Efficacy population: all eligible treated patients who completed ≥ 2 doses of nab-Paclitaxel

Phase 1: to describe the nab-Paclitaxel maximum tolerated dose-escalated phase 2 dose
• Dose-determining set: all patients who experienced a DLT or received 3 weekly nab- Paclitaxel doses during phase 1 (n = 41 RECIST and n = 3 MIBG/Curie)
• DLT-defined: Grade ≥ 2 toxicity leading to discontinuation, n (%) 1 (9) 1 (13) 1 (14) 1 (13) 1 (9) 1 (13)
• Grade 3 or 4 neutropenia or severe anemia
• Grade 3 or 4 nonhematologic toxicity (excluding transient transaminitis)
• Grade 3 or 4 peripheral neuropathy

Phase 1 Endpoints
• Phase 1: to determine the maximum tolerated dose of nab-Paclitaxel as part of a rolling-6 dose-escalation design
• Phase 1: to determine the optimal dose of nab-Paclitaxel for phase 2

Phase 2 Treatment Exposure and Safety
• Grade 1/2 peripheral neuropathy and hand-foot-syndrome occurred in 16% and 6% of patients
• At all dose levels, grade 3/4 AEs were mainly hematologic (Table 3)

Phase 1: to describe the nab-Paclitaxel maximum tolerated dose-escalated phase 2 dose, safety, and pharmacokinetic profile as well as preliminary clinical activity in pediatric patients with recurrent/metastatic solid tumors

Methodology
• Although solvent-based paclitaxel has demonstrated activity in children with refractory solid tumors, hypersensitivity and neurotoxic reactions have been dose-limiting and thereby compromise efficacy.
• nab-Paclitaxel is aalbumin-bound, solvent-free formulation of paclitaxel that has demonstrated efficacy and safety in adults with various solid tumors.

Objectives
• Phase 1: to describe the nab-Paclitaxel maximum tolerated dose-escalated phase 2 dose, safety, and pharmacokinetic profile as well as preliminary clinical activity in pediatric patients with recurrent/metastatic solid tumors

Study Design
• The recommended phase 2 dose was selected based on the results of the phase 1 study. The safety and efficacy of nab-Paclitaxel at the recommended phase 2 dose were further evaluated in a subsequent pediatric phase 2 trial.

Results
• Sixty-five patients were enrolled; 1 was ineligible for treatment and 64 were treated
• Of 44 efficacy-evaluable patients, 1 (2%) and 6 (14%) had complete and partial responses, leading to discontinuation, n (%) 1 (6) 0 1 (9) 1 (13) 1 (14)

Selection of Recommended Phase 2 Dose
• Of the 210 mg/m2 dose level, 9 patients were enrolled and 6 were treated
• Of the 240 mg/m2 dose level, 3 patients were enrolled and 2 were treated

DISCLOSURES
• All authors disclose no potential conflicts of interest

REFERENCES