Neutral Citation Number: [2021] EWCA Civ 278

Case No: C1/2020/2107

IN THE COURT OF APPEAL (CIVIL DIVISION)
ON APPEAL FROM THE QUEEN'S BENCH DIVISION
ADMINISTRATIVE COURT
His Honor Judge Bird (sitting as Deputy High Court Judge)
[2020] EWHC 3189 (admin)

Royal Courts of Justice
Strand, London, WC2A 2LL

Date: 02/03/2021

Before:

LADY JUSTICE KING
LORD JUSTICE BAKER
and
LADY JUSTICE ELISABETH LAING

- -

Between:

BASMA (SUING BY HER MOTHER AND LITIGATION FRIEND SARA BASMA)	**Appellants**
- and -	
MANCHESTER UNIVERSITY HOSPITALS NHS FOUNDATION TRUST & ANR	**Respondents**

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Tim Buley QC (instructed by **Irwin Mitchell Solicitors**) for the **Appellants**
Fenella Morris QC and Benjamin Tankel (instructed by **Manchester University NHS Foundation Trust and Great Ormond Street Hospital for Sick Children NHS Foundation Trust**) for the **Respondents**

Hearing date: 11 February 2021

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Approved Judgment

Covid-19 Protocol: This judgment was handed down remotely by circulation to the parties' representatives by email, release to BAILII and publication on the Courts and Tribunals Judiciary website. The date and time for hand-down is deemed to be at 10:00am on 2 March 2021.

Lady Justice King:

Introduction

1. Sophie Basma ("Sophie") is 10. She suffers from Type 3 Spinal Muscular Atrophy ("SMA"). SMA is a rare, genetic, neuromuscular disease which progressively leads to sufferers being unable to walk or sit unaided with devastating consequences on their quality of life. Sophie can no longer walk.

2. Sophie is under the care of Dr Imelda Hughes (consultant paediatric neurologist at the Royal Manchester Children's Hospital) and Dr Mariacristina Scoto (former locum consultant and now full consultant in Neuromuscular Translational Research at Great Ormond Street hospital for Children NHS Foundation Trust ("GOSH")) ('the Respondents').

3. In lay terms, the issue before HHJ Bird in judicial review proceedings brought on 13 August 2020 by Sophie's mother on her behalf, turned on whether the Respondents were acting unlawfully when they decided that they could not be satisfied that, at some stage between October 2018 and October 2019, Sophie was able to walk five steps in an upright position, with a straight back and with no contact with a person or object.

4. This seemingly simply matter was of critical importance to Sophie as her proven ability to walk those five steps determined whether she would qualify for treatment with a newly approved drug, Nusinersen (marketed and sometimes referred to as "Spinraza").

5. Nusinersen is agreed to be clinically appropriate for Sophie. Whilst it is unclear how effective it might be in the long term, there is good evidence of clinical effectiveness. It may have the potential to be life changing, in that it may help Sophie to regain her ability to walk and to maintain significant levels of upper body strength against, what is otherwise, an inevitable gradual decline to complete immobility.

6. Nusinersen is very expensive but that is not the issue here. Under the relevant, legally binding National Institute for Health and Care Excellence ("NICE") guidance in force since October 2019, Nusinersen is to be provided to Type 3 SMA sufferers provided that certain criteria ("the MAA Criteria") are satisfied. It is common ground that Sophie satisfies all but one of those criteria. The one that is controversial is "the 5 Steps Criterion" which relates to whether she was able to walk five steps unaided in the twelve months before she became entitled to be considered for the drug (October 2019).

7. Dr Hughes and Dr Scoto decided that, for reasons set out later in this judgment, Sophie was unable to satisfy the criteria and was therefore not entitled to be treated with Nusinersen.

8. These are judicial review proceedings. The appellant ("Ms Basma") is, therefore, obliged to couch her submissions in relation to the decision making of Dr Hughes and Dr Scoto in terms of 'unlawful' and 'irrational'. As was discussed during the hearing, such terms are professionally hurtful and upsetting to doctors such as Dr Hughes and Dr Scoto who are experts in their highly specialised fields. As will be seen in the

course of this judgment, I have concluded that decisions made by each of the doctors were both 'unlawful' and, 'irrational;' I should make it absolutely clear that in reaching those conclusions and by the use of those terms, I am not to be taken as doing other than having the highest regard for the expertise and dedication of each of these two consultants, nor should it be thought that their decisions were influenced by any financial considerations. It is clear from the papers that each of them had a strong desire to prescribe Nusinersen for Sophie, but each concluded that the interpretation they felt compelled to adopt of the 'the 5 Steps Criterion' meant that they were unable to do so.

9. On 23 November 2020, HHJ Bird sitting as a High Court Judge granted permission to Sophie's mother, Ms Basma to bring judicial review proceedings but dismissed the application for judicial review. It is against that decision that she now appeals.

Legal Framework

10. The legal framework is not in dispute and is set out at para. [11] – [17] of the judge's judgment ([2012] EWHC 3189 (Admin)). For the purposes of the appeal the relevant law can be stated shortly.

11. NICE was established by section 232 of the Health and Social Care Act 2012 ("the 2012 Act"). Its general duties are set out in section 233 of the 2012 Act. By section 237, the Secretary of State for Health may issue regulations authorising NICE to give "advice or guidance".

12. The resulting regulations are the Health and Social Care Information Centre (Functions) Regulations 2013 ("the 2013 Regs"). By Regulation 7 of the 2013 Regs, NICE may publish a "technology appraisal recommendation" ("TAC") for the use of a particular medicine or treatment. By Reg 7(6) 2013 Regs, "a relevant health authority must comply with a technology appraisal recommendation".

13. On 24 July 2019, NICE issued a TAC, *TA588 Nusinersen for treating spinal muscular atrophy*. Nusinersen was recommended as an option for treating Type 3 SMA "only if the conditions in the managed access agreement are followed". The recommendation took effect three months later on 24 October 2019.

14. Under the heading of "Your Responsibility" the TAC emphasises that the recommendations in the guidance are at the discretion of the healthcare professionals and their individual patients and "do not override the responsibility of healthcare professionals to make decisions appropriate to the individual patient".

15. Following a marketing authorisation for Nusinersen to Biogen Netherlands BV, Biogen, NICE and NHS England negotiated a "Managed Access Agreement" ("the MAA"). The MAA is not a creature of statute. It is a commercial agreement binding by way of the ordinary laws of contract. Its authority comes from the fact that the TAC (which does have a statutory footing) makes a binding recommendation that Nusinersen is an option for those who meet the conditions set out in the MAA. The MAA sets out (i) a statement of the clinical criteria for starting and stopping treatment with Nusinersen and (ii) a data collection plan to evaluate the performance of Nusinersen over the 5 years that the MAA is to run (para.2.3 MAA)

16. At paragraph 4.5, the MAA sets out seven entry criteria which have to be satisfied before a Type 3 SMA patient will become eligible for treatment with Nusinersen.

17. It is not in dispute that Sophie satisfies six of the seven criteria. The dispute has arisen as to whether she satisfied the 5 Steps Criterion (bullet point 5 in MAA 4.5), which says:

> "If gained independent ambulation prior to initiation of therapy must still be independently ambulant, with the exception of paediatric patients who have lost independent ambulation in the previous 12 months. Independent ambulation is defined as per the WHO definition: patient takes at least five steps independently in upright position with back straight. One leg moves forward while the other supports most of the body weight."

18. There is, within the MAA, recognition of the fact that the 5 steps Criterion is neither fixed by a single assessment on any particular day nor, as is conceded on behalf of the Respondents, is it fixed by reference only to a formal clinical assessment.

19. People with SMA have 'off days'; by way of example paragraph 5.6 in relation to the collection of data provides:

> "Any two entries need to be at least 4 months apart. Two data points a year will counteract the outcome variability due to "off" days and acute reversible illness. The time spacing is designed to coincide with either routine 6 monthly follow up clinic appointments or 4 monthly maintenance doses."

20. Similarly, in Table 1, which provides for endpoints and stopping rules, two consecutive measurements are required before stopping "in order to allow for confirmation of worsening and not an 'off' assessment day."

21. The MAA provides for NHS England to establish an expert Clinical Panel ("the Panel") whose role is to "provide advice to treating centres on interpretation of the MAA criteria, including starting and stopping criteria, and diagnosis". (Para. 5.17 MAA)

22. As the judge said at para.[18] "It is clear and not in dispute, that the person identified by NICE as the decision maker, is the treating consultant…. It is plain that the function of the panel is to "provide advice" to the decision maker and not to make decisions itself." I would add that equally, its role is not to act as an appeal or review body in relation to the decision of a treating consultant.

Evidence and decision making relating to Sophie's ability to walk 5 steps

23. The critical issue is Sophie's ability to walk five steps in accordance with the MAA specifications in the 12-month period preceding 24 October 2019.

24. It is common ground that during that period Sophie's ability to walk deteriorated but it is also clear that: (i) Sophie could walk in excess of 5 steps until at least January

2018 when her local physiotherapist recorded her as having walked '5/6 metres in November 2017' and 'approximately 2 metres' in January 2018; and (ii) that by September 2019 Sophie could no longer take any steps unaided.

25. The issue for the doctors was, therefore, whether Sophie had lost the ability to walk 5 steps before 24 October 2018.

26. What at first blush would seem to be a straightforward assessment was made difficult by the fact that during the critical period (October 2018 – October 2019) there was a dearth of formal assessments of Sophie. The interpretation of such evidence as there was, was made more difficult by the fact that during the 12-month period under consideration no one, whether health care professional, teacher or family and friends knew that whether Sophie could manage 4, 5 or even 10 steps had any particular relevance, as at that time Nusinersen had not been approved. As a consequence, no one paid specific attention or made any record of how far she walked with a view to satisfying, what would become 'the 5 Steps Criterion'.

Formal Assessments

27. A formal assessment of Sophie took place on 12 February 2018 at the Royal Manchester Children's Hospital. The physiotherapist, Ms Warner, conducted a full assessment using the 'Revised Hammersmith Scale for SMA'. The form records Sophie as having walked 4 steps unaided. Ms Morris QC, on behalf of the Respondents, drew the attention of the court to a note at the top of the form which records 'Walks with hand held'. Mr Buley QC, on behalf of Sophie, rightly observed that that could be a simple reference to the fact that meaningful walking requires her to have her hand held. I agree that no other inference can be drawn given that the reference to '4 steps' is found within a specific column (Column 18) which specifically asks if the patient can take at least 5 steps unaided, which column is completed to show Sophie walking '4 steps'.

28. This is the only entry in the records between February 2018 and September 2019 of Sophie being formally assessed as walking only 4 steps. There is no second entry, and so no opportunity for a decision maker to ascertain if Sophie was having an "off" day on 12 February 2018.

29. Given that no one knew the potential importance of this observation, Sophie's mother, Ms Basma, was not asked whether 4 steps were now the limit of Sophie's ability to walk or whether it perhaps varied from day to day. Following the physiotherapy appointment, Sophie was seen by Dr Hughes. No separate assessment of her ability to walk was undertaken. Dr Hughes' letter to Sophie's GP, written after the appointment, records Sophie 'as only walking with two people helping'. In my judgment, this takes the matter no further as it would not be in the least bit surprising if an ability to walk only 4 steps (or even 5) was not regarded as 'walking' in everyday language as opposed to the technical definition of 'independently ambulant'.

30. In May 2018 her physiotherapist referred to Sophie 'going off her feet quite quickly'; an observation which does not help in deciding if she could still walk 5 steps at that stage.

31. Sophie was next seen by a consultant on 8 October 2018; Dr McCullagh, in the absence of Dr Hughes. On this occasion there was no Revised Hammersmith Scale physiotherapy assessment. In his letter, Dr McCullagh refers to Sophie as being 'no longer ambulant and needs assistance for transfers'. When asked by Dr Hughes, Dr McCullagh (unsurprisingly) had no recollection of the meeting but thought what he wrote in the letter must have been based on information from Sophie's mother. Mr Buley submits that this takes the matter no further. All this does is confirm the undisputed picture of Sophie's ability to walk being now quite limited and deteriorating, but it does not assist on the critical questions of whether by that stage she could no longer walk 5 steps on her own, and, if so, when she ceased to be able to do so.

32. In July 2019, TAC 588 and the MAA were published to take effect in October 2019. Sophie was seen by Dr Hughes on 9 September 2019. On this occasion the Revised Hammersmith Scale physiotherapy assessment shows Sophie able to cruise at least 5 steps around furniture but no longer able to take steps unaided. Ms Basma described the meeting with Dr Hughes in her witness statement as a routine meeting at which she says she raised the issue of Nusinersen with Dr Hughes. Ms Basma said that Dr Hughes had told her that Sophie would not receive the treatment due to Ms Warner's assessment in February 2018. Ms Basma went on to say that this was presented as a decision already made and that even though she made it clear that she did not agree with Ms Warner's assessment she (Ms Basma), was not asked for any input into Sophie's ability to walk during the last 12 months,

33. In a letter to Sophie's GP dated 11 September 2019, Dr Hughes said:

> "…. Patients with Type 3 SMA are only eligible if they have been ambulant within the preceding 12 months. In February 2018 I documented that [Sophie] was no longer independently ambulant, at that time of her physiotherapy assessment her physiotherapist documented that she was only able to take four steps [Sophie]… was reviewed by my colleague Dr McCullagh in October who again recorded that she was no longer independently ambulant, unfortunately they attended late for the appointment so that it was not possible to perform her physiotherapy assessment. Mother disputes this and says that within the last 12 months Fifi has been able to walk more than 5 steps …. I have to be able to confirm that a patient meets the eligibility criteria and the evidence I have suggests that [Sophie] does not. I have suggested if mother has any previous videos of [Sophie]walking independently within the last 12 months I would be very happy to receive these. We will also make contact with her local physiotherapist. For difficult cases there will be an oversight panel who will advise on eligibility and when this panel has been appointed we can, of course consult them. For the moment I do not think I can confirm to NHS England that [Sophie] meets the eligibility criteria for Nusinersen."

34. A letter was sent to Ms Basma on 12 September explaining that when the unit had first become aware of Nusinersen, Sophie had been on the list of patients expected to receive it. The letter went on:

> "However when we read the terms of the NHS England Managed Access Agreement (MAA) we were dismayed by their criteria applicable to type 3 and realised that [Sophie] would not be eligible."

35. Ms Basma did not have a video of Sophie walking on her own during the relevant period but sent a video that she had taken. In her witness statement, Dr Hughes said:

> "When the MAA was published [ie in July 2019] we realized (*sic)* that [Sophie] was not eligible as our records suggested that she was not independently ambulant since at least February 2018."

36. Dr Hughes explained how she had sought additional information from Sophie's local physiotherapist, but that she had no formal assessments. Dr Hughes went on to say that Ms Basma had sent a video of Sophie walking but that she was holding onto a table, and sent a photo of Sophie standing holding the hands of two little boys from September 2018 and that Ms Basma said that on that day Sophie had walked from the car park to that position.

37. Mr Buley submits that it is plain that as soon as she saw the 5 Steps Criterion, Dr Hughes decided that Sophie was not eligible and that the only further evidence Dr Hughes has thereafter considered was the video and the single photograph. Dr Hughes did not take into account Ms Basma's protestations that Sophie had been able to walk 5 steps unaided at times during the period of time which was critical to the making of the decision but when Sophie had no contact with health care professionals. It is equally plain, Mr Buley submits, that Dr Hughes regarded the only alternative evidence which would allow the 5 Steps Criterion to be satisfied would be a video recording of Sophie walking 5 steps unaided during the critical timeframe.

Dr Scoto's involvement

38. After Dr Hughes told Ms Basma that Sophie would not be eligible for Nusinersen, Ms Basma sought a second opinion from Dr Scoto at GOSH. They met on 12 December 2019 when a full assessment of Sophie was carried out. Dr Scoto recorded her conclusions as follows:

> "Discussion about accessing to Nusinersen under the MAA:
>
> > We had a long discussion regarding the current inclusion criteria for patients with SMA type 3 who are no longer ambulant, to access Nusinersen under the MAA. [Sophie]'s loss of ambulation (defined as the ability to walk 5 steps independently) has been documented by the colleagues in Manchester in February 2018 when she managed to walk 4 steps during the physio assessment.

> The mother had strong reservation as she can definitely recall Sophie being able to walk more than 5 steps in daily activities till late 2018 (i.e. going from the parking area to the school entrance, or walking on the stage at school during a Christmas play in December 2018).
>
> *I have explained that in order for Sophie to access the drug there must be a formal documentation of her ability to walk and* that, should patients not regain the ability to walk after one year from starting the treatment with Nusinersen, this will be stopped.
>
> As clinicians we will be continuing working to collect evidence on the benefit of Nusinersen after losing ambulation and will keep an open dialogue with NICE and NHSE submitting evidence when available.
>
> *We would be available to review any video showing Sophie's ability to walk in the period between July-2018 and July 2019 and put her case forward for the MAA panel review* should she fit the inclusion criteria.
>
> In the meantime we have recommended to follow the physiotherapist' suggestions, remaining as much as possible active, doing standing in KAFOs and regular stretches." (my emphasis).

39. Dr Scoto referred Sophie's case to the NHS England Clinical Panel. The material sent to the Panel included a letter from Lucy Watson, Sophie's yoga teacher, who gave examples of Sophie walking 5 steps in January 2019 and during the summer of that year. Also enclosed was a letter from Sophie's one-to-one teaching assistant who described Sophie walking 3 metres in December 2018.

40. The Panel replied on 5 May 2020. The letter said that the Panel had concluded that Sophie did not meet the eligibility criteria and said that "Ideally any corroborating written evidence should be from a healthcare professional and must be dated and signed". That advice was subsequently withdrawn and solicitors representing Ms Basma were told that the Panel would "look again" at Sophie's case.

41. Meanwhile, further evidence was gathered by Ms Basma ("the family and friends' evidence") and sent, initially in unsigned form, by Ms Basma's solicitors to Dr Scoto on 7 July 2020. In the light of the additional information from the family and friends, Dr Scoto wrote again to the Clinical Panel on 20 July 2020 attaching a PowerPoint presentation.

42. It is not necessary to go into the detail of the family and friends' evidence. There was extensive evidence from Sophie's mother, family and teachers. It was produced in the form of witness statements for consideration by the clinicians and subsequently more formally, for the purpose of this claim. Sophie's mother filed two statements, and three family members each filed a single statement, as did a family friend. In addition, Sophie's one-to-one teaching assistant and her yoga teacher had each already filed a

statement. Each of these statements gave examples of Sophie walking unaided for at least 5 steps in the relevant period between October 2018 and October 2019. In the main, the witnesses referred to some incident or event which had made the occasion or occasions memorable. It has not been, and is not part of, the Respondent's case that these seven lay witnesses are other than giving honest accounts of what they believed they saw.

43. The letter and the PowerPoint sent by Dr Scoto to the Panel not only demonstrate her strong professional desire to prescribe Nusinersen to Sophie, who, she felt, would benefit from the drug, but they are also, in my judgment, of some importance when considering whether Dr Scoto made, in public law terms, an unlawful and irrational decision in relation to Sophie's eligibility to receive Nusinersen.

44. The relevant part of Dr Scoto's letter reads as follows:

> "*As a clinician I would consider it possible that this child could have walked unaided some time beyond her assessment in February 2018 where it is documented she could walk 4 steps.* Without any repeated formal assessment between then and my clinical review in December 2019, I find difficult to formally pinpoint a potential date/time for loss of ambulation before July 2018, which would exclude her from MAA.
>
> *As you will see, the family have provided statements and pictures (unfortunately no video was provided for review) supporting the possibility she was still independently ambulant between July-December 2018.* In order to ensure equitable access and in the patients' interest to receive the treatment in her regional centre if eligible, I am therefore consulting the panel for advice whether the provided information meets the conditions to be treated in the MAA, and a date/time of loss of ambulation could be deducted from the provided information rather than a formal assessment." (my emphasis).

45. The relevant slide has a photograph of Sophie standing up on the left-hand side and the following text on the right:

> "My questions to the expert panel are:
>
> In the absence of a formal assessment within the timeframe under consideration, and with the benefit of doubt that children can at times not perform at the maximum of their abilities when evaluated in clinic, is the evidence/statements provided by the family acceptable to consider her eligible?
>
> *I am sure that if the family knew that one step would have made such a huge difference, they would have filmed the child walking at any possible/favourable occasion.*

> *Unfortunately the child was not formally assessed for more than 12 months after Feb 2018, and only reviewed after the MAA criteria were published.*
>
> At our last physio session in Dec 2019, it was felt that she could stand and take few steps in KAFOs. Would this information support the possibility that this child was likely to be still independently ambulant in the previous 12 – 15 months?" (*my emphasis)*

46. My strong impression from reading those two documents is that Dr Scoto's view was that, taking into account all the circumstances and evidence available including her own clinical judgment, she for her part would conclude that it was at least possible that Sophie met criterion 5 and was therefore eligible for treatment with Nusinersen if the informal evidence could be taken into account. Her concern centred not upon the cogency of the evidence provided by the family and friends, but simply to the fact that the evidence was not in the form of a 'formal assessment'.

47. There followed a flurry of correspondence between the end of July 2020 and the Panel meeting on 7 August 2020, as Ms Basma was asked for more information to tie in the observations of the family witnesses with the criteria (straight back etc) and to provide, as best as could be achieved, a time line showing Sophie's gradual loss of ambulation over time.

48. A letter was sent by GOSH to Ms Basma's solicitors on 29 July 2020. In the letter they asked whether Ms Basma could obtain additional information to 'help Dr Hughes and Dr Scoto in their decision making'. The letter, however, also set out their approach to the ambulation criteria as follows:

> "The formal observations regarding ambulation are usually conducted by specialist physiotherapist, rather than even by doctors. Factors that are considered necessary to reach a conclusion of independent ambulation…. are taking five steps unaided, not holding on to anything or anyone, with the back in a sufficiently upright posture and without dragging of the feet in the course of making the steps. While neither Trust would insist upon an entry in the clinical records in order to confirm these criteria had been met for any given patient, it is hard for any treating clinician to come to a view that the MAA baseline criteria for provision of Nusinersen had been met without some evidence that could reasonably be said to address each of those factors.
>
> *If the best clinical baseline evidence for ambulation could be said to come from a physiotherapist or (failing that) another clinical professional or (failing that) at least someone with a solid prior understanding of the technical nature of the observations, both doctors accept that video evidence can form compelling evidence."* (my emphasis).

49. It would seem, therefore, that either clinical or video evidence was required before Sophie would satisfy the 5 Steps Criterion. Dr Scoto was diligent both in seeking additional information from Ms Basma and in referring it on to the Panel. On 6 August in an email, Dr Scoto said:

> "Again as a clinician, my request for the panel is to advice if this type of evidence, in the absence of a formal assessment, can be used surrogate to establish time of losing ambulation and therefore eligibility for the MAA."

50. Following the meeting of the Panel on 7 August 2020, the Panel wrote to Dr Scoto with its advice on 15 September 2020:

> "…..after lengthy discussion the Panel concluded that in its judgement there was not sufficient objective and clinical evidence demonstrating that the patient was able to walk in the relevant time period to change the original advice given by the Panel. The Panel would have placed greater weight on assessments by a regulated health care professional in a clinical setting as these are more likely to be objective, rigorous, consistent and accurately recorded at the time. The Panel placed less reliance on the evidence of family members and others, particularly where that evidence was based on recollections some time after the events in question. As such the Panel considered this evidence could be unreliable, but did not consider that it was intentionally incorrect.
>
> I hope the Panel's advice will assist you in reaching your decision as to your patient's eligibility under the MAA."

51. As Ms Morris rightly reminded the court, the Panel's advice is not the subject of this review. Whilst that is the case, my view is that in reality Dr Scoto adopted the Panel's 'advice' more in the manner of a decision in the form of a review or appeal than advice and as a result made no further independent decision herself. In those circumstances I feel it incumbent to comment that the Panel appear to have completely overlooked the fact that:

    i) So far as this child was concerned, there was never going to be an assessment by a healthcare professional available as she was not seen during the relevant period; and

    ii) In any event, no healthcare professional would have known to carry out an assessment with an eye to the criteria (as opposed to routine monitoring) as no one knew it would become necessary, given that Nusinersen had not yet been approved.

52. That, therefore, meant that the only available evidence with which to inform the Panel, or any decision maker, in relation to this life-changing decision for Sophie was the evidence of the family and friends which, it was accepted, was not 'intentionally incorrect', and Dr Scoto's potentially favourable clinical view as set out in the letter and PowerPoint sent to the Panel.

53. On 16 September 2020, the day after she received the letter of advice from the Panel, Dr Scoto filed a witness statement confirming her decision, made in December 2019, that Sophie did not satisfy the eligibility criteria. The statement was designed also to rebut the suggestion that she allowed the Panel to make the decision and that she failed to take into account the informal family and friends' evidence.

54. Dr Scoto said in her statement that the decision she made on 12 December 2019 was her own decision made conscientiously in the light of all the available evidence. So far as the informal evidence was concerned, Dr Scoto said that she had "proactively sought out informal video evidence of Sophie walking"… but that "no video were provided to me therefore what I received, it was unfortunately not what I had expected or needed to see in order to reach a different conclusion". Dr Scoto then went on to 'adopt as my evidence' the passage in the letter from GOSH set out at paragraph [47] above which concludes by saying that absent formal evidence, video evidence can form a 'compelling alternative'.

55. Dr Scoto concludes by saying that she had taken into account all the evidence but had not changed her view. With great respect to Dr Scoto, who tried her level best for Sophie, the contents of her statement, filed the very day after the Panel had given 'advice' to the same effect, sit most uncomfortably with the email and PowerPoint she submitted to the Panel in July 2020. The email and the PowerPoint were each based on precisely the same evidence which Dr Scoto now seemingly rejected and each were written in the knowledge that there was not, and never had been, the sort of video evidence which would have been a 'compelling alternative' to clinical baseline evidence.

The Judgment

56. By a claim form dated 13 August 2020, Ms Basma, as litigation friend of Sophie, issued judicial review proceedings. By Section 3 of the standard form, the detail of the decisions to be judicially reviewed was identified as the initial decisions by Dr Hughes and Dr Scoto not to prescribe Nusinersen, and a "continuing failure to take decisions to do so and or/ failing to take into account the evidence by the Claimant of her eligibility for the drug". Copies of Dr Hughes' letter of 11 September 2019 (see [32] above) and Dr Scoto's note of Sophie's appointment of 12 December 2019 (see [37] above) are attached as copies of the decisions subject to judicial review.

57. The judge granted permission for the judicial review to be brought but dismissed the application for judicial review.

58. The first issue addressed by the judge was an issue as to the interpretation of the 5 Steps Criterion. The judge considered whether the determination of the question of Sophie's ability to walk unaided for 5 steps in the relevant period was an issue of fact or of clinical judgment.

59. The Appellant submitted that this was a straightforward matter of fact whereas the Respondent said that it was a pure clinical question involving a "significant degree of evaluative clinical judgment especially at the borderline". The judge identified the difference in the approach as between the parties as being not as to the meaning of the words themselves, but as to "the process of evaluating the available evidence to decide if the criteria are met".

60. The judge concluded at [28] that:

> "…In my judgment the need to weigh evidence from competing sources and consider all relevant materials requires the exercise of clinical judgment and is not a straightforward matter of fact-finding. Only a clinician is in the position to conduct the balancing exercise."

61. The judge emphasised that NICE had nominated a clinician as the decision maker and that that decision maker has not only his or her own experience, but "can call upon others of the Clinical Panel for advice and that the decisions will not always be straightforward and will require the clinician to consider information from various sources". The judge was fortified in his view by four further factors to the effect that it would have been unlikely that the Panel would have been set up to advise on factual matters or, if it had been a simple question of fact, why there would have been a reference to the WHO criteria. In addition, the judge noted that each of the other six criteria undoubtedly require clinical judgment and, that to be meaningful, the answers must be "objective and measurable". The clinicians as experts, were, the judge said, best placed to answer the questions. The judge at para.29.4 of his judgment regarded the observations of physiotherapists applying standard tests as being 'at the top of the evidence pyramid' and anecdotal and undocumented evidence as being necessarily 'towards the bottom'.

62. The judge moved on to consider what is the appropriate intensity of review on the facts of this case. Mr Buley accepted that the court should be slow to interfere with a truly clinical judgment by a medical professional but that, he submitted, is not this case.

63. The judge directed himself by reference to a number of cases. Having referred to para. [31] of *The Queen (on the application of Cotter) v NICE* [2020] EWHC 435 (Admin), [2020] 2WLUK 444, the judge directed himself to consider (i) the impact of the determination under challenge and (ii) the degree to which the decision involves particular expertise that the court should respect. The judge reminded himself that "even a decision requiring specialist expertise is not immune from scrutiny." (*Cotter* paras. 50 – 52)

64. The judge concluded that the decision in the present case should be subjected to a "light touch review" because it is a decision of a "specialist body exercising expert judgment". In reaching this conclusion, the judge relied on *R (Campaign to End All Animal Experiments) v Secretary of State for the Home Department* [2008] EWCA Civ 417 ("*Campaign to End all Animal Experiments*"), a case which May LJ had said in his judgment was "at the further boundary of that which is suitable for judicial review" (para.[81]). The judge in deciding that this was a case for a light touch review relied upon May LJ's observations:

> "1…..Yet the nature of the claimants' case is to challenge a composite scientific judgment based more upon an expert analysis of scientific material than upon the application of hard-edged terms of a document amenable to lawyers' construction. The Guidance is susceptible to lawyers' analysis; but it is not a tax statute nor intrinsically difficult to understand. Its

> application requires scientific judgment. The scientific judgment is not immune from lawyers' analysis. But the court must be careful not to substitute its own inexpert view of the science for a tenable expert opinion. The appellants say that the judge was wrong to find that the Chief Inspector had misconstrued the Guidance; and wrong to find in consequence or at all that his Review reached a perverse conclusion. In my view, absent material misconstruction, the court should be very slow to conclude that this expert and experienced Chief Inspector reached a perverse scientific conclusion."

65. The judge decided, therefore, that the 5 Steps Criterion was not only a matter of clinical judgment, but a clinical judgment requiring such expertise as to bring it, not within what might be thought of as the conventional judicial review approach found in *Cotter,* but within the "hands off", light touch review of the type identified in *Campaign to End all Animal Experiments* where the decision had been heavily dependent on complex scientific judgment.

66. Having set his parameters in this way, the judge went on to set out the facts leading up to the making of the relevant decisions and of the involvement of the clinical panel. So far as the decisions of Dr Hughes and Dr Scoto were concerned, he concluded that:

> "59. No doubt because the Panel's advice had not changed neither Dr Scoto not Dr Hughes felt it necessary to re-confirm that the Claimant did not meet entry criterion 5."

67. So far as Dr Hughes was concerned, the judge at para. [70] noted that she had made further enquiries of the local physiotherapist, had indicated that she would be happy to look at video footage and had, in fact, looked at the video and photographs provided to her by Ms Basma. Dr Hughes was, the judge said, conscious of the gaps in the medical records and had discharged her duty to inform herself before taking the decision.

68. So far as Dr Scoto was concerned at para. [71]- [72], the judge held that Mr Buley was unfair in interpreting Dr Scoto's reference in the letter of 11 September to a "need for formal documentation of her ability to walk" to be Dr Scoto expressing the view that only an assessment or document carried out by a medical professional would suffice. The judge gave four reasons for reaching this conclusion: (i) English is not Dr Scoto's first language; (ii) it was clear that something short of a formal assessment namely a video would suffice; (iii) Dr Scoto had made it clear to Ms Basma that something more than her oral reassurance was necessary; and (iv) Dr Scoto was proactive in obtaining additional evidence and she satisfied her duty to inform herself before taking the decision.

69. The judge said that both Dr Hughes and Dr Scoto took their initial decisions before the Panel had been asked for any input. "There is no suggestion that the treating clinicians handed the decision over to the panel of experts".

70. The judge concluded that having "conducted a light touch, but not unduly deferential review", he was satisfied that the decision reached was not irrational but was fair and there was no public law ground on which to impugn the decision.

71. The judge [para. [84]-[85] relied on the note of the physiotherapist in May 2018, that Sophie was "going off her feet fairly quickly" and recorded Dr McCullagh's note as an 'important piece of evidence' before concluding that:

> "I can well understand that the clinicians regarded the totality of the medical evidence as a very powerful indicator that the Claimant had lost the ability to walk independently by October 2018."

72. The family's evidence must, the judge said, be viewed against that background and should not be considered in a vacuum. With respect to the judge he has, in my judgment, fallen into error in this part of his analysis for two reasons:

i) Neither of the two decision makers took into account either the evidence of Ms Beech or Dr McCullagh at the date of making the decision and in reviewing their decision the judge can only take into account the same factors as were considered by the decision makers;

ii) There is nothing in the evidence to suggest that either clinician regarded the totality of the medical evidence to be a 'powerful indicator' that Sophie had lost the ability to walk by October 2018. In my judgment; Dr Hughes felt constrained by the absence of formal documentation in the first place and unable to use other than video evidence as a surrogate for a formal clinical assessment and Dr Scoto, if anything, seemed to take the view that if she were permitted to give the family and friends informal evidence full weight, she might have taken the view that Sophie was still able to walk 5 steps during the relevant period.

Discussion

Ground 1: the 5 Steps Criterion: Clinical Judgment or Question of Fact?

73. Mr Buley QC, on behalf of the Appellant, rightly emphasised to the court that this seemingly abstract issue assumed considerable importance to the way the judge approached the rest of the case as it bore directly on the issue of the intensity of review.

74. Whilst I can see the temptation to say that Mr Buley is right and that the 5 Step Criterion is entirely fact-specific, on reflection I do not agree. Whilst there is a powerful factual aspect to the decision, there are also clinical aspects. The balance between those two aspects will vary from case to case. That that is so is perfectly encapsulated in Dr Scotos's email of 20 July (see para [43] above:

> "As a clinician I would consider it possible that this child could have walked unaided some time beyond her assessment in February 2018 where it is documented she could walk 4 steps……"

75. Dr Scoto is saying that from a clinical point of view it is possible the Sophie could still walk 5 steps, but she then goes on to ask the Panel if she can then look at the informal evidence in order to complete the picture. The decision to be made in this

case was, therefore, substantially factual but with clinical elements. There may well be cases where there is formal documentation and the matter will be a question of pure fact. The element of clinical judgment will depend in every case upon the evidence.

76. In my judgment, the judge was in error in categorising the decision in this case as one of 'expert clinical judgment', an error which, I believe, he made as a consequence of his having placed undue weight on the fact that the TAC makes the clinician the decision maker. It may well be that the clinician is the best person to make such decision, but that does not mean that the decision-making process in every case is solely a clinical judgment. A very substantial part of the task, in a case like this, is simply to ascertain the facts, which requires the decision maker to take into account all relevant evidence.

Ground 2: Intensity of Review

77. The judge's view was that the task of interpreting the 5 step Criterion is wholly one of clinical judgment. It follows that his consideration of the appropriate level of review was sharply influenced by that conclusion. In simple terms, the more the decision sought to be impugned can be categorised as clinical judgment of increasing complexity, the lighter the touch of the reviewing court and the greater the respect which the reviewing court must show to the decision made.

78. Although the judge referred to the judgment in *Cotter*, he reached a conclusion not by reference to *Cotter* but by reference to *Campaign to End all Animal Experiments*, a case at the furthest boundary of that which is suitable for judicial review, a case which depended upon: "a composite scientific judgment based more upon an expert analysis of scientific material than upon the application of hard-edged terms of a document amenable to lawyer's construction".

79. This is not such a case. In my judgment, the decisions under consideration are largely factual but with a clinical element. They are not dependent on hard-edged scientific interpretation or judgment.

80. *Cotter* was a case where NICE was asked to make a recommendation as to whether an 11-year-old child should have a highly beneficial, but expensive, medication provided to her by the NHS. The issue was whether the assessment should be carried out under its standard appraisal process or its highly specialised process, it being regarded as more likely that the necessary recommendation would be made if the specialised process was adopted. Cavanagh J decided that the intensity of review appropriate for the irrationality challenge in that case fell between the two extremes put forward by counsel:

> "62. Accordingly, I think that Mr Wise QC is right that the impact of the decision under challenge was very significant for the Claimant and for others in the same position, and the intensity of review should reflect this.
>
> 63. On the other hand, I think that Mr Stilitz QC is right that the criteria in question are matters that, to some extent at least,

> require the exercise of expert judgment, and the use of expert knowledge".

81. Cavanagh J concluded that:

> "69…..The views of the decision-makers should be given proper respect, whilst also bearing in mind, as I have said, that the impact of the decision was very significant on those whose chances of obtaining Kuvan on the NHS were thereby reduced.
>
> 70. I also bear in mind that, wherever one ends up with the issue of intensity of review, the central question, to which intensity of review is relevant, is whether the decision was irrational or perverse. There is always a high threshold for irrationality cases.

82. In my judgment, the proper approach in the present case is that set out in para.[69]-[70] by Cavanagh J and the judge fell into error in equating the level of intensity of review with that in *Campaign to End all Animal Experiments*.

83. This is a case for a conventional judicial review approach with deference being given to the fact that the matter under consideration involves an element of clinical judgment. On the facts of this case, the court should be conscious when determining the appropriate intensity of review, not only that there is a substantial factual element in the decision-making process but, importantly, of the considerable impact on this little girl of the decision. Not only is it common ground that Sophie would benefit from Nusinersen now, but also it is accepted that it has the potential significantly to delay the inevitable day when she will be rendered immobile. There is no other treatment available for her.

Ground 3: Exclusion of informal evidence

84. Miss Morris argues that, as it is apparent that both Dr Hughes and Dr Scoto were conscious of the need to take into account informal evidence, this ground should be dismissed as unarguable. Whilst the heading of this ground of appeal is as above, it is clear from the body of the ground as drafted, that the appeal goes to the treatment of the informal evidence by both Dr Hughes and Dr Scoto. Males LJ granted permission on the basis that it is arguable that the decision maker should have taken account of all the evidence as to the claimant's ability to walk, but had confined consideration to evidence amounting to formal documentation of this.

85. It is common ground that the family and friends' evidence was in principle relevant because there was no clinical evidence during the relevant period. That does not mean that it should be decisive, but the decision maker was bound to consider that evidence and to decide what weight it should have.

86. So far as the decision of Dr Hughes is concerned, I am satisfied that it was made upon receipt of the 5 Step Criterion in July 2019 and was communicated in the letter to the GP on 11 September. Her approach was also set out in her letter to Ms Basma on 12

September 2019 and confirmed in her witness statement which, it will be recollected, said that when the MAA was published she (Dr Hughes) had realised that Sophie was not eligible because of the assessment that she could only walk 4 steps in February 2018.

87. I am equally clear that Dr Hughes was of the view that only video evidence of Sophie walking 5 steps unaided would serve to displace that assessment, as can be seen in her letter to the GP on 11 September and in her witness statement. I am satisfied, therefore, that Dr Hughes failed to take into account relevant evidence, namely Ms Basma's evidence as to Sophie's ability to walk in the relevant period.

88. It goes without saying that Dr Hughes is to be commended for seeking out additional information from the physiotherapist and for seeking video evidence from Sophie's mother, but on a fair reading the letter indicates that Dr Hughes did not regard Ms Basma's assertions that Sophie could walk 5 steps during the relevant period as evidence or, if she did, she did not regard it as evidence sufficient to outweigh the medical records. I am satisfied that, absent a video or further clinical evidence, Dr Hughes did not believe she could review the decision she had made based on the February 2018 assessment.

89. I do not accept, as Ms Morris submits, that this was in effect a rolling decision and that Dr Hughes kept the decision constantly under review as Ms Basma produced additional (to her mind unsatisfactory) evidence. There is no indication within the voluminous papers of Dr Hughes remaking her decision, either in the light of further evidence or against the backdrop of her reconsidering the type of nonclinical evidence which would be capable of satisfying the eligibility criteria over and above a video recording of Sophie walking.

90. Turning to Dr Scoto; as detailed in [37] to [54] above, Sophie had an extensive assessment at GOSH on 12 December 2019. Dr Scoto's record says in terms that in order for Sophie to have access to the drug there had to be 'formal documentation of her ability to walk'. Dr Scoto said that she would be able to view a video showing Sophie walking and would then put her case forward to the MAA "review panel" should she fit the inclusion criteria. Dr Scoto's record cannot, therefore, mean other than that she excluded the informal evidence then available, namely Ms Basma's assertions.

91. Before moving on, I should be clear that in my view, as submitted by Mr Buley, the record of the appointment of 12 December 2019 is undoubtedly a record of a decision. A decision which, to use judicial review terminology, was unlawful as it was based on an incorrect basis, namely that formal documentation of Sophie's ability to walk was required or, failing that, a video of her walking unaided was necessary. As a consequence, Dr Scoto failed to take into account the informal relevant evidence, namely Ms Basma's evidence as to Sophie's ability to walk in the relevant period.

92. It should be noted that the record also appears to demonstrate a misunderstanding of the MAA's exclusively advisory role evidenced by the fact that Dr Scoto referred to "the MAA panel review" (my emphasis).

93. That leaves the question as to whether that is the end of the matter so far as Dr Scoto's decision making is concerned, or whether it can be properly argued that the record of

11 September 2018 can be categorised as a preliminary decision which was kept under constant review as Dr Scoto, who was undoubtedly doing all in her power to assist Ms Bamsa in achieving access to Nusinersen for Sophie, sought out and gathered together supporting evidence.

94. In my judgment, the decision of 12 December was not an initial view but a final decision. It is undoubtedly the case that Dr Scoto pulled together the extensive highly relevant evidence from the family and friends. If having seen the family and friends evidence Dr Scoto did not feel that Sophie met the 5 Steps Criterion, it was not because she had doubts as to the cogency of the evidence brought forward by the family and friends, but rather as is revealed in her email and PowerPoint presentation presented to the Clinical Panel for their meeting on 7 August 2020, it was because she was unclear as to whether she was permitted to use that evidence in order to decide Sophie's eligibility.

95. In the event, the Panel 'advice' set out at [49] above, said that what it needed was 'objective and clinical evidence' and that it regarded family recollection as potentially unreliable, even if not intentionally incorrect.

96. Ms Morris submitted that there was no necessity for Dr Scoto to remake her decision in formal terms as she had kept it under review. The advice from the Panel, she submitted, when factored into the February 2018 assessment served only to confirm her initial decision.

97. I do not agree with Ms Morris' analysis. In my judgment, Dr Scoto treated the role of the Panel not just as advisory, but as determinative. It is hard to see how else to understand Dr Scoto's complete change in approach to the informal evidence of the family and friends.

98. Dr Scoto's approach to the informal evidence was set out in the material she sent to the Panel for its meeting on 7 August. She had said: "As a clinician I would consider it possible that this child could have walked unaided sometime beyond her assessment in February 2018… as you will see the family provided statements and pictures (unfortunately no video for the period) supporting the possibility she was still independently ambulant" and in the PowerPoint she said: "I am sure that if the family knew one step would make such a huge difference, they would have filmed the child walking…"

99. Immediately after the Panel issued its advice Dr Scoto filed her witness statement now adopting the letter from GOSH of 29 July 2020 to the effect that a clinical baseline from a physiotherapist, a clinical professional or someone with a prior understanding of the technical nature of the observations was required, although a video could provide compelling evidence. Dr Scoto had said in the witness statement that the video she had been sent (of Sophie cruising) was "not what I had expected or needed to see in order to reach a different conclusion". No mention was made of the family and friend's evidence or of the fact that Ms Basma would undoubtedly have filmed Sophie walking had she known it would be vital for her future treatment. In my judgment, it can only have been the Panel's 'advice' that led Dr Scoto to approach the family's evidence in this way, a way which seems to be contrary to the independent clinical view she had expressed only weeks earlier.

100. Whilst I repeat my admiration for the tremendous efforts that Dr Scoto went to in order to try and get Sophie onto the Nusinersen programme, in my judgment her decision of 12 September was unlawful for the reasons given above and far from remaking her decision in the light of the family and friends evidence, Dr Scoto effectively delegated that to the Panel and adopted their view, notwithstanding that it could not stand up against the earlier opinion she had expressed as a treating consultant.

Ground 4: Irrationality

101. As the judge rightly said, and is accepted by Mr Buley, "an irrationality challenge requires substantial obstacles to be overcome", or as it is often said, it is a 'high hurdle'. The judge in deciding the irrationality challenge took into account his view that the decision whether Sophie satisfied the 5 Steps Criterion was 'of a clinical character reached by expert and experienced clinicians' and also that his review of the decision should be light touch although not made with 'reticence or timidity'. As I have already said, in my judgment the judge was in error in adopting a 'light touch' of the type used in *Campaign to End all Animal Experiments* and in reaching that conclusion he failed adequately to take into account the extremely serious consequences for Sophie if she is unable to receive this treatment on the one hand and, on the other, overstated the complexity of the clinical aspect of the decision making process.

102. I have considered with care whether the original decisions made by Dr Hughes and Dr Scoto respectively on 11 September 2019 and 11 December 2019 were unlawful, due to their failure to consider the relevant informal evidence, but also whether these decisions can be termed irrational, that is to say a decision which is so unreasonable that a reasonable decision maker, properly directing herself in law, could not have made it. I am, as always, conscious of the high hurdle which must be overcome before a court can review such a decision. In my judgment however each of the decisions can be so termed for the following reasons:

    i) The decision to be made, as already set out, had a very substantial factual element to it. Whilst Ms Morris spoke of the technical aspect to the decision, in my view the test is not complicated and any lay person if asked appropriate questions would have been able to describe whether a disabled child they saw was walking was unaided and was or was not in an upright position with the back straight and with one leg moving forward while the other supports most of the body weight;

    ii) There was no clinical evidence to assist in the decision making process during the relevant period;

    iii) The MAA makes it clear that children can have 'off days' and for that reason two points of reference are required for various matters, a view wholly endorsed, in relation to the 5 Step Criterion, by Dr Scoto in her email. It follows that the February 2018 assessment, when Sophie walked 4 (and not the critical 5 steps) at a time when no one thought it was a matter of importance, must be regarded with considerable caution;

iv) Dr McCullagh saw Sophie without the benefit of a physiotherapy assessment and had no recollection of the appointment;

v) No video evidence was available covering the relevant period;

vi) Even objectively, it is hard to overstate the impact on Sophie of the decision that was being made;

vii) It was not suggested that either Ms Basma or the family and friends were other than credible witnesses.

103. Against this back drop, I have concluded that the failure of both Dr Hughes and Dr Scoto to take into account the evidence of Sophie's mother in respect of Sophie's ability to walk from time to time during the period of time when there was no clinical evidence available, was irrational.

104. After the decision was made, Dr Scoto received substantially more evidence. Evidence which, when she put it together with her clinical judgment, suggested at the very least that if she were able to consider the family and friends' evidence, she would reconsider her decision. Dr Scoto was not only entitled to take that evidence into account, but as the Respondent's position has been throughout not to question the bona fides of those witnesses, she was obliged to take that evidence into consideration. Dr Scoto did not do so but relied upon the Panel's advice.

105. In my judgment, the failure of Dr Scoto to reconsider her decision in the light of the new evidence is irrational.

106. I emphasise again, that a finding of irrationality in a judicial review context, should not be taken to connote any personal criticism of either Dr Hughes or Dr Scoto who were each conscientious and well-intentioned.

Conclusion

107. It follows, therefore, that if My Lord and My Lady agree, I would allow the appeal on each of the four grounds of appeal and would quash the decisions of 11 September 2019 and 12 December 2019. I further order that the Respondents reconsider their decisions in the light of the judgment of this court together with any evidence Ms Basma has submitted to date and of any further evidence she might choose to submit.

Lord Justice Baker

108. I agree.

Lady Justice Elisabeth Laing

109. I also agree.