Mixed Methods: Using survey data and qualitative interviews to support content validity for **Spinocerebellar Ataxia (SCA) patients**

Background

- > Qualitative research enables an in-depth understanding of the patient experience,¹ however, attaining a sufficient sample size to determine the content validity of a Clinical Outcomes Assessment (COA) instrument can be challenging, especially in rare diseases.
- > The FDA Patient Focused Drug Development guidance mentions patient surveys as an appropriate method to inform content validity and add greater depth to data in rare diseases as part of a mixed methods analysis approach.²
- > We utilized survey data along with concept elicitation interview data from semi-structured interviews to achieve concept confirmation to support content validity for three COAs in the SCA population.

Methods

- > A cross-sectional survey was completed by 145 patients with Cerebellar Ataxia (CA). Respondents described the symptoms and quality of life (QoL) impacts associated with their disease.
- > Domains and concepts elicited from the surveys were utilized to inform questions in a semi-structured concept elicitation interview guide. Interviews were conducted with seven (n=7) SCA patients to explore patients' experiences of SCA including symptoms and impacts to daily living. Content analysis was used to assess the interviews via Atlas.Ti. Unique concepts reported by patients were identified and organized into a conceptual model.
- > Concept confirmation was evaluated by mapping concepts collected from both data sources (interviews and survey) against the three COAs of interest (f-SARA, PIFAS, FARS-ADL).

Figure 1. Overview of mixed methods approach

Survey (N=145) to elicit key domains and patient experiences

Interviews (N=7) for in-depth concept elicitation

Evaluation of content validity from survey and interview results

REFERENCES

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2. Patient-focused drug development guidance series for enhancing the incorporation of the Patient's voice in medical product development and regulatory decision making. (Food and Drug Administration)<u>https://www.fda.gov/drugs/development-approval-process-</u> drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporationpatients-voice-medicalDate accessed: April 3, 2024

Results



A A	A total of 40 signs/sy rom the interviews; & he survey (total of 68 domains and 60 impa sources). All concept PIFAS) were endorse I). A depth of informatio nterviews, while surve domains. For example stance/balance were additional stance/balance stance/balance were additional stance/balance stance/balance were additional stance/balance stance/balance were additional stance/balance the combination of unique and/or interviews tha The combination of so provided cumulative	mptoms and 44 impacts we 50 signs/symptoms and 30 5 unique signs/symptoms a acts across 7 domains from ts in the COAs (f-SARA, FA ed by the surveys and inter on was captured for individu vey data elucidated a broad le, although concepts relate originally elicited in the sur ance concepts were report ure 2 provides an overview e concepts elicited from the survey and interview data s evidence of domain releval pects related to patient exper-	ere reported impacts from across 17 h both RS-ADL, and views (Table al concepts in der range of ed to rvey, six ed from the v of the e survey As. ources nce and a	Activities of dai	ly life (i.e. perso activities)	nal care, dail Ir s	y nterview and urvey, 2	Stance	/balance	Survey	
ı Tablı	arger breattri of asp	firmation via nationt survey	and interviews	Interview only, 2	12	0	nly, 1	Intervie	ew and survey,	3 only, 1	
Item	Domain	Item concept	Confirmation from both patient survey	Dexterity		Swallowing			Bodily	Falls	
No.			and interview								
1 2 3 4 Instru	Gross motor function Speech	Gait/walking Stance Sitting Speech Rating Scale, Activities of Daily Living D	Of Ataxia (f-SARA) √ √ Omain (FARS-ADL)	Interview only,	Interview and						
1	Speech Swallowing	Swallowing		2	survey, 2		Interv	iew and	Interview and	Interview and	
3		Cutting Food and Handling Utensils	\checkmark	Sitting		Survey only,	2 surv	vey, 2	survey, 2	survey, 2	
4 5 6 7 8 9	Activities of daily living Gross motor function Bodily function	Dressing Personal Hygiene Falling Walking Quality of sitting position				Gait/walkin	g		Speech	Energy	
Instru	ument 3: Patient Impression	of Function and Activities Scale (PIFAS)							Intervi	
1	Energy	Tired	\checkmark	Interview only,		Interview ar	nd S	urvey	Interview and	and	
2	Gross motor function	Walking Climbing stairs		2	Survey only, 2	survey, 2		only	survey, 2	survey, 1	
4 5	Energy	Standing Lack of energy	✓ ✓	The size of and numerical value in each box corresponds to the number of							
6	daily living	Unable to do usual activities	\checkmark	unique si	gn and sympton	n concepts tl	hat was e	elicited f	from each data	asource	
7	Speech	Difficulty speaking	\checkmark		(9	survey, interv	view, or	both).			
8	Impact to emotional	Frustrated when people don't	\checkmark								
0	function	understand what I'm saying		Conclusions							
9 10	спегду	Trouble turning around	V				1				
11	Gross motor function	Fall down	\checkmark	Survey data wa	as combined with	qualitative in	terviews	as a mix	ed method app	roach to	
12	Swallowing	Swallowing	\checkmark	support the cor	ntent validation of	t three SCA C	OA instru	iments u	sed in a clinica	I trial.	
13	Impact to activities of daily living	Need help doing usual activities		This approach may be preferable to elicit a more comprehensive perspective of the patient experience, especially when recruiting large samples for qualitative interviews may be							
14	Swallowing	Lough when eating	✓			3 4 90 0		- 75010			

 A total of 40 signs/symptoms and 44 impacts were reported from the interviews; 50 signs/symptoms and 30 impacts from the survey (total of 65 unique signs/symptoms across 17 domains and 60 impacts across 7 domains from both sources). All concepts in the COAs (f-SARA, FARS-ADL, and PIFAS) were endorsed by the surveys and interviews (Table 1). A depth of information was captured for individual concepts in interviews, while survey data elucidated a broader range of domains. For example, although concepts related to stance/balance were originally elicited in the survey, six additional stance/balance concepts were reported from the interviews alone. Figure 2 provides an overview of the contribution of unique concepts elicited from the survey and/or interviews that are measured by the COAs. The combination of survey and interview data sources provided cumulative evidence of domain relevance and a 			Activities of daily life (i.e. personal care, daily activities) Interview and survey, 2				Stance/balance			
larger breadth of aspects related to patient experience.			Interview only	Interview only 12			Su Interview and survey 3		Survey	
I able Item No.	Domain	rmation via patient survey Item concept	Confirmation from both patient survey	Dexterity		Swallowin	g		Bodily functions	Falls
Instrum 1 2 3 4	Gross motor function	Scale for the Assessment and Rating Gait/walking Stance Sitting Speech	of Ataxia (f-SARA) ✓ ✓ ✓ ✓ ✓							
Instrum 1	nent 2: Friedreich's Ataxia Ra Speech	Swallowing	Domain (FARS-ADL) ✓	Interview only, 2	Interview and survey, 2		Intervi	ew and	Interview and	Interview and
2 3 4	Activities of daily living	Cutting Food and Handling Utensils Dressing		Sitting		Survey onl	y, 2 surv	ey, 2	survey, 2	survey, 2
5 6 7 8	Gross motor function	Personal Hygiene Falling Walking Quality of sitting position	\checkmark			Galt/Walk	ing		Speech	Energy
9 Instrur	Bodily function	Bladder function	✓ ✓							Intervi
1 2	Energy	Tired Walking		Interview only, 2	Survey only. 2	Interview a survey.	and Su	urvey only	Interview and survey. 2	and survev. 1
5 5	Energy	Standing Lack of energy	✓ ✓ ✓	The size of and numerical value in each box corresponds to the number of unique sign and symptom concepts that was elicited from each data source						
6	Impact to activities of daily living	Unable to do usual activities	\checkmark							
7 8	Speech Impact to emotional	Difficulty speaking Frustrated when people don't	 ✓ ✓ 			survey, inte	erview, or k	otn).		
9	tunctionEnergy	understand what I'm saying Fatigue	\checkmark	Conclusions						
10 11 12	Gross motor function Swallowing	Trouble turning around Fall down Swallowing	✓ ✓ ✓	 Survey data was combined with qualitative interviews as a mixed method approach to support the content validation of three SCA COA instruments used in a clinical trial. This approach may be preferable to elicit a more comprehensive perspective of the patient experience, especially when recruiting large samples for qualitative interviews may be 						
13	Impact to activities of daily living	Need help doing usual activities	\checkmark							
15	Gross motor function	Not able to walk more than a few feet without stopping		challenging.	-					
16 17	Impact to emotional function	Trouble keeping balance Frustrated being too tired to do the things I want	✓			www.pa	rexel.com			ß

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Figure 2. Tree Diagram: Contribution of unique concepts elicited from SCA patient survey and interviews



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