COWEN
AND COMPANY

Biotechnology

Zafgen

Equity Research

Earnings Update

August 14, 2014

Price: $17.83 (08/13/2014)

Price Target: $45.00

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: ZFGN
52-Week Range:	$21.01 - 17.06
Market Cap (MM):	$388.8
Net Debt (MM):	$(126.8)
Cash/Share:	$5.59
Dil. Shares Out (MM):	22.7
Enterprise Value (MM):	$261.2
ROIC:	NA
ROE (LTM):	NA
BV/Share:	$5.52
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	-	$(0.98)A	$(0.56)
Prior Q1	-	-	
Q2	-	$(2.96)A	$(0.58)
Prior Q2	-	*$(0.43)*	
Q3	-	$(0.86)	$(0.62)
Prior Q3	-	*$(0.84)*	
Q4	-	$(0.53)	$(0.63)
Prior Q4	-	*$(0.48)*	
Year	$(3.11)	$(3.25)	$(2.40)
Prior Year	-	*$(2.50)*	*$(2.45)*
P/E	NM	NM	NM
Revenue (MM)			
Year	$0.0	$0.0	$0.0

Reports Q2:Pipeline On-Track For A Data Rich 2015

The Cowen Insight

Zafgen provided Q2 financials and a pipeline update. Zafgen is on track to initiate Ph. III studies of beloranib in Prader-Willi Syndrome (PWS) in the U.S. during Q3 and in the EU in H1:15 with initial data expected in Q4:15. Beloranib is also in a Ph. IIa in craniopharyngioma-associated obesity with data anticipated in Q1:15. We expect beloranib's progress to drive outperformance in ZFGN.

Q2 Financials: Funding Into 2016 Through Multiple Milestones.

Zafgen reported a Q2 net loss of $6.4MM and ended the quarter with $134.2MM in cash following its recent IPO. Zafgen expects to end 2014 with greater than $95MM in cash and anticipates that its current cash balance will fund operations into 2016 through key pipeline milestones including results from beloranib's Ph. III studies in PWS and Ph. II trials in craniopharyngioma and severe obesity.

Beloranib's Ph. III In PWS To Begin In The U.S By End Of Q3:14 And In The EU By H1:15.

After meeting with the FDA and EMA, Zafgen expects to initiate a U.S. Ph. III trial in PWS by the end of Q3 followed by a Ph. III trial in EU in H1:15. Patients will receive beloranib at 1.8 mg or 2.4 mg or placebo via sub-Q injections twice-weekly for six months. Primary endpoints will evaluate improvements in total body fat mass and hyperphagia-related behaviors. Each trial will be considered positive if either endpoint is met with $p < 0.025$ or if both are met with $p < 0.05$. Zafgen anticipates that initial six-month data from the U.S. trial will be available by Q4:15.

Interim Data From Beloranib's Ph. IIa In Hypothalamic Injury Associated Obesity Expected In Q1:15.

Zafgen noted that the 14-patient Ph. IIa trial in hypothalamic injury associated obesity has achieved >50% enrollment and is on track for data in Q1:15. Hypothalamic injury in the trial is defined as patients with craniopharyngioma, stroke, brain trauma, or radiation injuries to the hypothalamus. Zafgen expects the majority of the enrollment to consist of craniopharyngioma patients. The primary endpoint will evaluate the change in body weight from baseline.

Beloranib's Ph. IIb In Severe Obesity On Track To Begin In Late 2014.

Zafgen plans to initiate a long-term Phase IIb study of beloranib in obesity in late 2014. Prior Ph. IIa data showed treatment with beloranib resulted in mean weight changes from baseline of 7 kg to 11 kg with favorable trends in CV parameters. Zafgen anticipates that preliminary six-month data will be available in Q4:15.

IND Filing For ZGN-839 On Track For H1:15

Zafgen is developing an orally-active second-generation MetAP2 inhibitor, ZGN-839. ZGN-839 has shown early efficacy in preclinical models of Nonalcoholic Steatohepatitis (NASH) and type 2 diabetes. Zafgen is conducting PK and PD studies in preclinical models and plans to submit an IND in H1:15.

At A Glance

Our Investment Thesis

Zafgen's lead asset beloranib is in development for the treatment of hyperphagia (insatiable appetite) and obesity in Prader-Willi syndrome, craniopharyngioma-associated obesity, and severe obesity in the general population. Zafgen has completed five clinical trials in over 200 subjects including obese volunteers and Prader-Willi Syndrome patients. In these trials beloranib has reduced fat mass and controlled hyperphagia while maintaining an acceptable tolerability and safety profile. Our consultants find the >50% decrease in hyperphagia produced in PWS patients particularly striking, as beloranib is the first agent shown to produce a reduction in food seeking behavior. Beloranib will enter a Phase III trial in Prader-Willi syndrome during H2:14, with initial data possible in Q4:2015. Our DCF suggests that Zafgen is undervalued based on beloranib's potential in Prader-Willi and craniopharyngioma alone, with no contribution from other indications or pipeline programs.

Forthcoming Catalysts

- Initiate Phase III trial of beloranib in Prader-Willi in U.S., H2:14
- Initiate Phase IIb trial of beloranib in severe obesity, H2:14
- Initial data from Ph. IIa of beloranib in craniopharyngioma, Q1:15
- Initial data from U.S. Ph. III trial of beloranib in Prader-Willi, Q4:15

Base Case Assumptions

- Beloranib is successfully developed for Prader-Willi and craniopharyngioma, achieving $1.0B in sales by 2026.
- Beloranib is not developed for any other indications.
- The rest of Zafgen's pipeline does not contribute significant value.

Upside Scenario

- Beloranib is successfully developed for Prader-Willi and craniopharyngioma, achieving >$1.0B in sales by 2026.
- Beloranib is also successfully developed for other indications.
- ZGN-839 is successfully developed for NASH and/or type 2 diabetes
- Another pipeline candidate contributes significant value.

Downside Scenario

- Beloranib is not successfully developed for Prader-Willi and/or craniopharyngioma.
- Beloranib does not achieve $1.0B in sales by 2026.
- The rest of Zafgen's pipeline does not contribute much value.

Price Performance

Source: Bloomberg

Company Description

Zafgen is a biopharmaceutical company dedicated to improving the health and well-being of patients affected by obesity. Zafgen's beloranib is a novel small molecule inhibitor of methionine aminopeptidase 2 (MetAP2). In addition to a Phase III trial of beloranib in Prader-Willi, Zafgen will also initiate a Phase IIb trial in general obesity during H2:14, with data possible in Q4:15. A Phase IIa trial in craniopharygioma-associated obesity is ongoing, with data expected during Q1:15. Prader-Willi syndrome and craniopharyngioma-associated obesity are orphan disorders that each afflict approximately 20K patients in the U.S. and EU. We estimate that beloranib could address a $1.5B+ opportunity in these two conditions. Behind beloranib, Zafgen is developing an orally active MetAP2 inhibitor, ZGN-839. ZGN-839 has shown early efficacy in preclinical models of Nonalcoholic Steatohepatitis (NASH) and type 2 diabetes. Zafgen plans to submit an IND for ZGN-839 in H1:15.

Analyst Top Picks

	Ticker	Price (08/13/2014)	Price Target	Rating
BioMarin Pharmaceutical	BMRN	$65.81	$95.00	Outperform
Gilead Sciences	GILD	$93.98	$95.00	Outperform
Portola Pharmaceuticals	PTLA	$26.82	$45.00	Outperform

Investment Thesis

Zafgen is a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity. Zafgen's lead asset, beloranib, is a novel small molecule inhibitor of methionine aminopeptidase 2 (MetAP2) that is in development for the treatment of hyperphagia (insatiable appetite) and obesity in Prader-Willi Syndrome, craniopharyngioma-associated obesity, and severe obesity in the general population. Zafgen has completed five clinical trials in over 200 subjects including obese volunteers and Prader-Willi Syndrome patients. In these trials beloranib has reduced fat mass and controlled hyperphagia while maintaining an acceptable tolerability and safety profile. Our consultants find the >50% decrease in hyperphagia produced in Prader-Willi patients particularly striking, as beloranib is the first agent shown to produce a reduction in food seeking behaviors in these patients, a key area of unmet need. Beloranib will enter a Phase III trial in Prader-Willi Syndrome during H2:14, with initial data possible in Q4:2015. Zafgen will also initiate a Phase IIb trial in general obesity, with data possible in Q4:2015. A Phase IIa trial in craniopharyngioma-associated obesity is ongoing, with data expected during Q1:2015. Prader-Willi Syndrome and craniopharyngioma-associated obesity are orphan disorders that each afflict approximately 20K patients in the U.S. and EU. We estimate that beloranib could address a $1.5B+ opportunity in these two conditions. Behind beloranib, Zafgen is developing an orally active second-generation MetAP2 inhibitor, ZGN-839. ZGN-839 has shown early efficacy in preclinical models of Nonalcoholic Steatohepatitis (NASH) and type 2 diabetes. Zafgen plans to submit an IND for ZGN-839 in H1:15. Our DCF analysis suggests that Zafgen is undervalued based on beloranib's potential in Prader-Willi and craniopharyngioma-associated obesity alone, with no contribution from other indications or pipeline programs. We maintain our Outperform rating and $45 price target.

Zafgen Upcoming Milestones

Milestone	Timing
Initiate Phase III trial of beloranib in PWS in U.S.	H2:14
Initiate Phase IIb trial of beloranib in severe obesity	H2:14
Initial data from Phase IIa of beloranib in craniopharyngioma-associated obesity	Q1:15
Initiate Phase III trial of beloranib in PWS in EU	H1:15
File IND for ZGN-839 in type II diabetes, NASH and abdominal obesity	H1:15
Interim 6-month Phase IIb data from beloranib in severe obesity (complete Phase IIb; development decision point)	Q4:15
Phase III data from beloranib in PWS in U.S.	Q4:15
Nomination of second-generation MetAP2i candidate	2016

Source: Cowen and Company

Zafgen Quarterly P&L ($MM)

	Q1:14A	Q2:14A	Q3:14E	Q4:14E	2014E	Q1:15E	Q2:15E	Q3:15E	Q4:15E	2015E
Boleranib	-	-	-	-	-	-	-	-	-	-
License/milestones revenue	-	-	-	-	-	-	-	-	-	-
Total Revenue	-	-	-	-	-	-	-	-	-	-
COGS	-	-	-	-	-	-	-	-	-	-
R&D	3.3	4.7	17.8	10.2	36.0	10.5	11.0	11.5	11.5	44.5
SG&A	1.2	1.3	1.8	2.0	6.3	2.1	2.2	2.7	3.0	10.0
Other	-	-	-	-	-					-
Operating Expenses	4.5	6.0	19.6	12.2	42.3	12.6	13.2	14.2	14.5	54.5
Operating Income / (Loss)	(4.5)	(6.0)	(19.6)	(12.2)	(42.3)	(12.6)	(13.2)	(14.2)	(14.5)	(54.5)
Interest Income	-	0.0	0.1	0.1	0.2	0.1	0.1	0.1	0.1	0.4
Interest Expenses	(0.0)	(0.4)	(0.0)	(0.0)	(0.5)	(0.4)	(0.4)	(0.4)	(0.4)	(1.6)
Foreign Currency Transaction Gains (Losses), n	0.1	0.0	-	-	0.1	-	-	-	-	-
Pretax net income	(4.5)	(6.4)	(19.5)	(12.1)	(42.5)	(12.9)	(13.5)	(14.5)	(14.8)	(55.7)
Accretion of redeemable convertible preferred st	(0.0)	(0.0)	-	-	-	-	-	-	-	-
Taxes	-	-	-	-	-	-	-	-	-	-
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
GAAP Net Income	(4.5)	(6.4)	(19.5)	(12.1)	(42.5)	(12.9)	(13.5)	(14.5)	(14.8)	(55.7)
GAAP EPS	**$ (0.98)**	**$ (2.96)**	**$ (0.86)**	**$ (0.53)**	**$ (3.25)**	**$ (0.56)**	**$ (0.58)**	**$ (0.62)**	**$ (0.63)**	**$ (2.40)**
Diluted Shares Outstanding (MM)	4.6	2.2	22.7	22.9	13.1	23.0	23.2	23.3	23.4	23.2

Source: Cowen and Company

Zafgen Annual P&L ($MM)

	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Boleranib	-	-	-	-	25.0	95.0	200.0	325.0
License/milestones revenue	-	-	-	-	-	-	-	-
Total Revenue	-	-	-	-	25.0	95.0	200.0	325.0
COGS	-	-	-	-	2.0	6.8	13.0	19.5
R&D	9.6	36.0	44.5	50.0	75.0	60.0	65.0	70.0
SG&A	4.2	6.3	10.0	29.0	40.0	50.0	60.0	75.0
Other	-	-	-	-	-	-	-	-
Operating Expenses	13.8	42.3	54.5	79.0	117.0	116.8	138.0	164.5
Operating Income / (Loss)	(13.8)	(42.3)	(54.5)	(79.0)	(92.0)	(21.8)	62.0	160.5
Interest Income	-	0.2	0.4	0.3	0.8	0.8	0.4	0.9
Interest Expenses	-	(0.5)	(1.6)	(0.8)	(0.4)	-	-	-
Foreign Currency Transaction Gains (Losses), net	(0.2)	0.1	-	-	-	-	-	-
Pretax net income	(14.0)	(42.5)	(55.7)	(79.5)	(91.6)	(21.0)	62.4	161.4
Accretion of redeemable convertible preferred stoc	(0.2)	-	-	-	-	-	-	-
Taxes	-	-	-	-	-	-	-	-
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
GAAP Net Income	(14.2)	(42.5)	(55.7)	(79.5)	(91.6)	(21.0)	62.4	161.4
GAAP EPS	**$ (3.11)**	**$ (3.25)**	**$ (2.40)**	**$ (2.75)**	**$ (3.10)**	**$ (0.70)**	**$ 2.05**	**$ 5.20**
Diluted Shares Outstanding (MM)	4.6	13.1	23.2	28.9	29.5	29.8	30.4	31.0

Source: Cowen and Company

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:
In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:
There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

Zafgen is developing candidates for the treatment of orphan disorders, obesity, and metabolic conditions. The majority of Zafgen's market capitalization is dependent upon the success of lead candidate beloranib. Beloranib's value could be adversely impacted should its clinical trials fail, should the regulatory agencies deny approval, or should its commercial opportunity not materialize as we project. In fact, all of Zafgen's drug candidates face clinical and regulatory risk. With the future development path depending on the evolution of clinical data, future revenue forecasts are uncertain. The commercial outlook for Zafgen's candidates could additionally be altered by safety/efficacy findings, emerging competition, alterations in the medical treatment paradigm, or changes in the pricing environment. Some of Zafgen's projected market exclusivity depends on patents, which are subject to challenge by generic drugmakers.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
BMRN	BioMarin Pharmaceutical
GILD	Gilead Sciences
PTLA	Portola Pharmaceuticals
ZFGN	Zafgen

Analyst Certification

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Cowen and Company, LLC and or its affiliates make a market in the stock of Zafgen, BioMarin Pharmaceutical, Gilead Sciences and Portola Pharmaceuticals securities.

Zafgen and Portola Pharmaceuticals have been client(s) of Cowen and Company, LLC in the past 12 months.

Cowen and Company, LLC and/or its affiliates expect to receive, or intend to seek, compensation for investment banking services in the next 3 months from Zafgen.

Zafgen and Portola Pharmaceuticals is or was in the past 12 months a client of Cowen and Company, LLC; during the past 12 months, Cowen and Company, LLC provided IB services.

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Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

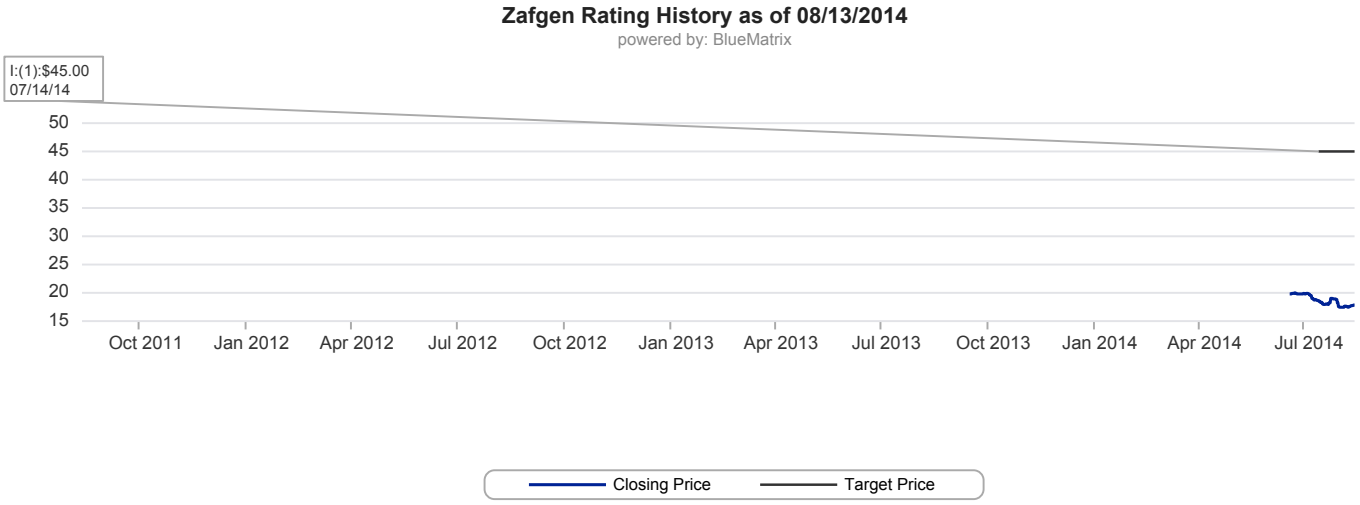
Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 06/30/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	417	58.57%	94	22.54%
Hold (b)	279	39.19%	7	2.51%
Sell (c)	16	2.25%	0	0.00%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.



Gilead Sciences Rating History as of 08/13/2014

powered by: BlueMatrix

Portola Pharmaceuticals Rating History as of 08/13/2014

powered by: BlueMatrix

Legend for Price Chart:

I = Initiation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | $xx = Price Target | NA = Not Available | S=Suspended

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