



Industry Updates and Panel Discussion - Why it's important to be in a Clinical Trial.

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How do drugs get approved?

FDA Mandate – *improve how a patient feels, functions, or survives.*

TRADITIONAL



Clinical Endpoint

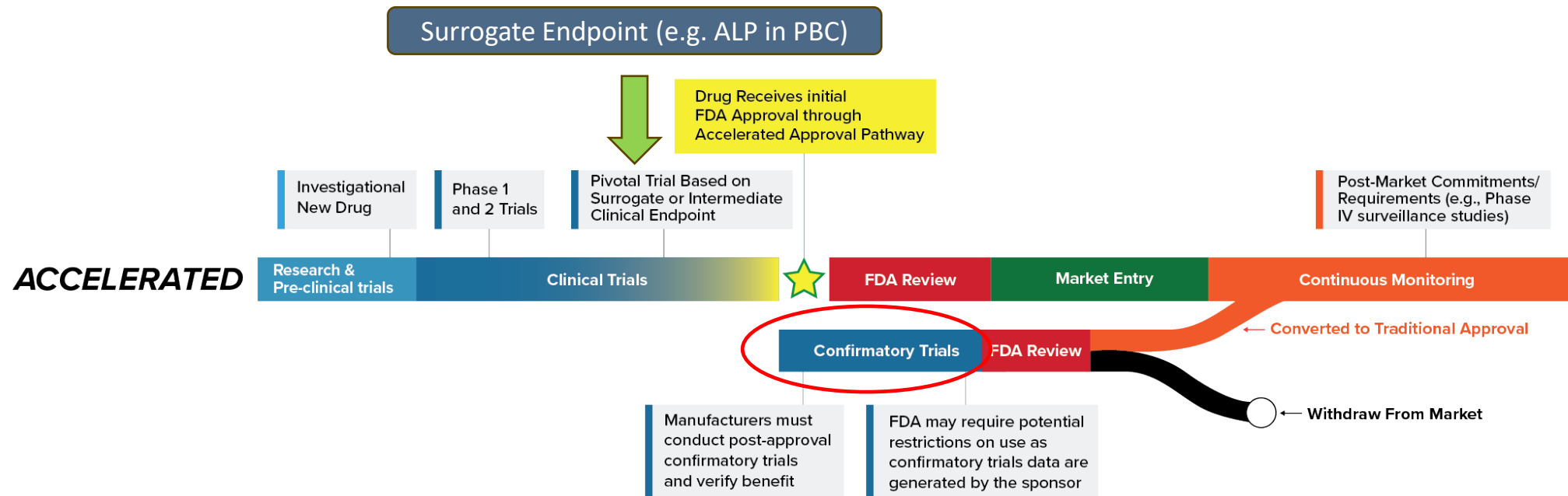
- Feels – *itching; fatigue*
- Functions – *hospitalizations, ERCPs*
- Survives – *cirrhosis, ascites, liver transplant.*

But chronic disease like PSC take years to impact survival. In these cases, there are **Validated Surrogate Endpoints**

- Hepatitis C Cure
- Improved A1c (diabetes)

There is no accepted validated surrogate endpoint for PSC

How do drugs get approved?



There is no accepted surrogate endpoint for PSC that is reasonably likely to predict how a patient feels, functions, or survives.

Current challenges and opportunities to drug development in PSC

- Challenges
 - Traditional clinical endpoints occur at a low rate
A clinical trial with endpoints of liver transplant or death would require hundreds of patients over a decade or more
 - No surrogate endpoint for accelerated approval
Lack of understanding of the mechanism of disease
- Opportunities
 - Expansion of clinical endpoints acceptable to FDA
Progression to cirrhosis, development of portal hypertension, cholangitis
 - Treatment for symptoms
However, limited application to those with the symptoms