PRISM | Promoting Rare-Disease Innovations through Sustainable Mechanisms

Accessing new treatments for rare diseases: What does rational, fair, and equitable look like?

publichealth.ualberta.ca

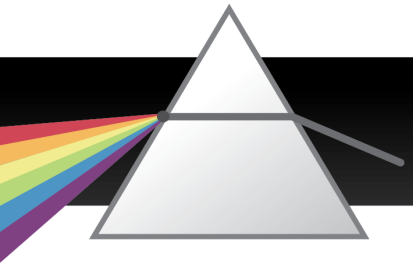
April 29th, 2025

OVER THE NEXT 70 MINUTES…

- How are new treatments for rare diseases made accessible to patients in Canada?
- How does this compare with other countries?
- What does *rational*, *fair*, and *equitable* access mean?
  - Industry perspective
  - Patient perspective
  - Clinician perspective
  - Public payer perspective

WHO PAYS?

Public drug plans	• Federal, provincial, and territorial governments • Coverage focused on certain populations
Private drug plans	• Often employer-sponsored, but sometime purchased by individuals • Limits on coverage per year or over life of patient
Pay out of pocket	• Individuals pay for drug themselves • Often not possible given high cost of drug
Patient support programs	• Offered by pharmaceutical companies • Provides access on a compassionate basis
Clinical trials	• Patients enroll in trial • Provides access to drugs still in development

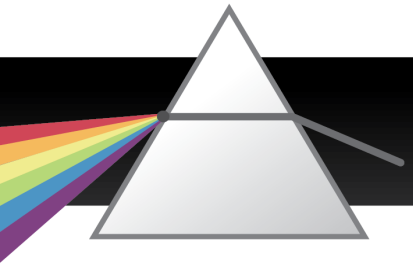


WHO PAYS?

Treatments for rare diseases: Mainly public drug plans

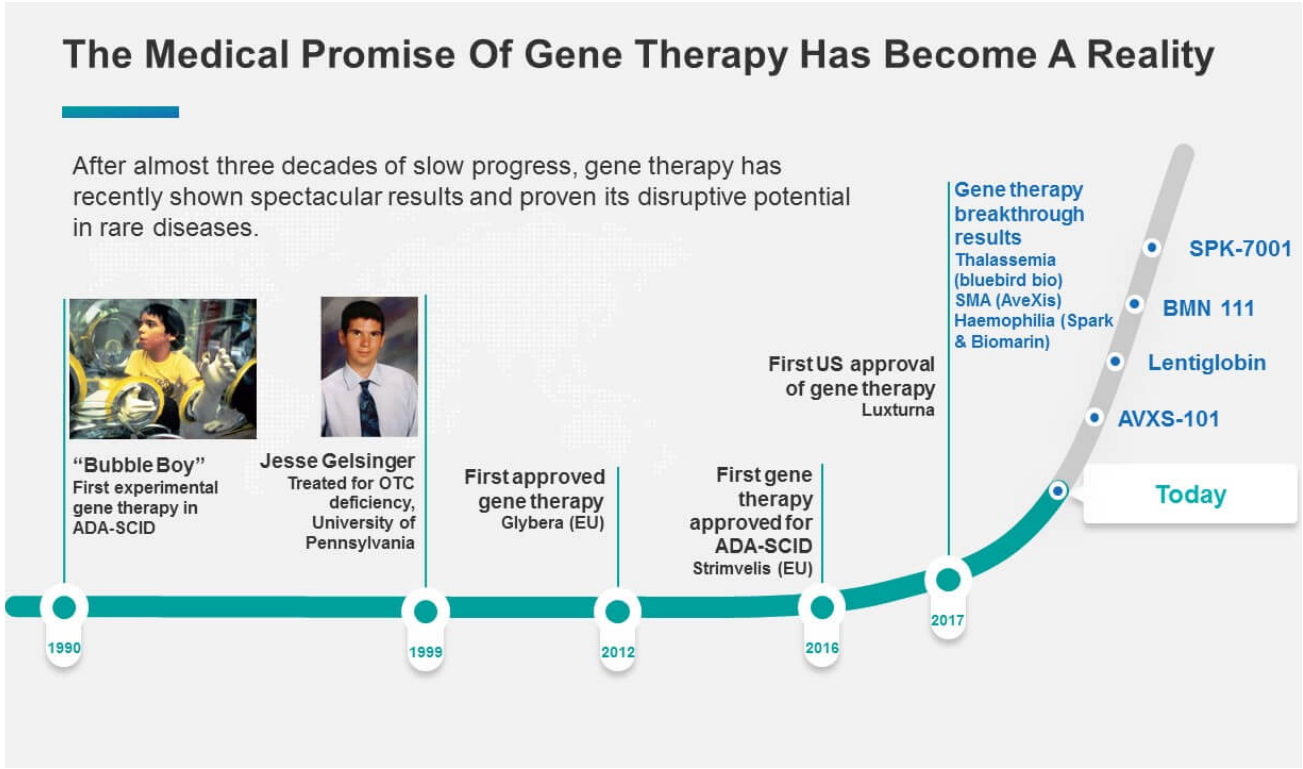
Cost of new gene therapy based treatments

Name	Rare inherited condition	Main symptoms	Response	Cost/patient (USD millions)
Luxturna	Retinal disease	Blindness	Preserves some eyesight	0.85
Zolgensma	Spinal Muscular Atrophy	Loss of muscle, loss of ability to breath, walk & swallow, death	Preserves muscle function	2.1
Casgevy/ Lyfgenia	Sickle cell anaemia	Crippling pain, organ damage, stroke, death	Eliminates pain crises	2.2-3.1
Hemgenix	Haemophilia B	Uncontrolled bleeding, death	Substantially reduces bleeds	3.5



WHO PAYS?

- US FDA estimates: 10-20 new gene therapies by next year
- This doesn't include:
  - Therapies that don't involve genes or cells e.g. enzyme replacement therapy
  - Therapies for hard-to-treat or rare cancers



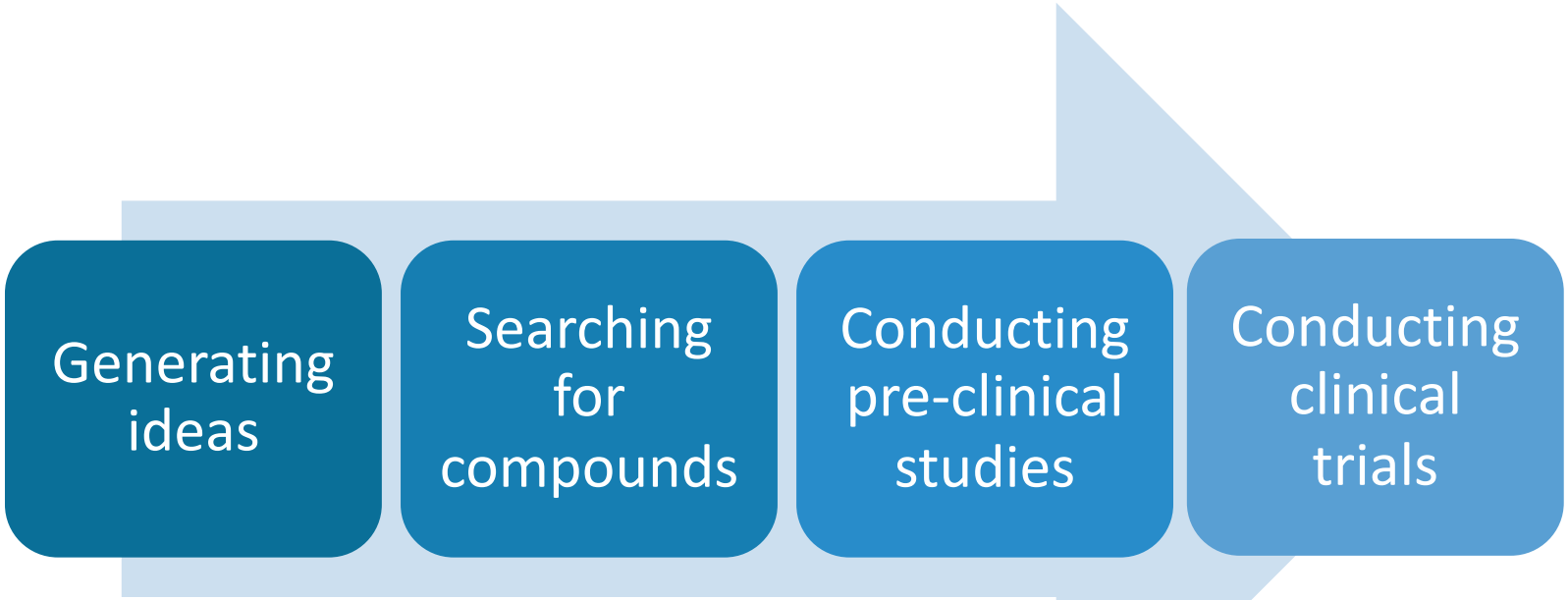
WHAT STEPS ARE INVOLVED IN ACCESSING NEW TREATMENTS?

Does the treatment appear promising?

Should the treatment be sold in Canada?

Should the treatment be covered or paid for?

DEVELOPMENT



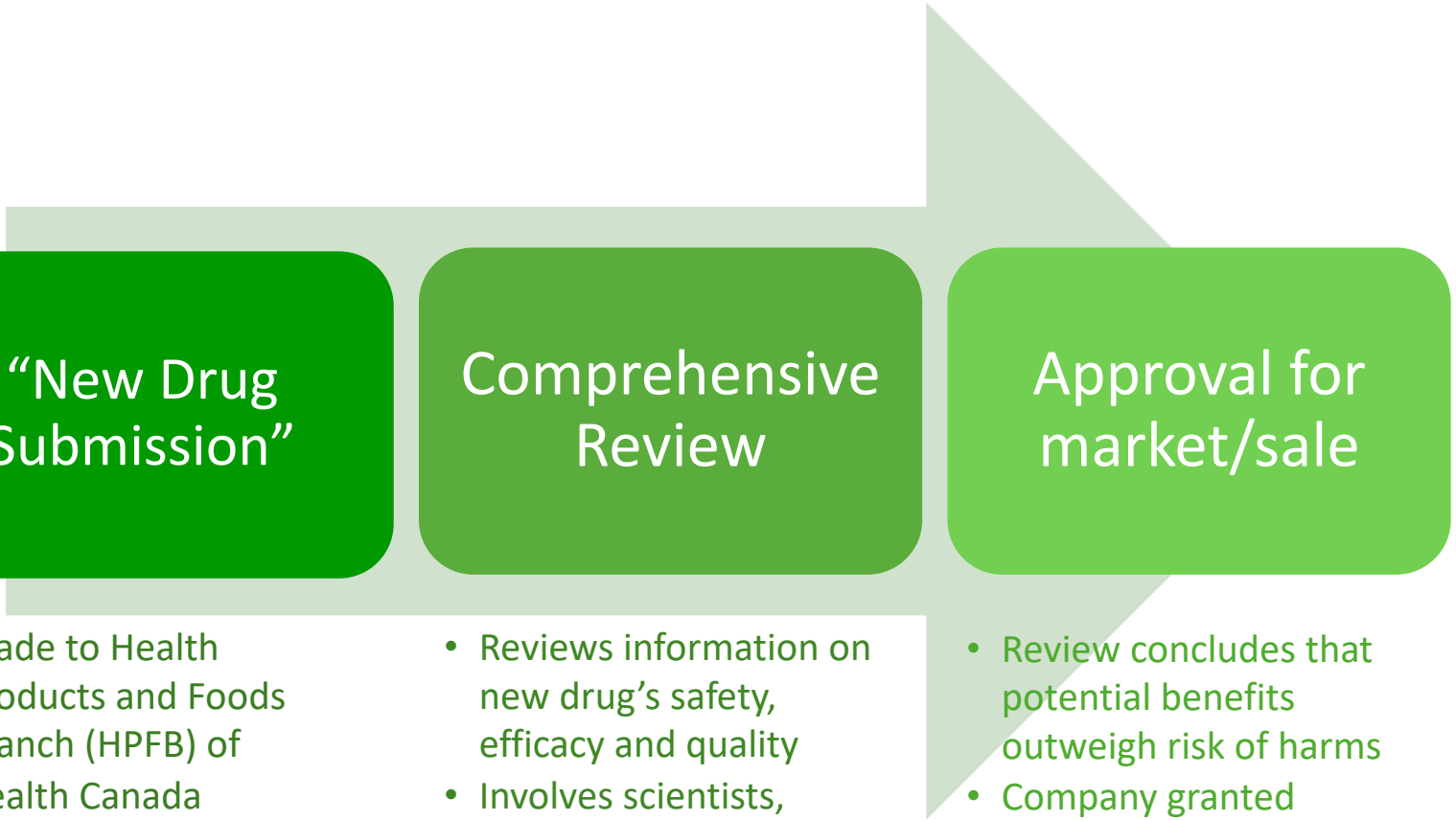
Basic science research to:
1) Understand a disease
2) Identify disease "targets"

- Millions of compounds screened for link with targets
- Structure of selected compounds modified optimize link with targets

Studies in animals to evaluate safety, efficacy and toxicity of modified compound (potential new therapy)

Trials in humans to assess:
- Phase I: Is it safe?
- Phase II: Does it work?
- Phase III: How well does it work compared to existing treatments or placebo?

MARKET (REGULATORY) APPROVAL



PUBLIC COVERAGE

Canada's Drug Agency (CDA)*

- Makes **recommendations** on whether a new drug should be publicly funded to participating drug plans
- Expert committee assesses "clinical effectiveness and cost-effectiveness, as well as patient and clinician perspectives, of a drug"

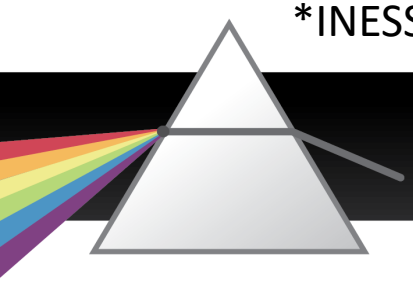
Pan-Canadian Pharmaceutical Alliance (pCPA)

- **Negotiates prices** on behalf of provincial, territorial and federal public drug plans
- Goal is to achieve greater value through combined purchasing power

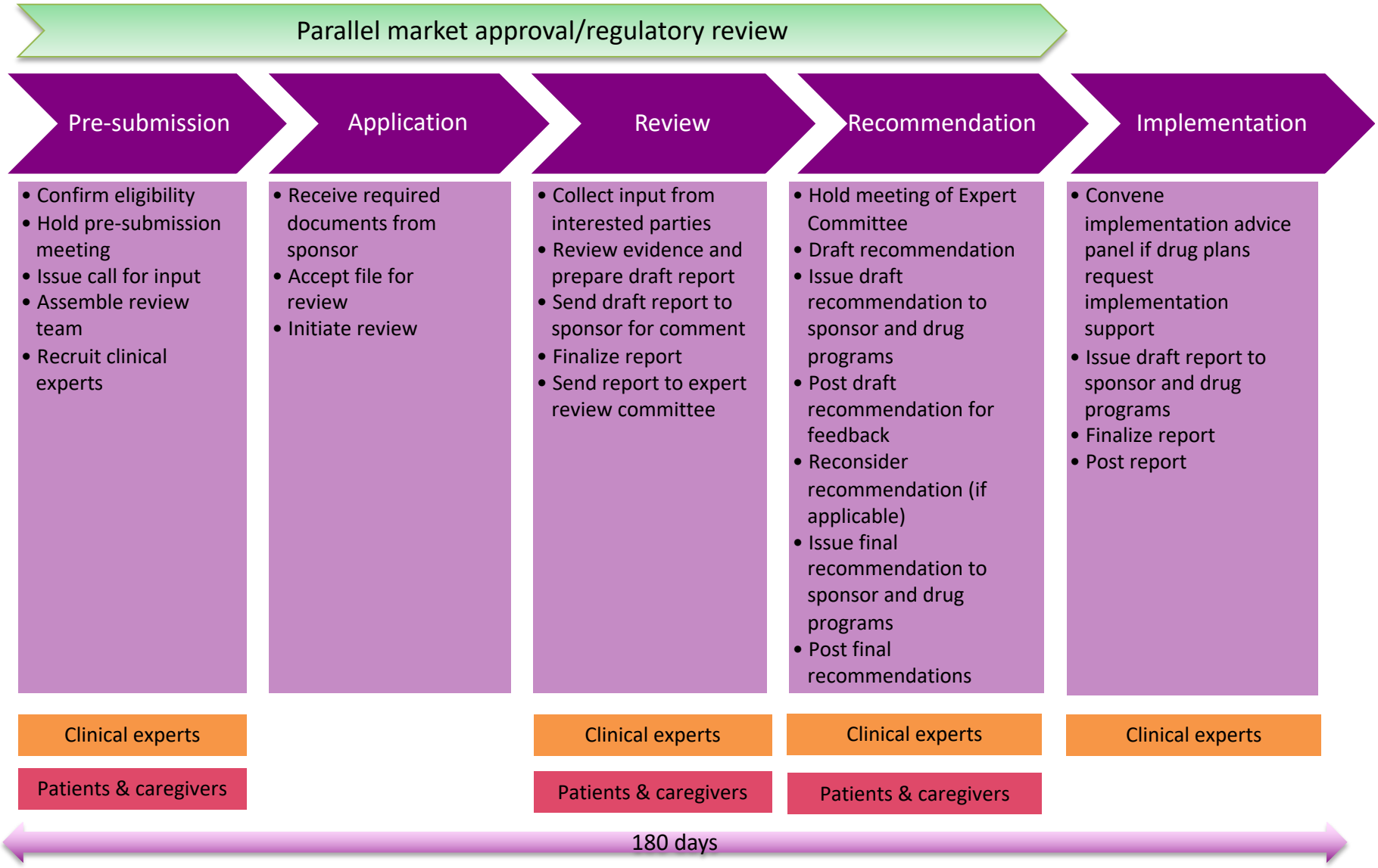
Individual public drug plans

- Makes final **decision** on whether to fund a drug
- Enters into a "listing agreement" with company

*INESSS in Quebec



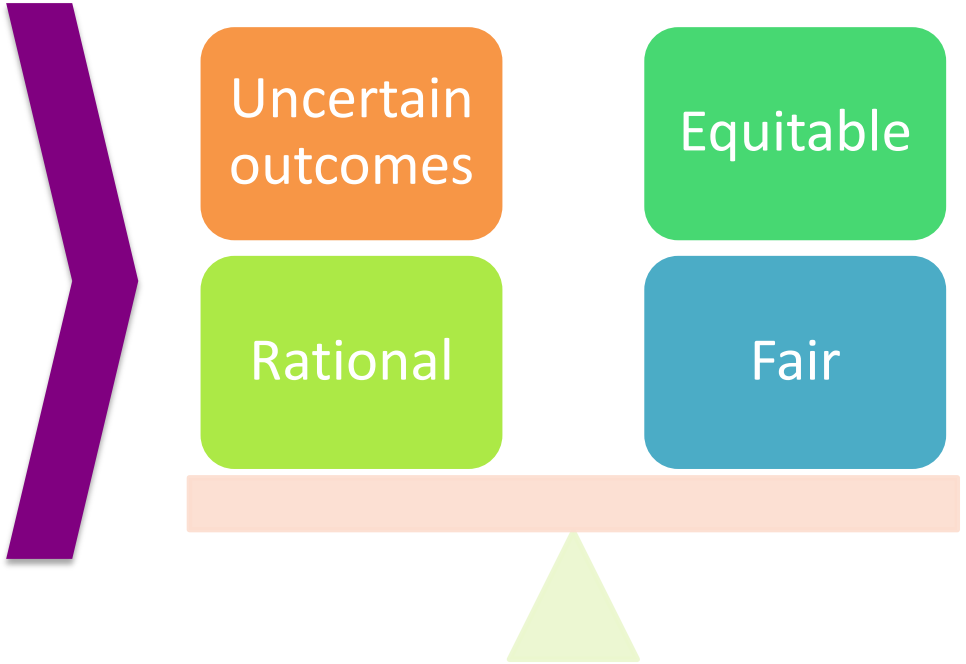
PAN-CANADIAN REIMBURSEMENT RECOMMENDATION PROCESS



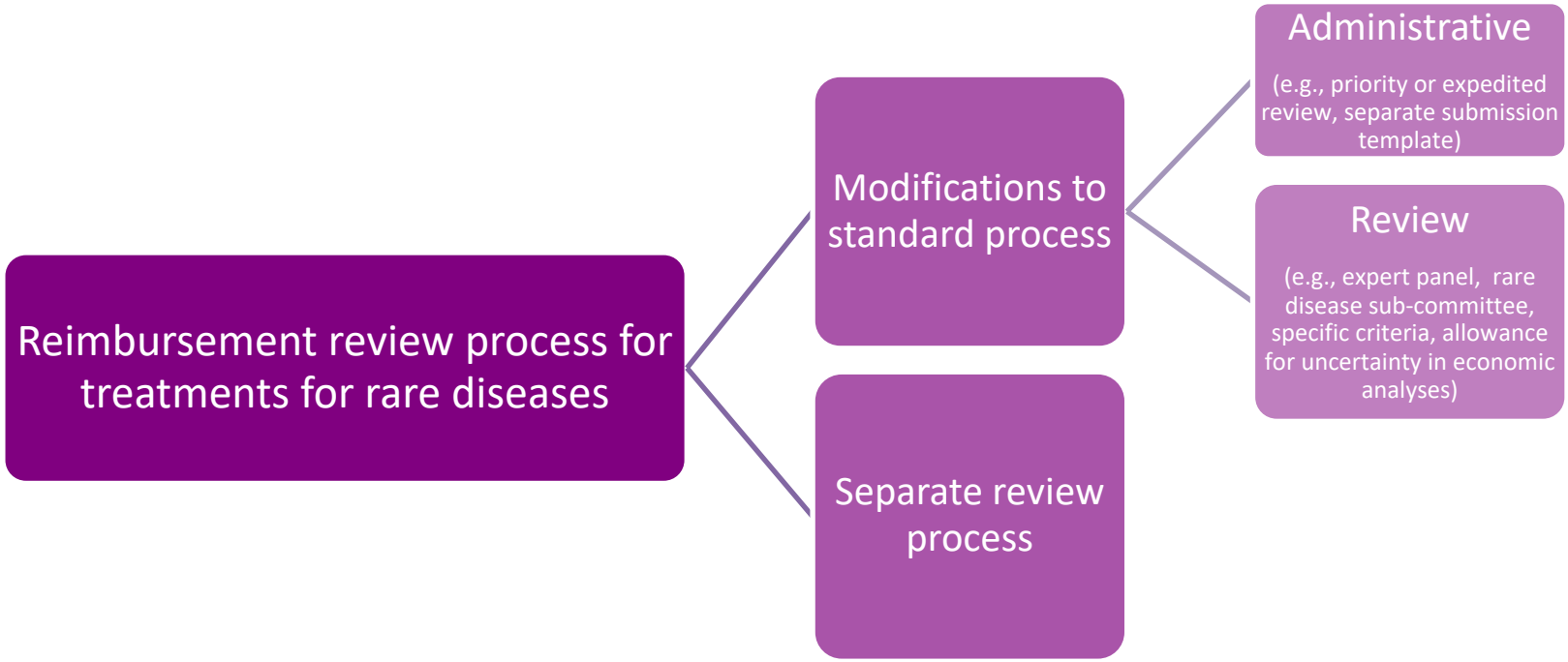
WHAT ABOUT SPECIAL CONSIDERATIONS FOR TREATMENTS FOR RARE DISEASES?

Features of most treatments for rare diseases

- For small numbers of patients
- For often inherited conditions that start in childhood
- Lack of alternative treatments
- Potentially life-changing or life-saving outcomes



WHAT ABOUT SPECIAL CONSIDERATIONS FOR TREATMENTS FOR RARE DISEASES?



WHAT ABOUT SPECIAL CONSIDERATIONS FOR TREATMENTS FOR RARE DISEASES?

Other countries with modified processes:

JURISDICTION	ELIGIBLE DRD	ANNUAL BUDGET IMPACT	MODIFIED PROCESS
France	Innovative: • Received "orphan drug" status* and • Meets one of the following conditions: 1) New type of care 2) May be clinically significant advance 3) Meets need not sufficiently covered	< €30 million	• No HTA required • Reimbursed at price set by manufacturer
		≥ €30 million	• HTA required • Fast tracked process
Germany	Received "orphan drug" status* at regulatory approval	< €50 million	• No HTA required • Reimbursed at price set by manufacturer
		≥ €50 million	• HTA required • Standard process
The Netherlands	Received "orphan drug" status* at regulatory approval	< €2.5 million	• No HTA required • Reimbursed at price set by manufacturer
		≥ €2.5 million	• HTA required • Standard process

*Special designation granted by regulatory bodies to drugs intended to treat rare diseases

WHAT ABOUT SPECIAL CONSIDERATIONS FOR TREATMENTS FOR RARE DISEASES?

Jurisdictions with modified processes affecting DRDs:

JURISDICTION	ELIGIBLE DRD	MODIFIED PROCESS
Italy	Received orphan drug status at regulatory approval	• HTA required • Fast tracked process
	Innovative drug: 1) Addresses unmet need 2) Significant added therapeutic value 3) High quality clinical trials	Access provided through special fund for up to 36 months
Scotland	Meets definition of ultra orphan drug 1) Prevalence of ≤ 1 in 50,000 2) European Medicines Agency (EMA) orphan designation 3) Condition is chronic and severely disabling 4) Condition requires highly specialised management	• HTA required • Made available for up to three years during which additional evidence is generated
Australia	Meets all of the following conditions: 1) For clearly definable disorder 2) Do not meet cost-effectiveness requirements but are considered clinically effective 3) No alternative non-therapeutic modality exists 4) Annual cost represents unreasonable burden to patient/family	• HTA required • May be considered through Life Saving Drug Program

WHAT ABOUT SPECIAL CONSIDERATIONS FOR TREATMENTS FOR RARE DISEASES?

Jurisdictions with completely separate process for DRDs:

JURISDICTION	ELIGIBLE DRD	DRD PROCESS
United Kingdom – NHS England	Meets all of the following conditions: 1) Target population so small that treatment is concentrated within a few centres 2) Chronic and severely debilitating condition 3) Technology is used within a highly specialised service 4) High acquisition cost 5) Need for national commissioning	• Specialized review committee with expertise in DRDs • Evaluation methods that account for challenges involved in treatments for small populations

PUBLIC COVERAGE

As of September 2022

Product (trade name)	CDA	INESSS	Australia	Catalonia (Spain)	Germany	Italy	Netherlands	Scotland	Sweden	UK
Asfotase alfa (Strensiq)	F	D	D	n/e	F	D	n/e	n/e	n/e	F
Burosumab (Crysvita)	F	D	n/e	n/e	F	F	n/e	F	F	F
Cerliponase alfa (Brineura)	F	F	F	n/e	F	F	n/e	n/e	n/e	F
Elosulfase alfa (Vimizim)	F	F	F	n/e	F	F	D	D	F	F
Lumacaftor/ ivacaftor (Orkambi)	D	D	F	n/e	F	F	F	D	F	D
Nusinersen (Spinraza)	F	F	F	F	F	F	D	F	F	F
Tolvaptan (Jinarc)	D	D	F	F	n/e	F	F	F	D	F

WHERE DO WE GO FROM HERE?

- Common challenges:
  - Affordable, sustainable access
  - Increasing number of high cost treatments

- Common responses?
  - How do we better manage clinical uncertainty?
  - How do we better manage financial uncertainty?

STAKEHOLDER PERSPECTIVES

SOME DEFINITIONS

Equitable: Everyone should have a fair opportunity to attain their full health potential; therefore, individuals may require different approaches or supports to achieve similar outcomes

Fair: Providing care and resources in a way that is just, unbiased and responsive to individual needs and circumstances; ensuring that individuals have an opportunity to achieve health without discrimination

Rational: making decisions based on evidence, logic, and sound reasoning that are typically aimed at achieving the best outcomes with the most efficient use of resources