Traditional Considerations in ODD

• Oncology drugs are too toxic to be tested in healthy volunteers (HV)

• PK in HV does not translate well to PK in oncology patients (i.e. target-mediated CL)

• Direct to Patient studies offer competitive advantages:
  – evaluation of early signals of efficacy
  – role of biomarkers: i.e. receptor occupancy, translational oncology
  – potential for a single, well-designed and adequately powered study to become registrational
Emerging Considerations

• Too many oncology drug trials and too few available patients
  – Logjam caused by companies hoping to rush ahead of competition
    • 2018 ASCO-SITC symposium:
      o > 4,000 ongoing IO trials;
      o ~1,500 combo IO trials; requiring > 150,000 patients
  – Expand access to trials for traditionally excluded patient populations
    • Minimize risk from extrinsic and intrinsic factors: i.e. DDIs, organ impairment

• Patient-centered drug development:
  – Maximize potential for benefit, minimize safety/tolerability risks
    • Minimize risk from inadequate or too high dosing
    • Informed consent
      – Evidence & reliability of data that a treatment will actually work
  – Minimize assessment burden