



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

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# Welcome, Introductions, & Disclaimer



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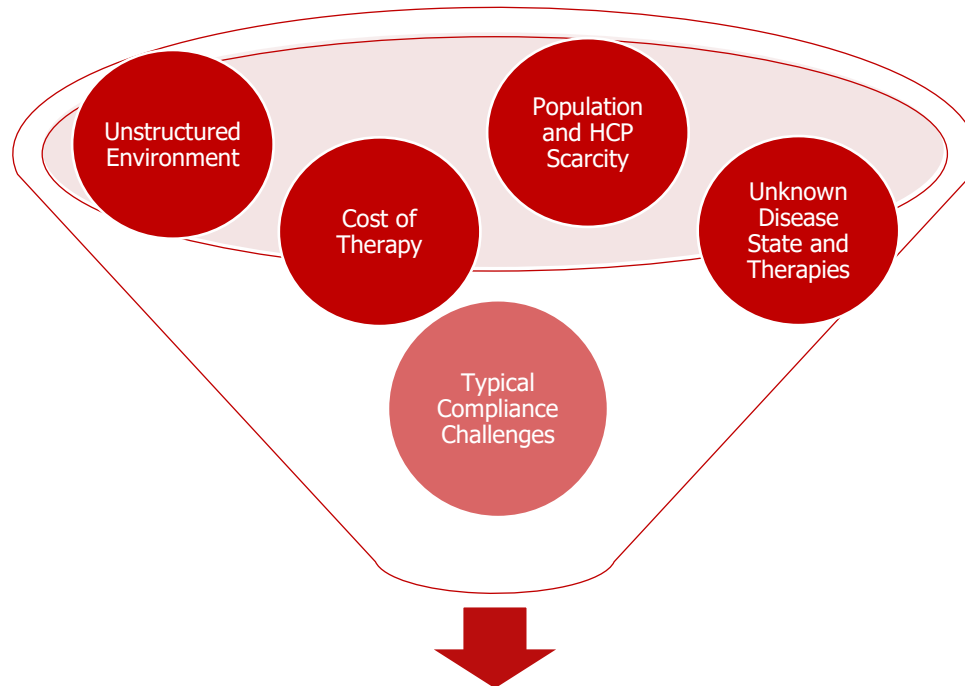
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**Any examples provided are hypotheticals, and should not be attributed to any individual company.**

# 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, MSAs, 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:**

### **Pre-prescribing Testing and Patient Genetic Testing**

- Parameters for genetic or pre-prescription tests (test kits)
- Scope of testing
- Revisiting patient testing after approval

### **Patient Discussions—Prior to Approval**

- What can be discussed?

### **Patient Consent, Patient Waivers**

- Considerations for information to be collected and when to collect it

### **Patient Identifiable Information and Confidential Information**

- Impact on interactions with patients and HCPs
- Considerations for handling confidential information & addressing HIPAA

### **Data Privacy, Patient privacy, and Data security**

- Duty to collect certain patient information
- Adverse event reporting

### **Interactions with Patient Groups**

- Kick-back concerns
- OIG Guidance

# 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:**

**Conduct Enterprise Risk Assessment or Compliance Risk Assessment**

**Gain understanding of company's risk tolerance**

**Develop policies and supporting documentation specifically geared towards patient interaction policies, market access reimbursement policies and others**

**Create a monitoring and auditing plan to address potential areas of increased risks or uncertainty (for activities/programs that may be unique to company or therapy)**

**Watch ongoing industry trends and be aware of ongoing investigations, settlements, CIAs, and OIG guidance to help drive compliance decisions**

**Ensure prior understanding of potential adverse events, pre-approval promotional enforcement risks, etc.**

Q&A

