Millendo Therapeutics Reports Second Quarter 2019 Operating and Financial Results

August 12, 2019

– Livoletide pivotal study in Prader-Willi syndrome (ZEPHYR) on track to report topline results in 1H20 –

– Nevanimibe CAH Phase 2b study now enrolling under amended protocol –

– New program, MLE-301, beginning preclinical development to enable first-in-human study –

ANN ARBOR, Mich.--(BUSINESS WIRE)--Aug. 12, 2019-- Millendo Therapeutics, Inc. (Nasdaq: MLND), a clinical-stage biopharmaceutical company primarily focused on developing novel treatments for orphan endocrine diseases with significant unmet needs, today provided a corporate update and reported financial results for the quarter ended June 30, 2019.

"Our ZEPHYR pivotal Phase 2b/3 clinical study in PWS patients is off to a strong start, and we remain on track to report topline results from the Phase 2b portion of the study in the first half of 2020," said Julia C. Owens, President and Chief Executive Officer of Millendo Therapeutics. "In addition, our Phase 2b clinical study of nevanimibe in CAH is enrolling patients under an amended protocol. We expect to provide an update on timelines for the CAH study in the second half of 2019.

"We are announcing today our decision to discontinue the Phase 2 study of nevanimibe in Cushing's syndrome, based on an analysis of the feasibility of patient recruitment and a reprioritization of resources. While this decision was a difficult one, we believe that we are now able to better optimize investments across our portfolio of potential novel therapies in areas of significant unmet need. In line with this commitment, we are excited today to announce the selection of a new compound to begin preclinical development required to support a first-in-human study, MLE-301 for the treatment of vasomotor symptoms associated with menopause. This selective NK3R antagonist enables us to leverage our expertise in VMS drug development."

Second Quarter 2019 and Recent Highlights

- **ZEPHYR on track to deliver topline results in 1H20:** A pivotal Phase 2b/3 study of livoletide for the treatment of Prader-Willi syndrome (PWS) was initiated in 1Q19. Clinical trial site activation has progressed well since study initiation with 26 sites recruiting in the U.S., Europe and Australia. The Phase 2b portion of the study has the potential to support an NDA submission for livoletide.
- **Fast Track designation has been granted by the U.S. Food and Drug Administration (FDA) to Millendo for the development of livoletide for PWS:** This designation is intended to expedite the development and regulatory review of new drugs that address unmet medical needs and have the potential to treat serious and life-threatening conditions.
- **Nevanimibe Phase 2b CAH study is enrolling under an amended protocol:** A Phase 2b study of nevanimibe in CAH that was initiated in 3Q18 is now recruiting patients under an amended protocol. The protocol amendment includes a 500 mg BID (twice a day) starting dose and an extension of the study treatment period to 16 weeks. An update on the CAH study timelines is planned for 2H19.
- **Nevanimibe Phase 2 study in Cushing's syndrome discontinued:** Millendo plans to discontinue the Phase 2 study of nevanimibe in Cushing's syndrome due to slower than anticipated enrollment. This strategic decision creates the opportunity for the company to redirect its resources toward other development programs.
- **New pipeline asset MLE-301 beginning preclinical development required to support first-in-human study:** MLE-301, a neurokinin 3 receptor (NK3R) antagonist, is intended for the treatment of vasomotor symptoms (VMS), defined as hot flashes and night sweats in menopausal women. MLE-301 is a novel NK3R antagonist, a target that Millendo and others have shown plays a key role in regulating the activity of KNDy (kisspeptin/NKB/dynorphin) neurons, which are overactive in menopausal women and play an important role in the generation of VMS. MLE-301 is currently in preclinical development and is expected to enter clinical trials in 2020.

"With the strategic reprioritization of our portfolio, we are pleased that our current cash position is expected to support our capital needs into the fourth quarter of 2020," commented Louis J. Arcudi III, Chief Financial Officer.

Second Quarter 2019 Financial Results

Cash Position: Cash, cash equivalents, marketable securities and restricted cash were $56.6 million at June 30, 2019, compared to $78.2 million at December 31, 2018.

Research and Development (R&D) Expenses: R&D expenses were $6.0 million for the second quarter 2019, as compared to $3.2 million for the same period in 2018. The increase in R&D expenses was primarily driven by increased spending on the company's pivotal Phase 2b/3 study of livoletide in PWS and higher compensation expenses as a result of increased headcount.

General and Administrative (G&A) Expenses: G&A expenses were $4.2 million for the second quarter 2019, as compared to $1.8 million for the

same period in 2018. The increase in G&A expenses was primarily driven by higher compensation expense as a result of increased headcount and increased professional and other fees to support ongoing business operations and obligations associated with being a publicly traded company.

Net Loss: The company's net loss for the quarter ended June 30, 2019 was $9.9 million as compared to $5.0 million for the same period in 2018.

2019 Financial Guidance

Millendo expects that its cash, cash equivalents, marketable securities and restricted cash will support the company's capital needs into the fourth quarter of 2020, beyond the readout for the topline results of the Phase 2b portion of the pivotal Phase 2b/3 study of livoletide in PWS, which is expected in the first half of 2020. This cash runway guidance is based on the company's current operational plans and excludes any additional funding or business development activities.

About Livoletide
Millendo's lead asset, livoletide, is an unacylated ghrelin analogue in late-stage clinical development for the treatment of Prader-Willi syndrome (PWS), a rare genetic disease characterized by hyperphagia, a chronic unrelenting hunger, that leads to obesity, metabolic dysfunction, reduced quality of life and early mortality. In March 2019, the company initiated a pivotal Phase 2b/3 clinical study of livoletide in patients with PWS. In a previous randomized, double-blind, placebo-controlled Phase 2 clinical trial in 47 patients with PWS, administration of livoletide once daily was associated with a clinically meaningful improvement in hyperphagia, as well as a reduction in appetite. For more information about Millendo's pivotal study of livoletide (ZEPHYR) please visit www.clinicaltrials.gov (NCT03790865) or the Patients and Families portion of our website.

About Nevanimibe
Nevanimibe decreases adrenal steroidogenesis through the inhibition of ACAT1 and is being studied for the treatment of classic congenital adrenal hyperplasia (CAH). CAH is a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses, which can make it difficult for physicians to appropriately treat CAH without causing adverse consequences. In a Phase 2 proof-of-concept clinical trial in patients with CAH, Millendo observed nevanimibe to be associated with clear signs of clinical activity in seven of 10 treated patients. A Phase 2b trial of nevanimibe in CAH (NCT03669549) is ongoing.

About MLE-301
MLE-301 is an investigational, neurokinin 3 receptor (NK3R) antagonist that acts to normalize the activity of KNDy (kisspeptin/NKB/dynorphin) neurons, which are overactive in menopausal women and play an important role in the generation of vasomotor symptoms (VMS). MLE-301 works by reducing neurokinin B signaling via KNDy neurons and is hypothesized to reduce the frequency and severity of VMS. Millendo is advancing MLE-301 for the treatment of VMS associated with menopause through preclinical development to enable first-in-human clinical studies.

About Millendo Therapeutics, Inc.
Millendo Therapeutics is a late-stage biopharmaceutical company primarily focused on developing novel treatments for orphan endocrine diseases where current therapies do not exist or are insufficient. As a leading orphan endocrine company, Millendo creates distinct and transformative treatments where there is a significant unmet medical need. The company is currently advancing livoletide for the treatment of Prader-Willi syndrome, nevanimibe for the treatment of classic congenital adrenal hyperplasia and MLE-301 for the treatment of vasomotor symptoms associated with menopause. For more information, please visit www.millendo.com.

Cautionary Statement Regarding Forward-Looking Statements
Certain statements contained in this press release regarding matters that are not historical facts, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. These include statements regarding Millendo's expectations regarding the potential for an NDA submission for livoletide, the timing of data from its clinical trials, including the timing of topline data from the Phase 2b portion of its ZEPHYR trial, and the timeline for its Phase 2b clinical study of nevanimibe in CAH, the potential and timing for MLE-301 to enter clinical trials, the potential of a future product launch, the potential advantages of Millendo's discontinuation of its Phase 2 study of nevanimibe in CS, the potential impact of Fast Track designation, Millendo's expectations regarding its 2019 and 2020 milestones, and Millendo's expectations regarding its cash runway, and, therefore, you are cautioned not to place undue reliance on them. In some cases, you can identify forward-looking statements by the words "may," "might," "will," "could," "would," "should," "expect," "intend," "plan," "objective," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue" and "ongoing," or the negative of these terms, or other comparable terminology intended to identify statements about the future. Such forward-looking statements are based on Millendo's expectations and involve risks and uncertainties; consequently, actual results may differ materially from those expressed or implied in the statements due to a number of factors, including that Millendo has incurred significant losses since inception, Millendo has a limited operating history and has never generated any revenue from product sales, Millendo will require additional capital to finance its operations, Millendo's future success is dependent on the successful clinical development, regulatory approval and subsequent commercialization of livoletide, nevanimibe and any future product candidates, preclinical studies or earlier clinical trials are not necessarily predictive of future results and the results of Millendo's clinical trials may not support Millendo's livoletide or nevanimibe claims, Millendo may encounter substantial delays in its clinical trials or Millendo may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside Millendo's control, Millendo's product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, or limit their commercial potential and Millendo faces substantial competition. You should refer to the risk factor disclosure set forth in the periodic reports and other documents we file with the SEC available at www.sec.gov, including without limitation our Annual Report on Form 10-K for our fiscal year ended December 31, 2018 and our Quarterly Report on Form 10-Q for our fiscal quarter ended June 30, 2019.

New factors emerge from time to time and it is not possible for Millendo to predict all such factors, nor can Millendo assess the impact of each such factor on the business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Forward-looking statements included in this press release are based on information available to Millendo as of the date of this press release. Millendo disclaims any obligation to update such forward-looking statements to reflect events or circumstances after the date of this press release, except as required by applicable law.

Millendo Therapeutics, Inc.

Condensed Statements of Operations
(in thousands except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Operating Expenses				
Research and development	$ 5,981	$ 3,200	$ 12,185	$ 5,969
General and administrative	4,179	1,786	8,632	3,405
Loss from operations	10,160	4,986	20,817	9,374
Other (income) expense, net	(289)	56	(580)	54
Net loss	(9,871)	(5,042)	(20,237)	(9,428)
Net loss attributable to noncontrolling interest	-	196	-	321
Net loss attributable to common stockholders	$ (9,871)	$ (4,846)	$ (20,237)	$ (9,107)
Net loss per share of common stock, basic and diluted	$ (0.74)	$ (6.82)	$ (1.51)	$ (12.82)
Weighted-average shares of common stock outstanding, basic and diluted	13,379,842	710,390	13,368,981	710,390

Millendo Therapeutics, Inc.
Condensed Balance Sheet Data
(in thousands)

	June 30, 2019	December 31, 2018
Cash, cash equivalents, marketable securities and restricted cash	$ 56,631	$ 78,155
Other assets	11,693	5,919
Total assets	$ 68,324	$ 84,074
Total liabilities	$ 13,299	$ 10,952
Total stockholders' equity	55,025	73,122
Total liabilities and stockholders' equity	$ 68,324	$ 84,074

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