				_			Ι .		Ι.		1 1/		1 14	N.
	A no and in 4. What is your view of the margini	B	C . NUIC	D	E		G	H	/-l	J	K	_ <u> </u>	M di) for	N N
	Appendix 1. What is your view of the possibil treatment of t									Knov	wn as	Evrys	ai) for	tne
2	Q1. Who completed this survey (as many as appl			oq Si	VIA: A	ALL K	LSPU	NOES) 					
3	Will completed this survey (as many as appr	y lickeu)											
4		%	Nos.											
5	Adult (age 18 years +) who has 5q SMA	7 0 51	70											
	Young person (age under 18 years) who has 5q	31	70											
6	SMA	1	1											
	Parent of someone who has 5q SMA	34	46											
	Grandparent of someone who has 5q SMA	6	8											
9	Partner of someone who has 5q SMA	1	2											
	Related in another way to someone who has 5q													
10	SMA	6	8											
	Bereaved by 5q SMA - parents of infants with SMA													
11	Type 1	1	2											
12	Other - family friend	1	1 1 1 1 1 1 1											
13	Total answering		137											
14	OO Amouvement record there are a company related to	41-			! 4									
16	Q2. Answered more than one survey - related to r	nore tha	an one	persor	1 Witr	ı əqər	VIA 		Τ					
	Adult (age 18 years +) who has 5q SMA		3											
18			4											
19	Total		7											
20	. • • • • • • • • • • • • • • • • • • •		-											
21	Q3. Bereaved by 5qSMA													
22														
	Parents of infants with SMA Type 1		2											
24	Adult with 5qSMA		3											
25	Total		5											
26														

	A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
27														
28	Q4. Ethnicity best describing the person who has	s / had 5	q SMA											
29		%	Nos											
30	Asian or Asian British Indian	1	2											
31	Asian or Asian British Pakistani	1	1											
32	Black or Black British African	1	1											
	Mixed White and Asian	2	3											
	White British	79	108											
	White Irish	4	5											
	White Other	12	17											
_	Prefer not to say	1	2											
38	Total answering		137											
39														
	Q5. Where the person with 5q SMA lives / lived													
41														
42		%	Nos											
	England	7	120											
	Scotland	0	10											
	Wales	5	0											
_	Northern Ireland	100	7											
47	Total answering		137											
48														
_	Q6. The clinical classification given by the diagno	osing cl	inician											
50														
51		%	Nos											
	Type 1	7	9											
	Type 2	49	67											
	Type 3	38	52											
	Type 4	3	4											
56	Don't know / can't remember	2	3											

	A	В	С	D	Е	F	G	Н	ı	J	К	L	М	N
57	Other (2 x Type 1/2)	1	2											
58	Total answering	100	137											
59	Q7. Age of the person with 5q SMA													
60														
61	In years	%	Nos											
62	0-4	5	7											
63	5 - 11	14	19											
	12 - 17	10	13											
	18-24	18	24											
	25-34	17	22			answe								
	35-44	10	13			skippe								
	45-54	17	22		2	berea	ved p	arents	not a	sked				
	55-64	5	6											
	65+	5	7											
71	Total answering	100	133											
72														ļ
_	Q8. How many SMN2 copies the person with 5q S	SMA has	1											
74														
75	Number	%	Nos											
76		1	1											
	2	8	11		405									
78 79	4	12 1	16 2			answe berea		oronto	not c	oleo d				
	5	1	1			berea	veu p ∣	arenis	1101 2	JONEU				
	6+	2	3											
	Don't know	75	101											
83	Total answering	100	135											
84	Total answering	.00	100											
84 85 86														_

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	N
87														
88														
89														
90	Q9. For each of the following physical milestone	s, the d	escripti	on tha	t bes	t appli	ies to	the p	ersor	า witl	h 5q S	MA		
91														
				Used	d to									
				be ab										
				do t										
		Never	been	but	•	Can	do							
92		able to	do this	any n	nore	this ı	now	Total						
93		%	Nos		Nos	%	Nos							
94	Sitting with support	1	1	12	15	88	113	129						
95	Sitting without support	13	18	32	43	55	74	135						
96	Standing with support	25	33	39	51	35	46	130		135	answ	ered		
97	Standing without support	51	69	36	48	13	17	134		2	be	reaved	parent	s not
98	Walking with support from a person/stick/frame	46	60	33	43	21	28	131						
99	Walking between 1 - 4 steps - without assistance	55	71	33	43	12	16	130						
100	Walking 5 steps or more - without assistance	56	75	33	44	11	15	134						
101				1	Total :	answe	ring	135						
102														
103	Q10. The best description of the person with 5q	SMA's c	urrent n	nobilit	У									
104														
105		%	Nos											
	No independent mobility - need to be reclined	5	7											
	Full-time independent electric wheelchair user - need	9	12											
	Full - time independent electric wheelchair user	53	71			answe							ļ	
_	Mainly electric wheelchair user	11	14			skippe							ļ	
	Mainly manual wheelchair user	8	10		2	bereav	ved pa	arents	not a	sked	1		<u> </u>	
	Walk with assistance - from stick, frame or person	6												
112	Walk without assistance	8	11											

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	N
113	Total answering	100	133											
114														
	Q11. If the person with 5qSMA has had any interv	vention	for a sc	oliosis	s (cur	ving c	of the	spine) - or	if any	y is pla	anned	(as ma	any as
115	apply ticked)						_							
116														
117		%	Nos											
	Yes - had spinal fusion surgery	54	38											
	Yes - had growth rods surgery	7	5			answe								
	Yes - surgery has been advised / is planned	7	5		2	berea	ved p	arents	not a	sked	П	T		
	Yes - have / had a spinal brace	37	26											
	Not sure / Don't know	3												
123		93												
124	Total answering		135											
125														
	Q12. If the person with 5qSMA has a scoliosis / s	-		•	ad or	plann	ed) t	nat is	likely	to pr	revent	a clini	ician fr	om
126	being able to safely administer a drug treatment	via luml	per pun	cture	T	1					T	1		
127														
128		%	Nos											
129	Yes - definitely	13	17											
130	Probably	21	27		127	answe	ered							
	Don't think so	12				skippe								
132		36			2	berea	ved p	arents	not a	sked				
	Other	2												
_	Don't know	15												
135	Total answering	100	127											
					1	1	1	1		1	1	1		
136														
136 137														
136 137 138														
136 137														

	A	В	С	D	Е	F	G	Н	ı	J	К	L	М	N
141														
142														
143	Q13. The impact of the person's 5q SMA on their	breathir	ng abilit	ty										
144														
145		%	Nos											
146	Breathing ability is not affected	51	69											
	Uses cough assist / airway clearance equipment /													
	chest physio when needed	43	58		135	ansv	wered							
	Uses a non-invasive ventilator (NIV such as BiPaP)													
	for less than 16 hours/day on a regular basis	25	34		2	bere	eaved p	arents	not a	sked				
	Uses a non-invasive ventilator (NIV such as BiPaP)													
	for more than 16 hours/day on a regular basis	2	3				1							
	Has a tracheostomy	3	4											
151	Total answering		135											
152														
153														
154														
	Q14. If the person with 5qSMA experiences musc	le contr	actures	(tigh	tness	<u>s)</u>								
156														
157		%	Nos											
158		15	20											
$\overline{}$	Yes - mild	30	40				wered							
	Yes - moderate	29	39			skip								
	Yes - severe	14	19		2	bere	eaved p	arents	not a	sked	ı			
-	Not sure / don't know	12	16							1				
163	Total answering		134											
164														
165														
166														
167														

	А	В	С	D	Е	F	G	Н	I	J	K	L	М	Ν
168														
169														
170														
171	Q15 Drug treatments for 5q SMA the person with	n 5qSMA	is rece	iving	/ has	receiv	/ed / I	nad re	ceive	d				
172														
173		%	Nos											
174	Continuing to receive Spinraza	16	22											
	Previously received Spinraza	1	2											
	Received Zolgensma via a clinical trial / private													
176	access	0	0											
	Continuing to receive risdiplam via a clinical trial /													
177	compassionate use /early access programme	2	3		137	answe	ered							
	Previously received risdiplam via a clinical trial /													
178	compassionate use / early access programme	0	0											
	Continuing to receive other drug treatment via a													
-	clinical trial	1	1											
	Previously received other drug treatment via clinical													
	trial	2	3											
	Never received any drug treatment	80	109											
182	Total answering		137								1			
183														
184														
185														
186														
187														
188														
189														
190														
191														
192														

	А	В	С	D	Ε	F	G	Н	I	J	K	L	М	N
193														
194														
	Q16. How important would improvements in diffe	erent ası	pects of	f the p	ersor	า with ร	5qSM	IA's h	ealth	and o	daily li	ving b	e if the	ese
195	could be affected by a drug treatment	•		•			•				,	J		
196														
			·			Neitl	ner							
						impor	_	N	ot	Not	at all			
197		Very im	portant	Impoi	tant	or n		impo	-		ortant	Total		
198			Nos	%	15		Nos		Nos		Nos			-
	Improved motor milestones - e.g. ability to sit, stand.				22	5	7	3	4	2		133		
	Improved breathing ability	66		16	21	11	14	5	7	2		132		
	Improved swallowing / ability to eat	64	85	14	19		16	5	7	2		132		
	Improved ability to communicate	44	57	18	24	24	31	9	12	9		131		
	Improved stamina and reduced fatigue	79	108	17	23	2	3	0	0	1	2	137		
204	Improved fine motor skills (e.g. movement of fingers	77	103	11	15	3	4	2	3	1	1	134		
205	Increased independence	84	115	15	20	4	5	1	1	1	1	137		
206	Reduced reliance on caregivers and personal assist	73	99	2	3	7	10	4	6	1	1	136		
207	See comments for responses								otal a	ınsw	ering	137		
208														
209	Q17. How many read the risdiplam summary info	rmation	provid	ed.										
210														
211		%	Nos		125	answe	red							
212	Chose to read the information	91	125		12	skippe	:d							
213														
214														
215														
216														
217														
218														
219														

	A	В	С	D	Е	F	G	Н	l	J	K	L	М	N
220														
221														
222	Q18. Advantages / disadvantages ratings of aspe	cts of ri	sdiplan	n treat	ment									
223														
		Stro	ong							Disa	dvant			
224		Advar	ntage							age				1
225		1		2		3	3	4	1		5	Total		
226		%	Nos	%	Nos	%	Nos	%	Nos	%	Nos			
227	How it is taken (syrup by mouth)	89	122	7	10	3	4	0	0	1	1	137		
228	How often it has to be taken (daily)	51	70	24	33	23	32	1	1	1	1	137		
229	How long it has to be taken for (as long as treatmen	48	66	23	32	27	37	1	2	0	0	137		
230	Where it can be taken (at home)	93	128	4	6	1	2	0	0	1	1	137		
231	Where it must be stored / kept (refrigerated)	48	66	23	32	25	34	4	5	0	0	137		
232									Total	answ	vering	137		

	A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
233														
234	Q19. Views on aspects of what is known so far a	bout ris	diplam											
235			_											
						Neit	her							
						posi	_							
						nc				V	ery			
236		Very p	ositive	Posi	tive	nega	tive	Nea	ative		ative	Don't	know	Total
237		%	Nos		Nos		Nos	_	Nos)	Nos		Nos	
238	Its safety profile	52	71	36		7	9	1	1	0	0	4	6	136
	Its recorded adverse events profile	25	34	36		29	40	3	4	1	1	6	8	136
	Its impact on motor milestones	59	80	34	46	4	6	0	0	0	0	3	4	136
241	Its impact on swallowing	46	62	30	41	13	17	0	0	0	0	12	16	136
242	Its impact on ability to communicate	35	48	35	47	15	20	0	0	0	0	15	21	136
243	Its impact on breathing ability	46	63	33		9	12	1	1	0	0	11	15	136
244	Its impact on frequency and duration of hospital stay	46	63	29		10	13	1	1	0	0	15	20	136
	Its impact on stamina and fatigue	53	73	35		3	4	0	0	0	0	9	12	137
246	Its impact on quality of life	66	91	25	34	2	3		0	0	0	7	9	137
247	Its impact on female menstruation	11	14	8	10	41	54	6	8	4	5	32	42	133
248	Its impact on female fertility	7	9	5	6	42	56	8	10	5	6	34	45	132
249	Its impact on male fertility	6	8	8	11	42	57	10	14	6	8	27	37	135
250											Total	answ	ering	137
251														
252	Q 20. How acceptable risdiplam as a whole													
										To	tally			
		Tota	ally								ccept			
253		accep	•								ble			
254				2	<u> </u>	3	}	4	1		5			
\vdash	Acceptability	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos			
256		88	120	10		2	3		0	0	0			
_	See comments for responses						_	_	answ	ering	137			

	А	В	С	D	Е	F	G	Н	I	J	К	L	М	N
258	Q21. Which groups should have access to risdip	lam												
259														
260		%	Nos											
261	Type 0	64	87											
262	Type 1	88	120											
263	Type 2	91	124											
264	Type 3	87	119											
	Type 4	70	96											
	Don't know	7	9											
267	Total answering		137											
268														
269	Q22. Which of the following groups should have	access	to risdi _l	olam										
270														
271		%	Nos											
	People with 1 SMN2 copy	87	119											
	People with 2 SMN2 copies	94	129											
	People with 3 SMN2 copies	92	126											
	People with 4 SMN2 copies	81	111											
	People with 5 SMN2 copies	72	99											
	People with 6+ SMN2 copies	69	94											
278			137											
279														
	Q23. If infants who are pre-symptomatic with a g	enetic d	iagnosi	s of 5	q SM	A shou	ıld ha	ve ac	cess	to ris	diplar	n		
281														
282		%	Nos											
	Yes	81	111											
284		1	2											
_	Don't know	18	24											
286	Total answering		137											
287														

				_				·		т.				
	A	В	С	D	E	F	G	Н	l	J	K	L	М	N
288							L	_						
	Q24. Any groups of people who have 5q SMA wh	o shoul	d have I	oriority	y acc	ess to	risdi	plam						
290														
291	\ \ \ \ \ \ \ \ \ \ \ \ \ \ \ \ \ \ \	%	Nos											
292		51	70											
293		26	35											
	Don't know	23	32											
	See comments for responses	51	70											
296	Total answering		137											
297						.	<u> </u>			<u> </u>	<u> </u>		<u> </u>	
	Q25. Would the respondent want the person who	o has 50	SMAth	ey are	rete	rring t	o in ti	his su	rvey,	to h	ave / r	nave h	ad acc	ess to
	NHS-funded risdiplam				1	ı		I	Ι	1				г
299														
						Don't		_						
_	Who is answering the question	Υe		N			sure	Total						
301			Nos	%	Nos	%	Nos							
302		95	73	5										
303	I am a parent / guardian of the person who has / had		47	16				57						
304	I have another relationship with the person who has	77	20	23	6	·	0							
305					Ans۱	wered		137						
	Q26. Willing for comments to be used													
307														
308		Υe	es	N	0	Total								
309		%	Nos	%	Nos									
310	In submissions to NICE	96	132	4	5	137								
	In any future submissions to the Scottish Medicines													
311	Consortium	95	126	5	7	133								
	In any survey summaries published on SMA UK's													
312	website	94	126	6	8	134								
	In any survey summaries published on MDUK's													
242	website	93	125	7	9	134								İ

	A	В	С	D	Е	F	G	Н	I	J	K	L	М	N
314			Total answering			137								