Aileron Therapeutics Announces Positive Interim Results from Phase 1b/2 Clinical Trial of ALRN-6924 for the Prevention of Topotecan-induced Toxicities During Treatment for Small Cell Lung Cancer

June 1, 2020

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Observed a protective effect against severe chemotherapy-induced anemia and thrombocytopenia across all dose levels

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Management to host a conference call and webcast today at 8:30 a.m. EDT

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WATERTOWN, Mass., June 01, 2020 (GLOBE NEWSWIRE) -- Aileron Therapeutics (NASDAQ:ALRN) announced today positive interim data from the open-label Phase 1b dose optimization part of its ongoing Phase 1b/2 clinical trial. In this trial, ALRN-6924 is being evaluated as an agent to protect patients against chemotherapy-induced toxicity, a concept known as chemoprotection (or myelopreservation). Patients in the trial have advanced, p53-mutated small cell lung cancer (SCLC) and are being treated with second-line topotecan following administration of ALRN-6924. 18 patients were enrolled across three ALRN-6924 dose levels in the dose optimization part of the trial of which 17 patients completed the first treatment cycle and therefore met protocol-defined criteria for evaluability. A protective effect against severe chemotherapy-induced anemia and thrombocytopenia was observed across all dose levels as compared to historical controls. In addition, patients treated with 0.3 mg/kg ALRN-6924 met the protocol-defined criteria for reduction of NCI CTC Grade 3/4 neutropenia to ≤50% in the first treatment cycle.

Key findings from the interim analysis of the Phase 1b dose optimization part of the trial are as follows:

- Patients treated with 0.3 mg/kg ALRN-6924 showed the lowest rate of NCI CTC Grade 3/4 hematological adverse events, including 17% with anemia, 33% with thrombocytopenia and 67% with neutropenia, across all treatment cycles.
- None of the patients treated with 0.3 mg/kg ALRN-6924 required transfusions of red blood cells or platelets. The expansion of the 0.3 mg/kg ALRN-6924 dose level from six to a total of 14 patients is underway.
- Across all three dose levels, patients showed NCI CTC Grade 3/4 hematological adverse events, including 24% with anemia, 35% with thrombocytopenia and 88% with neutropenia.
- Across all three dose levels, no patients experienced febrile neutropenia or NCI CTC Grade 3/4 nausea, vomiting, diarrhea or fatigue, which are severe toxicities commonly observed with topotecan-treatment in this patient population.

“Large randomized trials designed to evaluate the anti-tumor efficacy of topotecan and other drugs have historically presented safety data as physician-reported adverse events of hematological toxicity. In this study, Aileron has evaluated bone marrow toxicity based upon absolute laboratory values, which are fully objective measures when considering the toxicity of topotecan. While still preliminary, the results from our dose optimization trial show a strong chemoprotective effect on severe anemia and thrombocytopenia in SCLC patients treated with topotecan,” said Dr. Vojo Vukovic, Chief Medical Officer of Aileron Therapeutics.

"We and our investigators are very excited about the chemoprotective effects of ALRN-6924 observed in the dose optimization part of our ongoing trial in SCLC patients. Based on the interim data from the first 17 patients and published rates of single-agent topotecan-related toxicities in 2nd line SCLC patients, we now believe even more strongly that ALRN-6924 can selectively induce cell cycle arrest to protect cancer patients from the toxic side effects of chemotherapy. We believe these data represent an important step in our goal to drive a paradigm shift in the management of chemotherapy-induced toxicities, by creating a treatment that turns toxic chemotherapy into a form of well-tolerated, targeted therapy,” said Dr. Manuel Aivado, President and CEO of Aileron Therapeutics.

The key interim results from the Phase 1b dose optimization part of the trial as of the data cut-off date of May 13, 2020 are shown in the table below:

	ALRN-6924 0.3 mg/kg +	**ALRN-6924 (all doses) +**

Adverse Events	Topotecan 1.5 mg/m2	Topotecan 1.5 mg/m2
	N (%)	N (%)
***NCI CTC Grade ≥3**	N=6	N=17
All AEs	5 (83)	16 (94)
Neutropenia	4 (67)	15 (88)
Leukopenia	2 (33)	9 (53)
Thrombocytopenia	2 (33)	6 (35)
Anemia	1 (17)	4 (24)
Fatigue	-	-
Nausea	-	-
Neutropenia NCI CTC Grade 4**	2 (33)	8 (47)

*Based on lab values (except for fatigue and nausea) and all AEs presented are treatment-emergent
**In the first treatment cycle

The Phase 1b portion of the study is designed to identify a dose and a schedule of ALRN-6924 administration to reduce chemotherapy-induced toxicities such as severe anemia and thrombocytopenia, as well as other toxicities resulting from topotecan. In the ongoing dose optimization part of the study, ALRN-6924 is administered 24 hours before each dose of topotecan, which is administered daily on days 1 through 5 of every 21-day treatment cycle.

Phase 1b/2 Trial - Next Steps
The Company is enrolling patients into the expansion cohort of its 0.3mg/kg dose level and plans to initiate enrollment in the schedule optimization part of the Phase 1b/2 trial in June 2020.

As previously reported, the Company plans to report the top-line final data for the dose optimization and schedule optimization parts of the trial in the fourth quarter of 2020. The Company expects that these results will determine a recommended ALRN-6924 dose and schedule for subsequent trials.

The Company is carefully monitoring the effect of the coronavirus pandemic on its clinical trial sites and the healthcare system, which may impact the future timing of the trial and the Company's planned data announcements.

Conference Call
A conference call to discuss these interim data from the Phase 1b/2 study of ALRN-6924 as a chemoprotection agent has been scheduled for Monday, June 1, 2020 at 8:30 a.m. ET. To access the conference call, investors are invited to dial (877) 705-6003 (U.S. and Canada) or (201) 493-6725 (International). The conference ID number is 13702965. A live audio webcast can be accessed by visiting the investor relations section of the Company's website, www.aileronrx.com. A replay of the webcast will be archived on the Company's website following the event.

About ALRN-6924
ALRN-6924 is a first-in-class dual MDM2/MDMX inhibitor that is currently being evaluated in a Phase 1b/2 clinical trial to evaluate ALRN-6924 as a chemoprotective agent to protect against chemotherapy-related toxicities.

About Aileron
Aileron is a clinical-stage biopharmaceutical company advancing a proprietary platform of cell-permeating alpha-helical peptides. The stabilized helical structure of our peptides allows the design of cell-permeating therapeutic agents with large molecular surfaces for optimal target binding properties, resulting in drug candidates like ALRN-6924. Our current focus is to improve the standard of care for patients with cancer by developing safe and effective therapies that leverage our proprietary peptide platform. For more information, visit www.aileronrx.com, and for more information about our clinical trials please visit www.clinicaltrials.gov.

Forward-Looking Statements
Statements in this press release about Aileron's future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements about the Company's strategy and clinical development plans. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including whether Aileron's cash resources will be sufficient to fund its continuing operations for the periods and/or trials anticipated; whether results obtained in preclinical and nonclinical studies and clinical trials will be indicative of results obtained in future clinical trials; whether preliminary or interim results from a clinical trial such as the interim results presented will be indicative of the final results of the trial; whether Aileron's product candidates will advance through the clinical trial process on a timely basis, or at all; whether the results of such trials will warrant submission for approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether Aileron's product candidates will receive approval from regulatory agencies on a timely basis or at all; whether, if product candidates obtain approval, they will be successfully distributed and marketed; whether the coronavirus pandemic will have an impact on the timing of our clinical development, clinical supply and our operations; and other factors discussed in the "Risk Factors" section of Aileron's quarterly report on Form 10-Q for the period ended March 31, 2020, filed on May 11, 2020, and risks described in other filings that Aileron may make with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Aileron specifically disclaims any obligation to update any forward-looking statement, whether because of new information, future events or otherwise.

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