Compliance Considerations for Program Development for Gene Therapy and Ultra Rare Disease Products

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Welcome, Introductions, & Disclaimer

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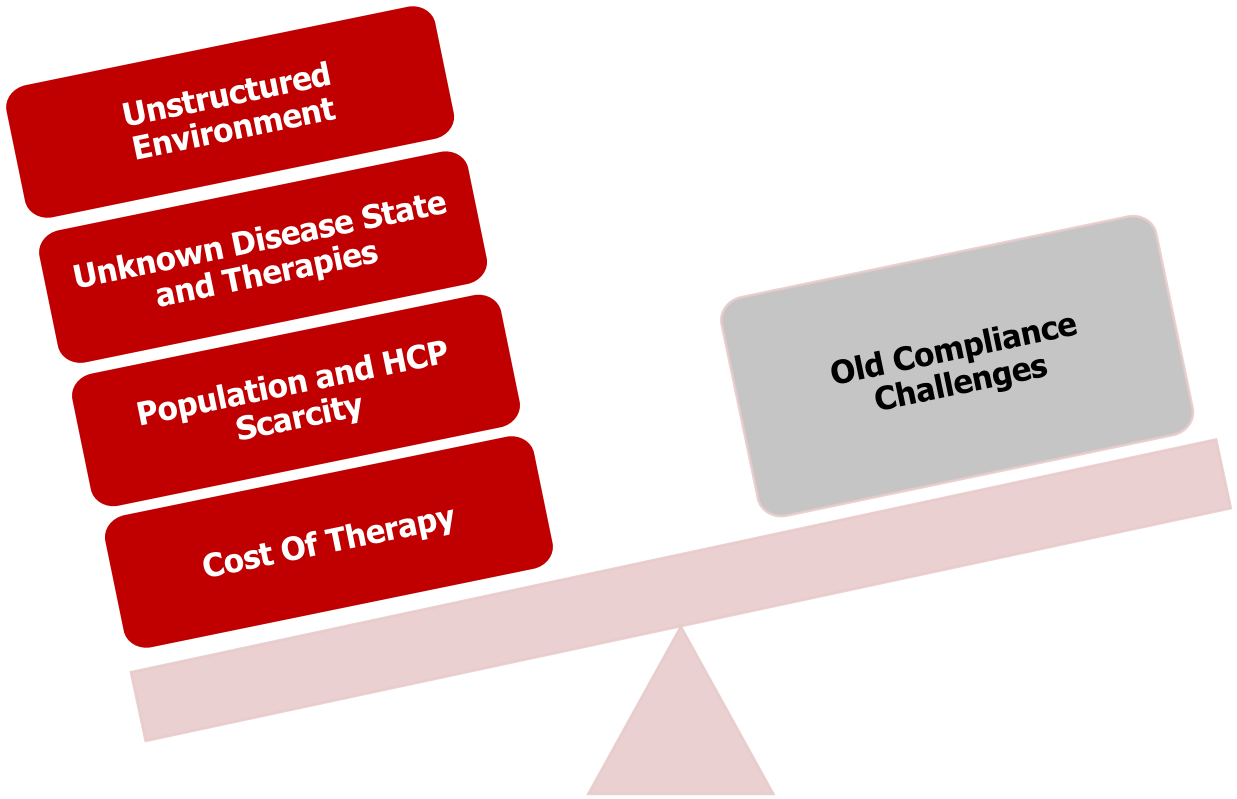
The views and ideas presented in this session are those of the speakers and are not necessarily shared by the presenters' employers.

Any examples provided are hypotheticals, and should not be attributed to any individual company.

Niche Pharmaceutical Products

NEW FRONTIERS

Niche pharmaceutical products such as gene therapy (GT) and ultra rare disease products face a complex landscape. While these products can be very different, the companies that develop these therapies face many of the same issues that are atypical of the traditional life sciences manufacturer experience.



New Challenges

INTERACTIONS WITH HCPS

Additional touchpoints with HCPs needed for Rare Disease and GT companies

- How to balance the additional legitimate needs for interactions with HCPs against the risks associated with more exchanges with HCPs?

Additional interactions with various company employees (i.e. sales reps, MSLs, patient support staff, market access)

- Additional Touchpoints
- Operational Considerations
- Personalized Medicine (impacts GT and other novel treatments)

Considerations when hiring HCP consultants

- Challenge to identify appropriate and knowledgeable HCPs who are limited in niche therapies

New Challenges

INTERACTIONS PRIOR TO THERAPY/DRUG APPROVAL

Interactions Prior to Therapy/Drug Approval

Disease State Awareness Parameters

- Different from traditional promotional programs for products post-approval

Patient Identification

- Discussions with HCPs

Single Product in Therapy Class

- Additional promotional considerations

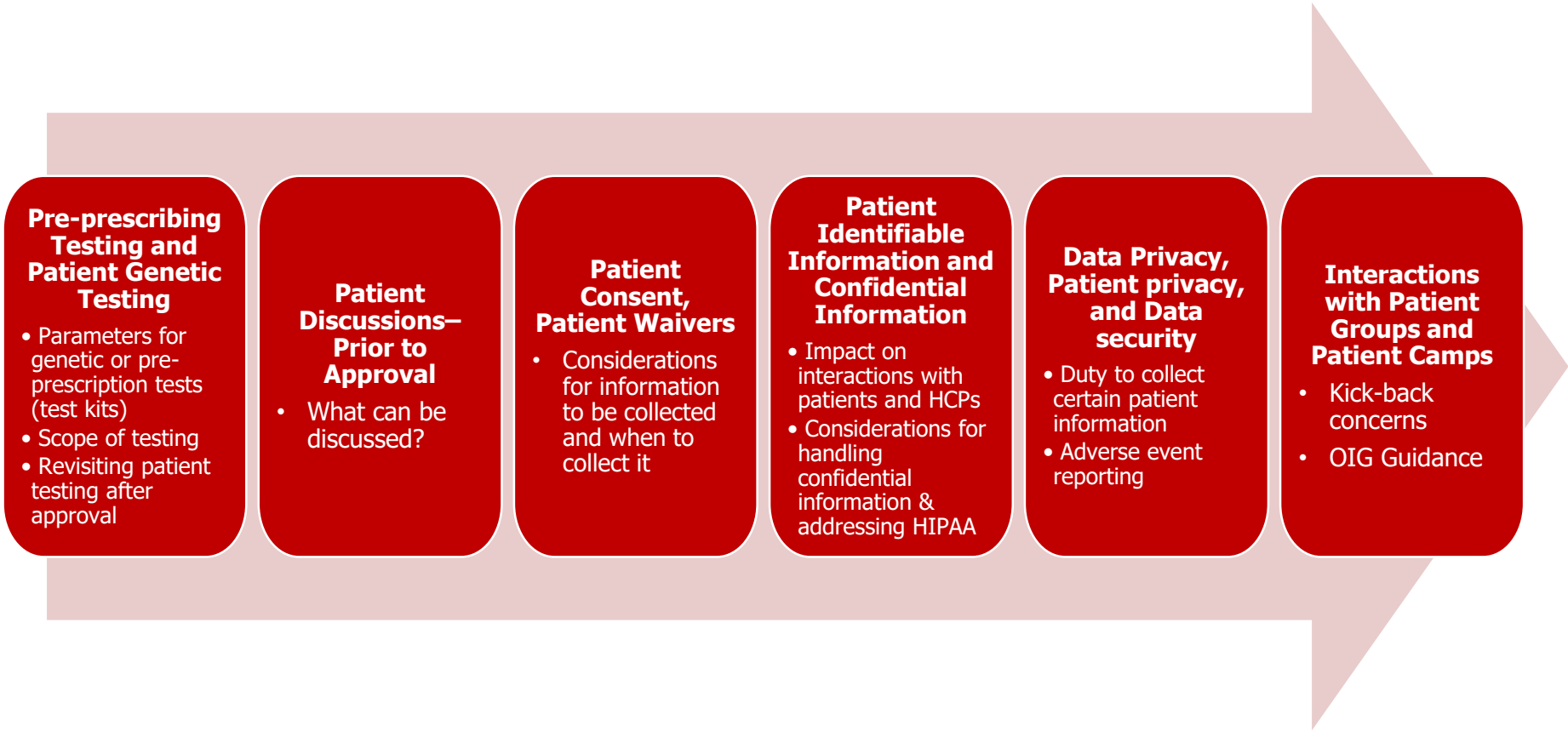
21st Century Cures Act

- Privacy issues and Payor concerns

New Challenges

PATIENT INTERACTIONS

There are a number of new challenges that face niche therapy companies when interacting with patients throughout the continuum of care:



New Challenges

KEY CONSIDERATIONS

There are a number of risks specific to this group of patients treated with this unique therapy and payer model; therefore companies should:

- Conduct Enterprise Risk Management activities
- Ensure all executive members understand risks and compliance challenges
- Understand the material impact of risks
- Ensure prior understanding of potential adverse events, pre-approval promotional enforcement risks, etc.
- Gain understanding of company's risk tolerance
- Create strategies to address and mitigate key risks
- Consider how the government may look at specific risks
- Develop patient interaction policies and documentation

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Q&A