#### **OUTPERFORM**

Joseph P. Schwartz (617) 918-4575 Joseph.Schwartz@Leerink.com

Paul.Matteis@Leerink.com

**Paul Matteis** (617) 918-4585

Reason for report:

**COMPANY UPDATE** 



## ZAFGEN, INC.

Positive Phase II data in HIAO, Readthrough for PWS; raising PT to

- Bottom Line: This morning ZFGN reported positive results from its Phase II trial of beloranib in Hypothalamic Injury-Associated Obesity (HIAO) with the primary endpoint of weight reduction meeting statistical significance (p=0.01). Given the phenotypic similarity of HIAO to Prader-Willi Syndrome (PWS), we view this as a positive read-through for its Phase III data in PWS, which is expected by YE15. We are updating our model with 80%/60% probabilities in PWS/HIAO, respectively, from 70%/50%, bringing us to a PT of \$42. Reiterate Outperform.
- · Mean weight loss of 3.4 kg and 6.2 kg after 4 and 8 weeks of beloranib treatment arm was statistically significantly improved (p =0.01) over placebo arm with mean weight loss of 0.3 kg after 4 weeks. Additional improvements in cardiovascular disease risk factors of lipid and inflammation (measured by C-reactive protein) were also observed. No information on the hyperphagia endpoint (constant hunger) was reported in the press release, and more detailed data will be presented at a future medical meeting. More clarity is expected on the structure of a registration-enabling study in HIAO following the company's interactions with US and EU regulatory authorities in 2015.
- The 1.8 mg twice-weekly subcutaneous (SC) dosing was welltolerated with no serious adverse events reported. Safety measures such as laboratory, electrocardiogram, and vital sign measurements revealed no signals of concern with all subjects randomized to beloranib completing the study. The trial enrolled 14 obese patients (9 women and 5 men) who were confirmed by MRI to have had hypothalamic injury.
- The robust effects seen in weight loss and other benefits on LDL/ HDL and trigs should bode well for the ongoing Phase III PWS study, expected to readout in 4Q15. Both PWS and HIAO presents a significant and orphan unmet medical needs with ~7K and ~6K patients in US respectively, with HIAO often referred to as "acquired PWS" by specialists given the similarity between the phenotypes of these 2 forms of disease.
- · Longer term, Beloranib's potential in the broader severe obesity market could drive further upside to our valuation, but currently comprises only ~\$4/share of our \$42 PT. In December 2014, ZFGN initiated its Phase II proof-of-concept study (NCT02324491) in obese pts with Type 2 Diabetes with primary endpoint of weight reduction expected in 4Q15. Label expansion to include such larger subtypes of an estimated total of ~16MM severe obesity patients in the US is likely to require larger late-stage trials and the generation of a robust safety database. In the meantime, we continue to believe that establishing broader proof of concept in orphan sub-populations such as PWS and HIAO offers a less risky and more rapid development path, and down the road could position ZFGN as a very attractive partnership or takeout target.

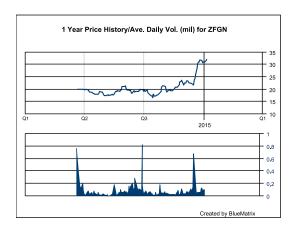
**Key Stats:** (OTC Un:ZFGN)

S&P 600 Health Care Index: 1,392.46 Price: \$32.17 Price Target: \$42.00 from \$35.00 Methodology: Probability-weighted DCF analysis, 12% discount rate

52 Week High: \$32 43 52 Week Low: \$16.01 Shares Outstanding (mil): 24.2 Market Capitalization (mil): \$778.5 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 and is now being studied in a registration-enabling Phase III PWS study expected to read out data by YE15. We project a 80% probability of PWS approval in 2017, and peak gross PWS sales of ~\$700MM worldwide in 2029. Zafgen is also developing beloranib in hypothalamicinjury associated obesity (HIAO), where it also recently released positive proof-of-concept Phase IIa data and expects to initiate a registration-enabling study in HIAO in the near future. We project 60% probability of HIAO approval in 2018, and peak gross HIAO sales of ~\$445MM 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 ~\$42 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 80% and 60% probability of beloranib approval in PWS and HIAO, respectively, which leads to our peak net sales estimates of ~\$560MM and ~\$270MM 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	-	-	-	-	-	-	-	-	-	-	-	-	4.6	53.2	144.
Beloranib HIAO	-	-	-	-	-	-	-	-	-	-	-	-	-	10.0	34.
Beloranib Severe Obesity	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue (p/w)	-	-	-	-	-	-	-	-	-	-	-	-	4.6	63.2	179.
cogs	-	-	-	-	-	-	-	-	-	-	-	-	0.5	6.3	17.
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	62.
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	44.2	62.
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	92.3	112.3	143.
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)	(87.6)	(49.2)	35.
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)	- 1	(0.0)				- 1			- 1	-	-
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)	(88.3)	(49.2)	35.
Тах	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
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)	(88.3)	(49.2)	35.
Accretion of Covert. Preferred	(0.2)	(0.0)	(0.0)	-	-	(0.1)	-	-	-	-	-	-	-	-	-
Net Income (Loss)	(14.2)	(4.5)	(6.4)	(14.7)	(18.5)	(44.2)	(19.6)	(20.8)	(22.0)	(23.1)	(85.5)	(82.5)	(88.3)	(49.2)	35.
Diluted EPS	\$ (19.53)	\$ (6.18) \$	(2.96) \$	(0.65) \$	(0.81)	\$ (3.65)	\$ (0.86)	\$ (0.90)	\$ (0.95) \$	(1.00)	\$ (3.71)	\$ (3.17)	\$ (3.09)	\$ (1.66)	\$ 1.1
	0.7		2.2	22.7	22.0		22.0	22.0	22.4	22.2	22.4	25.4	20.5	20.6	20
Basic Shares Outstanding	0.7 0.7	0.7	2.2 2.2	22.7 22.7	22.8 22.8	12.1	22.9	23.0	23.1	23.2 23.2	23.1	26.1 26.1	28.6 28.6	29.6 29.6	30.
Diluted Shares Oustanding Source: SEC Filings and Leerink Partners		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.

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
			4000	***						40.0	40.0			45.0	
Net Cash	35.5	31.1	126.9	119.6	95.0	95.0	71.4	52.3	31.8	10.2	10.2	34.1	46.0	15.0	63.4
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	54.6	15.0	63.4
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	-	-
Change in Cash	25.6	3.0	95.8	(7.2)	(19.6)	71.8	(19.1)	(20.2)	(21.5)	(22.7)	(83.4)	19.7	10.9	(39.6)	48.4
Operating Cash Flow	(15.0)	(4.0)	(6.9)	(6.7)	(24.1)	(41.8)	(18.0)	(19.1)	(19.4)	(19.7)	(76.3)	(71.1)	(73.5)	(29.6)	58.4
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)	(88.3)	(49.2)	35.8
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	10.6	12.5
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)	-	-	-	-	-	-	-	-	-
Investing Cash Flow	(0.0)	(0.0)	-	-	_	(0.0)	_	_	(1.0)	(2.0)	(3.0)	(5.0)	(7.0)	(10.0)	(10.0)
CapEx	(0.0)	(0.0)	-	-	-	(0.0)	-	-	(1.0)	(2.0)	(3.0)	(5.0)	(7.0)	(10.0)	(10.0)
Other	`-	- '	-	-	-	- '	-	-	`-	- 1	`- '	- '	, ,	, ,	, ,
Financing Cash flow	40.6	7.0	102.7	(0.5)	4.5	113.7	(1.0)	(1.0)	(1.0)	(1.0)	(4.2)	95.8	91.5	_	_
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)	(=,	,=,	,,	(=:=/	(/	(/	(0.0,	_	_

Source: SEC Filings and Leerink Partners Research

**ZAFGEN, INC.** January 7, 2015

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)	(30)	58	112	193	237	279	308	331	353	372	390	398	206	114	
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)	(89)	(40)	48	102	183	227	269	298	321	343	362	380	388	196	104	1061
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)	(27)	30	56	90	100	105	104	101	96	90	85	77	35	16	168

Sum NPV FCF (\$MM)	897
Net Cash 3Q14	120
Implied ZFGN Mkt Cap (\$MM)	\$ 1,017
ZFGN Per Share Value	\$ 41.99

Cost of Equity	12%
TG Rate	2%
Diluted Shares Oustanding	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 PWS Patients on Beloranib	0.0%	0.0%	0.0%	1.0%	8.0% 311	16.0% 627	21.0% 831	25.0% 998	28.0% 1.128	30.0% 1.219	32.0% 1.312	33.0% 1.366	34.0% 1.420	35.0% 1.475	35.0% 1.488	35.0% 1.501	17.5% 757	7.0% 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	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
US P(w) Beloranib PWS Revenues	\$0.0	\$0.0	\$0.0	\$4.6	\$42.0	\$115.3	\$175.7	\$220.4	\$256.2	\$282.9	\$305.2	\$322.8	\$335.7	\$348.9	\$357.1	\$360.3	\$181.8	\$73.4
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	400.000	400.000	400.000	400.000	155	251	380	511	709	846	984	1,059	1,136	1,213	1,292	1,373	692	279
Annual Cost of Therapy Gross Revenues	\$90,000 \$0.0	\$90,000 \$0.0	\$90,000 \$0.0	\$90,000 \$0.0	\$90,000 \$14.0	\$90,000 \$22.6	\$90,000 \$34.2	\$90,000 \$46.0	\$90,000 \$63.8	\$90,000 \$76.1	\$90,000 \$88.6	\$90,000 \$95.3	\$90,000 \$102.2	\$90,000 \$109.2	\$90,000 \$116.3	\$90,000 \$123.5	\$90,000 \$62.3	\$90,000 \$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% 157	4.0% 253	6.0% 383	8.0% 516	11.0% 715	13.0% 853	15.0% 993	17.0%	18.0% 1.213	19.0% 1.292	20.0% 1,373	10.0% 692	4.0% 279
PWS Patients on Beloranib	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000	1,136 \$90,000	\$90,000	\$90,000	\$90,000	\$90,000	\$90,000
Annual Cost of Therapy Gross Revenues (\$MM)	\$90,000	\$0.0	\$90,000	\$90,000	\$90,000	\$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	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
EU P(w) Beloranib PWS Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$11.2	\$29.4	\$45.6	\$64.4	\$88.2	\$112.4	\$132.3	\$147.8	\$163.6	\$174.7	\$186.1	\$197.7	\$99.7	\$40.2
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
WW Beloranib P(w) Sales	\$0.0	\$0.0	\$0.0	\$4.6	\$53.2	\$144.7	\$221.3	\$284.8	\$344.4	\$395.3	\$437.4	\$470.6	\$499.3	\$523.6	\$543.2	\$558.0	\$281.5	\$113.6

Assumptions	
Beloranib US Cost	\$150,000
Beloranib EU Cost	\$90,000
Probability of Approval	80%

Source: Company Presentations and Leerink Partners Research

**ZAFGEN, INC.** January 7, 2015

HIAO Revenue Model	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E
HIAO 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 HIAO 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	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%
US P(w) Beloranib HIAO Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$5.8	\$17.7	\$38.6	\$54.0	\$72.6	\$88.6	\$98.6	\$105.7	\$112.9	\$120.3	\$127.7	\$128.9	\$65.0	\$32.8
HIAO 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 HIAO 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	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%
EU P(w) Beloranib HIAO Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$4.2	\$16.8	\$33.8	\$51.2	\$68.9	\$86.9	\$96.5	\$106.2	\$116.1	\$126.1	\$136.4	\$137.6	\$69.4	\$35.0
WW Gross Beloranib HIAO 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
WW P(w) Beloranib HIAO Revenues	\$0.0	\$0.0	\$0.0	\$0.0	\$10.0	\$34.4	\$72.5	\$105.2	\$141.6	\$175.5	\$195.1	\$211.9	\$229.0	\$246.4	\$264.1	\$266.5	\$134.4	\$67.8

Assumptions	
Beloranib US Cost	\$150,000
Beloranib EU Cost	\$90,000
Probability of Approval	60%

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 HIAO 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
Beloranib	Initiate Phase III HIAO Data	2015

Source: Company Presentations and Leerink Partners Research



# Disclosures Appendix Analyst Certification

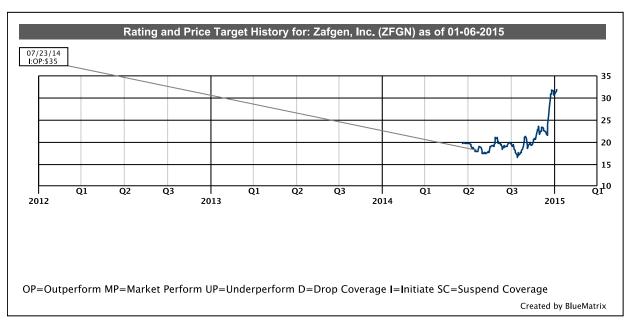
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

## **Valuation**

We derive a ~\$42 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 80% and 60% probability of beloranib approval in PWS and HIAO, respectively, which leads to our peak net sales estimates of ~\$560MM and ~\$270MM 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.





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

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

<u>Underperform (Sell):</u> 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.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction. MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

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.



©2015 Leerink Partners LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

Leerink Partners LLC Equity Research			
	Leerink Partners L	LC Equity Researc	<u>en</u>
Director of Equity Research	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
	,	(011) 010 1011	
Healthcare Strategy	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Biotechnology	Howard Liang, Ph.D.	(617) 918-4857	howard.liang@leerink.com
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Gena Wang, Ph.D., CFA	(212) 277-6073	gena.wang@leerink.com
	Paul Matteis	(617) 918-4585	paul.matteis@leerink.com
	Jonathan Chang, Ph.D.	(617) 918-4015	jonathan.chang@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
Life Science Tools	Dan Leonard	(212) 277-6116	dan.leonard@leerink.com
and Diagnostics	Justin Bowers, CFA	(212) 277-6066	justin.bowers@leerink.com
Pharmaceuticals/Major	Seamus Fernandez	(617) 918-4011	seamus.fernandez@leerink.com
•	Aneesh Kapur	(617) 918-4576	aneesh.kapur@leerink.com
Specialty Pharmaceuticals	Jason M. Gerberry, JD	(617) 918-4549	jason.gerberry@leerink.com
	Derek C. Archila	(617) 918-4851	derek.archila@leerink.com
Medical Devices, Cardiology	Danielle Antalffy	(212) 277-6044	danielle.antalffy@leerink.com
, 0,	Puneet Souda	(212) 277-6091	puneet.souda@leerink.com
& Orthopedics	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Ravi Misra	(212) 277-6049	ravi.misra@leerink.com
Healthcare Services	Ana Gupte, Ph.D.	(212) 277-6040	ana.gupte@leerink.com
Healthcare Technology	David Larsen, CFA	(617) 918-4502	david.larsen@leerink.com
& Distribution	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Digital Health	Steven Wardell	(617) 918-4097	steven.wardell@leerink.com
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com
Editorial	Cristina Diaz-Dickson	(617) 918-4548	cristina.diaz-dickson@leerink.com
Research Associate	Carmen Augustine	(212) 277-6012	carmen.augustine@leerink.com

**New York** 299 Park Avenue, 21<sup>st</sup> floor New York, NY 10171 (888) 778-1653 Boston One Federal Street, 37<sup>th</sup> Floor Boston, MA 02110 (800) 808-7525

San Francisco 201 Spear Street, 16<sup>th</sup> Floor San Francisco, CA 94105 (800) 778-1164