

INTRODUCTION ON POST-APPROVAL CHALLENGES, REIMBURSEMENT AND HTA

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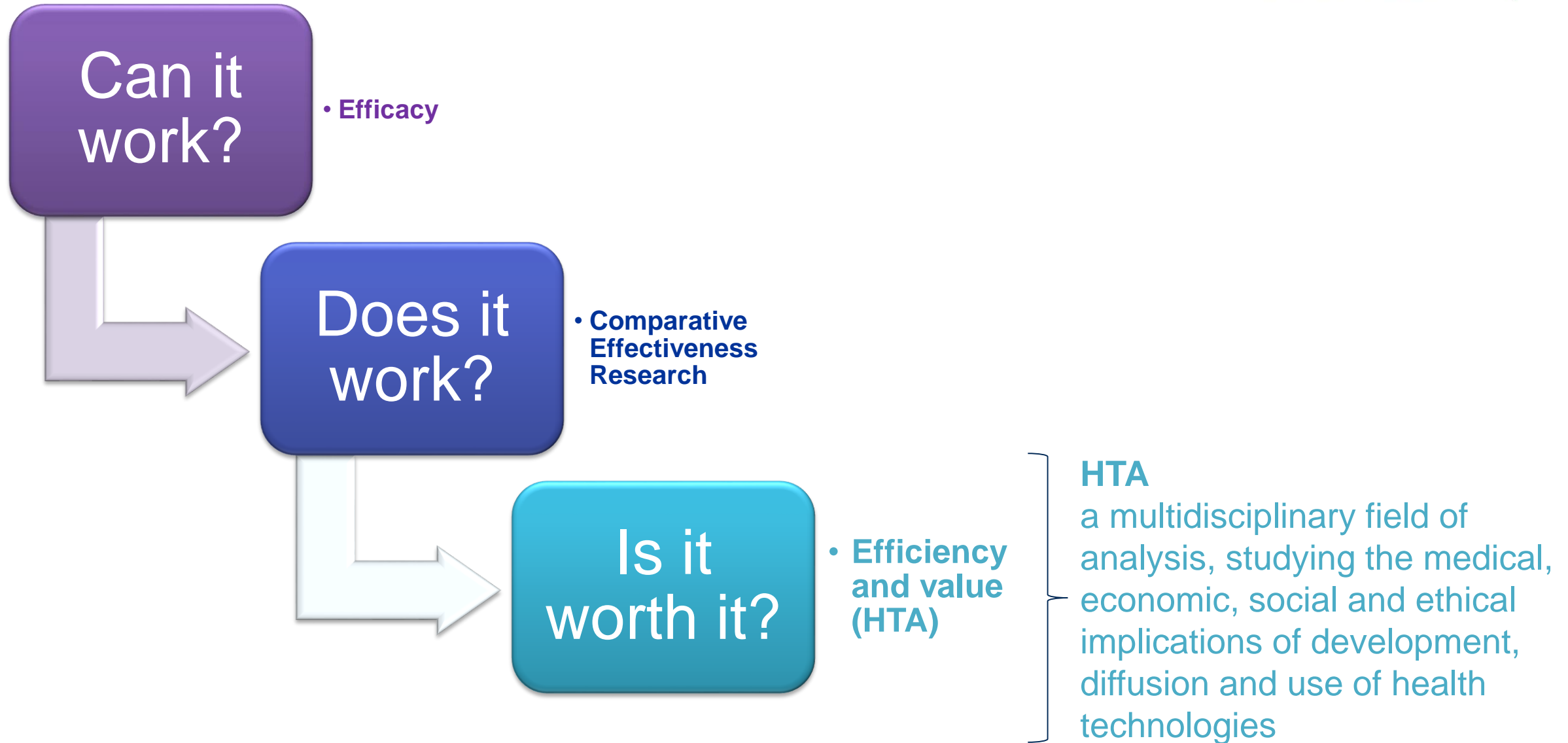
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FROM EVIDENCE GENERATION TO DECISION-MAKING



What are the typical identified gaps?



INTRODUCTION TO POST-APPROVAL CHALLENGES FOR CELL&GENE THERAPIES

Assessment of Clinical Effectiveness and Safety

Surrogate endpoint evidence

Rarity of condition

Small single-arm trials

Short-term evidence

Incorporating broader elements of value into decision-making

Severity of condition

Lack of alternatives

Paediatric population

Value to caregivers

Assessment of costs and affordability challenge

Time horizon and discount rates

Organizational arrangements

Alternative payment schemes

RECENT CELL&GENE THERAPIES ASSESSED BY HTA ORGANIZATIONS

	NICE	ICER	CADTH	SMC	AIFA
Kymriah DLBCL	✓			✓	✓
Kymriah ALL	✓	✓			✓
Yescarta	✓	✓		✓	
Luxturna	✓	✓	✓	✓	
Strimvelis	✓				
Imlygic	✓				
Alofisel	✓			✓	
Provenge	✓				
Glybera	✓				
Zolgensma	✓				✓

NICE = National Institute for Health and Care Excellence; ICER = Institute for Clinical and Economic Review; CADTH = Canadian Agency for Drugs and Technologies in Health; SMC = Scottish Medicines Consortium; AIFA = Italian Drug Agency

THANK YOU

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