NICE National Institute for Health and Care Excellence

Professional Expert Questionnaire

Technology/Procedure name & indication: **IP1994 Phrenic nerve pacing**

Your information

Name:	Ben Messer
Job title:	consultant in critical care medicine and long-term ventilation
Organisation:	Newcastle upon Tyne Hospitals NHS Foundation trust
Email address:	
Professional organisation or society membership/affiliation:	individual response
Nominated/ratified by (if applicable):	not applicable
Registration number (e.g. GMC, NMC, HCPC)	4742971

How NICE will use this information:

The information that you provide on this form will be used to develop guidance on this procedure.

☐ Please tick this box if you would like to receive information about other NICE topics.

Your advice and views represent your individual opinion and not that of your employer, professional society or a consensus view. Your name, job title, organisation and your responses, along with your declared interests will also be published online on the NICE website as part of public consultation on the draft guidance, except in circumstances but not limited to, where comments are considered voluminous, or publication would be unlawful or inappropriate.

For more information about how we process your data please see our privacy notice.

☐ I give my consent for the information in this questionnaire to be used and may be published on the NICE website as outlined above. If consent is NOT given, please state reasons below:

Click here to enter text.

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

1	Please describe your level of experience with the procedure/technology, for example: Are you familiar with the procedure/technology? Have you used it or are you currently using it? – Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake? – Is this procedure/technology performed/used by clinicians in specialities other than your own? – If your specialty is involved in patient selection or referral to another specialty for this	We have 1 patient under our long term ventilation service who has 1 of these systems in place. Not commonly used in the UK, however, patients are often very interested in this technology and its potential to reduce ventilator dependence. No. As above.

	procedure/technology, please indicate your experience with it.	
2	− Please indicate your research experience relating to this procedure (please choose one or more if relevant):	I have had no involvement in research on this procedure.
3	Does the title adequately reflect the procedure? How innovative is this procedure/technology, compared to the current standard of care? Is it a minor variation or a novel approach/concept/design? Which of the following best describes the procedure (please choose one):	no as there is no diagnostic group mentioned on this form but in the email CCHS is mentioned. This has been around as a concept for some years but is not widely used and would therefore be a significant innovation. Definitely novel and of uncertain safety and efficacy.
4	Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?	My very small experience would suggest that in patients with very high spinal cord injury levels, existing gold standard treatment with ventilation (either invasive or non-invasive) would still be required but ventilator dependency may be reduced.
5	Have there been any substantial modifications to the procedure technique or, if applicable, to devices involved in the procedure?	No

	Has the evidence base on the efficacy and safety of this procedure changed substantially since publication of the guidance?	No

Current management

6	Please describe the current standard of care that is used in the NHS.	ventilation which can be either invasive or non invasive.
7	Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this? If so, how do these differ from the procedure/technology described in the briefing?	direct diaphragm pacing placed laparoscopically which has been reviewed by NICE within the last 12 months. One stimulates the phrenic nerve, the other directly stimulates the diaphragm.

Potential patient benefits and impact on the health system

8	What do you consider to be the potential benefits to patients from using this procedure/technology?	a reduction in ventilator dependency and consequent improvement in independence and quality of life. It certainly enables the patient we look after to have periods of ventilator free breathing and improves his independence/ability to get out and about.
9	Are there any groups of patients who would particularly benefit from using this procedure/technology?	high spinal cord injured patients.
10	Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system? Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?	yes, it could reduce ventilator dependency. This may also reduce the complications of ventilation as well as improve quality of life. There is a significant burden of hospital admissions in this group which can be due to respiratory causes. In theory, this technology could help to reduce this.
11	What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?	commissioning arrangements would be important. Training of the surgical team would also be important.
12	Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?	yes, the surgical teams would need to undergo significant training on placement of this system.

Safety and efficacy of the procedure/technology

13	What are the potential harms of the procedure/technology? Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence:	the main harm would be related to the initial operation to do this. In general, if this were done early after a spinal cord injury (potentially when the patient still had a tracheostomy), these risks would be low.

	Adverse events reported in the literature (if possible, please cite literature) Anecdotal adverse events (known from experience) Theoretical adverse events	Unknown
14	Please list the key efficacy outcomes for this procedure/technology?	hours of ventilator dependence before and after the procedure. Hospital admissions. Quality of life metrics.
15	Please list any uncertainties or concerns about the efficacy and safety of this procedure/?	it has not currently been well investigated in large randomised controlled trials.
16	Is there controversy, or important uncertainty, about any aspect of the procedure/technology?	as above (section 15).
17	If it is safe and efficacious, in your opinion, will this procedure be carried out in (please choose one):	Fewer than 10 specialist centres in the UK. Cannot predict at present.

Abstracts and ongoing studies

18	Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work). Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a	None

	comprehensive reference list but it will help us if you list any that you think are particularly important.	
19	Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.	unknown
20	Please list any other data (published and/or unpublished) that you would like to share.	not applicable

Other considerations

21	Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?	Small numbers, probably 5-10 patients in our region.
22	Please suggest potential audit criteria for this procedure/technology. If known, please describe: − Beneficial outcome measures. These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured. − Adverse outcome measures. These should include early and late complications. Please state the post procedure timescales over which these should be measured:	Beneficial outcome measures: Quality of life, hours of ventilation in a 24 hour period, rate of decannulation of tracheostomy, hospital admissions, mortality. Adverse outcome measures: Perioperative adverse outcomes such as respiratory deterioration.

Further comments

23	If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe.	There have been no randomised controlled trials of this technology.

NICE National Institute for Health and Care Excellence

Declarations of interests

Please state any potential conflicts of interest relevant to the procedure/technology (or competitor technologies) on which you are providing advice, or any involvements in disputes or complaints, in the previous **12 months** or likely to exist in the future. Please use the NICE policy on declaring and managing interests as a guide when declaring any interests. Further advice can be obtained from the NICE team.

Type of interest *	Description of interest	Relevant dates	
		Interest arose	Interest ceased
Choose an item.	no declaration of interest.		
Choose an item.			
Choose an item.			

☐ I confirm that the information provided above is complete and correct. I acknowledge that any changes in these declarations during the course of my work with NICE, must be notified to NICE as soon as practicable and no later than 28 days after the interest arises. I am aware that if I do not make full, accurate and timely declarations then my advice may be excluded from being considered by the NICE committee.

Please note, all declarations of interest will be made publicly available on the NICE website.

Print name:	**Ben Messer**
Dated:	**03/04/2023**

NICE National Institute for Health and Care Excellence

Professional Expert Questionnaire

Technology/Procedure name & indication: **IP1994 Phrenic nerve pacing**

Your information

Name:	Dr Anita Saigal
Job title:	Respiratory Registrar
Organisation:	Royal Free Hospital
Email address:	
Professional organisation or society membership/affiliation:	General Medical Council/ British Thoracic Society Specialist Advisory Group
Nominated/ratified by (if applicable):	British Thoracic Society SAG
Registration number (e.g. GMC, NMC, HCPC)	GMC number: 7042640

How NICE will use this information:

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☐ Please tick this box if you would like to receive information about other NICE topics.

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For more information about how we process your data please see our privacy notice.

X I give my consent for the information in this questionnaire to be used and may be published on the NICE website as outlined above. If consent is NOT given, please state reasons below:

Click here to enter text.

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

1	Please describe your level of experience with the procedure/technology, for example: Are you familiar with the procedure/technology?	Yes I am familiar with the procedure and technology but have not seen it used in clinical practice.
	Have you used it or are you currently using it? − Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake? − Is this procedure/technology performed/used by clinicians in specialities other than your own? − If your specialty is involved in patient selection or referral to another specialty for this	We do not use it within our trust currently (I do not work in a specialist neurological/spincal centre). I am not aware of how widely it is used within the NHS. I believe this is used by clinicians specialising in neuromuscular disorders/ congential disorders and working in neurology/spinal units (in combination with the appropriate surgical teams).

	procedure/technology, please indicate your experience with it.	
2	– Please indicate your research experience relating to this procedure (please choose one or more if relevant):	I have had no involvement in research on this procedure. Other (please comment)
3	Does the title adequately reflect the procedure? How innovative is this procedure/technology, compared to the current standard of care? Is it a minor variation or a novel approach/concept/design? Which of the following best describes the procedure (please choose one):	Yes Novel approach (i.e not the standard of care for paediatrics) Definitely novel and of uncertain safety and efficacy in this target population .
4	Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?	Not to replace standard of care, but to allow ventilator free hours in group of patients dependent on this.
5	Have there been any substantial modifications to the procedure technique or, if applicable, to devices involved in the procedure? Has the evidence base on the efficacy and safety of this procedure changed	https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7503443/ - guidelines for CCHS from 2020 While unilateral pacing has been effective in adults, the paediatric population usually requires bilateral pacing to ensure adequate ventilation.

	substantially since publication of the guidance?	Two phrenic nerve pacing systems are currently commercially available: a monopolar electrode system from Avery® Biomedical Devices Inc. (New York, USA and a quadripolar electrode system from Atrotech® Ltd. (Tampere, Finland). I am not aware of any further evidence beyond this guidance.

Current management

6	Please describe the current standard of care that is used in the NHS.	Could not find NATIONAL UK guidance, only European guidance from 2020 and ATS guidance from 2010
7	Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this? If so, how do these differ from the procedure/technology described in the briefing?	No, not to the two devices above from 2020 guidance

Potential patient benefits and impact on the health system

8	What do you consider to be the potential benefits to patients from using this procedure/technology?	Ventilator free hours
9	Are there any groups of patients who would particularly benefit from using this procedure/technology?	CCHS as identified by this enquiry
10	Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system? Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?	Yes, ventilator free hours could be associated with reduced risk of ventilator associated pneumonias for example and therefore less invasive treatment and fewer hospital visits • Phrenic nerve pacing offers freedom from the ventilator during daytime in patients ventilated 24 h a day, thus increasing mobility and allowing sporting and professional activities
11	What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?	Adequate training/ MDT for decision making and monitoring of clinical service pathway in specialist neurosurgical/sleep and ventilation/paediatrics centres
12	Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?	Yes, extremely specialist - A surgical procedure is required for implanting electrodes and receivers. While some prefer a cervical approach, a majority of centres adopts intrathoracic implantation of the phrenic nerve electrodes

Safety and efficacy of the procedure/technology

13	What are the potential harms of the procedure/technology?	Infection, risk of failure, or repeated surgical intervention Risk of general anaesthetic Post operative risks (ICU stay etc)

	Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence: Adverse events reported in the literature (if possible, please cite literature) Anecdotal adverse events (known from experience) Theoretical adverse events	
)14	Please list the key efficacy outcomes for this procedure/technology?	Post operatively (short term) Mean hospital stay (days) Mean ICU stay (days) Longer term Symptoms Daytime sleepiness Functional capacity – with activities of daily living Exercise capacity Health-related Quality of life Ventilator free days Number of respiratory infections
15	Please list any uncertainties or concerns about the efficacy and safety of this procedure/?	Bilateral phrenic nerve pacing is suggested Unclear efficacy of unilateral
16	Is there controversy, or important uncertainty, about any aspect of the procedure/technology?	Optimal time to commence pacing post operatively i.e achievement of daily goal pacing times Optimal time to gain full hours of pacing post operatively (i.e. optimal incline in time daily) Number of times had to use back up battery

17	If it is safe and efficacious, in your opinion, will this procedure be carried out in (please choose one):	Fewer than 10 specialist centres in the UK/Cannot predict at present.

Abstracts and ongoing studies

18	Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work). Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a comprehensive reference list but it will help us if you list any that you think are particularly important.	https://pubmed.ncbi.nlm.nih.gov/25924848/ https://www.ijcasereportsandimages.com/archive/2017/002-2017-ijcri/CR-10755-02-2017-sardenberg/ijcri-1075502201755-sardenberg-full-text.php https://www.sciencedirect.com/science/article/abs/pii/S0022346814006526
19	Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.	Not aware and cannot find from search of clinical trials.gov
20	Please list any other data (published and/or unpublished) that you would like to share.	

Other considerations

21	Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?	Unknown

22	Please suggest potential audit criteria for this procedure/technology. If known, please describe: – Beneficial outcome measures. These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured. – Adverse outcome measures. These should include early and late complications. Please state the post procedure timescales over which these should be measured:	Beneficial outcome measures: Post operatively (short term) Mean hospital stay (days) Mean ICU stay (days) Longer term Symptoms Daytime sleepiness Functional capacity – with activities of daily living Exercise capacity Health-related Quality of life Number of respiratory infections Ventilator free hours (in those ventilator dependent for 12-24 hours) Adverse outcome measures: Early Post surgical implant infection Need for surgical removal/replacement of the implants Rate of pacer malfunction Late Rate of pacer malfunction

		Follow up of any studies should be a minimum of 2 years

Further comments

23	If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe.	

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Declarations of interests

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Type of interest *	Description of interest	Relevant dates	
		Interest arose	Interest ceased
Choose an item.			
Choose an item.			
Choose an item.			

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Please note, all declarations of interest will be made publicly available on the NICE website.

Print name:	**ANITA SAIGAL**
Dated:	**14/04/2023**

View results

Respondent

50 Anonymous

36:51
Time to complete

1. Project Number and Name - (Can be found on email) *

IP1994 Phrenic nerve pacing for congenital central hypoventilation syndrome

Your information

2. Name: *

Hui-leng Tan

3. Job title: *

Consultant in Paediatric Respiratory and Sleep Medicine

4. Organisation: *

Royal Brompton hospital

5. Email address: *

6. Professional organisation or society membership/affiliation: *

British Paediatric Respiratory Society

7. Nominated/ratified by (if applicable):

BPRS - Martin Samuels and Sonal Kalsa

8. Registration number (e.g. GMC, NMC, HCPC) *

GMC 4673578

How NICE will use this information:

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For more information about how we process your data please see our privacy notice: https://www.nice.org.uk/privacy-notice

9. I give my consent for the information in this questionnaire to be used and may be published on the NICE website as outlined above. *

- (●) I agree
- ( ) I disagree

The procedure/technology

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

10. Please describe your level of experience with the procedure/technology, for example:

    Are you familiar with the procedure/technology?

    Saw some patients when I did my sleep fellowship in the US. Have attended talks on it eg at ERS conference

11. Have you used it or are you currently using it?

    - Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake?

    - Is this procedure/technology performed/used by clinicians in specialities other than your own?

    - If your specialty is involved in patient selection or referral to another specialty for this procedure/technology, please indicate your experience with it.

    No

12. Please indicate your research experience relating to this procedure (please choose one or more if relevant):

- [ ] I have done bibliographic research on this procedure.
- [ ] I have done research on this procedure in laboratory settings (e.g. device-related research).
- [ ] I have done clinical research on this procedure involving patients or healthy volunteers.
- [ ] I have published this research.
- [x] I have had no involvement in research on this procedure.
- [ ] Other

13. Does the title adequately reflect the procedure?

- (•) Yes
- ( ) Other

14. Is the proposed indication appropriate? If not, please explain

Yes

15. How innovative is this procedure/technology, compared to the current standard of care? Is it a minor variation or a novel approach/concept/design?

Novel approach, but well established in other countries such as the USA

16. Which of the following best describes the procedure:

- (●) Established practice and no longer new.
- ( ) A minor variation on an existing procedure, which is unlikely to alter the procedure's safety and efficacy.
- ( ) Definitely novel and of uncertain safety and efficacy.
- ( ) The first in a new class of procedure.

17. Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?

Has potential to replace current standard care in some patients

Current management

18. Please describe the current standard of care that is used in the NHS.

Patients are ventilated when they are asleep. This may be via tracheostomy or non-invasively via mask ventilation

19. Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this?

    If so, how do these differ from the procedure/technology described in the briefing?

No

Potential patient benefits and impact on the health system

20. What do you consider to be the potential benefits to patients from using this procedure/technology?

Could come off ventilator, significant improvement to QOL and potentially safer

21. Are there any groups of patients who would particularly benefit from using this procedure/technology?

CCHS pts who are not obese

22. Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system?

    Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?

Less invasive treatment, better QOL

23. What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?

Surgeons need to be trained in the technique and physicians in how to manage pts who are being paced

24. Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?

Yes (as above) surgical expertise and pacing expertise training needed

Safety and efficacy of the procedure/technology

25. What are the potential harms of the procedure/technology?

    Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence:

    - Adverse events reported in the literature (if possible, please cite literature)
    - Anecdotal adverse events (known from experience)
    - Theoretical adverse events

    Adverse events would be those expected for an implantable neurostimulation device, such as lead component failure, lead dislodgment, implant site infection, impending pocket erosion, lead displacement, implant site haematoma. Airway obstruction during sleep may preclude decannulation in subset of patients

26. Please list the key efficacy outcomes for this procedure/technology?

    Coming off ventilation, whether ventilation is more stable (better oxygenation and carbon dioxide levels overnight), significant improvement in QOL

27. Please list any uncertainties or concerns about the efficacy and safety of this procedure/technology?

    In carefully selected patients, it is a very efficacious and safe procedure

28. Is there controversy, or important uncertainty, about any aspect of the procedure/technology?

    No

29. If it is safe and efficacious, in your opinion, will this procedure be carried out in:

- ( ) Most or all district general hospitals.
- ( ) A minority of hospitals, but at least 10 in the UK.
- (•) Fewer than 10 specialist centres in the UK.
- ( ) Cannot predict at present.

Abstracts and ongoing studies

30. Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work).

    Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a comprehensive reference list but it will help us if you list any that you think are particularly important.

    Established procedure in countries such as USA, so several papers already published which will be identified in any literature search

31. Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.

    Minimally invasive surgical approaches being developed

32. Please list any other data (published and/or unpublished) that you would like to share.

Other considerations

33. Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?

Incidence of CCHS estimated to be at 1/148,000–1/200,000 live births, not all will be eligible. The benefit of phrenic nerve pacing is greatest in patients, who need ventilator support 12–24 h/day. In these patients, the pacemaker offers freedom from the ventilator during the day. They use the small, battery-operated, and easily portable pacing transmitter during the day, allowing greater mobility and participation in sporting activities. During the night, patients should use a positive pressure ventilator. Pacing more than 12–16 h/day is generally not recommended

34. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Beneficial outcome measures.

These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured.

Ability to come off ventilator or be decannulated for those who are currently trachestomy ventilated. QOL

35. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Adverse outcome measures.

These should include early and late complications. Please state the post procedure timescales over which these should be measured:

short term post op complications
whether obstruction develops if considering decannulation
longer term complications eg pacer malfunction

Further comments

36. If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe *

Part of established care in several countries. Phrenic nerve pacing is described in the International CCHS guidelines. Would be a significant step forwards if it could be introduced in the UK

Declarations of interests

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37. Type of interest: *

- [ ] Direct: financial
- [ ] Non-financial: professional
- [ ] Non-financial: personal
- [ ] Indirect
- [x] No interests to declare

38. Description of interests, including relevant dates of when the interest arose and ceased. *

No interests to declare

39. I confirm that the information provided above is complete and correct. I acknowledge that any changes in these declarations during the course of my work with NICE, must be notified to NICE as soon as practicable and no later than 28 days after the interest arises. I am aware that if I do not make full, accurate and timely declarations then my advice may be excluded from being considered by the NICE committee.

    Please note, all declarations of interest will be made publicly available on the NICE website. *

    - (●) I agree
    - ( ) I disagree

Signature

40. Name: *

    Hui-leng Tan

41. Date: *

    29/11/2023

View results

Respondent

61 Anonymous

06:40
Time to complete

1. Project Number and Name - (Can be found on email) *

IP1994 Phrenic nerve pacing for congenital central hypoventilation syndrome and high cervical spinal injury (10:30am)

Your information

2. Name: *

Joel Dunning

3. Job title: *

Thoracic surgeon

4. Organisation: *

James cook university hospital

5. Email address: *

6. Professional organisation or society membership/affiliation: *

SCTS

7. Nominated/ratified by (if applicable):

Na

8. Registration number (e.g. GMC, NMC, HCPC) *

GMC 4443605

How NICE will use this information:

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- (●) I agree
- ( ) I disagree

The procedure/technology

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

10. Please describe your level of experience with the procedure/technology, for example:

    Are you familiar with the procedure/technology?

    Have performed diaphragm pacing and 10 years experience with other diaphragm and thoracic operations

11. Have you used it or are you currently using it?

    - Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake?

    - Is this procedure/technology performed/used by clinicians in specialities other than your own?

    - If your specialty is involved in patient selection or referral to another specialty for this procedure/technology, please indicate your experience with it.

    Used once here

12. Please indicate your research experience relating to this procedure (please choose one or more if relevant):

- [x] I have done bibliographic research on this procedure.
- [ ] I have done research on this procedure in laboratory settings (e.g. device-related research).
- [ ] I have done clinical research on this procedure involving patients or healthy volunteers.
- [ ] I have published this research.
- [ ] I have had no involvement in research on this procedure.
- [ ] Other

13. Does the title adequately reflect the procedure?

- (•) Yes
- ( ) Other

14. Is the proposed indication appropriate? If not, please explain

Yes

15. How innovative is this procedure/technology, compared to the current standard of care? Is it a minor variation or a novel approach/concept/design?

Should be standard of care

16. Which of the following best describes the procedure:

- (●) Established practice and no longer new.
- ( ) A minor variation on an existing procedure, which is unlikely to alter the procedure's safety and efficacy.
- ( ) Definitely novel and of uncertain safety and efficacy.
- ( ) The first in a new class of procedure.

17. Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?

Just needs to be performed

Current management

18. Please describe the current standard of care that is used in the NHS.

Only one hospital can offer it at the moment but its impossible to get a patient transferred to that hospital to get the surgery

19. Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this?

    If so, how do these differ from the procedure/technology described in the briefing?

Pacing of nerve or diaphragm are two options

Potential patient benefits and impact on the health system

20. What do you consider to be the potential benefits to patients from using this procedure/technology?

Get off ventilators

21. Are there any groups of patients who would particularly benefit from using this procedure/technology?

Quadriplegics

22. Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system?

    Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?

It is NHS approved

23. What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?

More centres must do it

24. Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?

The companies teach you

Safety and efficacy of the procedure/technology

25. What are the potential harms of the procedure/technology?

    Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence:

    - Adverse events reported in the literature (if possible, please cite literature)
    - Anecdotal adverse events (known from experience)
    - Theoretical adverse events

    Minimal

26. Please list the key efficacy outcomes for this procedure/technology?

    Getting off a ventilator

27. Please list any uncertainties or concerns about the efficacy and safety of this procedure/technology?

    None

28. Is there controversy, or important uncertainty, about any aspect of the procedure/technology?

    No

29. If it is safe and efficacious, in your opinion, will this procedure be carried out in:

- ( ) Most or all district general hospitals.
- ( ) A minority of hospitals, but at least 10 in the UK.
- (•) Fewer than 10 specialist centres in the UK.
- ( ) Cannot predict at present.

Abstracts and ongoing studies

30. Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work).

    Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a comprehensive reference list but it will help us if you list any that you think are particularly important.

    See previous nice guidance

31. Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.

    Yes

32. Please list any other data (published and/or unpublished) that you would like to share.

Other considerations

33. Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?

25

34. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Beneficial outcome measures.

These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured.

Getting off a ventilator

35. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Adverse outcome measures.

These should include early and late complications. Please state the post procedure timescales over which these should be measured:

Device failure

Further comments

36. If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe *

No

Declarations of interests

Please state any potential conflicts of interest relevant to the procedure/technology (or competitor technologies) on which you are providing advice, or any involvements in disputes or complaints, in the previous **12 months** or likely to exist in the future. Please use the NICE policy on declaring and managing interests as a guide when declaring any interests. Further advice can be obtained from the NICE team.

37. Type of interest: *

- [ ] Direct: financial
- [ ] Non-financial: professional
- [ ] Non-financial: personal
- [ ] Indirect
- [x] No interests to declare

38. Description of interests, including relevant dates of when the interest arose and ceased. *

None

39. I confirm that the information provided above is complete and correct. I acknowledge that any changes in these declarations during the course of my work with NICE, must be notified to NICE as soon as practicable and no later than 28 days after the interest arises. I am aware that if I do not make full, accurate and timely declarations then my advice may be excluded from being considered by the NICE committee.

    Please note, all declarations of interest will be made publicly available on the NICE website. *

    - (●) I agree
    - ( ) I disagree

Signature

40. Name: *

    Joel Dunning

41. Date: *

    04/02/2024

NICE National Institute for Health and Care Excellence

Professional Expert Questionnaire

Technology/Procedure name & indication: **IP1994 Phrenic nerve pacing**

Your information

Name:	Martin Samuels
Job title:	Consultant Respiratory Paediatrician
Organisation:	Great Ormond Street Hospital
Email address:	
Professional organisation or society membership/affiliation:	British Paediatric Respiratory Society
Nominated/ratified by (if applicable):	BPRS President & Secretary
Registration number (e.g. GMC, NMC, HCPC)	GMC 2732178

How NICE will use this information:

The information that you provide on this form will be used to develop guidance on this procedure.

☐ Please tick this box if you would like to receive information about other NICE topics.

Your advice and views represent your individual opinion and not that of your employer, professional society or a consensus view. Your name, job title, organisation and your responses, along with your declared interests will also be published online on the NICE website as part of public consultation on the draft guidance, except in circumstances but not limited to, where comments are considered voluminous, or publication would be unlawful or inappropriate.

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☒ I give my consent for the information in this questionnaire to be used and may be published on the NICE website as outlined above. If consent is NOT given, please state reasons below:

Click here to enter text.

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

1	Please describe your level of experience with the procedure/technology, for example: Are you familiar with the procedure/technology? Have you used it or are you currently using it? – Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake? – Is this procedure/technology performed/used by clinicians in specialities other than your own? – If your specialty is involved in patient selection or referral to another specialty for this procedure/technology, please indicate your experience with it.	I am a Consultant Respiratory Paediatrician with over 40 years' experience of working in the NHS and over 30 years as a consultant. During my career, I have looked after infants, children and young people with a wide variety of disorders of respiratory control and sleep-disordered breathing. This includes Congenital Central Hypoventilation Syndrome (CCHS) and I have international acknowledgment as an expert in this condition. Central hypoventilation is managed with long term respiratory support (assisted ventilation) provided via a tracheostomy or a face mask. While Phrenic Nerve Pacing (PNP) has not been available as an option for respiratory support of hypoventilation disorders in the UK, many patients undergo this procedure in several other countries around the world, including North America, Germany, Sweden, Russia and Japan. Phrenic nerve pacing has been in use for over 30 years, principally in high cervical spinal cord injury and for central hypoventilation disorders (including CCHS). In the UK, this procedure has been undertaken only in spinal cord injury units. PNP for spinal cord injury became commissioned by NHSE in 2013. I looked after 3 children, who underwent PNP in spinal units between 1997 and 2010. I attended a one-day workshop on Phrenic Nerve Pacing run by Avery Biomedical in New York in 2016. I work at Great Ormond Street Hospital, London, where we undertook implantation of phrenic nerve pacers in two cases during 2023, both in children with central hypoventilation (one with CCHS) and privately funded. These were undertaken with support from other centres outside the UK, and with a surgeon attending from Los Angeles. The use of PNP in the NHS remains at a very low level and currently exclusively (as far as I'm aware) in spinal injury units. The use of the procedure needs to be extended to allow implantation and use of PNP in other rare hypoventilation disorders.

		The procedure to implant pacers is undertaken by a surgeon, and then the pacing process is initiated and followed up long term by anaesthetic, ITU or respiratory specialists. The use of PNP is decided by a multi-disciplinary team including respiratory physician, anaesthetist / intensivist and surgeon. The two options for implantation of the device include cervical implantation, usually done by either a neurosurgeon, ENT surgeon or general surgeon, or thoracic implantation, usually done by a general surgeon or thoracic surgeon. My role in PNP has been to agree the selection of patients with the spinal unit / referring doctors, and secondly to initiate the pacing, usually undertaken a few weeks after implantation has occurred. I continue to look after patients who are on long term PNP.
2	− Please indicate your research experience relating to this procedure (please choose one or more if relevant):	**I have done bibliographic research on this procedure.** I led the development and publication of international guidelines on CCHS (2020) with a collaborative network of 9 EU countries, and this included reviewing the literature and making recommendations on the indications for, and management of PNP in CCHS. ~~I have done research on this procedure in laboratory settings (e.g. device-related research).~~ **I have done clinical research on this procedure involving patients or healthy volunteers.** The two patients who had PNP this year in GOSH both had physiological studies in laboratories to show that the pacing worked i) on submaximal exercise and ii) in conditions of hypoxia as encountered on an airline flight (modified hypoxic challenge test). **I have published this research.** This research was published as an abstract in the Great Ormond Street Conference 2023. ~~I have had no involvement in research on this procedure.~~ Other (please comment)
3	Does the title adequately reflect the procedure? How innovative is this procedure/technology, compared to the current standard of care? Is	The procedure should be called Phrenic Nerve Pacing (not diaphragmatic pacing, as the electrodes are implanted on the phrenic nerve, and not directly into the diaphragm). PNP has been used in many other countries in CCHS for more than 20 years. It has been used in the UK over this time in small numbers for high cervical spinal cord injury, but not in other conditions. The procedure is not new, but has been a standard of care in managing CCHS and

	it a minor variation or a novel approach/concept/design? Which of the following best describes the procedure (please choose one):	other rare central hypoventilation disorders in many other countries. In Los Angeles and in Munich, 40-50% of their CCHS patients are now managed with PNP. PNP should be available for the following rare central hypoventilation disorders: Congenital Central Hypoventilation Syndrome Rapid Onset Obesity, Hypothalamic Disorder, Hypoventilation and Autonomic Disorder (ROHHAD) Brainstem disorders (congenital) like Arnold Chiari malformation (where usual neurosurgical correction has already been tried) Brainstem disorders, acquired due to surgery, radiation or trauma High cervical spinal disorders from trauma, infection (myelitis) CCHS is likely to be the largest group of central hypoventilation disorders, all very rare conditions. Hypoventilation is defined as CO2 >50mmHg for >25% of sleep in the untreated patient. PNP is of most importance where there is both daytime and sleep-related hypoventilation, as it will allow greater mobility and independence. It will also be of value where there is nocturnal hypoventilation alone, especially where it simplifies care dependency (ie nursing/carers and monitoring). **Established practice [in countries outside the UK] and no longer new. The use of PNP for central hypoventilation disorders is a new indication for the UK, to supplement its use in high cervical spinal injury.** ~~A minor variation on an existing procedure, which is unlikely to alter the procedure's safety and efficacy.~~ ~~Definitely novel and of uncertain safety and efficacy.~~ ~~The first in a new class of procedure.~~
4	Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?	PNP would not replace the use of tracheostomy ventilation or mask ventilation in CCHS. However, it may be the more suitable technique to provide respiratory support for a substantial proportion of this cohort (up to 40-50% of patients, possibly 60-75 cases in the UK). It should be a standard of care for CCHS treatment choices, in addition to tracheostomy or mask ventilation.

5	Have there been any substantial modifications to the procedure technique or, if applicable, to devices involved in the procedure? Has the evidence base on the efficacy and safety of this procedure changed substantially since publication of the guidance?	No, the technique is standard and has been available in other countries for >30 years. The advances in the last 10 years include: surgeons now use thoracoscopy to implant the pacers into the right and left sides of the chest (aka minimally invasive surgery or 'keyhole' surgery); there has been an update to the pacer device by Avery Biomedical. NHSE commissioned use of PNP for high cervical spinal cord injury in 2013. Since then, there have been publications on the technique's use in CCHS, on surgical implantation methods, and on its long term safety.

Current management

6	Please describe the current standard of care that is used in the NHS.	Current treatment of CCHS and other central hypoventilation disorders involves respiratory support with either tracheostomy ventilation or mask ventilation. Outside the UK, PNP is also a choice and is used in up to 40-50% of patients with CCHS. In other central hypoventilation conditions, there is also only tracheostomy or mask ventilation. These usually need a heavy care package of carers / nurses, at least at night and sometimes in the day. These care packages cost between £200,000 and £500,000 per annum. The predominant reason for the care package is to look after the interface, ie the tracheostomy or the facemask. PNP does not have those same vulnerabilities, ie there is minimal likelihood of displacement or disconnection compared to a face mask / tracheostomy.
7	Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this? If so, how do these differ from the procedure/technology described in the briefing?	No. There are two pacing devices currently available with approval from the FDA, EU and BSI. These are devices made by Avery Biomedical (USA) and Atrotech (Finland).

Potential patient benefits and impact on the health system

8	What do you consider to be the potential benefits to patients from using this procedure/technology?	Providing mobile, discreet respiratory support in the daytime to ambulant patients, who currently find it difficult or impossible to use facemask or tracheostomy ventilation. Providing a safer and stable interface for assisted ventilation. Reducing the risks of hypoventilation, such as hypoxia, lower respiratory infections, hospital admission and sudden death. Reducing the need for high cost care packages.
9	Are there any groups of patients who would particularly benefit from using this procedure/technology?	Patients with central hypoventilation disorders, especially those with waking hypoventilation, but including those with only nocturnal hypoventilation. These disorders are rare, and include: Congenital Central Hypoventilation Syndrome Rapid Onset Obesity, Hypothalamic Disorder, Hypoventilation and Autonomic Disorder (ROHHAD) Brainstem disorders (congenital) like Arnold Chiari malformation (where usual neurosurgical correction has already been tried) Brainstem disorders, acquired due to surgery, radiation or trauma High cervical spinal disorders from trauma, infection (myelitis) Patients must have a central cause of hypoventilation (resulting from injury or disease of the brainstem or high cervical spine), intact phrenic nerves and functioning diaphragms.
10	Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system? Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?	The use of PNP in high cervical spinal injury has been shown to reduce the rate of lower respiratory infections and hospitalisation. The use of PNP is more stable than use of either tracheostomy or face mask ventilation, and may lessen the vulnerability of patients with central hypoventilation disorders. This may allow a reduction in daily monitoring, the number of hospital assessments, and care packages.
11	What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?	There needs to be a team available in centres who can assess central hypoventilation disorders and decide whether PNP is suitable for an individual patient. This team should include a respiratory consultant (adult / paediatric) and a surgeon with familiarity of the procedure. Whilst not technically demanding, any uncommon and risky surgical procedure is likely to have better outcomes when done by a surgeon who performs this on a regular basis.

		PNP needs a respiratory specialist and surgeon with expertise as a minimum. These specialists should be supported by teams (theatres, physiologists) used to undertaking the implantation, initiating pacing and providing long term monitoring and follow-up. Most patients would simply have an annual review with a brief (1-2 night) inpatient stay to assess the adequacy of treatment and make any adjustments.
12	Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?	Use of the device is not complex and experience can be gained readily to deal with the procedure and provide long term support to patients. There is no training programme specific to this procedure, although the company and experienced clinicians in other centres (eg Los Angeles and Uppsala) have provided excellent support.

Safety and efficacy of the procedure/technology

13	What are the potential harms of the procedure/technology? Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence: Adverse events reported in the literature (if possible, please cite literature) Anecdotal adverse events (known from experience) Theoretical adverse events	Risks of general anaesthesia Risks of operating around the phrenic nerve, heart, lungs and diaphragms, including phrenic nerve injury Risks of failure of the implant to function (uncommon), or subsequent breakdown of components of the device, including: – unilateral pacer malfunction eg displaced or broken antenna, low battery, increase in pacing threshold, phrenic nerve lesion – bilateral pacer malfunction (rare) eg low batteries both sides, broken antennae both sides, transmitter damage, thoracic or cervical trauma Avery Biomedical relates the following complications in their handbook related to the implants: – defective antennas especially at the connection to the stimulator – rarer are defects in the stimulator. – even rarer are defects in the implanted electrodes, this usually arising when the electrodes have been manipulated by the patient causing a break of the cable – rare problems with the receiver
14	Please list the key efficacy outcomes for this procedure/technology?	Effective provision of ventilation, monitored by adequate oxygen saturations and carbon dioxide levels. Patient tolerance and use of the pacer, if needed, during the daytime.

		Reduction in intercurrent lower respiratory infections, hospital attendances / admissions and level of care package.
15	Please list any uncertainties or concerns about the efficacy and safety of this procedure/?	The procedure has been long used in a few international centres with very good long term outcomes (>30 years). Initial concerns at the outset of this technique regarding the potential for damage to the phrenic nerves, and fatigue of the diaphragm have not been experienced.
16	Is there controversy, or important uncertainty, about any aspect of the procedure/technology?	The procedure has become a standard of care for CCHS in many countries and a favoured method of respiratory support. Controversy relates to its unavailability for patients in the UK.
17	If it is safe and efficacious, in your opinion, will this procedure be carried out in (please choose one):	~~Most or all district general hospitals.~~ ~~A minority of hospitals, but at least 10 in the UK.~~ **Fewer than 10 specialist centres in the UK – likely a few centres only, in order to keep experience centralised and at an adequate level.** Cannot predict at present.

Abstracts and ongoing studies

18	Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work). Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a comprehensive reference list but it will help us if you list any that you think are particularly important.	

19	Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.	
20	Please list any other data (published and/or unpublished) that you would like to share.	Key references of the experience of other international centres are as follows: Tsolakis N, Sindelar R, Markstrom A, Nilsson P, Jonzon A. Strategy of changing from tracheostomy and non-invasive mechanical ventilation to diaphragm pacing in children with congenital central hypoventilation syndrome. Acta Paediatrica 2022;111:1245-1247. (Sweden) Diep B, Wang A, Kun S, McComb JG, Shaul DB, Shin CE, Keens TG, Perez IA. Diaphragm pacing without tracheostomy in congenital central hypoventilation syndrome patients. Respiration 2015; 89: 534-538. (Los Angeles) Chin AC, Shaul DB, Patwari PP, Keens TG, Kenny AS, Weese-Mayer DE. Diaphragmatic pacing in infants and children with congenital central hypoventilation syndrome. In Kheirandish-Gozal L, Gozal D (eds). Sleep Disordered Breathing in Children, Respiratory Medicine, DOI 10.1007/978-1-60761-725-9_42, New York 2012. (Chicago & LA) International guidelines for the management of CCHS are found in the following publications: Kasi AS, Li H, Harford K-L et al. Congenital central hypoventilation syndrome: optimizing care with a multidisciplinary approach. J Multidisciplinary Healthcare 2022;15:455-469. (Atlanta) Trang H, Samuels M, Ceccherini et al. Guidelines for diagnosis and management of congenital central hypoventilation syndrome. Orphanet J Rare Diseases 2020;15:252. https://doi.org/10.1186/s13023-020-01460-2. Weese-Mayer DE, Berry-Kravis EM, Ceccherini I et al. An Official ATS Clinical Policy Statement: Congenital Central Hypoventilation Syndrome. Am J Respir Crit Care Med 2010;181:626-644. (International) References for incidence of CCHS Trang H, Dehan M, Beaufils F, Zaccaria I, Jeanne Amiel J, Gaultier C. The French Congenital Central Hypoventilation Syndrome Registry. Chest 2005;127; 72-79 *incidence of 1:200,000 births per year, ie 4 new cases per year (similar to UK)*

		Shimokaze T, Sasaki A, Meguro T, Hasegawa H, Hiraku Y, Yoshikawa T, Kishikawa Y and Hayasaka K. Genotype–phenotype relationship in Japanese patients with congenital central hypoventilation syndrome. Journal of Human Genetics 2015;60:473–477; doi:10.1038/jhg.2015.65 *A survey in Japan estimated an incidence at birth of 1:148,000*

Other considerations

21	Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?	For CCHS patients, perhaps 3-6 patients per year (assuming an incidence of double that). A similar number may exist for other rare central hypoventilation disorders. On introduction, there may be catch-up for some of the existing cohort of patients.
22	Please suggest potential audit criteria for this procedure/technology. If known, please describe: − Beneficial outcome measures. These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured. − Adverse outcome measures. These should include early and late complications. Please state the post procedure timescales over which these should be measured:	Beneficial outcome measures: Adequate pacing / ventilation, either alone, or if needed, in conjunction with tracheostomy / face mask ventilation (eg trach ventilation at night, PNP in the daytime) Patient comfort and well-being Fewer respiratory infections, hospital admissions Normal oxygen and carbon dioxide levels, reflecting adequate ventilatory assistance from PNP No complications from implantation or malfunction of the pacer/components. Adverse outcome measures: PNP complication of implantation of electrodes PNP not used because of patient dislike PNP failure

Further comments

23	If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe.	The implementation of a service for PNP implantation and follow-up needs to be provided from only a couple or few centres to ensure sufficient expertise in both the surgeons and the team initiating and monitoring progress; this needs to be provided for children and adults. A database of patients will need to be kept (already a requirement by the manufacturers of the pacing equipment) and appropriate long term follow up. Such a service could be provided for patients from outside the UK, either as part of a health care agreement, or on a private basis.

NICE National Institute for Health and Care Excellence

Declarations of interests

Please state any potential conflicts of interest relevant to the procedure/technology (or competitor technologies) on which you are providing advice, or any involvements in disputes or complaints, in the previous **12 months** or likely to exist in the future. Please use the NICE policy on declaring and managing interests as a guide when declaring any interests. Further advice can be obtained from the NICE team.

Type of interest *	Description of interest	Relevant dates	
		Interest arose	Interest ceased
Choose an item.			
Choose an item.			
Choose an item.			

☐ I confirm that the information provided above is complete and correct. I acknowledge that any changes in these declarations during the course of my work with NICE, must be notified to NICE as soon as practicable and no later than 28 days after the interest arises. I am aware that if I do not make full, accurate and timely declarations then my advice may be excluded from being considered by the NICE committee.

Please note, all declarations of interest will be made publicly available on the NICE website.

Print name:	**Click here to enter text.**
Dated:	**Click here to enter text.**

View results

Respondent

26 Anonymous

21:12
Time to complete

1. Project Number - (Can be found on email)

IP1994

Your information

2. Name: *

STEFANO GIULIANI

3. Job title: *

CONSULTANT NEONATAL AND PAEDIATRIC SURGEON

4. Organisation: *

GREAT ORMOND STREET HOSPITAL NHS FOUNDATION TRUST

5. Email address: *

6. Professional organisation or society membership/affiliation: *

BRITISH ASSOCIATION OF PAEDIATRIC SURGEONS - EUROPEAN ASSOCIATION OF PEDIATRIC SURGEONS - ROYAL COLLEGE OF SURGEONS OF ENGLAND

7. Nominated/ratified by (if applicable):

8. Registration number (e.g. GMC, NMC, HCPC) *

7008038

How NICE will use this information:

The information that you provide on this form will be used to develop guidance on this procedure.

Your advice and views represent your individual opinion and not that of your employer, professional society or a consensus view. Your name, job title, organisation and your responses, along with your declared interests will also be published online on the NICE website as part of public consultation on the draft guidance, except in circumstances but not limited to, where comments are considered voluminous, or publication would be unlawful or inappropriate.

For more information about how we process your data please see our privacy notice: https://www.nice.org.uk/privacy-notice

9. I give my consent for the information in this questionnaire to be used and may be published on the NICE website as outlined above. *

- (●) I agree
- ( ) I disagree

The procedure/technology

Please answer the following questions as fully as possible to provide further information about the procedure/technology and/or your experience.

10. Please describe your level of experience with the procedure/technology, for example:

    Are you familiar with the procedure/technology?

I have good experience with the procedure to implant diaphragmatic pacers in children. I have learnt the procedure during my Fellowship in Paediatric Surgery at Children's Hospital Los Angeles, USA, which is one of the centres with the largest number of diaphragmatic pacer insertions in the World. I have performed the first two cases of diaphragmatic pacer implantation in children in the UK in 2023.

11. Have you used it or are you currently using it?

    - Do you know how widely this procedure/technology is used in the NHS or what is the likely speed of uptake?

    - Is this procedure/technology performed/used by clinicians in specialities other than your own?

    - If your specialty is involved in patient selection or referral to another specialty for this procedure/technology, please indicate your experience with it.

This technology is very specialised and only used in a small number of adult centres for spinal injury in the NHS. There are no other centres in the UK which can offer this highly specialised procedure in children.

12. Please indicate your research experience relating to this procedure (please choose one or more if relevant):

- [ ] I have done bibliographic research on this procedure.
- [ ] I have done research on this procedure in laboratory settings (e.g. device-related research).
- [ ] I have done clinical research on this procedure involving patients or healthy volunteers.
- [ ] I have published this research.
- [x] I have had no involvement in research on this procedure.
- [ ] Other

13. Does the title adequately reflect the procedure?

- (•) Yes
- ( ) Other

14. Is the proposed indication appropriate? If not, please explain

yes

15. How innovative is this procedure/technology, compared to the current standard of care? Is it a minor variation or a novel approach/concept/design?

It is a novel procedure in children in the UK. As I mentioned before, we performed the first 2 cases in children at Great Ormond Street Hospital in 2023. We had support from an expert surgeon and the respiratory team from Children Hospital Los Angeles.

16. Which of the following best describes the procedure:

- (●) Established practice and no longer new.
- ( ) A minor variation on an existing procedure, which is unlikely to alter the procedure's safety and efficacy.
- ( ) Definitely novel and of uncertain safety and efficacy.
- ( ) The first in a new class of procedure.

17. Does this procedure/technology have the potential to replace current standard care or would it be used as an addition to existing standard care?

Yes, the Prenic Nerve Pacing will advance the care to children born with Hypoventilation Syndrome. It will change the standard of care which is non-invasive ventilation or tracheostomy.

Current management

18. Please describe the current standard of care that is used in the NHS.

Non-invasive ventilation and home tracheostomy with automated ventilation

19. Are you aware of any other competing or alternative procedure/technology available to the NHS which have a similar function/mode of action to this?

    If so, how do these differ from the procedure/technology described in the briefing?

No

Potential patient benefits and impact on the health system

20. What do you consider to be the potential benefits to patients from using this procedure/technology?

Large benefits for patients in term of independence from non-invasive and/or invasive ventilation. The children will become independent from external support with more freedom and better quality of life.

21. Are there any groups of patients who would particularly benefit from using this procedure/technology?

Yes, children born with Hypoventilation Syndrome/Ondine Syndrome/ ROARR/ other congenital or acquired forms of diaphragmatic palsy

22. Does this procedure/technology have the potential to change the current pathway or clinical outcomes to benefit the healthcare system?

Could it lead, for example, to improved outcomes, fewer hospital visits or less invasive treatment?

Yes, definitely it will lead to improved outcomes and better patient satisfaction/quality of life

23. What clinical facilities (or changes to existing facilities) are needed to do this procedure/technology safely?

A specialist children's hospital with all the paediatric specialties co-located.

24. Is any specific training needed in order to use the procedure/technology with respect to efficacy or safety?

No

Safety and efficacy of the procedure/technology

25. What are the potential harms of the procedure/technology?

    Please list any adverse events and potential risks (even if uncommon) and, if possible, estimate their incidence:

    - Adverse events reported in the literature (if possible, please cite literature)
    - Anecdotal adverse events (known from experience)
    - Theoretical adverse events

    Standard complications of thoracoscopy: bleeding, infection, injury to chest organs
    Malfunctioning of the device requiring replacement

26. Please list the key efficacy outcomes for this procedure/technology?

    Weaning from non-invasive ventilation and closure of tracheostomy

27. Please list any uncertainties or concerns about the efficacy and safety of this procedure/technology?

    None

28. Is there controversy, or important uncertainty, about any aspect of the procedure/technology?

    No

29. If it is safe and efficacious, in your opinion, will this procedure be carried out in:

- ( ) Most or all district general hospitals.
- ( ) A minority of hospitals, but at least 10 in the UK.
- (•) Fewer than 10 specialist centres in the UK.
- ( ) Cannot predict at present.

Abstracts and ongoing studies

30. Please list any abstracts or conference proceedings that you are aware of that have been recently presented / published on this procedure/technology (this can include your own work).

    Please note that NICE will do a comprehensive literature search; we are only asking you for any very recent abstracts or conference proceedings which might not be found using standard literature searches. You do not need to supply a comprehensive reference list but it will help us if you list any that you think are particularly important.

31. Are there any major trials or registries of this procedure/technology currently in progress? If so, please list.

32. Please list any other data (published and/or unpublished) that you would like to share.

Other considerations

33. Approximately how many people each year would be eligible for an intervention with this procedure/technology, (give either as an estimated number, or a proportion of the target population)?

5-10 children from the UK and 4-5 children from abroad.

34. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Beneficial outcome measures.

These should include short- and long-term clinical outcomes, quality-of-life measures and patient-related outcomes. Please suggest the most appropriate method of measurement for each and the timescales over which these should be measured.

Long term quality of life questionnaire

35. Please suggest potential audit criteria for this procedure/technology. If known, please describe:

Adverse outcome measures.

These should include early and late complications. Please state the post procedure timescales over which these should be measured:

Internal audit will be carried out for the first 5 years of practice

Further comments

36. If you have any further comments (e.g. issues with usability or implementation, the need for further research), please describe *

No

Declarations of interests

Please state any potential conflicts of interest relevant to the procedure/technology (or competitor technologies) on which you are providing advice, or any involvements in disputes or complaints, in the previous **12 months** or likely to exist in the future. Please use the NICE policy on declaring and managing interests as a guide when declaring any interests. Further advice can be obtained from the NICE team.

37. Type of interest: *

- [ ] Direct: financial
- [ ] Non-financial: professional
- [ ] Non-financial: personal
- [ ] Indirect
- [x] No interests to declare

38. Description of interests, including relevant dates of when the interest arose and ceased. *

NA

39. I confirm that the information provided above is complete and correct. I acknowledge that any changes in these declarations during the course of my work with NICE, must be notified to NICE as soon as practicable and no later than 28 days after the interest arises. I am aware that if I do not make full, accurate and timely declarations then my advice may be excluded from being considered by the NICE committee.

    Please note, all declarations of interest will be made publicly available on the NICE website. *

    - (●) I agree
    - ( ) I disagree

Signature

40. Name: *

    Stefano Giuliani

41. Date: *

    12/11/2023