PDG27

ARE ACUTE THERAPIES AND CURATIVE DRUGS MORE AFFORDABLE THAN CHRONIC TREATMENTS IN RARE DISEASES? AN ANALYSIS OF THE TOP 20 MOST EXPENSIVE DRUGS IN THE US

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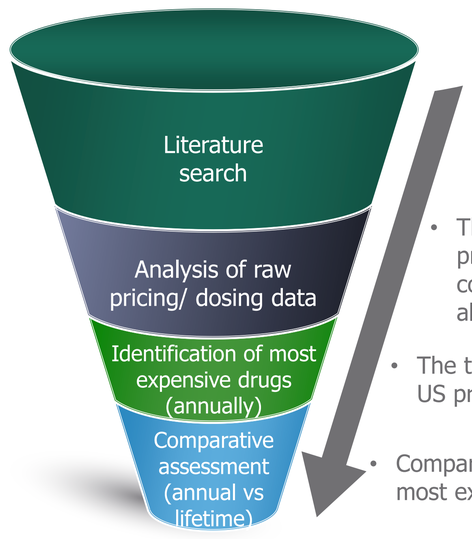
Introduction/objective

- The cost of innovative drugs for rare diseases which can prolong life or improve quality of life have continually pushed the upper pricing thresholds of payers in recent years.
- Recent drug launches and development pipelines highlight the addition of acute use gene-therapies and life-saving treatments to the market which promise a 'cure' or at a minimum, a life altering impact.
- Previous publications assess annual costs only and group together both gene/ acute therapies and chronic treatments, which may not provide a complete picture and places a focus on acute treatments being 'the most expensive drugs in the World'.
- This study investigates costs of gene/ acute therapies versus chronic treatments for rare diseases, on an annual and lifetime basis to provide a more balanced comparison.

Methods

- A targeted approach was used to assess the most expensive drugs globally on an annual and lifetime cost basis



- A literature review of PubMed and grey databases including terms: high cost, most expensive treatment, therapy, annual and lifetime was conducted to identify the most expensive drugs globally
- The most expensive 25 drugs were assessed. Analysis of price (USD EAC price) and dosing data to determine annual costs. Indication, age of onset and life-expectancy data was also used to estimate lifetime treatment costs
- The top 20 most expensive products globally (based on US prices) on an annual treatment basis were selected
- Comparison of annual and lifetime cost of the top 20 most expensive products

Results

- The assessment showed that the top 20 high cost drugs are all orphan drugs offering patients a 'cure' or 'life prolonging' impact. Analysis of raw cost data, taking dosing, indication, age of onset and life-expectancy allowed for comparisons to be made between the annual cost of each treatment (Chart 1) and the lifetime costs (Chart 2) and for trends to be noted across the selected basket of the 20 most expensive drugs (annually, first year) (Table 1).

Chart 1: Annual cost of the 20 most expensive drugs globally (US WAC)

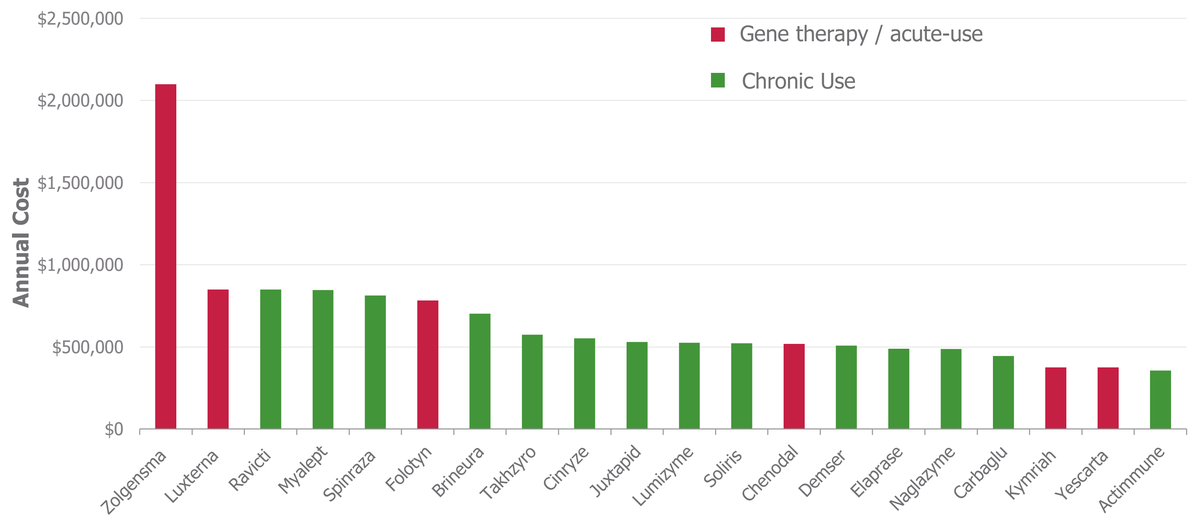


Chart 2: Lifetime cost of the 20 most expensive drugs (globally, US WAC)

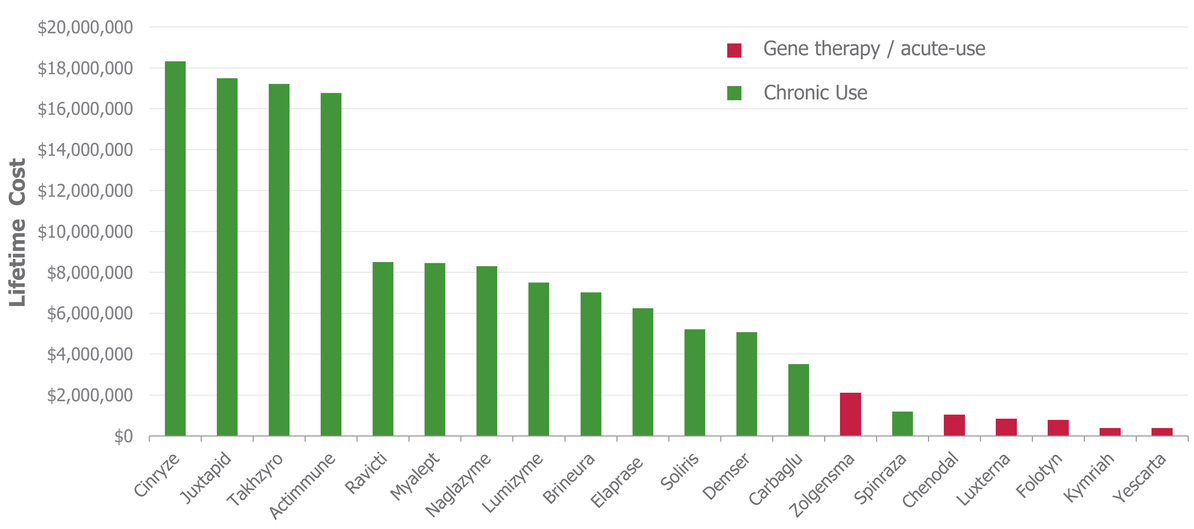


Table 1: Comparison of annual and lifetime costs of the 20 most expensive drugs globally (US WAC)

	Acute treatments (6)	Chronic treatments (14)
Most expensive annual cost	$2.1m	$849,808
Average annual USD WAC	$832,947	$585,571
Most expensive lifetime cost	$2.1m	>$18.0m
Average lifetime cost	$0.9m	$9.3m

- Of the top 20 most costly treatments (first year, annually) six are acute treatments (Zolgensma, Luxterna, Folotyn, Chenodal, Kymriah and Yescarta), with two of these representing the highest annual treatment costs in the basket (Zolgensma and Luxterna). The remaining 14 treatments are all used chronically.
- Interestingly, considering the lifetime costs of these top 20 (most costly, annual) products, of the acute treatments, the highest rank is #14 for Zolgensma.
- The remaining five acute treatments, are all ranked lower (#16-20) and would be unlikely to make an overall list of the top 20 products based on a lifetime cost assessment only.
- The average annual (first year) USD WAC for the six acute treatments is much higher at $832,947 compared to $585,571 for the 14 chronic treatments.
- However, the average lifetime costs of the 14 drugs used chronically, is generally far higher than the lifetime cost of the acute therapies with an average of $9.3m compared to just $0.9m for acute treatments.
- Furthermore, four of the chronic treatments (Cinryze, Juxtapid, Takhzyro and Actimmune) have lifetime costs potentially exceeding $16m, with the most costly, Cinryze, exceeding $18m.

Discussion and conclusions

- Overall our research suggests that acute and curative drugs may in-fact be more affordable than chronic treatments over a patient's lifetime.
- Whilst the emergence of gene-therapies in particular represents a new frontier offering a potential one-shot 'cure', concerns over affordability under existing paradigms of pricing / payment, represent a significant challenge for manufacturers [1,2].
  - In preparation for this new wave of therapeutics, the US ICER is undergoing a collaborative with agencies such as NICE and CADTH to develop and test alternative methods for HTA evaluation of potential 'cures' to establish how to translate results from CE analyses into recommendations for value-based price benchmarks to assist payer decision-making[3].
- Consideration of the comparative lifetime costs and overall budget impact, rather than annual treatment cost, of acute treatments vs existing chronic treatments is important to address the misconceptions relating to the 'high' annual / one-off prices. The focus should be on optimising healthcare budgets over the long-term[2].
- Development of novel payment agreements to assist with shorter-term budget concerns by payers may be necessary to reduce high upfront costs and also uncertainties over long-term clinical benefits of gene-therapies in particular in the real-world setting[2].
  - For example, Zolgensma (US), and Zynteglo (gene-therapy, EU, $1.8m) have outcomes-based instalments plans in place with some payers to spread the cost over five years. It is clear that the implementation and success of these plans to support patient access will continue to evolve.

CADTH=Canadian Agency for Drugs and Technologies in Health; CE=Cost-effectiveness; 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

*References: **1**. Marsden G, Towse A, Pearson S, et al. Gene Therapy: Understanding the Science, Assessing the Evidence and Paying for Value. Office of Health Economics. Institute for Clinical and Economic Review. March 2017; **2**. Towse, A Pricing and Paring for Cures: Early experience with HTA of gene therapy in the USA. Office of Health Economics Review. February 2019; **3**. ICER review article. ICER Launches International Collaborative to Develop New Methods to Guide Value-Based Pricing of Potential Cures. January 2019*

ISPOR 22nd Annual European Congress, 2-6 November 2019, Copenhagen, Denmark