



# Drug Trials Snapshots and Transparency: Opportunities and Challenges



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Clinical Trials: Assessing Safety and Efficacy for a Diverse Population  
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# FDA Action Plan

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



# FDA Action Plan

- Main action item: *Post demographic information from pivotal trials for newly-approved drugs and biologics*

# Drug Trials Snapshots

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**Drug Approvals and Databases**

- Approved Drug Products with Therapeutic Equivalence Evaluations (Orange Book)
- Bioresearch Monitoring Information System (BMIS)
- Clinical Investigator Inspection List (CLIL)
- Dissolution Methods Database
- Drug Establishments Current Registration Site
- ▶ **Drug Trials Snapshots**
- Drugs@FDA Database
- FDA Adverse Event Reporting System (FAERS)
- National Drug Code Directory
- Postmarket Requirements and Commitments
- Approved Drugs ▼

**Resources for You**

- Inside Clinical Trials: Testing Medical Products in People
- The FDA's Drug Review

## Drug Trials Snapshots

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### WHAT IS THE PURPOSE OF DRUG TRIALS SNAPSHOTS?

Drug Trials Snapshots provide consumers with information about who participated in clinical trials that supported the FDA approval of new drugs. The information provided in these Snapshots also highlights whether there were any differences in the benefits and side effects among sex, race and age groups. Drug Trials Snapshots is part of an overall FDA effort to make demographic data more available and transparent.

### HOW TO USE SNAPSHOTS:

Each Snapshot includes contains information about the drug in a question and answer format. At the end of each section of the Snapshot, there is a shaded bar with the words "MORE INFO". Click the "MORE INFO" bar for more technical and detailed content. At the bottom of each Snapshot, there is a link to the drug's Package Insert as well as the medical review.

### LIMITATIONS OF SNAPSHOTS:

The Snapshot is intended as one tool for consumers to use when discussing a drug's risks and benefits with their physician. Do not rely on Snapshots alone to make decisions regarding medical care. Do not use Snapshots to substitute for advice from your health care professional. Conclusions regarding how effective and safe a drug is among different sex, race, and age groups cannot always be made, often because the numbers of patients in some groups are too limited to allow for meaningful comparisons to other groups and to the overall results.

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# Drug Trials Snapshots

- Summary of information
  - *What is the drug for?*
  - *What are the benefits of the drug?*
  - *What are the possible side effects?*
  - *How were the clinical trials designed?*
- Demographic Information

# Key Snapshot Questions: Subgroups

- Who were in the clinical trials by **sex, race, and age** subgroups?
- Were there differences in efficacy and safety among **sex, race, and age** subgroups?

# Snapshots Brief History

- 2014: Pilot Program
- January 1, 2015: Snapshot written for every New Molecular Entity (NME) and Original Biologic approved
- Permanent program
- Goal to publish 30 days after approval
- Does not apply to previously approved drugs

# Who are Snapshots Written For?

- Consumers
- Physicians, Statisticians, anyone who is interested in the data and analyses
  - (MORE INFO)

# Snapshots are not a drug label

## Snapshots

- Intended for public
- Consumer-friendly language
- Focus on subgroup data and analysis
- Links to FDA reviews
- 30 days after drug approval

## Prescribing Information

- Intended for healthcare professionals
- Technical language
- Comprehensive resource for drug information
- Not linked to FDA reviews
- Published with drug approval



# 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<sup>®</sup> (panobinostat)

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

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### 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.

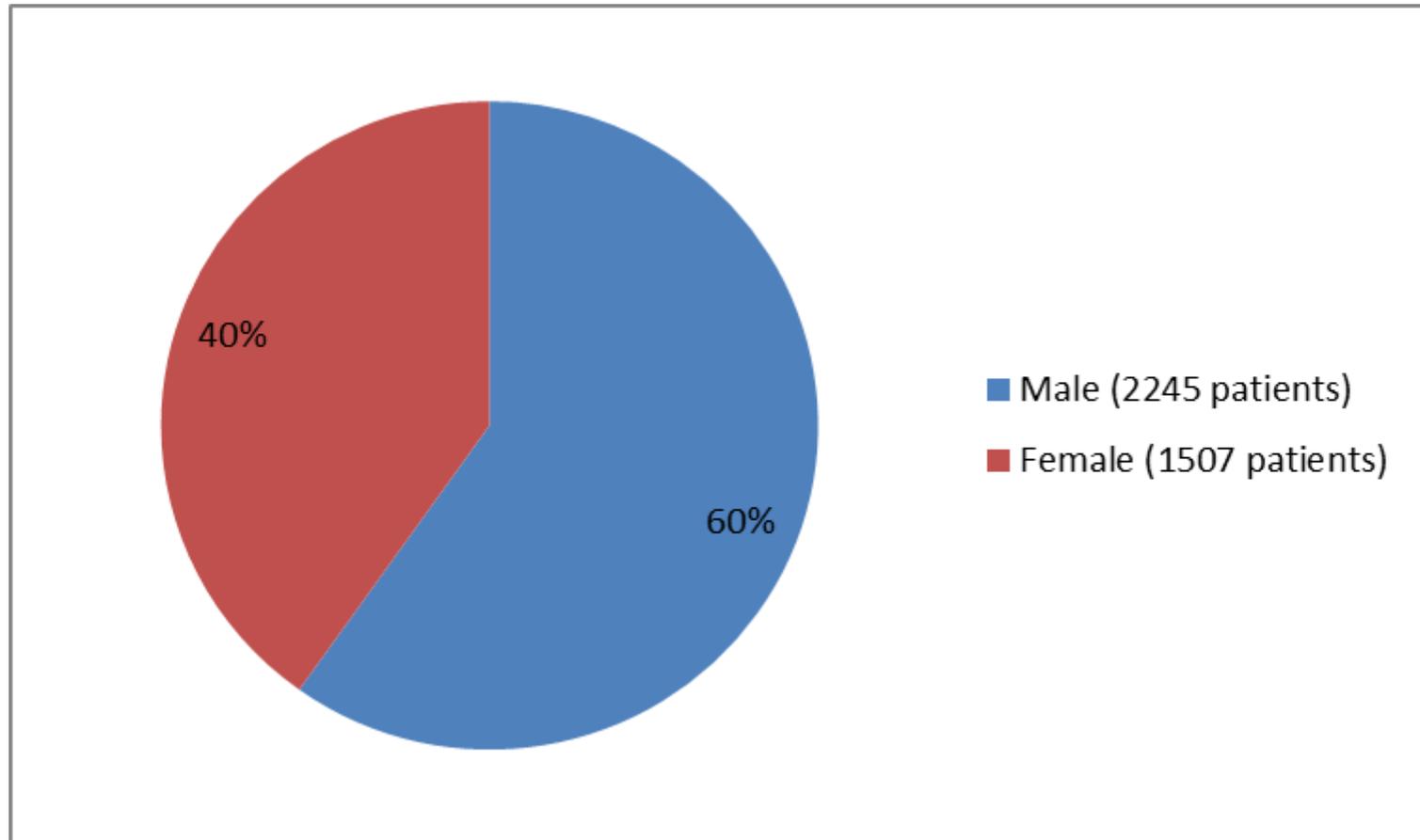
FARYDAK is approved to treat people with multiple myeloma who have received at least two prior standard therapies, including bortezomib, a type of chemotherapy, and a second type of therapy called an immunomodulatory agent. FARYDAK is to be used in combination with bortezomib and dexamethasone, which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

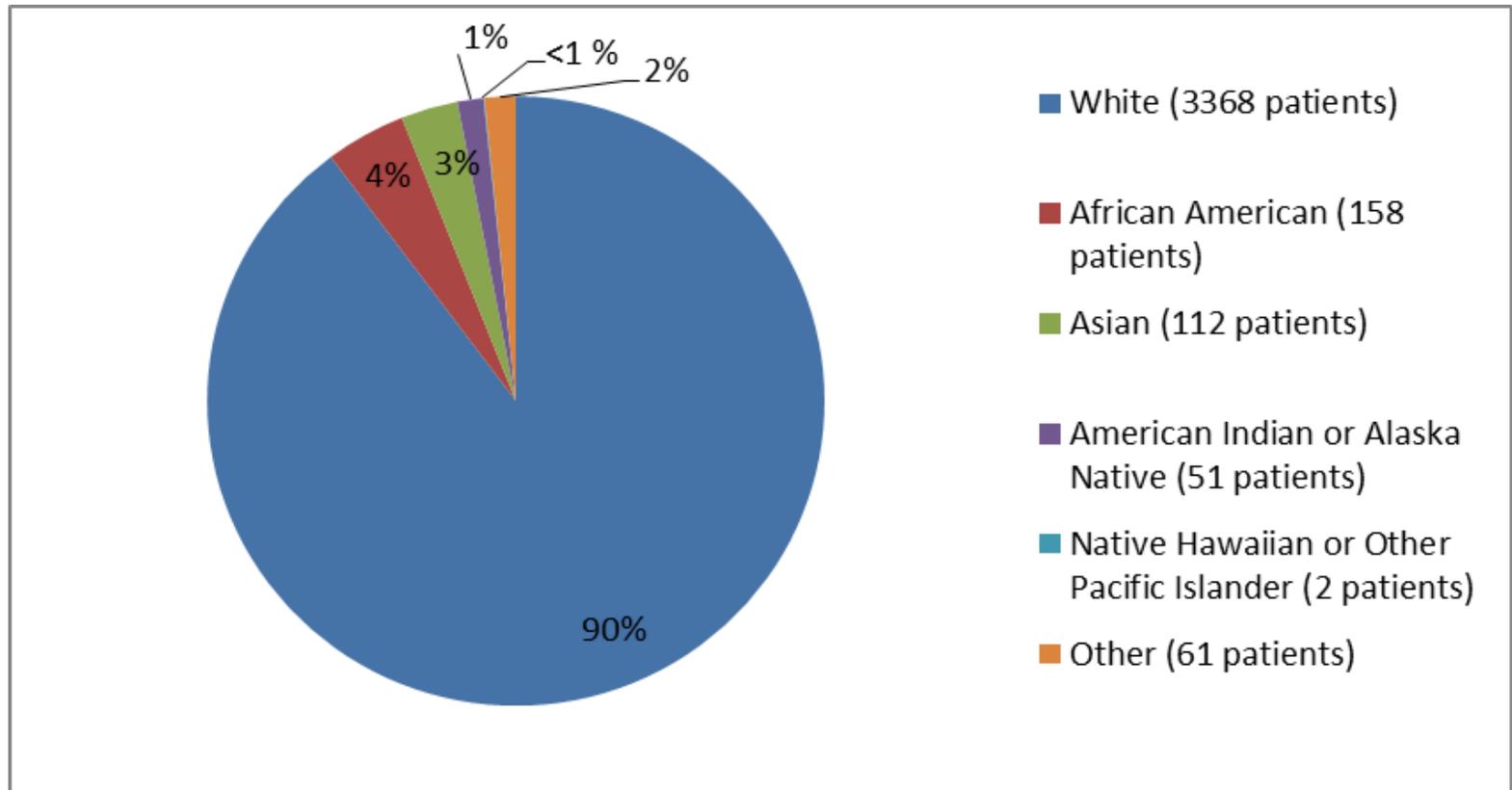
#### How is this drug used?

FARYDAK is a capsule that is taken three times a week with chemotherapy.

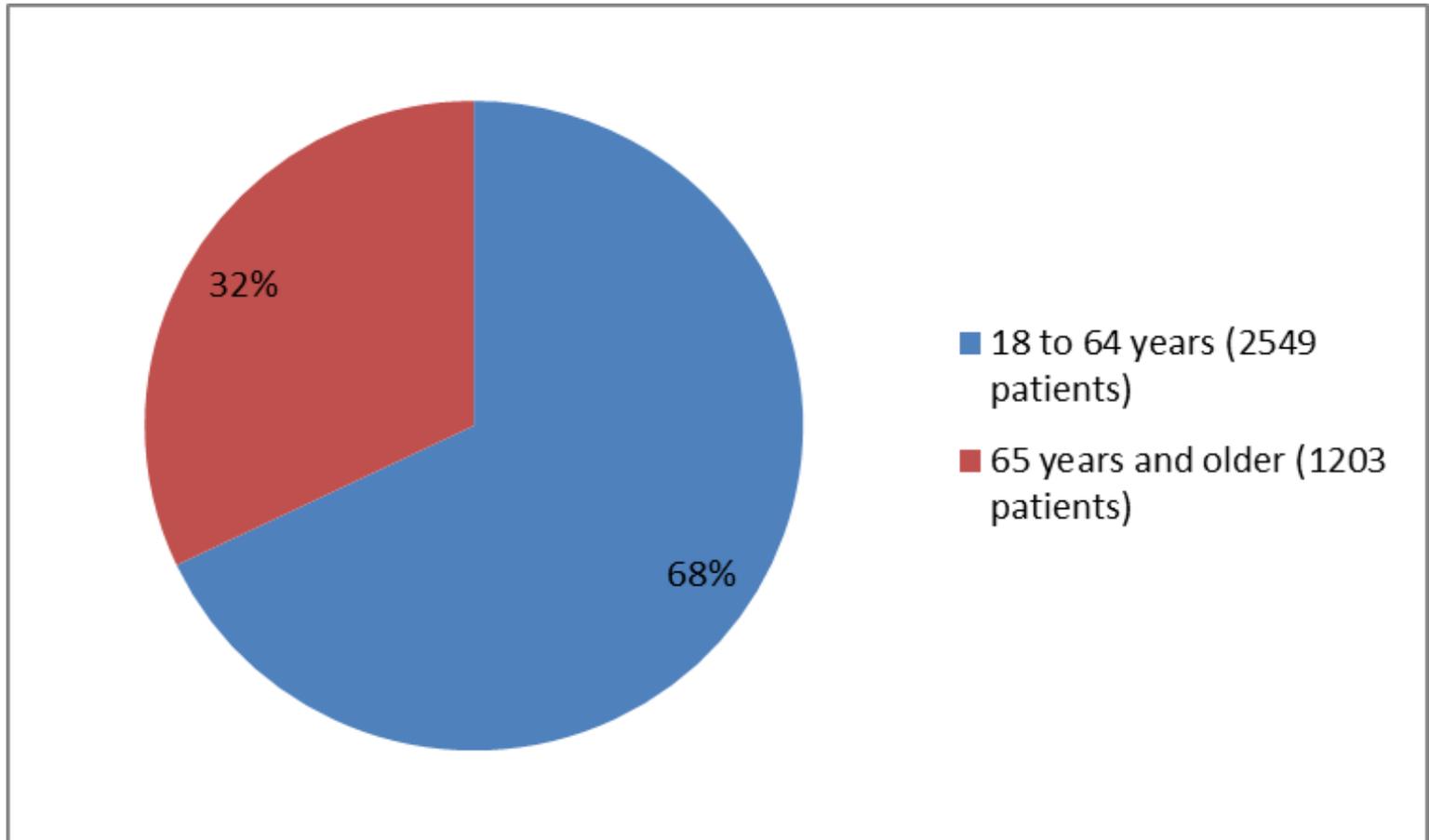
# Sex



# Race



# Age



# Efficacy and Safety Among Subgroups

## *Benefits:*

- The majority of patients in the trials were white. Differences in response to REPATHA among races could not be determined.*
- REXULTI worked similarly in all races studied.*

## *Side Effects:*

- The majority of patients in the trial were white. Differences in bleeding among races could not be determined.*

# Recent Activity

- Published over 40 Snapshots
- Over 53,000 people have visited the site since its launch
- Alerts of new Snapshots available through GovDelivery and Twitter

# Platform for Important Questions

- Are there enough data to make conclusions about efficacy and safety for all subgroups?
- How many patients per subgroup are needed?
- When is generalizability ok?
- When differences among subgroups are seen, when are differences clinically meaningful?

# Looking Forward

- Continuing discussion on variability in response to drugs among subgroups
- Deeper understanding of when subgroup differences are plausible
- Best practices for reporting subgroup differences to the public
- Commitment to continued transparency



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**THANK YOU**

**Naomi Lowy, M.D.**