	A	В	C	D	F	F	G	Н	ı	l ı	K	l ı	М	N
	Appendix 2. What is your view of the possibility of					to ris			o kno	wn as	Fyrys	hi) for		
1	of those who have 5q SMA											ai, 10i		atmont
2	Q1. Who completed this survey (as many as appli													
3	да предостава на		/											
4		%	Nos.											
5	Adult (age 18 years +) who has 5q SMA	99	70											
	Young person (age under 18 years) who has 5q													
6	SMA	1	1											
7	Total answering	100	71											
8														
9	Q2. Ethnicity best describing the person													
10		%	Nos											
11	Asian or Asian British Indian	3	2											
12	Asian or Asian British Pakistani	0	0											
13	Black or Black British African	0	0											
	Mixed White and Asian	1	1											
	White British	82	58											
	White Irish	3	2											
	White Other	10	7											
	Prefer not to say	3	2											
19			72											
20	Total answering		71											
21														
_	Q3. Where the person with 5q SMA lives													
23														
24		%	Nos											
	England	86	61											
	Scotland	10	7											
_	Wales	0	0											
28	Northern Ireland	4	3											

	A	В	C	D	Ε	F	G	Н	1	J	K	L	М	Ν
29	Total answering	100	71											
30	Q4. The clinical classification given by the diagno	osing cli	inician											
31		%	Nos											
32	Type 1	1	1											
	Type 2	25	34											
	Type 3	21	29											
35	Type 4	3	4											
36	Other (2 x Type 1/2 and 1 x later type 3)	2	3											
37	Total answering	52	71											
38														
39	Q5. Age of the person													
40	In years	%	Nos											
41	12 - 17	1	1								I.	1		
42	18-24	19	13											
43	25-34	27	19											
	35-44	17	12		1	skippe	ed							
	45-54	20	14											
	55-64	7	5											
47	65+	9	6											
48	Total answering	100	70											
49														
50	Q6. How many SMN2 copies the person has													
51														
52	Number	%	Nos											
53	1	1	1											
54	2	6	4				'					1		
55	3	6	4											
	4	1	1											
57	5	1	1											
58	6+	1	1											

	A	В	С	D	Е	F	G	Н	1	J	К	L	М	N
59	Don't know	83	59							-				
60	Total answering	100	71											
61	Q7. For each of the following physical milestone	s, the d	escript	on tha	t bes	t appli	ies to	the p	ersor	1				
62	<u> </u>		_					-						
				Used	l to									
				be ab										
				do ti										
		Never	been	but	,	Can	do							
63		able to				this		Total						
64		%	Nos		Nos		Nos							
65	Sitting with support	0				90		69						
66	Sitting without support	8	_		26	55		71						
67	Standing with support	22	15		34	29	20	69						
68	Standing without support	43	30	46	32	11	8	70						
69	Walking with support from a person/stick/frame	36	25	42	29	22	15	69						
70	Walking between 1 - 4 steps - without assistance	46	32	42	29	12	8	69						
71	Walking 5 steps or more - without assistance	49	34	40	28	11	8	70						
72				•	Total	answe	ering	71						
73														
74	Q8. The best description of the person's current	mobility	1											
75														
76		%	Nos											
	No independent mobility - need to be reclined	3	<b>.</b>											
	Full-time independent electric wheelchair user - need													
	Full - time independent electric wheelchair user	61	43											
_	Mainly electric wheelchair user	6												
	Mainly manual wheelchair user	7	5								1			
82	Walk with assistance - from stick, frame or person	8	<b>.</b>											
83	Walk without assistance	10												
84	Total answering	100	71											

	A	В	С	D	Е	F	G	Н	I	J	К	L	М	N
85														
86														
87	Q9. If they have had any intervention for a scolios	is (curv	ing of t	he spi	ne) -	or if a	ny is	plann	ed (as	s man	y as app	oly ticl	ked)	
88		%	Nos				Ī							
89	Yes - had spinal fusion surgery	31	22											
90	Yes - had growth rods surgery	1	1				•		•					
91	Yes - surgery has been advised / is planned	0	0											
92	Yes - have / had a spinal brace	17	12											
93	Not sure / Don't know	0	0											
94	No	55	39											
95	Total answering		71											
96														
	Q10. If they have a scoliosis / spinal intervention	(had or	planne	d) tha	t is li	kely to	pre	vent a	clinic	cian fr	om beir	ng able	e to sa	fely
97	administer a drug treatment via lumber puncture													
98		%	Nos											
99	Yes - definitely	14	9											
100	Probably	27	18		66	answe	ered							
101	Don't think so	11	7		5	skippe	ed							
102	No	29	19											
103	Other	2	1											
104	Don't know	18												
105	Total answering		66											
106														
107	Q11. The impact of their 5q SMA on their breathing	ng abilit	у											
108														
109		%	Nos											
-	Breathing ability is not affected	52												
	Uses cough assist / airway clearance equipment / ch					answe								
112	Uses a non-invasive ventilator (NIV such as BiPaP)	26	18		2	skippe	ed							
113	Uses a non-invasive ventilator (NIV such as BiPaP)	1	1											

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	N
114	Has a tracheostomy	1	1											
115	Total answering		69											
116	Q12. If they experience muscle contractures (tigh	ntness)												
117														
118		%	Nos											
119	No	17	12											
120	Yes - mild	30	21		70	answe	ered							
121	Yes - moderate	27	19		1	skippe	ed							
122	Yes - severe	19	13											
123	Not sure / don't know	7	5											
124	Total answering		70											
125														
126	Q13. Drug treatments for 5q SMA they are received	ing / hay	ve rece	ived										
127														
128		%	Nos											
129	Continuing to receive Spinraza	3	2											
	Continuing to receive risdiplam via a clinical trial /													
130	compassionate use /early access programme	1	1											
	Continuing to receive other drug treatment via a													
	clinical trial	1	1											
	Previously received other drug treatment via clinical													
	trial	4	3											
	Never received any drug treatment	93	66											
134	Total answering		71											
135														
136														
137														
138														
139														
140														

			•								,			
	A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
141														
142														
	Q14. How important improvements in different as	spects o	f health	and d	laily I	iving v	would	be if	these	coul	d be aff	ected	by a d	rug
143	treatment	•			•									•
144														
						Neit	her				l .			
						impoi	_	N	ot l	Not	at all			
145		Verv im	portant	Impoi	tant	or r		impo			ortant	Total		
146			Nos		Nos		Nos		Nos		Nos			
	Improved motor milestones - e.g. ability to sit, stand,			14	10	10		6	4	1	1	71		
	Improved breathing ability	65		17	12	10		7	5	0	·			
	Improved swallowing / ability to eat	65		14	10	12	8	7	5	1	1			
	Improved ability to communicate	38		22	15	20	14	12	8	9	6	69		
	Improved stamina and reduced fatigue	73	52	12	16	3	2	0	0	1	1	71	,	
152	Improved fine motor skills (e.g. movement of fingers	77	54	9	12	3	2	3	2	0	0	70		
153	Increased independence	82	58	7	10	3	2	1	1	0	0	71		
	Reduced reliance on caregivers and personal assist	71	50	8	11	6	4	7	5	0	0	70		
155	See comments for responses								Tot	al ans	wering	71		
156														
157	Q15. How many read the risdiplam summary info	rmation	provid	ed.										
158	· · · · · · · · · · · · · · · · · · ·													
159		%	Nos		66	answe	ered							
160	Chose to read the information	93	66		5	skippe	ed							
161														
162														
163														
164														
165														
166														
167														

	A	В	С	D	Е	F	G	Н	1	J	K	L	М	N
168														
169														
170	Q16. Advantages / disadvantages ratings of aspe	cts of ri	sdiplan	n treati	ment									
171														
		Str	ong											
172		Advar	ntage							Stı	ong			
173		1		2		3			1		5	Total		
174			Nos	%	Nos		Nos	%	Nos	%	Nos			
	How it is taken (syrup by mouth)	90		7	5	3		0	0	0	0			
	How often it has to be taken (daily)	44		25	18	30		1	1	0	0			
	How long it has to be taken for (as long as treatmen			24	17	34		3		0	_			
	Where it can be taken (at home)	96		3	2	0		0	0	1	1	71		
	Where it must be stored / kept (refrigerated)	39	28	25	18	28	20	7	5	0	0			
180									Tota	al ans	wering	71		
181														
182														
183														
184														
185														
186														
187														
188														
189														
190														
191														
192														
193														
194														
195														
196														

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	N
197														
198														
199														
200	Q17. Views on aspects of what is known so far a	bout ris	diplam											
201	-													
						Neit posi no	tive			V	ery			
202		Very p	ositive	Posi	tive	nega	tive	Neg	ative		ative	Don't	know	Total
203		%	Nos	%	Nos		Nos	)	Nos		Nos	%	Nos	
204	Its safety profile	43	30	41	29	11	8	0	0	0	0	4	3	70
	Its recorded adverse events profile	18	13	37	26	35	25	3	2	1	1	6	4	71
206	Its impact on motor milestones	54	38	41	29	4	3	0	0	0	0	1	1	71
207	Its impact on swallowing	41	29	31	22	15	11	0	0	0	0	13	9	71
208	Its impact on ability to communicate	28	20	38	27	17	12	0	0	0	0	17	12	71
209	Its impact on breathing ability	41	29	35	25	11	8	1	1	0	0	11	8	71
210	Its impact on frequency and duration of hospital stay			30	21	11	8	1	1	0	0	20	14	71
211	Its impact on stamina and fatigue	39	28	48	34	4			0	0	0	8	6	71
	Its impact on quality of life	56		34		3		0	0	0	0	7	5	71
	Its impact on female menstruation	3		7	5	46		7	5	7	5	30		70
	Its impact on female fertility	3		1		46		10	7	9	6	30		69
	Its impact on male fertility	3	2	6	4	41	29	14	10	11	8	24	17	70
	See comments for responses										Tota	l ansv	vering	71
217														
218	Q18. How acceptable risdiplam is as a whole													
219														
220		accep								unac	tally ceptabl e			
221			1	2		3	3	4	4		5			l

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	N
222	Acceptability	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos			
	See comments for responses	86	61	14	10	0	0	0	0	0	0			
224								Total	answ	ering	71			
225	Q19. Which groups should have access to risdip	lam												
226														
227		%	Nos											
_	Type 0	72	51											
	Type 1	89	63											
_	Type 2	90	64											
	Type 3	92	65											
	Type 4	73	52											
	Don't know	6	4											
234	Total answering		71											
235														
	Q20. Which of the following groups should have	access	to risdi <sub>l</sub>	plam										
237														
238		%	Nos											
	People with 1 SMN2 copy	86	61											
	People with 2 SMN2 copies	93	66											
	People with 3 SMN2 copies	92	65											
	People with 4 SMN2 copies	86	61											
	People with 5 SMN2 copies	75 70	53											
	People with 6+ SMN2 copies	73	52 <b>71</b>											
245	Total answering		71											
246	O24 If infants who are not considered in with a se		<b>! a.</b> a. u. a. a. a. !	f F		\ _l	- دا امار			ا داد ما	lin la :			
	Q21. If infants who are pre-symptomatic with a g	enetic d	iagnosi	S 01 50	J SIVIA	4 Snol	lia na	ive ac	cess	O risa	iipiam			
248		0/	Nina											
249	Voc	%	Nos											
	Yes	82 1	58											
251	NO .	1	1											

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	Ν
252	Don't know	17	12											
253	Total answering		71											
254														
255														
256	Q22. Any groups of people who have 5q SMA wh	o shoul	d have	priority	y acc	ess to	risdi	plam						
257														
258		%	Nos											
259	Yes	49	35											
260	No	32	23											
261	Don't know	18	13											
262	See comments for responses		36											
263	Total answering		71											
264														
265	Q23. Would they want access to NHS-funded risc	diplam												
266														
						Don't	know							
267	Who is answering the question	Υe	es	N	0	/ not	sure	Total						
268		%	Nos	%	Nos	%	Nos							
269	I am that person and have 5q SMA	100	71	0	0	0	0	71						
270				,	Total	answe	ering	71						
271	Q24. Willing for comments to be used													
272														
273		Υe	es	N	0	Total								
274		%	Nos	%	Nos									
275	In submissions to NICE	97	69	3	2	71								
	In any future submissions to the Scottish Medicines													
276	Consortium	96	66	4	3	69								
	In any survey summaries published on SMA UK's													
277	website	94	66	6	4	70								

	A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
	In any survey summaries published on MDUK's													
278	website	93	65	7	5	70								
279			Total	answe	ering	71								