

TYPE OF CANCER: Metastatic Solid Tumors
TYPE OF TRIAL: Phase I
TRIAL SPONSOR: Arqule

PRINCIPAL INVESTIGATOR: Lin Chi-Chen, M.D., Ph.D.
CONTACT PERSON: Sandy Lahr
(702) 822-5174

STUDY SUMMARY

A Phase 1 Dose Escalation Study of ARQ 621 in Adult Patients with Metastatic Solid Tumors

TREATMENT OVERVIEW

- ARQ 621 will be administered intravenously, weekly in a 28 day cycle
- Patient should be seen by the physician at least once a week
- Patients may continue to participate in the study unless they experience unacceptable toxicity or disease progression or withdrawal from study

PRE-TREATMENT ASSESSMENTS

- Informed consent
- Collection of archival tissue/Tumor biopsy, if applicable
- Serum pregnancy test (if applicable)
- Medical history
- 12-lead ECG
- Physical exam
- Vital signs
- Performance status
- Hematology and blood chemistry
- Coagulation panel
- Urinalysis
- Radiographic lesions evaluation

ENTRANCE CRITERIA FOR PARTICIPATION IN TRIAL

INCLUSION CRITERIA

1. Signed written informed consent must be obtained and documented according to International Conference on Harmonisation (ICH)- Good Clinical Practice (GCP), the local regulatory requirements, and permission to use private health information in accordance with the Health Insurance Portability and Accountability Act (HIPPA) prior to study-specific screening procedures
2. A histologically or cytologically confirmed solid tumor that is actively metastatic
3. Have a life expectancy of at least 12 weeks

4. ≥ 18 years of age
5. Measurable disease as defined by Response Evaluation Criteria in Solid Tumors
6. ECOG performance status ≤ 2
7. Male or female patients of child-producing potential must agree to use contraception or avoidance of pregnancy measures during the study and for 30 days after the last ARQ 621 dose
8. Females of childbearing potential must have a negative serum pregnancy test
9. Aspartate transaminase (AST) and alanine transaminase (ALT) $\leq 2.5 \times$ upper limit of normal (ULN) or $\leq 5.0 \times$ ULN with metastatic liver disease.
10. Hemoglobin (Hgb) ≥ 10 g/dl
11. Total bilirubin $\leq 1.5 \times$ ULN
12. Creatinine $\leq 1.5 \times$ ULN
13. Absolute neutrophil count $\geq 1.5 \times 10^9/L$
14. Platelets $\geq 100 \times 10^9/L$

EXCLUSION CRITERIA

1. Anti-cancer chemotherapy, radiotherapy, immunotherapy, or investigational agents within four weeks of first dose
2. Surgery within 4 weeks prior to first dose
3. Known untreated brain metastases (treated brain metastases need to be shown stable for at least 8 weeks)
4. Pregnant or breastfeeding
5. Uncontrolled intercurrent illness including, but not limited to ongoing or active infection, clinically significant non-healing or healing wounds, symptomatic congestive heart failure, unstable angina pectoris, cardiac arrhythmia, significant pulmonary disease (shortness of breath at rest or mild exertion), uncontrolled infection or psychiatric illness/social situations that would limit compliance with study requirements
6. Patients having a history of Thrombotic thrombocytopenic purpura (TTP) or Hemolytic-uremic syndrome (HUS) or HUS spectrum will be excluded from the study