

Introduction



Benoit Arnould,
PhD, Managing Director,
Mapi Values France

Acknowledgments to
Catherine Acquadro, Mapi Research Trust,
Aude Roborel de Climens, Mapi Values

1

- ▮ *How did the industry adapt to the new guidances including PRO?*
- ▮ *Was it successful?*
- ▮ *Where are we on the learning curve?*
- ▮ *What still has to be done?*

2

PRO recommendations in FDA and EMA guidances (1)

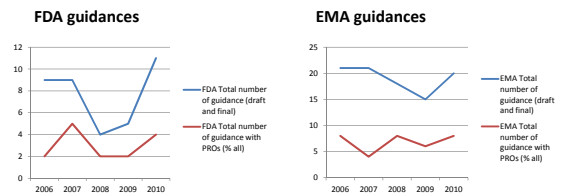
- ▮ Between January 1st 2006 and November 16th 2010, the inclusion of **PRO** endpoints in clinical trials was recommended in:
 - 34 of the 95 **EMA** guidance documents (36%)
 - 15 of the 38 **FDA** guidance documents (40%)
- ▮ The inclusion of **HRQL** endpoints was recommended in:
 - 22 of the 34 **EMA** guidances (65%), as primary or secondary endpoint
 - 3 of the 15 **FDA** guidances (20%), only as secondary endpoint

Marquis P. et al. *The Role of Health-Related Quality of Life Data in the Drug Approval Processes in the US and Europe: A Review of Guidance Documents and Authorizations of Medicinal Products from 2006 to 2010.* *Pharmaceutical Medicine* 2011

3

PRO recommendations in FDA and EMA guidances (2)

Evolution of the number of FDA and EMA guidances with PROs issued between January 1st 2006 and November 16th 2010



Marquis et al., 2011

4

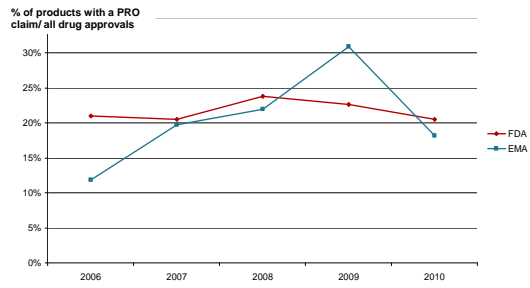
Product labels with PRO claims (1) mapi values

- † 93 of 432 products approved by the **FDA** between 2006 and 2010 with label claims included **PRO** endpoints (22%)
 - 8 products (9% of all products with a PRO claim) with **HRQL** in label
 - 79 products (85% of all products with a PRO claim) with **signs and symptoms** in label
- † 54 of 248 products approved by the **EMA** between 2006 and 2010 with label claims included **PRO** endpoints (22%)
 - 16 products (30% of all products with a PRO claim) with **HRQL** in label
 - 48 products (89% of all products with a PRO claim) with **signs and symptoms** in label

Marquis et al., 2011 5

Product labels with PRO claims (2) mapi values

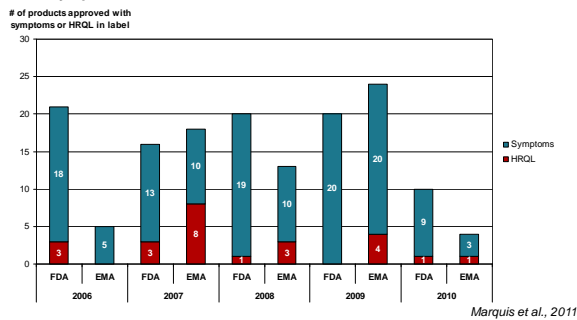
Evolution of the percentage of products with label claims including PRO endpoints among all products approved by the FDA and the EMA between January 1st 2006 and November 16th 2010



Marquis et al., 2011 6

Product labels with PRO claims (3) mapi values

Number of products approved by the FDA and the EMA between January 1st 2006 and November 16th 2010 with HRQL or signs and symptoms in label



Marquis et al., 2011

Key findings mapi values

- † Observable facts confirm the importance given by the FDA and EMA to the patients' perspective in clinical research since 2006
 - In guidances as well as in labels
- † Both agencies value patient-reported symptom data
 - HRQL endpoints still playing a minor role in product claims
 - EMA's receptivity to HRQL endpoints is greater than FDA's
- † The dynamic is stable, unlikely to be a transitional effect