Kiniksa Announces New Data from Phase 2 Trial of Mavrilimumab in Giant Cell Arteritis to be Presented at Late-Breaking Abstracts Session of American College of Rheumatology Convergence 2020

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- Mavrilimumab reduced risk of flare and increased sustained remission in Phase 2 giant cell arteritis clinical trial –
- Primary and secondary efficacy endpoints achieved statistical significance -
- Results were consistent across new onset and relapsing/refractory cohorts -

HAMILTON, Bermuda, Oct. 26, 2020 (GLOBE NEWSWIRE) -- Kiniksa Pharmaceuticals, Ltd. (Nasdaq: KNSA) ("Kiniksa"), a biopharmaceutical company with a pipeline of assets designed to modulate immunological pathways across a spectrum of diseases, today announced that new data from the global Phase 2 clinical trial of mavrilimumab in giant cell arteritis (GCA) will be presented at the late-breaking abstracts session during the American College of Rheumatology (ACR) Convergence 2020. Mavrilimumab is an investigational fully-human monoclonal antibody that targets granulocyte macrophage colony stimulating factor receptor alpha (GM-CSFRα). Both the primary and secondary efficacy endpoints achieved statistical significance, and there was a consistent trend of efficacy across the new onset and relapsing/refractory cohorts.

"There is a significant unmet need for safe and effective giant cell arteritis therapies. We believe mavrilimumab, with its upstream inhibition of two immune pathways implicated in giant cell arteritis, has the potential to provide differentiation from current standard of care therapies by addressing the underlying pathophysiology of the disease," said John F. Paolini, MD, PhD, Chief Medical Officer of Kiniksa. "In the Phase 2 giant cell arteritis trial, mavrilimumab was superior to placebo on the primary and secondary efficacy endpoints of time-to-flare and sustained remission at Week 26. These data further underscore the potential for mavrilimumab to offer a differentiated treatment option for patients with both relapsing/refractory disease as well as new onset disease."

Dr. Maria Cid[1], a co-principal investigator for the global Phase 2 trial, will deliver a virtual presentation entitled *Mavrilimumab (anti GM-CSF receptor α monoclonal antibody) Reduces Risk of Flare and Increases Sustained Remission in a Phase 2 Trial of Patients with Giant Cell Arteritis* at the late-breaking abstracts session (L06 - L11) on Monday, November 9, 2020 at 11:30 a.m. Eastern Time. Dr. Sebastian Unizony[2] is a co-principal investigator.

The Phase 2 trial randomized 70 patients 3:2 to mavrilimumab 150 mg (N=42) or placebo (N=28) biweekly injected subcutaneously, co-administered with a protocol-defined 26-week oral corticosteroid taper. Patients were stratified by new onset (n=35) or relapsing/refractory (n=35) disease.

The primary efficacy endpoint of time-to-first adjudicated GCA flare by Week 26 in all treated patients was statistically significant (Hazard Ratio = 0.38, p=0.0263).

- Median time-to-flare by Week 26 could not be estimated in mavrilimumab recipients due to the low number of flares in the mavrilimumab treatment arm. The median time-to-flare for placebo recipients was 25.1 weeks.
- There was a 62% lower risk of flare in mavrilimumab recipients compared to placebo recipients.

The secondary efficacy endpoint of sustained remission at Week 26 in all treated patients was also statistically significant.

- The sustained remission rate at Week 26 was 33.3 percentage points higher in mavrilimumab recipients (83.2%) compared to placebo recipients (49.9%) (p=0.0038).

While the study was not powered for disease cohorts, there was a consistent trend of efficacy across the new onset and relapsing/refractory cohorts.

New Onset Cohort

- There was a 71% lower risk of flare in mavrilimumab recipients compared to placebo recipients (Hazard Ratio = 0.29, p=0.0873).
- The sustained remission rate at Week 26 was 28.9 percentage points higher in mavrilimumab recipients (91.3%) compared to placebo recipients (62.3%) (p=0.0727).

Relapsing/Refractory Cohort

- There was a 57% lower risk of flare in mavrilimumab recipients compared to placebo recipients (Hazard Ratio = 0.43,

p=0.1231).

- The sustained remission rate at Week 26 was 30.6 percentage points higher in mavrilimumab recipients (72.2%) compared to placebo recipients (41.7%) (p=0.0668).

Mavrilimumab was well-tolerated; there were no drug-related serious adverse events, and the rates of drug-related treatment-emergent adverse events between mavrilimumab recipients and placebo recipients were similar.

The 12-week washout safety follow-up period is ongoing, and additional analyses of this Phase 2 trial are planned. Next steps for the development program in GCA will be further informed by anticipated discussions with the U.S. Food and Drug Administration (FDA).

The abstract is available through the ACR website.

Table 1 as submitted to ACR by the authors for publication and as referenced in the abstract follows below. The table was inadvertently omitted during the online posting of the abstract.

Table 1: Efficacy at Week 26.

Time to Flare by Week 26 and Sustained Remission at Week 26 - Total mITT Population

	Mavrilimumab 150 mg (N=42)	**Placebo (N=28)**
Number of Subjects with Flare, n (%)	8 (19.0)	13 (46.4)
Primary Efficacy Endpoint: Time to Flare (weeks) by Week 26 [1]		
Median, 95% CI	NE (NE, NE)	25.1 (16.0, NE)
Hazard Ratio (Mavrilimumab vs Placebo), 95% CI [2]	0.38 (0.15, 0.92)	
P-value [3]	0.0263	
Secondary Efficacy Endpoint: Sustained Remission at Week 26 (%), 95% CI [4]	83.2 (67.9, 91.6)	49.9 (29.6, 67.3)
Difference in Proportions (95% CI) [5]	33.3 (10.7, 55.8)	
P-value [5]	0.0038	

Time to Flare by Week 26 and Sustained Remission at Week 26 by Randomization Strata

	New-onset		**Relapsing/Refractory**	
	Mavrilimumab 150 mg (N=24)	**Placebo (N=11)**	**Mavrilimumab 150 mg (N=18)**	**Placebo (N=17)**
Number of Subjects with Flare, n (%)	3 (12.5)	4 (36.4)	5 (27.8)	9 (52.9)
Primary Endpoint: Time to Flare (weeks) by Week 26 [1]				
Median, 95% CI	NE (NE, NE)	NE (11.7, NE)	NE (16.4, NE)	22.6 (16.0, NE)
Hazard Ratio (Mavrilimumab vs Placebo), 95% CI [6]	0.29 (0.06, 1.31)		0.43 (0.14, 1.30)	
P-value [7] [8]	0.0873		0.1231	
Secondary Endpoint: Sustained Remission at Week 26 (%) , 95% CI [4]	91.3 (69.3, 97.7)	62.3 (27.7, 84.0)	72.2 (45.6, 87.4)	41.7 (17.4, 64.5)
Difference in Proportions (95% CI) [5]	28.9 (-2.7, 60.5)		30.6 (-2.1, 63.2)	
P-value [5][8]	0.0727		0.0668	

NE = Not estimable.
[1] Kaplan-Meier method used to estimate the survival functions for each treatment arm.
[2] Calculated based on a Cox proportional-hazards model with treatment as covariate and stratified by randomization strata.
[3] Comparison of KPL-301 and placebo with respect to time to flare calculated by using a log-rank test and stratified by randomization strata.
[4] Kaplan-Meier Survival Estimates with standard error and 95% CI for each arm.
[5] Two-sided p-value and 95% CI for the difference in sustained remission between two arms using normal approximation. Placebo arm is the reference.
[6] Calculated based on a Cox proportional-hazards model with treatment as covariate.
[7] Comparison of KPL-301 and placebo with respect to time to flare calculated by using a log-rank test.
[8] Subgroup analyses were not powered for significance; nominal p values reported.

The FDA recently granted Orphan Drug designation to mavrilimumab for the treatment of GCA.

Kiniksa intends to make the presentation available through the Science section of its website after the ACR embargo lifts.

Kiniksa is also evaluating mavrilimumab in severe COVID-19 pneumonia and hyperinflammation and is enrolling the Phase 2 portion of a global, randomized, double-blind, placebo-controlled adaptive design Phase 2/3 clinical trial. Additionally, the company expects to announce data from a randomized, double-blind, placebo-controlled investigator-initiated study in the U.S. in the fourth quarter of 2020.

[1] Hospital Clínic, University of Barcelona, Institut d'Investigacions Biomèdiques August Pi i Sunyer (IDIBAPS); [2] Massachusetts General Hospital,

Harvard University

About the Global Phase 2 Clinical Trial of Mavrilimumab in GCA
The randomized, double-blind, placebo-controlled, global Phase 2 clinical trial of mavrilimumab in GCA consists of a 6-week screening period, a 26-week double-blind placebo-controlled treatment period, and a 12-week washout safety follow-up period. Patients age 50 to 85 years with active GCA, confirmed by temporal artery biopsy and/or imaging, with erythrocyte sedimentation rate (ESR) ≥ 30 mm/hour or C-reactive protein (CRP) ≥ 1 mg/dL, and symptoms of GCA within 6 weeks from randomization, were included. All patients were required to have achieved corticosteroid-induced remission (resolution of symptoms, ESR < 20 mm/hour, CRP < 1 mg/dL) prior to randomization.

About Giant Cell Arteritis
Giant cell arteritis is a rare chronic inflammatory disease of medium-to-large arteries. Cranial giant cell arteritis typically presents with headache and jaw claudication as well as constitutional symptoms of fever and fatigue. Acute events can include permanent vision loss from diminished blood flow to the eye. The large vessel form of giant cell arteritis affects the branches of the aorta supplying the trunk and limbs. There is currently one FDA-approved treatment for giant cell arteritis as an adjunct to a corticosteroid taper.

About Mavrilimumab
Mavrilimumab is an investigational fully-human monoclonal antibody that targets GM-CSFRα. Mavrilimumab was dosed in over 550 patients with rheumatoid arthritis through Phase 2b clinical studies in Europe and achieved prospectively-defined primary endpoints of efficacy and safety. Kiniksa's lead indication for mavrilimumab is GCA, a rare inflammatory disease of medium-to-large arteries. Kiniksa is also evaluating mavrilimumab in COVID-19 pneumonia and hyperinflammation. The FDA granted Orphan Drug designation to mavrilimumab for GCA in 2020.

About Kiniksa
Kiniksa is a biopharmaceutical company focused on discovering, acquiring, developing and commercializing therapeutic medicines for patients suffering from debilitating diseases with significant unmet medical need. Kiniksa's product candidates, rilonacept, mavrilimumab, vixarelimab and KPL-404, are based on strong biologic rationale or validated mechanisms, target underserved conditions and offer the potential for differentiation. These pipeline assets are designed to modulate immunological pathways across a spectrum of diseases. For more information, please visit www.kiniksa.com.

Forward-Looking Statements
The information contained in this press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases, you can identify forward looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these identifying words. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation, statements regarding: mavrilimumab's potential to offer a treatment option for both patients with relapsing/refractory disease as well as those with new onset disease in giant cell arteritis ("GCA"); the unmet need for patients with GCA; the potential to provide differentiation from current standard of care therapies by mavrilimumab's potential to address the underlying pathophysiology of the disease; the presentation of data from the study in an upcoming medical conference; the timing of data from our clinical trials; and the potential for our clinical stage product candidates to offer differentiation.

These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including without limitation, the following: the impact of additional data from us, investigator-initiated studies or other companies; the potential for undesirable side effects to be caused by mavrilimumab; the potential inability to replicate in later clinical trials positive results from earlier studies or clinical trials; the impact of discussions with the FDA on our development program in GCA; our reliance on third parties to manufacture our product candidates and conduct our clinical trials and/or perform certain regulatory activities for our product candidates; drug substance and/or drug product shortages, including in connection with our engagement of a manufacturing organization to produce mavrilimumab beyond our current inventory; the potential impact of the COVID-19 pandemic and measures taken in response to the pandemic; changes in our operating plan and funding requirements; existing or new competition; and our ability to attract and retain qualified personnel.

These and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission ("SEC") on August 4, 2020 and our other reports subsequently filed with or furnished to the SEC could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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