Are acute therapies and curative drugs more affordable than chronic treatments in rare diseases?

An analysis of the top 10 most expensive drugs in the US compared with Germany

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INTRODUCTION

Recently there has been focus on acute treatments being 'the most expensive drugs in the world'. In 2019, we compared the costs of the most expensive gene/acute therapies and chronic treatments for rare diseases in the US on an annual and lifetime basis to contrast short- and long-term costs

- That research suggested that acute and curative drugs may be more affordable than chronic treatments over a patient's lifetime.
- This is an updated analysis to identify any changes since 2019

OBJECTIVES

1. To compare the annual and lifetime treatment costs of the 10 most expensive drugs in the US
2. To compare the costs from Objective 1, between the US and Germany

METHODS

Figure 1. Methods flow diagram

- A literature review of PubMed and grey databases was conducted. This included the following search terms: high cost, most expensive treatment, therapy, annual, lifetime
- The most expensive 25 drugs were assessed and prices and dosing data were identified and analysed to calculate annual treatment costs. Dosing assumptions were based on a product's SmPC, average weight or body surface area where applicable
- Age of onset and life-expectancy data were used to estimate lifetime treatment costs. The top 10 most expensive products on an annual treatment basis were selected based on US WAC price for the first year of treatment
- The annual and lifetime cost of the top 10 most expensive products between US and Germany was compared

RESULTS

- The top 10 most expensive drugs are a combination of acute therapies and chronic-use treatments, compared with 2019 where the top 10 were all acute therapies
- Of the top 10 most costly treatments, 3 acute treatments (Zolgensma, Luxterna, Folotyn) remain with the new addition of Spinraza since 2019 (Chart 1)
- Considering the lifetime costs of the top 10 products, based on US ex-factory price, the acute treatments are ranked #7-10 (Chart 2). Based on the German ex-factory price the most highly ranked acute treatment is #4 for Zolgensma
- Takhzyro, accrues the highest lifetime costs in both the US and Germany on a chronic basis.
- The average annual (first year) ex-factory price for the acute treatments remains higher than for the chronic treatments (Table 1). The average lifetime costs of the 6 drugs used chronically is far higher than the lifetime cost of the acute therapies with an average of $14.7m/€2.6m compared to $1.4m/€707k for acute treatments
- Furthermore, the annual and lifetime costs of the top 10 most expensive drugs has increased since 2019

Chart 1. Annual cost of the 10 most expensive drugs in the US compared to Germany (based on ex-factory price in EUR)

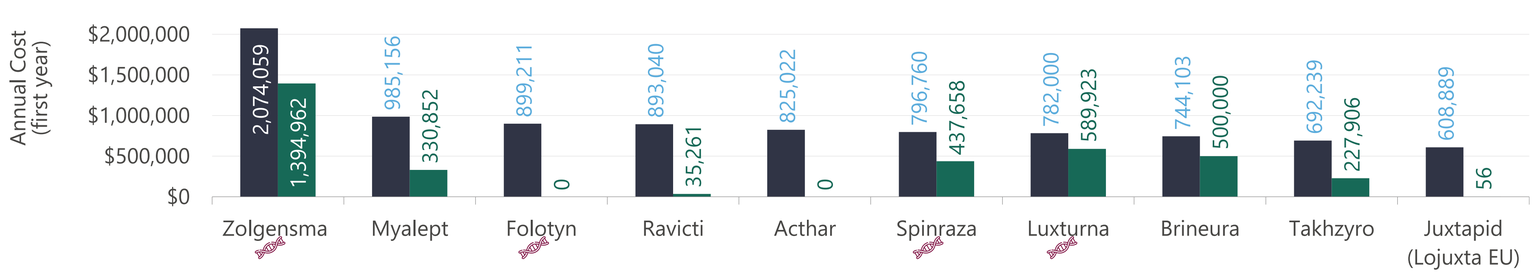

Chart 2. Lifetime cost of the 10 most expensive drugs in the US compared to Germany (based on ex-factory price in EUR)

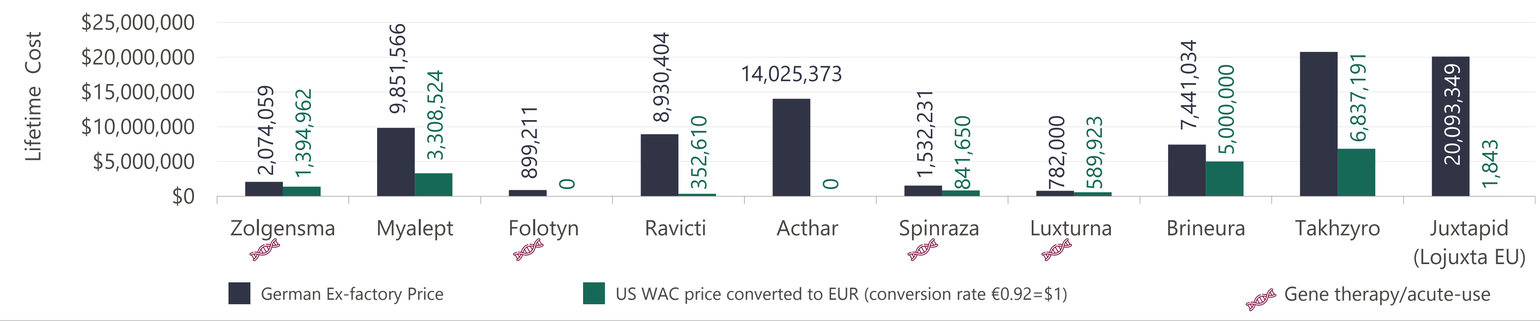

Table 1. Comparison of annual and lifetime costs of the 10 most expensive drugs globally, based on ex-factory price

	ACUTE TREATMENTS		ACUTE TREATMENTS 2019	CHRONIC TREATMENTS		CHRONIC TREATMENTS 2019
	🇺🇸	🇩🇰	🇺🇸	🇺🇸	🇩🇰	🇺🇸
Most expensive annual cost	$2.3m	€1.4m	$2.1m	$1.1m	€331k	$840k
Average annual cost	$1.2m	€606k	$833k	$860k	€156k	$586k
Most expensive lifetime cost	$2.3m	€1.4m	$2.1m	$22m	€6.8m	>$18.0m
Average lifetime cost	$1.4m	€707K	$0.9m	$14.7m	€2.6M	$9.3m

CONCLUSIONS

Overall, the research continues to suggest that acute and curative drugs may be more affordable than chronic treatments over a patient's lifetime.

1. **Existing pricing models tend to focus on short-term affordability.** Innovative assessment frameworks need to focus on the long-term, taking into consideration comparative lifetime costs and overall budget impact to assist payer decision-making and mitigate concerns relating to high 'one-off' prices
2. **The development of novel payment agreements to assist with short-term budget concerns by payers is necessary** to reduce high up-front costs and also uncertainties over long-term clinical benefits of gene-therapies in particular in the real-world setting.
3. **The study highlights the difference in drug prices between Europe and the US.** The Inflation Reduction Act IRA aims to make innovative medicines more affordable by providing greater negotiating power to Medicare and will require manufacturers to pay a rebate for drug list prices that exceed the rate of inflation
4. **Limitations of this research should be noted:** our analysis is based on publicly available pricing information and does not consider confidential discounts. Further, lifetime costs do not account for patients discontinuing treatment prior to death, or any survival benefits associated with the treatment if the analysis is based on historic life expectancy figures

REFERENCES

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Abbreviations: *APU=Manufacturer Sales Price; CADTH=Canadian Agency for Drugs and Technologies in Health; CE=Cost-effectiveness; EMA: European Medicines Agency; EU=Europe; HTA=Health technology assessment; ICER=Institute for Clinical and Economic Review; NICE=National Institute for Clinical Excellence; US=United States; USD=United States Dollars; WAC=Wholesale Acquisition Cost*

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