PPTA
Plasma Protein Therapeutics Association

The Role of Government in Plasma Therapeutics

Presentation to the

Advisory Committee on Blood Safety and Availability

January 28-29, 2004

Conclusions

US Government Role in Plasma Therapeutics is:

- Assuring Safety and Availability
  - Well regulated, high quality therapies
- Preserving Access and Choice
  - Appropriate payment systems that recognize the unique nature of plasma therapies

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Where to Plasma Therapies Fit?

National Resource

- Blood, tissue, organs
- Safety is paramount
- No substitutes
- Limited supply
- Market vigilance

Pharmaceutical

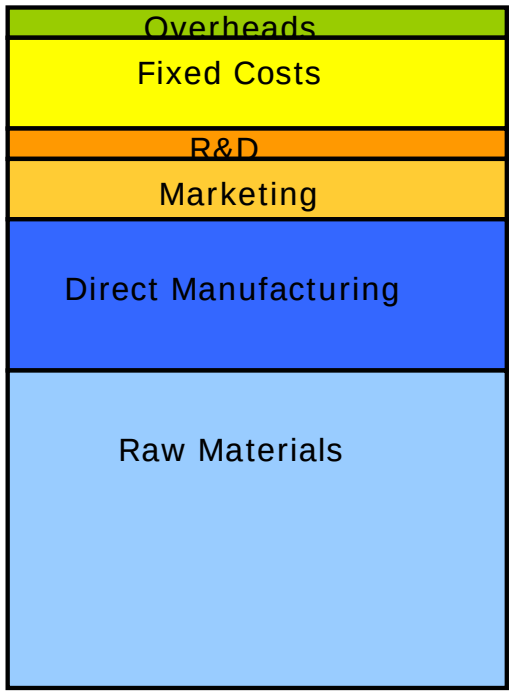
- Lipitor, Nexium, Vioxx
- Different safety profile
- Generics accepted
- Make more, sell more
- Free market

National Resource

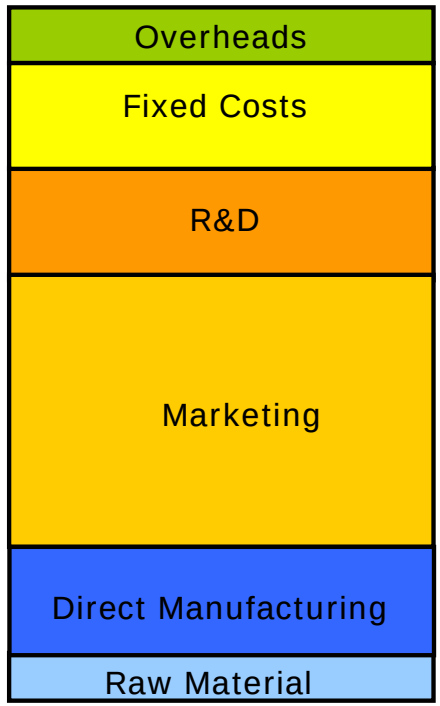
- Plasma Collection – France, Spain, Italy (others)
- Fractionation – BPL (U.K.), Sanquin (Holland)
- Distribution – Canadian Blood Services
- Payer – most national authorities
- Regulator – most national and supra national authorities (e.g., FDA, EMEA)

Plasma Therapies are not Pharmaceuticals

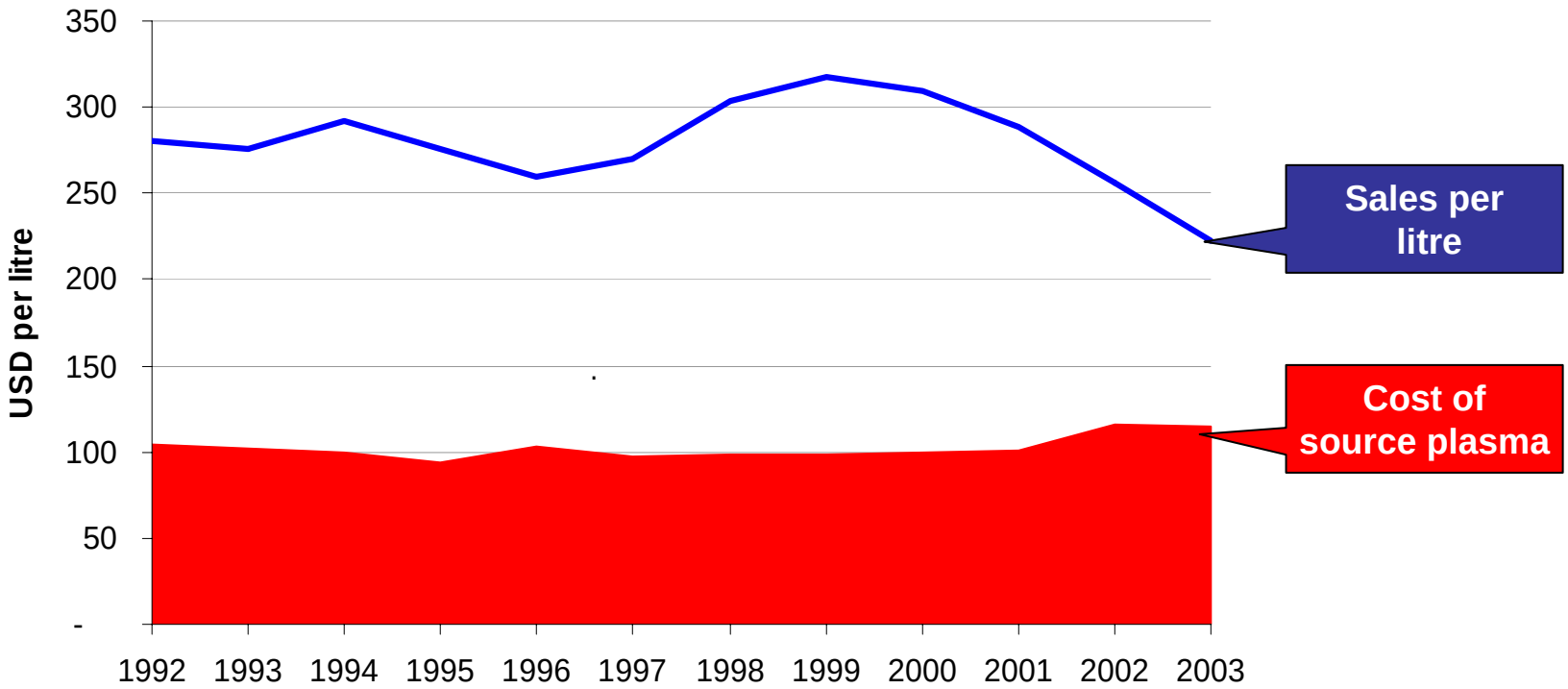
Plasma Industry
Cost Base



Pharmaceutical Industry
Cost Base



Real* sales per liter and real cost of plasma



* Prices and costs corrected for inflation – source MRB

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Unique Therapies

Need a paradigm shift:

- Focus on end-product safety
- Plan for Emerging Pathogens
- Facilitate R&D through clinical trial requirements
  - Broaden indications for core products
- **Establish appropriate payment methods**

Unique Therapies

Payment Methods:

- Medicare Part B –
  - IVIG listed as Generic: CMS disconnect with FDA
  - Provides payment at average sale price (ASP) plus 6% in 2005 and 2006
- HOPPS –
  - Therapies listed as non-innovator Multiple Source (46% AWP)
    - 50% less than Single Source rate (up to 95% AWP)
    - 30% less than innovator multi-source rate (68% of AWP)
  - Should be listed as Single Source

Unique Therapies

Medicaid Limitations on Access and Choice

- Prior Authorization
  - 37 states have some form of PA
- Reference and MAC pricing
  - Florida and Washington State
- Single Source provider contracts
  - Florida and Massachusetts

Conclusions

- Plasma Therapies are Unique: they are neither a “national resource” nor a pharmaceutical.
  - Payment systems must address plasma therapies
    - Traditional cost containment methods are not appropriate
  - Public Health Regulators should refocus:
    - End product safety
    - Address emerging pathogens
    - Facilitate clinical trials