



# NMIN's Early Health Technology Assessment (eHTA) Platform

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# Our mission

To support the translation of NMIN-funded technologies from bench to bedside by enabling the use of eHTA to inform R&D and commercialization-related decisions



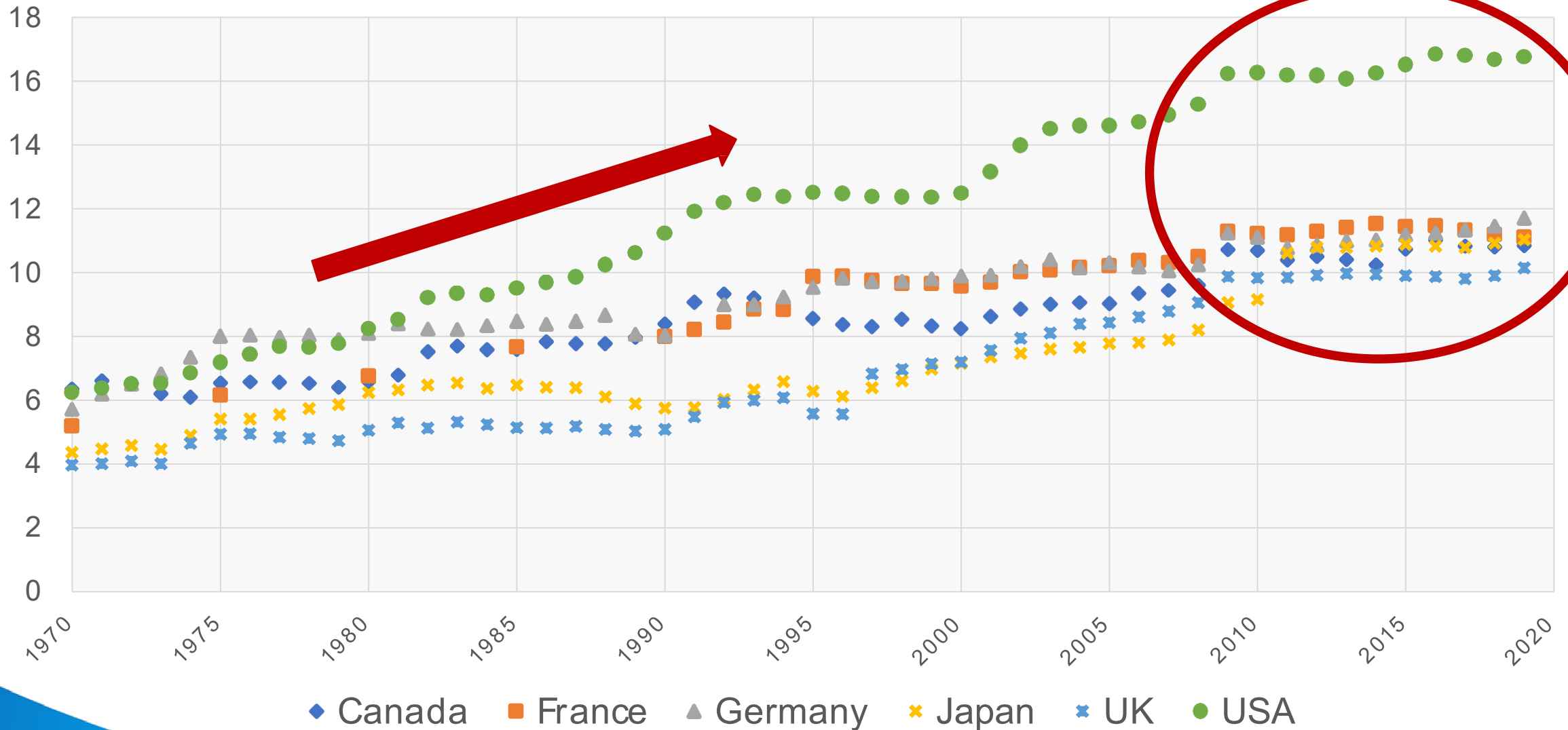
# What is eHTA?

- Use of health economics methods (e.g., cost-effectiveness analysis) early in the medical product life cycle
- Estimates potential value of proposed product for key healthcare stakeholders

Cost-effectiveness and budget impact are key drivers of **reimbursement decisions**



# HEALTHCARE SPENDING AS % OF GDP, 1970-2019





**\$2.8M  
gets you...**

- Zolgensma for 1 patient
- Statins for 14,000 patients
- Hospital stay for 456 patients

**Does Zolgensma deliver  
good value for money?**

PHARMA

STAT+

## At \$2.1 million, newly approved Novartis gene therapy will be world's most expensive drug

By ADAM FEUERSTEIN @adamfeuerstein / MAY 24, 2019

Reprints



Source: <https://www.statnews.com/2019/05/24/hold-novartis-zolgensma-approval/>

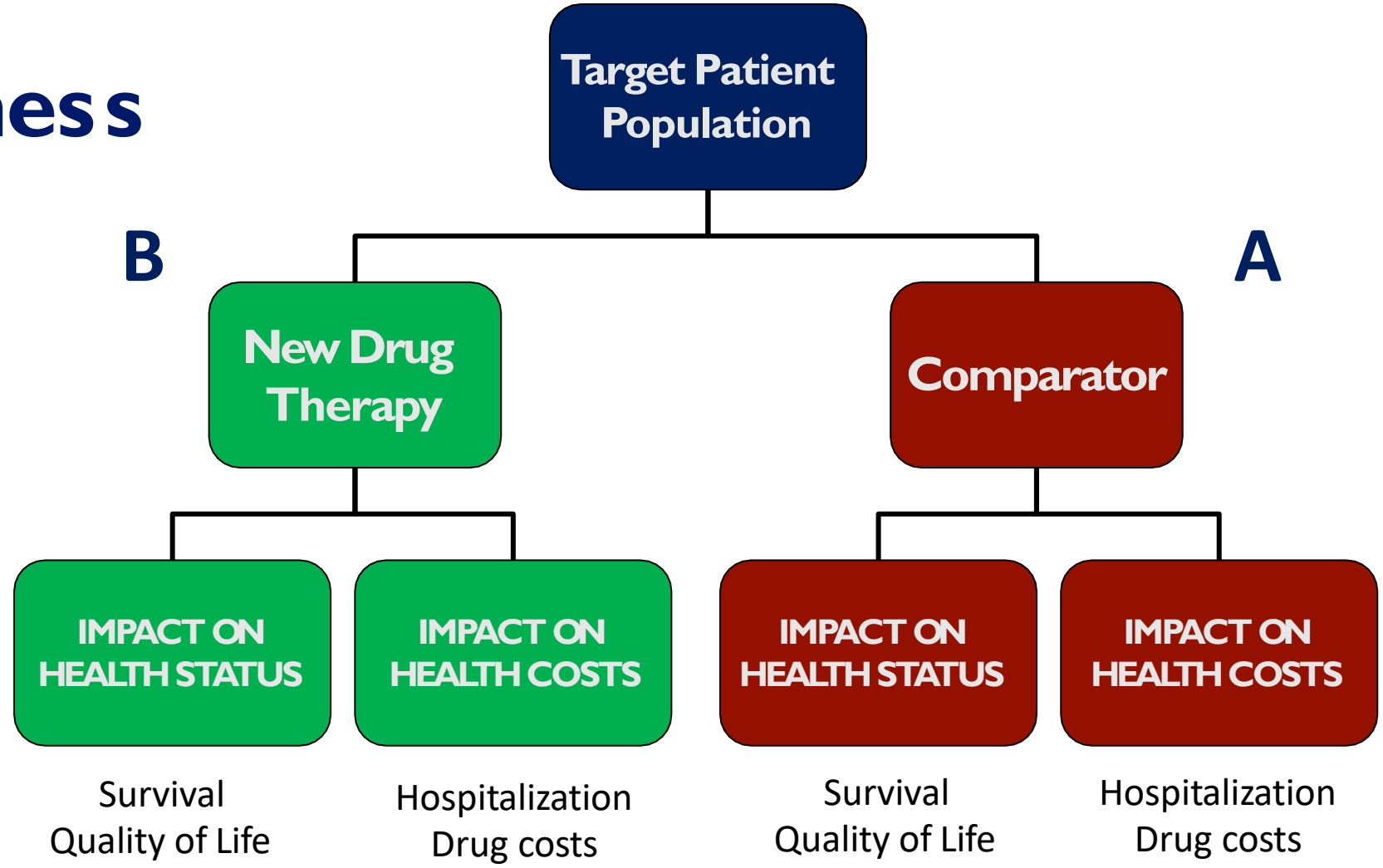


# Logic of cost-effectiveness analysis

Key metrics:

Quality-adjusted life-year (QALY)

Incremental cost-effectiveness ratio (ICER)



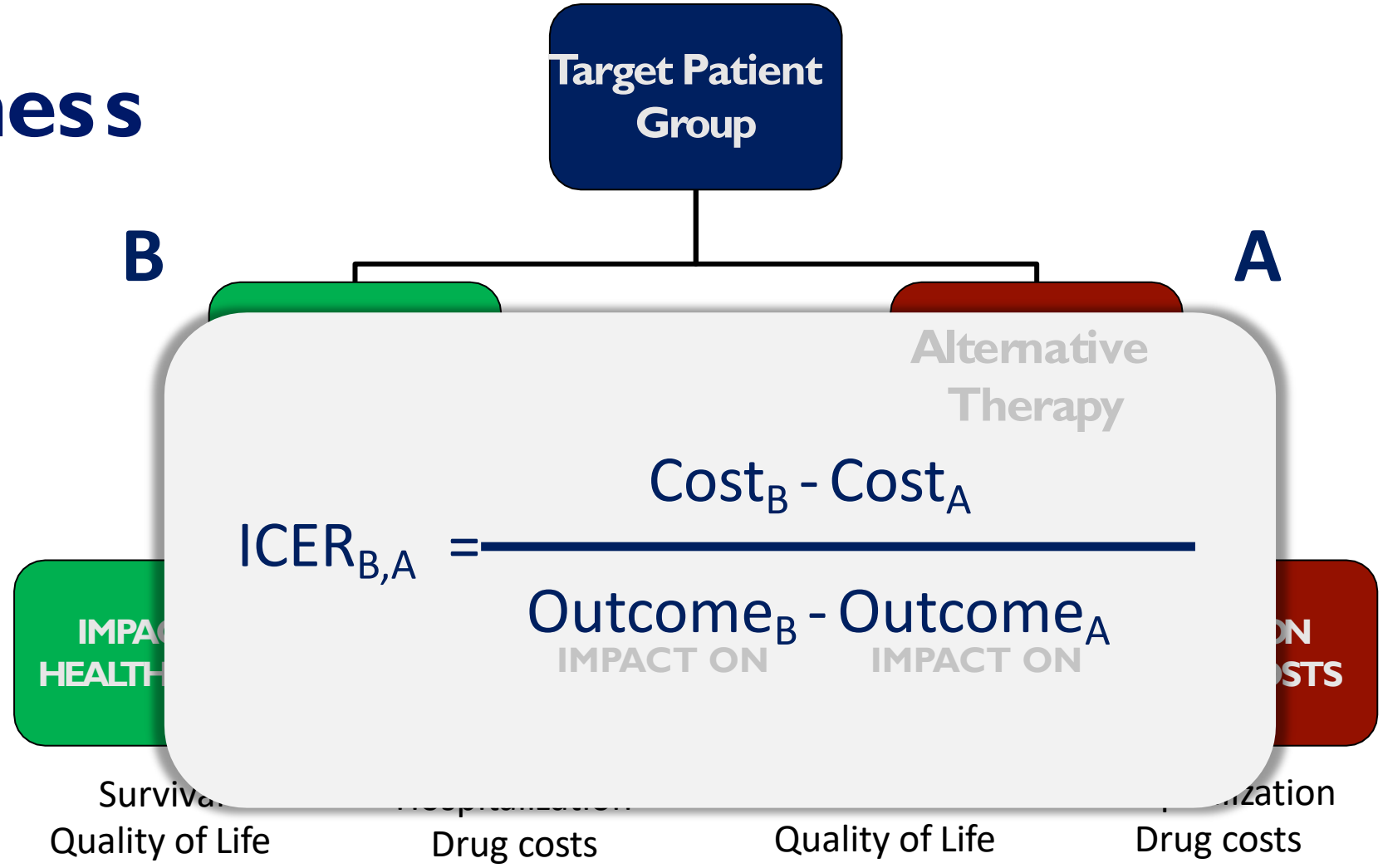


# Logic of cost-effectiveness analysis

Key metrics:

Quality-adjusted life-year (QALY)

Incremental cost-effectiveness ratio (ICER)



E.g., \$245,000 per QALY gained



# Cost-effectiveness influences coverage decisions

Specialty drug coverage by 17 US commercial health plans

- Is the ICER less than predefined threshold? (e.g. £20-30K/QALY in UK)
- ICER is not the only factor considered by payers
- But... larger ICERs *do* make reimbursement more difficult to achieve, even in the US market

ICER (\$/QALY)	% Restricted	Adjusted OR
< \$50,000	18	Reference
\$50K-\$175K	22	1.86
\$175K-\$500K	30	1.86
>\$500K	46	2.03

Source: Chambers et al. (2021) "Is an Orphan Drug's Cost-Effectiveness Associated with US Health Plan Coverage Restrictiveness? *Pharmacoeconomics*. Epub 26 Oct 2021





# Types of eHTA analyses

## Headroom analysis

- Assumes maximum clinical benefit for target population
- Estimates maximum reimbursable price for given indication
- Upper bound of market size given value-based pricing

## Threshold analysis

- Varies clinical parameters within plausible ranges
- Identifies drivers of cost-effectiveness (i.e., value)
- Minimum clinical performance required for cost-effectiveness

## Many others...

- Value of information analysis to inform clinical trial design
- Patient and provider preference studies
- Budget impact analysis to assess affordability for payers



# Potential eHTA Use Cases

- Screen possible indications for “headroom”
- Societal impact assessments for grant applications
- Identify target product profiles with strong value propositions
- Bottom-up market sizing for business plans
- Reimbursement risk assessment for investor pitches
- Inform evidence generation strategy at the clinical stage



# eHTA Platform Activities

## Community engagement

- Promote eHTA awareness and HQP capacity-building
- Gain understanding of user requirements for platform services
- Establish external partnerships within innovation ecosystem

## Collaborative eHTA research

- Partner with life sciences teams to secure grant funding for eHTA
- Economist-led eHTA studies (full-service contract research)
- User-led eHTA studies (coaching and consultancy services)

## Innovation in eHTA services

- Development of user-focused eHTA resources (e.g., self-assessment toolkit, user guide, case study examples)
- Iterative design and evaluation of standard operating protocols for economist-led and user-led eHTA studies



# eHTA Platform Services

## Initial Consultations

\* No cost to NMIN-funded teams

- ❖ Define eHTA needs, assess study options, suggest next steps

## Grant Development

\* No cost to NMIN-funded teams

- ❖ Joint applications to NMIN's SI-KTEE/Commercialization Support program or external funding opportunities
- ❖ Integrate eHTA into a larger grant application

## Planning Workshops

- ❖ Overview of eHTA methods and study options
- ❖ Co-design potential eHTA study with project team

## Contract Research

- ❖ ***Economist-led model*** - Traditional contract research model
- ❖ ***User-led model*** - Life science teams take the lead in eHTA study; Platform provides resources, coaching, and quality assurance



# Thank you!

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