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CLINICAL

Safety and efficacy of autologous CAR T cells targeting GD2 (GD2-CART01) in patients with high-risk metastatic, relapsed, or refractory neuroblastoma: updated analysis (was selected for ORAL presentation)

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Background: Anti-disialoganglioside (GD2), third-generation chimeric antigen receptor (CAR) T-cells, GD2-CART01, have shown high overall response rate (ORR) and promising survival in children with high-risk metastatic, relapsed, or refractory neuroblastoma (HR/R/Refr.-NB) at the interim analysis of a phase 1/2 clinical trial.

Methods:/Patients Herein we present the final results obtained in all 35 patients enrolled in the study (NCT03373097) and in 19 additional children selected with the same criteria of the trial and treated in a hospital exemption setting.

Results: GD2-CART01 persisted ≥12 months in 47% of the patients. No new safety signal was observed; grade 3 immune effector cell-associated neurotoxicity syndrome, diagnosed in 4 children, was rapidly controlled with the activation of the inducible caspase-9 suicide gene through the infusion of the dimerizing agent rimiducid. The ORR of the patients enrolled in the clinical trial is 66% (21/32- excluding the 3 patients treated in non-evidence of disease). With a median follow-up of 4.2 years, the 5-year overall survival (OS) for the CT cohort is 42,67%. Among the 54 patients of the whole study cohort, 38 children were treated with low disease burden at $10x10^6$ GD2-CART01 cells/kg (this representing the target population), including 8 patients consolidated in non-evidence of disease at the end of first-line. The ORR in the target population was 77% and the 5-year OS and event-free survivals (EFS) were 78% and 53%, respectively. Significantly superior 5-year OS and EFS were observed in patients treated after 1-2 lines of therapy as compared to those treated after >3 (89% and 71%, versus 43% and 32%, respectively). Better results were observed in patients whose lymphocyte collection was performed at time of NB diagnosis.

Conclusions: These results obtained confirm that GD2-CART01 can induce durable remissions in children with HR/R/Refr.-NB.

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Clinicobiological therapeutic stratification leads to excellent prognosis in *MYCN*-NA disseminated NB patients ≤12 m. Results of SIOPEN LINES trial (was selected for ORAL presentation)

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Background: In SIOPEN-LINES trial, disseminated *MYCN*-non-amplified neuroblastoma patients ≤12 months (m) were stratified according to metastatic compartment, life-threatening symptoms (LTS) and genomic profile (numerical/segmental, NCA/SCA). Groups-4/5/6 included patients without (group-4: NCA-only/LTS-negative; group-5: NCA-only/LTS-positive; group-6: SCA-positive) and with bone/lung/central nervous system (CNS) metastases (group-10). The aims of this study were to minimize treatment while maintaining event-free survival (EFS) and overall survival (OS) for groups-4/5, and keep moderate treatment for groups-6/10.

Methods: Group-4 underwent observation; if progression with LTS occurred, patients received chemotherapy according to group-5. Group-5 received 2 etoposide-carboplatin (VP/Carbo) +/- 2 cyclophosphamide-doxorubicin-vincristine (CADO) cycles until LTS-negative. In Group-6, LTS-negative patients received 4x[VP/Carbo]; LTS-positive received 2x[VP/Carbo]: if LTS resolved, they received additional 2x[VP/Carbo], if not, 2x[CADO]. Group-10 received up to 4x[VP/Carbo] +/- 4x[CADO] until metastatic remission. Groups-6/10 primary tumor was resected if no image-defined risk factors.

Results: 191 patients were enrolled into Groups-4/5/6/10 (median follow-up: 66.3m). In Group-4 (n = 49), n = 13 patients developed progression/LTS after observation (median time-to-progression, mTTP: 2.8m); n = 1 died of disease (DOD). In Group-5 (n = 52), n = 24 (46%) received VP/Carbo (median number courses, mNC: 2); n = 27 (52%) received VP/Carbo + CADO (mNC: 4); n = 5 relapsed/progressed (mTTP: 8m); n = 1 DOD. In group-6 (n = 37), n = 14 and n = 23 started with and without LTS, respectively; n = 31 (84%) received VP/Carbo (mNC: 4); and n = 5 (14%) received VP/Carbo + CADO (mNC: 4); n = 10 relapsed/progressed (mTTP 12.7m); n = 2 DOD. In group-10 (n = 53), n = 19 (36%) received VP/Carbo (mNC: 4); n = 34 (64%) received VP/Carbo + CADO (mNC: 8); n = 8 relapsed/progressed (mTTP: 8.5m); n = 1 DOD. Five-year EFS/OS were, respectively: Group-4 73.3 ± 6%/97.9 ± 2%; Group-5 87.9% ± 4%/98.1 ± 1%; Group-6 74.9 ± 7%/94.3 ± 3%; Group-10 84 ± 5%/100%.

Conclusions: In SIOPEN-LINES trial, therapeutic stratification according to clinical (metastatic compartment, symptoms) and biological (genomic profile) criteria allowed optimal survival. Patients without bone/lung/CNS metastases and NCA-only have good prognosis with low treatment burden. Patients without bone/lung/CNS metastases and SCA, and those with bone/lung/CNS involvement, need moderate treatment to reach same survival rates.

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Real-world data from SACHA-France on chemo-immunotherapy for relapsed high-risk neuroblastoma: predictive factors for outcome (was selected for ORAL presentation)

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