Module 6 Quiz

1. The limitations of using a phenotypic approach to drug discovery include:
    a. More time is needed to interrogate pathways the drug modulates
    b. Deconvoluting targets can be complex
    c. First in class drugs discovered this way may be met with skepticism by the scientific community
    d. All of the above
2. When deconvoluting active compounds from phenotypic screens, which of the following questions is the scientist trying to answer:
    a. What is the small molecule binding to?
    b. What biological pathways is the small molecule modulating?
    c. Are the pathways being modulated relevant to the disease I am studying?
    d. All of the above
3. Any cancer patient can participate in a Phase 1 clinical trial.
    a. True
    b. False
4. Select all that apply. What are the goals and purposes of a phase 3 clinical trial?
    a. Provide an initial assessment of the efficacy of the experimental compound
    b. Screen out ineffective compounds
    c. Determine efficacy of the experimental compound compared to standard therapy
    d. Determine efficacy of the experimental compound compared to placebo
5. Animal models can be used to do which of the following?
    a. Calculate a safe starting dose in humans for Phase 1 clinical trials.
    b. Identify potential side effects in humans and include related protections and monitoring in the in-human clinical trial study design.
    c. Both A and B.

Answer Key

1. All of the above
   When discussing some of the factors detracting from phenotypic drug discovery approaches, Dr. Marugan discussed the experimental issues as well as the potential for skepticism when introducing a compound with a novel mechanism of action.
2. All of the above
3. False
   Patients eligible for Phase 1 clinical trials must have a cancer that is not responsive to standard treatment or there is no remaining standard therapy to try. In addition, these individuals must have adequate organ function. Cancer that is not responsive to standard treatment is called refractory cancer or resistant cancer. It may be resistant from the beginning of treatment or may become resistant during the treatment.
4. Determine efficacy of the experimental compound compared to standard therapy
   The correct answer is c. Responses a and b are goals and purposes of a phase 2 clinical trial, which precedes a phase 3 trial. In addition, phase 2 clinical trials identify compounds to go on to phase 3 trials. In regard to response d, while placebos may be used in some phase 3 studies, they are never used alone if there is a treatment available that works.
5. Both A and B.