Million Dollar Therapies for Rare Conditions: Rethinking Value

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What Does Value Mean To Patients?

Clinical Outcomes Are Important

Clinical and quality-of-life improvements traditionally captured within a QALY-based, CEA/CUA analysis, should not be de-emphasized so long as:

1. those values translate to tangible patient experiences
2. Those values are patient-generated
But What About Non-Traditional Patient and Societal Values?

Patient Values

- Diagnostic advances
- Value of hope
- Family, caregivers, and friends
- Societal Inclusion
- Reduction in uncertainty

- Outcomes of altruistic contributions
- Resulting additional therapeutic advances
- Psychological values
- Value of First Treatment
Patient-Focused Values

Diagnostic Advances:
• LTD and diagnostic innovation
• Better coverage of diagnostics
• Newborn Screening eligibility

Value of Hope

Values to the Family:
• Ability to work, attend school, sleep through the night, have hobbies, etc.

Values to Caregivers:
• Better ability to care
• Less burdensome care

Patient-Focused Values

Values to Friends
• Fellow students, colleagues, friends, etc.

Societal Inclusion
• Not just productivity
• Ability to participate in social activities

Reduction in Uncertainty
• Predictability in outcomes and progression of disease
Patient-Focused Values

Values from Altruistic Contributions
• Results from clinical trial participation

Resulting Therapeutic Advances
• Snowball effect

Psychological Values
• Fewer feelings of isolation and worthlessness

Value from first treatment
• Added value in going from no treatments to one, compared to from 5th to 6th

Societal Values
Societal Values

- WTP higher for rare diseases
- Scientific advances
- Health equity
- Community value
- And more...

Thank you.