A Review of FDA PRO Labeling (2011-2015)

Ari Gnanasakthy
RTI Health Solutions

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Review of PRO labeling (2006-2010)

24.1% of new drug approved between 2006 and 2010 had PRO labeling

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Patient-Reported Outcomes

A Review of Patient-Reported Outcome Labels in the United States: 2006 to 2010

Ari Gnanasakthy, MSc[1,*], Margaret Mordin, MS[2], Marci Clark, PharmD[2], Carla DeMuro, MS[2], Sheri Fehnel, PhD[2], Catherine Copley-Merriman, MS[2]

[1]Novartis Pharmaceuticals Corporation, East Hannover, NJ, USA; [2]RTI Health Solutions, Durham, NC, USA

Percent of NMEs with PRO labeling (2011-2015)

Total number of new drugs approved = 182

Total number of new drugs with PRO labeling = 30

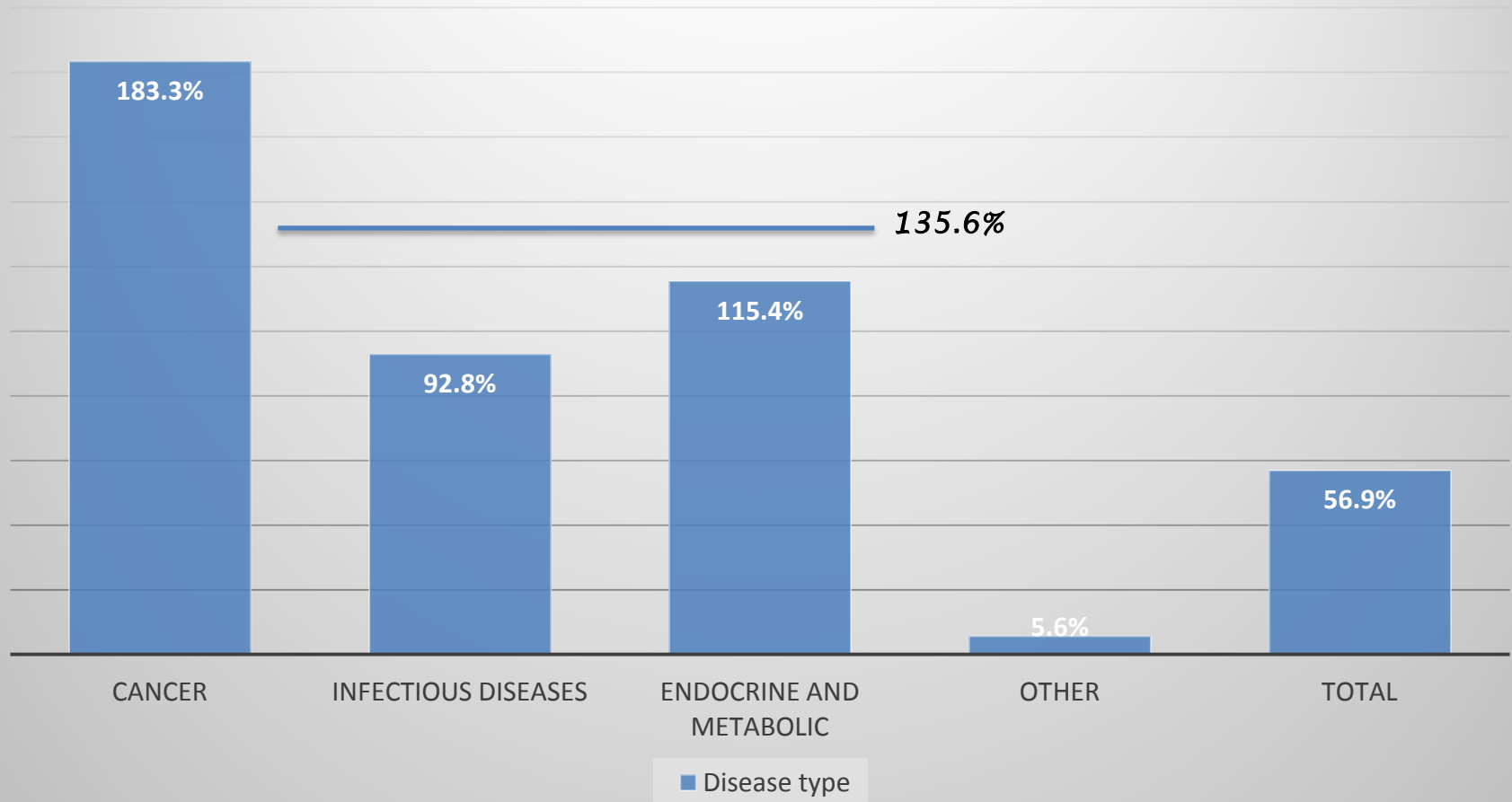**Percent of new drugs with PRO labeling (2011-2015) = 16.5%**

n = 116

Percent of new drugs with PRO labeling (2006-2010) = 24.1%

n = 182

There was noticeable increase in the number of NME approvals for three disease categories

**Change (%) in NME approvals
FDA 2006-2010 vs 2011-2015**

- CANCER — 183.3%
- INFECTIOUS DISEASES — 92.8%
- ENDOCRINE AND METABOLIC — 115.4%
- OTHER — 5.6%
- TOTAL — 56.9%
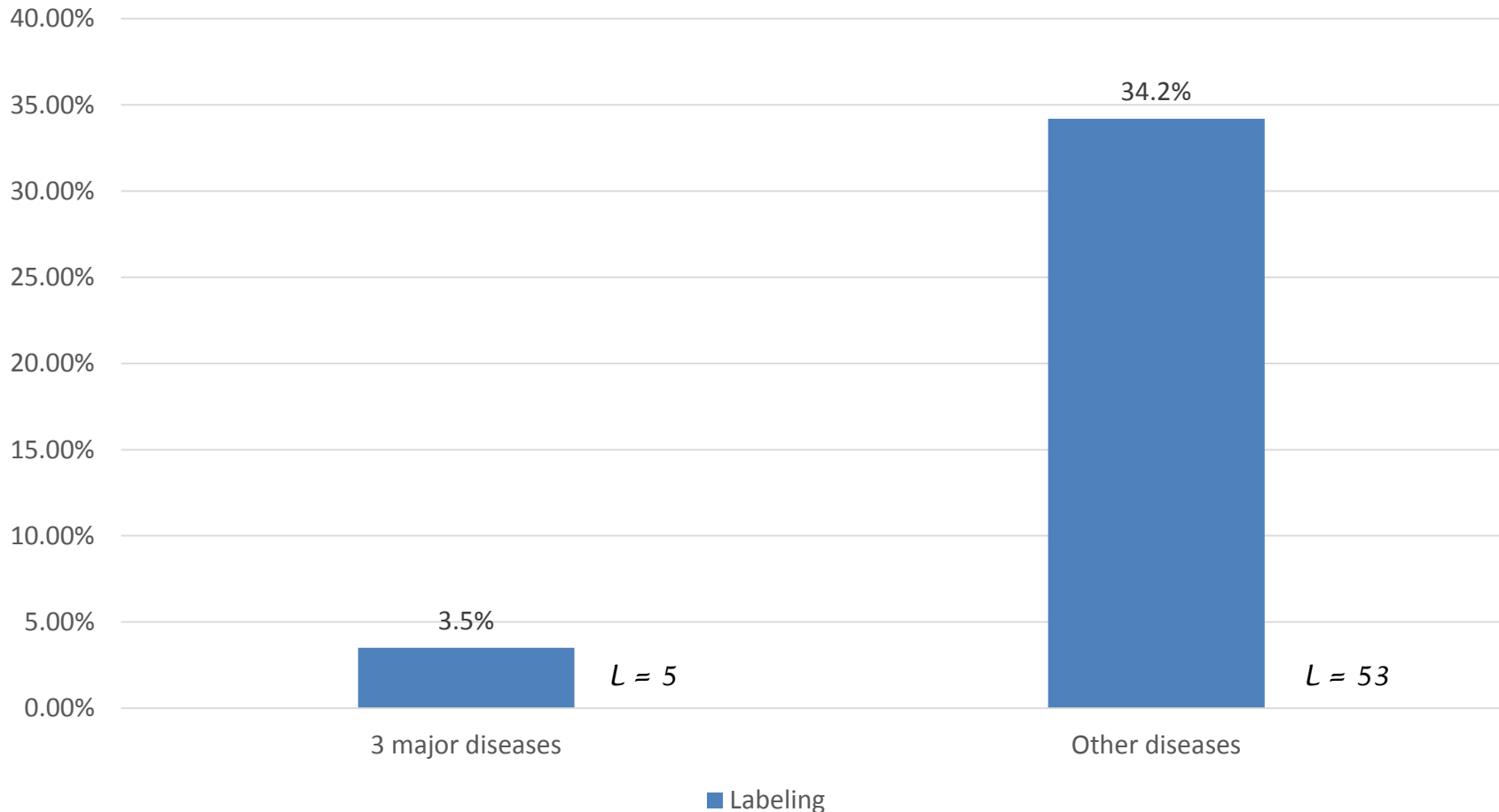
135.6%

■ Disease type

Number of approvals: 2006-2010 = 116; 2011-2015=182

Infectious diseases – infection and parasitic diseases; Endocrine and metabolic - Endocrine, nutritional and metabolic diseases

Only a few labeling in the 3 major diseases in the last 10 years (2006-2015)

PRO Labeling (FDA NMEs 2006-2015)
N=298; L=58

Number of approvals: 2006-2010 = 116; 2011-2015=182

3 major diseases: Neoplasms. infection and parasitic diseases & Endocrine, nutritional and metabolic diseases
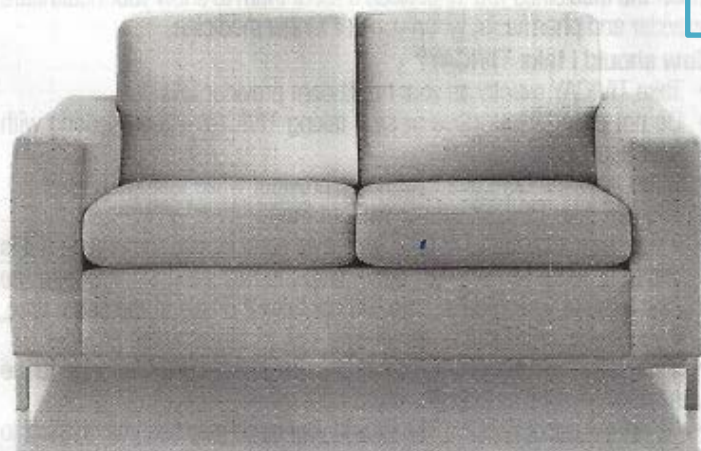
Let's be
HONEST
about HIV
treatment.

Our medication will not make you **younger**, **sexier**, or **smarter**.

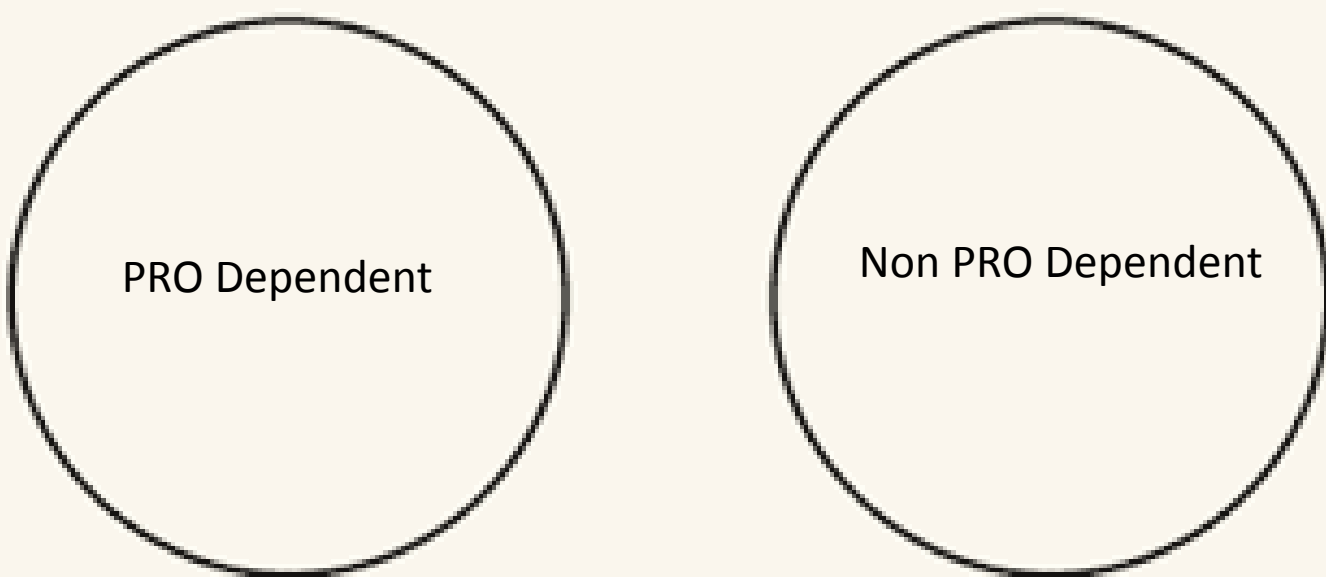It will not improve your jump shot or help make your dance moves "dancier."

However...

Taken in combination with other HIV medications, TIVICAY can lower your viral load and increase your CD4 count.

Tivicay®
(dolutegravir)
50 mg tablets
true to you

Two categories of diseases

PRO Dependent

Non PRO Dependent

Traditionally depends on PROs to demonstrate treatment benefit
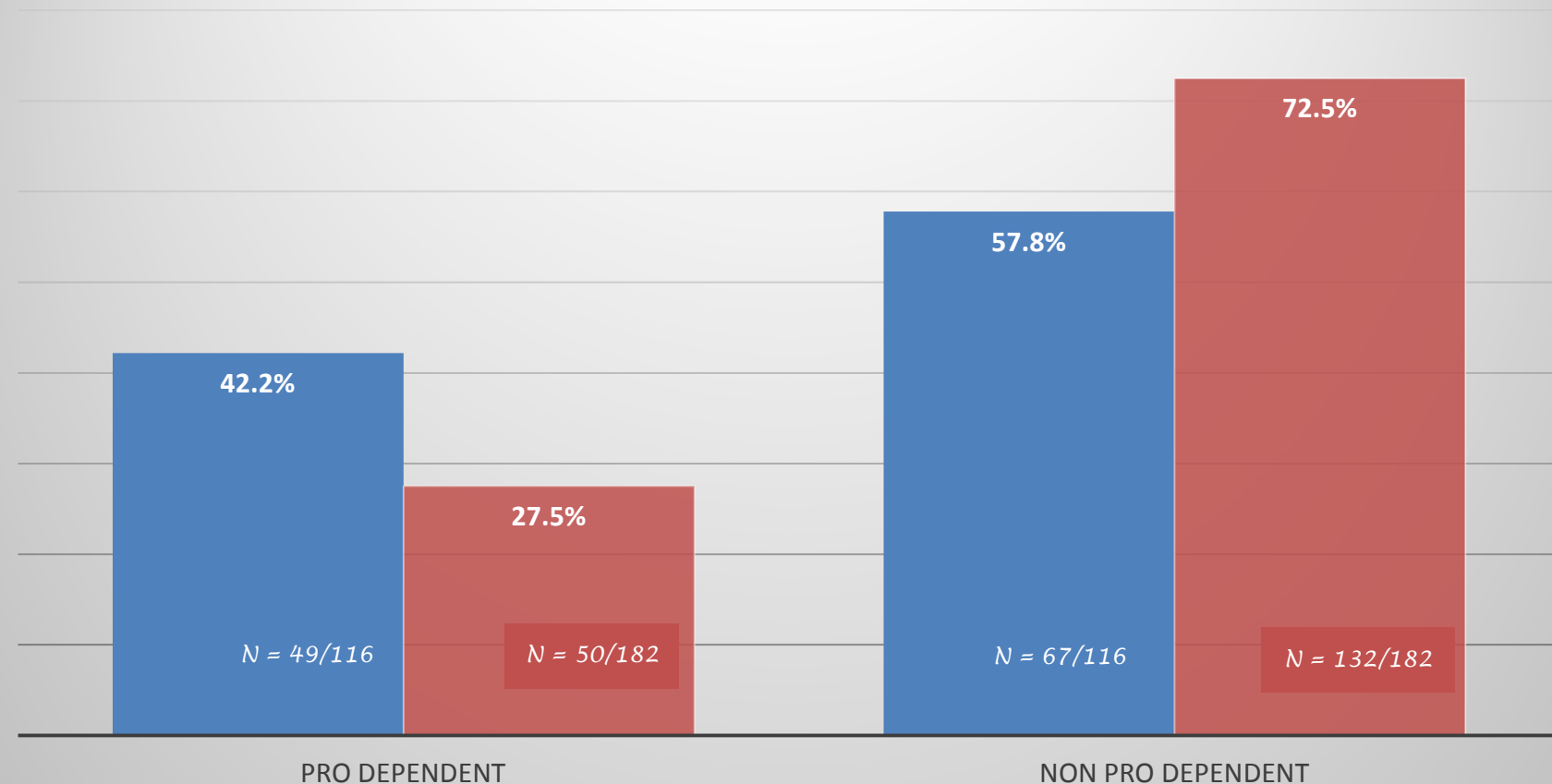
Non PRO-Dependent diseases

'Non PRO Diseases'	Examples
Neoplasms	Cancers (breast, ovarian, prostate etc.)
Infection and parasitic diseases	Tuberculosis of the lungs, HIV disease, Hepatitis C
Endocrine, nutritional and metabolic diseases	Diabetes, Hypophosphatasia, Familial hypercholesterolemia
Diseases of the circulatory system	Chronic heart failure, myocardial infarction, pulmonary heart disease
Pregnancy, childbirth and the puerperium	Spontaneous abortion, obstetric embolism, eclampsia in labor
Symptoms, signs and abnormal clinical and laboratory findings	Elevated blood glucose level, enlarged lymph nodes, functional quadriplegia
Injury, poisoning and certain other conditions of external causes	Injury to the head, frostbite, poisoning by overdose of substance
External causes of morbidity	Transport accidents, intentional self-harm, military operations
Factors influencing health status and contact with health services	Kidney transplant, smoking cessation, preventive pregnancy
Unclassified	Imaging agents

PRO-Dependent diseases

'PRO Diseases'	Examples
Blood and blood forming organs	Myelofibrosis, Iron deficiency anemia, hereditary angioedema
Mental, behavioral and neurodevelopmental disorders	Schizophrenia, anxiety disorders, major depressive disorder
Diseases of the nervous system	Insomnia, epilepsy, multiple sclerosis
Diseases of the eye and adnexa	Glaucoma, age-related macular degeneration, conjunctivitis
Diseases of the ear and mastoid process	Otosclerosis, hearing loss, tinnitus
Diseases of the respiratory system	Asthma, Chronic obstructive pulmonary disease, Idiopathic pulmonary fibrosis
Diseases of the digestive system	Irritable bowel syndrome, constipation, Ulcerative (chronic) pancolitis
Diseases of the skin and subcutaneous system	Psoriasis, urticaria, atopic dermatitis
Diseases of the musculoskeletal system	Osteoarthritis, gout, lupus
Diseases of the genitourinary system	Erectile dysfunction, overactive bladder, dyspareunia

There was a noticeable decrease in the percent of approvals relating to 'PRO Dependent Diseases'

NME Approvals
(FDA 2006 – 2015)

PRO DEPENDENT
- 42.2% — N = 49/116 (2006-2010)
- 27.5% — N = 50/182 (2011-2015)

NON PRO DEPENDENT
- 57.8% — N = 67/116 (2006-2010)
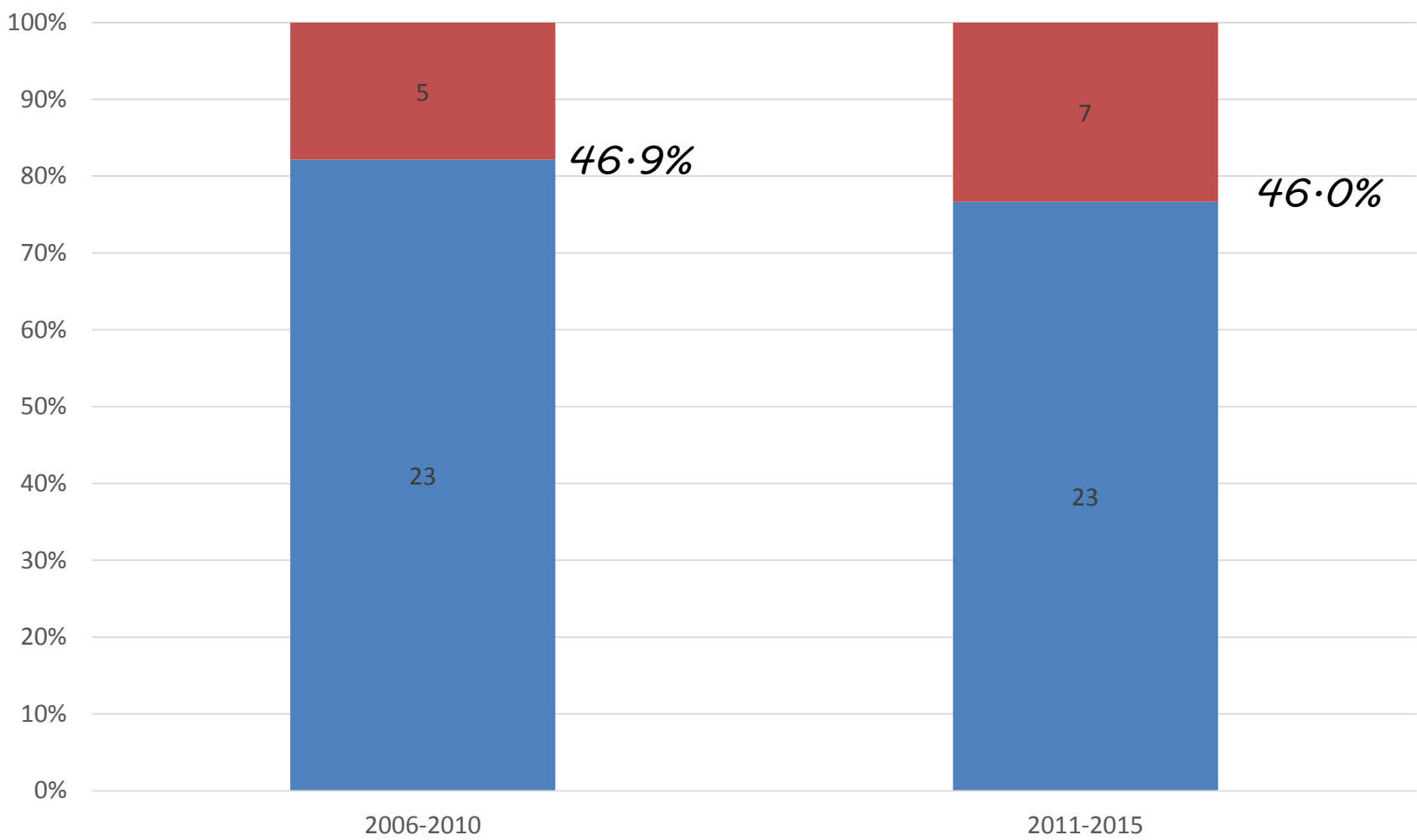- 72.5% — N = 132/182 (2011-2015)

Approvals in 2006-2010 = 116
Approvals in 2011-2015 = 182

■ 2006-2010   ■ 2011-2015

PRO labeling (%) was comparable for the periods 2006-2010 and 2011-2015 (FDA NMEs)

Approvals in 2006-2010 = 116
Approvals in 2011-2015 = 182

■ PRO Related ■ Non PRO Related

Summary

- ➤ Overall, PRO labeling declined from 24.1% to 16.5% between 2006-1010 and 2011-2015

- ➤ Number of new drugs approved that traditionally use PRO related endpoints to assess treatment benefit was about the same between 2006-2010 and 2011-2015
  - ▪ 2006-2010 = 49
  - ▪ 2011-2015 = 50

- ➤ For approvals that traditionally use PRO related endpoints to assess treatment benefit, labeling based on PROs was comparable for the periods 2006-2010 and 2011-2015
  - ▪ 2006-2010 = 46.9%
  - ▪ 2011-2015 = 46.0%