



Background/Significance

- A rare disease affects fewer than 200,000 people
 - Often life-limiting
 - Often requires constant caregiving.
- Children living with a rare disease are a heterogeneous group, thereby
 - Excluded from research
 - Creating a health disparity.
- New statistical methods overcome this limitation.
- According to the 2013 Shire Rare Disease Impact Report, caregivers experience significant depression, anxiety, stress, isolation, and worry based on future outlook and a lack of information.

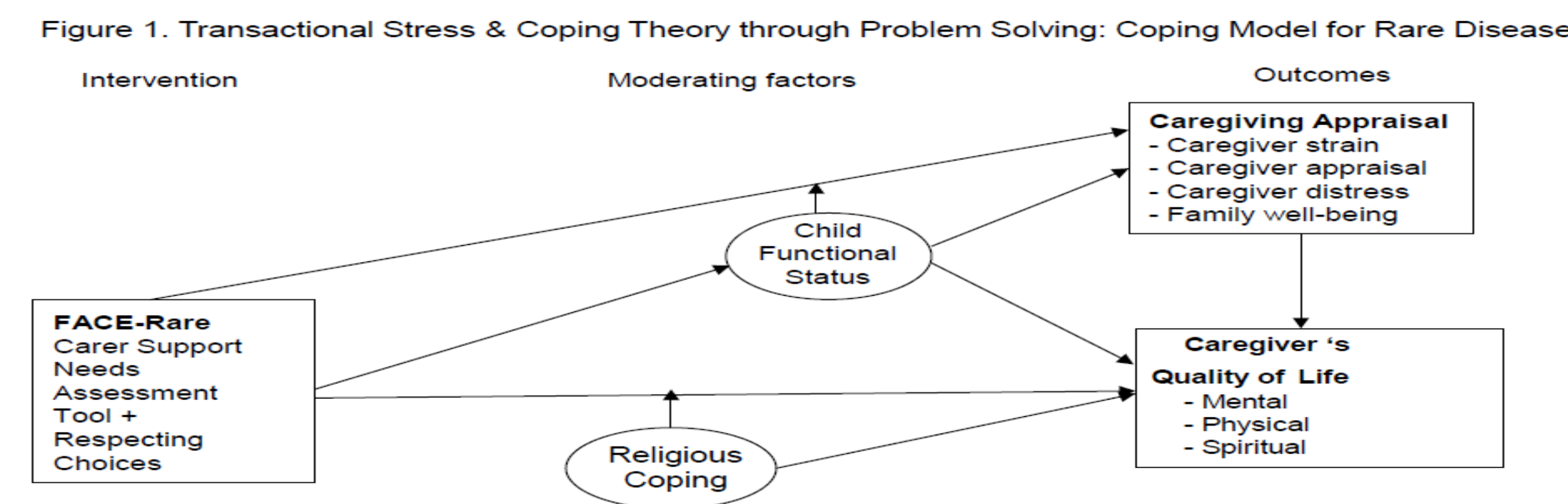
Objective

- To co-create/develop and beta test an intervention, FACE-Rare, designed to shift current clinical practice paradigms using a theoretically-based problem solving approach to:
 - Identify family-identified caregiver needs of children with rare diseases who cannot communicate for themselves.
 - Facilitate family-guided action plans.
 - Create advance care plans for their child.

Methods/Approach

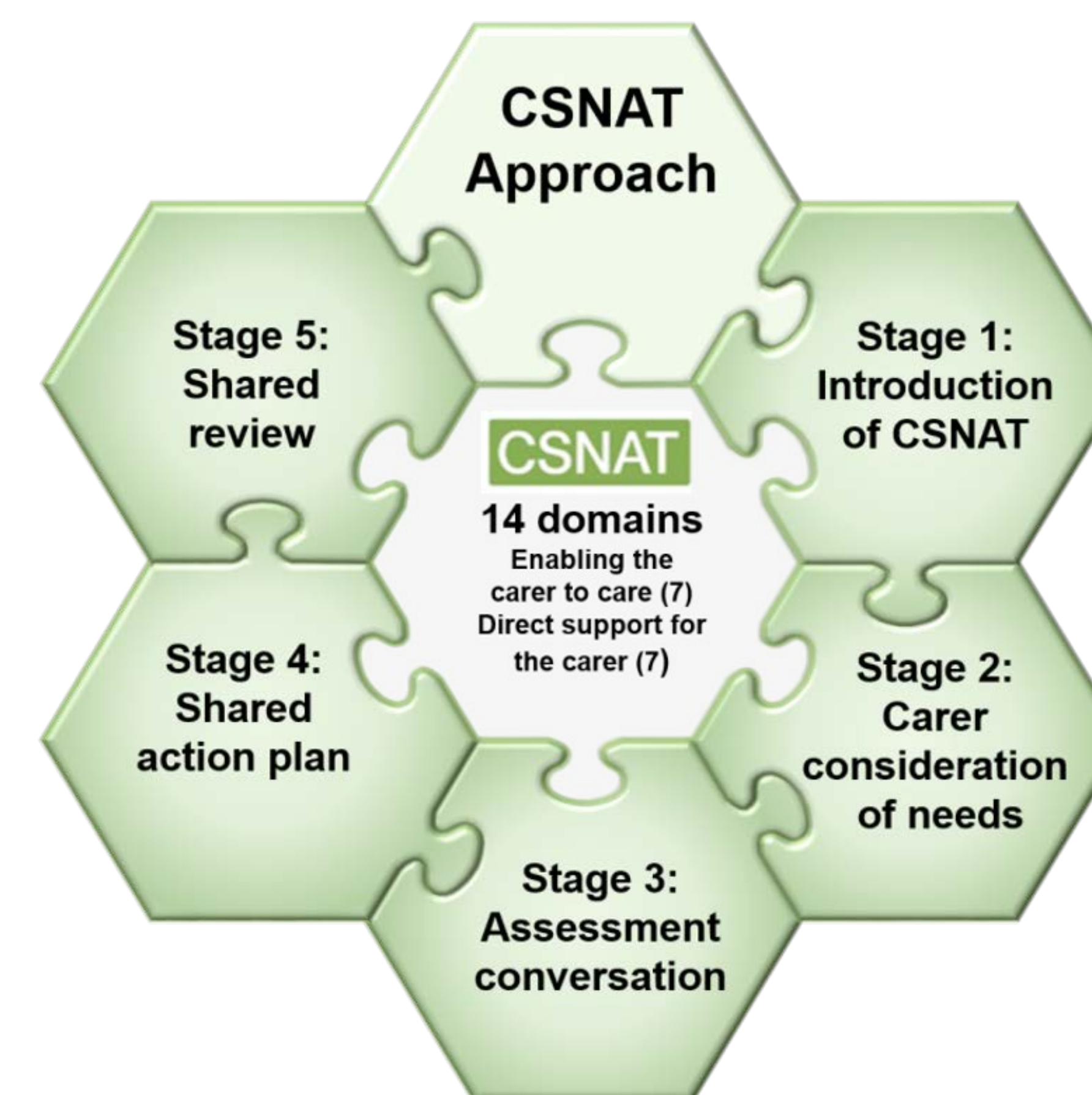
- Phase I: Interviews with key stakeholders to adapt/co-create intervention.**
 - Pediatric version of the Carer Support Needs Assessment Tool (CSNAT)© was adapted, finalized, and copyrighted.
 - Trained facilitator implemented the CSNAT© Paediatric with fidelity—Sessions 1 & 2.
 - Rare version of the Next Steps®: Respecting Choices™ pediatric ACP Conversation was adapted and finalized.
 - Trained facilitator implemented Respecting Choices pediatric ACP Conversation with fidelity—Sessions 3 & 4.
- Phase 2: Beta-testing** integration of two-evidence-based models:
 - Recruitment from outpatient Complex Care Clinic
 - 7 patient/family caregiver dyads were enrolled
- Telemedicine** was used successfully, based on family preference,

Theoretical Model



FACE-Rare Intervention

4 weekly 45-60 minute sessions:
Sessions 1 & 2 - CSNAT Paediatric©
Sessions 3 & 4 - Next Steps®: Respecting Choices™
pediatric ACP Conversation



Respecting Choices®
 PERSON-CENTERED CARE

- Stage 1** assess family's understanding of child's illness: hopes, fears, living well;
- Stage 2** explore family's experiences with child's hospitalization and end-of-life;
- Stage 3** review goals of care and set the stage for future healthcare decisions;
- Stage 4** explore goals for future healthcare, using a situation-based Advance Care Plan document. Unacceptable outcomes are explored;
- Stage 5** Summary; Questions for child's provider identified and written on post-card;
- Stage 6:** Follow-up Plan (Session 4 of this study).

Results – Caregiver Appraisal

Table2. Descriptive Statistics for Subscales of Family Appraisal of Caregiving Questionnaire (FACQ)

Subscale	Baseline (N=7)		2 weeks post-intervention (N=6)	
	Mean	Range	Mean	Range
Caregiver strain	3.1	1.4-4.3	3.6	2.8-4.3
Positive Caregiving Appraisal	4.5	3.6-5.0	4.7	3.9-5.0
Caregiver Distress	2.5	1.3-4.0	2.9	1.8-4.3
Family Wellbeing	3.9	2.5-4.7	4.1	3.2-5.0

Results – Spiritual Quality of Life

Table3. Descriptive Statistics for Subscales of FACIT-Sp-Ex IV

Subscale	Baseline (N=7)		2 weeks post-intervention (N=6)	
	Mean	Range	Mean	Range
Meaning/Peace	21.3	18-26	23.3	17-31
Faith	11.0	2-15	11.0	3-15
FACIT Total Score	66.0	52-78	65.0	50-83

Discussion

- Enrollment was greater than the 50% benchmark of those approached. One family caregiver declined and one withdrew after baseline assessment.
- Family caregivers were female with two male spouses and their child with a rare disease joining Respecting Choices Sessions 3 & 4.
- 86% completion rate indicates high levels of acceptability and feasibility.
- Families preferred to combine Sessions 3 & 4, creating a 3-session intervention.
- Mean Positive Caregiver Appraisal increased from 4.5 to 4.7. (Table 2)
- Mean Family Wellbeing increased from 3.9 to 4.1. (Table 2).
- Mean Caregiver Strain and Caregiver Distress increased at 2-week post-intervention. Reasons for this finding need further exploration.
- Mean score for Meaning/Peace increased from 21.3 to 23.3 (Table 3).
- 100% of 6 families who started FACE-Rare:
 - Completed a written advance care plan.
 - Described their preferences at this time for medical decision making, if their child had a critical health event.

Conclusions

- Technology-driven and medical treatments for life-threatening illnesses have prolonged life for medically fragile children living with rare diseases, leaving families "living on the precipice" with little psychosocial support.
- Need to address the challenges of being a parent of children with rare diseases who cannot communicate.
- Families often engage in complex medical decision-making for their child.
- Families were not too burdened to participate in advance care planning, but *first* wanted to identify their priority palliative care needs and to develop a support plan.

Acknowledgements

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Family Caregivers' Demographics

Variable, Total N=7	N (%)
Age	
Mean (SD) Years	40 (8)
Range Years	(30- 52)
Gender	
Female (Mothers)	7 (100)
Male (Fathers)	2 (29)
Race	
Bi-race	1 (14)
Black/African American	2 (29)
White	4 (57)
Ethnicity	
Not Hispanic or Latino	6 (86)
Hispanic or Latino	1 (14)

Variable, Total N=7	N (%)
Employment Status	
Full-time student	1 (14)
Part-time employed or self-employed	3 (43)
Full-time employed or self-employed	1 (14)
Unemployed	1 (14)
Disability / SSI or SSD	1 (14)
Residents in Household	
Two	1 (14)
Three	1 (14)
Four	3 (42)
Six	2 (28)
Housing Status	
Permanently housed	7 (100)
Marital Status	
Single, never married	2 (29)
Married	5 (71)

Results-Satisfaction

- 100% of participants found the study useful
- 100% of participants found the study helpful
- 6% of participants felt scared as a result of the study
- 26% of participants felt sad as a result of the study



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