Abstract 11565: Palbociclib in patients with soft tissue sarcoma with *CDK4* amplification: Results from the Targeted Agent and Profiling Utilization Registry (TAPUR) Study

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Background:

- TAPUR is a phase II basket study that evaluates anti-tumor activity of commercially available targeted agents in patients (pts) with advanced cancers with specific genomic alterations.
- **Results of a cohort of soft tissue sarcoma (STS) pts with *CDK4* amplification and no *RB* mutations treated with palbociclib are reported.**

Methods:

Study Design:

- **Eligible pts:** Advanced STS, no standard treatment (tx) options, ECOG PS 0-2, adequate organ function, measurable disease. Tx assigned according to pre-specified matching rules based on genomic tests selected by sites.
- Pts received palbociclib at 125 mg once daily for 21 days, followed by 7 days off until disease progression. Tumor evaluations performed at 8 and 16 wks then Q12 wks after treatment initiation.
- **Primary endpoint:** Disease control (DC) defined as objective response (OR) or stable disease (SD) at 16+ wks per RECIST v1.1. **Secondary endpoints:** Progression-free survival (PFS), overall survival (OS), and toxicity per CTCAE. Grade 3-5 adverse events (AEs) or serious adverse events (SAEs) at least possibly related to palbociclib are reported.

Statistical Methods:

- Simon's optimal two-stage design used to test null hypothesis of 15% DC rate vs. alternative of 35%. Power = 85%; 1-sided α = 10%.
- At least 7 of 28 pts must achieve DC to reject null hypothesis and consider treatment worthy of further study.

Palbociclib has anti-tumor activity in heavily pre-treated patients with soft tissue sarcoma with *CDK4* amplification.

Future Direction: Additional study is warranted to confirm the efficacy of palbociclib in this patient population.

Results:

- 29 pts enrolled July 2016 to November 2019.
- **Demographics**: Median age 64 y (range 41-85); 66% male; 24 pts (83%) liposarcoma.
- **Clinical characteristics:** 28% PS 0,72% PS 1; 52% received ≥3 prior systemic regimens; 48% received 1-2 prior regimens.
- **Outcomes**: 1 partial response (PR), and 12 SD16+ (Table 1 and Figure 1). Time on palbociclib among pts with SD16+ and OR is shown in Figure 2.
- **Safety:** 14 pts (48%) had ≥1 Grade 3-4 AE at least possibly related to palbociclib and consistent with known safety profile.

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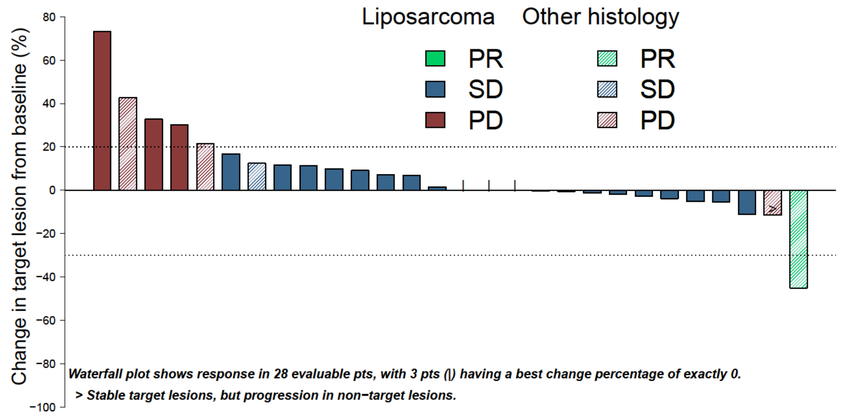
Table 1: Efficacy Outcomes (N=28)[1]	
DC rate, % (95% CI)	47 (30, 60)
OR rate, % (95% CI)	4 (0, 18)
Median PFS, wks (95% CI)	16.1 (11.6, 28.1)
1 year OS, % (95% CI)	53.6 (38.0, 75.6)

[1]1 *enrolled pt was not evaluable and excluded from efficacy analyses*

Figure 1: Best Percent Change from Baseline in Target Lesion Size (N=28)



Waterfall plot shows response in 28 evaluable pts, with 3 pts (|) having a best change percentage of exactly 0.
> Stable target lesions, but progression in non−target lesions.

Figure 2: Time on Treatment in Pts with SD16+ or OR (N=13)