

The case of rare disease drugs in the context of pricing bodies: lessons from Brazil, Canada, and the UK, implications for the US



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Introduction

Health technology assessment (HTA) bodies are becoming more common in developed and developing countries. Although they share similar goals, their development and ultimate structure differ considerably. This analysis compares the effects of three different HTA bodies on drug prices. Canada's HTA body was chosen for its unique structure of providing national coverage decisions for a province-based healthcare system, and for its longevity, having been first introduced in 1990 as the Canadian Coordinating Office for Health Technology Assessment (CCOHTA), turning into The Canadian Agency for Drugs and Technologies in Health (CADTH) in 2006¹. In contrast, Brazil's Coordenação Geral de Avaliação de Tecnologias em Saúde (CGATS), the Health Technology Assessment General Coordination, was chosen for its relatively recent institution as an HTA body². The National Institutes for Health and Clinical Excellence (NICE) was chosen as it is one of the most widely recognizable HTA bodies, having been developed in 1999 as an advisor to the single national provider of healthcare in the UK, the National Health Service³. In contrast to these three countries, the US does not have a coordinated HTA body, although there is some indication of HTA occurring within private health insurance plans and the US's Agency for Healthcare Research and Quality (AHRQ). Additionally, the recent development of the Federal Coordinating Council for Comparative Effectiveness Research suggests that there may be some interest in moving towards an evaluation of health technologies as they become available. Thus, the objective of this poster is to explore the pharmaceutical price implication for rare disease products in countries which have developed technology assessment and pricing processes with a look toward the potential implications for the United States.

Methods

Prices were obtained for the multiple sclerosis drugs Avonex[®] (interferon beta-1a) and Tysabri[®] (natalizumab) in Brazil, the UK, and the US. Prices were also obtained for the Gaucher's Disease drugs Cerezyme[®] (imiglucerase) and Zavesca[®] (miglustat) in Canada, the UK and the US. These diseases were chosen for their generally high prices and availability of data in countries of interest. Sources for this information are indicated below in Table 1.

Table 1: Dosing and sources of price data for the multiple sclerosis and Gaucher's Disease drugs used in this analysis

Drug	Dose	Country	Cost of yearly therapy (\$USD)*	Source
Avonex [®] (Multiple sclerosis)	30 µg once weekly	Brazil UK US	40 610.13 13 944.56 37 544.00	KairosWeb MIMS UK ProspectRX
Cerezyme [®] (Gaucher's Disease)	60 U/kg every other week	Canada UK US	499 108.36 342 631.77 285 120.00	PMPRB BNF ProspectRX
Tysabri [®] (Multiple sclerosis)	300 mg once every four weeks	Brazil UK US	31 260.04 24 093.82 42 787.55	Genzyme MIMS UK ProspectRX
Zavesca [®] (Gaucher's Disease)	100 mg three times daily	Canada UK US	499 108.36 160 949.09 84 169.98	PMPRB MIMS UK ProspectRX

µg: micrograms. U: units. kg: kilograms. mg: milligrams. MIMS: Monthly Index of Medical Specialties. PMPRB: Patented Medicines Price Review Board. BNF: British National Formulary. *Exchange rate determined from WSJ online data.

These prices were then used in conjunction with the dose indicated above in Table 1 to determine yearly treatment costs. It was assumed that there are 365.24 days in a year to account for leap years. In the case of Cerezyme[®], which is an infused product with dosing dependent on patient weight, it was assumed that the average patient weighs 50 kg. This assumption is based on cost effectiveness models of infused products, such as Fabrazyme[®] and Epogen[®], which use 50 kg as a patient's weight, despite this number being considerably lower than the weight of many adults. Annual therapy costs were converted to USD using exchange rates published online at the Wall Street Journal⁴. Price differentials were represented by calculating the newer product as a percentage of the older product.

Results

Analyses indicated that Tysabri[®] costs considerably more than Avonex[®] in the UK and US, while it costs less than Avonex[®] in Brazil. In contrast, Zavesca[®] is considerably less expensive than Cerezyme[®] in both Canada and the UK, but this price difference is not as large in the US. This can be seen in Tables 2 and 3 below.

Table 2: The price of Tysabri[®] as a percentage of the price of Avonex[®]

Country	Percentage
Brazil	77%
United Kingdom	114%
United States	173%

Table 3: The price of Zavesca[®] as a percentage of the price of Cerezyme[®]

Country	Percentage
Canada	25%
United Kingdom	25%
United States	56%

Discussion

One draw back of this examination is the limitations on data. Due to data limitations, Gaucher's Disease products were only explored in Canada, while only multiple sclerosis drugs were examined in Canada. This contributes to some difficulty in drawing conclusions from the results.

The difference in prices between the multiple sclerosis and Gaucher's Disease drugs might be accounted for by the novelty of the treatment or degree of technological advance. Tysabri[®] is the first monoclonal antibody treatment approved for multiple sclerosis indications, whereas Avonex[®] is an interferon, a class with multiple marketed products⁵. Thus, as a novel approach to treating multiple sclerosis, Tysabri[®] would command a price premium, as is apparent in the UK and US. That the price of Tysabri[®] remains lower than Avonex[®] in Brazil is an unexpected finding and the rationale behind this is unclear.

Similarly, Cerezyme[®] treats the underlying disease at a very fundamental level by reintroducing a missing enzyme, whereas Zavesca[®] simply decreases the amount of substrate the enzyme needs to act on. Additionally, Cerezyme[®] is the preferred treatment, with Zavesca[®] being used primarily in patients unable to tolerate Cerezyme[®]. Zavesca[®] was also developed later, limiting its ability to command a premium price. Interestingly, while the price of Zavesca[®] is lower than that of Cerezyme[®] in all of the countries examined, it is lower in those countries with HTA bodies. This suggests that development of cost effectiveness criteria as a condition of approval may apply downward pressure on the costs of new drugs, particularly in the case of those drugs which do not offer a significant new benefit to a disease area.

Additionally, price differences may be a reflection of differences in data requirements, methodologies, and economic modelling requirements amongst these HTA bodies. While CADTH and NICE have had explicit guidelines for HTA submissions for some time, Brazil's guidelines were only recently made available in 2009. Because Brazil is smaller and less developed, with limited infrastructure to collect data regarding health system utilization and patient populations, the data to develop assessments that are truly reflective of the country are limited.

The ability to use HTA recommendations to apply downward pressure on pharmaceutical and device products carries important implications for the US, which is currently the largest world market without an HTA body. For some time, the lack of HTA-associated rigors in the US caused many product developers to focus on this market, as it had the greatest potential for revenue. However, the development of evaluation bodies within health insurers and AHRQ suggests an imminent end to such strategies. While these bodies generally do not produce decisions that preclude patient access, the precedent suggests that such a situation may develop over time with continued HTA evolution⁷. Thus, the pricing implications of HTA body development, regardless of its ultimate structure, may play a crucial role in future market access.

Conclusions

It is clear that HTA bodies may be used to apply downward pressure on the prices of drugs, but this may be limited in the context of products which offer a strong therapeutic benefit or novel approach over existing therapies. The economic status of the nation may also contribute to applying downward pressure, although this result was obfuscated in this analysis. It is highly likely that differences in guidelines, methodologies, and evidence requirements have an impact. This result has important implications for the US, which may be moving toward a health economic environment.

References

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