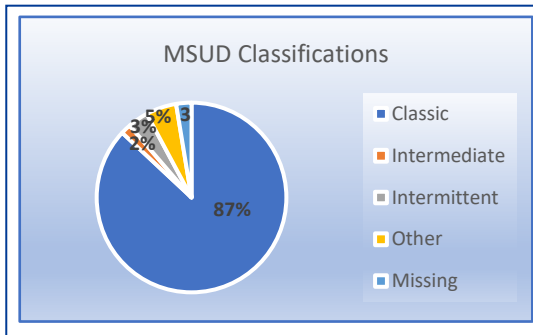


# Key Findings From the MSUD Registry



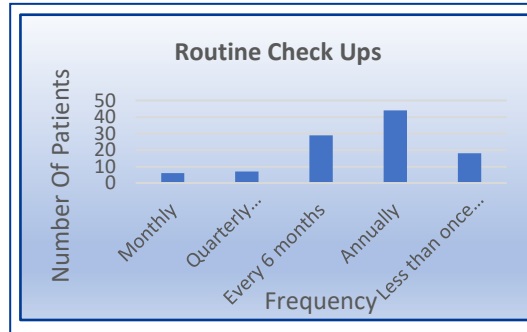
## MSUD Classifications

A clear majority of respondents indicate that they have "Classic" MSUD.



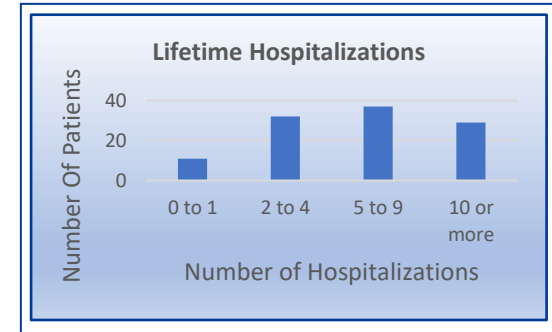
## Routine Visits To A Metabolic Specialist

Most MSUD patients visit their metabolic twice or once a year.



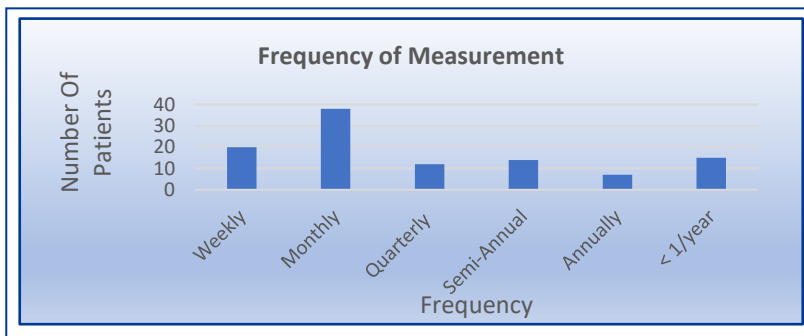
## Lifetime Hospitalizations

Most MSUD patients have been hospitalized at least five times in their lifetime..



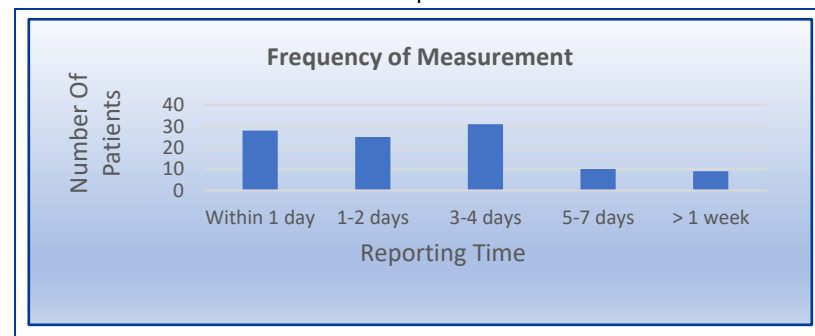
## Frequency of Blood Amino Acid Measurement

About half of MSUD patients measure their blood amino acid levels each week or month.



## Timeliness of Blood Amino Acid Measurement Results

About half of measurement results are reported within two days, however some results are not reported for more than a week.



# Key Findings From the MSUD Registry



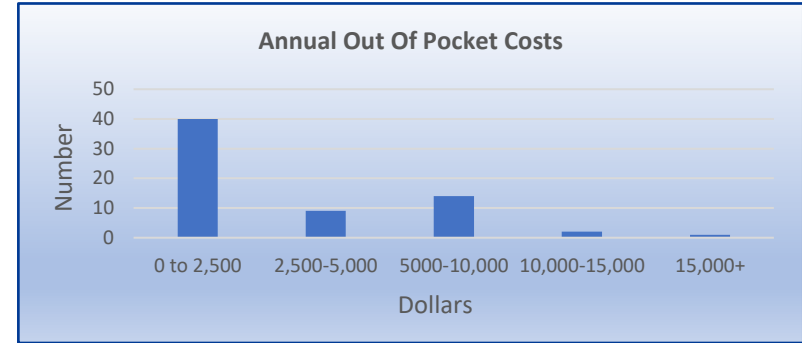
## Adherence To Diet

Most MSUD patients are “faithful” to their diet.

Prescribed diet statement	No.
The participant consistently consumes all daily formula and strictly follows their leucine/protein prescription and diet	47
The participant sometimes deviates from their daily formula or leucine/protein prescription and diet by minor amounts	27
The participant often deviates from their daily formula or leucine/protein prescription and diet by significant amounts	1
The participant generally does not follow a daily formula, their leucine/protein prescription, or diet	12

## Annual Out Of Pocket Costs Resulting From MSUD Diagnosis

Out of pocket costs vary widely but is most often less than \$2,500.



## Metabolic Formula's Used By MSUD Registry Participants

Over one-half of respondents reported using “Complex Essential MSD” or “Complex Amino Acid Blend MSD” as their metabolic formula.

Complex Essential MSD	Complex Amino Acid Blend MSD	Ketonex – 2	Complex Junior MSD	MSUD Cooler	Lophlex	BCAD 2	MSUD Gel	Complex Amino Acid Bars MSD	Anamix MSUD Junior	Ketonex – 1	MSUD Maxamum
39	24	12	10	6	5	4	3	2	1	1	1

Note: Nearly one-third (42 of 150) responses did not report which formula they used.

## Other Disorders Report By MSUD Registry Participants

Most respondents reported having been diagnosed with a behavioral disorder.

None	Anxiety Disorder	ADHD	Intellectual impairment	Mood Disorder	ADD	Panic Disorder	PTSD	Other	Major Depressive Disorder	Bipolar Disorder	Autism	Obsessive Compulsive Disorder	Personality Disorder	Eating Disorder	Schizophrenia
40	27	13	11	10	8	8	6	6	5	4	3	3	3	2	2

Note: twenty-two (of 173 respondents did not report a result.