



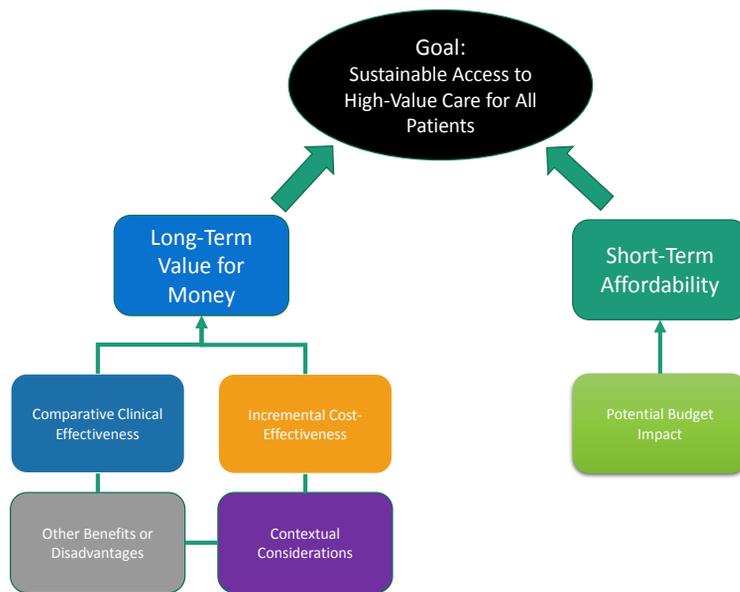
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## Current Value Frameworks – What's New?



## ICER Value Framework: Recent Developments

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## Modifications for “Treatments of Ultra-rare Disorders (URDs)”

- Eligible treatments
  - Condition/label for less than approximately 10,000 individuals (higher than EU definition)
  - No ongoing or planned clinical trials for populations >10,000
- No explicit change to different “standard” of evidence for judgments of comparative clinical effectiveness
- ICER will provide specific context regarding the potential challenges of generating evidence for these treatments
  - Conducting RCTs
  - Validating surrogate outcome measures
  - Obtaining long-term data on safety and durability of clinical benefit



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## Key Modifications for Treatments of URDs

- Cost-effectiveness thresholds included in the report expanded from \$50,000 per QALY to \$500,000 per QALY
- Value-based price benchmark remains \$100,000-\$150,000/QALY
  - But specific language to be included about history of decision-makers accepting higher thresholds
- *When impact of treatment on benefits and costs outside the health system are substantial in relation to health system costs, societal perspective analyses are presented in tandem with results from the health system perspective (co-base case).*



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## Key Modifications for Treatments of URDs

- Broader framework for other benefits and contextual considerations to reflect potential for impact on family, school, and community, as well as infrastructure for screening and care of affected individuals
- Manufacturers invited to supply information on costs of research and development, if they desire



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## Potential Other Benefits

- This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients who have failed other available treatments.
- This intervention will have a significant positive impact outside the family, including on schools and/or communities.
- This intervention will have a significant impact on the entire “infrastructure” of care, including effects on screening for affected patients, on the sensitization of clinicians, and on the dissemination of understanding about the condition, that may revolutionize how patients are cared for in many ways that extend beyond the treatment itself.



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## Contextual Considerations

- This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
- This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
- This intervention is the first to offer any improvement for patients with this condition.
- Compared to “the comparator,” there is significant uncertainty about the long-term risk of serious side effects of this intervention.
- Compared to “the comparator,” there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention.
- There are additional contextual considerations that should have an important role in judgments of the value of this intervention.



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## ICER Reports on Treatments for URDs

Drug	Dual base case?
Luxturna	Yes
Cystic Fibrosis • Kalydeco / Orkambi / Symdeko	No
Hemophilia A • Emicizumab (Hemlibra)	No
hATTR Amyloidosis • Inotersen / Patisiran	No
HAE • Lanadelumab / Haegarda / Cinryze	No
Spinal Muscular Atrophy • Spinraza / AVXS-101	TBD



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## Recent Evaluations: Luxturna

- ICERs
  - \$644,000/QALY health system perspective
  - \$480,000/QALY societal perspective
- VBPB
  - \$150-220K or \$360-430K for 15-year-olds
- Panel votes:
  - Adequate evidence of clinical effectiveness
  - Majority: Intermediate value for money
- Key policy recommendation
  - All stakeholders should realize that a growing stream of treatments for rare and ultra-rare disorders cannot all be priced at levels far above traditional cost-effectiveness thresholds without seriously threatening the financial sustainability of the health system.



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## Recent Evaluations: Emicizumab

- ICERs
  - Improved health and cost-saving
- VBPB
  - Not calculated
- Key policy recommendation
  - Innovation that addresses unmet clinical need and produces overall cost savings in the health system should be encouraged. However, in situations where new or emerging therapies appear cost-saving at a high price given high existing costs, reasonable value-based pricing requires consideration of a new paradigm for “shared savings” between innovators and society.



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## Challenges in Valuing Potential Cures

- *How should value-based prices for therapies reflect “cures” that produce magnitudes of lifetime health gains and cost offsets that are far beyond those generated by traditional therapies?*
- *How should value-based prices for therapies reflect uncertainty regarding inclusion of additional elements of value that may be important for potential cures, but which are not part of standard cost-effectiveness methods?*
- *How should value-based prices for therapies reflect social values related to treatments for very severe conditions, rapidly fatal conditions, rare conditions, illnesses that afflict children, and conditions that have a high lifetime burden of illness?*



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## What are Options for Value-Based Pricing of Cures?

- Full price at standard cost-effectiveness WTP thresholds -- untenable
- Abandon cost-effectiveness and use a different paradigm, e.g. fair profit
- *Cap the price at the level associated with health (QALY) gain no matter what cost offsets*
- *“Shared savings”*



Thank you