

Added value of Patient-Reported Outcomes: An Industry Perspective

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Recognize reflux disease

- Underdiagnosed

Beyond the reflux disease landscape

- Reflux esophagitis
- Symptomatic reflux disease
- Sleep disturbances
- Acid asthma
- Laryngitis
- Non cardiac chest pain

Symptoms last 30 days in heartburn sufferers

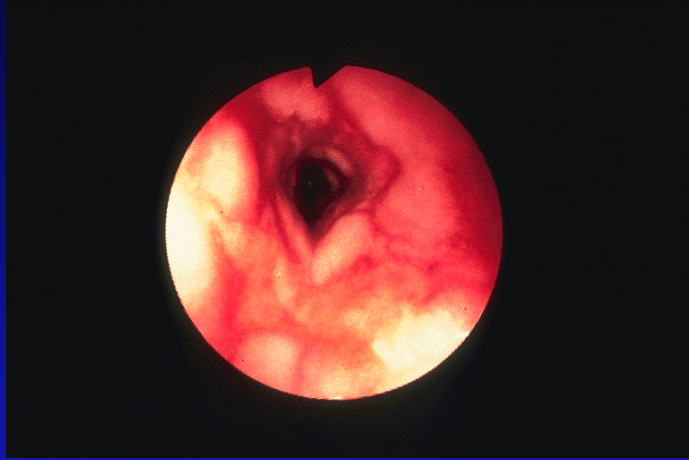
- 55% burning sensation in the chest
- 39% throat clearing
- 36% chronic sinus condition
- 20% feeling having a heart attack
- 18% hoarse voice
- 16% chronic coughing
- 13% asthma

Understanding heartburn

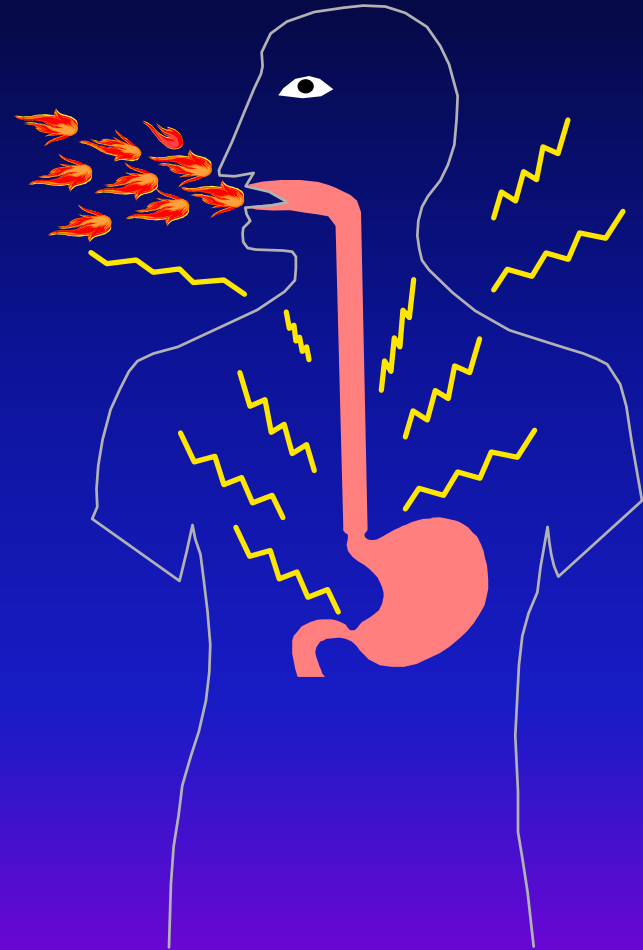
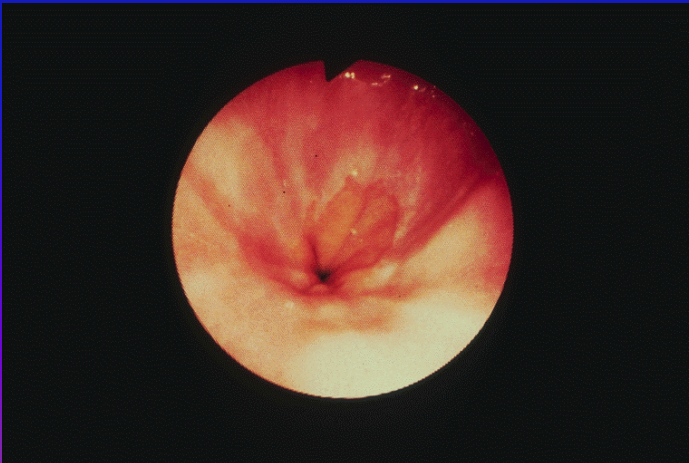
- 65% experience day-and nighttime heartburn
- Nighttime heartburn is the most prominent
- Nighttime heartburn significantly impacts on sleep
- 40% detect an impact on the ability to work
- Most sufferers have consulted a doctor

Heartburn from two perspectives

Inflamed oesophagus



Normal oesophagus



Recognize reflux disease

- Underdiagnosed
- Not recognized as a true medical condition
- Trivialized

Recognize reflux disease

- Underdiagnosed
- Not recognized as a true medical condition
- Trivialized
- Not well treated

Symptoms of GERD reported during four weeks of treatment with prescription heartburn medication (n=11 604)

Symptoms	n	%
Daily heartburn	7119	61.3
Night-time heartburn	6762	58.3
Acid reflux	7012	60.4
Difficulty swallowing	2263	19.5
Abdominal pain	3569	30.8

Recognize reflux disease

- Underdiagnosed
- Improve diagnosis

Why a diagnostic symptom based tool?

A diagnostic questionnaire such as RDQ can:

- facilitate a reflux diagnosis in general practice
- improve care by helping physicians identify reflux disease sufferers
- increase the likelihood of patients receiving the right treatment

Recognize reflux disease

- Underdiagnosed
- Not recognized as a true medical condition
- Trivialized
- Not well treated
- Improve diagnosis
- Raise awareness of burden to patients and society
- Counteract trivialization

Patient quotes:

"I feel I am "crazy". I can't have so much disability when all the tests carried out are almost normal."

"Unable to carry out usual household responsibilities."

"Needs help as symptoms are often started by various positions, i.e. bending over."

"Missing time from work, often requires help from co-workers to carry out responsibilities."

"Severe anxiety when invited to dinner due to worry of not being able to tolerate the food."

"I feel the disease is ruining my life."

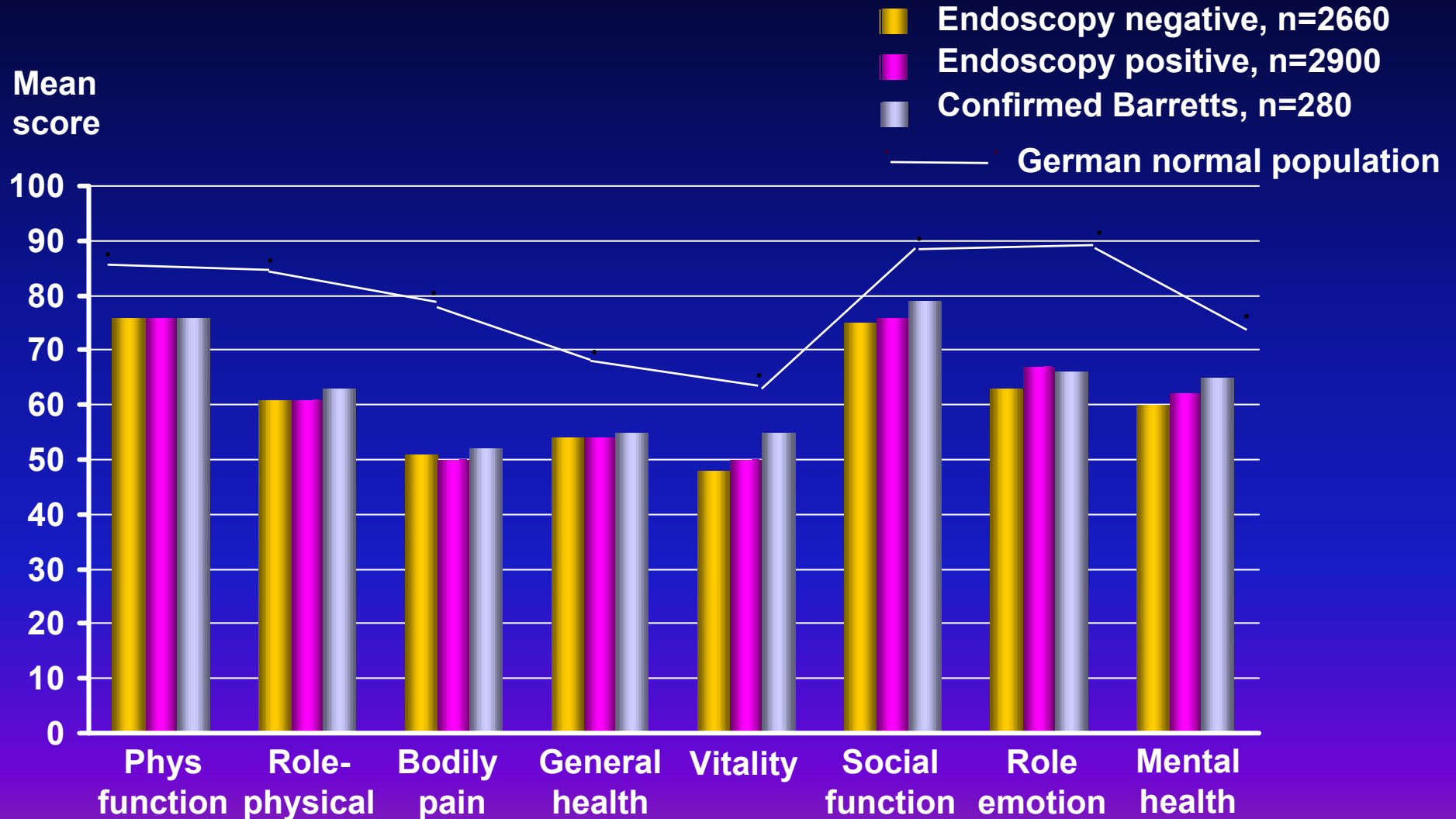
"I'm unable to go to dinner with others."

"I have trouble getting to sleep."

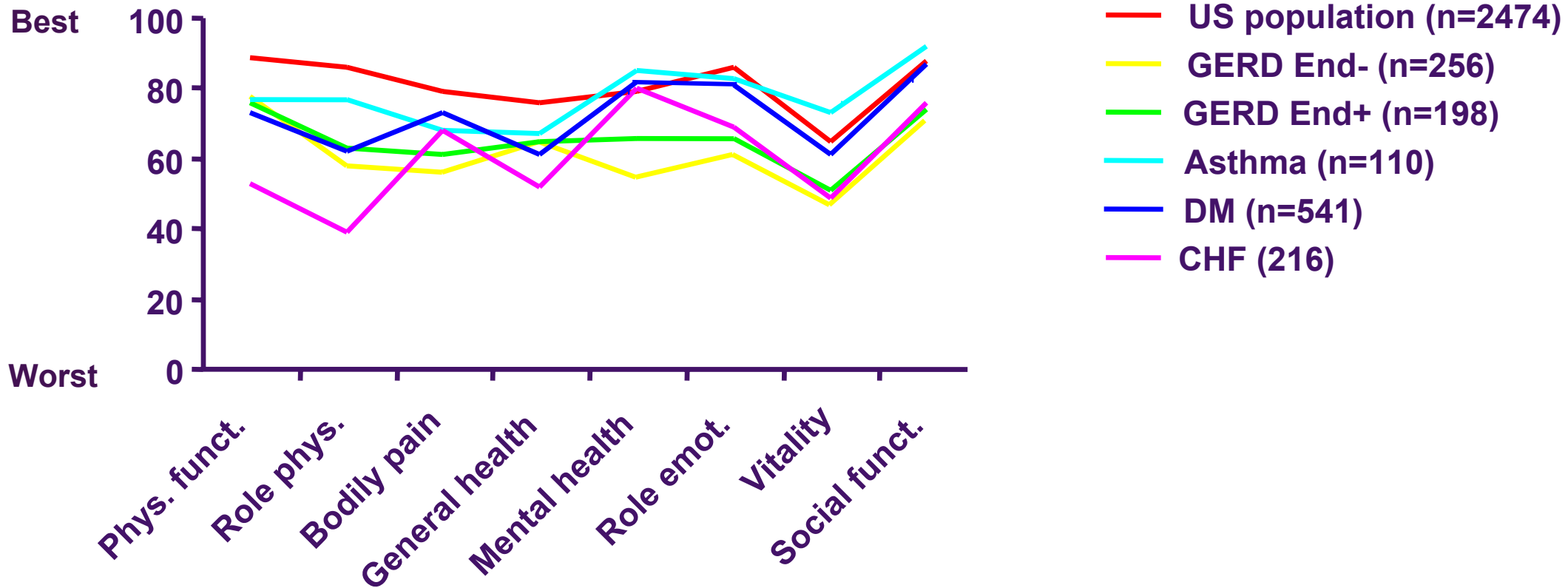
"I feel my family's life is disrupted."

A comparison of HRQL dimensions in patients with reflux disease before treatment

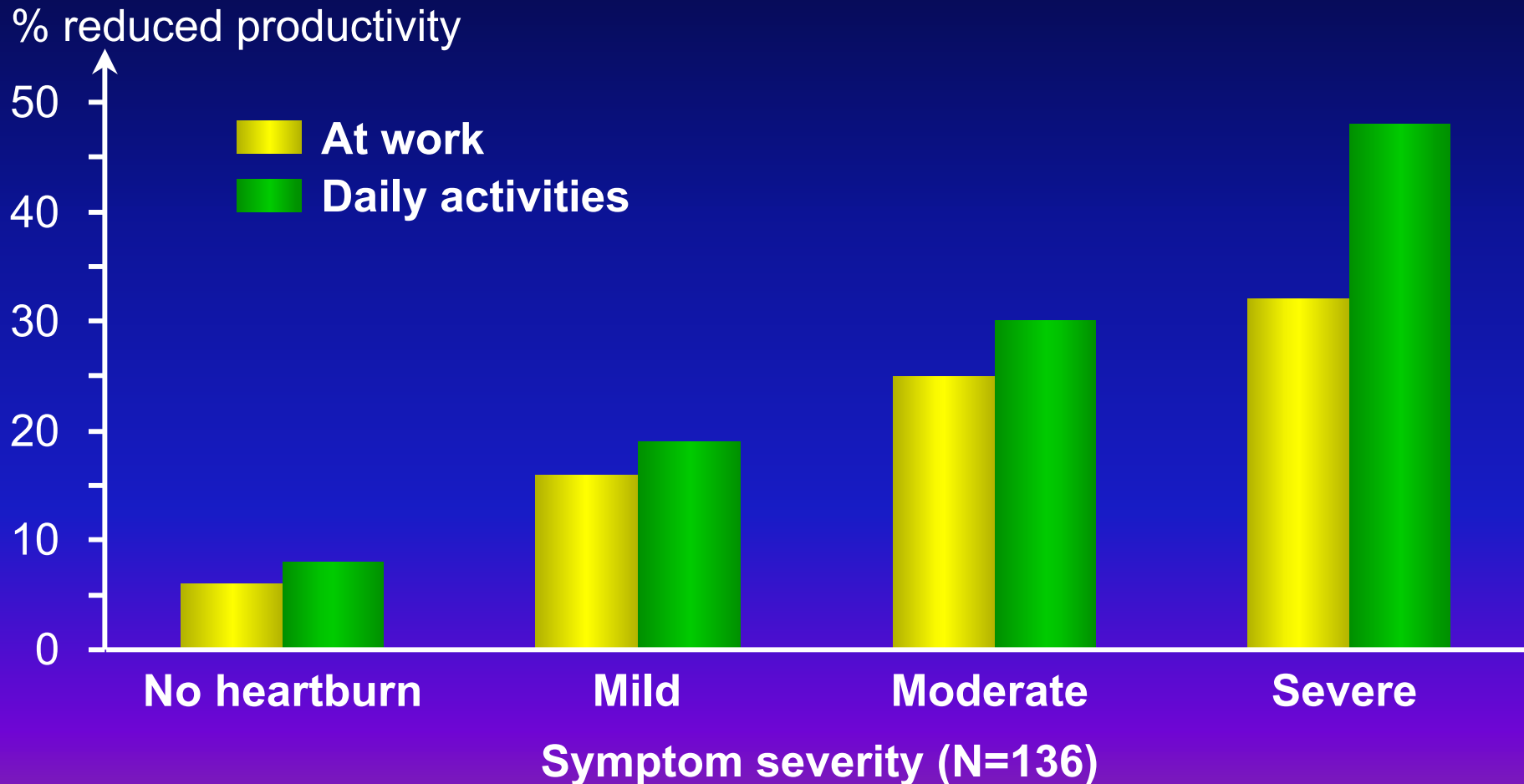
Mean HRQL scores (SF-36)



Mean SF-36 scores in GERD patients compared to US norms and to other chronic conditions



GERD symptoms incur a substantial impairment in productivity important to health officials, employers and patients

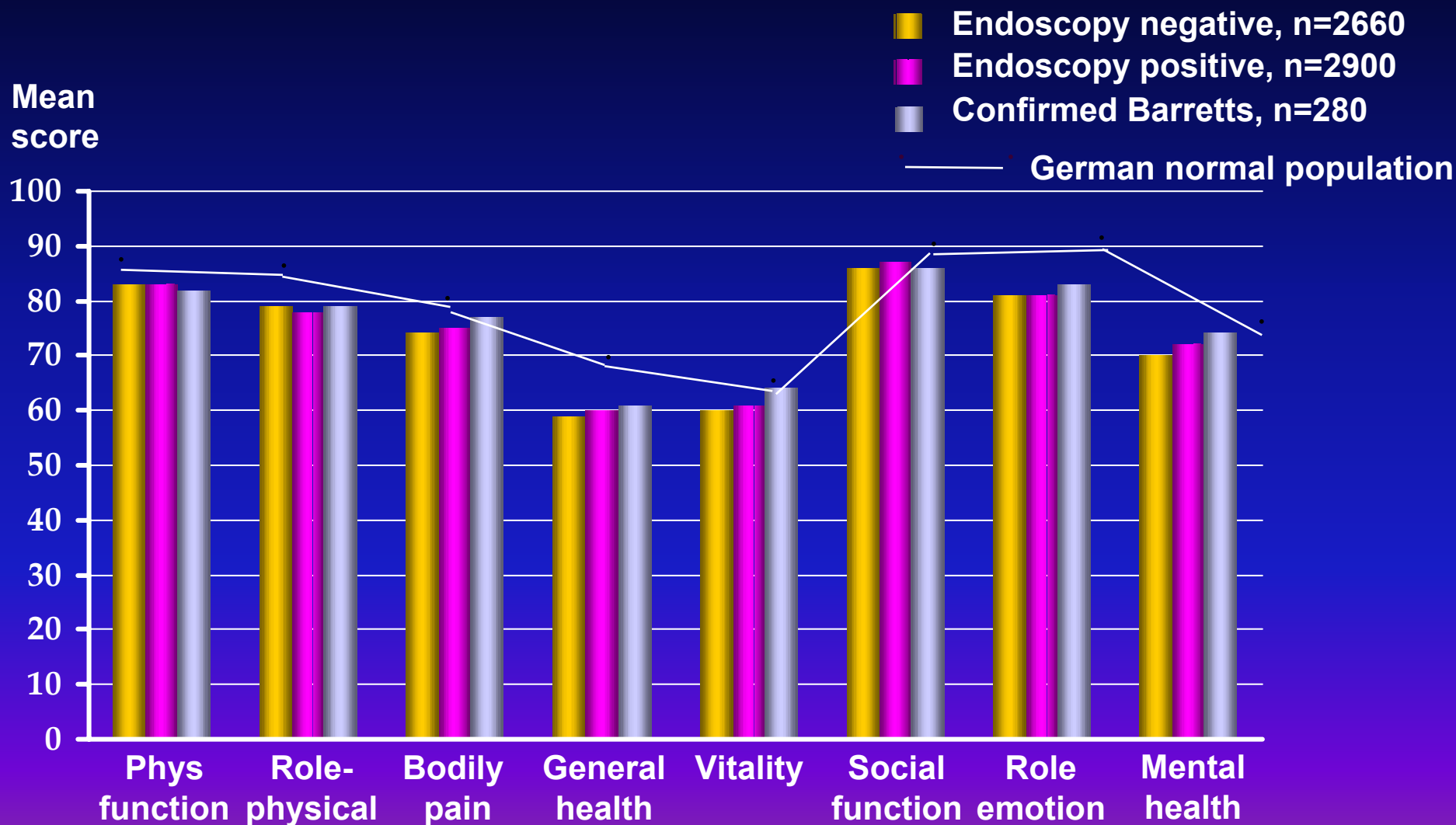


Recognize reflux disease

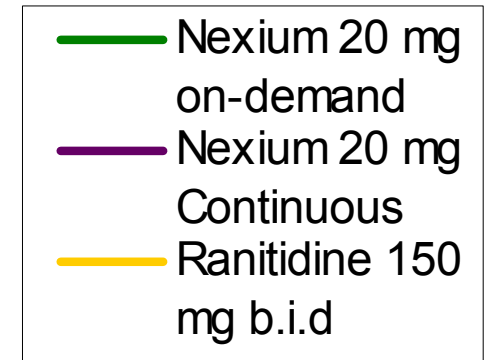
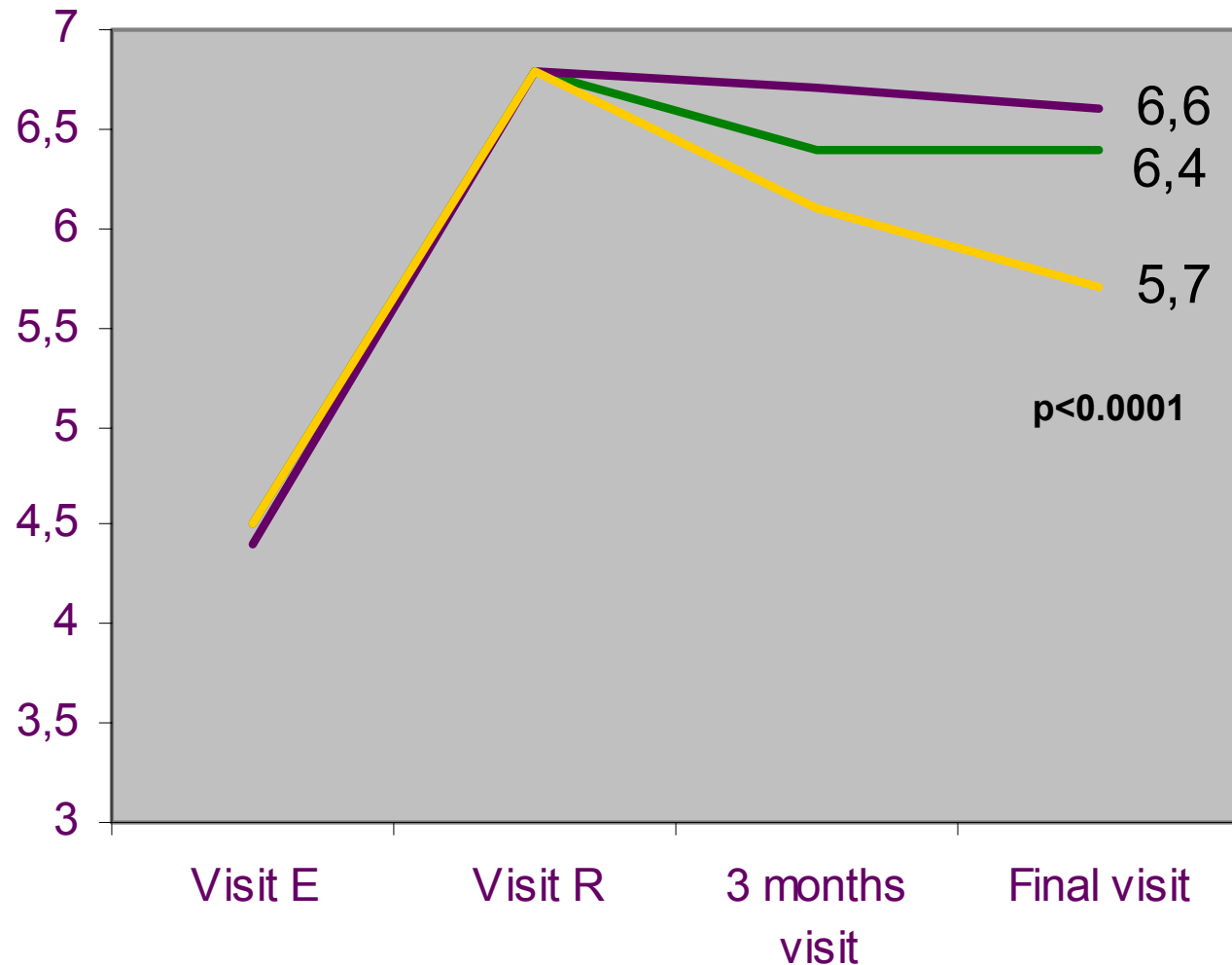
- Underdiagnosed
- Not recognized as a true medical condition
- Trivialized
- Not well treated
- Improve diagnosis
- Raise awareness of burden to patients and society
- Counteract trivialization
- Show superiority of effective treatment

A comparison of HRQL dimensions in patients with reflux disease after two weeks treatment with esomeprazole

Mean SF-36 scores



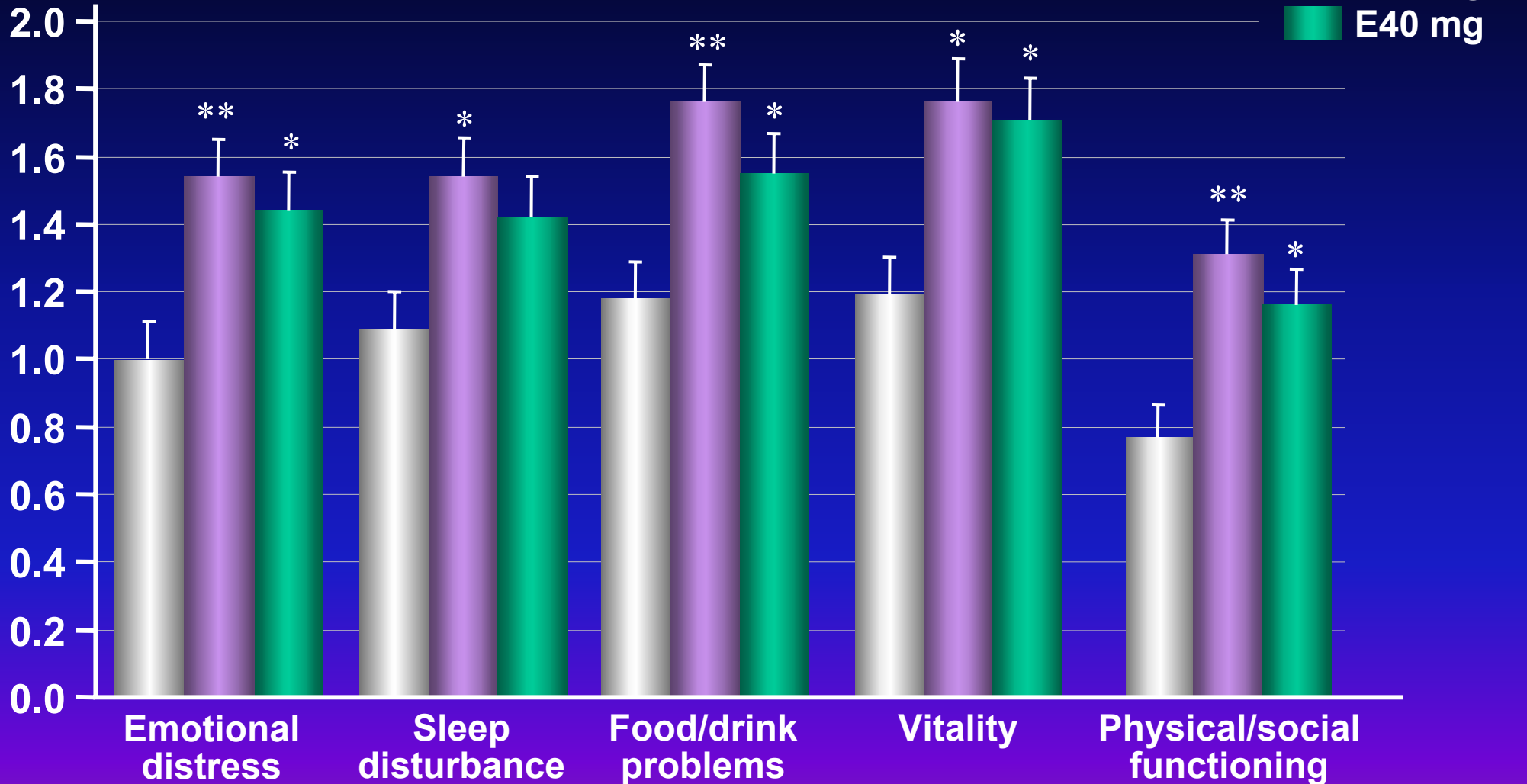
Sleep dimension scores (QOLRAD) along time



Improvement on QOLRAD dimensions

NASA 1

Improvement



* $p \leq 0.01$ vs placebo, ** $p \leq 0.001$ vs placebo (ANCOVA)

Sound Science
Clearly Communicated

The evolution of the use of PROs in clinical trials yesterday and today

Yesterday

- Investigator compiled ad hoc tools
- No or poor validation
- "Fishing expedition"
- PROs thrown into the study as an afterthought

Today

- Items derived from patients directly
- Psychometrically well validated scientific tools
- Clear hypothesis
- Well-integrated into the study protocol from the start

Evidentiary requirements for PRO outcomes

- Well-documented rationale for the outcome and measure
- Clear objectives and hypotheses
- Reliable and valid instruments
- Appropriate observation intervals
- Adequate sample size
- A priori data analysis plan (statistical & clinical significance)
- Careful implementation
- Interpretable results relative to clinical parameters
- Full and honest disclosure
- ***No more and no less than the requirements for clinical efficacy endpoints***

*Adapted from Leidy et al., Value in Health, 1999;
ISOQOL, 2000; Harmonization Meeting, 2002*

Price is what you ***pay***

Value is what you ***get!!!***

Value messages

- Relieves pain
 - Less pain with activity
 - Less difficulty with activity
- Improves functioning
- Improves well-being
- Improves health-related quality of life
- **Improves satisfaction with treatment**

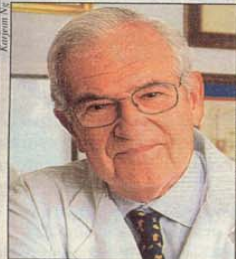
Communicating value

- Scientific Dissemination
- Direct To Consumer Marketing

How can you maximize your chances for good health care?

Patients, Know Your Rights

BY DR. ISADORE ROSENFELD



Karim N.

JWAC/Getty Stock Market

The importance of being fully informed about your medical condition—given the problems with America's health-care system today—cannot be overemphasized. In this article, PARADE's Health Editor spells out what patients need to know and do to get the best results from their physician, hospital and insurance provider.

THE TRUTH IS, medical care is rationed in this country. Physicians are spending less time with their patients. Many now work for insurance companies

or managed-care providers who have the last word on what tests or treatments they'll pay for. You may not even be told that there are better options than what you've been offered. So now, more than ever, you're essentially on your own when it comes to protecting your health and well-being.

What can you, the patient, do to maximize your chances of getting the best care possible? Basically, you must acquire as much information as you can

about your particular illness—and insist upon your rights. One of those rights is a say in the choice of a doctor. You also should be comfortable discussing all your treatment options before making or accepting a final decision.

As we go through life, we all experience a wide range of health problems. Symptoms appear that demand explanation and action. For example, suppose you've noticed that when you walk quickly up an incline, have sex



A patient receives a routine blood test—a simple procedure that can detect a large number of potential medical problems so they can be treated before matters get any worse. You have the right to have the results explained to you.

or are under stress, you develop discomfort or pressure in your chest, maybe even a little pain. It lasts only a few moments and doesn't really bother you.

If you have done your homework, you know that, depending on your age and sex, you probably have angina (due to narrowing of one or more arteries in the heart). That knowledge should lead you to call your doctor, who must then take the time to listen to your description of your symptoms.

Listening is extremely important. Doctors are so pressed for time these days that they often replace the critical face-to-face interview with a printed questionnaire with "Yes" or "No" answers. That's acceptable, as long as you also are given the time to elaborate on your symptoms. In this case, the diagnosis of angina can be made from your description alone.

Your doctor must now confirm what he or she suspects. There are several steps that can be taken. The preferable options may cost more and therefore not be offered to you (depending on your insurance coverage). You should know which

Be prepared when visiting a doctor: Know as much as you can about your illness—and what you are entitled to.



"I'm sorry doctor, but again I have to disagree"