Supporting adults living with mucopolysaccharide (MPS) diseases: Understanding current experiences and future challenges

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Introduction

Mucopolysaccharide (MPS) diseases are rare, inherited metabolic disorders caused by lysosomal enzyme deficiency.

They are life limiting, progressively debilitating conditions.[1]

There is no curative treatment but the improvement in diagnosis, care and the use of novel therapies have slowed disease progression and, as a result, more people are living into adulthood.[2]

Information about how adults are affected by MPS disease is limited.

Aims

This study aimed to understand:

- The challenges encountered by adults living with MPS disease
- How best to support them with their condition

Methods

Members of the MPS Society were invited to take part in an online survey investigating their views about living with MPS disease.

Participants were asked about:

- What they found difficult and challenging about living with their condition at present
- What they thought would be the biggest challenges or difficulties in the future
- What areas they felt they would need the most support in

Qualitative data was collected between September–October 2018.

Data included in this analysis comprise a subset of responses from adults (ages 16 years and over) with MPS.

Responses were analysed by applying an inductive thematic-content approach.

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Results

Patient characteristics

27 adults with MPS were included in this study

The study included adults with MPS I (Hurler, Hurler Scheie, Scheie; n=8), MPS II (Hunter; n=4), MPS IV (Morquio; n=13) and MPS VI (Maroteaux Lamy; n=2) (Figure 1).

Participants were divided into age categories which ranged from 16 to 64 (Table 1).

Table 1. Participants age categories, survey completions and overall percentage

Age categories	No. of survey completions	Percentage
16–19	3	11.1%
20–24	4	14.8%
25–34	8	29.6%
35–44	7	25.9%
45–54	4	14.8%
55–64	1	3.7%

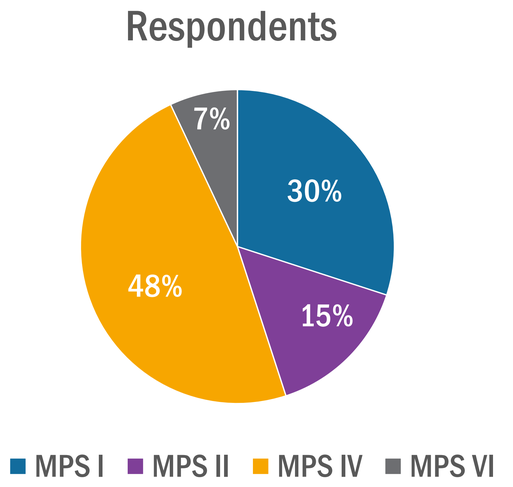


Figure 1. Study respondents' disease categories

Participants experience of living with MPS disease

More than 25% of participants were worried about getting older and how the progressive deterioration of their functions would affect them in the future.

The four most common challenges reported by participants included:

Loss of mobility/being unable to perform every-day tasks (37%)
"Losing my freedom"
"Restricted mobility affects my everyday life"
"Being unable to exercise"

Coming to terms with their condition (19%)
"Accepting the truth"
"Psychological adapting"
"Mental health, trying to understand and come to terms with my condition"

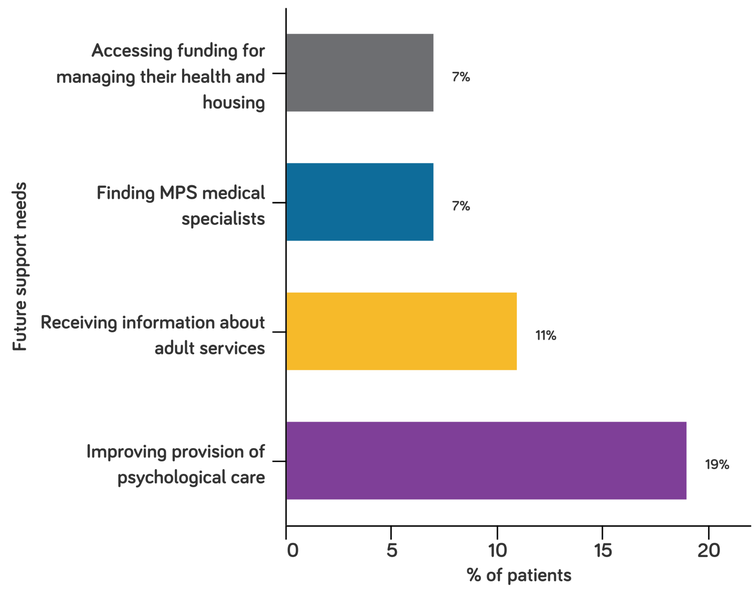
Being unable to find a job (15%)
"Finding a job that is compatible"
"Finding an employer that will see past my disability"
"I get tired and that affects my ability to work"

Losing their cognitive capabilities (7%)
"Ageing and loss of functions"
"Intellect deteriorating"
"Cognitive functionalities"

Future challenges

Common areas whereby patients felt support would be beneficial included help with funding for health and housing, accessing MPS specialists, adult services information and mental health (Figure 2).

Figure 2. Future support needs reported by adults with MPS disease



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Conclusion

The study has underlined that adults with MPS diseases are facing on-going difficulties and uncertainties as they age with their condition.
These difficulties are becoming more commonplace within the MPS community, as individuals are living longer and ageing with their condition.
Provisioning support that is tailored to individual needs would be an important aspect of improving quality of life and mental wellbeing in adults with MPS disease.