Cellular Therapeutics

Precision Medicine at Work Today

How America can Improve Public Health, Stimulate Industry and Capture National Competitiveness Opportunities through Cellular Therapeutics
Contents

① Breakthrough results from cellular therapeutics

② How cellular therapeutics are approved

③ Challenges in bringing treatments to the US public

④ Legislative changes needed

⑤ What should and should not change
Treatment of ALS-like symptoms

Age 58

Home
Houston, Texas

Diagnosis
Multiple System Atrophy (MSA)
Human breast reconstruction
Human breast reconstruction – 1 year follow-up
13 year old golden retriever with osteoarthritis
10 year old doberman with herniated disk

Pre-Treatment

April 30, 2012
Before we start – two important definitions

“Autologous”

*same donor and recipient*

“Homologous”

*similar in structure or function*
The problem: FDA approval process based on drugs

Under existing legislation, the FDA regulates cell-based therapies just like small drug molecules and biological products

- delayed patient benefits
- confusion for companies
- hindered growth
- loss of US competitive edge
Exempt
- Surgical procedures
- Bone marrow for homologous use in an autologous setting
- Minimally processed ADSC for homologous use in an autologous setting

Exempt from 21CFR 1271

IDE/510K Devices

361 HCT/P

351 HCT/P

Current FDA approach: one of three pathways

Therapeutic Product

- Devices
  - Autologous cells and scaffolds

- Bone marrow for homologous use in a non-autologous setting, or sorted or selected cells
  - Processed ADSC for homologous use in an autologous setting

- All allogenic, manufactured products
  - HLA matched unrelated donor
  - All non-homologous use products
  - All engineered or composite products

Exempt from 21CFR 1271

IDE/510K Devices

361 HCT/P

351 HCT/P
New category: “Autologous cellular therapeutics”

Exempt
- Surgical procedures
- Bone marrow for homologous use in an autologous setting
- Minimally processed ADSC for homologous use in an autologous setting

Exempt from 21CFR 1271

IDE/510K Devices

New Approval Category
- Devices,
  - Autologous cells and scaffolds
- All manufactured autologous cell products for both homologous and non-homologous use
  - Stems cells for autologous and/or HLA matched use
  - Banked cell use
- Bone marrow for homologous use in a non-autologous setting, or sorted or selected cells for homologous use
  - Processed ADSC for homologous use in an autologous setting

361 HCT/P

351 HCT/P

Items in red would move to the new category
- All allogenic, manufactured products
- HLA matched unrelated donor
- All non-homologous use of autologous cell products
- All engineered or composite products
Specific high-level recommendations

1. Create a new category of approval for autologous or HLA matched cell-based therapy
2. Include stem cell banking for allogeneic, autologous, or HLA matched cell use
3. Grant conditional approval for a time-limited period once initial safety confirmed
4. Long-term safety and efficacy must be demonstrated before full approval
Caution also needs to be applied

× Do not broaden the exempt category – it will reduce oversight
× No reduction in safety and quality requirements
× Avoid increase the regulatory burden
× Higher standards for new vs. existing therapies could stifle innovation
× No indefinite conditional approvals
THANK YOU.