Managing Drugs for Rare Genetic Diseases: Trends and Insights

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ABSTRACT

Managed care organizations generally pay for expensive drugs that treat rare genetic diseases because few patients have these conditions, the conditions are often life threatening, and the benefit design mandates coverage. In most cases, the cost-control measures and management restrictions applied to many other specialty pharmaceuticals do not make sense for the orphan drugs used to treat extremely rare genetic conditions because treatment alternatives usually are lacking for rare conditions (e.g., Gaucher’s disease) and because the per member per month costs for truly rare conditions are low. The increasing number of biologic and injectable therapies for more common conditions, however, is prompting managed care decision makers to manage these new therapies more actively. There is a potential that treatments for rare disorders will be swept up in this broad-based response. This article provides definitions, background, and stakeholder perspectives on this topic and describes recent trends and challenges in health system management of exceedingly rare genetic diseases. The author suggests that current protocols are appropriate for managing rare and ultra-rare diseases, and that applying more active management practices to less rare diseases is neither efficient nor productive; in fact, it may be counterproductive.

INTRODUCTION

Most managed care organizations routinely cover specialty pharmaceuticals for their approved indications, a policy that has been welcome (and, perhaps, life-saving) news to sufferers of the least common but most deadly of medical conditions. As stated by the National Organization of Rare Diseases (NORD): “Even though some orphan drugs may be priced higher than commonly used drugs, insurers … recognize that the small number of people using the product in any MCO will usually represent a small overall exposure to the health insurance company” (NORD 2004). With most health systems having internalized that rationale, today’s patients who have rare genetic diseases maintain full access to these highly specialized therapies (Milne 2002, U.S. DHHS 2001).

Learning objectives

Upon completion of this activity, participants will be able to:

• Offer several definitions of a “rare” disease.
• Explain perspectives on rare disease management held by many positions within the industry, including that of the manufacturer, an actuary, a physician, and the director of a managed care organization.
• Describe some existing and developing issues and difficult industry decisions related to management of patients with rare genetic diseases.
• Cite comparisons of management of therapies for rare genetic diseases and for more prevalent diseases.
• Formulate an opinion about whether reform of the current management protocols for rare diseases is required.

Cost concerns with specialty pharmaceuticals

The heightened, generalized concern of MCOs regarding the high costs of biologic andinjectable therapies is understandable. Currently, biological agents are the fastest grow-
ing category of drug expenditures. Moreover, the pipeline is brimming with these expensive and novel therapies (ExpressScripts 2002, Hoffman 2004, Johnson 2004, Rodgers 2003). Payers as well as insurers have valid concerns that certain biologic agents are likely to develop "mission creep," with their clinical use extending well beyond their original indications (Kocs 2003, Cuzzolin 2003, ASCO 2003).

The rising cost of specialty pharmaceuticals troubles insurers, employers, and health systems alike. Anecdotes abound about specialty drug costs for a single patient "breaking the bank" of small state Medicaid plans or private health plans. Many employers wonder if they can pay higher premiums to cover high-tech drugs. Not surprisingly, these cost concerns have translated into various internal management initiatives such as copayments or coinsurance. Yet, should all specialty therapies — including those for the most rare diseases — be aggressively managed to ensure proper use? When does such aggressive management make sense? What are the costs today, and what will they be tomorrow? In short, how can MCOs develop medical or pharmacy policies that continue coverage for ultra-rare genetic diseases while introducing appropriate and necessary controls on biologic and injectable agents used for more common conditions?

This article addresses these questions based on interactions with clinicians, pharmacists, researchers, and administrators in MCOs, insurance companies, employer groups, pharmacy benefit managers (PBMs), specialty pharmacy providers (SPPs), and research-based pharmaceutical and biotech companies. A special debt is due for ideas shared recently by members of the National Steering Committee on Rare Genetic Diseases, a meeting convened in March 2004 by the Zitter Group, under a grant from Genzyme Therapeutics.

### HOW RARE IS RARE, AND WHY IT MATTERS

When is a disease rare? For a primary care physician, it might be the condition seen once in a lifetime (Phillips 2004). The World Health Organization states that a rare disorder is one that affects, at most, 0.65 out of every 1,000 individuals; the European Union sets the rate at 5 out of 10,000; the Australians, Japanese, and Americans set countrywide prevalences of 2,000, 50,000, and 200,000, respectively, for a given rare disease (Lavandeira 2002).

In the United States, the definition of rare as less than 200,000, or about 1 per 1,000, is derived from the Orphan Drug Act of 1983. Before passage of the act, many patients with rare diseases did not have access to effective medications (Rados 2003, Schulman 1997).

The 7-year marketing exclusivity and grants provided under the Act prompted development of about 250 orphan drugs in the last 2 decades (Rados 2003, FDA 2004). For MCOs, the 200,000 demarcation provides an arbitrary but useful distinction between truly widespread conditions amenable to biologic therapy — e.g., severe allergic asthma, hepatitis C infections, and rheumatoid arthritis (RA) — and less common disorders. Table 1 shows how these less common diseases also can be subdivided into meaningful categories, ranging from the “near rare” multiple sclerosis (MS) and Crohn’s disease, to the “more-common rare” sickle cell ane-

### TABLE 1 Diseases treated with biologic or injectable agents

<table>
<thead>
<tr>
<th>Disease</th>
<th>(U.S.) Prevalence</th>
</tr>
</thead>
<tbody>
<tr>
<td>Psoriasis</td>
<td>5.8–7.5 million</td>
</tr>
<tr>
<td>Hepatitis C infections</td>
<td>3.9 million</td>
</tr>
<tr>
<td>Rheumatoid arthritis</td>
<td>2.1 million</td>
</tr>
<tr>
<td>Severe allergic asthma</td>
<td>500,000</td>
</tr>
<tr>
<td>Crohn’s disease</td>
<td>380,000–480,000</td>
</tr>
<tr>
<td>Multiple sclerosis</td>
<td>400,000</td>
</tr>
<tr>
<td><strong>“Rare” is &lt;200,000 as defined by Orphan Drug Act of 1983</strong></td>
<td></td>
</tr>
<tr>
<td>Alpha-1 antitrypsin deficiency</td>
<td>100,000</td>
</tr>
<tr>
<td>Sickle cell anemia</td>
<td>91,000</td>
</tr>
<tr>
<td>Multiple myeloma</td>
<td>63,000</td>
</tr>
<tr>
<td>Cystic fibrosis</td>
<td>30,000</td>
</tr>
<tr>
<td>Gastrointestinal stromal tumor</td>
<td>15,000</td>
</tr>
<tr>
<td><strong>“Ultra-rare” is generally defined as &lt;10,000</strong></td>
<td></td>
</tr>
<tr>
<td>Fabry’s disease</td>
<td>5,000</td>
</tr>
<tr>
<td>Gaucher’s disease</td>
<td>2,500</td>
</tr>
<tr>
<td>Tyrosinemia type 1</td>
<td>2,500</td>
</tr>
<tr>
<td>Mucopolysaccharidosis (MPS 1)</td>
<td>200</td>
</tr>
</tbody>
</table>

**Sources:** National Gaucher Foundation; National Heart, Lung, and Blood Institute; Calkins 1986; National Multiple Sclerosis Society; National Institute of Arthritis and Musculoskeletal and Skin Diseases; CDC 1998; Alpha One Foundation; Genzyme Corp.; and Rados 2003.
Drawing these finer, prevalence-based distinctions among diseases is useful because the prevalence of each disease affects various cost considerations. From the manufacturer’s perspective, for example, the development and commercialization costs are proportionally higher for the rarest of the rare diseases. This directly increases drug unit costs (see “The Manufacturer’s Perspective” at right). Within each MCO’s membership, the disease prevalence also determines the per member per month (PMPM) cost of treatment (see “The Actuary’s Perspective” on page 58).

This PMPM perspective on costs for rare diseases is important relative to the use of high-cost biological agents. The majority of biological agents are orphan drugs (Figure 1), an association encouraged by specific incentives within the Orphan Drug Act’s legislation (Haffner 2002a) and likely to be strengthened with further elucidation of the human genome (Haffner 2002b). These biological and injectable agents typically cost from $10,000 to $250,000 per year; national costs for these specialty drugs have increased 20 percent annually and are expected to reach $26 billion by 2006 (Tercero 2002, Hesselgrave 2003, Manag Care 2004). Injectables are used by only 0.2 percent of the population, while constituting about 8 percent of total medical costs ($140 million for each 1 million insured lives); injectable spending is projected to increase 20 percent to 40 percent annually (Johnson 2004).

Yet relatively little of the estimated $22 billion spent on biological and injectable agents goes to treating ultra-rare genetic diseases. In fact, over 75 percent of specialty drug spending goes to just five categories: oncology,
The actuary’s perspective

In typical health plans, only 0.1 percent (1 in 1,000) has claims that are over $250,000 to $300,000. The most expensive claims, such as for a patient with Gaucher’s disease — where the prevalence is about 1 in 100,000 — are typically a sliver of overall managed care system costs. Per member per month cost for a comprehensive commercial (non-Medicare) managed care member today is about $250 to $350, of which about 15 percent or $40 to $50 goes for drugs. About $5 or $10 of PMPM costs are for biologicals/specialty drugs, which may flow through the prescription drug or medical benefit. Although that may seem like a small fraction of the total cost, even a difference of $5 PMPM is not negligible for some employers.

“Medical management techniques as employed by managed care — for example, routine concurrent review or case management — usually have a limited impact for those few, very expensive cases involving patients with very rare diseases. However, most managed care payers will continue to apply such techniques to ensure that all reimbursed services are medically necessary and are covered under the policy. Adverse selection or random fluctuations can cause a concentration of orphan drug patients that could be financially catastrophic to plans, especially smaller health plans or self-insured employers. As the number of orphan and ultra-orphan drugs increases, the need for a national reinsurance program to spread the risk will increase.”

— Bruce Pyenson, FSA, MAAA, principal and consulting actuary, Milliman Inc., New York
Beyond the compelling life-or-death aspect of the situation, however, the inappropriateness of these rationing devices also can be defined in economic terms. The economic rationale for copayments and coinsurance is to discourage patients from consuming medical care with a value that is less than the cost to the insurer. Nevertheless, the efficacy of these economic tools depends on an elastic demand curve with choices of many alternative treatments. With drugs for rare diseases, where the demand is inelastic and cost of therapy is extremely high, there are few reasons to support patient cost-sharing once the health insurer has authorized reimbursement for the drug.

Furthermore, while copayments can help avert questionable use (i.e., “Well let’s treat just in case…”) in many more common diseases, the decision to treat an ultra-rare genetic disorder is usually clearer. The therapies are targeted to physiologic pathways that tend to be highly disease-specific rather than multifactorial (Grabowski 2004). Physician specialists generally know these ultra-rare diseases when they see them and are able to choose the proper therapy with little hesitation (see “The Physician’s Perspective” at right). Finally, since the rare condition is so narrowly defined in physiological and genetic terms, there is limited potential for use of the drug to expand beyond the original single indication.

Thus, while MCOs are highly motivated to ensure that biologic and injectable agents are used only for approved conditions, this well-founded desire to discourage inappropriate or questionable use of biologics needs to be instituted carefully — and applied selectively. The traditional techniques usually do not make economic sense in managing the products for the truly rare genetic diseases. This lack of fiscal rationale, combined with the likely lack of in-house clinical expertise on the disease, suggests that a quick referral to a COE (rather than any attempt to monitor with internal guidelines) may be the most efficient approach (see “The Managed Care Director’s Perspective” on page 63).

CHALLENGES AND STRATEGIES: MANAGING RARE GENETIC DISEASES

As more orphan products for rare diseases are introduced, the challenge of maintaining access to these products will remain a priority for MCOs, and decision makers will need to confront a range of related business challenges, including:

- Lack of evidence on drug dosing, safety, and outcomes for ultra-rare disease. Because the clinical trials for orphan drugs are necessarily small, providers and specialists will need to work alongside manufacturers to build and apply national registry data.
- Clarity and consistency in billing

The physician’s perspective

As doorkeeper to expensive therapies for most rare diseases, the specialist physician often sets de facto policy for managed care organizations, either as part of the MCO’s network or within a university-based Center of Excellence. For example, one new therapy now being handled by such specialists is agalsidase beta for Fabry’s disease, a serious metabolic genetic disorder that affects 1 in every 40,000 males. As part of the accelerated review and approval of its recombinant enzyme in April 2003, the manufacturer agreed to work with disease specialists to monitor long-term outcomes and maintain a patient registry. The decision to treat a patient with Fabry’s disease depends on the genotype and the presence of progressive disease (Figure 2, page 60); not all patients require immediate treatment, and this difficult decision is a prime example of why the experienced specialist who has seen several of these extremely rare patients is best suited to manage therapy.

Fabry’s disease, Gaucher’s disease, and mucopolysaccharidosis (MPS 1) are just three types of lysosomal storage diseases (LSDs) that specialist pediatricians can now treat with specific replacement enzymes. Similar orphan drugs eventually may be available for at least 35 other rare lipid disorders (e.g., MPS II, IV, and VI; Niemann-Pick B and C; Pompe disease; and the neuropathic LSDs). Considering that the LSDs represent just one fertile area of ongoing research, the role of specialist physicians as “MCO guides” to the expanding universe of rare disorders becomes clear (Wilcox 2004). While primary care physicians need not learn how to manage patients with rare genetic diseases, they should know how to recognize referral triggers. In many cases, generalists also can work with these exceptional patients to educate each other about the rare disease. Selective education of family physicians and internists is key in MCOs.

“We have tried over the years to develop Centers of Excellence in lysosomal diseases. So, for example, we’ve set up a hub in Cincinnati, and we have a series of spokes reaching out to local physicians. This works well, because I can send the patients back to their own doctors and [I can then] collaborate with these local physicians. It forces the stream of education. The local doctors enjoy seeing the patients get better. And I don’t need to see these patients every time they have a minor illness.” —Gregory A. Grabowski, MD, professor and director of human genetics, Cincinnati Children’s Hospital Research Foundation
administration. With different specialists taking the lead in specific orphan diseases (e.g., pediatricians instead of oncologists), MCOs can no longer assume that providers know the fine points of coding for use of expensive orphan products. MCOs need to find a way for providers to capture costs accurately while giving them a safe “crosswalk” in coding to allow prompt payment. One increasingly popular approach is to have these drugs delivered to the provider by specialty pharmacies, simultaneously removing the cost and coding issues from the physician’s office and allowing for cost-effective purchasing by the plan.

• Near-term paucity of competition and market price control. The small patient population makes development of multiple products for rare diseases less likely than is seen with drugs for other chronic conditions; the ultra-orphan markets are much smaller than others (e.g., MS and RA) for which more competing biologic products have been developed.

• A tipping point ahead? Currently, because ultra-rare genetic diseases occur so infrequently, the typical MCO’s PMPM cost is still low, and decisions on patient access, physician barriers, and internal treatment guidelines need to be tempered with this actuarial perspective. At some future point, however — especially as pharmacogenomic research continues to subdivide diseases into microdiseases for which microtreatments will be developed — MCOs and society will face ethical decisions about access to effective treatments. These difficult questions about reimbursement and rationing of health care will need to be reassessed continually in the decades ahead.

To face the immediate challenges, every MCO can generate strategies based on its structure, history, clinical expertise, and policies. Several new MCO policies may be required. For example, as new tests and treatments for rare diseases become more common, managing all patients with rare diseases as special cases might soon become untenable. At some point, an MCO might need to acknowledge and evaluate this trend and inculcate an institutional culture of continuous learning and practical policy-making aimed at handling ultra-rare diseases. Partnerships with COEs or with manufacturers can contribute to this internal learning process.

Before approving reimbursement, medical and pharmacy directors can gather highly focused information about each new and impending orphan product, soliciting information from network specialists, clinical investigators, COEs, and drug-maker representatives and consultants. A product dossier from the manufacturer can provide up-to-date guid-

\[\text{FIGURE 2} \quad \text{Fabry’s disease progression: When should therapy begin?}\]

In Fabry’s disease, the early damage caused by lipid deposition in small vessels usually remains unnoticed until major organs are affected later in life. A primary challenge for specialists in lysosomal disorders is to determine when to initiate therapy with the replacement enzyme in the course of this progression.

\[\text{SOURCE: GRABOWSKI 2004}\]
The managed care director’s perspective

An intelligent managed care organization benefit design would start with obligatory coverage of the label indication. It then would make critical distinctions between specialty injectable products for more common conditions and for truly rare conditions in deciding when to employ pharmacy or medical benefit design features (e.g., tiers, prior authorization, case management). A plan might apply a copayment for an injectable asthma drug, for example, to allow the member to share costs and to have a role in the decision to use the product. Likewise, in a category of drugs for multiple sclerosis, where there are several products, there may be one or two preferred drugs with fixed copayments and one or two drugs with co-insurances of up to 25 percent. This type of arrangement can drive utilization to the preferred products. Similarly, a single-agent category can be created for rare genetic diseases such as Gaucher’s, where the product is exempt from copayments and from prior authorization requirements.

“One quick solution to injectable costs is to throw them over the fence into pharmacy, and manage it as a pharmaceutical product. That’s tempting but I didn’t want to just play catch with myself, since I manage both medical and pharmacy subteams. So, last year, we redesigned our medical benefit to include a specialty pharmacy injectable benefit category. This medical benefit category looked, acted, and felt like a pharmaceutical benefit with copayments and two or three tiers — tier 1, for what we might call routine injectables for acute conditions; tier 2, for preferred specialty products; and tier 3 is being developed for products in areas like rheumatoid arthritis and psoriasis, where there are or will soon be choices of multiple specialty products.

“One way we deal with the growing cost burden to the employer is with a product design that shifts some costs to the employee. But care is needed, as there’s a relationship between the amount of member out-of-pocket spending and the potential to create a barrier to care. You have to titrate it carefully.” — Gary Owens, MD, medical director, Independence Blue Cross

MANAGING DRUGS FOR RARE DISEASES

Drugs are available for the treatment of rare diseases. The number of rare genetic diseases continues to grow, and many MCOs are struggling to manage the cost of these drugs. One solution is to redesign managed care plans to include specialty pharmacy injectable benefit categories. This can help control costs while still allowing patients to access necessary treatments.

Academic and non-academic inpatient hospital costs have increased by 8.8 percent across the United States, according to the Agency for Healthcare Research and Quality. However, inpatient costs have increased by only 1.6 percent for cardiac patients, who may account for the significant increase in inpatient hospital costs. This is a concern, as patients need to be able to access high-quality care. Providers need to consider how to manage costs while still offering quality care to patients.

Inpatient care continues to account for the majority of healthcare costs, but outpatient and ambulatory care costs are also increasing. The percentage of outpatient and ambulatory care costs has increased by 6.6 percent, according to the Agency for Healthcare Research and Quality. This is a concern, as providers need to consider how to manage costs while still offering quality care to patients.

The percentage of prescription drug costs accounted for by out-of-pocket spending has increased by 2.3 percent, according to the Agency for Healthcare Research and Quality. This is a concern, as patients need to be able to access necessary treatments. Providers need to consider how to manage costs while still offering quality care to patients.

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MANAGING DRUGS FOR RARE DISEASES

MD; David Meeker, MD; Bruce Pyenson, FSA, MAAA; and Gary Owens, MD.

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CE/CME quiz and answer sheet can be found on pages 66–67.
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CE/CME QUESTIONS

1. According to the author, which of the following is NOT a reason to discourage the use of aggressive cost techniques for truly rare diseases?

   a. What seems like a reasonable coinsurance percentage can quickly become unaffordable due to the expensive nature of rare disease therapies.

   b. Spending company time on such an aggressive endeavor is not practical, given the small number of people these diseases normally affect.

   c. Allowing the affordability of copayments/coinsurance to become a barrier to care for a life-threatening disease is ethically inappropriate.

   d. The elastic demand curve that justifies the use of copayments/coinsurance does not exist when dealing with diseases that have very few treatment options.

2. According to the author, what drives the cost of drugs for rare diseases so high?

   a. The burden of generating revenue for the pharmaceutical company and sharing the expense of developing the drug is dispersed over a small patient population.

   b. The lack of treatment options means there is little competition to drive down cost.

   c. The accumulation of unusually high “hidden” expenses such as patient advocacy, physician training, inventory management, etc.

   d. All the above.

3. The U.S. definition for rare diseases was defined by the ______________ and set at a prevalence of ______________ Americans.


   d. Biologics Control Act; 1:100.
4. Of the estimated $22 billion spent on biological and injectable agents, 75 percent is spent on just five categories: oncology, renal disease, transplant medications, HIV/AIDS, and __________._
   b. Central nervous system-related indications.
   c. Hemophilia.
   d. Autoimmune diseases.

5. According to the author, what should be done to overcome the lack of evidence on drug dosing, safety, and outcomes for ultra-rare diseases?
   a. Providers and specialists should partner with manufacturers to build and apply a national registry system.
   b. Clinical investigators should increase their trial recruitment efforts to fill more compelling study-sample sizes.
   c. Investigators should broaden the recruitment criteria to allow genetically susceptible people to become eligible.
   d. Researchers should use statistical methods to extrapolate as much information as possible from the data that is generated from current sample sizes.

6. According to the actuary’s estimation, what percentage of the per member per month cost is allocated for drugs?
   a. 10 percent.
   b. 15 percent.
   c. 20 percent.
   d. 25 percent.

7. The physician used Fabry’s disease in this article as an example of how specialists can:
   a. Use their position as doorkeepers to expensive therapies to set de facto policy for MCOs.
   b. Manage just about any disease with the proper training.
   c. Have his/her hands tied by unreasonable decisions made by managed care executives.
   d. All the above.

8. According to the managed care director’s perspective, why is it important for MCOs to make critical distinctions between specialty injectable products when used off-label for more common and rare conditions?
   a. To have a more complete understanding of the patient care being provided.
   b. To drive utilization toward preferred products when multiple agents are being used for more common disease states.
   c. So single-agent categories can be created for the truly rare diseases, and specialty products can be exempted from copayment and prior authorization requirements.
   d. Both “b” and “c.”

I have participated in the program Managing Drugs for Rare Genetic Diseases: Trends and Insights. I have reviewed all the materials and answered the CE/CME questions. I understand that a certificate for 0.1 CEU credits toward continuing pharmacy education or 1.0 credit of Category 1 PRA (Physician’s Recognition Award) will be mailed to me upon receipt of this form, provided I receive a passing score of 70 percent or higher. I also understand that no fee is required for me to receive credit. Credit can be awarded for submissions received through Jan. 12, 2008.

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Degree(s): _______________________
Title/Position: ____________________
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Business address:
Business telephone: _____________
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E-mail: __________________________
Signature: _______________________

Post-test answer sheet
Circle the letter of the correct answer.
1. a b c d
2. a b c d
3. a b c d
4. a b c d
5. a b c d
6. a b c d
7. a b c d
8. a b c d

Evaluation
Key:
1 = Strongly agree
2 = Agree
3 = Neutral
4 = Disagree
5 = Strongly disagree

Please circle the number of your response.
1. Overall, the materials were clear and to the point.
   1 2 3 4 5
2. The difficulty level of the material was appropriate.
   1 2 3 4 5
3. The program content was consistent with its description.
   1 2 3 4 5
4. The content had substance.
   1 2 3 4 5
5. The program content was relevant to my work.
   1 2 3 4 5
6. The instructional material addressed important practice problems.
   1 2 3 4 5
7. The printed material is likely to be used as a future reference.
   1 2 3 4 5
8. My learning objectives for this program were addressed.
   1 2 3 4 5
9. I gained new insight relevant to my work.
   1 2 3 4 5
10. The program was well organized.
    1 2 3 4 5