Targeted Agent and Profiling Utilization Registry (TAPUR) Study

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Precision Medicine

- Therapies designed to target the molecular alteration that aids cancer development

- A large proportion of cancers may contain at least one plausibly actionable genetic alteration
- The “long tail” means that the conventional clinical trial design approach may not be feasible

Problems

- Patient with advanced cancer; no standard treatment options
- Genomic profile test performed
- Potentially actionable aberration detected
- FDA-approved drug available for aberration

How to get the drug that might be beneficial?
How to learn from the treatment?
Overall Goals of TAPUR

• To learn from the real world practice of prescribing targeted therapies to patients with advanced cancer whose tumor harbors a genomic variant known to be a drug target, or to predict sensitivity to a drug.

• To educate oncologists about implementation of precision medicine in clinical practice.
Who Benefits?

• **Patients** receive targeted agent matched to molecular profile – broader eligibility criteria

• **Physicians** receive interpretation of molecular test results, guidance in treatment recommendations, access to drugs, clinical data on off-label use

• **Industry** receives data on drug use and outcomes to inform R&D plans and life cycle management

• **Oncology Community** receives data on extent and outcomes of off-label drug and test use and real world safety data
Eligibility

- Patients with advanced solid tumors, multiple myeloma or B cell non-Hodgkin lymphoma for which standard treatment options are no longer available and acceptable performance status and organ function
TAPUR Study Primary Objective

• To describe the anti-tumor activity and toxicity of commercially available, targeted anti-cancer drugs prescribed for treatment of patients whose tumors have a genomic variant known to be a drug target, or to predict sensitivity to a drug.
Study Endpoints and Analysis

- **Primary endpoint**: ORR or SD at 16 weeks per relevant response criteria
- **Other endpoints**: PFS, OS, time on treatment, grade 3-5 AEs per CTCAE, SAEs
- **Each tumor type-variant-drug is a “group”**
- **Enroll 10 patients/group. If 1 or fewer responses, stop**
- **If at least 2 responses, enroll additional 18**
- **7 or more responses/28, further study**
- **85% power and an alpha error rate of 10%**
How does TAPUR work?

A patient’s treating physician has results of a genomic profile of the patient’s tumor and determines that a study drug may benefit the patient.

The patient decides to participate in TAPUR and gives informed consent.

The Molecular Tumor Board—a group of experts convened by ASCO—is available for consult regarding the proposed treatment or to provide alternate treatment options.

A participating pharmaceutical company provides the study drug at no cost to the patient.

The patient is followed for standard toxicity and efficacy outcomes and data are collected for analysis.

The study’s Data and Safety Monitoring Board reviews results and determines whether a treatment is promising for a particular cancer and genomic variant.

ASCO publishes study findings in peer reviewed journals to inform clinical practice and future research.
Participating Pharmaceutical Companies

• Seven companies currently committed to participate
  – Providing free drug (ongoing access for responders)
  – Per-case payment for up to total of 1030 TAPUR participants
  – Infrastructure support for ASCO staff & study administration
Supporting Vendors

• Syapse Precision Medicine software
  – Electronic data collection platform & study workflows

• Cardinal Health Specialty Pharmacy
  – Central drug distribution
Key Milestones

• FDA reviewed and determined TAPUR Study IND-exempt (08/31/15)
• Chesapeake Institutional Review Board approval (02/09/16)
• Registered on ClinicalTrials.gov (02/11/16)
  – NCT ID# 02693535 granted (02/20/16)
• TAPUR Study Launch (03/14/16)
Public Website: TAPUR.org

• Visit www.TAPUR.org for more information

Find out more about getting involved with TAPUR.

About the TAPUR Study

The Targeted Agent and Profiling Utilization Registry (TAPUR) Study is a non-randomized clinical trial that aims to describe the performance (both safety and efficacy) of commercially available, targeted anticancer drugs prescribed for treatment of patients with advanced cancer that has a potentially actionable genomic variant. The study provides approved targeted therapies that are contributed to the program by collaborating pharmaceutical companies, catalogues the choice of genomic profiling test by clinical oncologists and aims to learn about the utility of registry data to develop hypotheses for additional clinical trials. To find responses to commonly asked questions, please visit our FAQ page and review our patient brochure.