Novartis presents results from first global registration trial of CTL019 in pediatric and young adult patients with r/r B-ALL

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- - 82% (41 of 50) of patients achieved complete remission or complete remission with incomplete blood count recovery in interim analysis of Novartis study (ELIANA)
- - ELIANA, the first global CAR T cell trial, will be the basis of a Biologics Licensing Application (BLA) to the FDA in early 2017
- - Novartis pivotal data from ELIANA is supported by CTL019 US multicenter trial (ENSIGN) as well as earlier single site trial in r/r pediatric and young adult patients with B-cell ALL
- - The University of Pennsylvania (Penn) will present findings evaluating overall response to CTL019 therapy among r/r DLBCL patients with poor prognosis

EAST HANOVER, N.J., Dec. 3, 2016 /PRNewswire/ -- Findings from a Novartis clinical trial (ELIANA) evaluating efficacy and safety of CTL019, an investigational chimeric antigen receptor T cell (CAR T) therapy, in relapsed/refractory (r/r) pediatric and young adult patients with B-cell acute lymphoblastic leukemia (ALL) will be presented today during an oral session at the 58th American Society of Hematology (ASH) annual meeting (Abstract #221, December 3, 4:00-5:30 p.m.). The global Phase II study found that 82% (41 of 50) of infused patients achieved complete remission or complete remission with incomplete blood count recovery at three months post CTL019 infusion. For all patients with complete remission, no minimal residual disease was detected. In addition, the estimated relapse-free rate among responders was 60% (95% CI: 36, 78) six months after infusion with CTL019.[1] The results set the stage for filing CTL019 with the US Food and Drug Administration (FDA) in early 2017 for pediatric and young adult patients with r/r B-cell ALL.

ELIANA is the first pediatric global CAR T cell registration trial with study enrollment having occurred across 25 centers in the US, EU, Canada, Australia and Japan. Forty-eight percent of patients in ELIANA experienced grade 3 or 4 cytokine release syndrome (CRS), a known complication of the investigational therapy that may occur when the engineered cells become activated in the patient's body. CRS was managed on a global scale using prior site education with implementation of the CRS treatment algorithm. There were no deaths due to CRS. Fifteen percent of patients experienced grade 3 neurological and psychiatric events including encephalopathy and delirium, with no grade 4 events seen.[1]

"These global multicenter trial data build on earlier encouraging research conducted at a single trial site, and advance the case for CTL019 as a potential treatment for children and young adults with relapsed or refractory B-cell ALL," said lead investigator Stephan Grupp, MD, PhD, the Yetta Deitch Novotny Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania (Penn), and Director of the Cancer Immunotherapy Frontier Program at the Children's Hospital of Philadelphia (CHOP).

In addition to filing CTL019 for approval with the FDA in early 2017, Novartis plans to file with the European Medicines Agency (EMA) later in 2017. The investigational therapy received PRIME (PRIority MEdicines) designation from the EMA earlier this year.

"This first-of-its-kind trial represents exciting progress toward our goal of helping children and young adults with relapsed or refractory B-cell ALL, a patient population with an urgent need for new treatment options," said Bruno Strigini, CEO, Novartis Oncology. "We are committed to advancing CTL019 and look forward to working closely with the FDA and EMA in the coming months."

Dr. Shannon Maude from CHOP will give a poster presentation highlighting data from ENSIGN, the first US multicenter Phase II trial for CTL019 in pediatric and young adults with B-cell ALL (Abstract #2801, December 4, 6:00-8:00 p.m.).[2] A separate poster presentation will also highlight an ongoing Phase IIa study led by Penn which investigated the efficacy and safety of CTL019 in poor prognostic groups of relapsed or refractory diffuse large B-cell lymphoma (DLBCL) patients (Abstract #3026, December 4, 6:00-8:00 p.m.).[3]

Because CTL019 is an investigational therapy, the safety and efficacy profile has not yet been established. Access to investigational therapies is available only through carefully controlled and monitored clinical trials. These trials are designed to better understand the potential benefits and risks of the therapy. Because of uncertainty of clinical trials, there is no guarantee that CTL019 will ever be commercially available anywhere in the world.

Disclaimer

The foregoing release contains forward-looking statements that can be identified by words such as "will," "investigational," "build on," "encouraging," "advance the case," "potential," "exciting," "goal," "committed," "look forward," "yet," or similar terms, or by express or implied discussions regarding potential marketing approvals for CTL019, or regarding potential future revenues from CTL019. You should not place undue reliance on these statements. Such forward-looking statements are based on the current beliefs and expectations of management regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that CTL019 will be submitted or approved for sale in any market, or at any particular time. Nor can there be any guarantee that CTL019 will be commercially successful in the future. In particular, management's expectations regarding CTL019 could be affected by, among other things, the uncertainties inherent in research and development, including unexpected clinical trial results and additional analysis of existing clinical data; unexpected regulatory actions or delays or government regulation generally; the company's ability to obtain or maintain proprietary intellectual property protection; general economic and industry conditions; global trends toward health care cost containment, including ongoing pricing pressures; unexpected safety, quality or manufacturing issues, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

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Novartis is on Twitter. Sign up to follow @Novartis at http://twitter.com/novartis and @NovartisCancer at http://twitter.com/novartiscancer.

References

1. Grupp, Stephen A. et al. Analysis of a Global Registration Trial of the Efficacy and Safety of CTL019 in Pediatric and Young Adults with Relapsed/Refractory Acute Lymphoblastic Leukemia (ALL). Session 614, Saturday, December 3, 5:00 p.m. PST. 58th American Society of Hematology Annual Meeting and Exposition: Abstract 221.
2. Maude, Shannon L. et al. Efficacy and Safety of CTL019 in the First US Phase II Multicenter Trial in Pediatric Relapsed/Refractory Acute Lymphoblastic Leukemia: Results of an Interim Analysis. Session 614, Sunday, December 4, 6:00 – 8:00 p.m. PST. 58th American Society of Hematology Annual Meeting and Exposition: Abstract 2801.
3. Schuster, Stephen J. et al. Treatment with Chimeric Antigen Receptor Modified T Cells Directed Against CD19 (CTL019) Results in Durable Remissions in Patients with Relapsed or Refractory Diffuse Large B Cell Lymphomas of Germinal Center and Non-Germinal Center Origin, "Double Hit" Diffuse Large B Cell Lymphomas, and Transformed Follicular to Diffuse Large B Cell Lymphomas. Session 626, Sunday, December 4, 6:00 – 8:00 pm PST. 58th American Society of Hematology Annual Meeting and Exposition: Abstract 3026.

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