U.S. Food and Drug Administration
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FDA's Patient-Focused Drug Development Initiative

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Drug Benefit-Risk Assessment

- Analysis of Condition
- Current Treatment Options

→ Provides regulators with the clinical context for weighing benefits and risks

- Benefit
- Risk
- Risk Management

→ Incorporates expert judgments based on evaluation of the efficacy and safety data and the expected impact of efforts to reduce and further characterize risks

The Patient Perspective

- Patients are uniquely positioned to inform FDA understanding of the clinical context
- FDA could benefit from a more systematic method of obtaining patients' point of view on the severity of a condition, and its impact on daily life, and their assessments of available treatment options
- Current mechanisms for obtaining patient input are often limited to discussions related to specific applications under review, such as Advisory Committee meetings

Patient-Focused Drug Development under PDUFA V

- **FDA is developing a more systematic way of gathering patient perspective**
  - Designed to provide greater patient input on benefit-risk
  - Input can inform FDA analyses both during and outside of review
- **Prescription Drug User Fee Act Commitment**
  - Patient-Focused Drug Development is part of FDA commitments under the fifth reauthorization of the Prescription Drug User Fee Act (PDUFA)
  - At least 20 meetings on specific disease over the next five years
  - Meetings will help develop this systematic approach to gathering input

Which Disease Areas would be the Focus of PDUFA V Meetings? Criteria for Nomination

- Disease areas that are chronic, symptomatic, and affect functioning and activities of daily living
- Disease areas for which important aspects of that disease are not formally captured in clinical trials
- Disease areas for which there are currently no therapies or very few therapies, or the available therapies do not directly affects how a patients feels, functions, or survives
- Disease areas that reflect a range of severity
- Disease areas that have a severe impact on identifiable sub-populations (such as children or the elderly)
- Disease areas that represent a broad range in terms of size of the affected population

What Questions to Ask?
Disease symptoms and daily impacts that matter most to patients (draft questions)

- Of all the symptoms that you experience because of your condition, which **1-3 symptoms** have the most significant impact on your life? (Examples may include chronic pain, constipation, difficulty concentrating, etc.)
- Are there **specific activities** that are important to you but that you cannot do at all or as fully as you would like because of your condition? (Examples of activities may include sleeping through the night, daily hygiene, driving, etc.)
- How has your condition and its symptoms **changed over time**?
- What **worries you most** about your condition?

What Questions to Ask?
Patients' perspectives on current approaches to treating (draft questions)

- **What are you currently doing** to help treat your condition or its symptoms? (Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.)
- **How well** does your current treatment regimen treat the most significant symptoms of your disease?
- What are the most significant **downsides to your current treatments**, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, going to the hospital for treatment, restrictions on driving, etc.)
- Assuming there is no complete cure for your condition, what specific things would you look for in an **ideal treatment** for your condition?

Identifying Disease Areas for PDUFA V Patient-Focused Meetings

- In September 2012, FDA published a Federal Register notice with a list of ~40 preliminary disease areas nominations for potential meeting focus
- Public input on these nominations was collected through an online docket and at a public meeting held in October 2012
- At the close of the docket in November 2012, about 4,500 comments had been received
- FDA analyzed comments and consulted with each drug review division to determine the set of diseases for the first 3 years of PDUFA V

Disease Areas to be the focus of meetings for FY 2013-2015

FY 2013

1. Chronic fatigue syndrome – April 25 (in conjunction with scientific workshop)
2. HIV – June 14
3. Lung cancer – June 28
4. Narcolepsy – September

FY 2014

1. Breast cancer – October (in conjunction with scientific workshop)
2. Neurological manifestations of inborn errors of metabolism (in conjunction with workshop)
3. Idiopathic pulmonary fibrosis
4. Fibromyalgia
5. Hemophilia A, Hemophilia B, von Willebrand disease, and other heritable bleeding disorders

Disease Areas to be the focus of meetings for FY 2013-2015 (cont)

FY 2014 (cont.)

6. Sickle cell disease
7. Pulmonary arterial hypertension

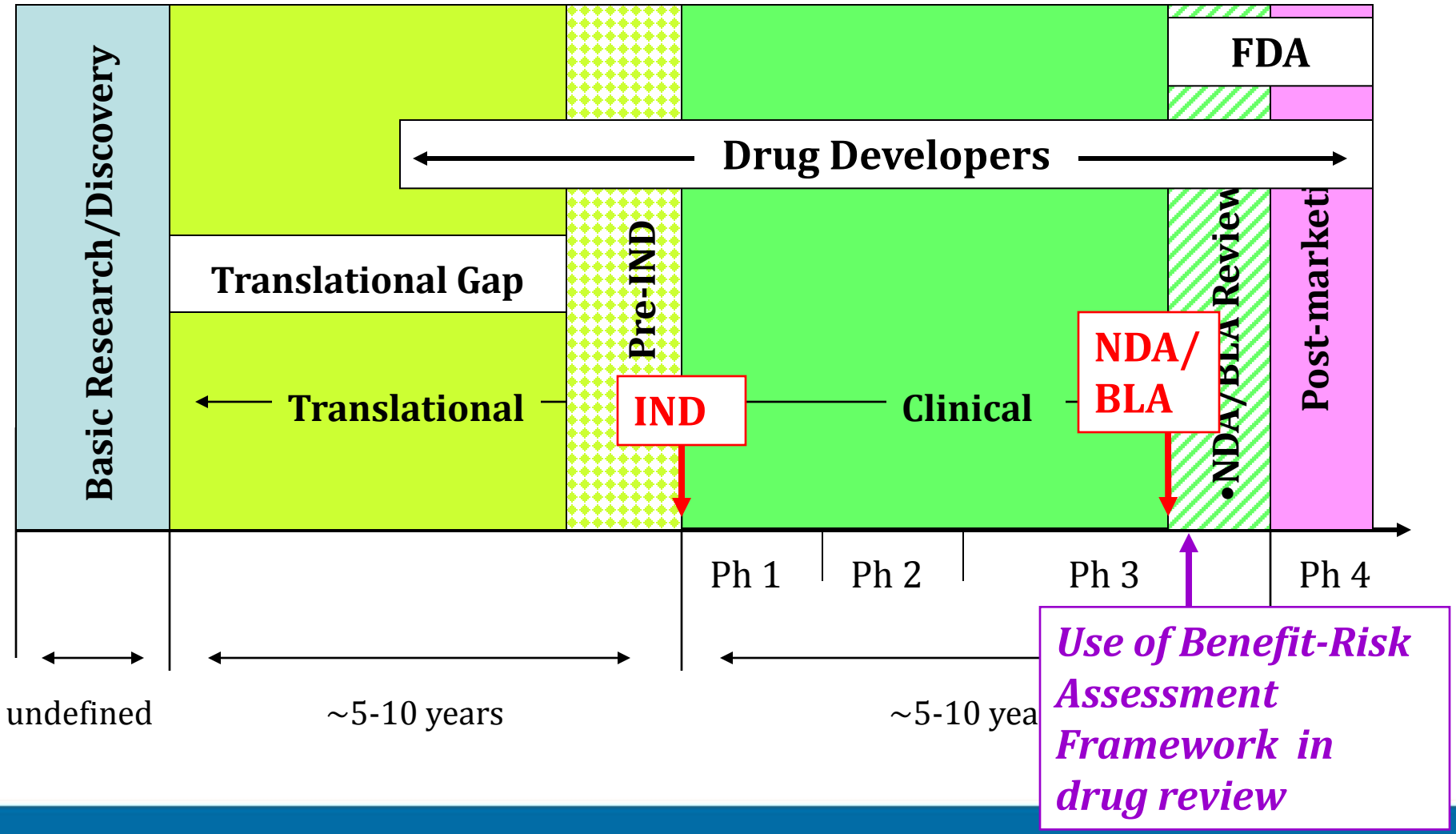
FY 2015

1. Irritable bowel syndrome, gastroparesis, and gastroesophageal reflux disease with persistent regurgitation symptoms on PPI
2. Parkinson's disease and Huntington's disease
3. Female sexual dysfunction (in conjunction with workshop)
4. Chronic Chagas disease
5. Alpha-1 antitrypsin deficiency

Product of PDUFA V
Patient-Focused Meetings

- Each meeting will result in a short meeting report that will be shared with FDA reviewers and posted on the FDA website
  - The patient perspectives captured in these reports will provide helpful insights for FDA reviewers conducting benefit-risk assessment for drugs to treat that disease

Reports generated by Patient-focused meetings will help inform drug Benefit-Risk Assessments

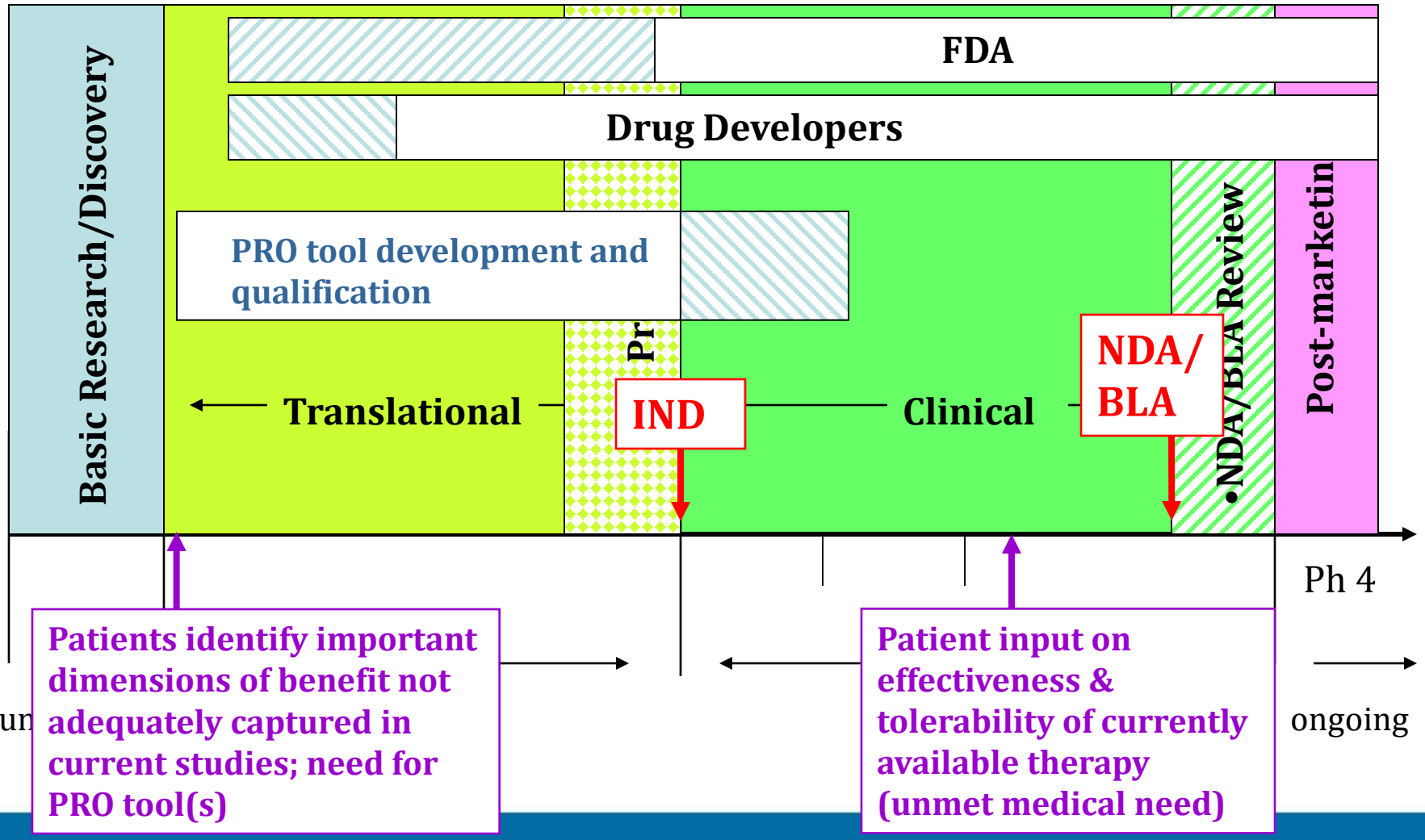


Other potential Impacts

- The narratives generated by patient responses to the questions posed in patient-focused meetings could point to the need for new outcome measures.
  - This identification of need could lead to work on development and qualification of new outcome measures

Meetings might stimulate longer-range development of new patient-focused outcome measures

For a specified disease area



Some Key Objectives For the PDUFA V Process

- Get broad patient input
- Use effective format(s) for collecting patient input
  - Faithfully capture patient views and represent transparently
  - Provide usable useful input to later FDA reviewer assessments
- Use venues/approaches that are both accessible and reliable

- FDA is holding bi-monthly meetings with patient stakeholders to discuss key process issues, such as:
  - How can FDA balance access for patient advocates who are local to FDA headquarters vs. those in other locations who have less physical access?
  - How can FDA ensure that certain sub-populations, such as patients with the most severe form of the disease, are represented?

Thank you