Novartis highlights new CTL019 Phase II data demonstrating 93% complete remission in pediatric patients with r/r ALL

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- - 55 of 59 patients experienced complete remissions in a single-arm study of the largest investigational CART therapy in children and young adults [1]
- - Additional data presented include analyses on safety and efficacy of CTL019 in r/r ALL and other CART therapies in r/r ALL and AML [2,3,4,5]

EAST HANOVER, N.J., Dec. 7, 2015 /PRNewswire/ -- The latest findings from an ongoing Phase II study of CTL019, an investigational chimeric antigen receptor T cell (CART) therapy, further support its potential in the treatment of children and young adults with relapsed/refractory acute lymphoblastic leukemia (r/r ALL). The study found that 55 of 59 patients (93%) experienced complete remissions (CR) with CTL019. These results will be presented in an oral session at the 57th American Society of Hematology (ASH) Annual Meeting on Monday, December 7 (Abstract #681, 3:15 p.m.)[1].

In the study, median follow up was 12 months, overall survival was 79% at 12 months (95% CI, 69-91%) and relapse-free survival was 76% at six months (95% CI, 65-89%) and 55% at 12 months (95% CI, 42-73%). Results found that 18 patients had ongoing CR after 12 months of therapy[1].

"This clinical trial of CTL019 is the largest study of a CART therapy in pediatric patients with relapsed or refractory acute lymphoblastic leukemia, and it is helping us better understand the therapy's potential to achieve durable responses in this patient population," 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 Translational Research in the Center for Childhood Cancer Research at the Children's Hospital of Philadelphia (CHOP). The ongoing study of CTL019 in pediatric patients with r/r ALL is being led by Dr. Grupp at CHOP and is sponsored by Penn.

Additionally, 52 of 59 (88%) patients developed Grade 1-4 cytokine release syndrome (CRS). CRS may occur after CTL019 infusion when the engineered cells become activated and multiply in the patient's body. During CRS, patients typically experience varying degrees of flu-like symptoms with high fevers, nausea, muscle pain, and in some cases, low blood pressure and breathing difficulties. Treatment for CRS was required for hemodynamic or respiratory instability in 27% of patients and was reversed in all cases with an IL-6 receptor antagonist[1].

"We have observed pediatric patients in this study achieve complete remissions with CTL019 treatment, in many cases without stem cell transplantation, which underscores the potential for CTL019 to fill an unmet medical need," said Usman Azam, MD, Global Head, Cell & Gene Therapies Unit, Novartis Pharmaceuticals. "These new longer-term data add to the growing understanding of CTL019 for patients with relapsed or refractory acute lymphoblastic leukemia who run out of treatment options."

Novartis recently expanded its own global multisite Phase II clinical trial of CTL019 in pediatric r/r ALL with the opening of study sites in Europe, Canada and Australia. A list of participating trial centers is available at

https://clinicaltrials.gov/.

Novartis and Penn have an exclusive global collaboration to research, develop and commercialize CART therapies for the investigational treatment of cancers. In July 2014, the FDA designated CTL019 as a Breakthrough Therapy for the treatment of pediatric and adult patients with r/r ALL under the Penn Investigational New Drug application (IND). Breakthrough Therapy designation is intended to expedite the development and review of drugs that treat serious or life-threatening conditions if the therapy has demonstrated substantial improvement over an available therapy on at least one clinically significant endpoint. Novartis holds the worldwide rights to CARs developed through the collaboration with Penn for all cancer indications, including the lead program, CTL019.

Additional key CART data presented at ASH include:

- Findings from a study on treatment with CTL019 in children with r/r ALL that face additional central nervous system (CNS) relapse complications (Abstract #3769, December 7, 6-8 p.m.)[2]
- Data on characterization and accurate early prediction of CRS in r/r ALL patients treated with CTL019 (Abstract #1334, December 5, 5:30 p.m.)[3]
- Preliminary efficacy and safety data on humanized CTL119 in children with r/r ALL (Abstract #683, December 7, 3:45 p.m.)[4]
- Preclinical data from a study on CART123 to mitigate toxicity in acute myeloid leukemia (Abstract #565, December 7, 10:30 a.m.)[5]

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 "investigational," "potential," "will," "ongoing," "encouraged," "growing," "Breakthrough Therapy," "intended," "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 issues; unexpected manufacturing or quality 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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Located in East Hanover, New Jersey, Novartis Pharmaceuticals Corporation is an affiliate of Novartis AG, which provides innovative healthcare solutions that address the evolving needs of patients and societies. Headquartered in Basel, Switzerland, Novartis offers a diversified portfolio to best meet these needs: innovative medicines, eye care and cost-saving generic pharmaceuticals. Novartis is the only global company with leading positions in these areas. In 2014, the Group achieved net sales of USD 58.0 billion, while R&D throughout the Group amounted to approximately USD 9.9 billion (USD 9.6 billion excluding impairment and amortization charges). Novartis Group companies employ approximately 120,000 full-time-equivalent associates. Novartis products are available in more than 180 countries around the world. For more information, please visit http://www.novartis.com.

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References

1. Grupp, Stephan A. et al. (7 December 2015). Durable Remissions in Children with Relapsed/Refractory ALL Treated with T Cells Engineered with a CD19-Targeted Chimeric Antigen Receptor (CTL019) [oral presentation]. 57th American Society of Hematology Annual Meeting & Exposition: Abstract 681

2. Rheingold, Susan R. et al. (7 December 2015). Efficient Trafficking of Chimeric Antigen Receptor (CAR)-Modified T Cells to CSF and Induction of Durable CNS Remissions in Children with CNS/Combined Relapsed/Refractory ALL [poster presentation]. 57th American Society of Hematology Annual Meeting & Exposition: Abstract 3769

3. Teachey, David T. et al. (5 December 2015) Characterization and Accurate Early Prediction of Cytokine Release Syndrome After Chimeric Antigen Receptor (CAR) T cell Therapy for Acute Lymphoblastic Leukemia (ALL) [poster presentation]. 57th American Society of Hematology Annual Meeting & Exposition: Abstract 1334

4. Maude, Shannon L. et al. (7 December 2015). Efficacy and Safety of Humanized Chimeric Antigen Receptor (CAR)-Modified T Cells Targeting CD19 in Children with Relapsed/Refractory ALL [oral presentation]. 57th American Society of Hematology Annual Meeting & Exposition: Abstract 683

5. Tasian, Sarah K. et al. (7 December 2015). Efficient Termination of CD123-Redirected Chimeric Antigen Receptor T Cells for Acute Myeloid Leukemia to Mitigate Toxicity [oral presentation]. 57th American Society of Hematology Annual Meeting & Exposition: Abstract 565

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List of links present in page

1. https://prod1.novartis.com/us-en/us-en/news/media-releases/novartis-highlights-new-ctl019-phase-ii-data-demonstrating-93-complete-remission-pediatric-patients-rr-all
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