Pfizer

Vi arbetar för en bättre hälsa i hela världen

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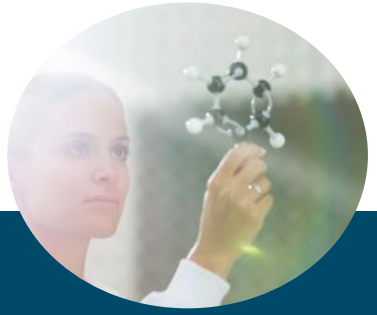
Pfizer i Sverige

Tillverkning

- En av norra Europas främsta tillverkningsenheter av biologiska läkemedel
- Ca 220 medarbetare

Leverantör

- Största leverantören av medicinska behandlingar till svensk hälso- och sjukvård
- > 140 produkter
- Ca 180 medarbetare
- Pfizer Healthcare Hub

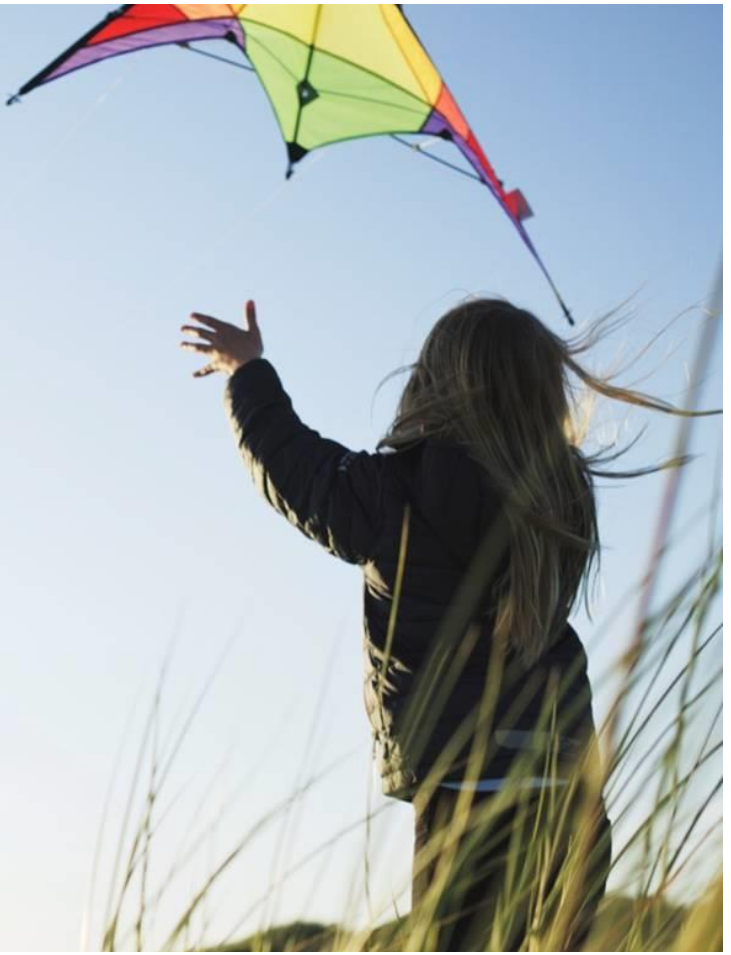


Forskning

Ny forsknings-/utvecklingsmodell

- Kliniska prövningar
- Samarbeten:
  - Mindre bolag
  - Akademin

Hälsoekonomi och avancerade behandlingar



Vad är problemet?

TLV - Beslutsgrunder för subvention

Behovs- och solidaritetsprincipen

- de som har det största medicinska behoven ska ha mer av vårdens resurser än andra patientgrupper

Människovärdesprincipen

- vården ska respektera alla människors lika värde

*Kostnadseffektivitetsprincipe*n

- kostnaderna för att använda ett läkemedel ska vara rimliga från medicinska, humanitära och samhällsekonomiska synpunkter

I tillägg kan sällsynthet (vid stor svårighetsgrad och god effekt) vägas in, praxis i utveckling, få exempel så långt…

Dessa principer ska vägas samman!

NT-rådets beslutsgrunder

- Tillståndets svårighetsgrad
- Tillståndets sällsynthet
- Interventionens effektstorlek
- Den hälsoekonomiska dokumentationens tillförlitlighet

I praktiken tämligen likartade TLVs beslutsgrunder.

Source :NT-rådet 2015, 2016

HTA

- HTA is used to assess the value of health care interventions as part of efforts to efficiently allocate resources in health care systems.

- HTA should be focused on helping decision-makers understand the full relative value of health care technologies for a broad range of stakeholders, considering differences in patient response to treatment.

- HTA studies should follow widely accepted principles of methodological rigor, transparency, and stakeholder involvement to ensure the greatest validity of results.

Evaluation of Orphan Drugs and its Limitations

If ordinary HTA procedures would apply for orphan drugs almost none of them would be considered "cost-effective"

Because of their rarity, the development costs are high. Therefore, in an analysis of the cost per patient the incremental cost per QALY is usually very high and exceeds the "normal" cost-effectiveness thresholds

The type of disease being treated usually makes it difficult to prove a significant increase in QALYs with any degree of certainty because of small populations

The difficulties in carrying out randomized clinical trials and the progressive nature of many rare diseases leads to a high cost per QALY

Source :Drummond et al. 2007

”Standard” methods for HTA might not consider societal preferences sufficiently:

- Studies have shown that the public are willing to give up highly cost effective treatments in order to help the most severly ill patients, or to help those that have no alternative treatments.

- The general population also tends to value helping ”visible”, identified, individuals more highly.

- People tend to value cure, and potential cure, at a premium.

Differs from expected utility, where a gained QALY is a QALY, regardless who gains it.

Source : e.g. NICE 2005, Ubel et al. 1996, Ubel et al. 1999

Gene therapy has the potential to have transformative impact on patient and their caregiver lives

Patients and caregivers

- Potential for a “cure” leading to improved quality of life and productivity
- Less burden on caregivers
- Eliminates burden of chronic treatment including adherence issues

Health care system

- For certain diseases, gene therapies could replace the existing cost of care
- Health care efficiencies - Improvement in patient health could lead to less use of health care resources which could be invested elsewhere

Realizing the promise of gene therapy :

- Transformational gene therapy innovation will require a different approach to health care delivery and financing
- We will all need to work together to find sustainable solutions that enable patient access

Gene therapies have some unique characteristics that we need to consider

Clinical evidence generation
• Large scale RCTs are not available • First-in-human studies are first-in-patient (i.e. Phase 1 & 2 combined) • Unmet medical need/no comparator • Clinical data is reliant upon surrogate outcomes • Dose-ranging will be limited
Assessing and Paying for Value
• Upfront cost for transformative treatment • Budget impact • Patients moving across payers • Surge populations for some treatments • Decision uncertainty – duration of effect/limited data • Quality of life considerations • Iterative nature of some regenerative medicines

Diversity – variability in products – all gene therapies are not the same and need to be treated differently

- Outcomes
- Durability of effect
- Patient Population Size / Concentration
- Unmet Morbidity / Natural Progression
- Treatment Regimen Period
- Existing treatment vs. No existing treatment
- Cost offsets
- Health related Quality of Life
- Safety
- Variability within therapeutic categories and across therapeutic categories

The demise of the Gold Standard, the blinded RCT?

- Two arm-trials not acceptable due to expected superiority of the experimental drug
- One-time interventions with long term effects? Information from comparator arm will become weak (cross-over, drop outs)
- Small populations and treatment genotype matched pathways
- Personalized treatment combinations? With multiple combinations, patient numbers necessary increase exponentially, essentially making RCT impossible.
- Interindividual variance: from noice to focus!

Source :Simon et al. 2015; Eichler et al 2016; Klauschen et al 2014

Uncertainty

Relative efficacy

- Survival
- Quality of life

Relative costs

- Costs related to treatment, or treatment failure
- Opportunity costs, alternative treatments

Rarity, number of patients?

What is fundamentally different with extremely good, curative, treatments?

Very little is known about what's happening in the world in the long run.

We assume that the world is constant, and that the new treatment only affects the world on the margin.

But in the long run, the world is far from constant…

Uncertainty about the contrafactual increases over time.

Divergence in flows?

Short treatment (and payment) period that (hopefully) leads to substantial health gains and/or savings over time:

-> Short run budgetrestrictions.

-> Will the payer or someone else benefit from the savings?

-> Especially important with few patients and products, no risk diversification.

-> Actuarial risk.

Collaboration between payers and manufacturers essential to find solutions

Curative therapies, promise significant clinical efficacy over time and could potentially provide long-term savings compared to current treatments

However, they present substantial challenges for both manufacturers and the health care systems:

Key Uncertainties	
Data uncertainties	• Lack of long-term clinical data and understanding of target populations • The current value-assessment framework fails to adequately capture the full benefit of a curative therapy
The current approach to reimbursement/ procurement isn't appropriate	• Patient identification key • Health systems are constrained by short-term budget cycles and finite budgets • Manufacturers seek to recoup investment through upfront payment (with or without net discounts)
Miss-alignment between cost-effectiveness and affordability	• Potential that high costs to health systems are concentrated upfront, which poses a risk to the system when cumulative technologies launch

Things to consider for annuity and outcome based payment frameworks

Reliable data at launch:

- Epidemiology data – to estimate prevalence and future incidence
- Costs associated with current treatment approach

On-going data collection:

- Single source of data capture needed, e.g., a national registry
- May need to limit treatment to a few specialist centres
- Tracking outcomes in the long-term may be difficult (patient migration)

Type and duration of agreement must work for all parties:

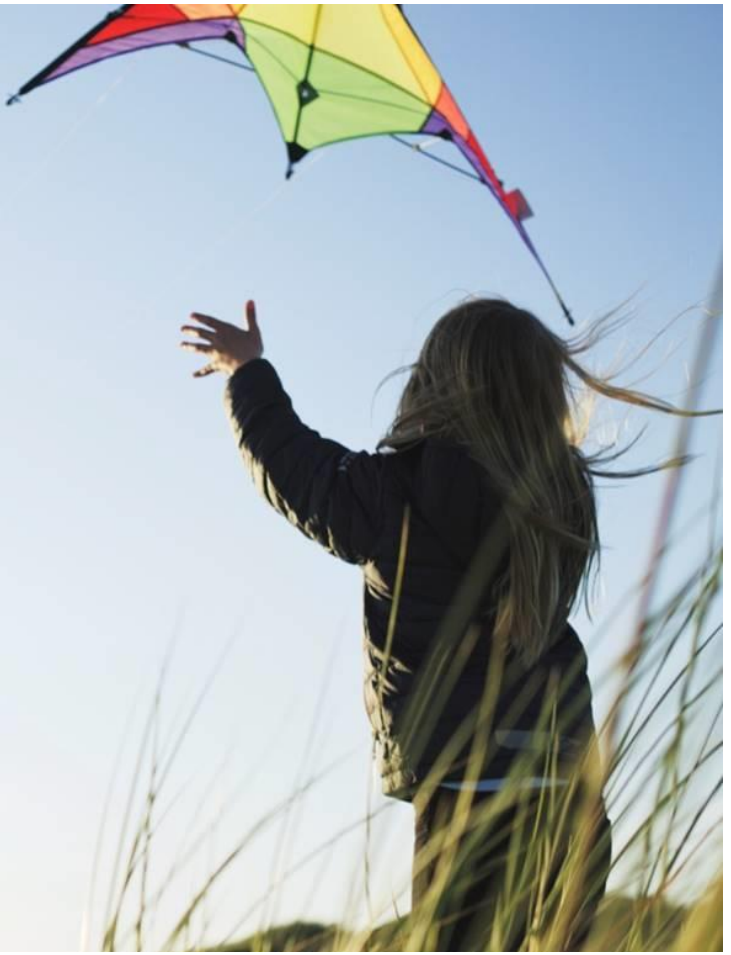
- What outcomes, how often measured?
- Annuity payments, for what period?
- On a per patient basis, i.e., when do payments stop?
- Option for re-negotiation after further data maturity?

Who should be the counter party?

- An actor who can see the treatment as an investment over time and across societal sectors.
- An actor that gets sufficient number of patients under its' umbrella to diminish actuarial risk and gain risk diversification.
- An actor that has a large jurisdiction so that few patients enter and exit it.
- I.e., the central government.

- It is essential with a strong funding and payment model. Without it, we'll get fewer important treatments.
- If the society wants curative treatments, we need clear incentives for it.

Tack och Hej!



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