



TRANSFORMING TREATMENT OF SPINA BIFIDA WITH PIONEERING OPEN FETAL SURGERY

According to **Neli Garbuzanova** and colleagues, collaborative working underpins the delivery of highly specialised open surgery for spina bifida

Open spina bifida is a lifelong condition that occurs in about six in every 10,000 births.¹ With clinical evidence pointing to improved outcomes for babies who received pioneering fetal surgery, NHS England Highly Specialised Services sought to establish a service that would enable eligible women in the UK to benefit from this treatment.

Following a collaborative provider-selection project delivered in partnership with NHS Arden & GEM Commissioning Support Unit (CSU), a new service is now in place, with two specialist centres providing this complex procedure.^{2,3} As well as serving the population of England, the service is accessible to women from Northern Ireland, Scotland, and Wales following agreement with their health commissioners. Between November 2019 and December 2020, 20 women from across the UK underwent open fetal surgery for congenital open spina bifida (figures derived from NHS England internal activity monitoring), which has a transformative impact on the lives of babies and their families (see Box 1).⁴

About the authors

Neli Garbuzanova (pictured)

Senior Procurement Manager, NHS Arden & GEM Commissioning Support Unit

✉ neli.garbuzanova@nhs.net

🐦 @NeliGarbuzanova

🐦 @ardengem

Bernie Stocks

Commissioning Manager, Highly Specialised Services, NHS England and NHS Improvement

Dr Ayesha Ali

Medical Advisor, Highly Specialised Services, NHS England and NHS Improvement

Fiona Marley

Head of Highly Specialised Commissioning, NHS England and NHS Improvement

Kate Steele

Chief Executive, Shine

ABOUT THE CONDITION

Public Health England describes spina bifida as ‘a neural tube defect ... where part of the baby’s neural tube does not develop or close properly. This leads to the baby’s spinal cord (the big bundle of nerves running from the brain down the baby’s back) or vertebrae (the bones of the spine) not developing as they should.’¹

There are different forms of the condition, but the most serious are forms of open spina bifida—myelomeningocele and myeloschisis—in which the spinal cord and nerve tissue bulge through a hole or area of thin membrane, referred to as the ‘defect’ or ‘lesion’, on the baby’s back.¹ Open spina bifida is typically accompanied by changes in the brain, which include hydrocephalus—excessive accumulation of fluid in the brain—and

Box 1: Case study⁴

Carrie, parent of Max and surgery recipient

'I had fetal surgery ... at UCLH to repair the defect on my unborn [baby's] spine. Max had ... myelomeningocele [Figure 1] and we were told he had around a 20% chance of walking with aids, but would most likely have no movement or feeling from the waist down and be in a wheelchair full time. He would need a shunt and have bowel and bladder issues. My local Fetal Medicine Unit ... were extremely knowledgeable and offered a pioneering surgery at UCLH as one of the options for me and my baby.'

'Max has absolutely thrived since surgery. His chiari II malformation reversed, the hydrocephalus levelled out and the fluid remained stable throughout my pregnancy. Max was born at 36 weeks [Figure 2] ... He came into the world kicking and screaming and hasn't stopped moving since. He has full feeling and movement all the way to his toes, the fluid on his brain has remained stable ... and he has full control of his bladder and bowels ... He is now a 10-month-old cheeky chappie, starting to take his first aided steps, and crawling around the house faster than I can catch him [Figure 3].'

UCLH=University College London Hospitals NHS Foundation Trust

fetal surgery, the likelihood of evolving hydrocephalus is significantly reduced, the leg weakness experienced is less severe than would have been predicted, and the abnormality at the back of the brain (Chiari II malformation) is often reversed or significantly improved.'

DEVELOPING A NEW SERVICE

Previously, open fetal surgery was not commissioned in the UK, although up to 10 women from England each year were travelling to mainland Europe, mostly Belgium, for this treatment via reciprocal healthcare arrangements with other European Union (EU) member states. Following a review by the NHS England Rare Diseases Advisory Group in December 2016, NHS England began work to commission a service in line with the *UK Strategy for Rare Diseases*, which would develop this speciality within England.^{11,12} With EU exit on the horizon, it was critical to establish an NHS-funded service with clear contractual arrangements in place with potential providers in the UK or overseas.

The project required a multidisciplinary approach from the outset. This began with the development of a detailed service specification drawing on specialist input from a wide range of stakeholders, including service users, clinicians, and providers, and through public consultation. This work was also supported by health commissioners and clinical teams from Northern Ireland. Shine is one of the specialist charities for spina bifida, and its team were instrumental in developing the specification and updating their existing patient communication materials for the new service. Since the start of the service, commissioners have been working in partnership with both Shine and Spina Bifida Hydrocephalus Scotland, which together cover the whole UK population.

The involvement of these charities has showcased the importance of the patient voice and the difference that it can make when developing and delivering

a herniation of the hindbrain through the foramen magnum known as Chiari II malformation.^{5,6}

The resulting disabilities vary according to the position of the lesion and extent of damage to the spinal cord, but can include curvature of the spine, weakness or paralysis of the lower limbs, as well as bladder and bowel dysfunction.⁶⁻⁸ Complications caused by hydrocephalus and its treatment can also lead to learning and behavioural issues.^{6,7}

The exact cause of spina bifida is unknown, but several factors are associated with the condition, including insufficient folate in the diet and hereditary factors.⁶

TREATMENT OPTIONS AND CHALLENGES

Traditionally, postnatal surgery has been carried out very soon after birth to close the lesion and reduce the likelihood of further damage or infection. However, prenatal treatment of spina bifida, where surgery is carried out to close the lesion before birth, has also become available around the world in recent years.⁹ This is extremely complex surgery, which involves opening the uterus, closing the spina bifida lesion, and then repairing the uterus (see Box 2).⁶

A randomised controlled trial—the Management of Myelomeningocele Study (MOMS)—was stopped early because of superior outcomes in the fetal surgery group, which included less need for a shunt to drain excess fluid from the brain, improvement in movement and motor function/walking at 30 months of age, and improvements in several secondary outcomes. However, the MOMS Trial identified additional risks for the mother and fetus and for future pregnancies, which have informed the suitability criteria for the surgery.¹⁰

... open fetal surgery for congenital open spina bifida ... has a transformative impact on the lives of babies and their families ...

The complexity of this surgery aside, the potential improvements in quality of life both for the baby and its parents are significant. Mr Dominic Thompson, Consultant Paediatric Neurosurgeon, Great Ormond Street Hospital (GOSH), explains: *'Following*

a high-quality, person-centred service. Patients have provided observations and feedback and been able to ask questions to continually inform and shape this service, which will potentially give babies and their parents a much better quality of life and hope for the future.

DESIGNING PROCUREMENT STRATEGIES TO OVERCOME CHALLENGES

NHS Arden & GEM CSU worked in partnership with commissioners from NHS England Highly Specialised Services to jointly design the procurement and provider-selection strategy for the new service. The hurdles to overcome in securing viable options for commissioning and delivering the new service included:

- the specialist nature of the treatment
- the small number of patients that would be eligible for treatment (approximately 10 per year)
- the small gestational age window for surgery
- the need to maintain expertise.

A collaborative approach was essential, and included setting up a multidisciplinary project group comprising clinical advisors, commissioners, procurement and communications leads, and experts in finance, contracting, quality, information governance, paediatric neurosurgery, and fetal medicine. An international expert in spina bifida surgery (from outside Europe, to avoid a conflict of interest) provided invaluable input, enabling the specification to incorporate global best practice. Two patient representatives actively participated in all stages of the process, keeping patients at the heart of service provision.

Dedicated market-engagement sessions for interested providers were set up to share provider selection intentions and open dialogue. Case studies, tips on developing successful proposals, and a dedicated session to discuss common bid mistakes were facilitated to give potential providers

Figure 1: Second-trimester ultrasound scan of Max showing myelomeningocele



Reproduced with permission.

Figure 2: The site of myelomeningocele repair on Max's back after birth



Reproduced with permission.

the best possible opportunity to make an informed submission.

Following analysis of clinical, financial, and market considerations, a lotting model was put forward suggesting two fetal surgery centres (FSCs). Finding the right balance between providing care close to patients and maintaining service quality was a challenge. Few UK centres had the necessary experience in this complex surgery, which involves a high level of risk for both mother and fetus, so two types of solution were invited:

those meeting the service specification in full; and those involving collaboration with other centres and specialists to acquire the level of expertise needed. Collaboration between both FSCs and referring regional fetal medicine units (RFMUs) was essential for developing and maintaining expertise; a requirement built into the provider-selection questionnaires and evaluation criteria. This process prioritised fully compliant and experienced centres, and led to the selection of two FSCs: one in the UK, and one in mainland Europe.

Figure 3: Max at 10 months of age



Reproduced with permission.

The two FSCs (UCLH and UHL), in conjunction with paediatric neurosurgery and general surgery teams at GOSH, operate a joint surgical decision-making team and provide cross cover, which effectively means one service across two sites. Patient assessments and surgeries are distributed between the two sites via geographic mapping based on traditional fetal medicine referral routes from the RFMUs.^{2,13,14} Enquiries and referrals are welcomed by the service from all RFMUs throughout the UK, which provide specialist maternity care and refer to the service when there is a concern for the health of the unborn child.

Comprehensive briefing documents describing the service and eligibility criteria have been provided to all RFMUs. Referring clinicians and their teams have also been invited to attend surgeries to increase their knowledge of the condition and build first-hand local experience and expertise. Reports to disseminate information to RFMUs and patient groups have been issued following two periodic clinical review meetings with the FSCs and NHS England commissioners.

As part of the service—and in order to deliver on the Government’s UK *Strategy for Rare Diseases* aim of co-ordination of care¹² and for all patients to have an ‘alert card’¹⁵—all those involved in the patient’s maternity care, including the GP and local midwifery unit, are fully briefed about the surgery and requirements for care of the mother between surgery and delivery of the baby in case the mother goes into spontaneous labour ahead of the planned caesarean section.

Shine and Spina Bifida Hydrocephalus Scotland offer additional emotional support and advice to the mother before, during, and after surgery to complete the service’s holistic approach.

OUTCOMES

According to Dr Michael Belfort, Specialist in Maternal–Fetal Medicine

MOBILISATION

Joint service initiation meetings were held with the wider clinical staff and management teams on consecutive days in preparation for the service starting. Weekly calls have been held by commissioners with clinical leads since November 2018 to troubleshoot any teething issues and consider any organisational, referral, or communications issues. Formal clinical review meetings have been held every 6 months, and the service has developed learning updates for referring clinicians to educate teams and improve understanding.

THE NEW SERVICE

For the next 4 years (2020–2024), eligible women carrying a fetus with open spina bifida will be offered high-quality surgery from two specialist FSCs: University College London Hospitals NHS Foundation Trust (UCLH), with specialist input from GOSH, in London; and University Hospitals Leuven (UHL), in Leuven, Belgium.³ Leuven is a very experienced specialist centre for this and other types of fetal surgery and, for many women, offers a shorter travel time than to the UK FSC. Some 10 surgeries are expected to take place each year in Leuven for women from England.

and Fetal Intervention at Texas Children's Hospital in the US and a key stakeholder in the procurement process, 'The impact of this fetal surgery on the life of the patient cannot be overstated. The reduction in the rate of hydrocephalus, and the increase in the rate of independent ambulation are simply life changing ... in some cases, the difference between a life in a wheelchair and a life of independent movement.'⁴

Between November 2019 and December 2020, 20 surgeries were delivered via the new service to women across the UK, all of whom went on to deliver their babies safely via planned caesarean section (figures derived from NHS England internal activity monitoring).

After surgery, women continue to receive care from the RFMU and their local maternity service until birth, which is via planned caesarean section at around 37 weeks of gestation as per the MOMS Trial.^{2,7,10} After delivery, babies are followed up at their regional paediatric neurosurgical spina bifida unit, which comprises experts on their coordinated care, including bladder assessment, physiotherapy, and neurodevelopment.

The regional unit liaises closely with the FSC to ensure that long-term outcomes are recorded—in this way, service quality can be continually reviewed. Weekly meetings are also held between the FSCs and NHS England to review outcomes and ensure that the necessary help is in place to support this new service.

This important project has made available an evidence-based, ground-breaking intervention for women, improving the outcomes for their babies. The success of the project is down to the collaborative efforts of everyone involved in the design, commissioning, mobilisation, and delivery of the new service. **SM**

REFERENCES

- Public Health England. *Spina bifida—information for parents*. www.gov.uk/government/

Box 2: Delivering highly specialised treatment

Professor Anna David, Clinical Lead, UCLH and UCL

'Our work begins from the moment a fetus is diagnosed with spina bifida. We work with families to explain what this means and the options available to them. Thanks to this project, those options now include fetal surgery, provided there are no other complications with the fetus, the mother is well, and type of spina bifida defect in the fetus meets the eligibility criteria. The surgery does carry risks for both the mother and fetus, which are carefully explained before consent is sought to proceed.'

Professor Jan Deprest, Fetal Surgeon, UHL and UCLH

'When surgery is carried out, it is a team effort—we are effectively performing two different surgeries on two patients at the same time. During the procedure, there are numerous specialists present, including two anaesthetists (one for the mother, one for the fetus) with supporting staff, two obstetricians, two neuro-paediatric surgeons, two people scanning and monitoring the fetus, a paediatric neonatologist, and the scrub team.'

UCLH=University College London Hospitals NHS Foundation Trust; UCL=University College London; UHL=University Hospitals Leuven

- publications/spina-bifida-information-for-parents/spina-bifida-information-for-parents (accessed 18 January 2021)
- NHS England. *Open fetal surgery to treat fetuses with open spina bifida*. London: NHS England, 2018. Available at: www.england.nhs.uk/commissioning/publication/open-fetal-surgery-to-treat-fetuses-with-open-spina-bifida/
- Shine. *Prenatal surgery*. www.shinecharity.org.uk/spina-bifida/prenatal-surgery (accessed 18 January 2021).
- Health Care Supply Association. *Transforming the lives of babies with spina bifida*. nhsprocurement.org.uk/transfoming-the-lives-of-babies/ (accessed 18 January 2021).
- Shine. *Chiari II malformation (also known as Arnold-Chiari Malformation, ACM, and hindbrain herniation)*. www.shinecharity.org.uk/related-conditions/chiari-ii (accessed 18 January 2021).
- University College London Hospitals NHS Foundation Trust. *Fetal spina bifida and surgical closure during pregnancy—information for pregnant women & families*. London: University College London Hospitals NHS Foundation Trust, 2018. Available at: www.ucl.ac.uk/womens-health/sites/womens-health/files/fetal_spina_bifida_and_surgical_closure_during_pregnancy.pdf
- University College London Hospitals NHS Foundation Trust. *Fetal surgery for spina bifida*. www.youtube.com/watch?v=or76PARXN10&feature=youtu.be (accessed 18 January 2021).
- Brock J, Thomas J, Baskin L et al; for the Eunice Kennedy Shriver NICHD MOMS Trial Group. Effect of prenatal repair of myelomeningocele on urological outcomes at school age. *J Urol* 2019; **202** (4): 812–818.
- Sacco A, Simpson L, Deprest J, David A. A study to assess global availability of fetal surgery for myelomeningocele. *Prenat Diagn* 2018; **38** (13): 1020–1027.
- Adzick N, Thom E, Spong C et al. A randomized trial of prenatal versus postnatal repair of myelomeningocele. *N Engl J Med* 2011; **364**: 993–1004.
- NHS England. *Urgent clinical commissioning policy statement: prenatal surgery for open spina bifida*. London: NHS England, 2018. Available at: www.england.nhs.uk/publication/urgent-clinical-commissioning-policy-statement-prenatal-surgery-for-open-spina-bifida/
- DH. *UK Strategy for Rare Diseases*. London: DH, 2013. Available at: assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/260562/UK_Strategy_for_Rare_Diseases.pdf
- NHS England. *E09. Specialised women's services*. www.england.nhs.uk/commissioning/spec-services/npc-crg/group-e/e09/ (accessed 18 January 2021).
- NHS England. *Women and children*. www.england.nhs.uk/commissioning/spec-services/npc-crg/group-e/ (accessed 18 January 2021).
- NHS England Specialised Services. *Implementation plan for the UK Strategy for Rare Diseases*. London: NHS England Specialised Services, 2018. Available at: www.england.nhs.uk/wp-content/uploads/2018/01/implementation-plan-uk-strategy-for-rare-diseases.pdf

The authors wish to acknowledge the following contributors:

Professor Anna David

Clinical Lead, University College London Hospitals NHS Foundation Trust and University College London

Professor Jan Deprest

Fetal Surgeon, University Hospitals Leuven and University College London Hospitals NHS Foundation Trust

Mr Dominic Thompson

Consultant Paediatric Neurosurgeon, Great Ormond Street Hospital