Targeted Agent and Profiling Utilization Registry (TAPUR™) Study

March 2018

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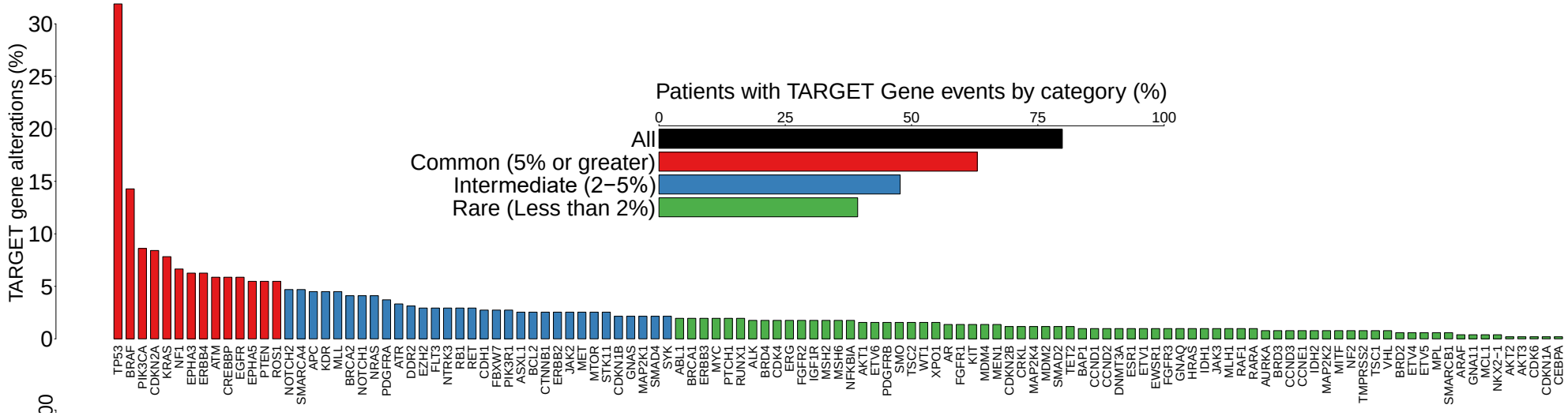
Targeted Agent and Profiling Utilization Registry Study

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**

Van Allen, Wagle et al., *Nature Med* 20, 682–688 (2014)

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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?

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Overall Goals of TAPUR

Learn from real world prescribing practices.

Educate oncologists about how to use genomically targeted drugs.

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Who Benefits?

Participants: Access to targeted study drugs matched to the genomic profiles of their cancers

Physicians: Assistance interpreting genomic results and identifying potential treatment options

Cancer Community: New uses of targeted anticancer drugs for patients who have exhausted standard options

Drug Manufacturers: Insights on new uses of existing drugs

Regulatory Authorities: Learn about side effects and treatment outcomes from use of approved drugs in other cancers

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Eligibility

- Advanced cancer (including solid tumors, multiple myeloma, and B cell non-Hodgkin lymphoma) with a genomic variant that can be targeted with a study drug
- No longer benefiting from standard treatment, or no standard treatment available
- Healthy enough to participate

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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

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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

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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.

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Participating Pharmaceutical Companies

- Seven companies currently committed to participate
  - **Providing free drug** (ongoing access for responders)
  - **Per-case payment for over 1200 TAPUR participants**
  - **Infrastructure support for ASCO staff and study administration**

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Supporting Vendors

- **Syapse Precision Medicine software**
  - Electronic data collection platform and study workflows

CardinalHealth™

- **Cardinal Health Specialty Pharmacy**
  - Central drug distribution

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Key Milestones

- FDA reviewed and determined TAPUR Study IND-exempt *(August 2015)*
- TAPUR Study Launch *(March 2016)*
- Addition of new study therapy; lowered enrollment age to 12 years of age *(May 2017)*
- Opened collaborations with international research groups and molecular testing companies *(June 2017)*
- Over 100 clinical site locations enrolling participants *(December 2017)*
- 1000th participant registered (consented to study) (*February 2018*)
- 750th participant enrolled (on study drug treatment) (*March 2018*)

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FOR PATIENTS FOR RESEARCHERS PARTICIPATING CENTERS STUDY MEMBERS

For more information:
www.TAPUR.org

ClinicalTrials.Gov:
NCT#02693535

About the TA

The Targeted Agent and Profil formance (both safety and efficacy) of commercially avail a potentially actionable genomic variant. The study provides approved targeted therapies that are contributed to the program by collaborating pharmaceutical companies, catalogues