

Pharmacovigilance for Cell Therapies

Industry Perspective

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Current Status

- ❖ HCT/Ps are currently reported using Form 3500A
- ❖ Reviewed by Tissue Safety Team
- ❖ Primary focus is transmission of infection
- ❖ Tracking focuses on communicable diseases
- ❖ Form is the same one used for devices and other mandatory reports

ISCT / Stakeholder Focus

- ❖ Applications for cell therapies is rapidly expanding
- ❖ Acute and long-term emerging adverse events include far more than transmission of communicable diseases
- ❖ Issues have out-stripped the current arrangement for adverse event reporting for cell therapies
- ❖ Issues are distinct from those of gene therapies

ISCT Proposal

- ❖ Cellular Therapies Stakeholder Community/FDA Collaborative effort to revise the form and accomplish the following:
 - » Differentiate type of cell therapy used (hematopoietic, mesenchymal, smooth muscle, etc.)
 - » Differentiate stem cell lineage (embryonic, non-embryonic)
 - » Capture information about cell source (autologous, allogeneic, matching criteria)

Other issues for a New AE Form

- ❖ Indication for use
 - » Bone marrow transplant
 - » Somatic pathology (cardiovascular, neurologic, etc.)
- ❖ AE Classifications specific to cell therapies
 - » Graft-versus-host-disease
 - » Atherosclerotic or re-stenosis complications
 - » Tumorigenesis
 - » Unexpected differentiation
 - » Metastases of infused cells

Create a Long-Term Pharmacovigilance Database Specific to Cell Therapies

- ❖ Detect and characterize specific and late complications of HCT/Ps
- ❖ Anticipate emerging technologies utilizing HCT/Ps
- ❖ Capture interactions between AEs associated with HCT/Ps and ancillary therapies used to support their use or manage their potential complications
- ❖ Capture interactions with other therapeutic modalities including drugs and devices

ISCT Proposal

- ❖ Joint FDA/Stakeholder working group focused on development of new form and dedicated database
- ❖ Pilot project in selected cell therapy sites
- ❖ Prospectively designed methods for signal detection
- ❖ Analysis and launch after initial pilot