VALUING A CURE:
ARE NEW APPROACHES NEEDED?

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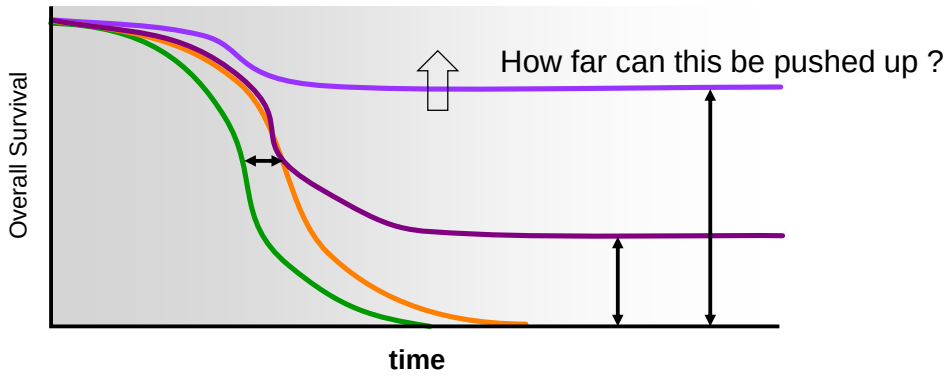
Why do we need new approaches for valuing cures?

- Significant advances in understanding the science and biology of complex diseases with high unmet need, eg. cancer, neurodegenerative disorders, monogenic rare diseases
- Novel approaches incl cancer immuno-therapies, gene therapies and cell therapies
  - While response rates are high, treatment outcomes may vary with potential for durable patient benefit and even cures
- How do we balance early access for treatments with high potential benefit with sufficient evidence?

We want proof, not promise
But what about the value of hope?



- Placebo control
- Cancer immuno-therapy
- Current standard of care including targeted therapies
- Combination: CIT+CIT, CIT+targeted therapy

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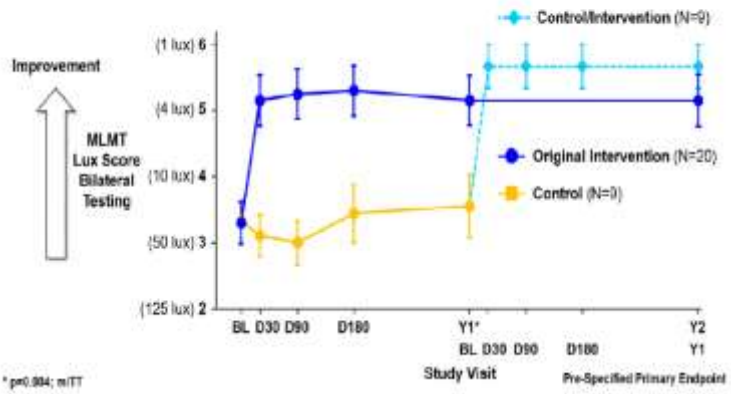
Figure 4.4. Observed Mean Bilateral MLMT Lux Score in Modified Intent-to-Treat Participants Out to 2-years in Phase III Study[a]



Institute for Clinical and Economic Review 2017

Specific issues that have to be resolved for curative treatments

- What is a cure?
- Are we treating incidence or prevalence?
- Who do the financial benefits accrue to?
- What is the evidence that we need for cure/durability of benefit?
- Is our concept of “surrogate endpoint” helpful here?

Different archetypes of treatment situations for cures

- **Novel breakthroughs**, that provide curative treatment in areas where nothing existed before (beta-thalassemia, SMA)
- **Orphan disruptors**: curative treatments for an orphan condition with an established treatment pathway, eg gene-therapies for Hemophilia A and B
- Durable response for **Oncology products** for tumors with high lethality, eg CAR-T therapies
- **Quantum leap**: Indications with very large incident and prevalent populations, representing a significant burden in therapeutic areas, such as cardiology, metabolic disorders, neurology and rheumatology

Newdigs: Designing financial solutions to ensure affordable access to cures. An overview of the MIT FoCUS project, 23 August 2018

Do we agree that cure is better than chronic treatment?

Chronic treatment that completely compensates the disease

- eg Glivec in CML, enzyme replacement therapies in Gaucher´s disease
- Payment is made in installments over the lifetime of a patient
- Treatment outcome depends on persistence
- treatment can be switched when not successful, or stopped in case of severe side effects
- Cumulative treatment costs can extend into USD/EUR millions per patient

One-off treatment that cures the disease

- Eg CAR-T and gene therapies
- Payment is made up front
- Treatment response observed immediately, no issue with persistence
- Not clear how long the benefit will persist or if we can retreat/redose in case of no response. Can we compensate for side effects?
- Are we willing to charge/pay USD/EUR 1 million+ up front?

Financial solutions that have been suggested

- Annuity payments
  - Why would we treat a cure like a chronic treatment from a payment perspective?
  - Significant accounting issues for manufacturer and payer
  - Can be combined with outcomes component (stop payment when defined outcomes are not met anymore)
- Performance based
  - Full payment up front, rebate in case defined treatment outcomes not reached
  - Reduced payment up front, bonus payment when defined treatment outcomes are reached

Do we agree that cure is better than chronic treatment?

- If a pharma company has the choice between developing a chronic and a curative therapy for a condition
  - What decision do we want the company to take?
  - Do we provide the right incentives/rewards?

Doing now what patients need next