

Private Health Insurance
&
Rare Diseases

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Private Health Insurance & Rare Diseases Overview

- Introductory comments
- What is the current situation?
Michael Russo, Partner, The Bruckner Group
- What can we do about it?
Case Study: Hemophilia
Laurie Kelley, President, LA Kelley Communications
- Questions

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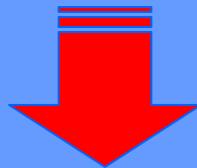
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Rare Disease Designation Impact on Private Insurers Decision-Making

Definitions	Designation	U.S. Prevalence
Federal Government	Orphan	< 200,000
Private Insurance		
Medical	Rare	< 150,000
Pharmaceutical	Rare	< 30,000



Even though “rare” is somewhat defined, there is
no separate set of coverage rules

Private Insurance Coverage of Rare Disease Treatment The Past

Generally,

- Rare and critical care diseases were off the radar screen for cost controls
- The reimbursement bar was lowered considerably
 - Less demand for proof of efficacy and need
- Only the most expensive and risky treatments were questioned
- Use of specialized and comprehensive care providers unrestricted

Private Insurance Coverage of Rare Disease Treatment

Why Have Things Changed?

Growing difference between what we are willing to spend and what we need

Small population and critical care patients no longer off limits

Private payer industry consolidation

Rise of outcomes based access

- Message from payers to manufacturers: “Prove it or we won’t pay for it.”
- Can be difficult to prove efficacy or rare disorders

Rare Disease Designation Impact on Private Insurers Decision-Making

That was then:

“Well, it’s just a few people. Let’s not concentrate on them.”

This is now:

“Well, it’s just a few people. It should be easy to target their high-costs.”

Private Insurance Coverage of Rare Disease Treatment What Are We Facing Now?

Overall, these changes have resulted in:

1. De-emphasis of the individual's needs
2. Attempts at applying cost-control programs to rare diseases that worked in other areas
3. Loss of patient choice over care providers and therapeutic regimens

Private Insurance Coverage of Rare Disease Treatment What Are We Facing Now?

These changes have had little effect thus far on:

1. VERY rare disorders
2. Disorders without a pharmaceutical standard of care
3. The availability of genetic testing

Private Insurance Coverage of Rare Disease Treatment What Are We Facing Now?

Rare diseases that the private insurance industry is targeting include:

Acromegaly

Alpha-one Antitrypsin Deficiency

ADA-deficiency

CIDP

Crohn's Disease

Fabry's Disease

Gaucher's Disease

Growth hormone deficiency

Hemophilia

ITP

Multiple Sclerosis

PID

Pulmonary Arterial Hypertension

Tyrosinemia

Private Insurance Coverage of Rare Disease Treatment What Are We Facing Now?

In many ways what they are really targeting is the use of specialty pharmaceuticals. These drugs include:

Adagen	Growth hormone
Antihemophilic factor VIII	IGIV
Antihemophilic factor IX	Prolastin
Aralast	Orfadin
Avonex	Rebif
Cerezyme	Remodulin
Enbrel	Tracleer
Fabrazyme	Ventavis
Flolan	Zemaira

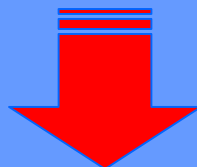
Private Insurance Coverage of Rare Disease Treatment Disease Management Programs

Positive side:

- Help patients to stabilize their chronic conditions through education, training, compliance reminders, and 24-hour nurse support.

Negative side:

- Interfere with individualized care and the patient-physician relationship
- Patients are matched to a program using a population-based analysis
- Patients are assessed by nurses and pharmacists
- Routine checks on patients can become invasive – more than once a week



Regardless of what is said to promote these programs,
their ultimate goal is to save money for plans and employers

Private Insurance Coverage of Rare Disease Treatment What Are We Facing Now?

When insurance programs cross the line:
Examples of cost-cutting efforts tracked by The Bruckner Group

Hemophilia

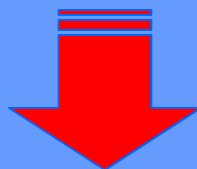
Challenges to utilization, loss of integrated care

CIDP

Challenges to IGIV use as off-label; prescribed regimen

Lysosomal Storage

Challenges to prescribed regimen



**Cost-cutting programs must not interfere with
physician-patient choice or the individual's standard of care**

What can we do?

Lessons from two years of engaging private insurance cost-cutting

Case Study: Hemophilia

- Inherited bleeding disorder
- Affects 1/10,000 male births
- Approximately 17,000 in US
- Untreated bleeding: crippling, pain, sometimes death
- Before treatments, life expectancy was about 11 years
- Treated with infusions of clotting factor
- Factor is one of the most expensive drugs

Case Study Hemophilia



What the Hemophilia Community Experienced

HIV in the blood supply in the 1980s

- Devastating consequences of “containing costs”
- Loss of 10,000 lives

Recombinants developed

- New products employing safer production methods
- Recombinant products: G1,2,3

Emergence of Homecare

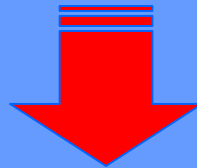
- Allows for more normal lives
- Avoids extreme numbers of ER visits

Creation of “Hemophilia, Inc.”

- Huge profits led to rapid growth of homecare
- Many companies only for hemophilia homecare
- Homecare owned by PWH, employing PWH
- Ownership changed hands many times
- New, “safer” products= higher prices

Exceptional Outcomes Driven by Specialized Care

- Home therapy saves money, promotes health
- Nursing services for babies, surgical patients
- Prophylaxis saves money over time, promotes health
- Choice of product means more brands to choose from
- Choice of provider allows for negotiation
- HTC's provide unique and expert care



With proper treatment, children with hemophilia can live typical lives.
Did this contribute to hemophilia becoming a test target for cost-cutting?

Targeting an Expensive Specialty Drug Current Threats to Hemophilia Care

Cost-cutting measures to reduce utilization and reduce price

- Single provider = no choice
- Single product = dependency, no choice, delays
- Prior authorization
- Preference for specific brands based on cost and contracting

Discourages further therapy advancements

Current Threats to Hemophilia Care
Are companies considering an exit?

Bayer divests plasma division
American Red Cross exits hemophilia industry

Current Threats to Hemophilia Care Emergence of PBMs

Pharmacy Benefit Managers (PBMs)

Massive resources

Cut out services/lack of expertise

Threat to treatment standards?

Undercut prices

Conflicts of interest?

Future of HTC's?

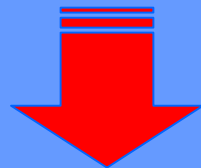
Current Threats to Hemophilia Care Distribution

Massive consolidation of distribution business

- HHS buys HRA
- Accredo buys HHS
- Medco buys Accredo
- Express Scripts buys CuraScript and Priority
- Caremark buys Advance PCS

Hemophilia Community Response

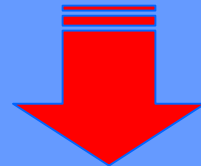
- Unprepared and not vigilant
- Emotional, “looking back,” entitlement
- Split: advocacy groups voice different priorities



Individual patients and families were unaware and unable to respond

Hemophilia Community BGI and LAKC Plan and Initiate a Strategy

- Publication and speaking campaign to educate
- Understand and engage the payers' position
- Make the individual central: understand own situation and priorities
- Create action-oriented planning tools
- Admit that costs are too high and targeted controls may be beneficial



Overwhelming response from families and support across the industry

Cost-cutting programs *must not* interfere with physician-patient choice or the individual's standard of care

