Issues with Biobanking

Mahendra Rao- Q Therapeutics & NYSCF
HSC & derivatives
- Cord Blood & ancillary tissue
- MSC & MSC like
- NSC & derivatives
- All other cells

Unregulated
- Medical Tourism
- Cosmeceuticals
- Medical Practice

Devices
- Unregulated
- Allogenic/autologous
- Composite/Tissue/organoids

Unregulated
- ADSC
  - Legal in a country but regulated in another
  - Not considered a medical product

Cultured
- HSC & derivatives
- Cord Blood & ancillary tissue
- MSC & MSC like
- NSC & derivatives
- All other cells

Selected

Engineered

Combination

Marrow
- Cartilage
- T cells
- NK cells
- Cord Blood HSC
- Endothelial cells

Compsoite
- NSC
- Oligos
- Astrocytes
- Neurons
- OEG
- Schwann cells
- Pancreatic Islet
- Cardiomyocytes
- Hepatocytes
- Skeletal muscle
- etc

Skin full thickness
- Valves & Vessels
- Ureter, Bladder, Urethra
- Trachea, Oesophagus
- Cardiac sheets / RPE sheets
- Pancreatic islets, liver organoids
- etc

Valves & Vessels
- Ureter, Bladder, Urethra
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- etc

Skin
- etc

etc

etc
Autologous processed therapy

Each lot is a single product and is processed by different groups with different protocols.

Very hard to consider a clinical trial as in a standard model.

In the absence of guidance, cell products are already being stored.

Recent advances suggest that gene editing may enhance the utility of the cells.
Banking and Storage of Cells

Companies now storing bone marrow from cadaveric donors

Expanded bone marrow with variability

Placental cell products

iPSC banks

The people storing cells are not the ones getting an IND
Non stem cells
- RBC’s
- Plateletes
- T
- B
- Monocytes
- NK cells

Stem & Progenitor cells
- CD34+
- EBC (< 1x10^6)
- MSC (<1x10^8)
- others

Cord derived
- MSC-Like

Placenta derived
- Membranes
- Matrix
- Cells - MSC-like, others

Bank other cells
- Ship and distribute
- Collect and process other samples
- Support development of novel uses

Plasma
- EBV transformed lines
- Immortalized progenitors
- iPSC

Commercial products available

Bank
- Public/Pvt

One cord Blood Unit

Collect and process other samples

Support development of novel uses
Cord, Cord Blood and Placenta

1: Amniotic Fluid cells
2: Amniotic membranes
3: Placental cells (maternal and Fetal)
4: Placental tissue
5: Wharton Jelly
6: Cord Tissue derived stem cells
7: Cord Blood
8: Growth factors harvested from placenta
9: Plasma

Commercial Products
1: CelGene
2: Cytomedix- Nuo Therapeutics
3: Reliance- Placental membranes
4: CBR and other private cord blood banks
5: Cultured or expanded derivatives- Cytomatrix
Issues

Each lot is a single product and is processed by different groups with different protocols.

Each group has received IND approval for a single indication and now cells are being used for multiple additional indications.

What happens to units that are stored?

What happens to autologous processed cells?
Suggestions and Questions

Biobanks which supply processed samples need to be regulated and draft guidances be developed.

Can the FDA advise us as to how they expect Biobanks to be regulated?

Are there any recommendations or guidances that are being considered for public comment?

Does the FDA consider state licenses for establishing a banking facility akin to blood banks to be sufficient?
Possible Recommendations

Separate and update tissue and cell procurement and processing for therapeutic use are needed

Comparability by end product should be considered

Autologous processed cells should be treated somewhat differently than allogeneic processed cells and these differences should be clarified

The FDA should consider harmonizing state licenses for establishing a banking facility akin to blood banks

Please consider a role for accreditation organizations
Summary

Thank You
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