



Million Dollar Therapies for Rare Conditions: Rethinking Value

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May 22nd, 2018



Alone we are rare. Together we are strong.™



NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.



What Does Value Mean To Patients?



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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



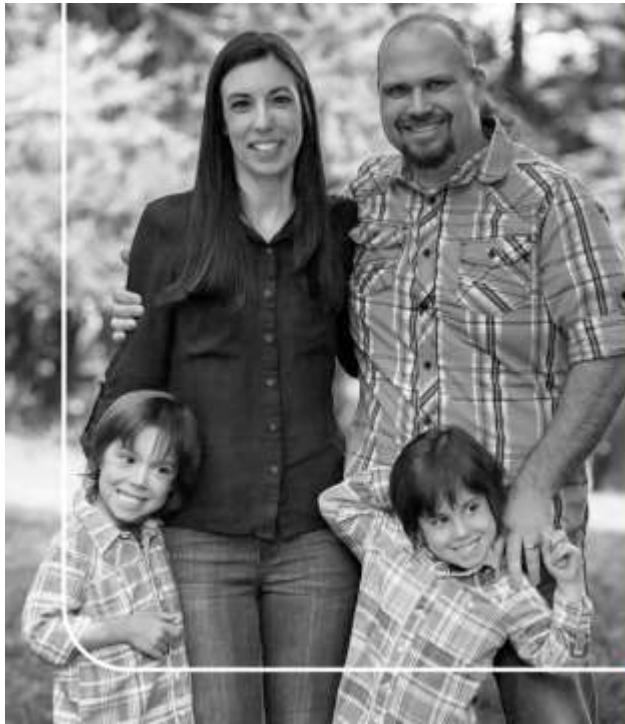
NORD was founded in **1983** along with the Orphan Drug Act

280+ NORD member organizations (& growing each year)

4,100+ RareAction advocates across the country



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But What About Non-Traditional Patient and Societal Values?



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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



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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

7,000
rare diseases exist.

95%
of rare diseases
have NO treatment.

80% of rare
diseases
are genetically based.

NORD
National Organization
for Rare Disorders

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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



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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



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Societal Values



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Societal Values



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



1 in 10
Americans
has a rare disease.
that's **30 million** people
two-thirds of which
are children

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