TOPOTARGET A/S

BELINOSTAT TRIAL IN CANCER OF UNKNOWN PRIMARY (CUP)

TOP-LINE DATA OF THE PRIMARY ANALYSIS

JUNE 29, 2012

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BelCaP in CUP – PXD101-CLN-17

An open-label randomized phase II trial of belinostat (PXD101) in combination with carboplatin and paclitaxel (BelCaP) compared to carboplatin and paclitaxel in patients with previously untreated carcinoma of unknown primary

Trial design/background

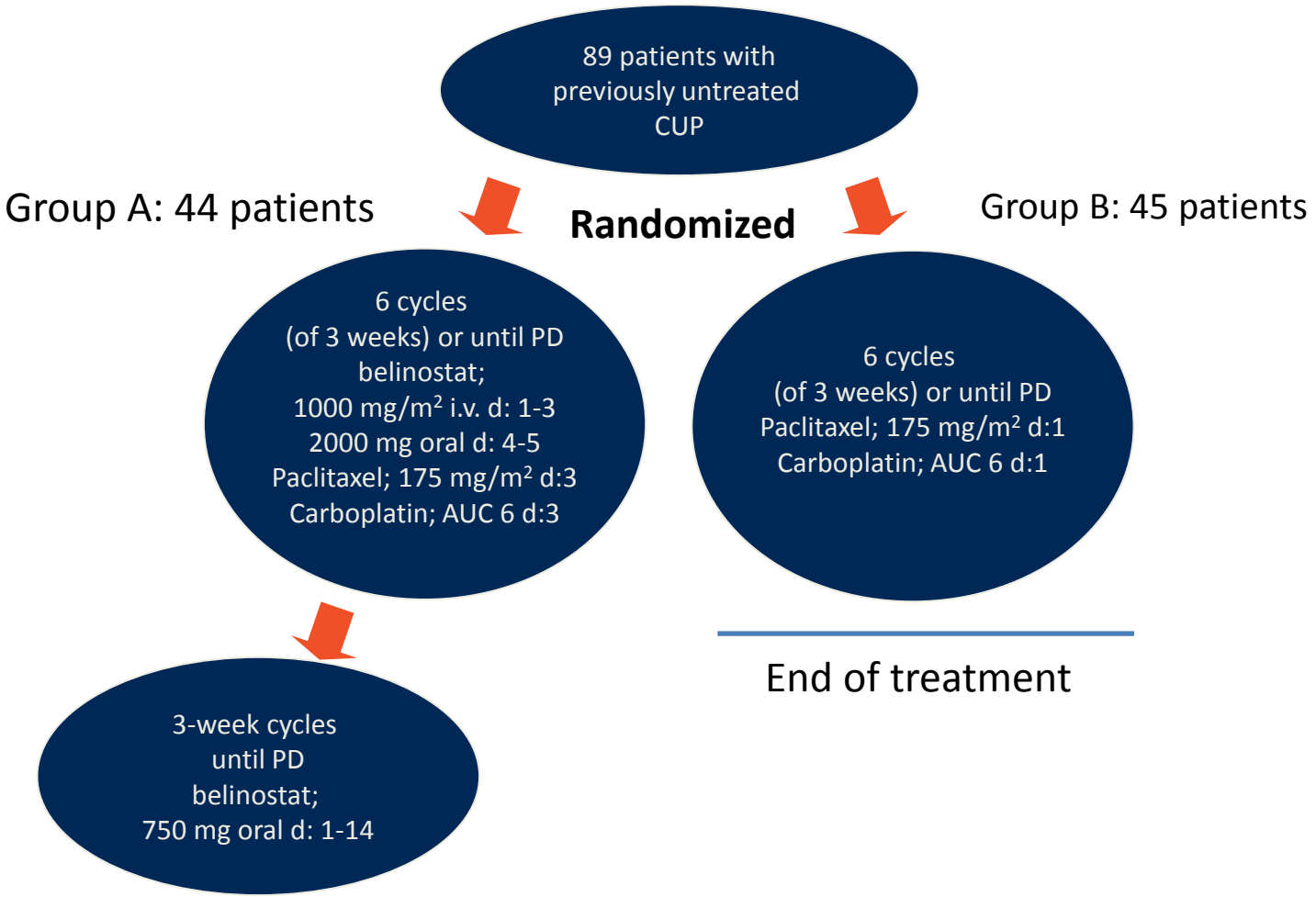
- **Group A**

  Belinostat (1000 mg/m^2) administered IV infusion once daily on days 1, 2, and 3, followed by belinostat 2000 mg (flat dose) administered orally once daily on days 4 and 5, every 3 weeks, in combination with paclitaxel (175 mg/m^2) administered IV, following the infusion of belinostat on cycle day 3, and carboplatin (AUC 6)

- **Group B**

  Paclitaxel (175 mg/m^2) administered as an IV infusion directly followed by carboplatin (AUC 6) on cycle day 1 of a 3-week cycle

BelCaP versus CaP in CUP: Trial design



Continue until PD or stop due to other reasons

Primary endpoint: PFS

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Main inclusion/exclusion criteria

- **Inclusion criteria:**
  - Patients with CUP where the primary site had not been revealed by complete history, physical examination, CT scan, etc.
  - At least one measurable lesion according to RECIST criteria
  - Performance status (ECOG) ≤2
  - Age ≥18 years

- **Exclusion criteria:**
  - Prior systemic anti-tumor therapy/investigational therapy <4 weeks prior to randomization, including chemotherapy administered in association to radiotherapy for sensitization, for CUP
  - Pregnancy

Objectives

Primary objective

- To provide an estimate of the hazard ratio of treatment effect when the combination of belinostat plus carboplatin and paclitaxel (Group A) is compared with the combination of carboplatin and paclitaxel (Group B) in terms of PFS for patients with CUP

Secondary efficacy objective

- To evaluate and compare further efficacy parameters (overall survival, objective response rate according to RECIST criteria, time to response, duration of response, and time to progression) in the randomized treatment groups

TRIAL RESULTS

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Summary

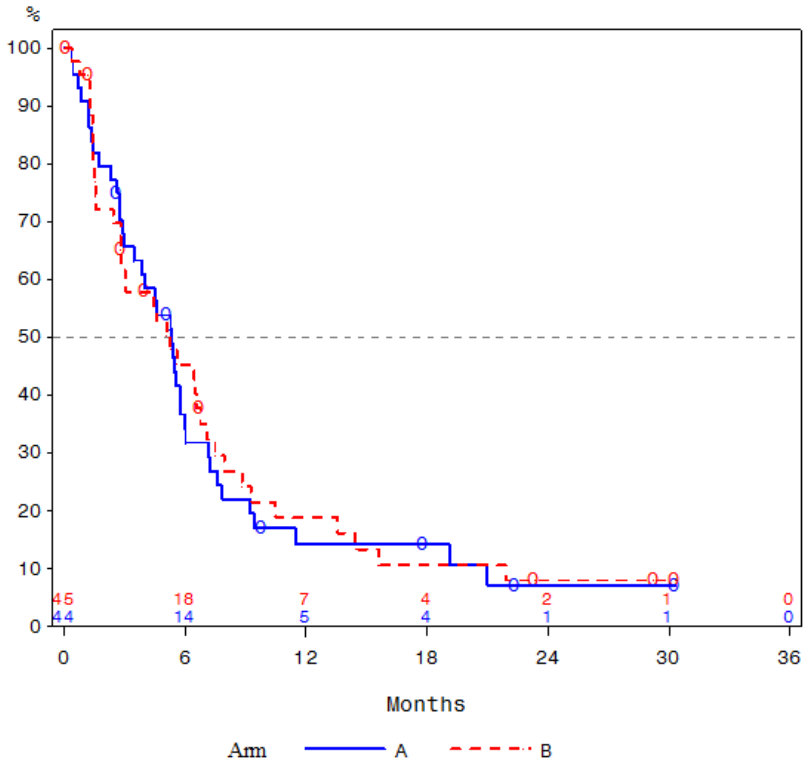
- 89 patients with CUP
  - ITT (intent to treat): 44 in Group A, 45 in Group B
  - PP (per protocol): 42 in Group A and 43 in Group B
- The two groups are well-balanced

Overall findings

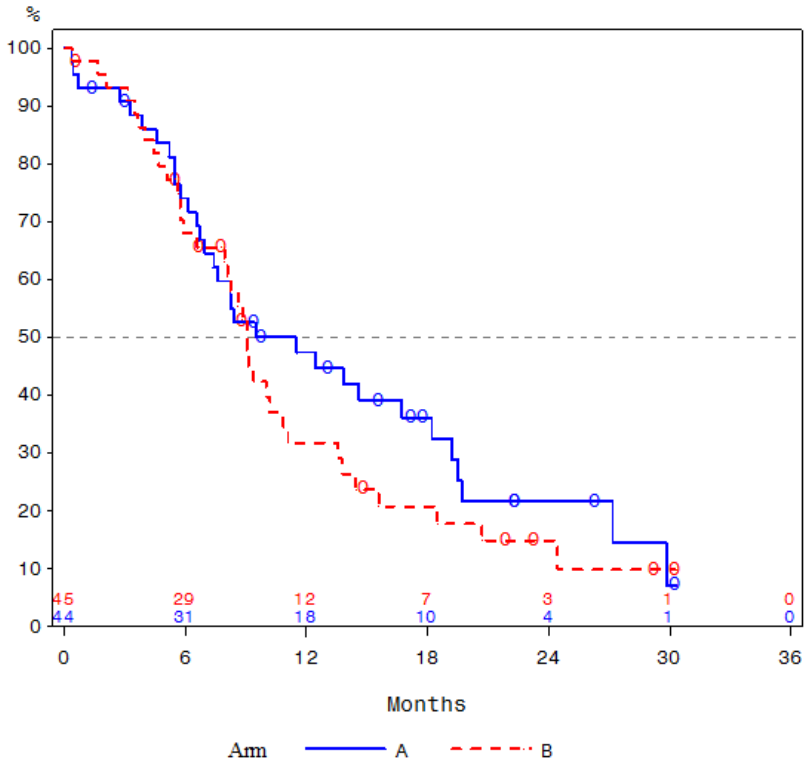
- The primary efficacy analysis of PFS for BelCaP versus CaP in ITT did not show statistical significance at the 10% level for the stratified, one-sided log-rank test. P-value is 0.5526 and median PFS is 5.4 versus 5.3 months
- The ORR in ITT, 43.2% versus 22.2%, is statistically significant, p = 0.0252
- The OS result is statistically not significant but indicates that the survival curves for OS separate after 8 months of treatment in favor of belinostat with p = 0.2906
- BelCaP compared to CaP during the first 6 cycles and during the maintenance where belinostat was given as mono treatment was generally well-tolerated

Endpoints

Progression-free survival (PFS)



Overall survival (OS)



ITT population

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BelCaP generally well-tolerated during the 6 cycles compared to CaP

	Belinostat + carboplatin + paclitaxel Group A		Carboplatin + paclitaxel Group B	
Safety analysis set	**42**		**44**	
	No of pts	**(%)**	**No of pts**	**(%)**
Any grade 3 or 4 TEAEs	**25**	**(59.5%)**	**19**	**(43.2%)**
Adverse Event term:				
Neutropenia/leucopenia	9	(21.4%)	7	(15.9%)
Neuropathy	2	(4.8%)	3	(6.8%)
Vomiting	7	(16.7%)	3	(6.8%)
Nausea	11	(26.2%)	5	(11.4%)
Fatigue	10	(23.8%)	6	(13.6%)
Diarrhoea	3	(7.1%)	1	(2.3%)
Anaemia/hemoglobin decreased	2	(4.8%)	2	(4.5%)
Pain (10 PT terms)	4	(9.5%)	4	(9.1%)
Thrombocytopenia/platelet count decreased	6	(14.3%)	6	(13.6%)
Drug hypersensitivity	4	(9.5%)	1	(2.3%)
Vena cava thrombosis/pulmonary embolism	6	(14.3%)	1	(2.3%)
Decreased appetite	1	(2.4%)	4	(9.1%)
Dehydration	4	(9.5%)	1	(2.3%)

Incidence of selected TEAE (grade 3 or 4) by treatment group - safety

Conclusion

- Currently no drugs are approved for the treatment of patients with CUP and there remains a high unmet medical need
- The study did not meet the primary efficacy endpoint of PFS
- There is a promising separation of OS – curves after eight months
- The signal for response rate is encouraging
- Maintenance treatment with oral belinostat is generally well-tolerated allowing for prolonged treatment past 6 cycles of the BelCaP combination
- Further analyses are on-going to evaluate potential benefit for specific patient groups