Objectives This was a single institution, phase II, Simon 2-stage with safety lead-in study of the oral androgen-receptor antagonist, enzalutamide, in patients with recurrent AR+ ovarian cancer with measurable disease and 1–3 prior lines of chemotherapy. The primary objective was to determine the proportion of patients surviving progression free for 6 months (PFS6) and overall response rate by RECIST 1.1 Criteria; with 7/58 responses or PFS of 13 being considered a positive study.

Methods Following consent, archival tissue was screened for AR+ by IHC with ≥5% considered positive. Enrolled patients were treated with enzalutamide 160mg po daily until progression of disease or treatment discontinuation. A cycle was 28 days. Adverse events were graded by CTCAE V 4.0.

Results Between 11/2013–7/2018 160 patients were screened and 59 patients [45 high grade serous(HGS), 14 low grade serous(LGS)] consented to treatment on the study (1 patient was replaced; efficacy cohort=n=58, safety cohort=n=59). There was 1 confirmed and 1 unconfirmed partial response (PR), PFS6 was 22% (90% CI: 15.1–100%) with PFS6 for those with HGS 19.8% (90% CI: 12.7–100%) and for LGS 38.5% (21.7%-100%). Median PFS was 3.5 months. There were no toxicities >grade 3 related to study drug. Related grade 3 toxicities occurred in 6 patients [fatigue (1), rash (2), hypertension (1), anemia (1), and transaminase elevation (1)].

Conclusions The study met its primary endpoint, with 13 patients (22%) remaining progression free at 6 months, however the response rate was low. Enzalutamide was well tolerated and may offer a good treatment option in select patients.