



May 12, 2015

## Zafgen Reports First Quarter 2015 Financial Results

*Quarter Highlighted by Accelerated Enrollment in bestPWS Clinical Trial - Six Month Data Now Expected in Early Q1 2016*

*Company on Track to Report Data from Ongoing ZAF-203 Clinical Trial in Late 2015/Very Early 2016*

*Ends Quarter with Cash and Marketable Securities of \$234 Million Following Recent Follow-on Offering*

BOSTON, May 12, 2015 (GLOBE NEWSWIRE) -- Zafgen, Inc. (Nasdaq:ZFGN), a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity and complex metabolic disorders, today announced its first quarter 2015 financial results.

### Zafgen Q1 2015 Business Highlights

- Announced an accelerated rate of patient enrollment in recent months in the bestPWS (Beloranib Efficacy Safety and Tolerability in PWS) clinical trial in patients with a rare genetic disorder, Prader-Willi syndrome (PWS) and has reached the original patient recruitment target of 102 patients. Enrollment closeout is expected to be completed in the near term and the total patients in the trial will slightly exceed the original 102 patient goal. Release of top line results is now expected to occur early in the first quarter of 2016.
- Based on constructive ongoing dialogue with the U.S. Food and Drug Administration (FDA), the Company has finalized its plan regarding the statistical analysis of primary efficacy endpoints for the bestPWS clinical trial. Both hyperphagia-related behaviors and body weight will be co-primary efficacy endpoints and will be evaluated at a significance level of p-value less than 0.05. With 102 patients enrolled, the trial is adequately powered to demonstrate these effects of beloranib.
- Enrolled approximately two-thirds of a target 150 patients in the Phase 2b clinical trial (ZAF-203) to establish the long-term weight loss benefits of MetAP2 inhibitor treatment with beloranib in patients with severe obesity complicated by type 2 diabetes, which began randomized treatment in December 2014. The Company remains on track to release six-month interim data in this subset of patients in late 2015 or very early 2016.
- In January 2015, announced positive results from a Phase 2 clinical trial of beloranib in patients with hypothalamic injury-associated obesity (HIAO), a rare, severe, and difficult-to-treat form of obesity. The Company also announced new weight loss and safety data from this Phase 2 clinical trial of beloranib in HIAO at the Endocrine Society Annual Meeting (ENDO 2015). Results from this trial showed that after four weeks of treatment, patients randomized to beloranib lost on average 3.40 kg of body weight vs. 0.25 kg on average for patients randomized to placebo (p=0.01). Results from the open-label extension phase of the trial suggested no apparent waning of the effect of beloranib over eight weeks of treatment.
- Raised approximately \$130 million through a follow-on public offering in January 2015 which included the full-exercise of the underwriters' option to purchase additional shares of common stock.

"We are very pleased with our accomplishments for the first quarter of 2015. We are thrilled to have seen a high rate of enrollment at our most recently-opened bestPWS clinical trial sites, which will enable us to release six month data from our first pivotal Phase 3 clinical trial early in the first quarter of 2016," said Dr. Thomas Hughes, Chief Executive Officer of Zafgen. "We are grateful to the patients and their families for their enthusiasm and commitment to making the bestPWS trial possible."

"With enrollment progressing well in our Phase 2b trial (ZAF-203) in patients with severe obesity complicated by type 2 diabetes, we remain on track to release important six month interim data in late 2015 or very early 2016," said Dr. Dennis Kim, Chief Medical Officer of Zafgen. "The ZAF-203 trial will provide our first look at the effects of beloranib treatment on both body weight and glycemic control in the setting of type 2 diabetes, an important and difficult to treat co-morbidity of obesity."

"We were pleased to have completed our recent follow-on offering in January 2015, bringing in total net proceeds of approximately \$130 million and ending the current quarter with a total cash position of approximately \$234 million," said Patricia Allen, Chief Financial Officer of Zafgen. "This is an incredibly productive and exciting time for Zafgen and we are investing this capital in the development of beloranib in multiple indications, along with advancing our ZAF-839 program in nonalcoholic steatohepatitis, or NASH, and our second-generation molecules in obesity. We are also expanding our organization to deliver on our multiple development programs and establish our commercial operations in anticipation of approval of beloranib in Prader-Willi syndrome."

### First Quarter 2015 Financial Results

## ***Cash and Cash Equivalents and Marketable Securities***

As of March 31, 2015, the Company had cash and cash equivalents and marketable securities totaling \$234.2 million.

In January 2015, the Company sold an aggregate of 3,942,200 shares of common stock through an underwritten public offering at \$35.00 per share, before underwriting discounts, which included the full-exercise of the underwriters' option to purchase an additional 514,200 shares of common stock at \$35.00 per share. The net proceeds to the Company were approximately \$129.6 million.

## ***Net Loss***

The Company reported a net loss for the first quarter of 2015 of \$13.5 million, or \$0.53 per share, compared to a \$4.5 million net loss, or \$6.18 per share, for the first quarter of 2014. The weighted average common shares (basic and diluted) outstanding used to compute net loss per share were 25,615,282 for the first quarter of 2015, compared to 729,391 for the first quarter of 2014.

## ***Research and Development Expenses***

Research and development expenses for the first quarter of 2015 were \$10.2 million, compared to \$3.3 million for the first quarter of 2014. The increase in research and development expenses for the quarter ended March 31, 2015 as compared to the first quarter of 2014 was primarily due to increased costs associated with the advancement of the Company's beloranib program, ZGN-839 and other early stage development programs (consisting of our second-generation MetAP2 inhibitors).

## ***General and Administrative Expenses***

General and administrative expenses for the first quarter of 2015 were \$3.0 million, compared to \$1.2 million for the first quarter of 2014. The increase in general and administrative expenses for the quarter ended March 31, 2015 as compared to the first quarter of 2014 was primarily due to increased personnel related costs, increased public company costs, increased travel and other related costs, and increased professional fees.

## ***2015 Financial Guidance***

The Company continues to expect that its cash and cash equivalents and marketable securities balance will be greater than \$145.0 million at December 31, 2015.

## ***Conference Call Information***

Zafgen will host an investor conference call today, May 12, 2015 at 4:30 p.m., Eastern Time, to discuss the Company's first quarter 2015 results as well as other forward-looking information about Zafgen's business. Investors and other interested parties may participate by dialing 844-824-7428 in the United States or 973-500-2177 outside the United States. The call will also be webcast live on the Company's website at <http://ir.zafgen.com/events.cfm>. You can access the replay for seven days by dialing 855-859-2056 in the United States and 404-537-3406 outside the United States and referencing conference ID number 42668578.

## ***About Beloranib***

Beloranib is a novel, first-in-class injectable small molecule therapy with a unique mechanism of action that reduces hunger while stimulating the use of stored fat as an energy source. Beloranib is a potent inhibitor of MetAP2, an enzyme that modulates the activity of key cellular processes that control metabolism. MetAP2 inhibitors work, at least in part, by directing MetAP2 binding to cellular stress mediators, and, thus, reducing the tone of signals that drive lipid synthesis by the liver and fat storage throughout the body. In this manner, MetAP2 inhibition increases metabolism of fat as an energy source. Zafgen holds exclusive worldwide rights (exclusive of South Korea) for the development and commercialization of beloranib. Zafgen exclusively licensed beloranib from Chong Kun Dang Pharmaceutical Corporation (CKD Pharma) of South Korea.

## ***About Prader-Willi Syndrome***

Prader-Willi syndrome (PWS), the most common known genetic cause of life-threatening obesity, results in constant and unrelenting hunger that drives patients with PWS to engage in problematic hunger-related behaviors and to gain excessive weight. As a result, many of those affected with PWS become morbidly obese and suffer significant mortality. Currently, there is no cure for this disease. Although the cause of PWS is complex, it results from a deletion or loss of function of a cluster of genes on the 15th chromosome. PWS typically causes low muscle mass and function, short stature, incomplete sexual development, and a chronic feeling of hunger that, when coupled with a metabolism that utilizes drastically fewer calories than

normal, can lead to excessive eating and life-threatening obesity. PWS occurs in males and females equally and in all races, with the same incidence around the world. Prevalence estimates have ranged from 1:8,000 to 1:50,000.

### **About Hypothalamic Injury-Associated Obesity (HIAO)**

When the hypothalamus, a small area of the brain responsible for many hormonal and metabolic functions including the desire to eat, is injured, a syndrome of intractable weight gain can ensue, resulting in severe obesity and a poor quality of life. This rare and complicated medical condition occurs in affected individuals most commonly due to a benign central nervous system tumor called craniopharyngioma, which presents as a mass in or near the hypothalamus. When the tumor is treated with surgical resection and radiation therapy, the hypothalamus often becomes severely damaged and/or dysfunctional, which can result in loss of appetite control and reduction in metabolic rate. Craniopharyngioma-associated obesity incidence estimates have ranged from 0.13 to 0.17 per 100,000 per year. Other comparably located tumors such as pituitary macroadenoma, medulloblastoma, and pineal germinoma, affect a smaller number of patients, but patients with these tumors can have a similar clinical presentation with respect to obesity. Rarely, this form of obesity also has been reported in cases of head trauma or stroke leading to injury to the hypothalamus.

### **About Zafgen**

Zafgen (Nasdaq:ZFGN) is a biopharmaceutical company dedicated to significantly improving the health and well-being of patients affected by obesity and complex metabolic disorders. Zafgen is focused on developing novel therapeutics that treat the underlying biological mechanisms through the MetAP2 pathway. Beloranib, Zafgen's lead product candidate, is a novel, first-in-class, twice-weekly subcutaneous injection being developed for the treatment of multiple indications, including severe obesity in two rare diseases, Prader-Willi syndrome, hypothalamic injury-associated obesity, including craniopharyngioma-associated obesity; and severe obesity in the general population. Zafgen is also developing ZGN-839, a liver-targeted MetAP2 inhibitor, for the treatment of nonalcoholic steatohepatitis, or NASH, and abdominal obesity. Zafgen aspires to improve the lives of patients through targeted treatments and has assembled a team accomplished in bringing therapies to patients with both rare and prevalent metabolic diseases.

### **Safe Harbor Statement**

Various statements in this release concerning Zafgen's future expectations, plans and prospects, including without limitation, Zafgen's expectations regarding beloranib as a treatment for PWS, HIAO and other forms of severe obesity, its expectations with respect to the timing and success of its clinical trials of beloranib and other product candidates, its plans regarding commercialization of beloranib and its expectations relating to available cash and cash equivalents and marketable securities at the end of 2015 may constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements can be identified by terminology such as "anticipate," "believe," "could," "could increase the likelihood," "estimate," "expect," "intend," "is planned," "may," "should," "will," "will enable," "would be expected," "look forward," "may provide," "would" or similar terms, variations of such terms or the negative of those terms. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Zafgen's ability to successfully demonstrate the efficacy and safety of its product candidates, the pre-clinical and clinical results for its product candidates, which may not support further development of such product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials, obtaining, maintaining and protecting intellectual property, Zafgen's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties, competition from others developing products for similar uses, Zafgen's ability to manage operating expenses, Zafgen's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, Zafgen's dependence on third parties for development, manufacture, marketing, sales and distribution of products, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Zafgen's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in Zafgen's subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent Zafgen's views only as of today and should not be relied upon as representing its views as of any subsequent date. Zafgen explicitly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

**ZAFGEN, INC.**

#### **CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

(In thousands, except share and per share data)

(Unaudited)

**Three Months Ended March 31,**

<b>2015</b>	<b>2014</b>
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Revenue	\$ —	\$ —
Operating expenses:		
Research and development	10,215	3,275
General and administrative	3,025	1,246
Total operating expenses	<u>13,240</u>	<u>4,521</u>
Loss from operations	<u>(13,240)</u>	<u>(4,521)</u>
Other income (expense):		
Interest income	39	—
Interest expense	(213)	(2)
Foreign currency transaction gains (losses), net	<u>(58)</u>	<u>65</u>
Total other income (expense), net	<u>(232)</u>	<u>63</u>
Net loss	(13,472)	(4,458)
Accretion of redeemable convertible preferred stock to redemption value	—	(49)
Net loss attributable to common stockholders	<u>\$ (13,472)</u>	<u>\$ (4,507)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.53)</u>	<u>\$ (6.18)</u>
Weighted average common shares outstanding, basic and diluted	<u>25,615,282</u>	<u>729,391</u>

**ZAFGEN, INC.**  
**CONSOLIDATED BALANCE SHEETS**  
(In thousands, except share and per share data)  
(Unaudited)

	<u>March 31,</u>	<u>December 31,</u>
	<u>2015</u>	<u>2014</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 106,934	\$ 58,103
Marketable securities	127,267	57,359
Tax incentive receivable	391	391
Prepaid expenses and other current assets	<u>1,734</u>	<u>1,345</u>
Total current assets	236,326	117,198
Property and equipment, net	101	79
Tax incentive receivable	205	—
Other assets	<u>89</u>	<u>242</u>
Total assets	<u>\$ 236,721</u>	<u>\$ 117,519</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 3,404	\$ 2,348
Accrued expenses	4,034	3,172
Notes payable, current	<u>2,093</u>	<u>1,381</u>
Total current liabilities	9,531	6,901
Notes payable, net of discount, long-term	<u>5,521</u>	<u>6,177</u>
Total liabilities	<u>15,052</u>	<u>13,078</u>
Commitments and contingencies		
Stockholders' equity:		
Preferred stock; \$0.001 par value; 5,000,000 shares authorized at March 31, 2015 and December 31, 2014; no shares issued and outstanding at March 31, 2015 and December 31, 2014	—	—

Common stock, \$0.001 par value; 115,000,000 shares authorized at March 31, 2015 and December 31, 2014; 26,856,329 and 22,879,160 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively

	27	23
Additional paid-in capital	340,538	209,838
Accumulated deficit	(118,857)	(105,385)
Accumulated other comprehensive loss	<u>(39)</u>	<u>(35)</u>
Total stockholders' equity	<u>221,669</u>	<u>104,441</u>
Total liabilities and stockholders' equity	<u>\$ 236,721</u>	<u>\$ 117,519</u>

This selected financial information should be read in conjunction with the consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K which includes the audited consolidated financial statements for the year ended December 31, 2014.

CONTACT: Zafgen, Inc.

Patricia Allen

Chief Financial Officer

617-648-9792

FTI Consulting

Kimberly Ha

Investor Relations

(212) 850-5612

Kimberly.Ha@fticonsulting.com

Shauna Elkin

Media Relations

(212) 850-5613

Shauna.Elkin@fticonsulting.com