

Reason for report:

PREVIEW

## ZAFGEN, INC.

## Beloranib HIAO Data 1Q15 - Strong Results Could Double Mkt. Oppty, Read on PWS

• **Bottom Line:** After taking a closer look at beloranib's mechanism-of-action and the compelling data generated to date, we remain positively biased on ZFGN shares ahead of proof-of-concept Hypothalamic Injury-Associated Obesity (HIAO) data in 1Q15. HIAO has the potential to double the beloranib market opportunity, and positive Phase I/II results in January could render the Street more bullish on Phase III data in Prader-Willi Syndrome (PWS), which is expected by YE15. **Reiterate OP on ZFGN and \$35 price target.**

• **HIAO presents a significant and orphan unmet medical need with ~6K patients in the US**, comparable to the prevalence of Prader-Willi (~7K patients). Without a hypothalamus to regulate their hunger cycles, HIAO patients gain weight very rapidly; at our Rare Disease Day in October, ZFGN noted that some patients had to be excluded from the HIAO Phase I/II because they had BMIs over 80 and were too large to fit into some of the equipment. Often referred to as "acquired PWS", specialists note that the phenotypes of HIAO and PWS are similar, and believe that beloranib's impact on both weight loss and hyperphagia (constant hunger) differentiates the drug from other obesity treatments, which only ameliorate the former. Importantly, given the neuroanatomical differences between PWS patients and those with HIAO (no hypothalamus), we believe the Street is unlikely to read too negatively on PWS if HIAO fails in light of previous signals that beloranib's activity may be at least somewhat CNS-mediated.

• **Based on its mechanism-of-action, we believe beloranib's robust weight-loss effects in both Prader-Willi and Severe Obesity settings should translate over to HIAO**, though we are unsure whether or not the drug will confer as strong an effect on hyperphagia. Beloranib works by inhibiting MetAP2 enzyme (found in the liver), which rebalances lipid metabolism and reduces hunger. However, data generated in PWS suggests that the drug may have some downstream neurological activity, as some treated patients experienced increased sleep latency and vivid dreams. This lack of visibility on the physiological source of beloranib's effect on hyperphagia makes us incrementally less certain in seeing as strong a hunger-effect in HIAO as in PWS, though if able to incur stat. sig. weight loss we'd expect beloranib to garner uptake in HIAO regardless, given its other benefits on LDL/HDL and trigls.

• **We see a +50%/-35% up/down for ZFGN shares in best case/worst case data scenarios**, though with multiple efficacy endpoints/outcomes we expect a stock move somewhere in between. Our sensitivity analysis is informed by our conversations with investors and by solving for the stock price with our model. At ~\$30, we believe the stock is pricing-in ~70%/-30% probabilities in PWS/HIAO, respectively, and a negligible amount of value for severe obesity; changing these to 80%/60% or 60%/0% adds/detracts ~\$15/share & ~\$11/share, respectively.

## Key Stats:

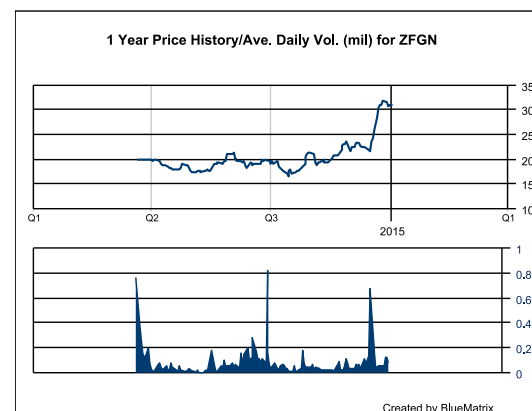
(OTC Un:ZFGN)

**S&P 600 Health Care Index:** 1,415.44  
**Price:** \$30.92  
 Price Target: \$35.00  
 Methodology: Probability-weighted DCF analysis, 12% discount rate

52 Week High: \$32.25  
 52 Week Low: \$16.01  
 Shares Outstanding (mil): 24.2  
 Market Capitalization (mil): \$748.3  
 Book Value/Share: \$0.00  
 Cash Per Share: \$4.94  
 Dividend (ann): \$0.00  
 Dividend Yield: 0.0%

*Shares Outstanding (mil): Diluted; includes stock options*

*Cash Per Share: On a net basis as of 3Q14*



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	--	--	--	--	0.0	--	--	--	--	(\$19.53)	NM
2014E	0.0A	0.0A	0.0A	0.0	0.0	(\$6.18)A	(\$2.96)A	(\$0.65)A	(\$0.81)	(\$3.65)	NM
2015E	0.0	0.0	0.0	0.0	0.0	(\$0.86)	(\$0.90)	(\$0.95)	(\$1.00)	(\$3.71)	NM

Source: Company Information and Leerink Partners LLC Research  
 GAAP EPS presented

## INVESTMENT THESIS

We rate ZFGN Shares Outperform. Zafgen (NASDAQ: ZFGN) is a biopharmaceutical company dedicated to the development of medicines to address the unmet need in obesity, with an initial focus on two orphan diseases that offer a potentially streamlined development path and high margin business model. We believe that ZFGN has an experienced management team with an outstanding track record executing in the field of drug discovery and commercialization. ZFGN's lead asset, beloranib, is a MetAP2 inhibitor that has generated compelling Phase II data in Prader-Willi syndrome (PWS) and severe obesity on a number of clinically relevant endpoints, in our view, and is entering a PWS Phase III program beginning in 2H14. We project a 70% probability of PWS approval in 2017, and peak gross PWS sales of ~\$700MM worldwide in 2029. Zafgen is also developing beloranib in hypothalamic-injury associated obesity (HIAO), and data from a Phase IIa study is expected to be reported in 1Q15. We project 50% probability of HIAO approval in 2018, and peak gross HIAO sales of ~\$440MM worldwide in 2029. The commercial opportunity presented by severe obesity holds the potential to be orders of magnitude larger than PWS and HIAO, though ZFGN will likely need support from a larger partner to unlock its full potential. Thus, while severe obesity afflicts ~16MM Americans in the US, we only model ~\$140MM in peak beloranib sales in non-PWS/HIAO patients, though in a partnership/acquisition (P&A) scenario, ZFGN is likely to receive considerably more value for beloranib in high prevalence indications. In the meantime, we believe that establishing broader proof-of-concept in orphan sub-populations offers a less risky and more rapid development path, and over the long term could position ZFGN as a very attractive partnership or takeout target. Likewise, a second generation MetAP2 inhibitor in preclinical development for general obesity, and a novel chemical class MetAP2 inhibitor in preclinical development for NASH/diabetes could provide significant upside to our price target as clinical catalysts are realized.

## VALUATION

We derive a ~\$35 per share value for ZFGN using a 12% discount rate and a 2% terminal growth rate, representing a ~\$860MM market capitalization. Our price target assumes a 70% and 50% probability of beloranib approval in PWS and HIAO, respectively, which leads to our peak risk-adjusted sales estimates of ~\$490MM and ~\$220MM in each indication. We only model ~\$140MM in peak sales in severe obesity, which we believe holds the potential to be very conservative if/when ZFGN generates pivotal beloranib data in orphan indications.

## RISKS TO VALUATION

Risks to our valuation include disappointing clinical data, regulatory setbacks, dilution risk from an additional equity offering, and commercial shortfalls. Because ZFGN has only one late stage product, the occurrence of any of these could impact the stock significantly.

ZFGN P&L (\$MM) GAAP	2013	1Q14	2Q14	3Q14	4Q14E	2014E	1Q15E	2Q15E	3Q15E	4Q15E	2015E	2016E	2017E	2018E	2019E
Beloranib PWS	-	-	-	-	-	-	-	-	-	-	-	-	4.0	46.5	126.6
Beloranib Craniopharyngioma	-	-	-	-	-	-	-	-	-	-	-	-	-	8.3	28.7
Beloranib Severe Obesity	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
<b>Total Revenue (p/w)</b>	-	-	-	-	-	-	-	-	-	-	-	-	<b>4.0</b>	<b>54.9</b>	<b>155.3</b>
COGS	-	-	-	-	-	-	-	-	-	-	-	-	0.4	5.5	15.5
R&D	9.6	3.3	4.7	12.1	15.0	35.0	16.0	17.0	18.0	19.0	70.0	60.0	61.8	61.8	54.4
SG&A	4.2	1.2	1.3	2.3	3.3	8.1	3.6	3.8	4.0	4.1	15.5	21.7	30.0	38.4	54.4
Operating Expenses	13.8	4.5	6.0	14.4	18.3	43.2	19.6	20.8	22.0	23.1	85.5	81.7	92.2	105.7	124.2
Operating Income	(13.8)	(4.5)	(6.0)	(14.4)	(18.3)	(43.2)	(19.6)	(20.8)	(22.0)	(23.1)	(85.5)	(81.7)	(88.2)	(50.8)	31.1
Interest Income (Expense)	-	(0.0)	(0.4)	(0.2)	(0.2)	(0.9)	(0.3)	(0.3)	(0.3)	(0.3)	(1.2)	(0.8)	(0.7)	-	-
FX Gains/Losses	(0.2)	0.1	0.0	(0.1)	-	(0.0)	-	-	-	-	-	-	-	-	-
Total Other Income (expense)	(0.2)	0.1	(0.4)	(0.3)	(0.2)	(0.9)	-	-	-	-	-	(0.8)	(0.7)	-	-
EBT	(14.0)	(4.5)	(6.4)	(14.7)	(18.5)	(44.1)	(19.6)	(20.8)	(22.0)	(23.1)	(85.5)	(82.5)	(88.8)	(50.8)	31.1
Tax	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Comprehensive Income (Loss)	(14.0)	(4.5)	(6.4)	(14.7)	(18.5)	(44.1)	(19.6)	(20.8)	(22.0)	(23.1)	(85.5)	(82.5)	(88.8)	(50.8)	31.1
Accretion of Covert. Preferred	(0.2)	(0.0)	(0.0)	-	-	(0.1)	-	-	-	-	-	-	-	-	-
<b>Net Income (Loss)</b>	<b>(14.2)</b>	<b>(4.5)</b>	<b>(6.4)</b>	<b>(14.7)</b>	<b>(18.5)</b>	<b>(44.2)</b>	<b>(19.6)</b>	<b>(20.8)</b>	<b>(22.0)</b>	<b>(23.1)</b>	<b>(85.5)</b>	<b>(82.5)</b>	<b>(88.8)</b>	<b>(50.8)</b>	<b>31.1</b>
<b>Diluted EPS</b>	<b>\$ (19.53)</b>	<b>\$ (6.18)</b>	<b>\$ (2.96)</b>	<b>\$ (0.65)</b>	<b>\$ (0.81)</b>	<b>\$ (3.65)</b>	<b>\$ (0.86)</b>	<b>\$ (0.90)</b>	<b>\$ (0.95)</b>	<b>\$ (1.00)</b>	<b>\$ (3.71)</b>	<b>\$ (3.17)</b>	<b>\$ (3.11)</b>	<b>\$ (1.72)</b>	<b>\$ 1.02</b>
Basic Shares Outstanding	0.7	0.7	2.2	22.7	22.8	12.1	22.9	23.0	23.1	23.2	23.1	26.1	28.6	29.6	30.6
Diluted Shares Outstanding	0.7	0.7	2.2	22.7	22.8	12.1	22.9	23.0	23.1	23.2	23.1	26.1	28.6	29.6	30.6

Source: SEC Filings and Leerink Partners Research

ZFGN BS & CFS (\$MM) GAAP	2013	1Q14	2Q14	3Q14E	4Q14E	2014E	1Q15E	2Q15E	3Q15E	4Q15E	2015E	2016E	2017E	2018E	2019E
<b>Net Cash</b>	<b>35.5</b>	<b>31.1</b>	<b>126.9</b>	<b>119.6</b>	<b>95.0</b>	<b>95.0</b>	<b>71.4</b>	<b>52.3</b>	<b>31.8</b>	<b>10.2</b>	<b>10.2</b>	<b>34.1</b>	<b>45.5</b>	<b>12.2</b>	<b>54.2</b>
Cash & Equivalents	35.5	38.5	134.2	127.0	107.4	107.4	88.3	68.1	46.6	23.9	23.9	43.7	54.0	12.2	54.2
Debt	-	7.4	7.4	7.4	12.4	12.4	16.9	15.8	14.8	13.8	13.8	9.6	8.5	-	-
<b>Change in Cash</b>	<b>25.6</b>	<b>3.0</b>	<b>95.8</b>	<b>(7.2)</b>	<b>(19.6)</b>	<b>71.8</b>	<b>(19.1)</b>	<b>(20.2)</b>	<b>(21.5)</b>	<b>(22.7)</b>	<b>(83.4)</b>	<b>19.7</b>	<b>10.4</b>	<b>(41.8)</b>	<b>41.9</b>
<b>Operating Cash Flow</b>	<b>(15.0)</b>	<b>(4.0)</b>	<b>(6.9)</b>	<b>(6.7)</b>	<b>(24.1)</b>	<b>(41.8)</b>	<b>(18.0)</b>	<b>(19.1)</b>	<b>(19.4)</b>	<b>(19.7)</b>	<b>(76.3)</b>	<b>(71.1)</b>	<b>(74.1)</b>	<b>(31.8)</b>	<b>51.9</b>
Net Income (Loss)	(14.0)	(4.5)	(6.4)	(14.7)	(18.5)	(44.1)	(19.6)	(20.8)	(22.0)	(23.1)	(85.5)	(82.5)	(88.8)	(50.8)	31.1
SOE	0.4	0.2	0.4	0.9	1.1	2.5	1.6	1.7	1.8	1.8	6.8	7.4	9.2	10.0	10.9
D&A	0.0	0.0	-	-	-	0.0	-	-	0.8	1.6	2.4	4.0	5.6	9.0	10.0
Other	(1.4)	0.3	(0.9)	7.1	(6.7)	(0.2)	-	-	-	-	-	-	-	-	-
<b>Investing Cash Flow</b>	<b>(0.0)</b>	<b>(0.0)</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>(0.0)</b>	<b>-</b>	<b>-</b>	<b>(1.0)</b>	<b>(2.0)</b>	<b>(3.0)</b>	<b>(5.0)</b>	<b>(7.0)</b>	<b>(10.0)</b>	<b>(10.0)</b>
CapEx	(0.0)	(0.0)	-	-	-	(0.0)	-	-	(1.0)	(2.0)	(3.0)	(5.0)	(7.0)	(10.0)	(10.0)
Other	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
<b>Financing Cash flow</b>	<b>40.6</b>	<b>7.0</b>	<b>102.7</b>	<b>(0.5)</b>	<b>4.5</b>	<b>113.7</b>	<b>(1.0)</b>	<b>(1.0)</b>	<b>(1.0)</b>	<b>(1.0)</b>	<b>(4.2)</b>	<b>95.8</b>	<b>91.5</b>	<b>-</b>	<b>-</b>
Equity Issuance (Buyback)	40.8	0.4	102.7	-	-	103.1	-	-	-	-	-	100.0	100.0	-	-
Debt Issuance (Retirement)	-	7.4	-	(0.5)	4.5	11.4	(1.0)	(1.0)	(1.0)	(1.0)	(4.2)	(4.2)	(8.5)	-	-
Other	(0.2)	(0.8)	-	-	-	(0.8)	-	-	-	-	-	-	-	-	-

Source: SEC Filings and Leerink Partners Research

ZFGN DCF Analysis	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	TV
Cash Flow From Operations (\$MM)	(42)	(76)	(71)	(74)	(32)	52	98	168	206	242	267	287	305	322	337	344	179	99	
Cash Flow From Investing (\$MM)	(0)	(3)	(5)	(7)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	(10)	
Net Borrowing (Repayment) (\$MM)	4	(4)	(4)	(9)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	
Free Cash Flow (\$MM)	(38)	(83)	(80)	(90)	(42)	42	88	158	196	232	257	277	295	312	327	334	169	89	912
Discount Periods	-	0.25	1.25	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25	12.25	13.25	14.25	15.25	16.25	
NPV FCF (\$MM)	(9)	(81)	(70)	(69)	(29)	26	48	78	86	91	90	87	83	78	73	66	30	14	145

Sum NPV FCF (\$MM)	736
Net Cash 3Q14	120
Implied ZFGN Mkt Cap (\$MM)	\$ 855
ZFGN Per Share Value	\$ 35.33

Cost of Equity	12%
TG Rate	2%
Diluted Shares Outstanding	24.2

Source: Leerink Partners Research

Prader Willi Syndrome Revenue Model	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E
PWS Patients in the US	7,500	7,568	7,636	7,704	7,774	7,844	7,914	7,985	8,057	8,130	8,203	8,277	8,351	8,426	8,502	8,579	8,656	8,734
% >12 years old	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
US PWS Patients >12 yr/old	3,750	3,784	3,818	3,852	3,887	3,922	3,957	3,993	4,029	4,065	4,102	4,138	4,176	4,213	4,251	4,289	4,328	4,367
% treated with Beloranib	0.0%	0.0%	0.0%	1.0%	8.0%	16.0%	21.0%	25.0%	28.0%	30.0%	32.0%	33.0%	34.0%	35.0%	35.0%	35.0%	17.5%	7.0%
PWS Patients on Beloranib	-	-	-	39	311	627	831	998	1,128	1,219	1,312	1,366	1,420	1,475	1,488	1,501	757	306
Annual Cost of Therapy	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000
Gross Revenues (\$MM)	\$0.0	\$0.0	\$0.0	\$5.8	\$46.6	\$94.1	\$124.6	\$149.7	\$169.2	\$182.9	\$196.9	\$204.9	\$213.0	\$221.2	\$223.2	\$225.2	\$113.6	\$45.9
%<12 years old	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
US PWS Patients <12 yr/old	3,750	3,784	3,818	3,852	3,887	3,922	3,957	3,993	4,029	4,065	4,102	4,138	4,176	4,213	4,251	4,289	4,328	4,367
% treated with Beloranib	0.0%	0.0%	0.0%	0.0%	1.0%	8.5%	16.0%	21.0%	25.0%	28.0%	30.0%	32.0%	33.0%	34.0%	35.0%	35.0%	17.5%	7.0%
PWS Patients on Beloranib	-	-	-	-	39	333	633	838	1,007	1,138	1,230	1,324	1,378	1,433	1,488	1,501	757	306
Annual Cost of Therapy	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000
Gross Revenues (\$MM)	\$0.0	\$0.0	\$0.0	\$0.0	\$5.8	\$50.0	\$95.0	\$125.8	\$151.1	\$170.7	\$184.6	\$198.6	\$206.7	\$214.9	\$223.2	\$225.2	\$113.6	\$45.9
Approval Probability	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%
<b>US P(w) Beloranib PWS Revenues</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$4.0</b>	<b>\$36.7</b>	<b>\$100.9</b>	<b>\$153.7</b>	<b>\$192.8</b>	<b>\$224.2</b>	<b>\$247.6</b>	<b>\$267.0</b>	<b>\$282.4</b>	<b>\$293.8</b>	<b>\$305.2</b>	<b>\$312.5</b>	<b>\$315.3</b>	<b>\$159.1</b>	<b>\$64.2</b>
PWS Patients in the EU	12,000	12,108	12,217	12,327	12,438	12,550	12,663	12,777	12,892	13,008	13,125	13,243	13,362	13,482	13,604	13,726	13,850	13,974
% >12 years old	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
EU PWS Patients >12 yr/old	6,000	6,054	6,108	6,163	6,219	6,275	6,331	6,388	6,446	6,504	6,562	6,621	6,681	6,741	6,802	6,863	6,925	6,987
% treated with Beloranib	0.0%	0.0%	0.0%	0.0%	2.5%	4.0%	6.0%	8.0%	11.0%	13.0%	15.0%	16.0%	17.0%	18.0%	19.0%	20.0%	10.0%	4.0%
PWS Patients on Beloranib	-	-	-	-	155	251	380	511	709	846	984	1,059	1,136	1,213	1,292	1,373	692	279
Annual Cost of Therapy	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000
Gross Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$14.0	\$22.6	\$34.2	\$46.0	\$63.8	\$76.1	\$88.6	\$95.3	\$102.2	\$109.2	\$116.3	\$123.5	\$62.3	\$25.2
%<12 years old	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
US PWS Patients <12 yr/old	6,000	6,054	6,108	6,163	6,219	6,275	6,331	6,388	6,446	6,504	6,562	6,621	6,681	6,741	6,802	6,863	6,925	6,987
% treated with Beloranib	0.0%	0.0%	0.0%	0.0%	0.0%	2.5%	4.0%	6.0%	8.0%	11.0%	13.0%	15.0%	17.0%	18.0%	19.0%	20.0%	10.0%	4.0%
PWS Patients on Beloranib	-	-	-	-	-	157	253	383	516	715	853	993	1,136	1,213	1,292	1,373	692	279
Annual Cost of Therapy	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000
Gross Revenues (\$MM)	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$14.1	\$22.8	\$34.5	\$46.4	\$64.4	\$76.8	\$89.4	\$102.2	\$109.2	\$116.3	\$123.5	\$62.3	\$25.2
Approval Probability	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%
<b>EU P(w) Beloranib PWS Revenues</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$9.8</b>	<b>\$25.7</b>	<b>\$39.9</b>	<b>\$56.3</b>	<b>\$77.2</b>	<b>\$98.3</b>	<b>\$115.8</b>	<b>\$129.3</b>	<b>\$143.1</b>	<b>\$152.9</b>	<b>\$162.8</b>	<b>\$172.9</b>	<b>\$87.3</b>	<b>\$35.2</b>
WW Beloranib Gross Sales	\$0.0	\$0.0	\$0.0	\$5.8	\$66.5	\$180.8	\$276.6	\$356.0	\$430.5	\$494.1	\$546.8	\$588.2	\$624.1	\$654.5	\$679.0	\$697.5	\$351.9	\$142.0
<b>WW Beloranib P(w) Sales</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$4.0</b>	<b>\$46.5</b>	<b>\$126.6</b>	<b>\$193.6</b>	<b>\$249.2</b>	<b>\$301.4</b>	<b>\$345.9</b>	<b>\$382.8</b>	<b>\$411.8</b>	<b>\$436.9</b>	<b>\$458.1</b>	<b>\$475.3</b>	<b>\$488.2</b>	<b>\$246.3</b>	<b>\$99.4</b>

Assumptions	
Beloranib US Cost	\$150,000
Beloranib EU Cost	\$90,000
Probability of Approval	70%

Source: Company Presentations and Leerink Partners Research

Craniopharyngioma Revenue Model	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E
Craniopharyngioma Patients in the US	6,260	6,316	6,373	6,431	6,488	6,547	6,606	6,665	6,725	6,786	6,847	6,908	6,971	7,033	7,097	7,160	7,225	7,290
% with post-treatment hypothalamic dysfunction	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
beloranib craniopharyngioma candidates	3,130	3,158	3,187	3,215	3,244	3,273	3,303	3,333	3,363	3,393	3,423	3,454	3,485	3,517	3,548	3,580	3,612	3,645
% treated with Beloranib	0.0%	0.0%	0.0%	0.0%	2.0%	6.0%	13.0%	18.0%	24.0%	29.0%	32.0%	34.0%	36.0%	38.0%	40.0%	40.0%	20.0%	10.0%
Patients on Beloranib	-	-	-	-	65	196	429	600	807	984	1,095	1,174	1,255	1,336	1,419	1,432	722	364
Annual Cost of Therapy	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000	\$150,000
Gross Revenues (\$MM)	\$0.0	\$0.0	\$0.0	\$0.0	\$9.7	\$29.5	\$64.4	\$90.0	\$121.1	\$147.6	\$164.3	\$176.2	\$188.2	\$200.4	\$212.9	\$214.8	\$108.4	\$54.7
Approval Probability	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
<b>US P(w) Beloranib Cranio Revenues</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$4.9</b>	<b>\$14.7</b>	<b>\$32.2</b>	<b>\$45.0</b>	<b>\$60.5</b>	<b>\$73.8</b>	<b>\$82.2</b>	<b>\$88.1</b>	<b>\$94.1</b>	<b>\$100.2</b>	<b>\$106.4</b>	<b>\$107.4</b>	<b>\$54.2</b>	<b>\$27.3</b>
Craniopharyngioma Patients in the EU	14,850	14,984	15,119	15,255	15,392	15,530	15,670	15,811	15,953	16,097	16,242	16,388	16,536	16,684	16,835	16,986	17,139	17,293
% with post-treatment hypothalamic dysfunction	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
beloranib craniopharyngioma candidates	7,425	7,492	7,559	7,627	7,696	7,765	7,835	7,906	7,977	8,049	8,121	8,194	8,268	8,342	8,417	8,493	8,569	8,647
% treated with Beloranib	0.0%	0.0%	0.0%	0.0%	1.0%	4.0%	8.0%	12.0%	16.0%	20.0%	22.0%	24.0%	26.0%	28.0%	30.0%	30.0%	15.0%	7.5%
Patients on Beloranib	-	-	-	-	77	311	627	949	1,276	1,610	1,787	1,967	2,150	2,336	2,525	2,548	1,285	648
Annual Cost of Therapy	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000	90,000
Gross Revenues (\$MM)	\$0.0	\$0.0	\$0.0	\$0.0	\$6.9	\$28.0	\$56.4	\$85.4	\$114.9	\$144.9	\$160.8	\$177.0	\$193.5	\$210.2	\$227.3	\$229.3	\$115.7	\$58.4
Approval Probability	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
<b>EU P(w) Beloranib Cranio Revenues</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$3.5</b>	<b>\$14.0</b>	<b>\$28.2</b>	<b>\$42.7</b>	<b>\$57.4</b>	<b>\$72.4</b>	<b>\$80.4</b>	<b>\$88.5</b>	<b>\$96.7</b>	<b>\$105.1</b>	<b>\$113.6</b>	<b>\$114.7</b>	<b>\$57.8</b>	<b>\$29.2</b>
WW Gross Beloranib Craniopharyngioma Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$16.7	\$57.4	\$120.8	\$175.4	\$235.9	\$292.5	\$325.1	\$353.2	\$381.7	\$410.7	\$440.2	\$444.1	\$224.1	\$113.0
<b>WW P(w) Beloranib Craniopharyngioma Revenues</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$8.3</b>	<b>\$28.7</b>	<b>\$60.4</b>	<b>\$87.7</b>	<b>\$118.0</b>	<b>\$146.2</b>	<b>\$162.6</b>	<b>\$176.6</b>	<b>\$190.8</b>	<b>\$205.3</b>	<b>\$220.1</b>	<b>\$222.1</b>	<b>\$112.0</b>	<b>\$56.5</b>
<b>Assumptions</b>																		
Beloranib US Cost	\$150,000																	
Beloranib EU Cost	\$90,000																	
Probability of Approval	50%																	

Source: Company Presentations and Leerink Partners Research

Product	Event	Timing
Beloranib	Initiate US Phase III PWS Trial	3Q14
Beloranib	Initiate Phase IIb Severe Obesity Trial	4Q14
Beloranib	Phase IIa HAO Data	1Q15
ZGN-839	File NASH/Type II Diabetes IND	1H15
Beloranib	6 Month Interim Severe Obesity Data	4Q15
Beloranib	6 Mo. Phase III PWS Data	4Q15

Source: Company Presentations and Leerink Partners Research

## Disclosures Appendix

### Analyst Certification

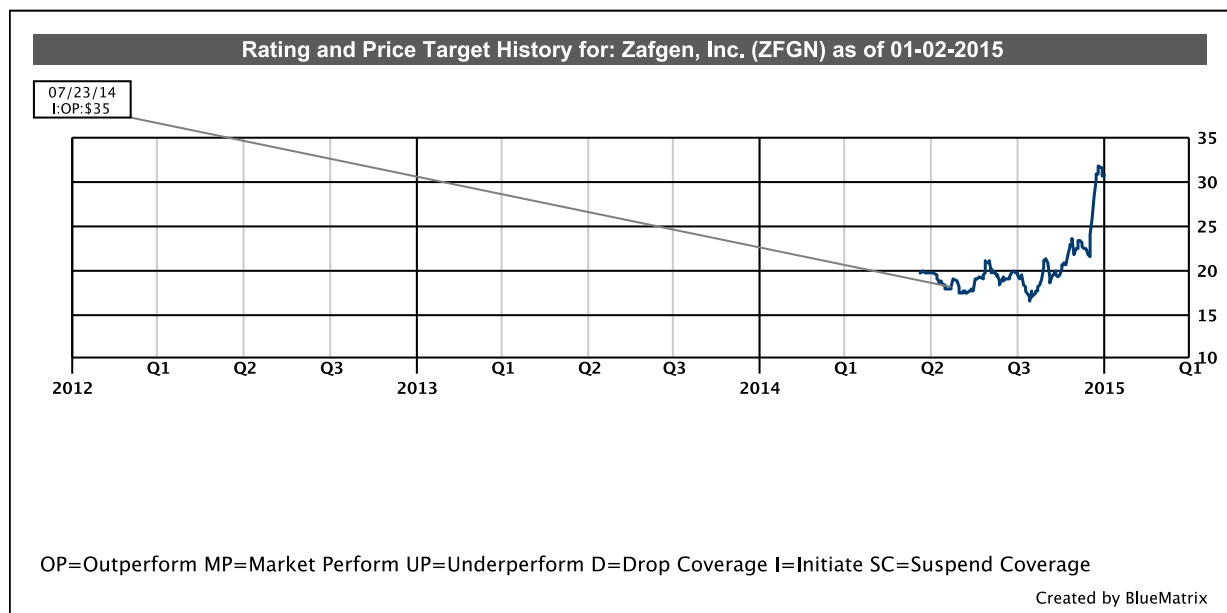
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### Valuation

We derive a ~\$35 per share value for ZFGN using a 12% discount rate and a 2% terminal growth rate, representing a ~\$860MM market capitalization. Our price target assumes a 70% and 50% probability of beloranib approval in PWS and HIAO, respectively, which leads to our peak risk-adjusted sales estimates of ~\$490MM and ~\$220MM in each indication. We only model ~\$140MM in peak sales in severe obesity, which we believe holds the potential to be very conservative if/when ZFGN generates pivotal beloranib data in orphan indications.

### Risks to Valuation

Risks to our valuation include disappointing clinical data, regulatory setbacks, dilution risk from an additional equity offering, and commercial shortfalls. Because ZFGN has only one late stage product, the occurrence of any of these could impact the stock significantly.





Distribution of Ratings/Investment Banking Services (IB) as of 09/30/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	138	69.30	51	37.00
HOLD [MP]	61	30.70	2	3.30
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

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

Leerink Partners LLC makes a market in Zafgen, Inc.

Leerink Partners LLC has acted as the manager for a public offering of Zafgen, Inc. in the past 12 months.

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