A Policy Analysis of Spinraza: Orphan Drug for Spinal Muscular Atrophy

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Introduction

Spinal Muscular Atrophy (5q-SMA) is the leading genetic cause of infant mortality.

5q-SMA

- An ultra rare disease
- Affects <200,000 persons
- Leads to profound physical disability, with intact intellectual capacity and functioning
- Prior to December 2016, only supportive care was available to patients with 5q-SMA
- December 23, 2016 – FDA approved Spinraza
  - The first treatment for 5q-SMA

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Problem

- Spinraza treatment cost is phenomenal
  - $120,000 per dose
  - 1st year: $750,000
  - Subsequent years: $375,000
- Facility and administrative costs are additional
- Current price is unsustainable long term
- Zolgensma treatment cost is $2.125 million

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Problems Identified

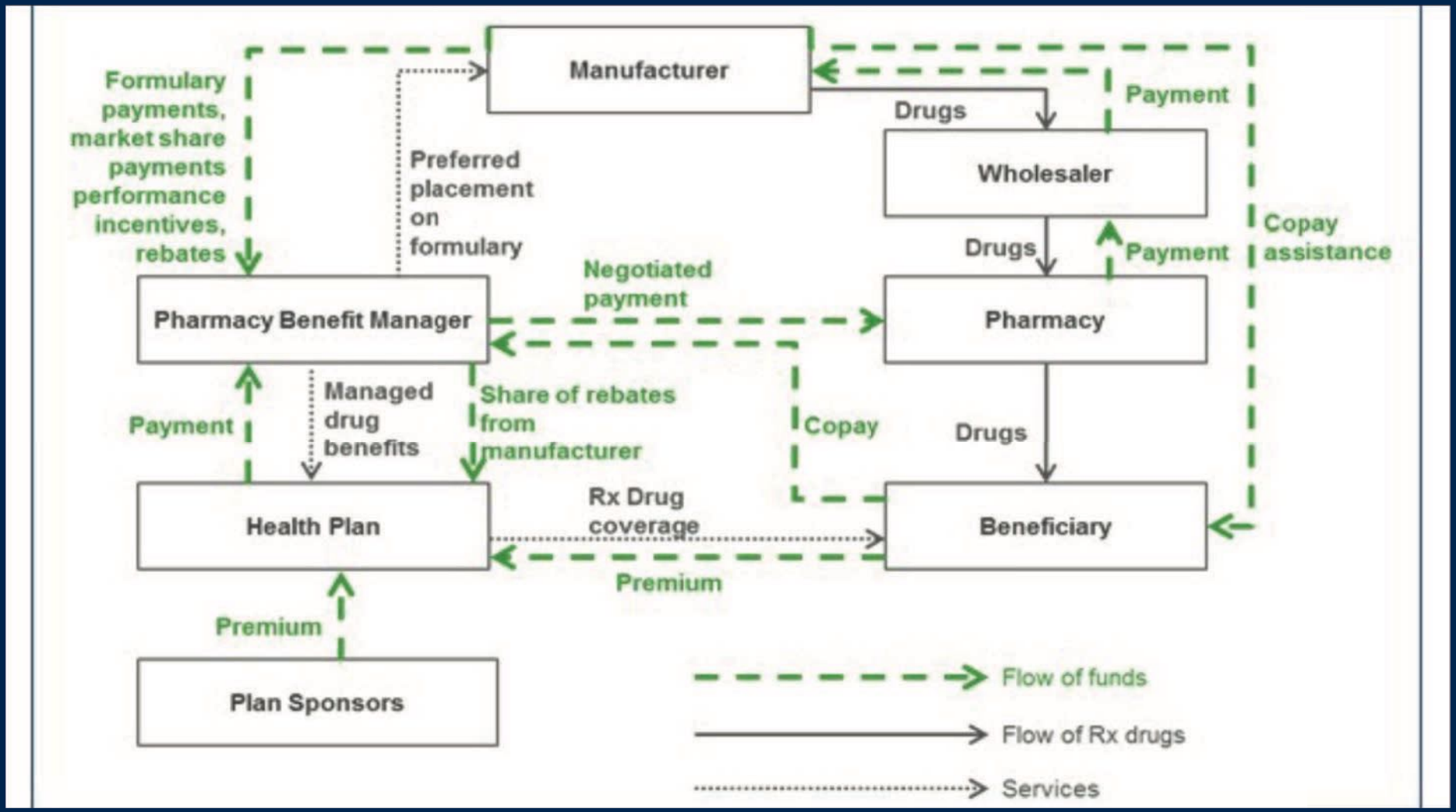
- Pharmaceutical manufacturing Research and Development costs lack transparency
- The operation and profit of the Pharmacy Benefit Managers and Drug Wholesalers lack transparency
- FDA's responsibility is to make sure a drug is safe and clinically better than doing nothing, not to control drug costs
- Longitudinal efficacy studies are needed for Spinraza, Zolgensma and functional motor exams

Current Policy Incentives

- Eliminate or reduce pharmaceutical manufacturers exclusive market timeframes
  - 1983 Orphan Drug Act – 7 years of market exclusivity
  - 1997 Food and Drug Administration Modernization Act – 6 months of market exclusivity
  - 2002 Best Pharmaceuticals for Children Act – 6 months of market exclusivity
  - 2015 Orphan Product Extensions Now Act – 6 months of market exclusivity

Kesselhein et al., (2017)

Flow of Money Through Pharmaceutical System



Policy Restructuring Recommendations

- Restructure pharmaceutical funding incentives to directly subsidize research
- Offer tax breaks for research and development of orphan drugs
- Organizational support for neurology clinicians to have time away from clinical duties
- Continue longitudinal efficacy studies of Spinraza
- Global policies and regulations of ultra-orphan drugs is a red tape quagmire

DNP Implications

- Pediatric Neurology DNPs
  - Can identify political obstacles to policy legislation and implementation and as content experts for legislators should provide expert testimony
  - Should be involved on professional organizational committees crafting of SMA guidelines, and position statements for collaboration with insurance payers and pharmaceutical manufacturers
  - Should be involved in the outcomes studies and determining sustainability of Spinraza
  - Can evaluate and influence the global health polices of Spinraza

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Natural History

Hope in a bottle

Realizing a dream

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