NICE National Institute for Health and Care Excellence

Patient organisation submission

Risdiplam for treating spinal muscular atrophy in children and adults [ID1631]

Thank you for agreeing to give us your organisation's views on this technology and its possible use in the NHS. You can provide a unique perspective on conditions and their treatment that is not typically available from other sources. To help you give your views, please use this questionnaire with our guide for patient submissions. You do not have to answer every question – they are prompts to guide you. The text boxes will expand as you type. [Please note that declarations of interests relevant to this topic are compulsory].

Information on completing this submission

- Please do not embed documents (such as a PDF) in a submission because this may lead to the information being mislaid or make the submission unreadable
- We are committed to meeting the requirements of copyright legislation. If you intend to include **journal articles** in your submission you must have copyright clearance for these articles. We can accept journal articles in NICE Docs.
- Your response should not be longer than 10 pages.

About you	
1.Your name	**Liz Ryburn and Kate Adcock**
2. **Name of organisation**	Spinal Muscular Atrophy UK (SMA UK) and Muscular Dystrophy UK (MDUK)

3. **Job title or position**	Support Team Manager (Liz Ryburn) Director of Research and Innovation (Kate Adcock)
4a	**Brief description of the organisation (including who funds it). How many members does it have?**
	SMA UK is a charity (previously known as the Jennifer Trust / SMA Support UK; merged in 2018 with The SMA Trust) that, since 1985, has provided free information and support to anyone affected by any form of SMA in the UK and has also funded research-related initiatives. We currently have contact with some 775 households of adults with SMA / parents living with a child with SMA. We estimate this to be over 60% of the total UK SMA population. We are also in touch with 364 households of parents bereaved by SMA. These figures exclude households of other relatives / friends. SMA UK is accredited to the Information Standard. Our SMA-related guides are signposted by the NHS website. Our Research Correspondents (a clinical and a research doctor) and Research Coordinator report to the SMA Community on the development of all drug treatments and clinical trials. We have regular contact with the SMA REACH UK paediatric and adult clinical networks. SMA UK's funding comes from donations, gifts, grants, trusts and merchandise sales. In 2019 / 20 we raised £925,870, comprising £795,531 donations and gifts, £124,302 Lotteries grant, £5,262 from merchandise sales and £775 from investment income. Founded in 1959, **Muscular Dystrophy UK** (previously known as the Muscular Dystrophy Campaign) brings together over 60 rare neuromuscular conditions, affecting around 70,000 children and adults in the UK. We fund research, provide vital information, advice, resources and support for people with these conditions, their families and the professionals who work with them. MDUK's funding comes from donations, gifts, grants and trusts. In 2019 / 20, we raised £6.2m, comprising £5.9m in fundraised income, £200k in investments and £100k other income. We have also received a grant of £2m Changing Places grant to be distributed on behalf of the Department for Transport.
4b	**Has the organisation received any funding from the manufacturer(s) of the technology and/or comparator products in the last 12 months? If so, please state the name of manufacturer, amount, and purpose of funding.**
	SMA UK Since 1st November 2019, we have received the following funds from pharmaceutical companies:

Date	Manufacturer	Amount	Purpose of Funding
Jan 2020	Roche	£21,000	Towards two-year events programme – cancelled due to covid – agreed in view of Covid's impact on charity income that this could cover running costs
May 2020	AveXis	£40,000	Covid emergency grant to support core services
May 2020	Biogen	£39,124	Covid emergency grant to support core services
June 2020	Roche	£25,000	Covid emergency grant to support core services
June 2020	Roche	£8,200	Grant to support Community Connections project
	Total	**£133,324**	

This was 16.3% of our income during this period. Our applications for help to maintain services were driven by the huge impact of Covid-19 on income experienced across the economy and charity sector. In the financial year 2019 / 20, 6.8% of total income was from pharmaceutical grants.

MDUK

Manufacturer	Amount	Purpose of Funding
PTC Therapeutics International	£40,412	MDUK / NorthStar funding to support data collection for ataluren MAA (four x quarterly payments of £10,103)
PTC Therapeutics International	£15,000	Sponsorship of the Muscles Matter online seminar series; Living with a muscle-wasting condition in 2020 and beyond
BIOGEN IDEC LIMITED	£6,000	Sponsorship for 2017 Neuromuscular Translational Research Conference
Roche	£20,000	Grant to support MDUK services during Covid-19 pandemic

4c.	**Do you have any direct or indirect links with, or funding from, the tobacco industry?**
	SMA UK - No **MDUK** - No
5	**How did you gather information about the experiences of patients and carers to include in your submission?**

	Though the SMA Community has been inundated with surveys over the last 4 years and, as one respondent put it, "*I'm tired of filling out a million surveys explaining my view, my life, my experiences",* we decided that this was still one important way to obtain a cross section of views about risdiplam. Our survey was advertised via SMA UK and MDUK communication channels. It was open 25th Sept - 18th Oct 2020. We received **137 responses**: 71 adults / young adults who have SMA (52%); 32 parents of young people < 18 years old who have SMA (23%); 32 other relatives (23%); 2 parents bereaved by SMA (2%). The clinical classification given to the person with SMA who was subject of the survey was: Type 1 - 7%; Type 2 - 50%; Type 3 - 38%; Type 4 - 3%. (See Appendices 1 - 7). Rather than repeat the same questions, we have also drawn on results of the joint charities' (SMA UK, MDUK, TreatSMA) 2018 survey which were submitted to NICE as part of the nusinersen appraisal. These included 128 returns describing the health-related impacts of SMA (full survey results not included as appendices but available here: smauk.org.uk/our-surveys-about-the-impact-of-sma-and-views-about-access-to-nusinersen Our submission is also informed by the contact our Support & Outreach Service has with many adults and families and our community contact networks.
Living with the condition	
6	**What is it like to live with the condition? What do carers experience when caring for someone with the condition?**
	Although 5q SMA is clinically classified into different 'Types' (see also Q.12) which reflect the potential severity of its impact, it is considered a spectrum. For children and adults, the severity of the condition varies from person to person, both within and between 'Types' - each child and adult is affected differently. Care and management as recommended in the 'International Standards of Care for SMA' should always be provided. The physical milestones describing people's ability to sit, stand and walk are increasingly important when it comes to care and management decisions. For simplicity, the summary words 'non-sitters', 'sitters' and 'walkers' are often used. The 128 respondents to our 2018 survey were either adults / teenagers with SMA or parents of children who have SMA, with the person with SMA ranging in age from < 2 years to 66+ years. They vividly described their day-to-day experiences in many pages of responses to the question, '***What are the biggest challenges of living with SMA?***' A few representative quotes: "*The hardest part of SMA for me is the regression...to watch your child lose his greatly achieved milestone it's heart-breaking, you can't explain to him why he can't do that thing he was doing two months ago*." **Father – child age 0-2 years** "*My grandson is unable to walk or stand and can sit only with support. He is susceptible to serious respiratory problems…this leads to frequent emergency admissions to PHDU and PICU for up to 5 weeks at a time - the stress placed both on the child and probably more so on the parents in these dangerous situations is immeasurable*." **Grandparent – child age 3-4 years**

	In terms of mobility, 83% used powered wheelchairs, 68% used manual wheelchairs and 21% used Wizzybugs - designed for children age 18 months - 3 years who are unable to walk. "*As he gets older and bigger the strain of moving and carrying him means more adaptations are needed in the home and less places are accessible. Joining in at school is becoming more difficult. Not being able to go to friends and family's homes. Needing to be turned in the night. Struggling with weight gain. Watching him become less balanced, not being able to sit unaided. Everything getting weaker*." **Aunt / Uncle – child age 5-12 years** "*My grandson is now unable to walk unaided and uses a wheelchair all the time. He is also slowly losing the strength in his arms. Until the age of 15 he was at least able to walk albeit slowly so you can imagine how frightening it is for the whole family to see how quickly he is deteriorating. It affects us all emotionally, and my grandson physically and practically. He has days when he just can't come to terms with what is happening to him."* **Grandparent – child age 13-17 years** Full support - more than would be expected considering the age of the person - was needed for people to go to the toilet (78%), wash (74%), dress (81%), transfer (80%), eat and drink (31%) and, for those who required this, to prepare meals (75%). Between 10 – 42% of others required some support with these tasks. 66% required night care as they were unable to turn over at night or were, for example, needing night-time invasive ventilation (29%). For 64% of these, this care was needed between 3 – 6+ times each night. "*I cannot do the simplest things on my own: lift my hand to my face, pick up a cup with water, keep my head upright….I cannot go to meet my friends on my own, I cannot go to their houses (not accessible), I cannot hang out with them without having everything pre-arranged so a carer is present*." **Young person - age 13-17 years** "*My son …has become more isolated, doesn't want his friends to see that he can't hold his head up if it falls forward so avoids putting himself in a position where he might need to ask for help and has slowly been pulling away from going out."* **Mother - child age 13-17 years** Support was needed because of people's muscle weakness and the other health impacts of the condition: contractures (84%), pain (62%), scoliosis (60%), fatigue with oral feeding (50%), constipation (45%), bone weakness (41%), breathing difficulties (40%) and other health problems. "*Physically, I am unable to do anything for myself as all my muscles are that weak now; I cannot walk, stand, transfer, change position independently, hold a pen to write, cannot move or turn over a piece of paper, send a text, use a cash point, clean my teeth, blow my nose, brush my hair, shake your hand, put make up on, scratch an itch, wipe my bottom, feed myself, hold a cup, cuddle my son…"* **Adult age 46-55 years** 48% had no paid support, 25% had between 1 – 10 hours each 24-hour period and 27% had between 11- 24 hours. Respondents described unpaid support for the 128 people with SMA coming from a range of 146 different people with 75% of respondents receiving support from parents. These unpaid carers had other caring responsibilities as well. 51% cared for other children, 32% for ageing relatives. Additionally, 39% of the 146 carers had had to give up work completely due to their caring responsibilities, 25% had dropped to part-time.

	"*I am a qualified professional and would love to return to work full time…I am unable to sleep at night as I have to roll my daughter frequently….All the hospital appointments, treatments, surgeries, etc take up a lot of our time….I have to do all of the household chores…while my kids are at school, because as soon as my disabled daughter is home she needs my help with everything (bathing, toileting, physio, getting dressed, doing homework, etc). My able-bodied daughter often feels neglected…and I am constantly torn and feel guilty…SMA has had a huge negative impact on the whole family in every area of our lives - financial, emotional, marital, personal, self-fulfilment and physical health*." **Mother - child age 5-12 years** All those affected by and living with the condition and their carers described in their different ways the emotional impact of the condition – the 'chronic sorrow' associated with their ongoing living loss.
Current treatment of the condition in the NHS	
7.	**What do patients or carers think of current treatments and care available on the NHS?**
	Management interventions, particularly for infants, focus on **correct positioning** and ameliorating **breathing difficulties**. These include: chest physiotherapy; oral suctioning; medication to reduce secretions; cough assist; non-invasive ventilation. This is very time-consuming for parents and can be distressing for both them and their child. In the 2020 survey responses, breathing ability was affected and interventions needed for 59% of children and 48% of adults. **Spinal scoliosis**, with its physical and emotional impact, is often managed initially with a lycra suit, spinal brace or jacket but surgery may be recommended if it is contributing to breathing difficulties, preventing comfortable sitting or the curvature has progressed beyond a certain point. In the 2020 survey response, 61% of adults and 68% of the children had had interventions due to scoliosis. **Physiotherapy** helps manage contractures (2020 survey: moderate / severe experienced by 50% of children and 46% of adults) and resultant pain, chest physiotherapy helps manage breathing difficulties. Few adults have access to the physio they need, and many children miss out. Interventions to manage choking, swallowing, fatigue with feeding, digestion, constipation and managing weight, may include **tube feeding, gastrostomy, medication** and **dietary management**. A major management tool, also, is vigilance and time on the part of carers and with this comes the stress of being constantly 'on high alert'. To manage the impact of their condition, the children, young people and adults who responded to the 2018 survey were having to use **powered wheelchairs** (83%), **manual wheelchairs** (68%), **wheelchair accessible vehicles** (66%), **specialist beds** (63%), **hoists** (60%), **orthotics** (54%), **specialist seating** (50%), **assisted cough machines** (38%), **nebulisers** (31%) and **assistive technology** (30%), as well as other equipment. They required **adaptations to toilet and bathroom facilities** (73%) as well as **other home adaptations** (69%).

	"*Practically our house is full of medical devices and equipment. If we want to go on a trip overnight there is an assisted cough machine and a nebuliser to take, as well as a sleep aid and maybe a specialised chair. Our 'normal' is very different from most peoples'*." **Father - child age 0-2 years** Many described the frustrations they experience in their efforts to secure the support they need in their day-to-day lives: "*Being on a wheelchair referral waiting list for so long. Waiting for possible adaptions to house, ground floor bedroom for son as stairs a hazard. As a parent the emotional stress of watching my son's strength quickly deteriorating is unbearable*."**Mother - child age 5-12 years** For 57%, **the number of health and social care professionals** involved range from 6 - 20. Attending appointments and generally managing to coordinate care and support depends on the complexity of the individual's condition and can be very time consuming. **Many of the interventions / equipment to manage the condition were not, and still are not, funded by the NHS and, although funding may be secured via other statutory sources, many are invariably secured privately or via charitable funding, creating significant financial pressure on families. For example, for these respondents, the NHS was not funding 50% of their powered wheelchairs, 27% of hoists, 36% of toilet and bathroom adaptations, 52% of other home adaptations. The majority of children under the age of 3 years could not, and still cannot, access NHS funded powered chairs so 71% of families find funding for their 'Wizzybugs'.** As best supportive care is the comparator, we have not referenced views on nusinersen treatment here. We do though note that: as it is delivered by lumber puncture, spinal scoliosis / intervention can prevent safe administration; the current Managed Access Agreement combined with the very slow roll out of treatment, in particular for adults, means that its availability is limited.
8.	**Is there an unmet need for patients with this condition?**
	Yes – best supportive care does not prevent the progressive weakening of muscles. A number of adults in their 2020 responses clearly state this and their unmet need: *"It's a devastating disease no matter what type you are. It steals your abilities and in turn steals your life."* "*It would mean a lot to be able to continue to support my neck and head as this is so important for safe eating and swallowing. It is so important that my muscles maintain as much strength and stability possible to make breathing and fighting illness easier. I really want to be able to keep my independence and carry on using my hands to drive my wheelchair, hold my toothbrush, use my phone, write and use the computer for as long as I possibly can*."

	"*SMA is unpredictable and can progress at any speed, at any moment. Anything to delay that progression, or maintain existing strength, will do the world of good to people's physical and mental health. It's not fun thinking you've reached middle age at 15, and society has a lot to do to make the world more inclusive and accessible*." *"Tiny margins of increase or halting decrease would have a huge impact on all areas of my life."* In terms of access to any new drug treatment, we note the comparator is best supportive care, also that of the 2020 survey respondents, 38% of children and 93% of adults had not had access to any clinical trial / new drug treatment.
Advantages of the technology	
9.	**What do patients or carers think are the advantages of the technology?**
	To accompany our 2020 survey, our Scientific Research Correspondent compiled summaries of the clinical trial evidence to date (Appendices 5-6). This was made available to all 137 respondents and was read by 91%. These were the views expressed: **Q. What in your view are the advantages / disadvantages of aspects of risdiplam treatment**

	Strong Advantage		**Advantage**		**Neither Advantage nor Disadvantage**		**Disadvantage**		**Strong Disadvantage**		
	1		2		3		4		5		Total
	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	
How it is taken (syrup by mouth)	89	122	7	10	3	4	0	0	1	1	137
How often it has to be taken (daily)	51	70	24	33	23	32	1	1	1	1	137
How long it has to be taken for (as long as treatment continues)	48	66	23	32	27	37	1	2	0	0	137
Where it can be taken (at home)	93	128	4	6	1	2	0	0	1	1	137
Where it must be stored / kept (refrigerated)	48	66	23	32	25	34	4	5	0	0	137
See also Appendix 4 for additional comments									**Total answering**		**137**

Q. Views on aspects of what is known so far about risdiplam

	Very positive		Positive		Neither positive nor negative		Negative		Very negative		Don't know		Total
	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	%	Nos	
Its safety profile	52	71	36	49	7	9	1	1	0	0	4	6	136
Its recorded adverse events profile	25	34	36	49	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
Its impact on swallowing	46	62	30	41	13	17	0	0	0	0	12	16	136
Its impact on ability to communicate	35	48	35	47	15	20	0	0	0	0	15	21	136
Its impact on breathing ability	46	63	33	45	9	12	1	1	0	0	11	15	136
Its impact on frequency and duration of hospital stays	46	63	29	39	10	13	1	1	0	0	15	20	136
Its impact on stamina and fatigue	53	73	35	48	3	4	0	0	0	0	9	12	137
Its impact on quality of life	66	91	25	34	2	3	0	0	0	0	7	9	137
Its impact on female menstruation	11	14	8	10	41	54	6	8	4	5	32	42	133
Its impact on female fertility	7	9	5	6	42	56	8	10	5	6	34	45	132
Its impact on male fertility	6	8	8	11	42	57	10	14	6	8	27	37	135
See also Appendix 4 for additional comments										**Total answering**			**137**

As one adult put it: "*It's non-invasive and can be self-administered at home without medical professionals. That's a milestone*."

A family member stated:
"The lack of requirement to have a surgical procedure with risk of infection is a plus. Loss of school days for visiting hospital 150 miles return in a day is of enormous benefit. At a time when hospital visits are only possible in emergency cases home treatment and administration is a definite positive to reduce risk of catching COVID 19 on journeys and in hospital. It releases clinicians to do other essential work."

100% of the 71 adults with SMA responding said they would want access to risdiplam. 97% (30) of the 31 parents of < 18-year olds with SMA responding said they would want their child to have access. We note that this includes 17 children under 18 years and one adult who are all currently receiving nusinersen treatment and live in England (see Appendix 7).

Disadvantages of the technology	
10.	**What do patients or carers think are the disadvantages of the technology?**
	Please see above tables.
Patient population	
11	**Are there any groups of patients who might benefit more or less from the technology than others? If so, please describe them and explain why.**
	In general, both clinical trial and real-world evidence for all current drug development suggests that early treatment may be necessary to maximise the potential benefits. Though we acknowledge this, the importance of stabilisation or even the smallest benefit for people impacted by a progressive muscle wasting condition cannot be stressed enough. In 2019, **96.7% of 1,327 validated responses to SMA Europe's SMA Community survey** stated they would "*consider it to be progress if there was a drug to stabilize their current clinical state*." All who have 5q SMA should have the opportunity to have NHS funded access to this treatment with a decision to go ahead or not based on a grounded and realistic discussion with their clinician about the potential benefits and any risks to them individually.
Equality	
12	**Are there any potential <u>equality issues</u> that should be taken into account when considering this condition and the technology?**
	The clinical classifications and 'Typing' of SMA was introduced in 1990 by a committee of clinicians and geneticists to promote collaborative studies between different centres and to identify the genes of SMA. Their classification was based primarily on the age of onset and the age of death, with the ability to sit unaided and stand and walk unaided added on. The classifications were never meant as a way to make decisions about who should / should not have access to treatment (V Dubowitz writing in 'SMA Disease Mechanisms and Therapy' edited by Summer, Paushkin & Ko, 2016).

	As one respondent put it, "*The diagnosis needs to be as dynamic as the condition…The etymology of the disease dictates that wherever people start on the continuum of sma they are on an ever-decreasing scale. As such if you start as a type 3 or type 2 eventually those people have the same end point."* To tie access to this treatment to 'Type' would be discriminatory. This includes those who are labelled as 'Type 4' who have the same genetic cause for their condition. As one person put it: "*In the past all research has been focusing on type 1 and 2 and 3. Nothing on type 4. Is type 4 not as important? Is my life over with nothing to look forward to except caregivers and an old folks' home?"* We note that: young adults may 'deny' symptom onset or have symptoms dismissed; that the road to diagnosis can be very delayed; in some countries, where the clinical classification of Type 3b and Type 4 is sometimes viewed as less distinct, drug treatment may be possible for some individuals with SMA symptom onset over the age of 19 years of age; numbers with this clinical classification are very small; life expectancy is normal and a treatment that could stabilise or improve progressive muscle weakness would greatly improve its quality. We are very concerned about the potential for geographical inequalities in accessing treatment for SMA. We know some neuromuscular centres have not been able to provide nusinersen for adults with SMA who are eligible for treatment. In addition, there have been challenges in equitably rolling out the Early Access to Medicines Scheme for risdiplam across all sites (again for adults). Many adults and children with SMA are powerchair users who, with the support of personal assistants and / or parents / carers, manage a complex and challenging disability due to their progressive muscle weakness. Travel is always a logistical challenge and though this is a treatment that is taken at home, at least initial health assessments will need to be centre based. It is therefore vital that access to treatment is offered at a centre as close as possible to where people live.
Other issues	
13	**Are there any other issues that you would like the committee to consider?**
	We recommend **access for all** but recognise that when it comes to NICE making a recommendation and NHS England and clinicians rolling out an access programme, there will need to be prioritisation. To some extent this will be impacted by what access to other treatments is possible for different groups. We suggest priority needs to be given to **those who have no other treatment option**, in particular those who are prevented from accessing nusinersen due to the Managed Access Agreement's eligibility, starting and stopping criteria and, if funded, those infants who are, for clinical reasons, unable to access onasemnogene abeparvovec. It will be vital to have:

- accurate evidence-based, user-friendly summaries about each treatment and how they compare to each other that clinicians can use to discuss options with patients and their families
- comparable clinical outcomes recorded for all treatments on the SMA REACH UK paediatric and adult databases linked with appropriate databases / ways of recording patient reported outcomes
- publication of reliable accurate evidence-based, user-friendly updates and reviews that compare the performance of new treatments

14. **Please outline what carers and patients consider to be meaningful treatment outcomes for each SMA type**

The above comments (Q.11) on the value of 'stabilisation' needs to be borne in mind when considering the following responses to our 2020 survey question as to what outcomes would be valued by respondents. For '*improvements'* the vast majority of people would substitute '*stabilisation*' as a meaningful outcome. The value, meaning and measurement for each of these outcomes should not be determined by Type, but be based on how an individual's SMA is impacting on them at the time treatment starts.

Q16. How important would improvements in different aspects of the person with 5qSMA's health and daily living be if these could be affected by a drug treatment

	Very important		Important		Neither important or not		Not important		Not at all important		Total
	%	Nos	%	15	%	Nos	%	Nos	%	Nos	
Improved motor milestones - e.g. ability to sit, stand, walk	79	105	17	22	5	7	3	4	2	2	133
Improved breathing ability	66	87	16	21	11	14	5	7	2	2	132
Improved swallowing / ability to eat	64	85	14	19	12	16	5	7	2	3	132
Improved ability to communicate	44	57	18	24	24	31	9	12	9	12	131
Improved stamina and reduced fatigue	79	108	17	23	2	3	0	0	1	2	137
Improved fine motor skills (e.g. movement of fingers)	77	103	11	15	3	4	2	3	1	1	134
Increased independence	84	115	15	20	4	5	1	1	1	1	137
Reduced reliance on caregivers and personal assistants	73	99	2	3	7	10	4	6	1	1	136
See Appendix 4 for all additional comments										**Total answering**	**137**

	These adults and parents clearly illustrate what are meaningful outcomes for so many: "…*maintain the milestone of sitting up in my wheelchair and ensure I can maintain the ability to type, use my phone, and put on my make-up. I would love it if I could one day open a packet of crisps. I thrive off independence and I would be so much less reliant on people if I could open a pen to write, open a door, or open a bottle of water. I am terrified of losing my ability to swallow and communicate."* **Adult** *"Ability to move in bed, possibly go to the toilet or make a cup of tea would be amazing."* **Adult** *"Maintaining strength for independence and mental health is vital*." **Parent** *"Walking 5 independent steps is by far not the most valuable…Improvement in back and neck strength, the ability to transfer, cut up food is of far greater importance…"* **Parent** *"Self-confidence and mental health would improve dramatically with treatment as well as my daughter's general belief of self-worth, which she has very little of currently because of her SMA condition!"* **Parent** *"She is of an age where image and independence Is key as much as stamina to keep up with peers at secondary school where workload has quadrupled!"* **Parent**

15. Key messages

In up to 5 bullet points, please summarise the key messages of your submission:

- Best supportive care does not prevent the progressive weakening of muscles.
- The importance of stabilisation and even the smallest benefit of a treatment for people impacted by a progressive muscle wasting condition cannot be stressed enough.
- **All** who have 5q SMA should have the opportunity to have NHS funded access to risdiplam treatment, with a decision to go ahead or not based on a grounded and realistic discussion with their clinician about the potential benefits and any risks to them individually.
- The value, benefit and measurement for each meaningful outcome should not be determined by 'Type' but be based on how an individual's SMA is impacting on them at the time treatment starts. The classifications by 'Type' were never meant as a way to make decisions about who should / should not have access to treatment.
- We suggest priority needs to be given to those who have no other treatment option.

Thank you for your time. Please log in to your NICE Docs account to upload your completed submission.

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