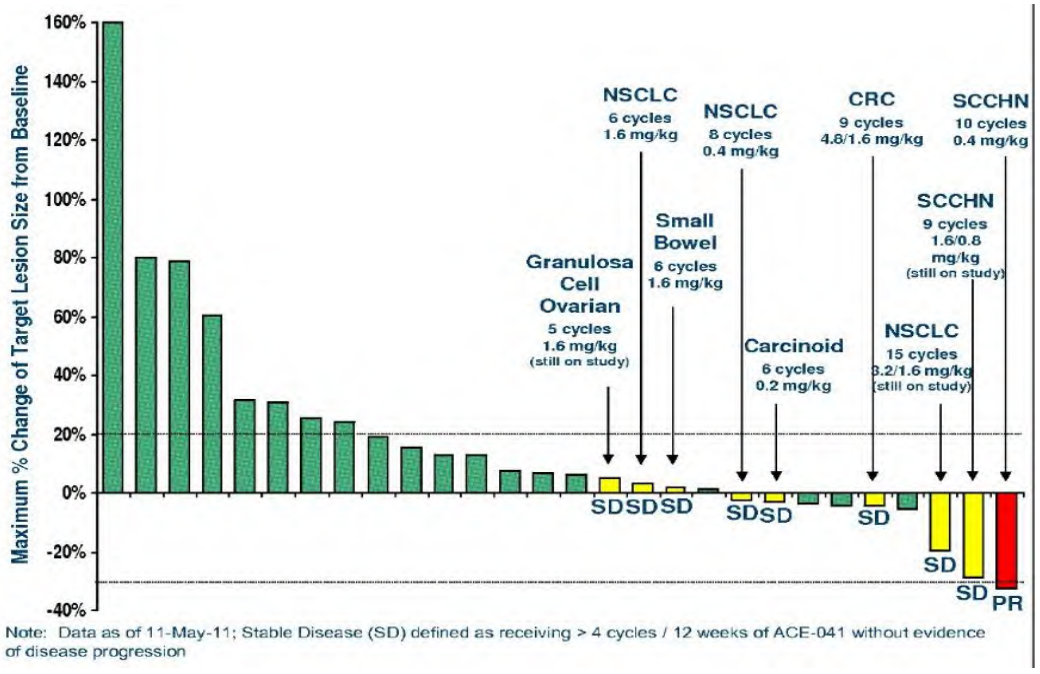
Preferred Term	ACE-041 Dose Level (mg/kg)						
	0.1 (n=3)	0.2 (n=3)	0.4 (n=3)	0.8 (n=4)	1.6 (n=17)	3.2 (n=6)	4.8 (n=3)
Peripheral edema	0	0	1 (33)	1 (25)	10 (59)	6 (100)	1 (33)
Fatigue	0	1 (33)	2 (67)	2 (50)	9 (53)	3 (50)	1 (33)
Anemia	0	0	0	0	6 (35)	2 (33)	3 (100)
Dyspnea	0	0	0	1 (25)	8 (47)	1 (17)	1 (33)
Anorexia	0	0	1 (33)	1 (25)	2 (12)	4 (67)	1 (33)
Nausea	0	1 (33)	0	1 (25)	2 (12)	3 (50)	1 (33)
Headache	0	1 (33)	1 (33)	0	2 (12)	2 (33)	2 (67)
Epistaxis	0	0	0	0	4 (24)	2 (33)	1 (33)
Pyrexia	0	0	0	0	4 (24)	1 (17)	1 (33)
Telangiectasia	0	0	0	0	4 (24)	1 (17)	0
Dizziness	0	0	0	1 (25)	2 (12)	1 (17)	0
Vomiting	1 (33)	0	0	1 (25)	0	1 (17)	0
Diarrhea	0	0	0	0	2 (12)	1 (17)	0
Dehydration	0	0	1 (33)	0	1 (6)	0	1 (33)
Dysgeusia	0	0	1 (33)	0	1 (6)	1 (17)	0
Night Sweats	0	0	1 (33)	0	0	2 (33)	0

Table 3. ACE-041 Related Grade ≥ 3 TEAEs
Any ACE-041 Related Event ≥ Grade 3 [n (%)]

Preferred Term	ACE-041 Dose Level (mg/kg)						
	0.1 (n=3)	0.2 (n=3)	0.4 (n=3)	0.8 (n=4)	1.6 (n=17)	3.2 (n=6)	4.8 (n=3)
Anemia	0	0	0	0	1 (6)	0	3 (100)
CHF	0	0	0	0	1 (6)	0	1 (33)
Thrombocytopenia	0	0	0	0	0	1 (17)	0
LV dysfunction	0	0	1 (33)	0	0	0	0
Fatigue	0	0	1 (33)	0	0	0	0
Dyspnea	0	0	0	0	1 (6)	0	0

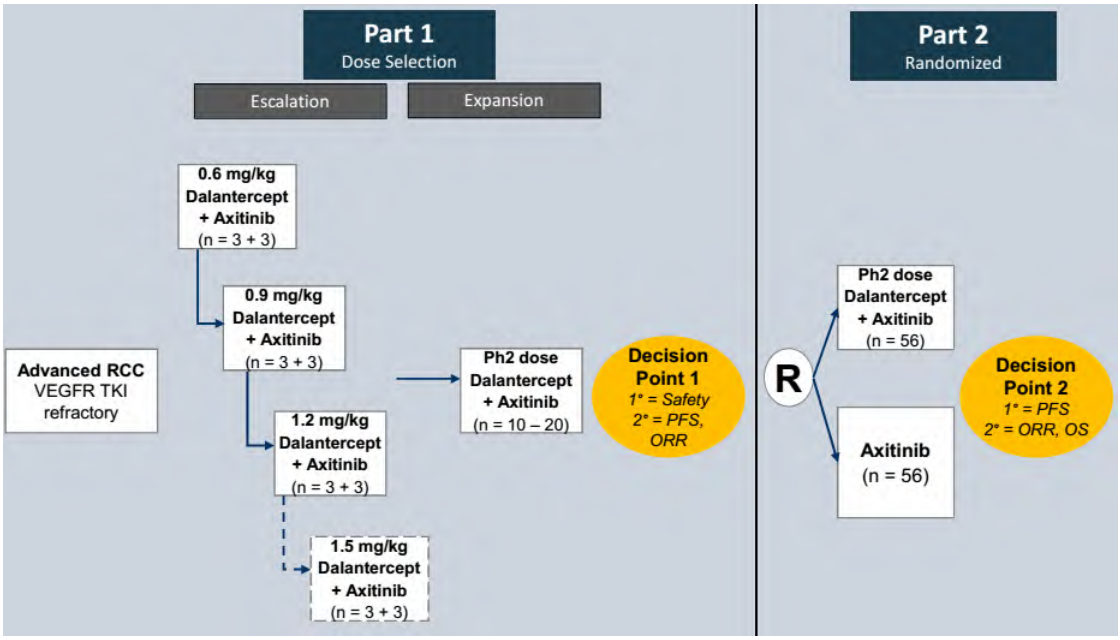
Source: Bendell et al, ASCO, 2011

FIGURE 28. Dalantercept Phase I Tumor Response by RECIST



Source: Bendell et al, ASCO, 2011

FIGURE 29. Dalantercept Phase II Trial Design and Preliminary Results



Source: Acceleron company presentation

Commercial Opportunity

In assessing the commercial opportunity with dalantercept, we have projected revenues related to its use in the treatment of RCC, beginning in second-line and migrating toward front-line use with expanded approval. Our model assumes marketing approval for second-line RCC in the U.S., beginning in 2018, followed by a moderate ramp in market share to 50% peak penetration by 2023. For front-line RCC, we assume expanded marketing approval in 2020 and moderate peak penetration of 20% by 2024.

For Europe and Japan, we model initial marketing approval in 2021 and 2021, respectively, with similar front-line adoption and market share dynamics to those described for the U.S. We also anticipate ex-U.S. sales being led by a global commercial partner, delivering a straight line 20% royalty to Acceleron. All told, we forecast worldwide dalantercept sales of ~$800MM by 2022 ($690MM in sales and royalty revenue to Acceleron) and ~$2.0B in sales by 2025 (~$1.35 to Acceleron). A summary of our dalantercept revenue projections by geography is presented in Figure 30, while a segmented model of dalantercept adoption in the U.S. is presented in Figure 31.

FIGURE 30. Summary of Dalantercept Revenues, by Geography

Dalantercept Revenue Summary	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E
Dalantercept Sales													
WW Sales	$ -	$ -	$ -	$ -	$ -	$ 41.9	$ 90.1	$ 213.8	$ 457.2	$ 792.9	$ 1,157.8	$ 1,547.2	$ 1,988.5
US Sales	-	-	-	-	-	41.9	90.1	213.8	416.1	662.9	866.7	1,047.0	1,191.8
Ex-US Sales	-	-	-	-	-	-	-	-	41.1	130.1	291.2	500.2	796.8
Royalty on ex-US sales						*20%*	*20%*	*20%*	*20%*	*20%*	*20%*	*20%*	*20%*
Royalty revenue	-	-	-	-	-	-	-	-	8.2	26.0	58.2	100.0	159.4
Dalantercept Revenue to XLRN	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 41.9**	**$ 90.1**	**$ 213.8**	**$ 424.3**	**$ 688.9**	**$ 924.9**	**$ 1,147.1**	**$ 1,351.1**
Breakdown by Geography													
US	-	-	-	-	-	41.9	90.1	213.8	416.1	662.9	866.7	1,047.0	1,191.8
EU	-	-	-	-	-	-	-	-	41.1	117.7	246.8	396.2	616.9
JPN	-	-	-	-	-	-	-	-	-	12.4	44.4	104.0	179.8

Source: JMP Securities LLC and Company Reports

FIGURE 31. Segmented Dalantercept Revenue Model in RCC, U.S.

US													
Dalantercept in RCC ($MM)	**2013E**	**2014E**	**2015E**	**2016E**	**2017E**	**2018E**	**2019E**	**2020E**	**2021E**	**2022E**	**2023E**	**2024E**	**2025E**
Renal Cell Carcinoma Incidence, US	66,275	66,938	67,607	68,283	68,966	69,656	70,352	71,056	71,766	72,484	73,209	73,941	74,680
% Growth	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*
% pts with advanced RCC (stage IV metastatic)	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*
% pts receiving targeted front-line TKI therapy	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*	*90%*
Front-line RCC population	14,912	15,061	15,212	15,364	15,517	15,673	15,829	15,988	16,147	16,309	16,472	16,637	16,803
Dalantercept in front-line RCC													
Market Penetration								***2%***	***6%***	***10%***	***12%***	***17%***	***20%***
Duration of therapy (months)								13.2	13.5	13.5	13.5	13.5	13.5
Total patient months in front-line								4,221	13,079	22,017	26,685	38,181	45,368
Dalantercept in second-line RCC													
% pts failing front-line therapy	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*	*85%*
Second-line RCC population	12,675	12,802	12,930	13,059	13,190	13,322	13,455	13,589	13,725	13,863	14,001	14,141	14,283
Market Penetration						***5%***	***10%***	***18%***	***28%***	***40%***	***50%***	***50%***	***50%***
Duration of therapy (months)						7.4	7.5	7.6	7.6	7.6	7.6	7.6	7.6
Total patient months in front-line						4,929	10,091	18,590	29,207	42,142	53,205	53,737	54,274
Total patient months on therapy						4,929	10,091	22,811	42,287	64,159	79,889	91,918	99,642
Cost of therapy (per month)						$8,500	$8,925	$9,371	$9,840	$10,332	$10,848	$11,391	$11,960
% price increase							*5%*	*5%*	*5%*	*5%*	*5%*	*5%*	*5%*
US sales of Dalantercept ($MM)						**$41.9**	**$90.1**	**$213.8**	**$416.1**	**$662.9**	**$866.7**	**$1,047.0**	**$1,191.8**
% growth							*115%*	*137%*	*95%*	*59%*	*31%*	*21%*	*14%*

Source: JMP Securities LLC and Company Reports

Competitive landscape

It goes without saying that RCC is an increasingly crowded market, with five approved VEGF targeted agents and two agents targeting the mTOR pathway. We tend to believe that if a dalantercept-axitinib combination proves to be well tolerated and incrementally more effective than axitinib alone, dalantercept could see appreciable off-label adoption with other RCC anti-VEGF TKI therapy. In that respect, we view front-line TKI drugs (i.e., sunitinib, pazopanib, sorafenib) less as competitors than as guideposts defining that market opportunity.

From a competitive, developing therapy standpoint, we highlight Pfizer's fully human, monoclonal ALK1 antibody PF03446962 (PF'962). Phase I clinical results show PF'962 to have a manageable safety profile (pancreatic enzyme increase and transient thrombocytopenia being the adverse events of greatest concern). Out of 44 patients with advanced solid tumors enrolled in the study, partial responses were observed in three patients, including one NSCLC, one RCC and one HCC, each of which had been treated with prior VEGF therapy. PFE'962 development is ongoing, as evidenced in open Phase II trials evaluating the drugs activity in second-line malignant pleural mesothelioma and in combination with best supportive care in the treatment for recurrent liver cancer.

SUMMARY AND CONCLUSION

We recommend the purchase of Acceleron shares to those investors who have a long-term perspective and a vision toward what kind of company Acceleron can grow into over the course of the next few years. We hope that we have convinced the reader of this report that the company has all the requisite ingredients to become one of the leading developers of unique biologic therapeutic agents amongst publicly traded biotechnology companies. We reiterate our view that shares of XLRN, which currently trade with a market cap of roughly $600MM, can be worth several billion dollars over the course of the next few years, and many times today's market cap over the longer term.

APPENDIX A – BIOGRAPHIES

Senior Management Team

John L. Knopf, Ph.D., President and CEO. Dr. Knopf co-founded Acceleron in 2003 and currently serves as the company's Chief Executive Officer and President. Dr. Knopf served on the company's board of directors from 2003 to 2004, and has also served from 2007 to present. 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 key scientist at the Genetics Institute (GI) from 1982 to 1998, where he participated in the development of pioneering biopharmaceutical products, including the first treatment for hemophilia, recombinant factor VIII Recombinate® and helped establish GI as a premier biopharmaceutical company. Dr. Knopf received a BS in biology from SUNY Stonybrook and his Ph.D. in biology at SUNY Buffalo.

Kevin F. McLaughlin, SVP, Chief Financial Officer, and Treasurer. Mr. McLaughlin joined Acceleron in November 2010. Previously, he served most recently, from 2009 through 2010, as Senior Vice President and Chief Financial Officer of Qteros, Inc. He was a co-founder of Aptius Education, Inc. and from 2007 through 2009, he worked as the Chief Operating Officer and a director. From 1996 through 2007, Mr. McLaughlin held several executive positions with PRAECIS Pharmaceuticals, Inc. He joined PRAECIS as their first Chief Financial Officer where he had responsibility for private financings, partnership financings, the company's initial public offering, and subsequent stock offering. Later, Mr. McLaughlin became COO, and then President and CEO, and he served as a member of the board of directors. In this capacity, he was responsible for negotiating the sale of the company to GlaxoSmithKline. He began his career in senior financial roles at Prime Computer and Computer vision Corporation. Mr. McLaughlin received a BS in business from Northeastern University and an MBA from Babson College.

Matthew L. Sherman, M.D., SVP, and Chief Medical Officer. Dr. Sherman joined Acceleron in May 2006. Previously, he served as Senior Vice President and Chief Medical Officer at Synta Pharmaceuticals where he was responsible for clinical research, clinical operations, biostatistics, data management, regulatory affairs, quality assurance, and program management. Prior to that, Dr. Sherman worked at Genetics Institute and Wyeth Pharmaceuticals in various capacities, including Therapeutic Area Director for Oncology. While at Wyeth, Dr. Sherman provided senior oncology and hematology leadership for worldwide clinical development for both small molecule and biologic therapeutics, including the submission and approval of Mylotarg® by the FDA. 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 D. Ertel, SVP and Chief Business Officer. Mr. Ertel joined Acceleron in January 2006. Mr. Ertel established Acceleron's business development function and currently leads the company's business development, commercial strategy, and program management functions. Mr. Ertel has over 20 years of experience in the biotechnology industry at Vivus, Inc., Genentech, Inc., Biogen Idec, Inc., and Synta. His responsibilities at these companies included program management for pre-clinical and clinical-stage programs, commercial strategy for clinical-stage programs, the market launch of a novel biologic agent, and business development. 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.

Ravindra Kumar, Ph.D., VP and Chief Scientific Officer. Dr. Kumar joined Acceleron in March 2004. Dr. Kumar established and currently leads the company's discovery research. Previously, Dr. Kumar worked for 12 years at Genetics Institute and Wyeth Pharmaceuticals. At Genetics Institute, Dr. Kumar was a key member of the Small Molecule Drug Discovery group and was responsible for cell biology. Following the integration of discovery functions from GI and Wyeth Pharmaceuticals, Dr. Kumar served as Senior Scientist in the Biological Chemistry group. Dr. Kumar is the author of several key scientific manuscripts in the area of protein glycosylation and is named as an inventor of several patents. Dr. Kumar received his BS in chemistry from Rohilkhand University, his MS in chemistry from Meerut University, his Ph.D. from the University of New Brunswick and he completed his post-doctoral fellowship at Albert Einstein College of Medicine, in Bronx, NY.

Robert J. Steininger II, SVP, Manufacturing. Mr. Steininger joined Acceleron in March 2007. He currently serves as a director of the Massachusetts Accelerator for Biomanufacturing, PBE Corporation, and Sunopro. He was previously the Vice President of Process Sciences at Millennium Pharmaceuticals (now Takeda, JP-4502, NC). In this capacity, he was responsible for managing the processes for the bulk production of both large and small molecule clinical candidates. Mr. Steininger also served as a Vice President within the Millennium Product and Portfolio Management organization. Prior to joining Millennium, he held multiple roles at GI from 1984 to 2000, including the Director of Clinical Production, Director of Process Technology, Director of Regulatory Affairs, and Senior Director of Research, Genomics. Mr. Steininger received a SB in chemistry from the Massachusetts Institute of Technology and an MS in chemical engineering from the University of California, Berkeley.

John D. Quisel, J.D., Ph.D., VP, General Counsel and Secretary. Dr. Quisel joined Acceleron in October 2006. Prior to joining the company, Dr. Quisel worked at the Boston office of Ropes & Gray LLP and prior to that, the Boston office of Foley Hoag LLP. In his work at law firms, Dr. Quisel has, through strategic in-licensing and protection of internal research programs, assembled and licensed product and platform-focused intellectual property portfolios for numerous biotechnology ventures. Over his entire career, Dr. Quisel's experience spans many aspects of biotechnology law, including the negotiation of intellectual property licenses and product development collaborations, patent prosecution, and litigation. Dr. Quisel received an AB in biology from Harvard University, an MS in biology from Stanford University, a Ph.D. in biology from the Massachusetts Institute of Technology and a J.D. from Harvard Law School.

Source: Company website

Board of Directors

Anthony B. Evnin, Ph.D. has served as a member of Acceleron's board of directors since 2004. Since 1975, Dr. Evnin has been a Partner at Venrock, Inc. and then VR Management, LLC, both part of Venrock Associates, a venture capital firm, where he focuses largely on life sciences investments and, in particular, biotechnology investments. Before this, he served as a manager of business development at Story Chemical Corporation and a research scientist at Union Carbide Corporation. Dr. Evnin currently serves on the boards of AVEO Pharmaceuticals, Infinity Pharmaceuticals, Inc., Constellation Pharmaceuticals, Inc. and Metabolex, Inc. During the last five years, Dr. Evnin served as a director of Altea Therapeutics Corporation, Celladon Corporation, Kenet, Inc., Boston-Power, Inc., Memory Pharmaceuticals Corp., Sunesis Pharmaceuticals, Inc., Renovis, Inc., Icagen, Inc., Coley Pharmaceutical Group, Inc., and Pharmos Corporation. Dr. Evnin is a Trustee of The Rockefeller University and for The Jackson Laboratory, Trustee Emeritus of Princeton University, a Member of the Boards of Overseers and Managers of Memorial Sloan-Kettering Cancer Center, and a Director of the New York Genome Center. Dr. Evnin received an AB in chemistry from Princeton University and a Ph.D. in chemistry from the Massachusetts Institute of Technology.

Jean M. George has served as a member of Acceleron's board of directors since 2005. Since 2002, Ms. George has been a Managing Director at Advanced Technology Ventures (ATV), and, concurrently since April 2013, Ms. George has been a Managing Director at LSV Capital Management. She joined ATV in 2002 and serves as the firm's East Coast lead partner for healthcare investments. Prior to joining ATV, Ms. George was a Director at BancBoston Ventures, where she led the healthcare team's investment activity in NuGenesis Technologies Corp., Microbia, Inc., Syntonix Pharmaceuticals, Inc. and Neurometrix, Inc. Before BancBoston Ventures, she worked at Genzyme Corporation from 1988 to 1998, where she held a variety of operational roles in marketing, product development, and business development, including Vice President of Global Sales and Marketing. She also worked as a Vice President and Founder of Genzyme's Tissue Repair Division. She is currently a Director of Calithera Biosciences, Hydra Biosciences, Inc., Zeltiq Aesthetics, Inc. and Portola Pharmaceuticals, Inc. Ms. George was a Director of Hypnion, Inc., Critical Therapeutics, Inc. and Proteolix, Inc. She was named a member of the Scientific Advisory Board for the Massachusetts Life Sciences Center. Ms. George received a BS in biology from the University of Maine and an MBA from Simmons College Graduate School of Management.

George Golumbeski, Ph.D. has served as a member of Acceleron's board of directors since 2011. Since 2009, Dr. Golumbeski has been a Senior Vice President of Business Development for Celgene Corporation, where he is responsible for the full array of business development activities, including identification and evaluation of opportunities, structuring and negotiating transactions, in-licensing, M&A, out-licensing, and alliance management. At Celgene, these activities are focused primarily within the therapeutic areas of oncology and inflammation. From 2008 to 2009, Dr. Golumbeski served as the CEO of Nabriva Therapeutics AG. Prior to Nabriva, Dr. Golumbeski served as Vice President of Business Development, Licensing and Strategy for Novartis-Oncology. During his tenure at Novartis, Dr. Golumbeski's group closed a significant number of collaboration agreements which bolstered the development pipeline. Earlier in his career, Dr. Golumbeski held senior positions at Elan Pharmaceuticals and at Schwarz Pharma, where he led the effort to in-license rotigotine and lacosamide (now both approved agents). Dr. Golumbeski received a BA in biology from the University of Virginia and a Ph.D. in genetics from the University of Wisconsin-Madison.

Carl L. Gordon, Ph.D., CFA has served as a member of Acceleron's board of directors since 2006. Dr. Gordon has served as a General Partner and Co-Head of Private Equity of OrbiMed Advisors LLC, or OrbiMed, which he co-founded in 1998. From 1995 to 1997, he was a senior biotechnology analyst at Mehta and Isaly, and from 1993 to 1995 he was a Fellow at The Rockefeller University. Dr. Gordon currently serves on the board of directors of Selecta Biosciences, Singulex, Inc. and other companies. He has also served on the boards of Complete Genomics, Inc., BioCryst Pharmaceuticals, Inc., Arius Research, Inc. and numerous other companies. Dr. Gordon received a BS in chemistry from Harvard and a Ph.D. in molecular biology from the Massachusetts Institute of Technology.

Edwin M. Kania, Jr. has served as a member of Acceleron's board of directors since 2004. Since 2000, Mr. Kania has been Managing Partner and Chairman of Flagship Ventures, a Boston-based venture capital firm that he co-founded and that also manages the Applied Genomic Technology Capital Fund, L.P. (AGTC Fund), as well as funds raised by OneLiberty Ventures. Prior to co-founding Flagship Ventures, Mr. Kania was a General Partner at OneLiberty Ventures and its predecessor firm, Morgan Holland Ventures, which he joined in 1985. Mr. Kania currently serves on the boards of several private companies. Mr. Kania has also served on the boards of Aspect Medical, EXACT Sciences, and other public and private companies. Mr. Kania's direct investment experience covers over 100 companies, and he has been intimately involved in the launch and development of more than a dozen companies as the founding, lead, or co-lead investor, and has, on occasion, assumed operating roles in support of management. Mr. Kania received a BS in physics from Dartmouth College and an MBA from Harvard Business School.

Tom Maniatis, Ph.D. co-founded Acceleron in 2003 and has served as a member of Acceleron's board of directors and chairman of the Scientific Advisory Board since 2003. Since 2010, he has been a Professor and Chair of the Department of Biochemistry & Molecular Biophysics at the Columbia University College of Physicians and Surgeons. Prior to working at Columbia, Dr. Maniatis was a professor at Harvard University, where he studied the mechanisms of transcription and RNA splicing in eukaryotes. Dr. Maniatis currently serves on the board of Constellation Pharmaceuticals, Inc. Dr. Maniatis is also a co-founder of the Genetics Institute and ProScript Inc., where he served on the board of directors. Dr. Maniatis is a member of the U.S. National Academy of Sciences, and has received numerous awards for his research contributions, including the Eli Lilly Research Award in Microbiology and Immunology, the Richard Lounsbery Award for Biology and Medicine from the U.S. and French National Academies of Science, and the 2012 Lasker-Koshland Special Achievement Award in Medical Science. Dr. Maniatis received a BA in biology, an MS in chemistry from the University of Colorado at Boulder, and a Ph.D. in molecular biology from Vanderbilt University.

Terrance G. McGuire has served as a member of Acceleron's board of directors since 2005. Mr. McGuire co-founded Polaris Partners in 1996 and is currently one of their general partners. Prior to starting Polaris Partners, Mr. McGuire spent seven years at Burr, Egan, Deleage, & Co., investing in early stage medical and information technology companies. He serves on the board of directors of Adimab/Arsanis, Aero Designs/Wiki Cells, Arsenal Medical/480 Biomedical, Iora Health, Ironwood Pharmaceuticals, Life Line Screening, MicroCHIPS, NextCode, Pulmatrix, SustainX, and Trevena. He has served on the board of directors of numerous other companies, including Remon Medical Technologies, GlycoFi, Akamai Technologies, Aspect Medical Systems, Cubist Pharmaceuticals, Transform Pharma, and deCODE genetics. Mr. McGuire is the former chairman of

the National Venture Capital Association, chairman of the board of the Thayer School of Engineering at Dartmouth College, and a member of the boards of The David H. Koch Institute for Integrative Cancer Research at the Massachusetts Institute of Technology and The Arthur Rock Center for Entrepreneurship at Harvard Business School. Mr. McGuire received a BS in physics and economics from Hobart College, an MS in engineering from The Thayer School at Dartmouth College, and an MBA from Harvard Business School.

Richard F. Pops has served as a member of Acceleron's board of directors since 2004. Since 2011, Mr. Pops has served as Chief Executive Officer and Chairman of the board of Alkermes plc, the parent company of Alkermes. From 2009 to 2011, Mr. Pops served as Chief Executive Officer and Chairman of the Board of Alkermes, from 2007 to 2009 he served as the Chairman of the board of Alkermes, and from 1991 through 2007 he served as the Chief Executive Officer of Alkermes. Mr. Pops also serves on the board of directors of Neurocrine Biosciences, Inc., Epizyme Inc., the Biotechnology Industry Organization (BIO), and Pharmaceutical Researcher and Manufacturers of America (PhRMA). He has previously served on the board of directors of Sirtris Pharmaceuticals from 2004 to 2008, and CombinatoRx, Inc. from 2001 to 2009. Mr. Pops also served on the board of directors of Reliant Pharmaceuticals, a privately held pharmaceutical company purchased by GlaxoSmithKline in 2007, and on the advisory board of Polaris Venture Partners. He was a member of the Harvard Medical School Board of Fellows from 2002 through June 2012. Mr. Pops received a BA in economics from Stanford University.

Joseph S. Zakrzewski has served as a member of Acceleron's board of directors since 2011. Since 2010, Mr. Zakrzewski has been Executive Chairman and Chief Executive Officer of Amarin Corporation. From 2007 to 2010, Mr. Zakrzewski served as President and Chief Executive Officer of Xcellerex. From 2005 to 2007, Mr. Zakrzewski served as the Chief Operating Officer of Reliant Pharmaceuticals, overseeing the launch of Omacor®, a drug to treat elevated triglyceride levels. From 1988 to 2004, Mr. Zakrzewski served in a variety of positions at Eli Lilly and Company including as Vice President, Corporate Business Development from 2003 through 2004. In addition, Mr. Zakrzewski served as a Venture Partner with OrbiMed in 2010 and 2011. Mr. Zakrzewski also currently serves on the board of directors of Amarin and Insulet Corporation and has also served on the board of directors of Xcellerex, Azelon/Zelos Therapeutics and Promedior. Mr. Zakrzewski received a BS in Chemical Engineering and an MS in Biochemical Engineering from Drexel University, as well as an MBA in Finance from Indiana University.

Source: Company website

FIGURE 32. Income Statement, Acceleron Pharma

Income Statement ($MM)	1Q13E	2Q13E	3Q13E	4Q13E	2013E	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E
Product Sales and Royalties:																		
Sotatercept / ACE-536 Royalty Revenue											-	3.3	34.7	123.9	252.5	429.8	583.0	743.4
Dalantercept											-	-	-	41.9	90.1	213.8	424.3	688.9
Total Product Sales and Royalties	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	3.3	34.7	165.8	342.6	643.5	1,007.3	1,432.3
Collaboration Revenue:																		
Licensing and milestones	12.5	22.9	0.0	0.0	35.4	0.0	7.0	0.0	15.0	22.0	17.5	60.0	50.0	100.0	50.0	0.0	90.0	0.0
Cost-sharing, net	2.5	3.5	4.3	4.7	15.0	4.9	5.2	5.4	5.9	21.4	25.4	28.0	30.8	33.8	37.2	40.9	45.0	49.5
Contract Manufacturing																		
Total Revenue	15.0	26.4	4.3	4.7	50.5	4.9	12.2	5.4	20.9	43.4	42.9	91.2	115.4	299.6	429.8	684.5	1,142.3	1,481.8
Cost of Goods Sold														4.2	8.1	19.2	37.4	59.7
Gross Profit	15.0	26.4	4.3	4.7	50.5	4.9	12.2	5.4	20.9	43.4	42.9	91.2	115.4	295.4	421.7	665.2	1,104.9	1,422.1
Operating Expenses:																		
Research and Development	8.8	8.9	9.1	9.3	36.1	9.6	9.8	10.0	10.3	39.7	57.5	69.0	79.3	87.3	95.1	103.7	113.0	123.2
General and administrative	3.1	3.4	3.8	3.9	14.2	4.1	4.3	4.6	4.9	17.9	19.7	21.7	30.4	34.9	38.8	41.9	44.8	47.7
Cost of contract manufacturing revenue																		
Total operating expenses	11.9	12.3	12.9	13.2	50.3	13.7	14.1	14.6	15.2	57.6	77.2	90.7	109.7	122.2	133.9	145.6	157.9	170.9
Operating income (loss)	3.1	14.2	(8.6)	(8.5)	0.2	(8.8)	(2.0)	(9.2)	5.8	(14.2)	(34.3)	0.5	5.7	173.2	287.7	519.6	947.0	1,251.2
Other income (expense):																		
Other expense, net	(1.1)	(0.4)	0.0	0.0	(1.3)	0.0	0.0	0.0	0.0	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2
Interest income	0.0	0.0			0.0													
Interest expense	(0.4)	(0.7)			(1.2)													
Total other income, net	(1.5)	(1.1)	0.0	0.0	(2.5)	0.0	0.0	0.0	0.0	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2
Pretax income (loss)	1.6	13.1	(8.5)	(8.5)	(2.3)	(8.7)	(1.9)	(9.1)	5.8	(14.0)	(34.1)	0.7	5.8	173.3	287.9	519.8	947.2	1,251.3
Income tax benefit (provision)										2.1	8.5	(0.2)	(2.0)	(60.7)	(100.8)	(181.9)	(331.5)	(438.0)
Tax Rate										*15%*	*25%*	*30%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
Comprehensive income (loss)	1.647	13.1	(8.5)	(8.5)	(2.3)	(8.7)	(1.9)	(9.1)	5.8	(14.0)	(25.6)	0.5	3.8	112.7	187.1	337.9	615.7	813.4
Pro forma net income (loss) applicable to	2.7	10.1	(8.5)	(8.5)	(4.2)	(8.7)	(1.9)	(9.1)	5.8	(14.0)	(25.6)	0.5	3.8	112.7	187.1	337.9	615.7	813.4
Pro forma Basic EPS to common shareholders	$ 0.13	$ 0.48	$ (0.36)	$ (0.32)	$ (0.17)	$ (0.33)	$ (0.07)	$ (0.34)	$ 0.22	$ (0.53)	$ (0.95)	$ 0.02	$ 0.13	$ 3.90	$ 6.32	$ 11.12	$ 19.79	$ 20.22
Pro forma Diluted EPS to common shareholders	$ 0.12	$ 0.44	$ (0.36)	$ (0.32)	$ (0.17)	$ (0.33)	$ (0.07)	$ (0.34)	$ 0.14	$ (0.53)	$ (0.95)	$ 0.01	$ 0.10	$ 2.85	$ 4.64	$ 8.22	$ 14.68	$ 15.92
Basic shares outstanding	20.5	21.0	23.7	26.4	24.3	26.4	26.5	26.6	26.6	26.6	26.9	27.5	28.2	28.9	29.6	30.4	31.1	40.2
Diluted shares outstanding	22.2	23.1	23.7	26.4	24.3	26.4	26.5	26.6	40.6	26.6	26.9	38.8	38.7	39.5	40.3	41.1	41.9	51.1

Source: Acceleron Pharma and JMP Securities LLC

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 TGFβ signaling pathway. These fusion proteins have shown clinical potential in the treatment of anemia disorders related to β-thalassemia and myelodysplastic syndromes, as well as in the treatment of solid cancers, muscle wasting disorders, and other indications impacted by dysregulated TGFβ.

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 ≥20% royalty on worldwide sales, by our estimates. Sotatercept and ACE-536 are currently in Phase II trials for the treatment of β-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 to the company's share price.

Financial. Following its IPO, we estimate that Acceleron will end 4Q13 with approximately $87MM in cash and cash equivalents - adequate resources to fund operations into 2015, according to Acceleron 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

Analyst Certification:

The research analyst(s) who prepared this report does/do hereby certify that the views presented in this report are in accordance with my/our personal views on the securities and issuers discussed in this report. As mandated by SEC Regulation AC no part of my/our compensation was, is or will be directly or indirectly related to the specific views or recommendations expressed herein. This certification is made under the obligations set forth in SEC Regulation AC. Any other person or entity may not use it for any other purpose. This certification is made based on my/our analysis on the date of this report's publication. I/We assume no obligation to update this certification to reflect any facts,circumstances or events that may subsequently come to my/our attention. Signed Michael G. King

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JMP Securities currently makes a market in the securities of Acceleron Pharma Inc., Alnylam Pharmaceuticals, Inc., Astex Pharmaceuticals, Inc., AVEO Pharmaceuticals, Inc., Biogen Idec Inc., Celgene Corporation, Epizyme, Inc., Infinity Pharmaceuticals, Inc., Regeneron Pharmaceuticals, Inc., Synta Pharmaceuticals Corp., Threshold Pharmaceuticals, Inc. and ZIOPHARM Oncology, Inc.

JMP Securities was manager or co-manager of a public offering for Acceleron Pharma Inc., Alnylam Pharmaceuticals, Inc. and Epizyme, Inc. in the past 12 months.

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.
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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	244	*61.31%*	Buy	244	*61.31%*	75	*30.74%*
MARKET PERFORM	Hold	148	*37.19%*	Hold	148	*37.19%*	22	*14.86%*
MARKET UNDERPERFORM	Sell	6	*1.51%*	Sell	6	*1.51%*	0	*0%*
TOTAL:		398	*100%*		398	*100%*	97	*24.37%*

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.

In order to obtain these (6 or more) stock price charts or additional applicable disclosures and information concerning JMP's recommendations of companies under coverage mentioned in this report, please contact JMP Securities at (877) 263-1333 or visit www.jmpsecurities.com

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