

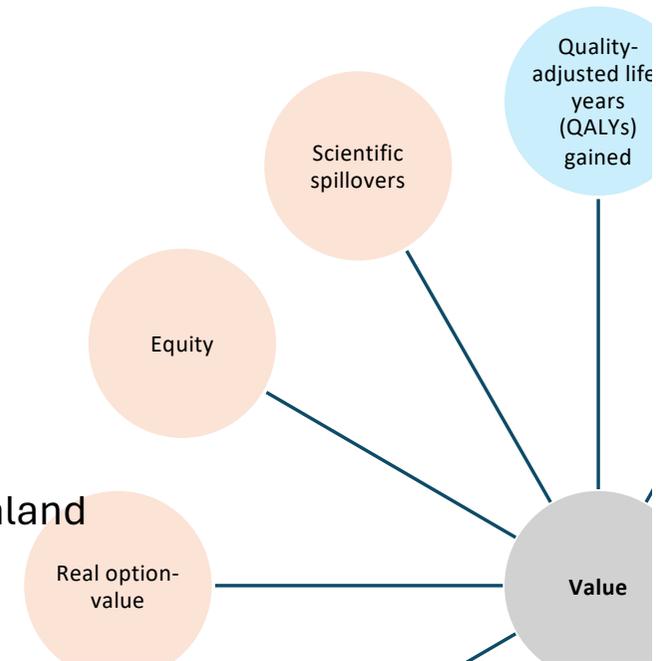
Plucking the petals: Dissecting value claims in gene therapy funding decisions

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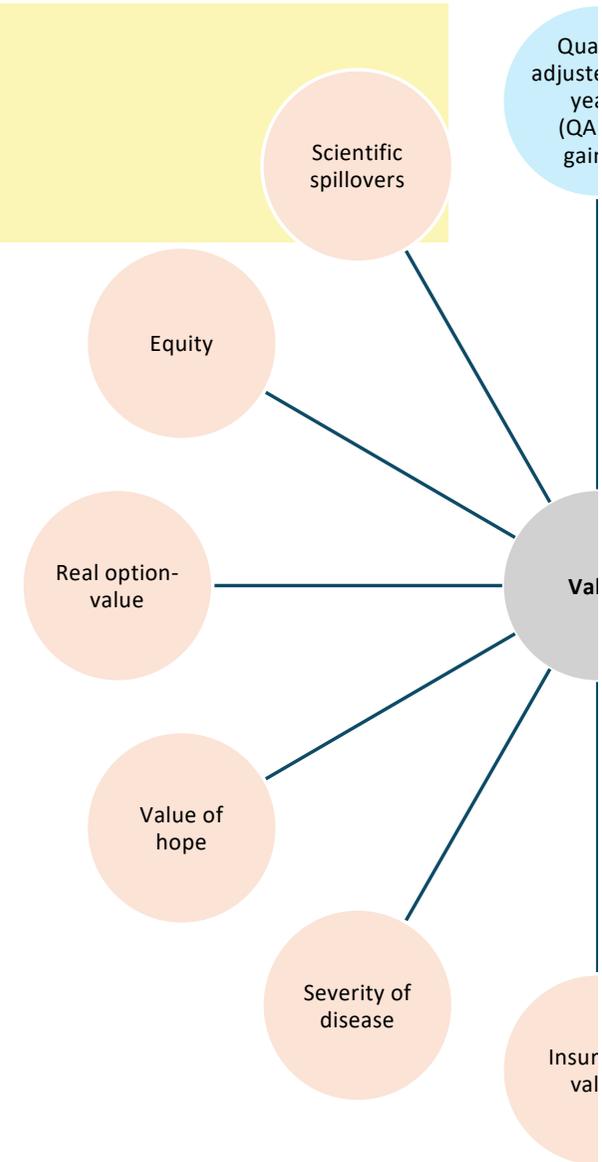
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Outline

- Gene therapies – challenges for funders
- Current and emerging responses
- Research aim and method
- Findings
 - diverse perspectives on revising HTA
 - moral cases for and against expanding value considerations
- Critical observations from a bioethical perspective





Gene therapies – the basics

- Work by repairing, deactivating, or replacing genes
- Potential cures for rare and serious conditions with few or no treatment options
- Over 300 million people worldwide are affected by rare, mostly genetic conditions
- Gene therapies may also address common genetic disorders such as haemophilia, thalassaemia, and sickle cell anaemia
- Typically cost US\$150k-500, but some are US\$3 million per treatment



The challenge for funders

- Uncertainty – lack of evidence that treatments are cost-saving, or at least cost-effective
 - Some show poor post-approval performance, serious side effects, diminishing effectiveness over time, withdrawal
- Manufacturer, clinician, and patient/carer/consumer advocacy to depart from evidentiary standards to remove this barrier to access
- Arguments sometimes invoke values beyond effectiveness, e.g., hope and technological innovation, which are hard to hard to define, quantify, and dispute
- Allocating in accordance with these values would be unaffordable



Current and emerging approaches

- Ongoing debate about:
 - how cost-effectiveness should be defined
 - where thresholds should be set
 - when and how additional ethical, legal, patient, and social factors are to be considered (Norris et al., 2020)
- Approaches to overcoming the barrier caused by strict adherence to cost-effectiveness:
 - case-by-case
 - special funding schemes, like those for rare diseases and cancer
- Analysis (generally via deliberation) can include factors such as:
 - disease severity or youth (where these imply greater health gains for the same cost) and productivity costs/gains
 - other values such as equity, unmet medical need, support for innovation, and preserving future treatment options
- Some HTA scholars advocate formalising this via extending the range of values considered in the analysis
 - quantifying the value(s) OR
 - including the value(s) in deliberation

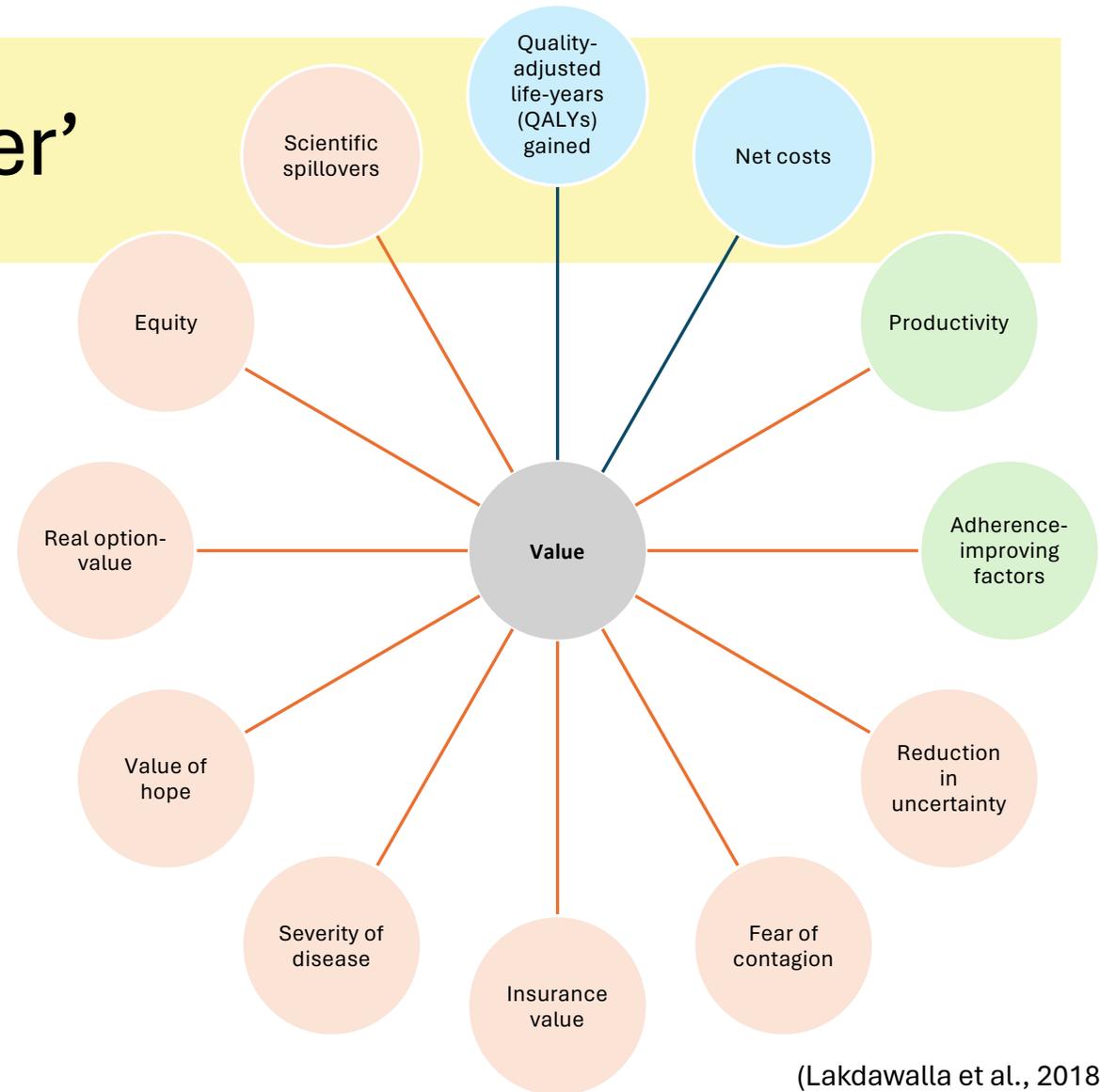


How is a wide definition of value meant to help?

- An economic rationale for paying a premium for gene therapies—over and above the price justified solely on health improvement grounds
- Narrows the gap between payers' willingness to pay and manufacturers' price demands
- Example of potential impacts:
 - the value of ***peace of mind*** from knowing there is a potentially curative therapy for a condition that may affect oneself or family, when aggregated, yields 'enormous economic value' (Garrison et al., 2023a)



The 'value flower'



Circles:

blue: core elements

green: common but inconsistently used elements

pink: potential novel elements of value

Lines:

blue: included in traditional payer perspective

orange: included in societal perspective

(Lakdawalla et al., 2018)



Research question and method

Research question: Whether and how are ethical considerations and frameworks reflected in the academic discourse surrounding values proliferation in health technology assessments of gene therapies

Pragmatic narrative review of the health technology assessment, ethics, social sciences, health humanities literature

- Ovid MEDLINE, Ovid Embase, Scopus, Philpapers, and Philosopher's Index
- (*"gene* therap*" OR "cell* therap*" AND ("health technology assessment" OR HTA OR funding OR reimburs* OR registration OR listing OR subsid* OR "resource allocation" OR values*)*)
- Mar-Apr 2024; refreshed April 2025
- Data were analysed thematically and charted in an Excel spreadsheet



Findings

Type of article

analytical studies (13)

literature reviews (10)

qualitative studies (8)

mixed methods studies (4)

perspective pieces (5)

theoretical papers (4)

conference reports (4)

total (48)

Research focus

broad perspective on evaluation and funding of gene therapies (13)

how value is considered in HTAs (10)

technical and modelling aspects (15)

community or patient perspectives (5)

reimbursement approaches (3)

high-level ethical principles (2)

Region of interest: global or general view (28); Europe (11); USA (8); African countries (1)



High-level overview

- Cost-effectiveness analysis (CEA) remains the most just method for allocating health care resources and should continue to underpin HTAs of gene therapies
- Current approaches have problems – but including novel elements of value is not the solution
- Integrating a wider range of values *is* appropriate
 - quantification of any incorporated values
 - dealing with additional values via deliberation
- The function the term ‘values’ played in the articles and their deeper meaning and implications were rarely explained
- Most articles were concerned with the development of technical solutions to assessing value
- There is enthusiasm in HTA academic community and stakeholders about extra value elements and they are being examined in a *technically* thorough way; however few articles engaged critically with the *ethical* implications of expanding the range of values considered



Moral arguments for adding values

- To avoid withholding potentially curative treatments from those who might benefit
- To recognise the catastrophic nature of many genetic diseases and the potential for cure
- To address limitations of cost-effectiveness in capturing the full value of gene therapies, e.g.,
 - the considerable burdens genetic conditions place on caregivers and families
 - the preferences and risk-tolerance of people with severe, declining health status
 - economic value to the broader health system, caregivers, families and society
- To recognise societal values, e.g.,
 - disease severity, stage (and proximity to death)
 - lack of treatment alternatives
 - youth
- To promote equity (and sometimes redress injustices), the right to ‘the highest attainable level of health’, or freedom of choice



Moral critiques of adding values /1

Distributive injustice

- New values introduced into HTA conflict with existing distributive justice values, particularly efficiency, e.g.,
 - creating a costs and caregiver burden in respect of those who might not survive otherwise
 - demanding considerable infrastructure and capital expenses
 - diverting resources from other essential clinical infrastructure, sectors, and programs to fund gene therapies
- They may drive up ('value-based') prices
- Paradoxically, they may heighten inequities
- There are other therapies with high upfront costs and uncertainty about long-term outcomes
- If additional values are to be considered in gene therapy assessments they ought to apply to other therapies as well



Moral critiques of adding values /2

Procedural injustice

- Compounding uncertainty
 - definition, selection, and operationalisation of novel value elements is unclear
 - information on community preferences is insufficient
- Failure to consider the 'right' values, e.g., value to patients as defined by patients
- Bias and lack of transparency in some methods, such as deliberation
- Other concerns
 - effect of commercial confidentiality stipulations
 - inadequacy of manufacturers' justifications for departing from accepted standards



Critical observations from a bioethical perspective

Deeper articulation of values and value-related reasoning and deliberation e.g.:

- defining values and other key concepts
- exploring whether gene therapies ought to be conceptualised purely as treatments, and the ethical implications of imbuing them with additional purposes such as innovation, and with non-health related values such as insurance value and the value of hope
- exploring how procedural justice frameworks might assist with transparency around deliberation in situations of uncertainty

Critique of the phenomenon of extending value considerations itself e.g.:

- a philosophical examination of adding further elements of value – just to tip the scales – in the absence of evidence that this approach delivers safe, effective, and affordable outcomes

Identifying missing values e.g.:

- unpacking the assumption that HTA is ‘self-sufficient’ in values (none of the articles engaged explicitly with communitarian values e.g., solidarity)

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