<table>
<thead>
<tr>
<th>Study ID</th>
<th>Clinical Trial Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>NCT01472783</td>
<td>Veliparib Monotherapy for Relapsed Ovarian Cancer With BRCA Mutation (Veli-BRCA)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th><strong>Phase</strong></th>
<th>I/II</th>
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</thead>
<tbody>
<tr>
<td><strong>Drug Class</strong></td>
<td>PARP Inhibitor</td>
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<tr>
<td><strong>Drug Name</strong></td>
<td>Veliparib</td>
</tr>
<tr>
<td><strong>Alternate Drug Names</strong></td>
<td>BKM120</td>
</tr>
<tr>
<td><strong>Eligible Participant</strong></td>
<td>Germline BRCA1/2-mutated platinum-resistant or partially platinum-sensitive recurrent ovarian cancer</td>
</tr>
<tr>
<td><strong>Patients Enrolled</strong></td>
<td>16/32</td>
</tr>
<tr>
<td><strong>Therapy Setting</strong></td>
<td>Recurrence</td>
</tr>
<tr>
<td><strong>Study Design</strong></td>
<td>Open Label, Non-Randomized</td>
</tr>
<tr>
<td><strong>Endpoints</strong></td>
<td>Recommended Phase 2 Dose (RP2D), ORR evaluated per RECIST; PFS; OS</td>
</tr>
<tr>
<td><strong>Biomarkers</strong></td>
<td>BRCA1/2 germline status</td>
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</tbody>
</table>

**Efficacy**

- **RP2D:** 300 mg BID
- **ORR:** 46.9% (6.3% CR, 40.6% PR)
- **PFS:** 5.5 months (4.9-7.3 months)
- **OS:** 15.2 months (10.0-17.3 months)

**Clinically Significant Adverse Events**

- Dose Limiting Toxicities: none
- RP2D: Serious AE: none
  - Grade 3-4 AE: none

**Conclusion**

Promising activity in BRCA MUT cancer

**Reference**

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).
RR: Response Rate—percentage of patients whose cancer shrinks or disappears after treatment.
PR: Partial Response -- Number of partial tumor responses to treatment, divided by the number of patients evaluated.
CR: Complete Response -- Number of complete tumor responses to treatment, divided by the number of patients evaluated.
ORR: Objective Response Rate -- Sum of complete and partial 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.