

Isabelle Lousada¹, Kelsey Barrell², Ruth Masterson Creber³, Dena Heath⁴, Lisa Mendelson⁵, Michael Pollock⁶, Jason Shore⁷, Sascha Tuchman⁸, Melissa Warner¹, Stephen Heitner⁹

¹Amyloidosis Research Consortium, Newton, MA, USA; ²University of Utah, Salt Lake City, UT, USA; ³Weill Cornell Medical Center, New York, NY, USA; ⁴Northern California Support Groups, San Francisco, CA, USA; ⁵Boston University School of Medicine, Boston, MA, USA; ⁶Akcea Therapeutics, Boston, MA, USA; ⁷Patient Discovery, Boston, MA, USA; ⁸University of North Carolina, Chapel Hill, NC, USA; ⁹Oregon Health and Sciences University, Portland, OR, USA

BACKGROUND

- Amyloidosis is a group of complex, progressive and life-threatening diseases.
- Patients often feel overwhelmed by the complexity of their symptoms, treatments, and care management.
- Amyloidosis can affect the heart, kidneys, liver, nervous system, and digestive tract, leading to multiple physician appointments, and complexities in care.
- Symptoms of, and treatments for, the leading types of amyloidosis are known to affect health-related quality of life but may not be evaluated as part of a routine visit.
- Understanding the patient's perspective and goals is essential to the development of a comprehensive strategy for care.
- The Amyloidosis Appointment Companion (AAC) was created to help patients identify their goals of care, changes in their condition, and challenges they are facing and to share these with their healthcare providers (HCPs).
- The AAC has been collaboratively developed by the Amyloidosis Research Consortium and Patient Discovery.
 - The Amyloidosis Research Consortium (ARC) was founded in 2015 with its mission dedicated to changing the way research is being done and focusing on what will have the greatest impact on improving the lives of patients.
 - Patient Discovery is a leader in patient-provider decision technologies and the tools being developed are aimed at improving the way patients with complex conditions are understood, supported, and treated.

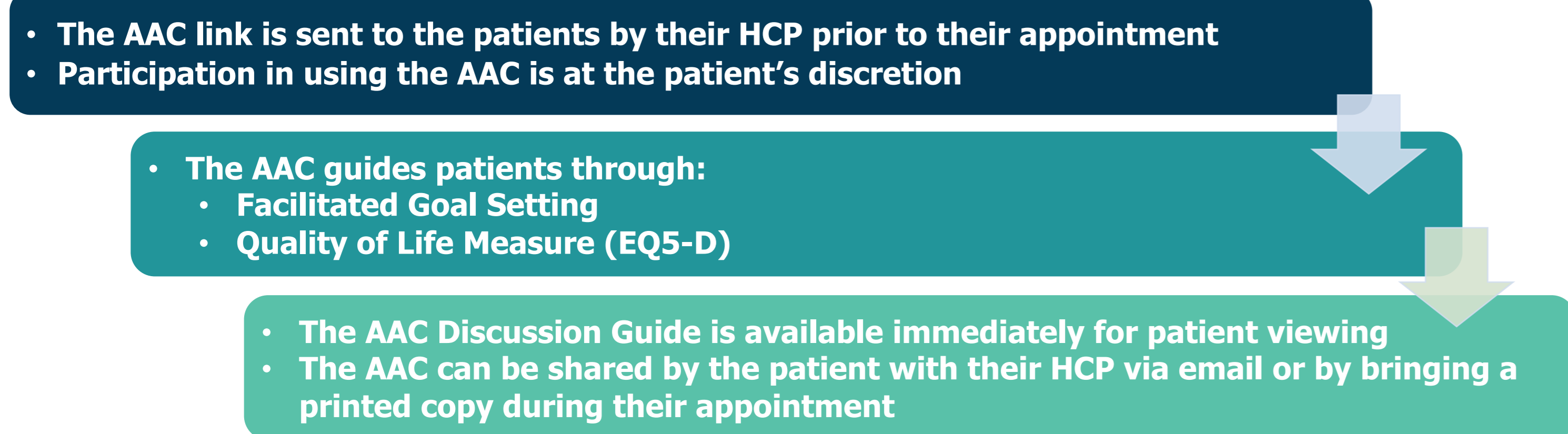
OBJECTIVE

- To evaluate the feasibility of the AAC as a tool to enable patients to identify their concerns, healthcare priorities, and goals and share these with their HCP with the aim of improving quality of care.
- This abstract reports findings and feasibility data from the first pilot study of the AAC with 25 patients.

METHODS

- Patients were invited to sign into the AAC either by their healthcare provider prior to their appointment, or through their patient support group leader.
 - The AAC is a HIPAA compliant online tool.
- Patients are guided through an interactive survey to capture basic demographics, type of amyloidosis, current treatments, treatment satisfaction, and quality of life (using the EQ-5D).
- Patients are then taken through two sections which identify their concerns and goals of care.
- Patients have the additional opportunity to share their questions and concerns using their own words in free text boxes allowing a better understanding of their needs and expectations. This qualitative data can be used in conjunction with the clinical data collected by HCPs to create a more holistic view of the patient and the management of the disease.
- Answers are compiled and prioritized in a one-page 'Discussion Guide.' This can be shared either directly through email with their HCP prior to their appointment or can be printed and shared during the appointment.

Workflow



- A steering group of amyloidosis patients, HCPs, and industry representatives was assembled to evaluate aggregated de-identified data, discuss opportunities to improve care and oversee data collection. The AAC was tested in a soft launch with 4 amyloidosis centers within the US to review the dissemination process and collected data.

RESULTS

Participants

A total of 25 amyloidosis patients were successfully referred to the AAC, used the tool, and created their Discussion Guide.

- They were referred by four amyloidosis centers, as well as one amyloidosis patient support group in the US [figure 1]

The Participants self-identified their type. Most respondents had AL amyloidosis.

- 68% AL amyloidosis
- 12% Hereditary TTR amyloidosis
- 8% Wild-type TTR amyloidosis
- 12% Untyped amyloidosis

They also indicated whether they were currently on any treatment for their amyloidosis or not. 12 reported being newly diagnosed [figure 2].

Figure 1: How Patients Were Referred to The AAC

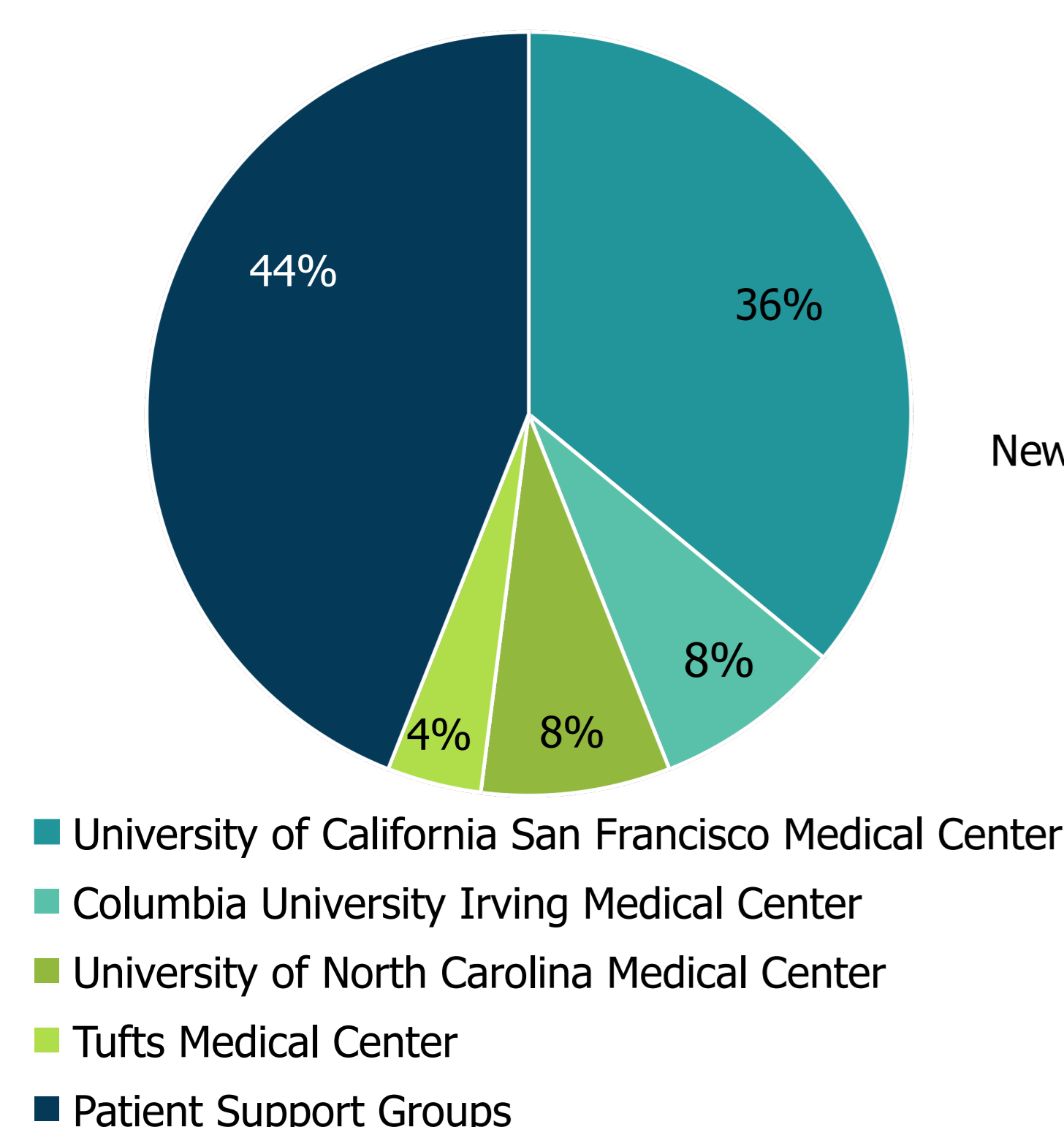
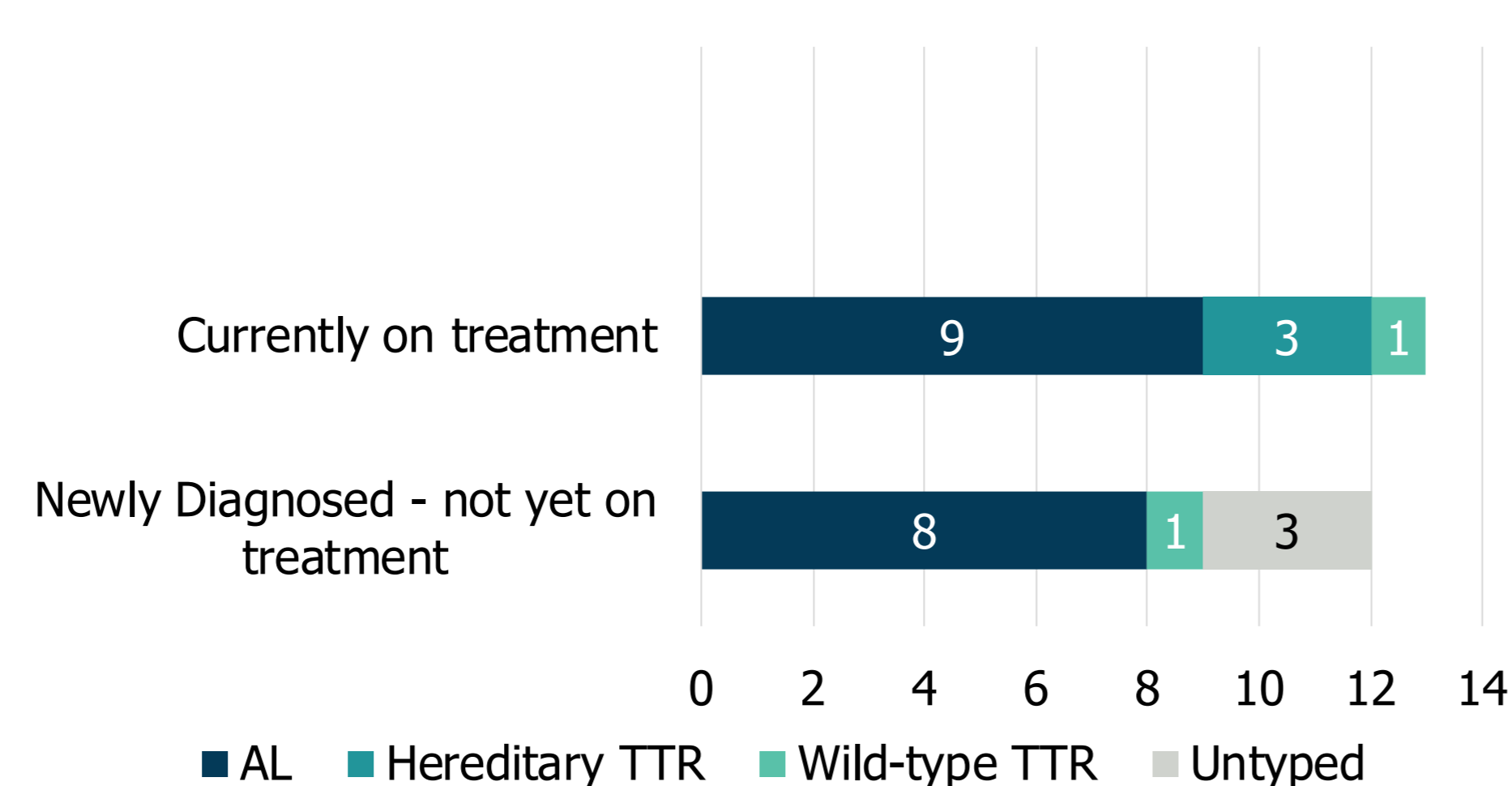


Figure 2: Patients' Diagnosis & Treatment



Treatment Satisfaction and Quality of Life

While responses to the EQ-5D quality of life measures varied, a majority of the patients, 64% (N=16), reported moderate levels of anxiety or depression [figure 4]. Over half of the patients, 52% (N=13) reported being on treatment [figure 2]. Of these 13 patients, 46% (N=6), reported being satisfied with their treatment while 54% (N=7), reported being neutral or dissatisfied [figure 3].

Figure 3: Treatment Satisfaction

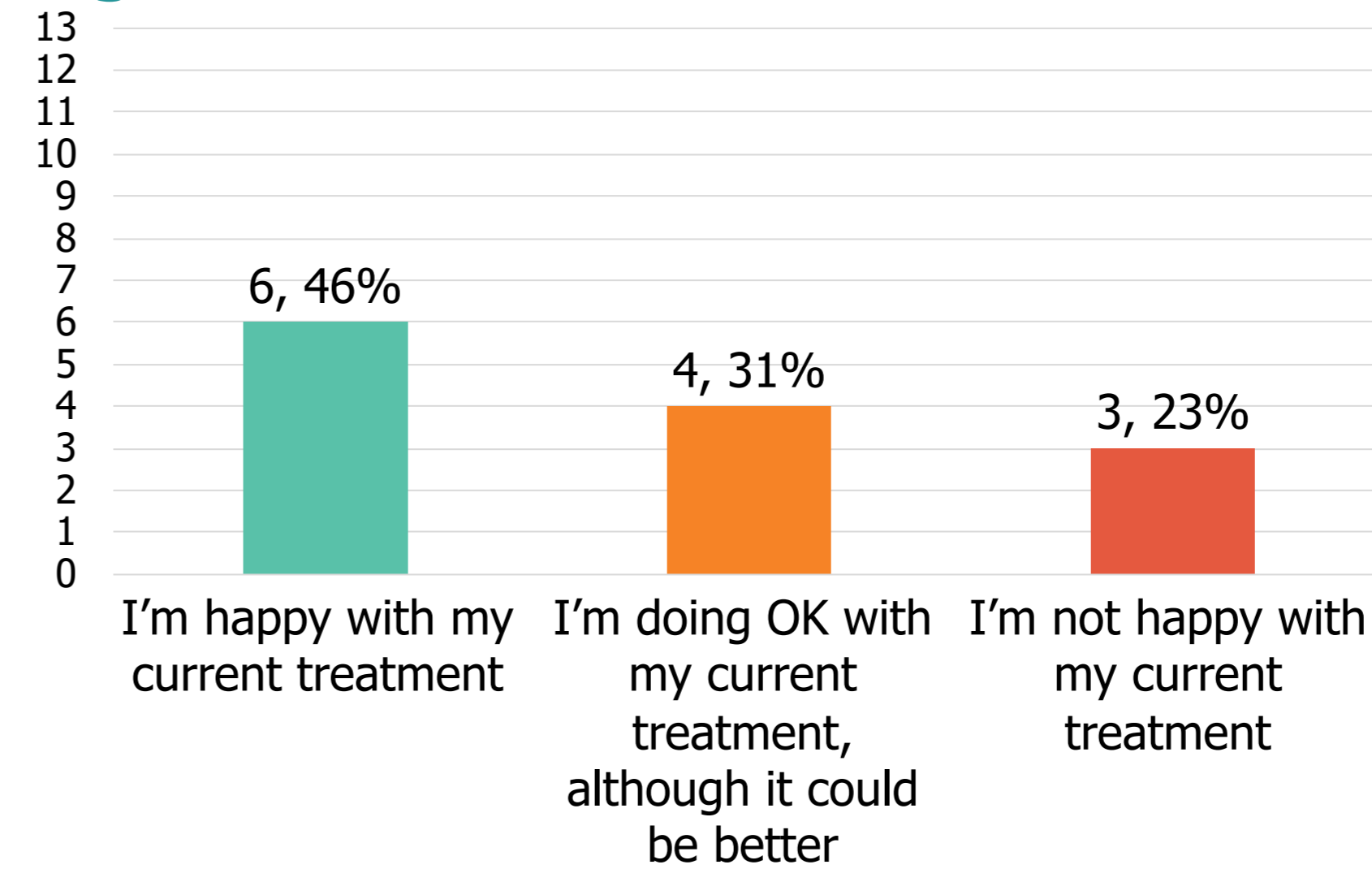
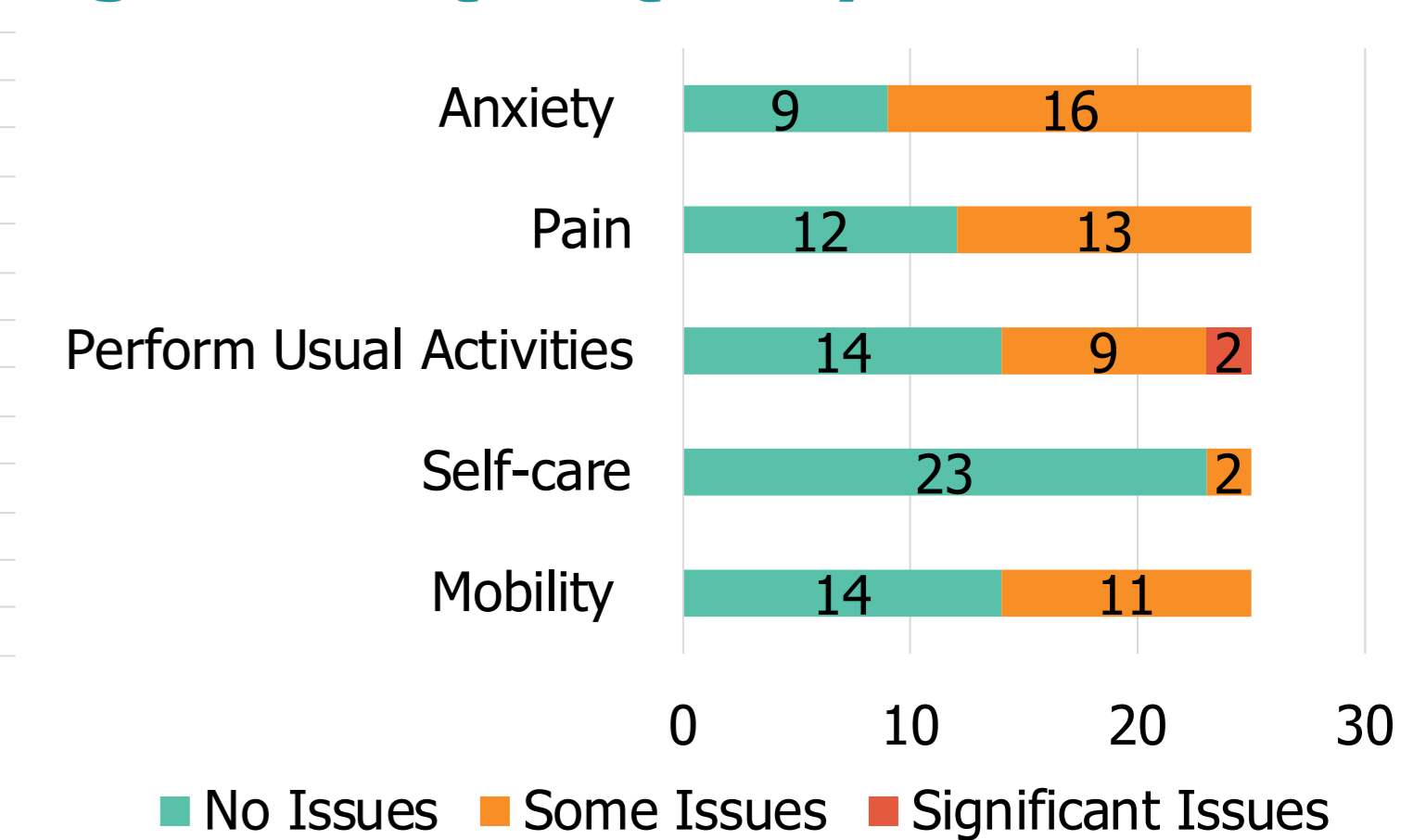


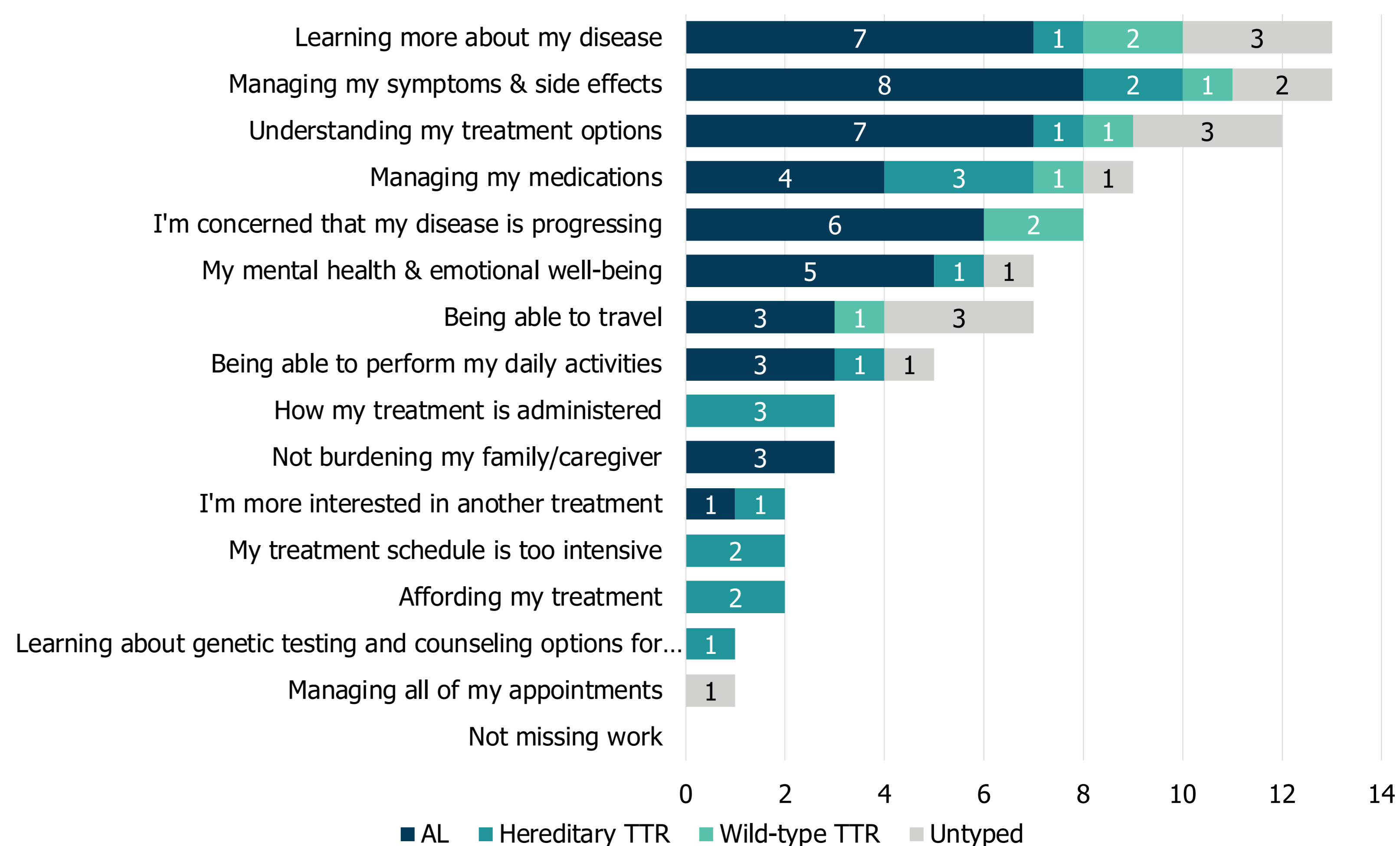
Figure 4: EQ5D Quality of Life Measure



Selected Discussion Topics

Selected discussion topics varied widely between types. Over half the patients, 52% (N=13), selected 'learning more about their disease' as a topic to discuss. 'Managing symptoms and side effects' was also a priority for many, 52% (N=13), as was 'understanding treatment options,' 48% (N=12).

Figure 5: Topics Patients Selected to Discuss at Their Upcoming Appointment



Self Reported Goals of Care

Two main types of reported goals of care were identified: 1) short term, isolated goals (such as attending a specific life event) and 2) long term, lifestyle goals (such as regaining exercise tolerance or living independently).

Table 1: Short Term Goals

- "want to see my twin granddaughters born at Christmas."
- "To travel to Ohio for my granddaughter's wedding in mid-July"
- "better understanding of current medical condition"
- "More convenient treatment. Intervals, method & location"
- "Good control of diarrhea."
- "discuss side effects of my treatment"

Table 2: Long Term Goals

- "To live independently in my own apt"
- "To participate in my normal daily social activities, with fewer side effects (fatigue, occasional loose bowels, neuropathy, occasional nausea)."
- "Optimizing cardiac and renal recovery to the point where I can resume lap swimming. Having sufficient stamina to travel overseas for pleasure or business, including destinations as far as Asia, while taking account of my age (72) and health."
- "Many goals, but right now, would like to focus on walking normally. Of course I'd like to explore experimental treatments, drug options, etc."
- "Gain strength and muscle mass. Improve balance"
- "Ability to play tennis. Control breathing."

CONCLUSION

- Both patients and HCPs report the tool enhanced communication during appointments.
- Feedback from HCPs and the steering group highlight the particular importance of asking questions relating to patients' goals of care as well as their treatment satisfaction. These questions provide novel information that is crucial for managing individual patient expectations, treatment decisions, and appointment schedules.
- Outcomes from this tool are useful to both patients and HCPs for improving individual patient care through identification of priorities, concerns, and misconceptions.
- Furthermore, review of aggregated data with industry and physicians shows promise for improving patient care.
 - Insights from this data provide powerful information that may help improve the development of assistance programs, clinical trials, and research.
 - Insights from this limited study population show there is a great unmet educational need for patients.

