Zafgen Reports Fourth Quarter and Full Year 2018 Operating and Financial Results and Provides Key Program and Business Updates

March 11, 2019

Recently announced positive results for second cohort of Phase 2 clinical trial of ZGN-1061

Updates on progress preparing for FDA Type A meeting for ZGN-1061

Suspends IND filing plans for ZGN-1258 based on nonclinical finding in long-term toxicology studies

Expanded executive team with highly experienced drug development leader, Dr. Priya Singhal, as Head of Research and Development

Company to host conference call today at 5:00 p.m. ET

BOSTON, March 11, 2019 (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 fourth quarter and full year 2018 operating and financial results along with key program updates for ZGN-1061 and ZGN-1258 and other business updates.

Program Updates

ZGN-1061
In January 2019, Zafgen announced positive data for the second cohort of its Phase 2 clinical trial of ZGN-1061 in patients with type 2 diabetes. The clinical trial met all of its primary objectives at the 1.8 mg dose, which included glycemic control, or change in A1C, and safety and tolerability. Treatment with ZGN-1061 showed a statistically significant reduction in A1C for 1.8 mg versus placebo, with a 1.1% reduction. A1C levels continued to decline with no waning of effect for both doses through Week 12. Progressive and notable reduction in body weight was observed at the 1.8 mg dose with no evidence of waning effect. The data also showed a favorable safety and tolerability profile for ZGN-1061 through 12 weeks of treatment, with no treatment-related serious adverse events and no cardiovascular (CV) safety signals observed.

Zafgen has made important progress in preparation for a Type A meeting to address the concerns raised by the U.S. Food and Drug Administration (FDA) with the clinical hold for ZGN-1061. The Company plans to provide key new data, including: newly developed *in vitro* assays of human plasma coagulation using endothelial cells and assessment of tissue factor expression with endothelial cells, and other supportive new assays, all of which Zafgen believes continue to substantially differentiate ZGN-1061 from the Company's prior compound; and the efficacy data and the full safety data from the second cohort of the Phase 2 clinical trial. An update is anticipated in the second quarter of 2019.

ZGN-1258
Zafgen is also announcing its decision to suspend plans to file an investigational new drug (IND) application for ZGN-1258, the Company's candidate for rare metabolic disorders including Prader-Willi syndrome (PWS), based on a recent, unexpected finding in muscle tissue in four- and six-month long-term rodent toxicology studies. Nonclinical data showed degeneration and other anomalies in rat muscle tissue to different degrees in both vehicle and dose arms of the studies. The effects were absent from other animal species in long term models, and importantly, this finding has not been observed in any of the Company's other MetAP2 inhibitors or clinical trials and appears to be specific to ZGN-1258. Zafgen will provide an update on plans for ZGN-1258 at a later time, if warranted, following further evaluation.

"Our mission at Zafgen to develop safe, effective and potentially transformative treatment options to patients who could benefit, centers on applying the highest scientific rigor to our development efforts and letting the data lead us. For ZGN-1061, we are very pleased with the strong data that continue to support the profile of this novel MetAP2 inhibitor for patients with type 2 diabetes. I am proud of the team's diligent, solution-oriented approach to addressing the FDA's comments on the ZGN-1061 IND application, and we look forward to presenting these new, more sophisticated assays and full safety results as the basis of what we hope is a productive and collaborative dialogue to advance the ZGN-1061 program," said Jeffrey Hatfield, Chief Executive Officer, Zafgen.

Mr. Hatfield continued, "We are, of course, extremely disappointed to suspend our IND filing plans for ZGN-1258. We proactively initiated long-term toxicology studies prior to filing an IND, and that decision now allows us to take the necessary steps to assess the unexpected effects we observed. The entire team at Zafgen is driven by a deep commitment to people with PWS and their families, and we'll continue to evaluate ZGN-1258, as well as explore other potential options within our portfolio of MetAP2 inhibitors, to address the devastating hyperphagia experienced by those with PWS. Zafgen has a strong cash position through 2020 to execute on our strategic objectives."

PATH for PWS, Zafgen's natural history study conducted in collaboration with the Foundation for Prader-Willi Research (FPWR), is independent of any specific development program and continues enrollment, with more than 400 of the 500-participant goal now enrolled. The data from this study are intended to inform the development and clinical trial design of potential new treatments.

Corporate Updates

Last week, Zafgen appointed a key executive to its leadership team, Priya Singhal, M.D., M.P.H., as Head of Research and Development, who brings nearly a decade of senior drug development experience in R&D strategy, drug safety and benefit-risk management. Dr. Singhal is responsible for leading and overseeing research, clinical and manufacturing strategy and implementation across the Company's portfolio of investigational MetAP2 inhibitors.

Zafgen is also announcing that Dennis Kim, M.D. has resigned from his position as the Company's Chief Medical Officer (CMO) to serve as a senior consultant in a CMO capacity for multiple biotechnology companies. Dr. Kim will support a transition process over the next several weeks.

"Priya brings an invaluable depth of R&D expertise and leadership to Zafgen and is ideally suited to drive efforts across all our key areas of focus. We are so thrilled that she has joined the Zafgen team," said Mr. Hatfield. "We also want to thank Dennis for his many contributions to Zafgen and his commitment to the patients and families that are at the heart of our work. We wish him all the best in this new chapter of his career."

Fourth Quarter and Full Year 2018 Financial Results

Cash, Cash Equivalents and Marketable Securities

As of December 31, 2018, the Company had cash, cash equivalents and marketable securities totaling $118.1 million.

Net Loss

The Company reported a net loss for the fourth quarter of 2018 of $14.6 million, or $0.39 per share, compared to a net loss of $13.1 million, or $0.48 per share, for the fourth quarter of 2017. For the full year 2018, the Company reported a net loss of $61.4 million, or $1.90 per share, compared to $52.0 million, or $1.90 per share, for the full year 2017.

The weighted average common shares (basic and diluted) outstanding used to compute net loss per share were 37,036,065 for the fourth quarter of 2018 compared to 27,489,397 for the same quarter of 2017. For the full year 2018, weighted average common shares (basic and diluted) outstanding used to compute net loss per share were 32,228,721 compared to 27,433,239 for the full year 2017.

Research and Development Expenses

Research and development expenses for the fourth quarter of 2018 were $11.5 million compared to $10.9 million for the fourth 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 nonclinical studies progressed during the quarter. These increases in research and development costs were partially offset by a decrease in non-cash stock-based compensation expense and nonclinical and manufacturing costs associated with our ZGN-1061 program in the fourth quarter of 2018 as compared to the fourth quarter of 2017.

For the full year 2018, research and development expenses were $47.9 million, compared to $40.8 million for the full year 2017. The increase in research and development expenses for the full year period was primarily due to increased costs related to the ZGN-1258 program as nonclinical studies progressed during the year. There were also increases in personnel related costs and non-cash stock-based compensation expense for the full year 2018 as compared to the full year 2017. The increase was partially offset by a decrease in nonclinical and manufacturing costs associated with our ZGN-1061 program during the full year 2018.

General and Administrative Expenses

General and administrative expenses for the fourth quarter of 2018 were $3.2 million, compared to $2.4 million for the fourth 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 and non-cash stock-based compensation expense.

For the full year 2018, general and administrative expenses were $13.2 million, compared to $12.2 million for the full year 2017. The increase in general and administrative expenses for the full year 2018 as compared to the prior year period was primarily due to an increase in personnel related costs and public company costs.

Conference Call Information

Zafgen will host an investor conference call today, March 11, 2019, at 5:00 p.m., Eastern Time, to discuss the Company's fourth quarter and full year 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 2067548. 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 8:00 p.m. ET on March 11, 2019 through March 18, 2019 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 2067548.

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 researching or advancing programs for type 2 diabetes, Prader-Willi syndrome and liver diseases. 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 use of ZGN-1258, ZGN-1061, ZGN-1345 and other second-generation MetAP2 inhibitors as treatments for metabolic diseases including Prader-Willi syndrome, 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, Zafgen's expected cash, cash equivalents and marketable securities balance as of December 31, 2018, and Zafgen's expectations regarding the length of its cash runway, may constitute forward-looking statements for the 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 demonstrate the efficacy and safety of ZGN-1258, ZGN-1061, ZGN-1345 and its product candidates and to differentiate them 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 successfully engage with the FDA concerning the clinical hold on a clinical trial of ZGN-1061 and to design and conduct a nonclinical study or clinical trial demonstrating sufficient data to exclude cardiovascular risk to an acceptable degree, Zafgen's ability to overcome the full clinical hold place on ZGN-1061 by the FDA and obtain regulatory approval, Zafgen's ability to continue to evaluate ZGN-1258 and to advance the program in nonclinical and clinical development, 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.

Media/Investor Relations Contacts:
Zafgen, Inc.
Patricia Allen
Chief Financial Officer
617-648-9792

Media
Krystle Gibbs
Ten Bridge Communications
krystle@tenbridgecommunications.com
508-479-6358

Investors
John Woolford
Westwicke
john.woolford@westwicke.com
443-213-0506

ZAFGEN, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)
(Unaudited)

	Year Ended December 31,		
	2018	**2017**	**2016**
Revenue	$ -	$ -	$ -
Operating expenses:			
Research and development	47,929	40,839	39,936
General and administrative	13,193	12,160	18,289
Total operating expenses	61,122	52,999	58,225
Loss from operations	(61,122)	(52,999)	(58,225)
Other income (expense):			
Interest income	1,889	996	894
Interest expense	(1,898)	(165)	(529)
Foreign currency transaction (losses) gains, net	(237)	140	(18)
Total other (expense) income, net	(246)	971	347
Net loss	$ (61,368)	$ (52,028)	$ (57,878)
Net loss per share, basic and diluted	$ (1.90)	$ (1.90)	$ (2.12)
Weighted average common shares outstanding, basic and diluted	32,228,721	27,433,239	27,297,934

ZAFGEN, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended December 31,	
	2018	**2017**
Revenue	$ -	$ -
Operating expenses:		

Research and development	11,457	10,911
General and administrative	3,234	2,447
Total operating expenses	14,691	13,358
Loss from operations	(14,691)	(13,358)
Other income (expense):		
Interest income	675	256
Interest expense	(499)	(8)
Foreign currency transaction (losses) gains, net	(55)	25
Total other income, net	121	273
Net loss	$ (14,570)	$ (13,085)
Net loss per share , basic and diluted	$ (0.39)	$ (0.48)
Weighted average common shares outstanding, basic and diluted	37,036,065	27,489,397

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

	December 31, 2018	2017
Assets		
Current assets:		
Cash and cash equivalents	$ 49,331	$ 40,777
Marketable securities	68,735	61,275
Tax incentive receivable	1,536	946
Prepaid expenses and other current assets	1,728	1,927
Total current assets	121,330	104,925
Property and equipment, net	375	528
Other assets	57	57
Total assets	$ 121,762	$ 105,510
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,590	$ 3,020
Accrued expenses	4,261	4,273
Notes payable, current	5,455	-
Total current liabilities	13,306	7,293
Notes payable, long-term	15,185	20,000
Total liabilities	28,491	27,293
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of December 31, 2018 and 2017; no shares issued and outstanding as of and December 31, 2018 and 2017	-	-
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of December 31, 2018 and 2017; 37,287,221 and 27,489,457 shares issued and outstanding as of December 31, 2018 and 2017, respectively	37	27
Additional paid-in capital	444,212	367,825
Accumulated deficit	(350,945)	(289,577)
Accumulated other comprehensive loss	(33)	(58)
Total stockholders' equity	93,271	78,217
Total liabilities and stockholders' equity	$ 121,762	$ 105,510

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 Company's audited consolidated financial statements for the year ended December 31, 2017.

Source: Zafgen, Inc.