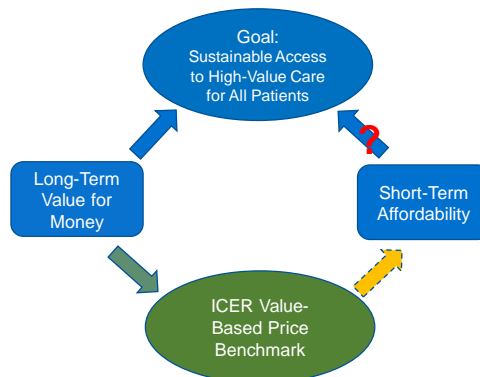

Valuing a Cure: Are New Approaches Needed?



ICER value-based price benchmark



Challenges in Valuing Potential Cures

- Uncertainty regarding clinical effectiveness due to limitations in study design, outcome measures, and the size and duration of clinical trials
- 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



Potential Other Benefits particularly relevant for potential cures

- This intervention offers a novel mechanism of action that will allow successful treatment of patients for whom other treatments have failed.
- 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.

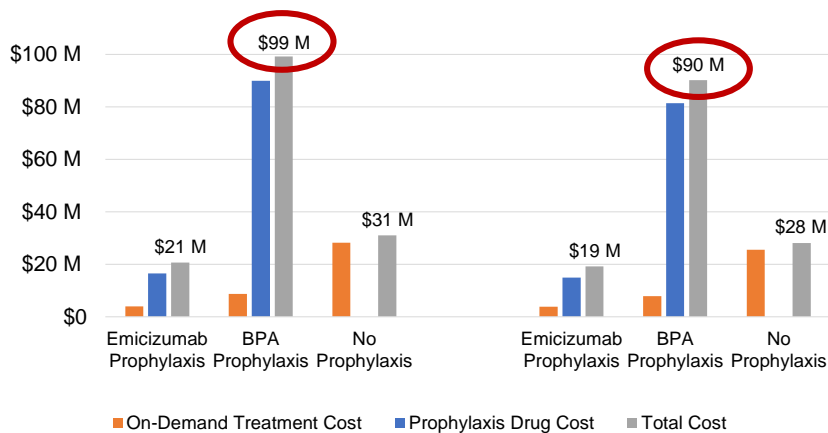


Challenges in Valuing Potential Cures

- Magnitudes of lifetime health gains and cost offsets that are far beyond those generated by nearly all traditional therapies



Lifetime costs of Hemophilia A with need for bypassing agent (BPA) prophylaxis



Options for value-based pricing of a cure

- New cure of a fatal disease of a 5 year-old child who would die in 10 years with standard Rx
- Assumed WTP threshold of \$100,000/QALY

	Cost per year of current Rx	QALY gained	QALY gain price	Cost offset price component	"Value-based" price
Standard CEA	\$400,000	50	\$5 million	\$4 million	\$9 million
QALY Price cap	\$400,000	50	\$5 million	\$0	\$5 million
Re-priced cost offset at \$100,000 per QALY	\$400,000	50	\$5 million	\$1 million	\$6 million
Shared savings 50%	\$400,000	50	\$5 million	\$2 million	\$7 million



Options for value-based pricing of a cure

- What are the criteria or principles by which we would choose to move to a modified approach?
 - Relative uncertainty in the durability of benefit
 - Magnitude of the health gain and/or cost offset
 - Federal investment in the basic science
 - Size of the intended population
 - Ability to pay through installments

	Cost per year of current Rx	QALY gained	QALY gain price	Cost offset price component	"Value-based" price	Annual payment over 10 years
Standard CEA	\$100,000	50	\$5 million	\$1 million	\$6 million	



Presentations