

UNMET NEEDS AMONG PATIENTS WITH TRANSTHYRETIN AMYLOIDOSIS (ATTR) AND CALL FOR ADDITIONAL RESEARCH

Makenna A. Paule^a, Jocelyn M. Ashford^a, Muriel Finkel^b, Dena Heath^c
^aBridgeBio Pharma; ^bAmyloidosis Support Groups; ^cAmyloidosis Research Consortium

BACKGROUND

Transthyretin (TTR) amyloidosis (ATTR) is a progressive, debilitating form of amyloidosis caused by the aggregation and deposition of misfolded TTR protein in organs and tissues, resulting in damage and dysfunction. Evidence suggests that ATTR is markedly underdiagnosed. In addition, the needs of this patient population have not yet been thoroughly studied.

METHODS

To understand the unmet needs of ATTR patients, a series of four virtual focus groups were conducted with individuals (n=11; 1 female, 10 male) with ATTR (7 ATTRv; 4 ATTRwt) who were geographically dispersed across the U.S. and represented a range of demographic profiles, pathways to diagnosis, and access to care and treatment. The focus groups were conducted using methods commonly employed in process-oriented psychotherapeutic support groups.

Demographic Information					
Demographic Profile		n (N=11)	Demographic Profile		n (N=11)
Age (years)	41-55	1	Community	Rural	3
	56-70	1		Urban	3
	71+	9		Suburban	5
Race	White	10	Type of Insurance	Private	2
	Black	1		Medicare	9

Figure 1. Focus Group participant demographics

RESULTS

Unmet needs were demonstrated in the following dimensions of care and quality of life: material and physical well-being; relationships with other people; social, community, and civic activities; and recreation (fig. 2). Relevant experiences along the patient journey are summarized in fig. 1. Focus group participants also provided recommendations regarding opportunities to address these unmet needs with targeted, collaborative efforts between industry, advocacy groups, and health care professionals.

CONCLUSION

The findings from focus groups among patients with ATTR indicate that additional research on the effects of this rare, chronic disease on mental health and the effectiveness of prescribed treatments and interventions is necessary.

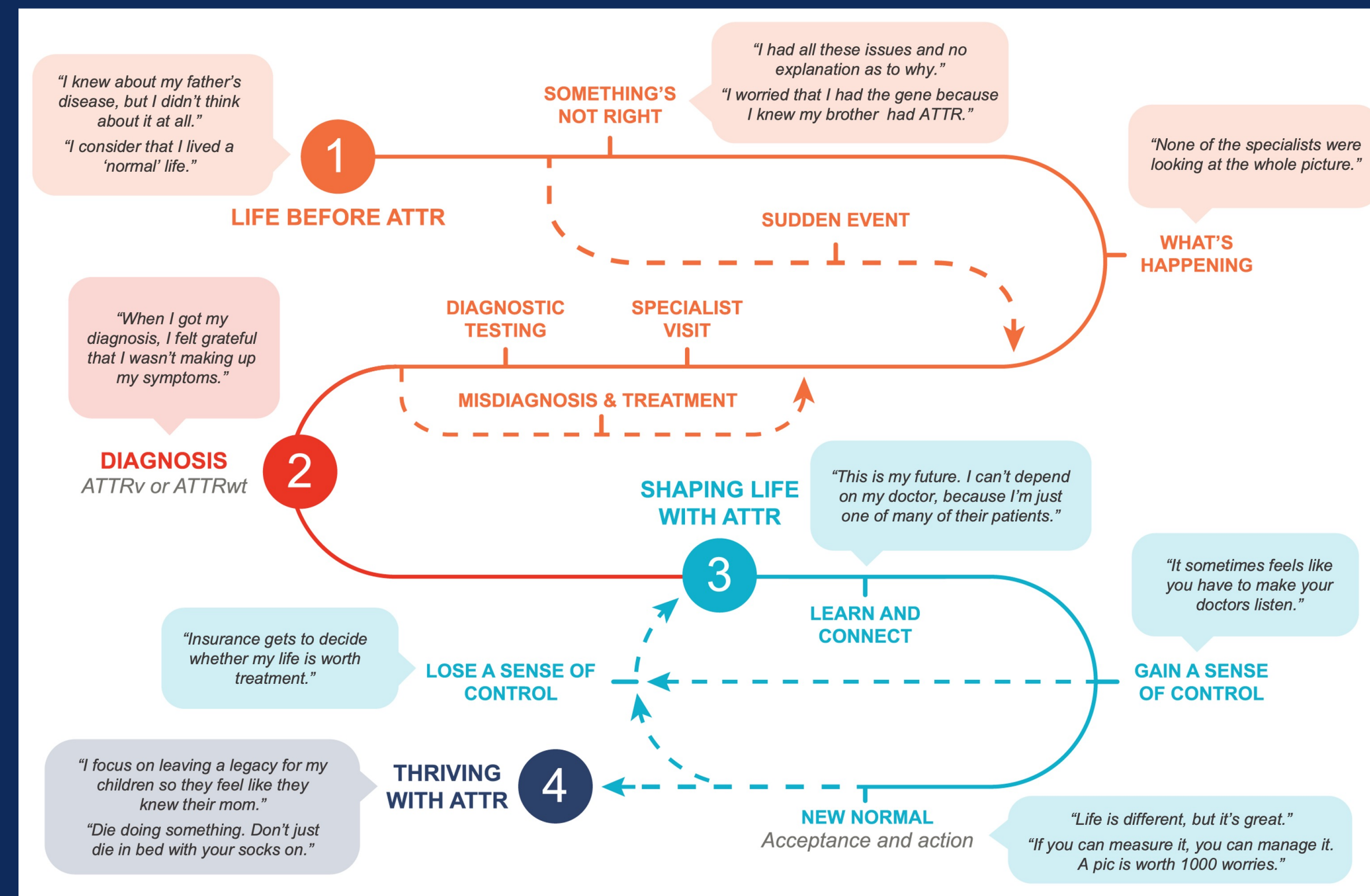


Figure 2. ATTR Patient Experience Map validated by focus group participants including direct quotes from ATTR patients.

“It is impossible to understand or explain the feeling one gets when you are told you have a terminal illness, meaning it can’t be fixed. Of course, we are all mortal, we are all dying, but hope still persists.”
 – Focus Group Participant



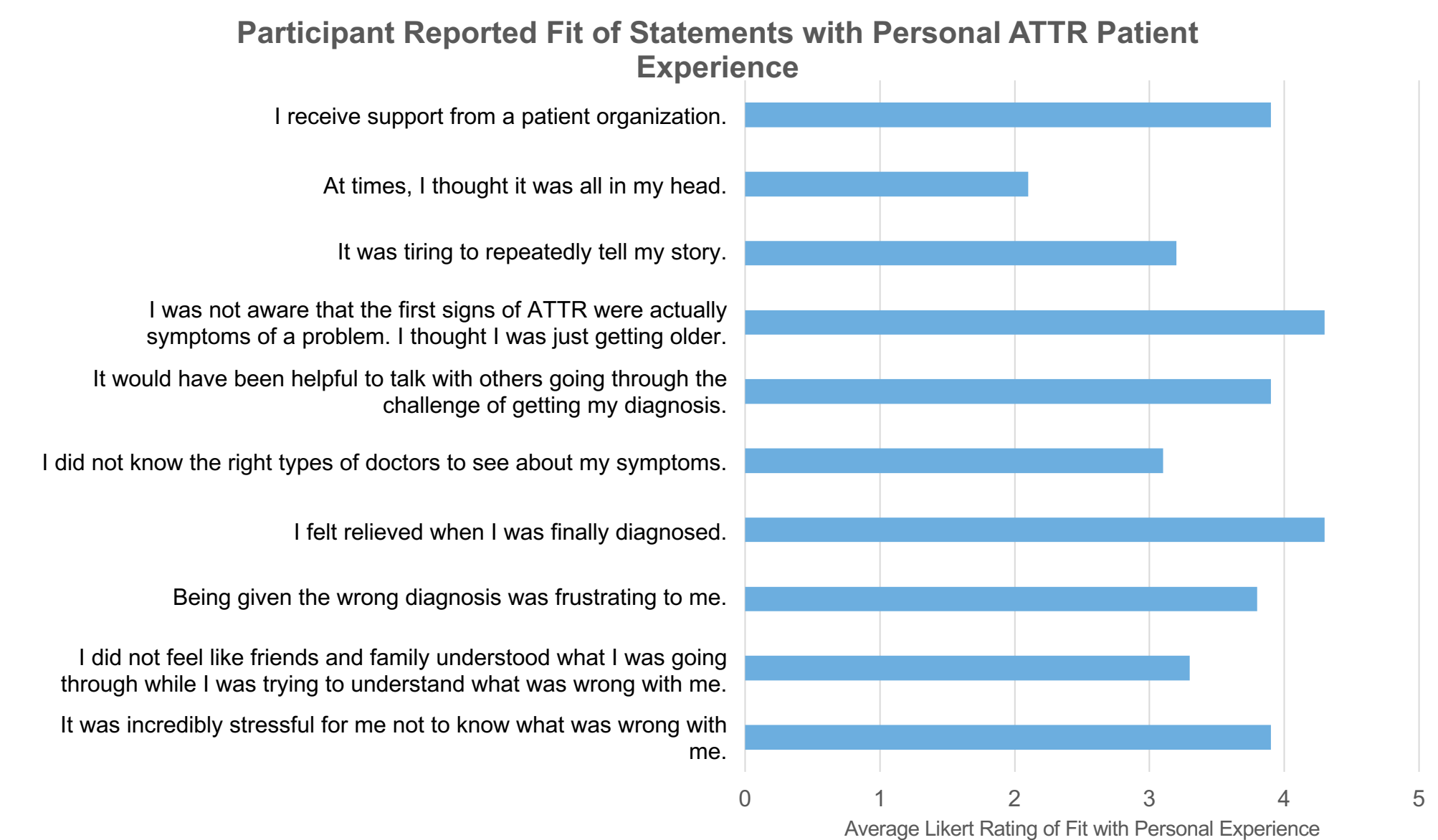
For more information, scan the QR code, or email MedInfo@eidostx.com.



DISCUSSION

Participants reported variable experiences of disease onset and progression, diagnostic processes and timelines, and experiences post-diagnosis. However, some experiences (fig. 3) related to unmet needs were common across the patient journey (fig. 2). It was determined that ATTR has a significant negative impact on participants’ quality of life (fig. 4). Participants suggested that advocacy organizations, industry, and healthcare teams collaborate to develop resources such as psychotherapeutic support groups, accessible educational materials, tools for tracking disease progression, and peer-to-peer mentorship and support programs.

FIGURE 3



Average Likert rating of fit of statements with personal experience as an ATTR patient reported by focus group participants.

FIGURE 4

QOLS Category	% Focus Group Participants who reported negative impacts due to ATTR
Material & Physical Well-being	100%
Relationships with other people	27%
Social, Community, and Civic Activities	45%
Personal Development and Fulfillment	0%
Recreation	45%

Percentage of focus group participants who reported negative impacts on dimensions of quality of life specified by the Flanagan Quality of Life Scale due to ATTR.

DISCLOSURES

Makenna Paule and Jocelyn Ashford are employees of BridgeBio Pharma, Inc.

