FDANEWS

May 10, 2018
San Francisco

The West Coast Symposium on Expanded Access

BUILDING EXPANDED ACCESS INTO THE CLINICAL DEVELOPMENT PROCESS

Theme of this Workshop

- A modern, constructive analysis of the commercial opportunities in pre-approval treatment access
  - **Engaging more patients**
  - **Learning more from patients**

Takeaways

- **Clarity** on the regulatory and business conditions of pre-approval access
- **Best practices** for industry and health systems
- Thorough **assessment of narratives** and roadblocks
- **Emerging solutions** to the hardest challenges
- **Opportunities** for value generation

What is Expanded Access?

- U.S.: Expanded Access Programs (EAPs)
  - ***Clinical trials for groups of patients who cannot get into regular clinical trials***
    - *Investigational Therapeutics and Diagnostics*
    - *Serious or Immediately Life Threatening Conditions*
    - *For Treatment Use*
- U.S.: Single-Patient Exemptions for Exceptional Cases (Individual IND)
- Non-U.S.: Regulatory Channels for Treatment-Use of Pre-Market products.

= "Pre-Approval Access"

Unmet Medical Need: A Legacy Problem

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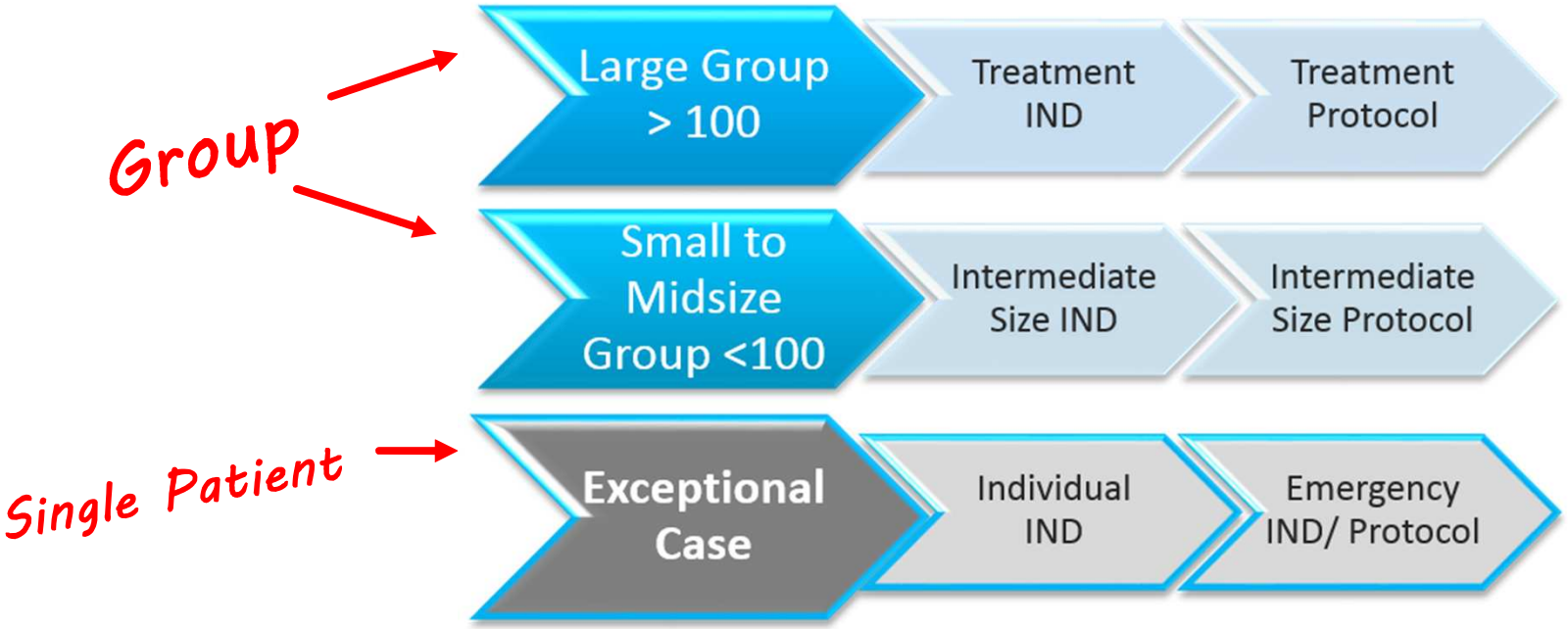
Condition	Annual Mortality	High-Risk Population*	Population Definition
Heart Failure	634,000	6,000,000	Currently at Failure
Cancer	596,000	5,500,000	Stage Diagnosis
CLRD (Respiratory)	156,000	3,000,000	Stage Diagnosis
Cerebrovascular	140,000	2,000,000	3-Year Stroke History
Alzheimer's	111,000	2,000,000	Stage Diagnosis
Diabetes Mellitus	80,000	1,500,000	Hazard Ratio
Flu / Pneumonia	57,000	1,000,000	Hazard Ratio
Nephritis	50,000	1,000,000	Hazard Ratio
TOTAL	**1,824,000**	**22,000,000**	

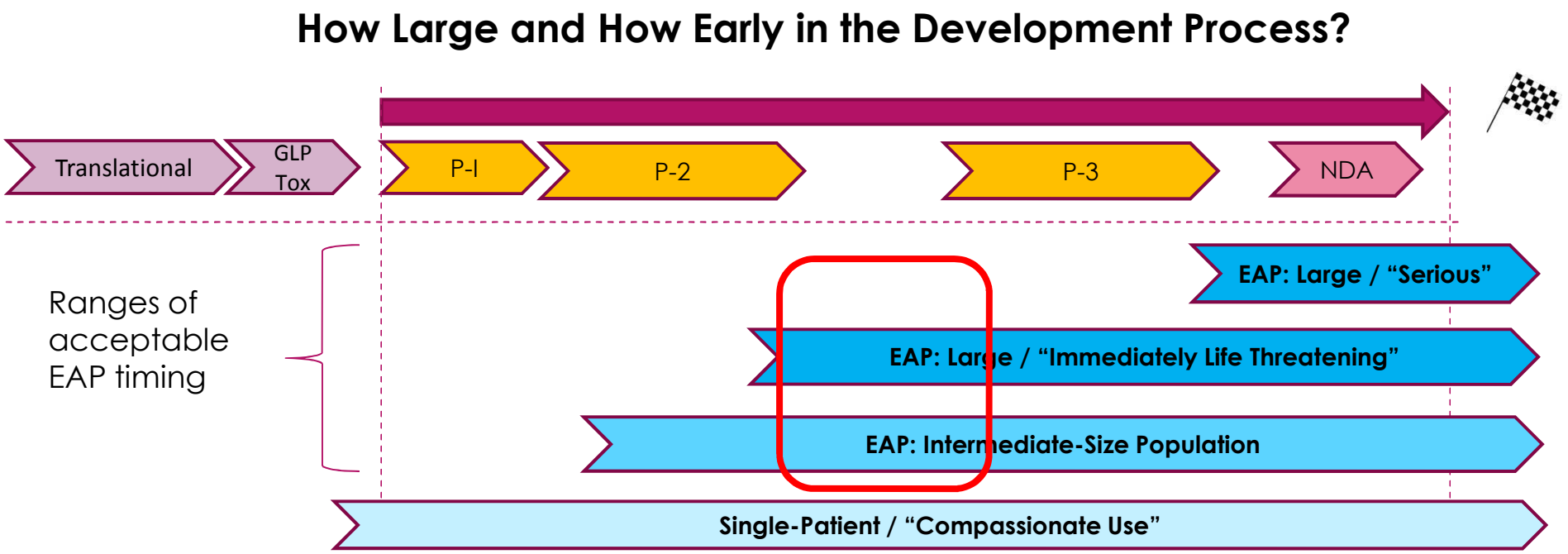
*Calculated from CDC reported statistics, relative mortality rates, and independent range estimates.

1. Background

U.S. Two Channels (21 CFR 312.310, .315, .320)



1. Background



Presenters

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Agenda

1. The Foundations
2. Strategy
3. Commercial Value
4. Roundtable Challenge
5. Innovation