Aim: When this trial was planned, A was believed to be a promising new anthracycline agent for sensitive-relapsed SCLC. While re-challenge of P that had been used for the first-line treatment was also believed to be effective for sensitive-relapsed SCLC, although prospective evaluation of it had not been reported. Thus this randomized phase 2 study was conducted to select A or P for future phase 3 trial.

Methods: Sensitive-relapsed SCLC pts were randomized to receive A (40mg/m², day1-3, every 3 weeks) or P (every 3-4 weeks). The modification of P such as the 20%-dose reduction of combined third-generation or change of platinum agent from cisplatin to carboplatin according to patient’s condition was permitted. The primary endpoint was overall response rate (ORR), and secondary endpoint were progression-free survival (PFS), overall survival (OS), and toxicity profile. According to the Simon’s two-stage phase 2 design, 28 pts were required in each arm (p0=0.3, p1=0.5, alpha=0.01, beta=0.02), and the treatment that achieved not less 12 out of 28 pts with partial response would be judged as effective.

Results: From February 2008 to June 2013, 60 pts were enrolled from 14 institutions. Two patients in the A arm and one patient in the P arm did not receive any protocol treatment due to rapid disease progression. Evaluated patients’ characteristics were as follows: Male/Female, 53/4; median age, 66 (range 44-80); performance status 0/1/2, 32/21/4. The median number of treatment cycles was 4 (range 2-8) in A arm and 3 (range 1-7) in P arm. ORRs and disease control rates were 67% (95%CI, 50-84) and 86% for A, and 43% (95%CI, 25-61) and 80% for P, respectively. Median PFS and OS were 5.4 months and 14.4 months in A arm, and 5.1 months and 14.3 months in P arm. Grade 3 toxicity was observed in 33% of patients in A arm including 19% of febrile neutropenia, while 17% in P arm without any febrile neutropenia. There was no grade 4 toxicity or treatment-related death in this trial.

Conclusions: Both treatments met the primary endpoint. Since A produced higher ORR and longer median PFS than P with acceptable toxicity, we select A for subsequent phase 3 trial.

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