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CAR-T 細胞の臨床応用／CAR-T in Clinics

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2019/09/26 13:00~15:30 Room2（２F　Room A）

小児固形がんに対する CAR-T 細胞療法の開発

Preclinical evaluation of *piggyBac* transposon mediated CAR-T cells in the treatment of solid tumors

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Background Chimeric antigen receptor T (CAR-T) cells engineered by non-viral gene transfer can be produced at an affordable cost as compared to virally engineered CAR-T cells. In this study, we developed novel chimeric antigen receptor T cells using *piggybac* (PB) transposon-based gene transfer for the clinical application of these cells in the treatment of pediatric solid tumors.
Methods and results We have developed the optimized protocol for clinical scale manufacture of three PB-based CAR-T cells targeting rhabdomyosarcoma, neuroblastoma and osteosarcoma, respectively. All of CAR-T cells were successfully generated during 14 days culture with robust cell expansion, good CAR positivity and predominant population of central memory phenotype. PB-CART cells demonstrated sustained killing activity against tumor cells even in multiple tumor re-challenges in vitro, and debulked tumors in vivo.
Conclusion PB-based CART cell therapy is promising for the treatment of solid tumors. A non-clinical study of PB-based CART cell therapy targeting pediatric soft tissue sarcoma and osteosarcoma is underway, precluding future clinical trials in Japan.