Why do we need new approaches for valuing cures?

- Significant advances in understanding the science and biology of complex diseases with high unmet need, e.g., 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?

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**Overall Survival**

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

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**We want proof, not promise**

*But what about the value of hope?*

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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[^1]

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[^1]: p<0.001; mTT
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
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