

# **Patient-Reported Outcomes (PROs) as Endpoints in Medical Product Development**

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**Jane A. Scott, Ph.D**  
**Endpoint Reviewer**

**FDA/CDER/Office of New Drugs - Immediate Office**

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# Use of PROs in Medical Product Development

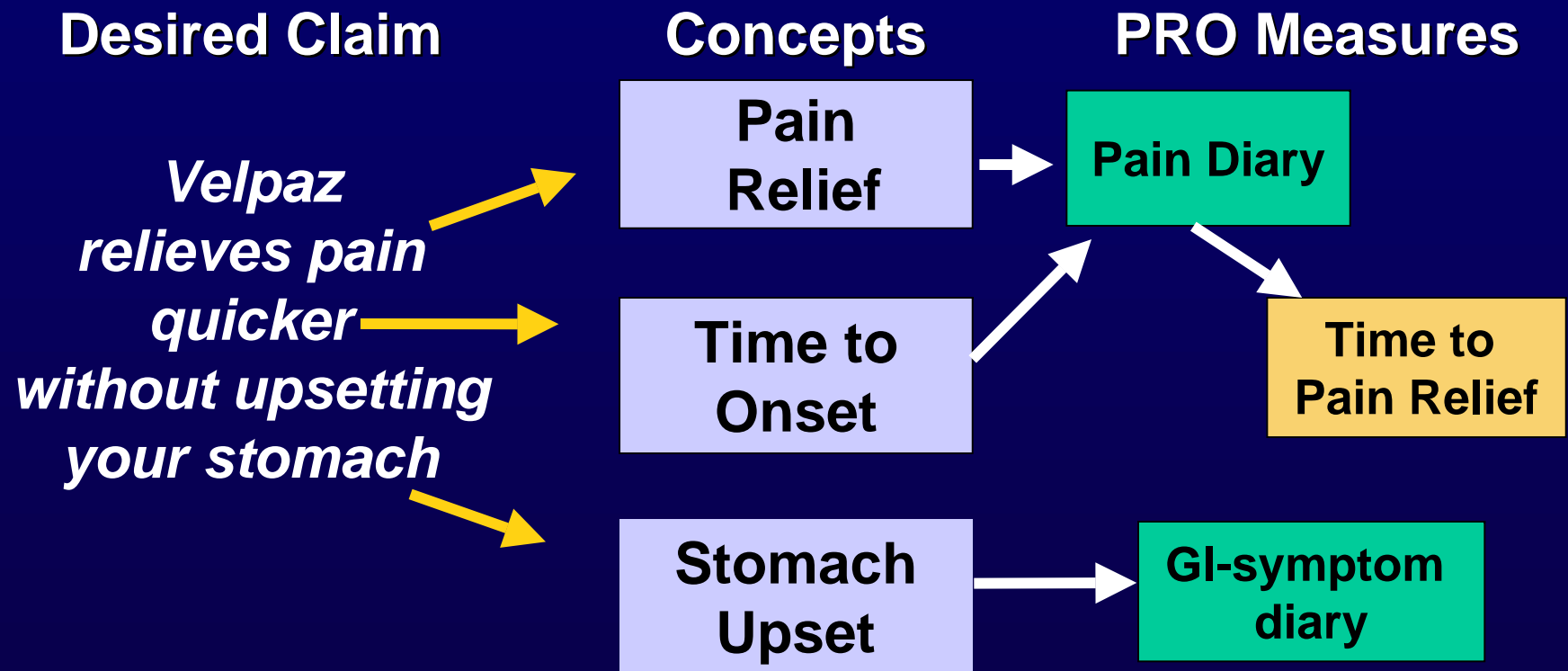
- **When treatment success / failure can only be known from a patient's perception or sensation (e.g., pain, fatigue, discomfort)**
- **Symptomatic conditions where clinical assessment does not perfectly correlate with patients' experience (e.g., rhinitis)**
- **Impact of disease or treatment on a patient's daily functioning or perceived well-being**

# Patient Reported Outcomes in Drug Development

- PRO data in 30% of labels for new products
- PRO assessments are used in clinical trials to
  - Describe patient populations
  - Characterize disease severity
  - Determine eligibility for trials
  - Evaluate treatment effects
    - Record otherwise unknowable information
    - Corroborate other endpoints
    - Help evaluate tradeoff between benefit and risk of tx



# Linking Desired Claims to PRO Endpoints



# **What Determines Whether a PRO is Adequate to Serve as an Endpoint?**

- **PRO concept matches claim sought**
- **Appropriate development process**
- **Adequate measurement properties**
- **Able to detect change**
- **Interpretable**
- **Proper implementation**



# Identify PRO for Each Concept

- Identify requirements for PRO in this trial
- 3 sources for PROs for trials



- Pretest in relevant patient population

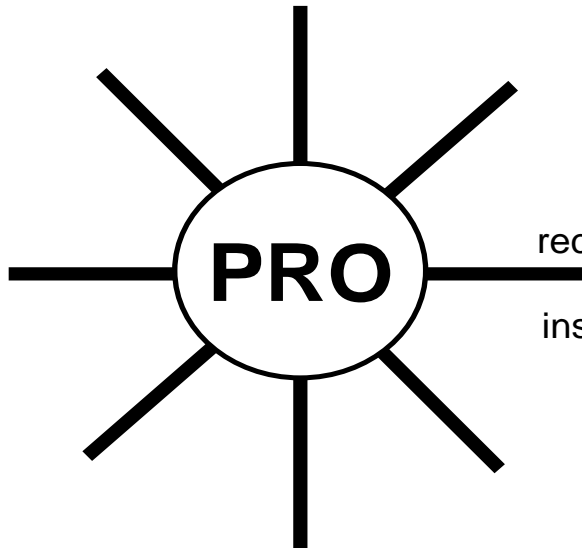


## Identify Concepts & Develop Conceptual Framework

Identify concepts & domains that are important to patients.  
Determine intended application and population.  
Hypothesize expected relationships among concepts.

## Modify Instrument

Change concepts measured,  
populations studied,  
research application,  
instrumentation  
or method of administration.



## Create Instrument

Generate items.  
Choose method of data  
collection,  
recall period & response options.  
Draft instructions. Format  
instrument. Draft procedures for  
scoring & administration. Pilot  
test draft instrument. Refine  
instrument & procedures.

## Assess Measurement Properties

Assess score reliability, validity, and ability to detect change.  
Evaluate administrative & respondent burden. Add, delete, or revise items.  
Identify meaningful differences in scores. Finalize instrument formats,  
scoring, procedures & training materials.



# Essential Measurement Properties for PRO Endpoints

- **Reliability**
- **Validity:**
  - Content / Face validity
  - Concurrent validity
  - Convergent / Discriminant validity (item and scale)
  - Predictive validity
- **Ability to detect change**
- **Interpretable scores (what is success?)**



# New Challenges: New Ways to Administer PROs



Computerized  
& Web-based  
Assessment



IVRS



PDA



# Conclusions

- Outcome assessments are important in drug approval
- Novel therapies and novel drug development approaches demand novel assessments, including both “objective” and patient-reported outcomes
- New endpoints (i.e., new to drug development) require thoughtful development and proper validation and/or experience
- An important part of that development/validation is providing clinical context to the outcomes data about what a change in scores means

