HELIO HEALTH

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

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Welcome, Introductions, & Disclaimer

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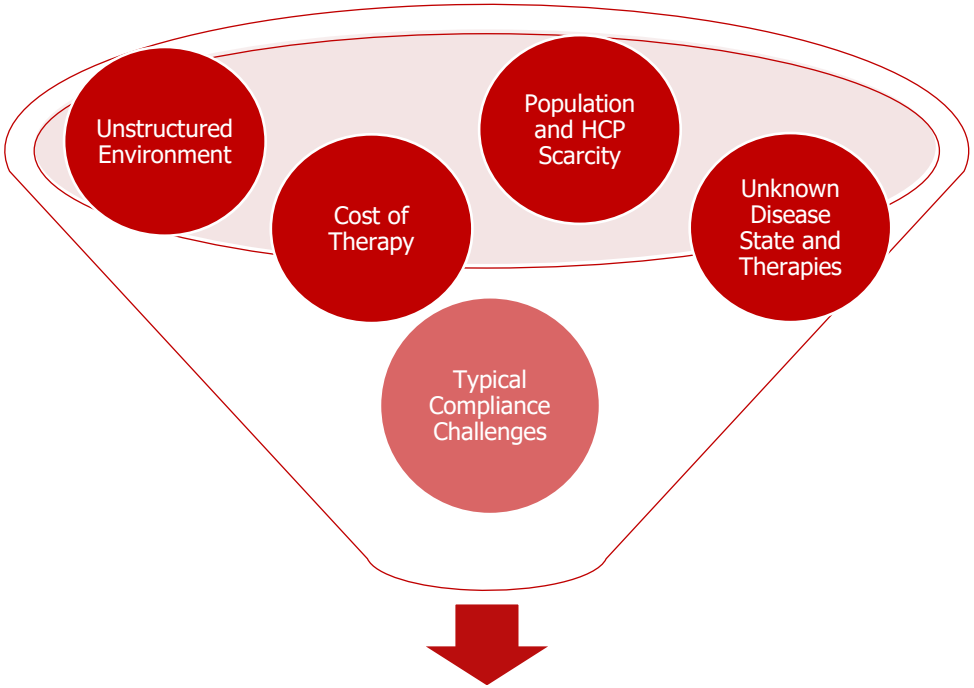
The views and ideas presented in this session are those of the speakers and are not necessarily shared by the presenters' employers.

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

Niche Pharmaceutical Products

NEW FRONTIERS

Manufacturers of niche products such as gene therapy (GT) and ultra rare disease products face a complex landscape. Not only must they deal with the challenges of bringing a successful life sciences product to market, but they must also address new risks and challenges that are atypical of the traditional manufacturer. However, due to the evolving regulatory landscape and the novelty of these therapies, there are also unique ways in which companies can drive value and create efficiencies.



New Compliance Challenges & Efficiencies for Niche Product Manufacturers

New Challenges

INTERACTIONS WITH HCPS

Additional and new touchpoints with HCPs

- Balance additional legitimate needs for interactions with HCPs against the risks associated with more exchanges with HCPs
- Risks and challenges associated with a narrowly-defined approved label (since many of these therapy labels are much less narrow than overall disease state); traditional promotional calls must be separated from disease state awareness calls – ensure appropriate transition statements
- Additional considerations related to consultant arrangements
- Disease state awareness

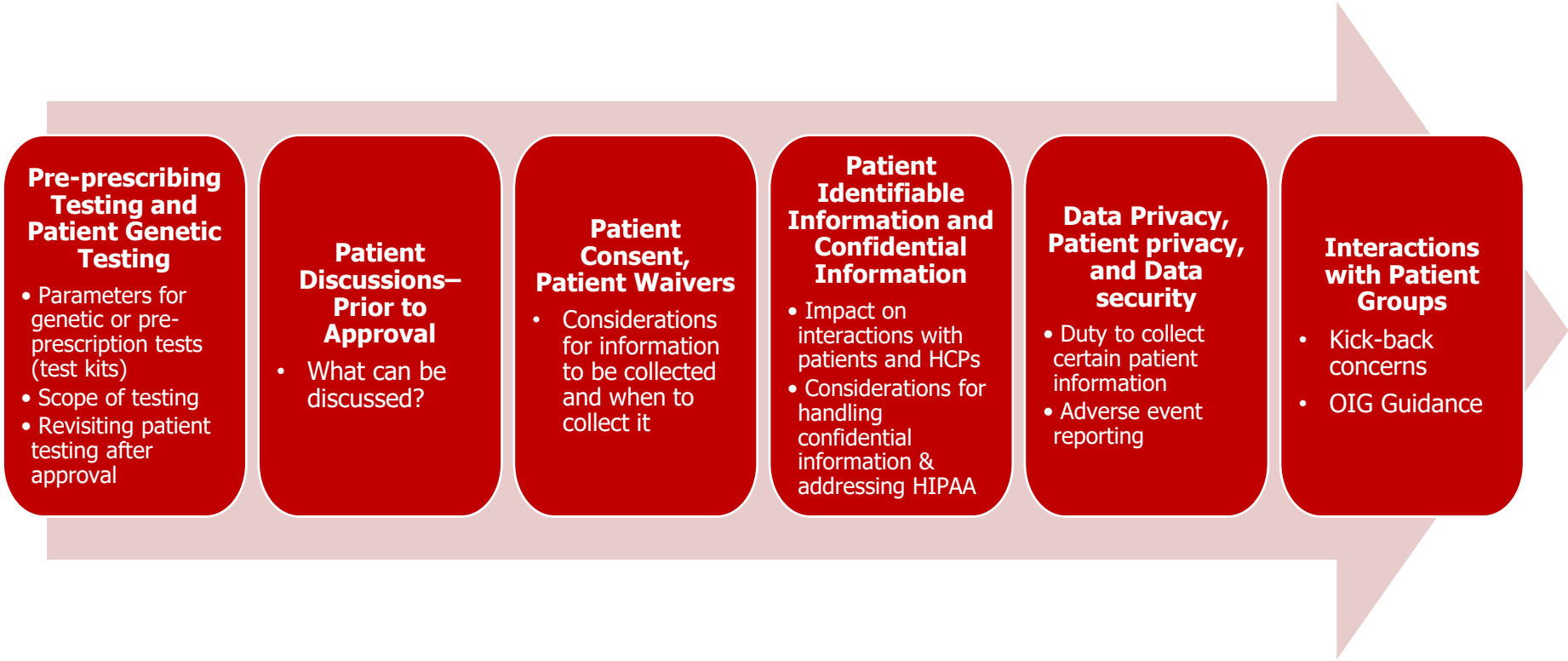
Additional interactions with company employees (i.e. sales reps, MSLs, patient support staff, market access)

- Medical interactions with Commercial
- Patient services interactions with other functional areas
- Additional staff considerations with different requirements than traditional field that require significant time including nurse educators, sales reimbursement specialists, etc.

New Challenges

PATIENT INTERACTIONS

Niche therapy manufacturers face several new challenges with the increasing number of patient interactions they have throughout the continuum of care:



New Challenges

KEY CONSIDERATIONS

There are several risks that should be considered when interacting with patients and HCPs for unique therapeutic areas; therefore companies should:

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Q&A