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OUTPERFORM

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LEERINK

Reason for report:

COMPANY UPDATE

ACCELERON PHARMA

Very Encouraging New SOT/ACE-536 Data in MDS & TD β-Thal; Reit OP & $52PT

• **Bottom Line:** Top-line data in European Hematology Association (EHA) abstracts released today show very encouraging new data for ACE-536 in MyeloDysplastic Syndrome (MDS) patients and new data for Sotatercept (SOT)/ACE-536 in Transfusion Dependent (TD) β-Thalassemia (β-Thal.). In MDS, ACE-536 is demonstrating impressive efficacy (in a small trial) across both TD and Non-Transfusion-Dependent (NTD) patients. In β-Thal., NTD data for both SOT-ACE-536 build upon previous updates and are maturing nicely. New for β-Thal. Is that 100% (4/4) of ACE-536 treated TD patients are showing reductions in transfusion requirements while respective data for SOT will be presented at the meeting. These data demonstrate solid management execution in these programs (i.e., achieving >2g/dL Hgb increases in NTD patients) and while they suggest efficacy goals are already being met further dose escalation is continuing in order to provide additional dosing information. We reiterate our Outperform rating and $52 price target (PT).

• **New (presented for the first time) ACE-536 MDS data demonstrate impressive efficacy across both TD and NTD patients (EHA presentation 6.15.14; 8:45 CEST).** Emerging efficacy data are available for 15 patients (5 NTD, 10 TD) treated in the first 4 cohorts (0.125, 0.25, 0.5, or 0.75mg/kg). For TD patients, 40% (4/10) experience a ≥50% reduction in transfused units during an 8-week treatment interval vs. the 8 weeks prior to treatment with 1 patient who was previously ESA and lenalidomide non-responsive being transfusion-free while on study (~22 weeks). 5 NTD patients showed dose-dependent hemoglobin (Hgb) increased ranging from 0.8-3.3g/dL. For NTD patients 60% (3/5) in the 0.75mg/kg were either erythropoietin-stimulating agent (ESA) refractory or ESA non-responders and showed Hgb increases of 1.6, 1.9, and 3.3g/dL with 1 of these patients experiencing a ≥1.5g/dL sustained response for ~15 weeks. Additional data at EHA will include TD transfusion burden for patients treated at 1.0mg/kg.

• **New for β-Thal. is that 100% (4/4) of ACE-536 treated TD patients are showing reductions in transfusion requirements while respective data for SOT will be presented at the meeting (EHA presentations 6.14.14; 8:15/8:45 CEST).** So far, available efficacy data for 4/4 TD patients treated at 0.6 or 0.8mg/kg have experienced reductions in transfusion requirements. Key at EHA will be data for all patients at all 4 dose groups through Day 112 (5 cycles+7 days) and data in NTD patients treated at 0.8mg/kg. For SOT in TD β-Thal. transfusion burden changes will be presented for 0.1, 0.3. 0.5 and 0.75mg/kg groups.

• **New in Sickle Cell Disease (SCD) will be a positive ACE-536 preclinical poster (EHA presentation 6.13.14; 5:45-7PM CEST).** These data demonstrate that after 1 month of treatment, RAP-536 (a murine version of ACE-536) significantly increased red blood cell (RBC) numbers, Hgb and reduced reticulocytes vs. placebo in SCD mice.

Key Stats:	**(NASDAQ:XLRN)**
S&P 600 Health Care Index:	**1,234.31**
Price:	**$31.76**
Price Target:	$52.00
Methodology:	DCF analysis; 10% discount rate; 1% terminal growth rate
52 Week High:	$57.89
52 Week Low:	$15.00
Shares Outstanding (mil):	30.3
Market Capitalization (mil):	$962.3
Book Value/Share:	$1.32
Cash Per Share:	$7.06
Dividend (ann):	$0.00
Dividend Yield:	0.0%
Cash Per Share:	

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	$15.0	$26.4	$4.3	$11.5	$57.2	$0.13	$0.64	($0.66)	($0.64)	($4.15)	NM
2014E	$3.3A	0.0	$30.0	0.0	$33.3	($0.30)A	($0.55)	$0.39	($0.60)	($1.06)	NM
2015E	--	--	--	--	0.0	--	--	--	--	($2.29)	NM

Source: Company Information and Leerink Partners LLC Research
Revenue in MM.
GAAP EPS presented.

INVESTMENT THESIS

We rate XLRN Outperform. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). XLRN has multiple significant data read-out catalysts during almost every quarter until YE14. Pivotal catalysts through 2014: (1) Preliminary Dalantercept Phase II RCC data in 2Q14; (2) top-line Sotatercept and ACE-536 Phase II MDS and β-Thal. (4 trials) data at EHA in 2Q14; (3) final Sotatercept and ACE-536 Phase II MDS and β-Thal. (4 trials) data at ASH in 4Q14; (4) initiate pivotal MDS and/or β-Thal. trials by YE14. MEDACorp KOLs are very bullish and encouraged by emerging pipeline data and science. We assume probability of success in the low 30s percent range for Sotatercept/ACE-536 in MDS, 40% for β-Thal., 20% in end-stage renal disease (ESRD) patients on hemodialysis, and low 30s percent range for Dalantercept in 2nd-line RCC.

ASCO 2014 Titles

Dalantercept:				
Abstract #	Title	Time/Session	Poster Board	Presenter
5594	Phase II evaluation of dalantercept, a soluble recombinant activin receptor-like kinase 1 (ALK1) receptor-fusion protein, for treatment of recurrent/persistent endometrial cancer: GOG-0229N.	5/31(Sat) 8-11:45am S Hall A2 General poster Track(s): Gynecologic Cancer	376	Vicky Makker, MD
6045	Phase 2 study of dalantercept in recurrent or metastatic squamous cell carcinoma of the head and neck	5/31 (Sat) 1:15-5pm S Hall A2 General poster Track(s): Head and Neck Cancer	80	Antonio Jimeno, MD, PhD
4566	**A two-part phase 2 randomized study of dalantercept and axitinib versus placebo plus axitinib in advanced renal cell carcinoma: Results from the part 1 dose escalation cohorts.**	**6/2 (Mon) 1:15-5pm S Hall A2 General Poster Track(s): Genitourinary Cancer**	**134**	**Michael B. Atkins, MD**

Source: ASCO.org, Leerink Partners estimates

Milestones

Product	Partner	Indication	Phase	Timing	Milestone
ACE-536	CELG	MDS + β-Thal.	Ph. II	**2Q14**	**Phase II dose escalation data for MDS + β-Thal. at EHA-2014**
				4Q14	**Final Phase II in MDS and β-Thal. data**
				YE14/ early-15	Initiate Phase III trial for MDS and/or β-Thal.
				2018	Approval and launch
Sotatercept (ACE-011)				**2Q14**	**Phase II dose escalation β-Thal. data at EHA-2014 +** Preclinical data in sickle cell anemia
				4Q14	**Final Phase II in MDS + β-Thal. data**
				2018	Approval and launch
				YE14/early-15	Initiate Phase III trial for MDS and/or β-Thal.
	CELG	ESRD	Ph. II	**11.11-16.14**	**Phase II Part-1 data (0.7mg/kg cohort+BMD/Vasc Calcificat)**
Dalantercept (ACE-041)	Proprietary	Oncology	Ph. II	**early-June -14**	**Interim Part-1 Phase II RCC combo data (ASCO-2014)**
				2Q14	Initiate Phase II Dal-sorafenib combo trial in HCC
				YE14	Preliminary Phase II Dal-sorafenib combo RR data in HCC
				2H15	**Phase II RCC Part-2 data**
				2014	Phase II data in SCCHN
				2018	Approval and launch in RCC
ACE-083		Muscle	PC	2H14	Advance ACE-083 into clinic for Muscle Loss
New TGF-β Candidates		Fibrosis	PC	2015	Advance Fibrosis (i.e., PAH) candidate into clinic

Source: Company reports, Leerink Partners estimates

VALUATION

Our $52 12-month price target of XLRN shares reflects probability-adjusted royalty revenue at 20% from ESRD patients on hemodialysis. Our valuation is based on a discounted cash flow analysis. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). We apply a discount rate of 10% and a terminal growth rate of 1%, which translates to an 11x terminal multiple, which we believe is comparable to biotechnology companies in a similar development stage.

RISKS TO VALUATION

An investment in XLRN is fundamentally a high-risk, high-reward investment, in our opinion. XLRN may face significant clinical, regulatory, and commercial risks for pipeline products. Most important is clinical risk for Phase II Sotatercept and ACE-536 trials in MDS (myelodysplastic syndromes) and β-Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS, β-Thal., and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.

XLRN P&L ($000s, except per share data)	2013A	1Q14A	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E
Revenues																		
Sotatercept/ACE-536 WW Revenue in MDS to CELG										$66,089	$141,589	$227,505	$324,937	$435,091	$559,283	$698,955	$855,680	$1,031,180
Probability of Success										*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue										$21,148	$45,308	$72,801	$103,980	$139,229	$178,971	$223,666	$273,818	$329,978
Risk Adjusted Sotatercept/ACE-536 WW Royalties in MDS										**$4,230**	**$9,515**	**$16,016**	**$23,915**	**$32,023**	**$42,953**	**$55,916**	**$68,454**	**$82,494**
Sotatercept/ACE-536 WW Revenue in NTD β-Thal. to CELG										$3,659	$60,642	$127,092	$226,546	$339,098	$466,097	$609,020	$767,222	$904,639
Probability of Success										*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*	*40%*
Risk Adjusted Sotatercept/ACE-536 WW Revenue in NTD β-Thal.										$1,464	$24,257	$50,837	$90,619	$135,639	$186,439	$243,608	$306,889	$361,856
Risk Adjusted Sotatercept/ACE-536 WW Royalties in NTD β-Thal.										**$293**	**$4,851**	**$10,676**	**$19,936**	**$29,841**	**$42,881**	**$58,466**	**$73,653**	**$90,464**
Dalantercept WW Revenue in 2nd-line RCC										$68,061	$131,647	$210,325	$298,864	$398,173	$509,233	$633,101	$770,918	$877,863
Probability of Success										*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*	*32%*
Risk Adjusted Dalantercept WW Revenue in 2nd-line RCC										**$21,780**	**$42,127**	**$67,304**	**$95,637**	**$127,415**	**$162,954**	**$202,592**	**$246,694**	**$280,916**
Sotatercept US Revenue in ESRD Patients on Hemodialysis												$301,866	$819,107	$1,418,781	$1,915,379	$2,064,199	$2,220,226	$2,383,756
Probability of Success												*20%*	*20%*	*20%*	*20%*	*20%*	*20%*	*20%*
Risk Adjusted Sotatercept US Revenue in ESRD Patients on Hemodialysis												$60,373	$163,821	$283,756	$383,076	$412,840	$444,045	$476,751
Risk Adjusted Sotatercept US Royalties in ESRD Patients on Hemodialysis												**$12,075**	**$36,041**	**$65,264**	**$91,938**	**$103,210**	**$111,011**	**$119,188**
Collaboration Revenue	$57,231	$3,307	-	$30,000	-	$33,307	-	$40,000	$30,000	$22,400	$8,000	-	$8,000	-	$8,000	$8,000	$8,000	$8,000
Total Revenue	**$57,231**	**$3,307**	**-**	**$30,000**	**-**	**$33,307**	**-**	**$40,000**	**$30,000**	**$48,702**	**$64,493**	**$106,071**	**$183,529**	**$254,543**	**$348,727**	**$428,184**	**$507,813**	**$581,062**
Costs and Expenses																		
Probability Adjusted Dalantercept COGS	-					-	-	-	-	$3,267	$6,319	$10,096	$9,564	$12,742	$16,295	$20,259	$24,669	$28,092
Research and Development	$36,051	$11,765	$12,200	$12,500	$13,280	$49,745	$55,217	$61,291	$68,033	$34,016	$35,717	$37,503	$39,378	$41,347	$43,415	$45,585	$47,864	$50,258
SG&A (Risk Adjusted from Time of Dalantercept Launch)	$14,227	$3,750	$4,500	$5,100	$5,200	$18,550	$20,405	$22,446	$24,690	$31,090	$34,199	$36,935	$38,782	$40,721	$42,757	$44,895	$47,139	$49,496
Total Costs and Expenses	**$50,278**	**$15,515**	**$16,700**	**$17,600**	**$18,480**	**$68,295**	**$75,622**	**$83,736**	**$92,723**	**$68,373**	**$76,235**	**$84,534**	**$87,724**	**$94,810**	**$102,467**	**$110,739**	**$119,673**	**$127,846**
Operating Income (EBIT)	**$6,952**	**($12,208)**	**($16,700)**	**$12,400**	**($18,480)**	**($34,988)**	**($75,622)**	**($43,736)**	**($62,723)**	**($19,671)**	**($11,742)**	**$21,537**	**$95,805**	**$159,733**	**$246,260**	**$317,445**	**$388,140**	**$453,217**
Y/Y growth																		
Other Income (Expenses)	($27,710)	$3,088				-	-	-	-	-	-	-	-	-	-	-	-	-
Interest Income	$20																	
Interest Expense	($1,161)		($378)	($298)	($219)	($895)	($521)	-	-	-	-	-	-	-	-	-	-	-
Income Before Taxes	**($21,898)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($19,671)**	**($11,742)**	**$21,537**	**$95,805**	**$159,733**	**$246,260**	**$317,445**	**$388,140**	**$453,217**
Provision for Taxes						-	-	-	-	-	-	-	-	-	29,317	107,931	131,967	154,094
Tax Rate						0%	0%	0%	0%	0%	0%	0%	0%	0%	12%	34%	34%	34%
Net income	**($21,898)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($19,671)**	**($11,742)**	**$21,537**	**$95,805**	**$159,733**	**$216,942**	**$209,514**	**$256,172**	**$299,123**
Net income (loss) applicable to common stockholders—diluted	**($39,003)**	**($9,120)**	**($17,078)**	**$12,102**	**($18,699)**	**($32,795)**	**($76,143)**	**($43,736)**	**($62,723)**	**($19,671)**	**($11,742)**	**$21,537**	**$95,805**	**$159,733**	**$216,942**	**$209,514**	**$256,172**	**$299,123**
Change in fair value of warrants	$1,500																	
EPS (LPS) Basic	**($4.15)**	**($0.30)**	**($0.55)**	**$0.39**	**($0.60)**	**($1.06)**	**($2.29)**	**($1.30)**	**($1.85)**	**($0.57)**	**($0.34)**	**$0.62**	**$2.72**	**$4.48**	**$6.03**	**$5.76**	**$6.98**	**$8.07**
Basic Shares (000)	**9,407**	**30,321**	**31,140**	**31,171**	**31,202**	**30,931**	**33,241**	**33,573**	**33,909**	**34,248**	**34,590**	**34,936**	**35,286**	**35,639**	**35,995**	**36,355**	**36,719**	**37,086**

Source: Leerink Partners estimates and company reports.
NTD=non-transfusion dependent.

DCF Calcuation

Discount rate	*10%*
Terminal Growth Rate	*1%*
Valuation ($M)	$1,668
Valuation / Share	**$52**

Source: Leerink Partners estimates.

XLRN DCF Valuation/Share Sensitivity Analysis

Terminal Growth Rate \ Discount Rate	8.0%	9.0%	10.0%	11.0%	12.0%
0.0%	$69	$57	$48	$41	$36
1.0%	$76	$62	**$52**	$44	$38
2.0%	$86	$69	$57	$47	$40
3.0%	$99	$78	$62	$51	$43
4.0%	$120	$90	$70	$57	$47

Source: Leerink Partners estimates.

Disclosures Appendix

Analyst Certification

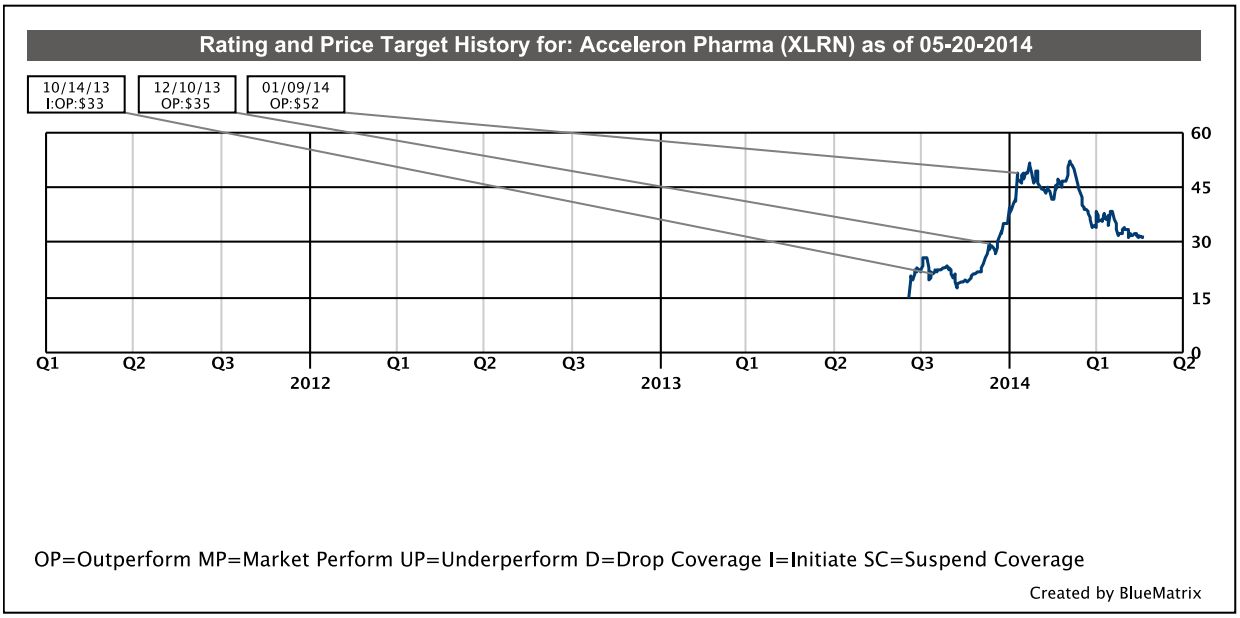
I, Marko Kozul, M.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

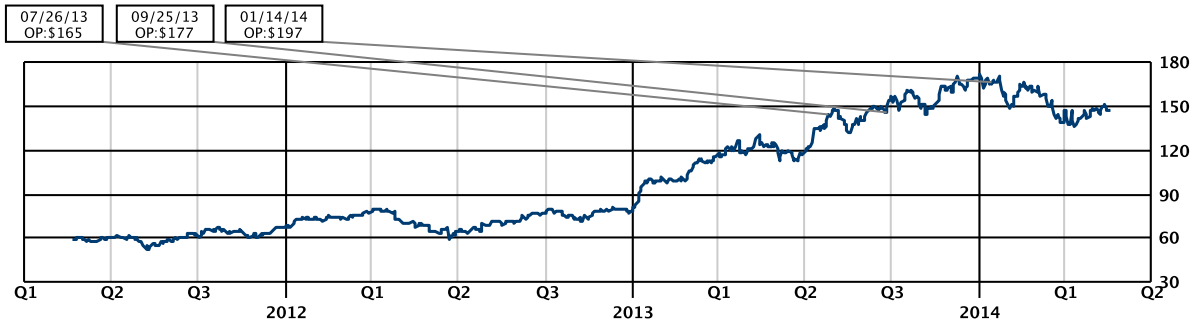
Our $52 12-month price target of XLRN shares reflects probability-adjusted royalty revenue at 20% from ESRD patients on hemodialysis. Our valuation is based on a discounted cash flow analysis. We believe XLRN shares are poised to appreciate near/longer term driven by progress with CELG-partnered compounds Sotatercept/ACE-536 and proprietary Dalantercept (ACE-041). We apply a discount rate of 10% and a terminal growth rate of 1%, which translates to an 11x terminal multiple, which we believe is comparable to biotechnology companies in a similar development stage.

Risks to Valuation

An investment in XLRN is fundamentally a high-risk, high-reward investment, in our opinion. XLRN may face significant clinical, regulatory, and commercial risks for pipeline products. Most important is clinical risk for Phase II Sotatercept and ACE-536 trials in MDS (myelodysplastic syndromes) and β-Thal. as well as Dalantercept/Axitinib in RCC. There is also competitive risk from emerging MDS, β-Thal., and RCC therapies. Finally, XLRN may face financing risk beyond 1H15.



Rating and Price Target History for: Celgene, Inc. (CELG) as of 05-20-2014



Leerink Swann initiated coverage of CELG with an Outperform rating on February 7, 2003. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

Distribution of Ratings/Investment Banking Services (IB) as of 03/31/14				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	131	68.23	46	35.11
HOLD [MP]	61	31.77	3	4.92
SELL [UP]	0	0.00	0	0.00

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months.The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

Important Disclosures

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Editorial Department at One Federal Street, 37th Floor, Boston, MA 02110.

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In the past 12 months, the Firm has received compensation for providing investment banking services to Acceleron Pharma .

Leerink Partners LLC makes a market in Acceleron Pharma and Celgene, Inc.

Leerink Partners LLC has acted as the manager for a public offering of Acceleron Pharma in the past 12 months.

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