Solving Kids' Cancer Submissions on Human Rights

and

the Rights of the Child (Ground 1b)

Introduction

1. This note sets out the submissions of Solving Kids' Cancer on why NICE has exceeded its powers by the Appraisal Committee not recommending dinutuximab in breach of several human rights under the European Convention of Human Rights (Convention), as transposed into national law under the Human Rights Act 1998 (HRA). Additionally, this note provides our submissions on why the Appraisal Committee's decision breaches the rights of the child under the UN Convention on the Rights of the Child (UN Convention).

2. It is important to be clear at the outset that Solving Kids' Cancer recognises and accepts that NICE has difficult decisions to make about the allocation of resources. However, decisions about whether to recommend a technology must be taken rationally, in accordance with the obligations imposed on public bodies by the HRA, the UN Convention, the Equality Act 2010 and the Children Act 2004. The human rights context of this appeal, therefore, also impacts upon Solving Kids' Cancer submitted appeal ground that NICE's decision not to recommend dinutuximab was perverse.

3. NICE, as a public body, is bound in its appraisals to take account of human rights legislation. There are numerous references to this obligation in NICE guidance: see, for example, NICE Guide to Methods of Technology Appraisals at §§1.4.3, 3.1.4, 6.1.3 and 6.1.8; and NICE Social Value Judgments principles at §§3.1 and § 9. Further, NICE as a body corporate may only exercise its functions on the direction of the Secretary of State for Health and/or NHS England and subject to those directions and therefore is bound to take into account the State's obligation to protect rights of the child in discharging its functions.

4. Solving Kids' Cancer is mindful that the Appeal Panel has previously considered and ruled on the human rights aspects of NICE appraisals, specifically in relation to the appraisal of azacitidine for the treatment of myelodysplastic syndromes, chronic myelomonocytic leukaemia and acute myeloid leukaemia (the Azacitidine Decision) and the appraisal of dasatinib for the treatment of imatinib-resistant chronic myeloid leukaemia (the Dasatinib Decision). However, neither the Panel nor the domestic courts, as far as Solving Kids' Cancer is aware, have considered the human rights of the child in the context of NICE appraisals.

5. In this appeal, Solving Kids' Cancer submits that the decision of the Appraisal Committee not to recommend dinutuximab contravenes four Articles of the Convention and two Articles of the UN Convention for the following reasons:

    5.1. the recommendation deprives children with high-risk neuroblastoma to extra life (Article 2 of the Convention) and to the highest attainable standard of health (Article

24 of the UN Convention), thereby the recommendation is not in the best interests of the child (Article 3 of the UN Convention);

5.2. refusing dinutuximab to children with high-risk neuroblastoma amounts to inhumane and degrading treatment (Article 3 of the Convention);

5.3. refusing dinutuximab to children with high-risk neuroblastoma denies such children the right of a family and privacy (Article 8 of the Convention); and

5.4. the recommendation is discriminatory in that it disproportionately affects children and therefore is indirectly discriminatory in respect of age (Article 14 of the Convention).

6. Solving Kids' Cancer will address in turn below why each of the above Convention Articles is infringed by the decision not to recommend dinutuximab and the interplay of those Articles with the rights of the child under the UN Convention.

Article 2 of the Convention

7. The Appraisal Committee's decision not to recommend dinutuximab engages the right to life. Essentially, the decision deprives children with high-risk neuroblastoma of a US Food and Drug Administration (FDA) and European Medicines Agency (EMA) approved drug that is recognised to both increase median event free survival and overall survival of patients. In other words, dinutuximab represents a life-saving treatment for children and the Appraisal Committee accepted that for those patients that it does not represent a cure, dinutuximab extends the life of the child on average up to 2.81 life years (approximately 33.7 months) compared with isotretinoin alone (Final Appraisal Determination at §4.22).

8. Article 2 obliges the State to refrain from depriving persons of life intentionally, but also imposes a positive obligation to fund life-saving treatments. For example, in *Scialacqua v Italy* DR 81, 35, the European Commission of Human Rights assumed that Article 2 imposes on States *"... the obligation to cover the costs of certain medical treatments or medicines that are essential in order to save lives."*

9. The domestic courts also have recognised that Article 2 obliges the State to provide life-saving healthcare treatment and that such treatment is, save in exceptional circumstances, in the best interests of the patient. For example, *NHS Trust A v M* [2001] Fam. 348 at §24 affirms this principle:

> *"... I bear in mind the positive (though not absolute) obligation imposed by Article 2 of the European Convention on Human Rights to give life-sustaining treatment where responsible medical opinion is of the view that such treatment is in the patient's best interests and the observation of Munby J approved in R (Burke) v General Medical Council [2005] EWCA 1003 at paragraph 61;*
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> > *"**There is a very strong presumption in favour of taking all steps to prolong life, and save in exceptional circumstances**, or where the*

patient is dying, ***the best interests of the patient will normally require such steps to be taken.*** *In case of doubt, that doubt falls to be resolved in favour of the preservation of life."* (Emphasis added)

Accordingly, the court was recognising that Article 2 is engaged by a decision whether or not to approve life-saving and prolonging treatment.

10. In the Azacitidine Decision, the Appeal Panel accepted that Article 2 imposes certain positive obligations on the State but the Panel was, with respect, wrong to conclude that Article 2 did not extend an obligation to recommend a treatment within a national health service (§52) and to dismiss Celgene's point that access to treatment should be provided in exceptional circumstances taking into account the limits to public funding (§54).

11. Solving Kids' Cancer also recognises that public resources are finite, and that Article 2 does not impose an obligation on the State to provide unlimited resources for medical treatment. Rather, Article 2 is engaged only in exceptional life-saving situations, as in this case.

12. The present case is exceptional in that dinutuximab is for the paediatric population and can cure patients with the life-threatening condition high-risk neuroblastoma, or at a minimum, significantly prolongs their lives. In the context of the paediatric population, the extension of life provided by dinutuximab of nearly 3 years is even more acute given the median life expectancy for children with high-risk neuroblastoma is 4 years. In regard the latter, the EMA's Committee for Medicinal Products for Human Use recommended the authorisation of dinutuximab given the *"significant and sustained improvement in overall survival [with dinutuximab] in this paediatric patient population."*[1] Dinutuximab is the only drug approved by both the EMA and FDA for the treatment of high-risk neuroblastoma in children. Given the recognised clinical effectiveness of dinutuximab, it is clearly in the best interests of paediatric patients to receive treatment. The present case can be distinguished from the Azacitidine and Dasatinib Decisions, which concerned treatments for adult patients with terminal cancer that shortly prolonged rather than saved patients' lives. It is therefore unjustified and hence contrary to Article 2 for the Appraisal Committee to refuse to recommend dinutuximab.

UN Convention - Articles 3 and 24

13. Further or alternatively, the failure of NICE to take appropriate steps to adapt the appraisal system for the special position of children is contrary to the rights of the child and to the procedural obligation contained in Article 2 of the Convention.

14. The UN Convention obliges the State to give particular attention to the needs of children when formulating and implementing its strategies, policies, programmes, projects and activities that bear upon access to medicines. Section 11 of the Children Act 2004, reinforces the general principle that a public health body should take into consideration

[1] European Public Assessment Report for dinutuximab, p. 96. Available at: http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Public_assessment_report/human/002800/WC500192794.pdf

the rights of the child when discharging its functions. In the recent case of *S (a child) v NHS England* [2016] EWHC 1395 (Admin) at §38, the domestic courts acknowledged the fundamental importance of taking into consideration the needs of children, and the relevance of Section 11 of the Children Act 2004, in the context of a public body's decision to provide access to medicines.

15. Article 24(1) of the UN Convention codifies a child's fundamental right to enjoyment of the highest attainable standard of health and states that:

> *"**States Parties recognize the right of the child to the enjoyment of the highest attainable standard of health and to facilities for the treatment of illness** and rehabilitation of health. States Parties shall strive to **ensure that no child is deprived of his or her right of access** to such health care services."* (Emphasis added)

Further, Article 3(1) of the UN Convention provides that:

> *"**In all actions concerning children**, whether **undertaken by public** or private social welfare **institutions**, courts of law, administrative authorities or legislative bodies, **the best interests of the child shall be a primary consideration**."* (Emphasis added)

16. NICE as a body corporate may only exercise its functions relating to the provision of NHS services, public health services, or social care in England on the direction of the Secretary of State for Health and/or NHS England and subject to those directions (see, for example, Section 237 of the Health and Social Care Act 2012) and therefore is bound to take into account the State's obligation to protect the rights of the child in discharging its functions.

17. There can be no doubt, therefore, that NICE appraisals engage the rights of the child under Article 3 and 24 of the UN Convention. However, the Appraisal Committee either had no regard, or failed to have proper regard to the needs of children and NICE's heightened scrutiny obligation when appraising dinutuximab for the paediatric population. According to *R (Burke) v General Medical Council,* it is, save in exceptional circumstances, in the best interests of the patient to provide life-saving healthcare treatment. Furthermore, Article 3(1) of the UN Convention requires NICE to have at the forefront of its mind the best interests of the child when appraising medicines for the paediatric population.

18. However, the Appraisal Committee failed to consider the special needs of children and applied NICE's standard end-of-life and clinical effectiveness criteria to dinutuximab. NICE's 2-year life-expectancy threshold for its end-of-life criteria is arbitrary, unfair and biased against children, as this vulnerable population typically live longer than adults with cancer. Further, the Committee's appraisal of dinutuximab through the standard appraisal technology process, and thereby applying NICE's standard clinical effectiveness criteria, was always likely to produce a negative recommendation for the orphan drug and amounts to a breach of a child's right of access to the highest attainable standard of health and facilities for the treatment of illness.

19. In the case of a life-saving paediatric orphan drug such as dinutuximab, the Appraisal Committee's failure to apply any special approach taking into account the unique needs of children is contrary to Article 2 of the Convention for the reasons set out above coupled with a breach of Article 3 and 24 of the UN Convention to treat children's best interests as a primary consideration in access to medicine decisions.

Article 3 of the Convention

20. Article 3 of the Convention prohibits inhuman or degrading treatment. Solving Kids' Cancer recognises that Article 3 does not routinely apply to a failure to provide healthcare, since such failure will not usually meet the minimum threshold required to constitute inhuman or degrading treatment.

21. Solving Kids' Cancer, however, submits that the present case is exceptional given NICE's refusal to recommend dinutuximab deprives children with high-risk neuroblastoma of a potential cure or alternatively, a significantly prolonged and improved quality of life of nearly 3 years and amounts to inhuman treatment in breach of Article 3.

22. As a result of the Appraisal Committee's non-recommendation of dinutuximab, patients in England will not have access to an EMA and FDA approved treatment for their condition. In order to increase their poor chances of survival, patients will have to either fund the cost of dinutuximab or alternatively, enrol in a clinical trial to gain access to a similar or other treatment type for their condition. Both these options amount to inhuman and degrading treatment. In relation to the latter, parents acting in their child's best interest will be forced to make an impossible choice to either enrol their child in a trial exposing them to the unknown risks of an experimental treatment whilst an approved drug exists or accept their child's poor prognosis. Further, dinutuximab is standard of care in Europe and the US and there is evidence of families making public appeals for money and travelling abroad to access this drug at an enormous financial and emotional cost.

23. The Appraisal Committee failed to apply NICE's end-of-life and standard clinical effectiveness criteria in a way that is appropriate for children taking into account their best interests, as set out above. Inevitably this lead to a negative recommendation for dinutuximab and therefore, NICE failed to reduce the suffering of children to the fullest extent possible contrary to Article 3.

Article 8 of the Convention

24. Even if, contrary to Solving Kids' Cancer submissions set out above, the Panel does not accept that the consequences of the Appraisal Committee's decision are sufficiently grave to constitute a violation of Article 2 and/or 3 of the Convention, it constitutes an interference with the right to respect for private and family life of paediatric patients with high-risk neuroblastoma contrary to Article 8.

25. Article 8(1) requires that *"everyone has the right to respect for his private and family life."* The right is qualified and Article 8(2) provides that "*there shall be no interference by a public authority with the exercise of this right except such as in accordance with the law and is necessary in a democratic society…"*

26. The European Court of Human Rights has interpreted Article 8 broadly and assumed that the right is engaged with regard to medical treatment: see, for example, *Pentiacova v Moldova* (2005) 40 EHRR SE 23, *Tysiac v Poland* (2007) 45 EHRR 42 and *Sentges v Netherlands* (2003). Further, Article 8 protects not only private and family life, but also the wider concept of physical and moral integrity, which are a vital precondition to the effective enjoyment of private life: see, for example, *Bensaid v UK* 2001-I, §46-47.

27. Dinutuximab cures or at least significantly extends the life of children with high-risk neuroblastoma by up to 3 years and therefore patients treated with dinutuximab enjoy a longer family life. Accordingly, having regard to the substantial duration of additional life gained, Solving Kids' Cancer submits that denying paediatric patients access to dinutuximab constitutes an interference with their private and family life contrary to Article 8(1) of the Convention.

28. In the Azacitidine and Dasatinib Decisions the Panel was, with respect, wrong to hold that although access to treatment is within the ambit of Article 8(1) that the right was not engaged on the basis that the high threshold was not met (§55 and §102, respectively). Solving Kids' Cancer agrees with the Panel's legal advisors in those appeals that Article 8 is engaged where a drug provides substantial additional life.

29. Solving Kids' Cancer recognises that an interference with private and family life may be justified in accordance with the requirements of Article 8(2) and accepts that NICE is pursuing a legitimate objective in appraising dinutuximab in order to fairly allocate resources to maximise the health of the population in general. However, for an interference to be justified under Article 8(2), it must be "necessary in a democratic society".

30. The key issue, therefore, is whether the Appraisal Committee's non-recommendation is "necessary". This is a question of fact that the Appeal Panel must determine having regard to the special characteristics of this case, in particular the need to act in the best interests of the child, whether denying paediatric patients access to dinutuximab is justified under Article 8(2).

31. In the present case, the Appraisal Committee's decision deprives children of the right of access to dinutuximab, a life-saving treatment or at a minimum a significant life-prolonging treatment. The Appraisal Committee accepted that dinutuximab extends the life of patients by nearly 3 years. In the context of high-risk neuroblastoma patients, who on average live for 4 years, this represents nearly double their life expectancy. However, the Appraisal Committee failed to consider the clinical and cost effectiveness of dinutuximab in light of the special needs of the paediatric population contrary to NICE's obligation under the UN Convention, as set out above. No proper balancing appears to have been carried out between the interests and impact of the decision on the paediatric population as against the importance of NICE's budgetary aim. No real consideration was given to the impact of the Appraisal Committee's non-recommendation of dinutuximab on children. Accordingly, the Appraisal Committee's decision not to recommend dinutuximab is disproportionate and in breach of Article 8 of the Convention.

Article 14 of the Convention

32. Article 14 prohibits discrimination in the enjoyment of other Convention rights. Accordingly, for Article 14 to be engaged, one of the other Convention rights must be applicable (although not necessarily infringed). For the reasons set out above, each of Articles 2, 3 and 8 are engaged, such that Article 14 applies. In the Azacitidine and Dasatinib Decisions, the Panel accepted that since access to treatment is within the ambit of Article 8, Article 14 was engaged (§57 and §104, respectively).

33. Where a public body, such as NICE, is providing a public service, it is bound by Article 14 to ensure that it does so in a non-discriminatory fashion: see, for example, *Belgian Linguistic* Case (No 2) (1968) I EHRR 252. By refusing to recommend dinutuximab, the Appraisal Committee has taken a decision that will disproportionately affect children. The decision therefore constitutes indirect discrimination against paediatric patients. The Appraisal Committee's decision therefore is indirectly discriminatory in respect of age. Article 14 prohibits indirect as well as direct discrimination, that is an apparently age-neutral provision (such as the decision not to recommend dinutuximab), which as a matter of fact has a disproportionate and unjustified impact on members of a particular group (children). The disproportionate discriminatory effect of the recommendation is compounded by the fact that NICE failed to consider the rights of the child during the appraisal process, as set out above.

34. The Appraisal Committee's decision, therefore, does not discriminate in a manner that is "objectively and reasonably justified" within the meaning of Article 14 and is unlawful for that reason.

35. Furthermore, although NICE is entitled to identify clinical priorities and allocate its limited funds accordingly, where its decisions give rise to a discriminatory effect, NICE may not justify that discrimination on budgetary grounds alone: see, for example, Baroness Hale in S*eldon v Clarkson Wright & Jakes* [20 12] UKSC 16; [20 12] 2 C.M.L.R. 50 at §46. Accordingly, the decision is not a proportionate means of achieving a legitimate aim and therefore constitutes unlawful discrimination under Section 29(6) of the Equality Act 2010.

36. Section 149 of the Equality Act 2010 also obliges NICE to have due regard to the need to eliminate discrimination. In the present case, the Appraisal Committee should have had specific regard when appraising dinutuximab to the need to eliminate discrimination because of age and the vulnerability of the group (children) affected by its decision. For example, in *R (on the application of Rahman) v Birmingham City Council [2011] EWHC 944* (Admin), the domestic courts noted that limited financial resources did not excuse compliance with the public sector equality duty and indeed there was much to be said for the proposition that in straitened times the need for clear, well-informed decision-making when assessing the impacts on less-advantaged members of society was as great, if not greater. However, NICE failed to have due regard to the rights of the child and the impact of the Appraisal Committee's decision on the vulnerable paediatric population during the appraisal process, as set out above. The Appraisal Committee's decision, therefore, amounts to a breach of NICE's public sector equality duty under Section 149 of the Equality Act 2010 and is unlawful.