EQUITY RESEARCH

RBC Capital Markets

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Zafgen, Inc.

PWS Phase III data pushed out a bit; Obesity, PWS data expected YE15, 2Q16

Our view: On its 4Q14 call, ZFGN announced that topline data from the bestPWS Phase III trial are now expected "early 2Q16" (vs. prior YE15) due to slightly slower enrollment. A DSMB in the trial cleared the 2.4mg dose of beloranib to be used, and Zafgen decided to make it the only dose used in the ZAF-312 PWS EU trial expected to start mid-2015.

Key points:

On its earnings call, ZFGN updated timelines for lead asset belonarib and provided guidance for increased spending in 2015. The key updates are:

1) Topline Phase III data in PWS now expected "(probably early) 2Q16" vs. prior YE15. ZFGN pushed out expected timing for topline data in the ZAF-311 Phase III trial of beloranib due to slower than expected recruitment, despite fewer than expected dropouts in the trial.

2) The 102-patient trial enrolling in 15 US sites is now 2/3 enrolled. ZFGN will add CAN sites, and will lower the BMI inclusion criteria to 27 from 30 to speed up enrollment. Mgmt also reiterated the recently disclosed decision to go back to change in body weight (used to be change in body fat mass) and hyperphagia as co-primary endpoints in the trial.

3) Higher dose cleared to use in Phase III; EU trial will use just this dose (2.4mg). A DSMB safety look into the early part of the bestPWS trial has cleared the 2.4mg dose to be used in the trial. ZFGN has also decided to use just the 2.4mg dose in the second Phase III trial, ZAF-312, which will be conducted in the EU and is still expected to start in mid-2015.

Adjusting our price target post-full HIAO data. Given the positive news on the 2.4mg dose getting the DSMB green light from a safety standpoint, and the recently presented full HIAO dataset, we are adjusting our probability of success in the PWS and HIAO trials to 65% and 60%, from 60% and 50%, respectively, resulting in our $58 price target (raised from $53).

What's next for ZFGN: 1) Submit IND for oral ZGN-839 program in NASH/T2DM, mid-2015; **2)** Initiate second PWS trial (ZAF-312) in EU, mid-2015; **3)** *Interim Ph IIb ZAF-203 data in severe obesity, YE15;* **4)** *6-month Ph III bestPWS readout, 2Q16.*

4Q/FY14 numbers: Spent $35.5M in 2014, has $245M in proforma cash. ZFGN spent $10.7M in 4Q14 ($7.3M in R&D, $3.3M in G&A) vs. $3.8M in 4Q13 ($2.5M in R&D, $1.2M in G&A). In 2014, operating expenses were $35.5M ($27.4M in R&D, $8.1M in G&A) vs. $13.8M ($9.6M in R&D, $4.2M in G&A) in 2013. ZFGN ended the year with $115.5M cash and raised $130M net proceeds in a January 2015 equity offering at $35/share, bringing its pro-forma cash position to $245M, sufficient "for at least the next 18 months".

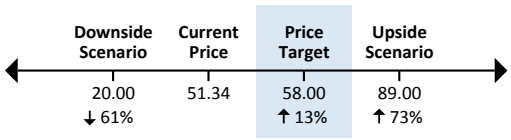
Outperform
Speculative Risk

NASDAQ: ZFGN; USD 51.34

Price Target USD 58.00 ↑ 53.00

WHAT'S INSIDE	
☐ Rating/Risk Change	☑ Price Target Change
☐ In-Depth Report	☑ Est. Change
☐ Preview	☐ News Analysis

Scenario Analysis*



*Implied Total Returns

Key Statistics

Shares O/S (MM):	28.5	Market Cap (MM):	1,463
Dividend:	0.00	Yield:	0.0%
		Avg. Daily Volume:	150,863

RBC Estimates

FY Dec	2014A	2015E	2016E
Revenue	0.0	0.0	0.0
EPS, Ops Diluted	(3.00)	(2.51)	(2.23)
Prev.	(2.90)	(1.55)	(1.45)

Revenue	Q1	Q2	Q3	Q4
2014	0.0A	0.0A	0.0A	0.0A
2015	0.0E	0.0E	0.0E	0.0E
EPS, Ops Diluted				
2014	(6.18)A	(2.96)A	(0.65)A	(0.48)A
Prev.				(0.41)E
2015	(0.56)E	(0.60)E	(0.65)E	(0.70)E
Prev.	(0.37)E	(0.38)E	(0.39)E	(0.41)E

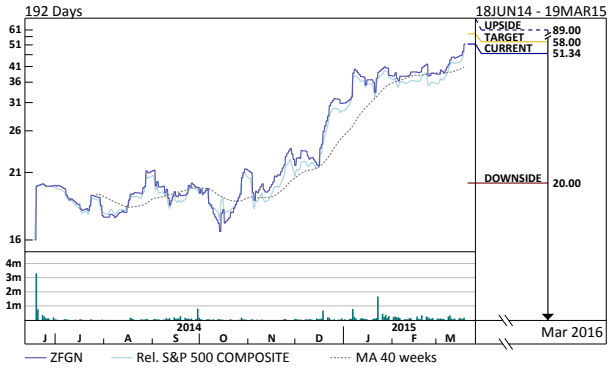
All values in USD unless otherwise noted.

Priced as of prior trading day's market close, EST (unless otherwise noted).

For Required Conflicts Disclosures, see Page 12.

Target/Upside/Downside Scenarios

Exhibit 1: Zafgen, Inc.



Source: Bloomberg and RBC Capital Markets estimates for Upside/Downside/Target

Target price/base case

To value ZFGN shares at $58, we use a sum-of-the-parts methodology, and estimate the probability-adjusted NPV of the following: 1) beloranib sales in PWS ($32/share), 2) beloranib sales in HIAO ($20/share), and 3) the company's projected net cash position (~$6/share). We assign a probability of success of 65% for PWS and 60% for HIAO, and model peak US/EU sales of beloranib in PWS and HIAO of $750MM and $630MM, respectively.

Upside scenario

Our upside scenario of $89/share assumes positive data from the Phase IIb trial of beloranib in severe obesity, an indication which is currently not part of our valuation. Zafgen's stated plan under this scenario is to preserve beloranib's premium pricing for the rare disease settings and develop a follow-on molecule for the large severe obesity indication. Given the impressive weight loss efficacy observed with beloranib and the fact that it comes without the need for concomitant diet and exercise, we believe that positive data in this Phase IIb trial could provide an additional $31/share to our estimates.

Downside scenario

The downside scenario of $20/share assumes the Phase III bestPWS trial fails. By removing sales in PWS, reducing beloranib penetrations in HIAO, and lowering the probability of success to 50% in HIAO, we arrive at a valuation of beloranib sales in HIAO of ~$14/share.

Investment summary

A really impressive injectable drug for obesity… Beloranib acts via a novel mechanism of action to lead to rapid and impressive weight loss in the obese patients it has been tested on in early stage Phase I and II trials. Using a novel mechanism different from the ones used by other drugs currently on the market, this methionine aminopeptidase 2 (MetAP2) inhibitor leads to significant weight loss (up to 10% of body weight) in just 12 weeks.

…but it will not get to the market for obesity first: Despite the compound's impressive efficacy, the company has decided to try a different approach and not go with obesity as its lead indication for beloranib. If successful, this would possibly allow the drug to reach market faster, in addition to gaining other benefits along the way, including premium pricing.

Prader-Willi Syndrome: a rare, genetic, obesity-related disease. There are an estimated 21,000 PWS cases in the US, with 7,500 of them identified. PWS patients are characterized by hyperphagia, the inability to feel satiety, which leads them to overeat and (most) to become obese. After promising data from a 17-patient Phase II trial, Zafgen is testing the drug in the bestPWS Phase III trial, with data expected YE15. A second trial to be conducted in Europe is expected to start soon, and will be required for US and EU approval. Based on the efficacy observed thus far, we expect the drug to be approved. Zafgen plans to market beloranib on its own, via a specialty salesforce, and we project that it can become a significant product in PWS alone, reaching $750MM in peak US/EU 2029 sales.

HIAO doubles beloranib's commercial potential: Zafgen recently reported positive data from a 14-patient, Phase II trial of beloranib in patients with hypothalamic injury-associated obesity (HIAO). These patients develop obesity due to uncontrollable hunger, somewhat akin to an "acquired PWS", following surgery to remove craniopharyngiomas. Positive pivotal data in this setting would make HIAO the second rare disease indication for beloranib, doubling its market potential.

Potential catalysts: 1) Phase III beloranib PWS data (2Q16), 2) IND application for ZGN-839 in NASH/T2DM (mid-15), 3) interim data from beloranib severe obesity trial (YE15).

Risks: 1) Delays in US or EU PWS trials, 2) negative data in Phase III PWS trials, and 3) unanticipated safety signals of beloranib in ongoing trials. These risks, that if materialized, may result in significant volatility, and the fact that the majority of the value comes from a single product leads to the Speculative Risk qualifier on our rating.

Exhibit 2: ZFGN Sum-of-the-parts Valuation ($MM)

ZFGN NPV (probability-adjusted)	
Beloranib PWS NPV - US	$17
Beloranib PWS NPV - EU	$15
Beloranib HIAO NPV - US	$11
Beloranib HIAO NPV - EU	$9
Projected Net Cash	$6
Sum-of-the-parts value for ZFGN	**$58**

Source: RBC Capital Markets estimates

Exhibit 3: ZFGN Quarterly P&L ($MM)

($MM)	FY 2013A	Q1: 14A	Q2: 14A	Q3: 14A	Q4: 14A	FY 2014A	Q1: 15E	Q2: 15E	Q3: 15E	Q4: 15E	FY 2015E	FY 2016E
US beloranib PWS sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
US beloranib HIAO sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total US beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
EU beloranib PWS sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
EU beloranib HIAO sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total EU beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Total US/EU beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Royalties paid to CKD Pharma	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Royalties paid to Children's	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total royalties paid	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Total revenue	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
COGS	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
R&D	**9.6**	**3.3**	**4.7**	**12.1**	**7.3**	**27.4**	**11.5**	**11.9**	**13.0**	**14.5**	**50.9**	**52.0**
SG&A	**4.2**	**1.2**	**1.3**	**2.3**	**3.3**	**8.1**	**3.4**	**3.9**	**4.2**	**4.3**	**15.8**	**15.9**
Total Operating Expenses	**13.8**	**4.5**	**6.0**	**14.4**	**10.7**	**35.5**	**14.9**	**15.8**	**17.2**	**18.8**	**66.7**	**67.9**
Operating Income (loss)	**(13.8)**	**(4.5)**	**(6.0)**	**(14.4)**	**(10.7)**	**(35.5)**	**(14.9)**	**(15.8)**	**(17.2)**	**(18.8)**	**(66.7)**	**(67.9)**
Total other expenses, net	(0.2)	0.1	(0.4)	(0.3)	(0.3)	(0.9)	(0.1)	(0.2)	(0.1)	(0.1)	(1.9)	(3.3)
Net loss and comprehensive loss	(14.0)	(4.5)	(6.4)	(14.7)	(10.9)	(36.5)	(15.0)	(16.0)	(17.3)	(18.9)	(67.2)	(71.2)
Accretion of redeemable convertible stock	(0.2)	(0.0)	(0.0)	0.0	0.0	(0.1)	(0.0)	(0.0)	(0.0)	(0.0)	(0.1)	(0.1)
Pretax income	(14.2)	(4.5)	(6.4)	(14.7)	(10.9)	(36.6)	(15.1)	(16.0)	(17.3)	(18.9)	(67.3)	(71.3)
Income tax expense	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Tax rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net income (loss)	**(14.2)**	**(4.5)**	**(6.4)**	**(14.7)**	**(10.9)**	**(36.6)**	**(15.1)**	**(16.0)**	**(17.3)**	**(18.9)**	**(67.3)**	**(71.3)**
GAAP EPS												
Basic	($19.53)	($6.18)	($2.96)	($0.65)	($0.48)	($3.00)	($0.56)	($0.60)	($0.65)	($0.70)	($2.51)	($2.23)
Diluted	($19.53)	($6.18)	($2.96)	($0.65)	($0.48)	($3.00)	($0.56)	($0.60)	($0.65)	($0.70)	($2.51)	($2.23)

Source: Company reports and RBC Capital Markets estimates

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Exhibit 4: ZFGN Annual P&L ($MM)

($MM)	FY 2013A	FY 2014A	FY 2015E	FY 2016E	FY 2017E	FY 2018E	FY 2019E	FY 2020E	FY 2021E	FY 2022E	FY 2023E	FY 2024E	FY 2025E	FY 2026E	FY 2027E	FY 2028E	FY 2029E	FY 2030E	FY 2031E	FY 2032E	FY 2033E
US beloranib PWS sales	0.0	0.0	0.0	0.0	0.0	89.5	157.0	211.4	285.9	293.9	302.1	310.5	319.2	328.1	337.2	346.6	356.2	366.2	376.4	38.7	19.9
US beloranib HIAO sales	0.0	0.0	0.0	0.0	0.0	0.0	74.2	128.8	186.3	251.9	258.9	266.2	273.6	281.2	289.0	297.1	305.3	313.9	322.6	33.2	17.0
Total US beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**89.5**	**231.2**	**340.2**	**472.2**	**545.8**	**561.0**	**576.7**	**592.7**	**609.2**	**626.2**	**643.7**	**661.6**	**680.0**	**699.0**	**71.8**	**36.9**
EU beloranib PWS sales	0.0	0.0	0.0	0.0	0.0	0.0	88.7	174.9	251.6	338.4	345.9	353.6	361.5	369.5	377.7	386.1	394.7	40.4	20.6	21.1	21.6
EU beloranib HIAO sales	0.0	0.0	0.0	0.0	0.0	0.0	0.0	66.6	130.5	197.2	296.5	303.1	309.8	316.7	323.8	331.0	236.8	34.6	17.7	18.1	18.5
Total EU beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**88.7**	**241.5**	**382.1**	**535.6**	**642.4**	**656.7**	**671.3**	**686.2**	**701.5**	**717.1**	**631.6**	**74.9**	**38.3**	**39.2**	**40.0**
Total US/EU beloranib sales	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**89.5**	**319.9**	**581.7**	**854.3**	**1081.5**	**1203.5**	**1233.4**	**1264.0**	**1295.5**	**1327.7**	**1360.8**	**1293.1**	**754.9**	**737.3**	**111.0**	**76.9**
Royalties paid to CKD Pharma	0.0	0.0	0.0	0.0	0.0	4.5	16.0	29.1	42.7	54.1	60.2	61.7	63.2	64.8	66.4	68.0	64.7	37.7	36.9	5.5	3.8
Royalties paid to Children's	0.0	0.0	0.0	0.0	0.0	2.7	9.6	17.5	25.6	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total royalties paid	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**7.2**	**25.6**	**46.5**	**68.3**	**54.1**	**60.2**	**61.7**	**63.2**	**64.8**	**66.4**	**68.0**	**64.7**	**37.7**	**36.9**	**5.5**	**3.8**
Total revenue	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**82.4**	**294.3**	**535.1**	**786.0**	**1027.4**	**1143.3**	**1171.7**	**1200.8**	**1230.7**	**1261.3**	**1292.7**	**1228.5**	**717.2**	**700.4**	**105.4**	**73.1**
COGS	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**10.7**	**38.4**	**69.8**	**94.0**	**119.0**	**120.3**	**123.3**	**126.4**	**129.5**	**132.8**	**136.1**	**129.3**	**75.5**	**73.7**	**11.1**	**7.7**
R&D	**9.6**	**27.4**	**50.9**	**52.0**	**53.0**	**53.8**	**54.9**	**55.7**	**56.6**	**54.4**	**52.4**	**50.8**	**49.4**	**48.2**	**47.1**	**46.3**	**45.6**	**45.1**	**44.7**	**44.4**	**44.3**
SG&A	**4.2**	**8.1**	**15.8**	**15.9**	**16.0**	**43.3**	**49.6**	**51.5**	**53.3**	**54.1**	**55.0**	**55.9**	**56.8**	**57.7**	**58.6**	**58.5**	**58.4**	**50.1**	**48.1**	**46.3**	**44.7**
Total Operating Expenses	**13.8**	**35.5**	**66.7**	**67.9**	**69.0**	**107.9**	**142.9**	**177.0**	**203.8**	**227.5**	**227.8**	**230.0**	**232.5**	**235.4**	**238.5**	**240.8**	**233.3**	**170.7**	**166.5**	**101.8**	**96.7**
Operating Income (loss)	**(13.8)**	**(35.5)**	**(66.7)**	**(67.9)**	**(69.0)**	**(25.5)**	**151.4**	**358.1**	**582.1**	**799.9**	**915.5**	**941.7**	**968.3**	**995.3**	**1022.8**	**1051.9**	**995.2**	**546.5**	**533.9**	**3.6**	**(23.6)**
Total other expenses, net	(0.2)	(0.9)	(1.9)	(3.3)	(3.8)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net loss and comprehensive loss	(14.0)	(36.5)	(67.2)	(71.2)	(72.8)	(25.5)	151.4	358.1	582.1	799.9	915.5	941.7	968.3	995.3	1022.8	1051.9	995.2	546.5	533.9	3.6	(23.6)
Accretion of redeemable convertible stock	(0.2)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)
Pretax income	(14.2)	(36.6)	(67.3)	(71.3)	(72.9)	(25.6)	151.3	358.0	582.0	799.8	915.4	941.6	968.2	995.2	1022.7	1051.8	995.0	546.4	533.8	3.5	(23.7)
Income tax expense	0.0	0.0	0.0	0.0	0.0	0.0	24.2	64.4	193.8	279.9	320.4	329.6	338.9	348.3	357.9	368.1	348.3	191.2	186.8	0.7	(4.7)
Tax rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*16%*	*18%*	*33%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*20%*	*20%*
Net income (loss)	**(14.2)**	**(36.6)**	**(67.3)**	**(71.3)**	**(72.9)**	**(25.6)**	**127.1**	**293.6**	**388.2**	**519.9**	**595.0**	**612.0**	**629.3**	**646.9**	**664.7**	**683.7**	**646.8**	**355.2**	**347.0**	**2.8**	**(19.0)**
GAAP EPS																					
Basic	($19.53)	($3.00)	($2.51)	($2.23)	($2.21)	($0.75)	$3.63	$8.15	$10.47	$13.61	$15.12	$15.10	$15.07	$15.04	$15.01	$14.99	$13.76	$7.34	$6.96	$0.05	($0.36)
Diluted	($19.53)	($3.00)	($2.51)	($2.23)	($2.21)	($0.75)	$3.44	$7.71	$9.90	$12.87	$14.31	$14.29	$14.26	$14.23	$14.20	$14.18	$13.02	$6.94	$6.59	$0.05	($0.36)

Source: Company reports and RBC Capital Markets estimates

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Exhibit 5: Beloranib PWS NPV Analysis - US ($MM)

($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
Total US Beloranib PWS Sales	0.0	0.0	0.0	89.5	157.0	211.4	285.9	293.9	302.1	310.5	319.2	328.1	337.2	346.6	356.2	366.2	376.4	38.7	19.9
Total US Beloranib PWS Revenue	**0.0**	**0.0**	**0.0**	**89.5**	**157.0**	**211.4**	**285.9**	**293.9**	**302.1**	**310.5**	**319.2**	**328.1**	**337.2**	**346.6**	**356.2**	**366.2**	**376.4**	**38.7**	**19.9**
Total Paid Royalties for US PWS Beloranib Sales	0.0	0.0	0.0	7.2	12.6	16.9	22.9	14.7	15.1	15.5	16.0	16.4	16.9	17.3	17.8	18.3	18.8	1.9	1.0
COGS	0.0	0.0	0.0	10.7	18.8	25.4	31.5	32.3	30.2	31.1	31.9	32.8	33.7	34.7	35.6	36.6	37.6	3.9	2.0
R&D	7.1	6.5	6.6	6.7	6.8	6.9	7.0	6.3	5.6	5.1	4.6	4.1	3.7	3.3	3.0	2.7	2.4	2.2	2.0
G&A	4.0	4.0	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.8	4.9	5.0	5.1	5.2	5.3	5.4	5.5
Sales expense	0.0	0.0	0.0	7.5	8.0	8.2	8.3	8.5	8.7	8.8	9.0	9.2	9.4	9.6	9.8	8.8	7.9	7.1	6.4
Marketing expense	0.0	0.0	0.0	2.0	2.5	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	1.5	1.5	1.5	1.5
Tax adjusted EBIT	(11.0)	(10.5)	(10.6)	51.3	87.4	120.4	139.4	146.1	152.7	157.6	162.5	167.5	172.7	177.9	183.3	190.5	196.8	13.4	1.2
Tax rate	*0%*	*0%*	*0%*	*0%*	*16%*	*18%*	*33%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*20%*	*20%*
Beloranib sales free cash flow	**(11.0)**	**(10.5)**	**(10.6)**	**51.3**	**87.4**	**120.4**	**139.4**	**146.1**	**152.7**	**157.6**	**162.5**	**167.5**	**172.7**	**177.9**	**183.3**	**190.5**	**196.8**	**13.4**	**1.2**
Discount period	*0.79*	*1.79*	*2.79*	*3.79*	*4.79*	*5.79*	*6.79*	*7.79*	*8.79*	*9.79*	*10.79*	*11.79*	*12.79*	*13.79*	*14.79*	*15.79*	*16.79*	*17.79*	*18.79*
Discount factor	*0.93*	*0.84*	*0.77*	*0.70*	*0.63*	*0.58*	*0.52*	*0.48*	*0.43*	*0.39*	*0.36*	*0.33*	*0.30*	*0.27*	*0.24*	*0.22*	*0.20*	*0.18*	*0.17*
PV of Beloranib free cash flow	**(10.2)**	**(8.8)**	**(8.2)**	**35.8**	**55.4**	**69.4**	**73.0**	**69.5**	**66.1**	**62.0**	**58.1**	**54.5**	**51.0**	**47.8**	**44.8**	**42.3**	**39.7**	**2.5**	**0.2**

Discount Rate	10%
Perpetual Growth Rate	0%
Final year FCF	$0
Terminal Value	$0
Discount Factor	0.18
Present Value of Terminal Value	**$0**
Present Value of Cash Flows	**$745**
Present Value of Total Cash Flows	**$745**
Fully Diluted Shares Outstanding (MM)	28.5
NPV of US beloranib free cash flow	**$26.15**
Probability of success	***65%***
NPV of beloranib free cash flows (probability-adjusted)	**$17.00**

Source: RBC Capital Markets estimates

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Exhibit 6: Beloranib PWS NPV Analysis - EU ($MM)

($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
Total EU Beloranib PWS Sales	0.0	0.0	0.0	0.0	88.7	174.9	251.6	338.4	345.9	353.6	361.5	369.5	377.7	386.1	394.7	40.4	20.6	21.1	21.6
Total EU Beloranib PWS Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**88.7**	**174.9**	**251.6**	**338.4**	**345.9**	**353.6**	**361.5**	**369.5**	**377.7**	**386.1**	**394.7**	**40.4**	**20.6**	**21.1**	**21.6**
Total Paid Royalties for EU Beloranib Sales	0.0	0.0	0.0	0.0	7.1	14.0	20.1	16.9	17.3	17.7	18.1	18.5	18.9	19.3	19.7	2.0	1.0	1.1	1.1
COGS	0.0	0.0	0.0	0.0	10.6	21.0	27.7	37.2	34.6	35.4	36.1	37.0	37.8	38.6	39.5	4.0	2.1	2.1	2.2
R&D	7.1	6.5	6.6	6.7	6.8	6.9	7.0	6.3	5.6	5.1	4.6	4.1	3.7	3.3	3.0	2.7	2.4	2.2	2.0
G&A	4.0	4.0	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.8	4.9	5.0	5.1	5.2	5.3	5.4	5.5
Sales expense	0.0	0.0	0.0	2.5	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.2	3.8	3.4	3.1	2.8	2.5
Marketing expense	0.0	0.0	0.0	1.5	2.0	2.0	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5	1.0	1.0	1.0	1.0
Tax adjusted EBIT	(11.0)	(10.5)	(10.6)	(14.8)	45.3	100.6	123.9	173.4	180.1	184.6	189.1	193.8	198.4	203.6	208.7	14.3	3.7	5.3	5.9
Tax rate	*0%*	*0%*	*0%*	*0%*	*16%*	*18%*	*33%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*20%*	*20%*
Beloranib sales free cash flow	**(11.0)**	**(10.5)**	**(10.6)**	**(14.8)**	**45.3**	**100.6**	**123.9**	**173.4**	**180.1**	**184.6**	**189.1**	**193.8**	**198.4**	**203.6**	**208.7**	**14.3**	**3.7**	**5.3**	**5.9**
Discount period	*0.79*	*1.79*	*2.79*	*3.79*	*4.79*	*5.79*	*6.79*	*7.79*	*8.79*	*9.79*	*10.79*	*11.79*	*12.79*	*13.79*	*14.79*	*15.79*	*16.79*	*17.79*	*18.79*
Discount factor	*0.93*	*0.84*	*0.77*	*0.70*	*0.63*	*0.58*	*0.52*	*0.48*	*0.43*	*0.39*	*0.36*	*0.33*	*0.30*	*0.27*	*0.24*	*0.22*	*0.20*	*0.18*	*0.17*
PV of Beloranib free cash flow	**(10.2)**	**(8.8)**	**(8.2)**	**(10.3)**	**28.7**	**57.9**	**64.9**	**82.6**	**77.9**	**72.6**	**67.7**	**63.0**	**58.7**	**54.7**	**51.0**	**3.2**	**0.8**	**1.0**	**1.0**

Discount Rate	10%
Perpetual Growth Rate	0%
Final year FCF	$0
Terminal Value	$0
Discount Factor	0.18
Present Value of Terminal Value	**$0**
Present Value of Cash Flows	**$648**
Present Value of Total Cash Flows	**$648**
Fully Diluted Shares Outstanding (MM)	28.5
NPV of beloranib free cash flow	**$22.75**
Probability of success	***65%***
NPV of beloranib free cash flows (probability-adjusted)	**$14.79**

Source: RBC Capital Markets estimates

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Exhibit 7: Beloranib HIAO NPV Analysis - US ($MM)

($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
Total US Beloranib HIAO Sales	0.0	0.0	0.0	0.0	74.2	128.8	186.3	251.9	258.9	266.2	273.6	281.2	289.0	297.1	305.3	313.9	322.6	33.2	17.0
Total US Beloranib HIAO Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**74.2**	**128.8**	**186.3**	**251.9**	**258.9**	**266.2**	**273.6**	**281.2**	**289.0**	**297.1**	**305.3**	**313.9**	**322.6**	**33.2**	**17.0**
Total Paid Royalties for US Beloranib Sales	0.0	0.0	0.0	0.0	5.9	10.3	14.9	12.6	12.9	13.3	13.7	14.1	14.5	14.9	15.3	15.7	16.1	1.7	0.9
COGS	0.0	0.0	0.0	0.0	8.9	15.5	20.5	27.7	25.9	26.6	27.4	28.1	28.9	29.7	30.5	31.4	32.3	3.3	1.7
R&D	7.1	6.5	6.6	6.7	6.8	6.9	7.0	6.3	5.6	5.1	4.6	4.1	3.7	3.3	3.0	2.7	2.4	2.2	2.0
G&A	4.0	4.0	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.8	4.9	5.0	5.1	5.2	5.3	5.4	5.5
Sales expense	0.0	0.0	0.0	7.5	8.0	8.2	8.3	8.5	8.7	8.8	9.0	9.2	9.4	9.6	9.8	8.8	7.9	7.1	6.4
Marketing expense	0.0	0.0	0.0	2.0	2.5	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	3.0	1.5	1.5	1.5	1.5
Tax adjusted EBIT	(11.0)	(10.5)	(10.6)	(20.3)	31.8	66.2	85.6	123.1	128.9	133.1	137.3	141.7	146.1	150.6	155.2	161.6	167.1	9.6	(0.7)
Tax rate	*0%*	*0%*	*0%*	*0%*	*16%*	*18%*	*33%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*20%*	*20%*
Beloranib sales free cash flow	**(11.0)**	**(10.5)**	**(10.6)**	**(20.3)**	**31.8**	**66.2**	**85.6**	**123.1**	**128.9**	**133.1**	**137.3**	**141.7**	**146.1**	**150.6**	**155.2**	**161.6**	**167.1**	**9.6**	**(0.7)**
Discount period	*0.79*	*1.79*	*2.79*	*3.79*	*4.79*	*5.79*	*6.79*	*7.79*	*8.79*	*9.79*	*10.79*	*11.79*	*12.79*	*13.79*	*14.79*	*15.79*	*16.79*	*17.79*	*18.79*
Discount factor	*0.93*	*0.84*	*0.77*	*0.70*	*0.63*	*0.58*	*0.52*	*0.48*	*0.43*	*0.39*	*0.36*	*0.33*	*0.30*	*0.27*	*0.24*	*0.22*	*0.20*	*0.18*	*0.17*
PV of Beloranib free cash flow	**(10.2)**	**(8.8)**	**(8.2)**	**(14.1)**	**20.2**	**38.1**	**44.8**	**58.6**	**55.8**	**52.4**	**49.1**	**46.1**	**43.2**	**40.5**	**37.9**	**35.9**	**33.7**	**1.8**	**(0.1)**

Discount Rate	10%
Perpetual Growth Rate	0%
Final year FCF	$0
Terminal Value	$0
Discount Factor	0.18
Present Value of Terminal Value	**$0**
Present Value of Cash Flows	**$517**
Present Value of Total Cash Flows	**$517**
Fully Diluted Shares Outstanding (MM)	28.5
NPV of US beloranib free cash flow	**$18.13**
Probability of success	***60%***
NPV of beloranib free cash flows (probability-adjusted)	**$10.88**

Source: RBC Capital Markets estimates

Exhibit 8: Beloranib HIAO NPV Analysis - EU ($MM)

($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
Total EU Beloranib HIAO Sales	0.0	0.0	0.0	0.0	0.0	66.6	130.5	197.2	296.5	303.1	309.8	316.7	323.8	331.0	236.8	34.6	17.7	18.1	18.5
Total EU Beloranib Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**66.6**	**130.5**	**197.2**	**296.5**	**303.1**	**309.8**	**316.7**	**323.8**	**331.0**	**236.8**	**34.6**	**17.7**	**18.1**	**18.5**
Total Paid Royalties for EU Beloranib Sales	0.0	0.0	0.0	0.0	0.0	5.3	10.4	9.9	14.8	15.2	15.5	15.8	16.2	16.5	11.8	1.7	0.9	0.9	0.9
COGS	0.0	0.0	0.0	0.0	0.0	8.0	14.4	21.7	29.6	30.3	31.0	31.7	32.4	33.1	23.7	3.5	1.8	1.8	1.8
R&D	7.1	6.5	6.6	6.7	6.8	6.9	7.0	6.3	5.6	5.1	4.6	4.1	3.7	3.3	3.0	2.7	2.4	2.2	2.0
G&A	4.0	4.0	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.8	4.9	5.0	5.1	5.2	5.3	5.4	5.5
Sales expense	0.0	0.0	0.0	2.5	4.0	4.1	4.2	4.2	4.3	4.4	4.5	4.6	4.7	4.2	3.8	3.4	3.1	2.8	2.5
Marketing expense	0.0	0.0	0.0	1.5	2.0	2.0	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5	1.0	1.0	1.0	1.0
Tax adjusted EBIT	(11.0)	(10.5)	(10.6)	(14.8)	(14.3)	29.6	58.5	96.4	152.8	156.7	160.6	164.6	168.6	173.1	121.5	11.1	2.1	3.2	3.8
Tax rate	*0%*	*0%*	*0%*	*0%*	*16%*	*18%*	*33%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*20%*	*20%*
Beloranib sales free cash flow	**(11.0)**	**(10.5)**	**(10.6)**	**(14.8)**	**(14.3)**	**29.6**	**58.5**	**96.4**	**152.8**	**156.7**	**160.6**	**164.6**	**168.6**	**173.1**	**121.5**	**11.1**	**2.1**	**3.2**	**3.8**
Discount period	*0.79*	*1.79*	*2.79*	*3.79*	*4.79*	*5.79*	*6.79*	*7.79*	*8.79*	*9.79*	*10.79*	*11.79*	*12.79*	*13.79*	*14.79*	*15.79*	*16.79*	*17.79*	*18.79*
Discount factor	*0.93*	*0.84*	*0.77*	*0.70*	*0.63*	*0.58*	*0.52*	*0.48*	*0.43*	*0.39*	*0.36*	*0.33*	*0.30*	*0.27*	*0.24*	*0.22*	*0.20*	*0.18*	*0.17*
PV of Beloranib free cash flow	**(10.2)**	**(8.8)**	**(8.2)**	**(10.3)**	**(9.0)**	**17.0**	**30.7**	**45.9**	**66.1**	**61.6**	**57.5**	**53.5**	**49.9**	**46.5**	**29.7**	**2.5**	**0.4**	**0.6**	**0.6**

Discount Rate	10%
Perpetual Growth Rate	0%
Final year FCF	$0
Terminal Value	$0
Discount Factor	0.18
Present Value of Terminal Value	**$0**
Present Value of Cash Flows	**$416**
Present Value of Total Cash Flows	**$416**
Fully Diluted Shares Outstanding (MM)	28.5
NPV of beloranib free cash flow	**$14.60**
Probability of success	***60%***
NPV of beloranib free cash flows (probability-adjusted)	**$8.76**

Source: RBC Capital Markets estimates

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Exhibit 9: US Beloranib PWS Revenue Model ($MM)

Beloranib PWS Revenue Model ($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
US Beloranib PWS Revenue Model																			
US population	321,347,572	323,821,948	326,315,378	328,828,006	331,359,982	333,911,453	336,482,572	339,073,487	341,684,353	344,315,323	346,966,551	349,638,193	352,330,407	355,043,351	357,777,185	360,532,070	363,308,166	366,105,639	368,924,653
Population growth	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*
PWS prevalence	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*
# of estimated PWS cases	21,423	21,588	21,754	21,922	22,091	22,261	22,432	22,605	22,779	22,954	23,131	23,309	23,489	23,670	23,852	24,035	24,221	24,407	24,595
% of PWS cases that have been identified	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
# of identified PWS cases	7,498	7,556	7,614	7,673	7,732	7,791	7,851	7,912	7,973	8,034	8,096	8,158	8,221	8,284	8,348	8,412	8,477	8,542	8,608
% of PWS patients ≥12 years old	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
# of PWS patients ≥12 years old	3,749	3,778	3,807	3,836	3,866	3,896	3,926	3,956	3,986	4,017	4,048	4,079	4,111	4,142	4,174	4,206	4,239	4,271	4,304
Beloranib penetration	*0%*	*0%*	*0%*	*17%*	*29%*	*38%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*5%*	*3%*
# of PWS patients treated with beloranib	0	0	0	652	1,121	1,480	1,963	1,978	1,993	2,009	2,024	2,040	2,055	2,071	2,087	2,103	2,119	214	108
Annual revenue/patient				$137,280	$140,026	$142,826	$145,683	$148,596	$151,568	$154,600	$157,692	$160,845	$164,062	$167,344	$170,690	$174,104	$177,586	$181,138	$184,761
Total US beloranib sales in PWS ($MM)			**$0**	**$90**	**$157**	**$211**	**$286**	**$294**	**$302**	**$311**	**$319**	**$328**	**$337**	**$347**	**$356**	**$366**	**$376**	**$39**	**$20**

Source: RBC Capital Markets estimates

Exhibit 10: EU Beloranib PWS Revenue Model ($MM)

EU Beloranib PWS Revenue Model	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
EU population	512,559,969	513,687,601	514,817,713	515,950,312	517,085,403	518,222,991	519,363,081	520,505,680	521,650,793	522,798,424	523,948,581	525,101,268	526,256,491	527,414,255	528,574,566	529,737,430	530,902,853	532,070,839	533,241,395
Population growth	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*
PWS prevalence	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*	*0.000067*
# of estimated PWS cases	34,171	34,246	34,321	34,397	34,472	34,548	34,624	34,700	34,777	34,853	34,930	35,007	35,084	35,161	35,238	35,316	35,394	35,471	35,549
% of PWS cases that have been identified	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*	*35%*
# of identified PWS cases	11,960	11,986	12,012	12,039	12,065	12,092	12,118	12,145	12,172	12,199	12,225	12,252	12,279	12,306	12,333	12,361	12,388	12,415	12,442
% of PWS patients ≥12 years old	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
# of PWS patients ≥12 years old	5,980	5,993	6,006	6,019	6,033	6,046	6,059	6,073	6,086	6,099	6,113	6,126	6,140	6,153	6,167	6,180	6,194	6,207	6,221
Beloranib penetration	*0%*	*0%*	*0%*	*0%*	*14%*	*27%*	*38%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*5%*	*3%*	*3%*	*3%*
# of PWS patients treated with beloranib	0	0	0	0	845	1,632	2,303	3,036	3,043	3,050	3,056	3,063	3,070	3,077	3,083	309	155	155	156
Annual revenue/patient					$105,019	$107,120	$109,262	$111,447	$113,676	$115,950	$118,269	$120,634	$123,047	$125,508	$128,018	$130,578	$133,190	$135,854	$138,571
Total EU beloranib sales in PWS ($MM)				**$0**	**$89**	**$175**	**$252**	**$338**	**$346**	**$354**	**$361**	**$370**	**$378**	**$386**	**$395**	**$40**	**$21**	**$21**	**$22**

Source: RBC Capital Markets estimates

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Exhibit 11: US Beloranib HIAO Revenue Model ($MM)

Beloranib HIAO Revenue Model ($MM)	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
US Beloranib HIAO Revenue Model																			
US population	321,347,572	323,821,948	326,315,378	328,828,006	331,359,982	333,911,453	336,482,572	339,073,487	341,684,353	344,315,323	346,966,551	349,638,193	352,330,407	355,043,351	357,777,185	360,532,070	363,308,166	366,105,639	368,924,653
Population growth	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*	*0.77%*
craniopharyngioma prevalence	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*
# of estimated craniopharyngioma patients	6,427	6,476	6,526	6,577	6,627	6,678	6,730	6,781	6,834	6,886	6,939	6,993	7,047	7,101	7,156	7,211	7,266	7,322	7,378
% of craniopharyngioma cases who develop obesity (HIAO)	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
# of HIAO patients	3,213	3,238	3,263	3,288	3,314	3,339	3,365	3,391	3,417	3,443	3,470	3,496	3,523	3,550	3,578	3,605	3,633	3,661	3,689
Beloranib penetration	*0%*	*0%*	*0%*	*0%*	*16%*	*27%*	*38%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*5%*	*3%*
# of HIAO patients treated with beloranib	0	0	0	0	530	902	1,279	1,695	1,708	1,722	1,735	1,748	1,762	1,775	1,789	1,803	1,817	183	92
Annual revenue/patient				$137,280	$140,026	$142,826	$145,683	$148,596	$151,568	$154,600	$157,692	$160,845	$164,062	$167,344	$170,690	$174,104	$177,586	$181,138	$184,761
Total US beloranib sales in HIAO ($MM)			**$0**	**$0**	**$74**	**$129**	**$186**	**$252**	**$259**	**$266**	**$274**	**$281**	**$289**	**$297**	**$305**	**$314**	**$323**	**$33**	**$17**

Source: RBC Capital Markets estimates

Exhibit 12: EU Beloranib HIAO Revenue Model ($MM)

EU Beloranib HIAO Revenue Model	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
EU population	512,559,969	513,687,601	514,817,713	515,950,312	517,085,403	518,222,991	519,363,081	520,505,680	521,650,793	522,798,424	523,948,581	525,101,268	526,256,491	527,414,255	528,574,566	529,737,430	530,902,853	532,070,839	533,241,395
Population growth	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*	*0.22%*
craniopharyngioma prevalence	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*	*0.00002*
# of estimated craniopharyngioma patients	10,251	10,274	10,296	10,319	10,342	10,364	10,387	10,410	10,433	10,456	10,479	10,502	10,525	10,548	10,571	10,595	10,618	10,641	10,665
% of craniopharyngioma cases who develop obesity (HIAO)	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*
# of HIAO patients	5,126	5,137	5,148	5,160	5,171	5,182	5,194	5,205	5,217	5,228	5,239	5,251	5,263	5,274	5,286	5,297	5,309	5,321	5,332
Beloranib penetration	*0%*	*0%*	*0%*	*0%*	*0%*	*12%*	*23%*	*34%*	*50%*	*50%*	*50%*	*50%*	*50%*	*50%*	*35%*	*5%*	*3%*	*3%*	*3%*
# of HIAO patients treated with beloranib	0	0	0	0	0	622	1,195	1,770	2,608	2,614	2,620	2,626	2,631	2,637	1,850	265	133	133	133
Annual revenue/patient					$105,019	$107,120	$109,262	$111,447	$113,676	$115,950	$118,269	$120,634	$123,047	$125,508	$128,018	$130,578	$133,190	$135,854	$138,571
Total EU beloranib sales in HIAO ($MM)				**$0**	**$0**	**$67**	**$131**	**$197**	**$296**	**$303**	**$310**	**$317**	**$324**	**$331**	**$237**	**$35**	**$18**	**$18**	**$18**

Source: RBC Capital Markets estimates

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Valuation

To value ZFGN shares at $58, we use a sum-of-the-parts methodology, and estimate the probability adjusted NPV of the following: 1) beloranib sales in PWS ($32/share), 2) beloranib sales in HIAO ($20/share), and 3) the company's projected net cash position (~$6/share). We assign a probability of success of 65% for PWS and 60% for HIAO, and model peak US/EU sales of beloranib in PWS and HIAO of $750MM and $630MM, respectively.

Price target impediments

As the majority of Zafgen's value lies with the beloranib asset, and particularly in the rare disease indications, any further delays to the filing timeline for the beloranib would negatively affect our valuation. Other factors that can negatively influence our valuation include increased competition, regulatory setbacks, and lower than projected penetrations for beloranib. Factors that can positively affect our valuation include more favorable pricing, positive data from other early-stage Zafgen programs (such as ZGN-839 or a new compound for general obese individuals), and M&A, which we have not included in our valuation.

Company description

Zafgen is a clinical-stage biotechnology company developing MetAP2 inhibitors for the treatment of individuals with obesity. The company went public by issuing a $110MM IPO in June 2014 to fund its ongoing clinical trials for its lead compound beloranib, a first-in-class MetAP2 inhibitor. Beloranib is currently in clinical trials to treat patients with Prader-Willi Syndrome (PWS), hypothalamic injury (HIAO), and general obesity. Zafgen's pipeline also includes an oral MetAP2 inhibitor, ZGN-839, in preclinical studies for the treatment of nonalcoholic steatohepatitis (NASH) and type 2 diabetes. Founded in 2005, Zafgen is based in Cambridge, MA and currently has 24 full-time employees.

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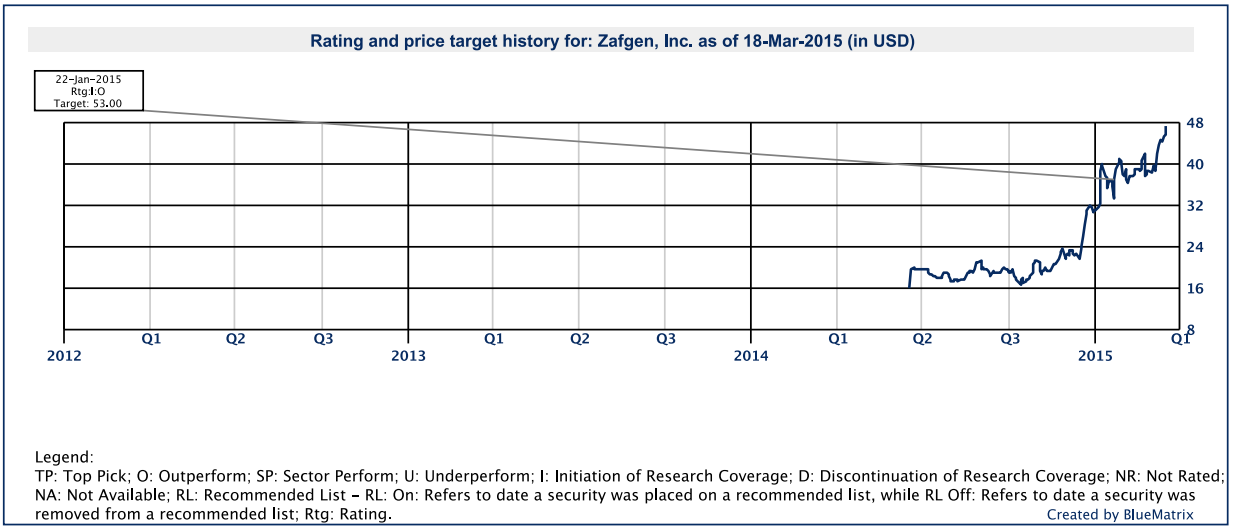

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