**Phase I, Open-Label, Dose-Escalation Study of the Safety, Tolerability, and Pharmacokinetics of DMUC4064A Administered Intravenously to Patients With Platinum-Resistant Ovarian Cancer or Unresectable Pancreatic Cancer**

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<th><strong>Phase</strong></th>
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<tbody>
<tr>
<td><strong>Drug Class</strong></td>
<td>Tumor Specific Targeted Agents: Antibody Drug Conjugate (ADC)</td>
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<tr>
<td><strong>Drug Name</strong></td>
<td>DMUC4064A</td>
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<td><strong>Alternate Drug Names</strong></td>
<td>Anti-MUC16 Thiomab™ Drug Conjugate (TDC)</td>
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<td><strong>Eligible Participant</strong></td>
<td>Platinum-resistant ovarian cancer</td>
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<td><strong>Patients Enrolled</strong></td>
<td>Dose escalation: 44 ovarian, median of 6 prior therapies (1-14), Expansion: 20 ovarian</td>
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<td><strong>Therapy Setting</strong></td>
<td>Recurrence</td>
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<td><strong>Study Design</strong></td>
<td>Open Label, Non-Randomized</td>
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<td><strong>Endpoints</strong></td>
<td>Recommended Phase 2 Dose (RP2D); ORR; PFS</td>
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<td><strong>Biomarkers</strong></td>
<td>MUC16 IHC score</td>
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| **Efficacy** | RP2D: 5.2mg/kg  
Dose escalation:  
**ORR**: 23% (1 CR, 6 PR, n=30) at doses ≥3.2 mg/kg and MUC16 IHC score 2+/3+  
Expansion cohort at RP2D (5.2mg/kg) (n=20):  
**ORR**: 45% (1 CR, 8 PR)  
**PFS**: 5.8 months |
| **Clinically Significant Adverse Events** | Dose Limiting Toxicities: at 5.2 mg/kg: grade 3 colitis, hyperglycemia, and hypokalemia (n=1)  
Serious AE: none  
Grade 3-4 AE: hyponatremia (11%), ascites (7%), hyperglycemia (7%), keratitis (10%) |
| **Conclusion** | Anti-tumor activity with acceptable safety profile |
| **Reference** | Liu JF et al. Targeting MUC16 with the THIOMAB™-drug conjugate DMUC4064A in patients with platinum-resistant ovarian cancer: a Phase I escalation study  
AACR (2017) Abstract CT009 [http://cancerres.aacrjournals.org/content/77/13_Supplement/CT009](http://cancerres.aacrjournals.org/content/77/13_Supplement/CT009)  
Moore K et al., Targeting MUC16 with the THIOMAB™-drug conjugate DMUC4064A in patients with platinum-resistant ovarian cancer: A phase I expansion study  
**Legend**

**Therapy Setting**

**First-line** – Therapy given to patients on initial diagnosis of disease as the first, best treatment option.

**Maintenance** – Therapy given to patients to help keep cancer from coming back after it has responded to therapy.

**Recurrence** – Therapy given to patients in whom disease has returned after prior therapy.

**Study Design**

**Randomized** -- A study in which participants are assigned by chance to the separate study groups.

**Non-randomized** -- A study in which participants are NOT assigned by chance to the separate study groups.

**Efficacy Endpoints**

**PFS: Progression-Free Survival**—length of time during and after treatment during which the cancer does not get worse (usually reported as the time when the cancer for half—or median—of the people in the treatment group gets worse).

**OS: Overall Survival**—length of time from the start of treatment that patients are still alive (usually reported as the time when half—or median—of the people in the treatment group are still alive).

**CR: Complete Response** -- The disappearance of all signs of cancer in response to treatment.


**SD: Stable Disease Response** -- Cancer that is neither decreasing nor increasing in extent or severity.

**ORR: Objective Response Rate** -- Sum of complete and partial tumor responses to treatment, divided by the number of patients evaluated.

**DCR: Disease Control Rate** -- Sum of complete, partial and stable disease tumor responses to treatment, divided by the number of patients evaluated.

**HR: Hazard Ratio**—measures survival in the treatment group compared to the control group. An HR = 1 means that there is no difference in survival between the groups. An HR < 1 means that the treatment group has a lower risk of death compared to the control group. Range in parentheses is 95% Confidence Interval (CI).

**RECIST: Response Evaluation Criteria in Solid Tumors** -- Set of rules, based on measurements of the change in tumor size that define when cancer patients improve, stabilize, or worsen during a treatment regimen.

**CA125: GCIG CA125 Criteria** -- Set of rules, based on measurements of the CA125 biomarker level that define when cancer patients improve, stabilize, or worsen during a treatment regimen.

**Clinically Significant Adverse Events** (Based on National Cancer Institute--Common Terminology Criteria for Adverse Events (CTCAE))

**AE: Adverse events** -- any undesirable experience associated with the use of a drug.

**SAE: Serious adverse events** – untoward event associated with drug treatment e.g., death, life-threatening, requiring of hospitalization, persistent or significant incapacity; usually graded from 1-5.