Drug Trials Snapshots
Making Minority Demographic Data More Available and Transparent

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Organizational Structure

Center for Drug Evaluation and Research

Office of the Center Director

Professional Affairs and Stakeholder Engagement

Safe Use Team  Engagement Team  Education Team

Drug Trials Snapshots
PASE’s Mission

To establish relationships with various CDER stakeholders in order to:

- Enhance two-way communication
- Provide a focal point for advocacy
- Establish measures, processes, and outcomes to assess effectiveness of CDER initiatives
Stakeholders

Healthcare professionals/societies

Patients/patient advocates

Health plans, insurers

State/federal regulatory bodies
BiDil®: Where Have We Come in 10 Years?
Inter-racial differences in response to a drug

- Pathophysiological differences
- Genetics
- Physiological differences
- Multifactorial
- Environmental factors
- Unknown

Adapted from Ramamoorthy et al, 2014
True or False?

- There is a regulatory requirement for companies to include patients of different races as participants in clinical trials.
  - False

- There is a regulatory requirement that New Drug Applications include analyses of safety and effectiveness by demographic subgroups.
  - True
True or False?

- Congress mandated that FDA provide a report addressing demographic subgroups in clinical trials.

True

*Drug Trials Snapshots*
The Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA): Section 907

• Response to concerns from groups representing racial minorities and women
• Directed FDA to publish and to provide Congress a report that addresses demographic subgroups

Sex, Race, Age, and Ethnicity
FDA Action Plan

• In August 2014, FDA delivered its *Action Plan to Enhance the Collection and Availability of Subgroup Data*

• The plan includes three overarching priorities for subgroups:
  – Quality of Data
  – Greater Participation
  – *Increased Transparency*
Transparency

- Making demographic subgroup data more available and transparent
- Main action item: *Post demographic information from pivotal trials for newly-approved drugs and biologics*
Important Questions for Subgroups

In New Drug and Biologic applications submitted to FDA:

1. What is the extent of clinical participation by demographic subgroups?
2. What is the extent of safety and effectiveness data by demographic subgroups?
Drug Trials Snapshot Publication

• In November 2014, first snapshots were published
  – 6 indications for 5 NMEs
  – Covered 2-month period in 2014
  – Public Comment Period after publication

• Going forward, FDA publishing snapshots for all approved NMEs and original biologics

• 30 days after drug approval
Drug Trials Snapshot

WHAT IS THE PURPOSE OF DRUG TRIALS SNAPSHOT?

The FDA has developed Drug Trials Snapshots to provide information to the public about who participated in the clinical trials for new FDA approved drugs. Drug Trials Snapshot is part of a pilot project to provide information about the sex, age, race and ethnicity of clinical participants for a small group of recently approved drugs. In addition to information about who participates in the trial, each Snapshot also includes information on how the study was designed, results of the efficacy and safety studies and, if known, differences in efficacy and side effects among sex, race and age (referred to as subgroups).
<table>
<thead>
<tr>
<th>Clinical Trial Demographic</th>
<th>Active Ingredient</th>
<th>Original Approval or Tentative Approval Date</th>
<th>What It's Used For</th>
<th>Drugs@FDA Label</th>
</tr>
</thead>
<tbody>
<tr>
<td>DALVANCE</td>
<td>daibavancin hydrochloride</td>
<td>May 23, 2014</td>
<td>Serious bacterial skin infections known as acute bacterial skin and skin structure infections (ABSSI)</td>
<td>Dalvance drug label</td>
</tr>
<tr>
<td>ENTYVIO</td>
<td>vedolizumab</td>
<td>May 20, 2014</td>
<td>Crohn's Disease</td>
<td>Entyvio drug label</td>
</tr>
<tr>
<td>ENTYVIO</td>
<td>vedolizumab</td>
<td>May 20, 2014</td>
<td>Ulcerative Colitis</td>
<td>Entyvio drug label</td>
</tr>
<tr>
<td>JUBLIA</td>
<td>efinaconazole</td>
<td>June 6, 2014</td>
<td>Fungal infection of the toenails (onychomycosis) due to two common forms of fungi</td>
<td>Jublia drug label</td>
</tr>
<tr>
<td>SIVEXTRO</td>
<td>tedizolid</td>
<td>June 20, 2014</td>
<td>Serious bacterial skin infections known as acute bacterial skin and skin structure infections (ABSSI)</td>
<td>Sivextro drug label</td>
</tr>
<tr>
<td>ZONTIVITY</td>
<td>vorapaxar</td>
<td>May 8, 2014</td>
<td>People who have had a heart attack or reduced blood flow in their legs (peripheral arterial disease)</td>
<td>Zontivity drug label</td>
</tr>
</tbody>
</table>
Sample Snapshot

Drug Trials Snapshot
FARYDAK

HOW TO USE THIS SNAPSHOT:
The information provided in Snapshots highlights who participated in the clinical trials that supported the FDA approval of this drug, and whether there were differences among sex, race and age groups. The "MORE INFO" bar shows more detailed, technical content for each section. The Snapshot is intended as one tool for consumers to use when discussing the risks and benefits of the drugs.

LIMITATIONS OF THIS SNAPSHOT:
Do not rely on Snapshots to make decisions regarding medical care. Always speak to your health provider about the risks and benefits of a drug. Refer to the FARYDAK Prescribing Information for complete information.

FARYDAK® (panobinostat)
(FAYR-ah-dak)
Novartis Pharmaceuticals Corporation
Approval date: February 23, 2015

DRUG TRIALS SNAPSHOT SUMMARY:

What is the drug for?
Multiple myeloma is a form of blood cancer that begins in a type of white blood cell called a plasma cell. Multiple myeloma causes plasma cells to rapidly multiply and crowd out other healthy blood cells from the bone marrow. FARYDAK works by slowing the over-development of plasma cells in patients with multiple myeloma or causing the death of these dangerous cells.

FARYDAK is approved to treat people with multiple myeloma who have received at least two prior standard therapies, including bortezomib, a type of chemotherapy, and a second type of therapy called an immunomodulatory agent. FARYDAK is to be used in combination with bortezomib and dexamethasone, which is also used to kill myeloma plasma cells.

FARYDAK was approved under FDA's accelerated approval program, which provides earlier patient access to a promising new drug while the company continues to conduct clinical trials to confirm that the drug works well.

How is this drug used?
FARYDAK is a capsule that is taken three times a week with chemotherapy.
Snapshots: Racial Demographics

FARYDAK Baseline Demographics (193 Patients)

- 122 Patients (63.2%)
- 63 Patients (32.6%)
- 6 Patients (3.1%)
- 2 Patients (1%)
Snapshots: Racial Demographics

**SAVAYSA Baseline Demographics**

- Caucasian: 7,008 patients (80.9%)
- Black: 278 patients (1.3%)
- Asian: 2,894 patients (13.8%)
- Other: 845 patients (4.0%)
Snapshots: Gender Demographics

SAVAYSA Baseline Demographics

- Male: 13,020 (62%)
- Female: 8,006 (38%)
Differences in Effectiveness Among Subgroups

- Were there any differences in how well the drug worked in clinical trials among sex, race, and age?
  - SAVAYSA was similarly effective in men and women.
  - The number of non-white patients was limited; therefore, differences in response to NATPAR between white and non-white patients could not be determined.
  - FARYDAK was similarly effective in patients above and below age 65.
Differences in Safety Among Subgroups

- Were there any differences in side effects among sex, race, and age?
  - The overall incidence of bleeding was greater in women, primarily due to an increase in vaginal bleeding.
  - The number of patients in the non-White subgroup was limited. Therefore, differences among races could not be detected.
  - The risk of side effects was similar in patients below and above age 65 years.
What do Snapshots Provide?

• Transparency on:
  – Who is participating in clinical trials?
  – Are there differences in safety and efficacy among subgroups?

• Tool for physicians and patients, not meant to replace Prescribing Information

• Consumer-friendly information about the drug and the trials that supported FDA approval
Snapshots Generate Questions

• Why does it matter and when does it matter?
• Is subgroup analysis relevant for every trial for every drug?
• What numbers are required for proper statistical analyses of subgroups?
• How do we recruit more minority subgroups?
Looking Forward

• Collaboration with internal and external colleagues
• Scientific Conference
• Feedback from stakeholders
Barbra Streisand and Snapshots

The Food and Drug Administration’s new online “Drug Trial Snapshots” reveal the persistent gender and racial imbalances in pharmaceutical trials. I hope these revelations will spark changes in how products and devices are approved.

“The battle for women’s hearts—and lives”. Washington Post, April 10, 2015
Questions?
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www.fda.gov/drugtrialssnapshot

THANK YOU
Naomi Lowy, M.D.