Zafgen Reports Third Quarter 2018 Operating and Financial Results

November 7, 2018

ZGN-1061 1.8 mg cohort for the Phase 2 proof-of-concept trial now fully enrolled; on track for topline results early 2019

ZGN-1258 nonclinical safety and efficacy data sets presented at Foundation for Prader-Willi Research (FPWR) Annual Conference

'PATH for PWS' natural history study collaboration with FPWR opened for participation; robust enrollment seen in first month

Oral, liver targeted MetAP2 inhibitor ZGN-1345 formally named a development candidate

Company to host conference call today at 4:30 PM ET

BOSTON, Nov. 07, 2018 (GLOBE NEWSWIRE) -- Zafgen, Inc. (Nasdaq:ZFGN), a clinical-stage biopharmaceutical company leveraging its proprietary knowledge of MetAP2 systems biology to develop novel therapies for patients affected by a range of metabolic diseases, today reported its third quarter 2018 operating and financial results.

"We are excited this quarter about the emerging potential of our broader pipeline," said Jeffrey Hatfield, Chief Executive Officer. "In addition to the steady progress of ZGN-1061, we have also made solid progress on ZGN-1258's path towards the clinic and advanced an oral, novel MetAP2 inhibitor, ZGN-1345, to development candidate status. Further, the resurgence of our active engagement with the Prader-Willi community, including numerous meaningful interactions at the FPWR annual conference last month, is a tremendous step forward in our heartfelt mission to help families affected by PWS."

Recent Corporate and Program Highlights

ZGN-1061

- Enrollment for the additional 1.8 mg dose cohort is complete and exceeded our targets. As previously announced, Zafgen initiated this additional cohort of its Phase 2 proof-of-concept clinical trial to explore full MetAP2 target engagement efficacy. Data from this cohort are on track for topline results in early 2019.

ZGN-1258

- Zafgen presented three ZGN-1258 posters on nonclinical efficacy and safety at the FPWR Annual Conference October 4-6th, 2018. The data presented include:
  - ZGN-1258 effects on hyperphagia and obesity, demonstrating nonclinical efficacy results replicating those previously shown with Zafgen's prior molecule for Prader-Willi syndrome (PWS), and directly aligned with the primary pathologies of PWS
  - Novel ZGN-1258 effects on additional behavioral manifestations commonly observed in PWS, such as anxiety and obsessive-compulsive behaviors
  - ZGN-1258 differentiation versus prior compound on nonclinical safety measures including cardiovascular (CV) measures

- PATH for PWS, Zafgen's natural history study collaboration with FPWR, opened enrollment at the FPWR annual conference. There is high interest and engagement from the PWS community around this initiative, and enrollment has been robust since opening, with approximately 190 of the 500-participant goal enrolled in the first month.

- Zafgen has successfully completed all of the investigational new drug application (IND) enabling studies necessary for submission of an IND to the U.S. Food and Drug Administration (FDA), with the exception of one in which the contract research organization (CRO) improperly conducted the study. Zafgen is in the process of completing this last requirement with a different CRO, and now expects the IND allowance and Phase 1 start date in the first quarter of 2019.

ZGN-1345

- Zafgen announced that ZGN-1345, an orally dosed MetAP2 inhibitor specifically targeting the liver, has been formally advanced to development candidate stage as a differentiated third asset within the Company's pipeline. Nonclinical models have shown positive preliminary results in multiple liver disease indications.

Corporate

- On July 2, 2018, Zafgen closed an underwritten public offering of 9.2 million shares of its common stock at a price to the public of $7.50 per share, including 1.2 million shares pursuant to the underwriters' exercise in full of their option to

purchase additional common shares. The offering resulted in total gross proceeds of $69.0 million and net proceeds of approximately $64.6 million, after deducting the underwriting discounts and commissions and other offering costs.

- Zafgen has continued to add to its management team with key hires that bring significant expertise to areas of focus for the Company including:
  - Lisa Percival, Vice President of Regulatory, who brings nearly 20 years of global regulatory experience and previously co-led the Pediatric Center of Excellence at Bristol-Myers Squibb (BMS)
  - Erin Brubaker, Vice President of Commercial Strategy and Business Development, who brings 25 years of experience including global leadership roles in New Product Planning, Portfolio Strategy, Alliance Management and Commercial while at GlaxoSmithKline (GSK)

Third Quarter 2018 Financial Results

"We ended the quarter with a strong cash position of $127.8 million of cash, cash equivalents and marketable securities, which includes $64.6 million of net proceeds from our public offering of common stock which closed in early July 2018, and extends our runway to achieve several potential value-creating inflection points throughout our pipeline," said Patricia Allen, Chief Financial Officer.

Cash, Cash Equivalents and Marketable Securities

As of September 30, 2018, the Company had cash, cash equivalents and marketable securities totaling $127.8 million.

Net Loss

The Company reported a net loss for the third quarter of 2018 of $15.1 million, or $0.41 per share, compared to a net loss of $12.6 million, or $0.46 per share, for the third quarter of 2017.

The weighted average common shares (basic and diluted) outstanding used to compute net loss per share were 36,619,575 for the third quarter of 2018 compared to 27,483,550 for the same quarter of 2017.

Research and Development Expenses

Research and development expenses for the third quarter of 2018 were $11.8 million compared to $9.7 million for the third quarter of 2017. The increase in research and development expenses compared to the prior year period was primarily due to increased costs related to the ZGN-1258 program as IND enabling studies progressed during the quarter as the program advances towards the filing of an IND. There were also increases in personnel related costs and non-cash stock-based compensation expense in the third quarter of 2018 as compared to the third quarter of 2017. These increases in research and development costs were partially offset by a decrease in nonclinical and manufacturing costs associated with our ZGN-1061 program.

General and Administrative Expenses

General and administrative expenses for the third quarter of 2018 were $3.3 million, compared to $3.1 million for the third quarter of 2017. The increase in general and administrative expenses as compared to the prior year period was primarily due to an increase in personnel related costs, partially offset by a decrease in professional fees and non-cash stock-based compensation expense.

2018 Financial Guidance

The Company expects that its cash, cash equivalents and marketable securities balance will be greater than $100 million as of December 31, 2018.

Conference Call Information

Zafgen will host an investor conference call today, November 7, 2018 at 4:30 p.m., Eastern Time, to discuss the Company's third quarter 2018 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 and referencing conference ID number 7891309. The call will also be webcast live on the Company's website at https://zafgen.gcs-web.com/events-and-presentations. A replay of this conference call will be available beginning at 7:30 p.m. ET on November 7, 2018 through November 14, 2018 by dialing (855) 859-2056 in the United States or (404) 537-3406 outside the United States. To access the replay please provide Conference ID number 7891309.

About Zafgen

Zafgen (Nasdaq:ZFGN) is a clinical-stage biopharmaceutical company leveraging its proprietary MetAP2 biology platform to develop novel therapies for patients affected by complex metabolic diseases. Zafgen has pioneered the study of MetAP2 inhibitors in both common and rare metabolic disorders and is currently advancing programs for type 2 diabetes, Prader-Willi syndrome and liver diseases. The Company's lead product candidate, ZGN-1061, a MetAP2 inhibitor for difficult-to-control type 2 diabetes, has successfully completed the initial part of a Phase 2 clinical trial. Learn more at www.zafgen.com.

Safe Harbor Statement

Various statements in this release concerning Zafgen's future expectations, plans and prospects, including without limitation, Zafgen's expectations regarding the collection of medical history and medical events from PATH for PWS participants to inform development and clinical trial design for potential new treatments for PWS, including ZGN-1258, and the use of ZGN-1258, ZGN-1061, ZGN-1345 and other second-generation MetAP2 inhibitors as treatments for metabolic diseases including PWS, type 2 diabetes, liver diseases and obesity and Zafgen's expectations with respect to the timing and success of its ability to collect and analyze PATH for PWS data for development and clinical trial design and with respect to its nonclinical studies and clinical trials of ZGN-1258, ZGN-1061, ZGN-1345 and its other product candidates, may constitute forward-looking statements for purposes of the safe harbor provisions of 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 collect and analyze medical history and medical events from PATH for PWS participants, the capacity for such data to inform clinical trial design and potential areas for future study, Zafgen's ability to successfully demonstrate the efficacy and safety of ZGN-1258, ZGN-1061, ZGN-1345 and its other product candidates and to differentiate ZGN-1258, ZGN-1061, ZGN-1345 and its other product candidates from first generation MetAP2 inhibitors, such as beloranib, the nonclinical and clinical results for ZGN-1258, ZGN-1061, ZGN-1345 and its other product candidates, which may not support further development and marketing approval, actions of regulatory agencies, which may affect the initiation, timing and progress of nonclinical studies and clinical trials of its product candidates, Zafgen's ability to obtain, maintain and protect its 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 when needed, Zafgen's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, 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, including without limitation Zafgen's Quarterly Reports on Form 10-Q. 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.

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Condensed Consolidated Statements of Operations

(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	11,830	9,723	36,472	29,928
General and administrative	3,339	3,117	9,959	9,713
Total operating expenses	15,169	12,840	46,431	39,641
Loss from operations	(15,169)	(12,840)	(46,431)	(39,641)
Other income (expense):				
Interest income	623	266	1,214	740
Interest expense	(475)	(31)	(1,399)	(157)
Foreign currency transaction (losses) gains, net	(46)	20	(182)	115
Total other income (expense), net	102	255	(367)	698
Net loss	$ (15,067)	$ (12,585)	$ (46,798)	$ (38,943)
Net loss per share, basic and diluted	$ (0.41)	$ (0.46)	$ (1.53)	$ (1.42)
Weighted average common shares outstanding, basic and diluted	36,619,575	27,483,550	30,608,664	27,414,314

Condensed Consolidated Balance Sheets

(In thousands, except share and per share data)

(Unaudited)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 45,141	$ 40,777
Marketable securities	82,691	61,275
Tax incentive receivable	1,245	946
Prepaid expenses and other current assets	2,392	1,927
Total current assets	131,469	104,925
Property and equipment, net	419	528
Other assets	57	57
Total assets	$ 131,945	$ 105,510
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,627	$ 3,020
Accrued expenses	4,901	4,273
Notes payable, current	3,636	—
Total current liabilities	11,164	7,293
Notes payable, long-term	16,844	20,000
Total liabilities	28,008	27,293
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of September 30, 2018 and December 31, 2017; no shares issued and outstanding as of September 30, 2018 and December 31, 2017	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of September 30, 2018 and December 31, 2017; 36,865,817 and 27,489,457 shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively	37	27
Additional paid-in capital	440,303	367,825
Accumulated deficit	(336,375)	(289,577)
Accumulated other comprehensive loss	(28)	(58)
Total stockholders' equity	103,937	78,217
Total liabilities and stockholders' equity	$ 131,945	$ 105,510

Source: Zafgen, Inc.