



Drivers of Socially Acceptable Costs in Outcomes Based Agreements and access

Van der Lelie LG¹, Yap EYS¹, Severens JL²



subject

New treatments like gene therapy (GT) often have uncertainties regarding effect, related to their (high) costs. Outcomes Based Agreements (OBA's) can reduce these uncertainties and can as such be

patients

physicians

producers

payers

a solution towards access (figure 1). Lack of a broadly supported, general description for socially acceptable cost (SAC) can block implementation. Clear and predictable SAC (elements) can help

the industry to have realistic pricing strategies and can help payers to better substantiate their willingness to pay, preparing the way for agreement on the cost element of OBA's.

Fig. 1. How agreement on SAC and an OBA can facilitate access to new (expensive) therapies

Case Specifics

trial circumstances

uncertainties

real world circumstances

other specifics

General Description for SAC

aim to improve effect per investment

conditions:

- outcome
- costs
- data

OBA specific circumstances **ACCESS**

research

Which decisive elements are in your top-4 determining SAC for gene therapies in OBA? 2023 Survey amongst Payers (authorities, government, insurers) and

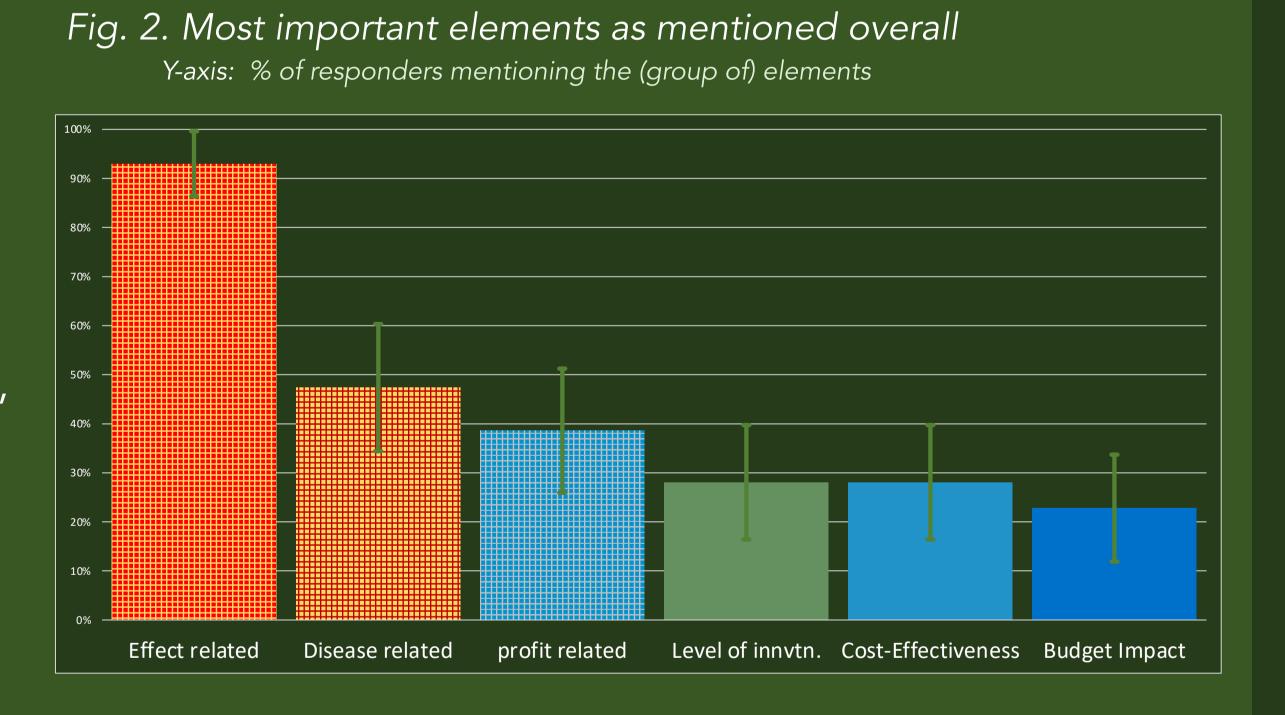
Patients (organisations), market access Experts (scientific, reimbursement/HTA) and Industry (companies and umbrella organisations).

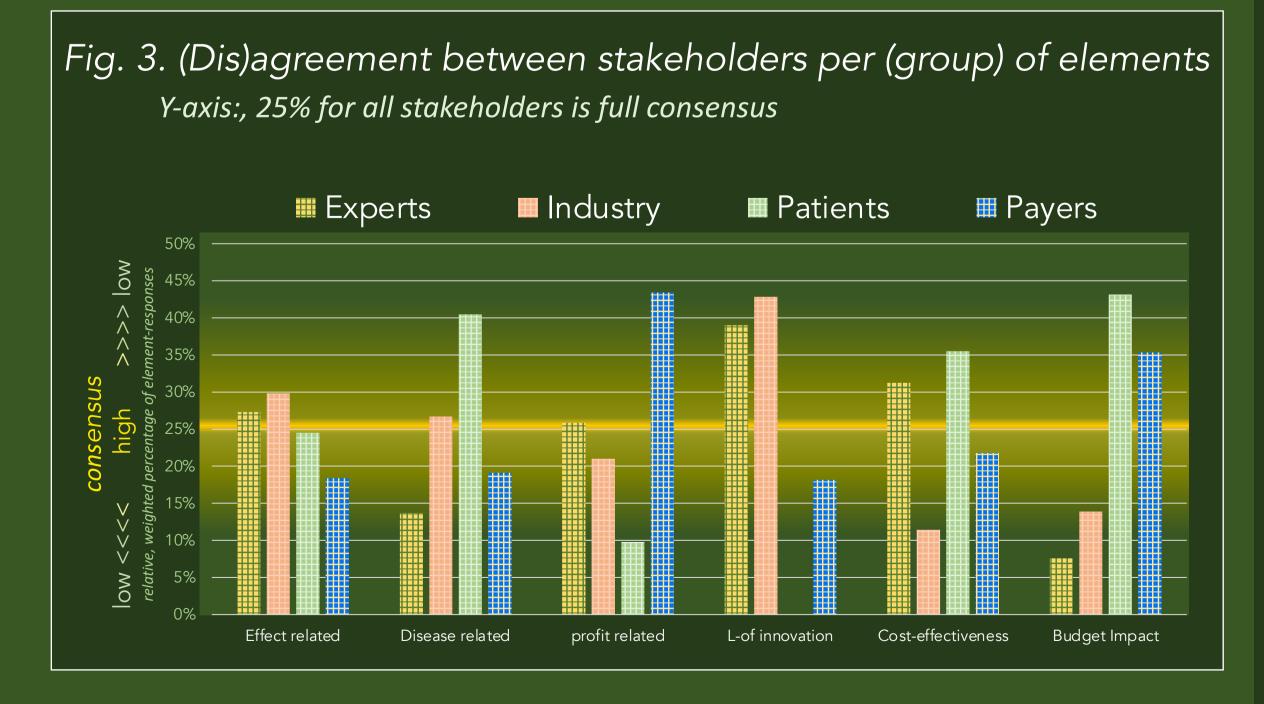
results

Effect-related elements clear winner

All relevant stakeholders are represented in this convenience sample: Industry (37%), Experts (25%), Payers (19%) and Patients (19%). Respondents (n=57) are international, of which 64% is Dutch.

- 93% of respondents indicated effect related elements (i.e. duration of effect, effect size, quality of life) as most important (figure 2).
- The level of consensus between stakeholders seems lower for elements other than effect-related (figure 3).
- Trends on stakeholder top-elements:
 - > Patients: disease related elements
 - Payers and authorities: profit related (e.g. research and manufacturing cost and expected product profit)
 - > Industry: the level of innovation element





conclusion

Other elements require stakeholder discussion to come to a general SAC description for GT

Effect-related elements are valued by all stakeholders as an important driver for SAC. There is more ambiguity on other SAC elements.

Implementation of OBA's and thus access can be facilitated by more mutual understanding, more discussion and, ideally, more stakeholder alignment on the importance and feasibility of all elements to describe SAC.

There may be disagreement on which stakeholders should be involved to develop the general SAC description.

follow up

Follow up Survey:

How to incorporate all relevant views, when developing a general description of socially acceptable costs? Agreeing on driver SACelements should be jointly discussed among relevant stakeholders. Share your opinion in this short survey Which relevant stakeholders should (not) be involved in developing a general SAC description?

