	A	В	C	D	Е	F	G	Н	1		Ικ	1	М	N
	Appendix 3. What is your view of the possibility of	_	Ŭ			to ris			o kno	own as		li) for		
1	of those who have 5q SMA? I											,		
2	Q1. Who completed this survey										<u> </u>			
3														
4		%	Nos.											
	Parent of someone who has 5q SMA and is <													
5	18years		32											
6	Total answering		32											
7														
8														
9	Q2. Ethnicity best describing their child													
10														
11		%	Nos											
	Asian or Asian British Pakistani	1	1											
	Black or Black British African	1	1											
	Mixed White and Asian	1	1											
	White British	18	25											
	White Other	4	5											
17	Total answering		32											
18														
	Q3. Where their child lives													
20														
21		%	Nos											
	England	84	27											
	Scotland	13	4											
	Wales	0	0											
	Northern Ireland	3	1											
26	Total answering		32											
27														-
28														

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	N
29														
30	Q4. The clinical classification given by the diagno	osing cl	inician											
31														
32		%	Nos											
	Type 1	16	5											
	Type 2	66	21											
	Type 3	19	6											
36	Total answering	100	32											
37														
38	05.4													
	Q5. Age of their child													
40	la con a un	0/	NI											
41	In years	%	Nos											
	0-4	22	7											
	5 - 11 12 - 17	44 34	14 11											
45	Total answering	100	32									1		
46	Total allswering	100	32											
	Q6. How many SMN2 copies their child has													
48	QU. Flow many owner copies their crima has													
49	Number	%	Nos											
50		0	0											
51		13	4											
52		34	11											
	Don't know	53	17											
54	Total answering	100	32											
55														
56														
57														
58														

	A	В	С	D	Е	F	G	Н	I	J	К	L	М	N
59														
60														
61	Q7. For each of the following physical milestone	s, the d	escripti	on tha	t bes	t appli	es to	their	child					
62														
				Used	d to									
				be ab	le to									
				do t										
		Never	been	but	•	Can	do							
63		able to	do this	any n	nore	this r	now	Total						
64			Nos	%	Nos		Nos							
65	Sitting with support	0	0	13		88	28	32						
	Sitting without support	16	5	25	8	59	19	32						
67	Standing with support	25	8	22	7	53	17	32						
68	Standing without support	75	24	16	5	9	3	32						
69	Walking with support from a person/stick/frame	66	21	16	5	19	6	32						
70	Walking between 1 - 4 steps - without assistance	75	24	13	4	13	4	32						
71	Walking 5 steps or more - without assistance	78	25	13	4	9	3	32						
72					Total	answe	ering	32						
73														
74	Q8. The best description of their child's current r	nobility												
75														
76		%	Nos											
77	No independent mobility - need to be reclined	10												
	Full-time independent electric wheelchair user - need	0	_											
	Full - time independent electric wheelchair user	60												
	Mainly electric wheelchair user	10			2	skippe	d							
	Mainly manual wheelchair user	10								Г				
	Walk with assistance - from stick, frame or person	3												
-	Walk without assistance	7	2											
84	Total answering	100	30											

	A	В	С	D	Е	F	G	Н		J	К	L	М	N
85			_											
							1	1	1		1		1	
86	Q9. If their child has had any intervention for a so	coliosis	(curvin	g of th	e spi	ne) - c	or if a	ny is	plann	ed (as	many a	ıs appl	ly ticke	:d)
87														
88		%	Nos											
	Yes - had spinal fusion surgery	22	7											
	Yes - had growth rods surgery	9	_											
	Yes - surgery has been advised / is planned	13												
	Yes - have / had a spinal brace	25	8											
93	Not sure / Don't know	0												
	No	38												
95	Total answering		32											
96														
	Q10. If their child has a scoliosis / spinal interver	ntion (ha	ad or pla	anned) tha	t is lik	ely to	prev	ent a	clinici	an from	being	able to	o
97	safely administer a drug treatment via lumber pu	ncture												
98														
99		%	Nos											
100	Yes - definitely	13	4											
	Probably	16	5											
	Don't think so	13	4											
103		47	15								T.			
	Other	3												
	Don't know	9												
106	Total answering	100	32											
107														
108														
109														
110														
111														
112														

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	N
113														
114	Q11. The impact of 5q SMA on their child's breatl	ning abi	lity											
115														
116		%	Nos											
117	Breathing ability is not affected	41	13											
	Uses cough assist / airway clearance equipment /													
118	chest physio when needed	56	18											
	Uses a non-invasive ventilator (NIV such as BiPaP)													
119	for less than 16 hours/day on a regular basis	38	12											
	Uses a non-invasive ventilator (NIV such as BiPaP)		_											
	for more than 16 hours/day on a regular basis	6	2						I		T			
	Has a tracheostomy	0	0											
122	Total answering		32											
123														
_	Q12. If their child experiences muscle contractur	es (tight	tness)											
125														
126		%	Nos											
	No	13	4											
	Yes - mild	34	11											
	Yes - moderate	38	12											
	Yes - severe	13	4						1		1			
	Not sure / don't know	3												
132	Total answering		32											
133														
_	Q13. Drug treatments for 5q SMA their child is re			eceive	d									
135		%	Nos											
	Continuing to receive Spinraza	56	18											
137	Previously received Spinraza	3	1											
	Continuing to receive risdiplam via a clinical trial /													
138	compassionate use /early access programme	6	2											

	Δ.	В		_		г	_	l	, 1	<u> </u>	l _V		1.4	
120	Never received any drug treatment	B 38	C 12	D	E	F	G	Н	I	J	K	L	М	N
140	· ·	36	32											
140														
	Q14. How important would improvements in diffe	erent as	pects of	their	child	's hea	lth an	id dai	y livir	ng be	if these	could	be affe	ected
	by a drug treatment		ı			ı					1			T
142														
						Neit	her							
						impo			ot	_	t at all			
143		•	portant			or r			rtant		ortant	Total		
144			Nos	%	15		Nos		Nos		Nos			
	Improved motor milestones - e.g. ability to sit, stand,			6	2	0			0	0				
	Improved breathing ability	83		13	4	3		0		0				
	Improved swallowing / ability to eat	84	26	10	3				0	0				
	Improved ability to communicate	68	21	6	2	16				1	2			
	Improved stamina and reduced fatigue	88		13	4	0				0				
	Improved fine motor skills (e.g. movement of fingers		29	9	3	0				0				
	Increased independence	94	30	6	2	0			0	0		_		
	Reduced reliance on caregivers and personal assist	75	24	16	5	9	3	0	0	0				
	See comments for responses								Tot	tal ans	swering	32		
154														
155	Q15. How many read the risdiplam summary info	rmation	provid	ed.										
156														
157		%	Nos		28	answe	ered							
158	Chose to read the information	81	26		4	skippe	ed							
159														
160														
161														
162														
163					_									
164														
165														

A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
166													
167													
168 Q16. Advantages / disadvantages ratings of aspe	cts of ri	sdiplan	1 treat	ment									
169													
	Stro	ong							Disad	vantage			
170	Advar												
171	1		2		3	}		1		5	Total		
172	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos			
173 How it is taken (syrup by mouth)	91	29	3		3		0	0			32		
174 How often it has to be taken (daily)	69		16		13		0			1	32		
175 How long it has to be taken for (as long as treatmen			16		16		0						
176 Where it can be taken (at home)	97	31	0		3		0			0			
177 Where it must be stored / kept (refrigerated)	66	21	13	4	22	7	0)		0			
178								To	tal ans	swering	32		
179													
180													
181													
182													
183													
184													
185													
186													
187													
188													
189													
190													
191													
192													
193													
194													

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	Ν
195														
196														
197														
198	Q17. Views on aspects of what is known so far a	bout ris	diplam											
199														
						Neit posi no	tive			V	/ery			
200			ositive	Posi		nega)	ative		gative		know	Total
201		%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	
	Its safety profile	66		28		0	0	0		0	0			32
	Its recorded adverse events profile	41	13			16	5	3		0	0	•		32
	Its impact on motor milestones	63		28		3	1	0		0	0	•		32
	Its impact on swallowing	53		31	10	6	2	0	0	0		_		32
	Its impact on ability to communicate	47	_				3			0				32
	Its impact on breathing ability	59						0	0	0				32
	Its impact on frequency and duration of hospital stay			25		0				0				32
	Its impact on stamina and fatigue	69		25						0				32
	Its impact on quality of life	75		19						0	_	_		32
	Its impact on female menstruation	22		3		25	8	9	3	0			13	32
	Its impact on female fertility	13				31	10	9	3	0				32
	Its impact on male fertility	2	3	3	1	34	11	13	4	0	_		13	32
_	See comments for responses										Tota	ıl ansv	vering	32
215														
216	Q18. How acceptable risdiplam is as a whole													
217														
218		accep	Totally acceptable			_					otally ceptabl e			
219		·	1	2	2	3	}	4	1		5			

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	Ν
220	Acceptability	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos			
221		94	30	3	1	3	1	0	0	0	0			
222	See comments for responses							Total	answ	ering	32			
223	Q19. Which groups should have access to risdip	lam												
224														
225		%	Nos											
	Type 0	78	25											
	Type 1	100	32											
	Type 2	100	32											
	Type 3	88	28											
	Type 4	75	24											
	Don't know	0	0											
232	Total answering		32											
233														
234	Q20. Which of the following groups should have	access	to risdi _l	olam										
235														
236		%	Nos											
	People with 1 SMN2 copy	94	30											
	People with 2 SMN2 copies	97	31											
	People with 3 SMN2 copies	97	31											
	People with 4 SMN2 copies	75	24											
	People with 5 SMN2 copies	66	21											
	People with 6+ SMN2 copies	63	20											
243	Total answering		32											
244														
245	Q21. If infants who are pre-symptomatic with a g	enetic d	iagnosi	s of 5	q SMA	A shou	ıld ha	eve ac	cess	to risd	liplam			
246														
247		%	Nos											
	Yes	97	31											
249	No	3	1											

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	N
250	Total answering		32											
251														
252														
253														
254	Q22. Any groups of people who have 5q SMA wh	o shoul	d have i	oriorit	y acc	ess to	risdi	plam						
255														
256		%	Nos											
257	Yes	50	16											
258	No	25	8											
259	Don't know	25	8											
260	See comments for responses	38	12											
261	Total answering		32											
262														
263	Q23. Would they want their child to have access	to NHS-	funded	risdip	lam									
264														
						Don't	know							
265	Who is answering the question	Υe	es	Ν	0	/ not	sure	Total						
266		%	Nos	%	Nos	%	Nos							
267	. 0	97	31	0				32						
268					Total	answe	ering	32						
269	Q24. Willing for comments to be used													
270														<u> </u>
271		Υe		N		Total								
272		%	Nos	%	Nos									
273	In submissions to NICE	97	31	3	1	32								
	In any future submissions to the Scottish Medicines													
274	Consortium	94	30	6	2	32								
	In any survey summaries published on SMA UK's													
275	website	94	30	6	2	32								

	А	В	С	D	Е	F	G	Н	ı	J	K	L	М	N
	In any survey summaries published on MDUK's													
276	website	91	29	6	2	31								
277			Total	answe	ering	137								