

Outcomes Based Agreements for Gene Therapy: towards Socially Acceptable Costs and better access

Ensuring access to costly gene therapy with Outcomes Based Agreements (OBA)

Gene therapy (GT) is a cutting-edge treatment option that holds great promise for a range of diseases. On the other hand, the high costs have led to lengthy negotiations with limited or no access for patients, especially in case these costs are considered to be not socially acceptable. OBAs have emerged as a potential solution to the challenges of providing access to high-cost gene therapies. To date, OBAs have hardly been implemented, because of the challenges associated with them, including the difficulty of determining appropriate outcomes and how to collect the data. Despite these challenges, OBAs represent a promising avenue for addressing the access barriers posed by high-cost gene therapies and are considered the best option for the future according to Dutch stakeholders.¹

Ambiguity about Socially Acceptable Costs (SAC) blocks successful OBA implementation

The main challenge with access of gene therapy is the high cost in combination with the expected durability of effect versus the uncertainty of this durability. An OBA could be an answer to this challenge, because the payments are linked to the outcome, taking away this type of uncertainty. However, to be able to come to an OBA, there should be agreement in the appraisal and negotiation phase about the specific main elements that determine what is socially acceptable. In gene therapy, main elements are e.g. the uncertainty of duration of effect and the development cost. Without a general, broadly supported definition of what is socially acceptable and how to determine it, this is a difficult task. The 'social acceptability' of costs is unclear, difficult to predict and difficult to anticipate. If government and manufacturer disagree, patient access could be at stake.

Questionnaire

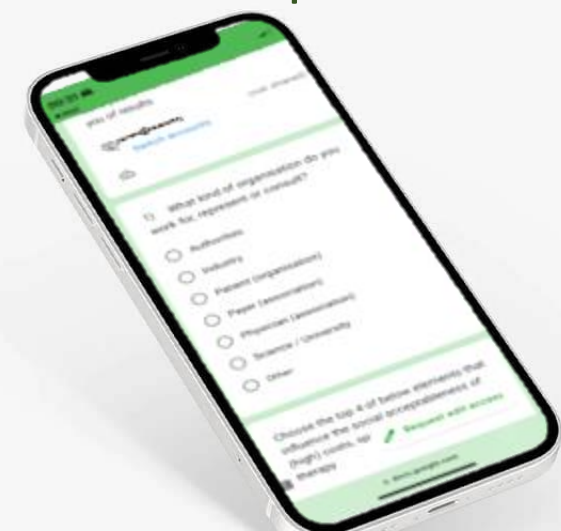
You are invited to share what elements associated with socially acceptable cost you consider to be the most important when setting up an OBA for gene therapies. E.g. Gene therapy's value of innovation, the level of uncertainty in the duration of effect or expected profit for the manufacturer.

Scan the QR-code



on your phone

3 short questions

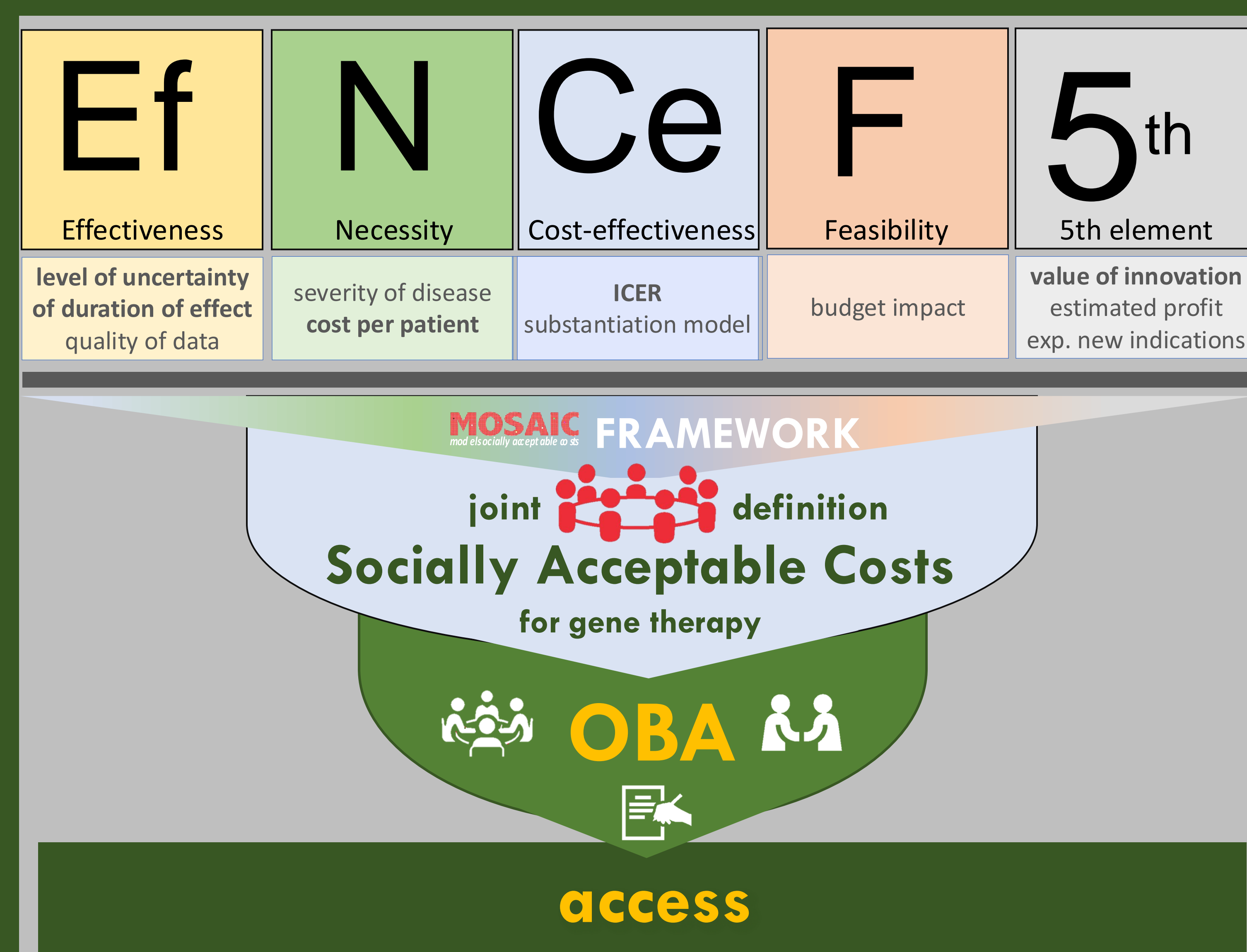


Defining SAC with a Mosaic framework

Several initiatives were launched to come to a definition of SAC, several with a scientific background, also some from a payer's perspective and recently an initiative involving legal action.

One specific project is Mosaic, this initiative is based on broad support in the society, including relevant organisations like patients, HCP's and industry in this project. The Mosaic framework is based on a set of elements that should have impact on the costs and thus on the acceptability of cost to society. These elements include the package criteria and elements associated with it: effectiveness, cost-effectiveness, severity of disease, budget impact and additional elements that are considered in the appraisal e.g. expected profit for the manufacturer, expected new indications or less need for scarce health care employees. The intention of such a framework is to jointly improve the definition of what a treatment may cost according to society. Therefore, it should be developed by, and based upon, broad support by all involved stakeholders, including manufacturers and payers. The development phase will already be helpful in discussions and negotiations, contributing to faster and better access to treatments at socially acceptable costs.² With a Mosaic-like framework, the jointly defined SAC for GT, based on the main elements specific to GT, can be used as input for OBAs leading to faster and more fair access. (Figure 1)

Figure 1. From Mosaic via SAC and OBA to access for gene therapy



Conclusion

A broadly accepted definition of Socially Acceptable Costs is crucial to implement OBA's for gene therapies.

Further insights of all involved parties are to be gathered to jointly discuss and determine in a Mosaic-like framework the key elements associated with SAC to set up an OBA.

1.) Callenbach MHE, Vreman RA, Mantel-Teeuwisse AK, Goettsch WG. When Reality Does Not Meet Expectations-Experiences and Perceived Attitudes of Dutch Stakeholders Regarding Payment and Reimbursement Models for High-Priced Hospital Drugs. *Int J Environ Res Public Health*. 2022 Dec 26;20(1):340. doi: 10.3390/ijerph20010340. PMID: 36612665; PMCID: PMC9819658.

2.) Yap EYS, van der Lelie LG. Mosaic helpt om 'eerlijke' prijs van nieuw medicijn te vinden. *Pharm weekbl* 16 juni 2017;152-24