GUIDELINES

Spasticity in children and young people with non-progressive brain disorders: summary of NICE guidance

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This is one of a series of *BMJ* summaries of new guidelines based on the best available evidence; they highlight important recommendations for clinical practice, especially where uncertainty or controversy exists.

Further information about the guidance, a list of members of the guideline development group, and the supporting evidence statements are in the full version on bmj.com.

Spasticity is a form of hypertonia[1] and is associated with conditions such as cerebral palsy, which affects 110 000 people in the United Kingdom.[2] More than 2000 children born this year in the UK will develop spasticity, which, if unmanaged, will cause pain, affect quality of life, and may lead to complications requiring major surgery. Children and young people with spasticity need early referral to local services that will meet their individual needs and allow them access to the range of interventions that will encourage their motor development. This article summarises the most recent recommendations from the National Institute for Health and Clinical Excellence (NICE) on the management of spasticity in children and young people with non-progressive brain disorders, including those with cerebral palsy.[3]

Recommendations

NICE recommendations are based on systematic reviews of best available evidence and explicit consideration of cost effectiveness. When minimal evidence is available, recommendations are based on the Guideline Development Group's experience and opinion of what constitutes good practice. Evidence levels for the recommendations are in the full version of this article on bmj.com.

Principles of care

Delivering care

- Children and young people with spasticity should have access to a network of care that uses agreed care pathways supported by effective communication and integrated team working, and provides access to healthcare professionals experienced in the care of such people. The network team should provide local expertise in paediatrics, nursing, physiotherapy, and occupational therapy. Access to other expertise, including orthotics, orthopaedic surgery (and/or neurosurgery), and paediatric neurology, may be provided locally or regionally.

Box 1 | Possible indicators of hip displacement

- Pain arising from the hip
- Clinically important difference in leg length
- Deterioration in hip abduction or range of hip movement
- Increasing muscle tone in the hip
- Deterioration in sitting or standing
- Increasing difficulty with perineal care or hygiene

- If a person receives treatment for spasticity from healthcare professionals outside the network team, this should be planned and undertaken in discussion with the network team to ensure integrated care and effective subsequent management.

Management programmes

- After diagnosis, ensure that all children and young people with spasticity are referred without delay to an appropriate member of the network team.
- Offer a management programme that is *(a)* developed and implemented in partnership with the individual and his or her parents or carers, tailored to the individual, and goal focused; and *(b)* takes into account the programme's possible impact on the person and family.
- Carefully assess the impact of spasticity in people with cognitive impairments: be aware that any benefit of treatments may be more difficult to assess in those with limited communication; and ensure that they have access to all appropriate services.
- Record the individualised goals and share these goals with the network team and, where appropriate, other people involved in their care.
- Offer relevant, and age and developmentally appropriate information, and educational materials; regular opportunities for discussion; and advice on individuals' developmental potential and how treatment options may affect this.

Monitoring

- Monitor for response to treatments; worsening of spasticity; secondary consequences of spasticity (for example, pain or contractures); and the need to change individualised goals.
- Recognise clinical findings that are possible indicators of hip displacement (hip migration greater than 30%) (box 1).
- Offer hip radiography to assess for hip displacement if there are clinical concerns about possible hip displacement or at 24 months in children with bilateral cerebral palsy.

Physical therapy (physiotherapy and/or occupational therapy)

General principles

- Everyone referred to the network team should be promptly assessed by a physiotherapist and, where necessary, an occupational therapist.

- Offer a programme of physical therapy (physiotherapy and/or occupational therapy) that is tailored to the individual's needs and goals, such as enhancing skill development, function, and ability to participate in everyday activities; and preventing consequences such as pain or contractures.
- When deciding who should deliver physical therapy, take into account whether the person and parents or carers can deliver this therapy, what training they might need, and what their wishes are.
- Encourage individuals and their parents or carers to incorporate physical therapy into daily activities—for example, standing while brushing their teeth (to stretch leg muscles).

Specific strategies

- Consider including 24 hour postural management strategies *(a)* to prevent or delay the development of contractures or skeletal deformities in those at risk of developing these; and *(b)* to enable participation in activities appropriate to their stage of development.
- Consider task focused, "active use" therapy, such as "constraint induced movement therapy" (temporary restraint of an unaffected arm to encourage use of the other arm) followed by bimanual therapy (unrestrained use of both arms) to enhance manual skills.
- Consider muscle strengthening therapy when the assessment indicates that muscle weakness is contributing to loss of function or postural difficulties.
- Provision of an adapted physical therapy programme is essential after treatment with botulinum toxin type A, continuous pump administered intrathecal baclofen, orthopaedic surgery, or selective dorsal rhizotomy.

Orthoses

General principles

- Consider orthoses based on individual needs and goals, such as improving posture, upper limb function, or walking efficiency; preventing or slowing hip migration or development of contractures; relieving discomfort, pain, or pressure points.

Specific uses

- Consider using elbow gaiters to maintain extension and improve function; rigid wrist orthoses to prevent contractures and limit flexion deformity; and dynamic orthoses to improve hand function (for example, a non-rigid thumb abduction splint to allow some movement with a "thumb in palm" deformity).
- Consider ankle-foot orthoses for those with serious functional limitations (level IV or V in the gross motor function classification system; box 2) to aid sitting, transfers between sitting and standing, and assisted standing.
- If an orthosis is used overnight, check that it is acceptable to the child or young person and does not cause injury or disturb sleep.

Box 2 | Gross motor function classification system

Level I—Walks without restrictions
Level II—Walks without assistive devices
Level III—Walks with assistive devices
Level IV—Has limited self mobility
Level V—Has severely limited self mobility even with assistive devices

Continuing assessment

- The network team should review the use of orthoses at every contact with the person. Ensure the orthosis remains acceptable to the person and parents or carers; remains appropriate to treatment goals; is being used as advised; remains well fitting and in good repair; and is not causing adverse effects such as discomfort, pain, sleep disturbance, injury, or excessive muscle wasting.

Oral drugs

- Consider oral diazepam or oral baclofen if spasticity is contributing to discomfort or pain; muscle spasms (for example, at night); or functional disability. Diazepam is particularly useful if a rapid effect is desirable (for example, in a pain crisis), and baclofen for a sustained long term effect (for example, to relieve continuous discomfort or improve motor function).
- Give oral diazepam treatment as a bedtime dose. If the response is unsatisfactory consider increasing the dose or adding a daytime dose.
- Start oral baclofen treatment with a low dose and increase the dose stepwise over about four weeks to achieve the optimum therapeutic effect.
- Continue using oral diazepam or oral baclofen if they have a clinical benefit and are well tolerated, but consider stopping the treatment whenever the child's or young person's management programme is reviewed and at least every six months.
- If the response to oral diazepam and oral baclofen used individually for four to six weeks is unsatisfactory, consider a trial of combined treatment using both drugs.
- If stopping oral diazepam and/or baclofen after several weeks of use, ensure that the dose is reduced in stages to avoid withdrawal symptoms.
- If dystonia is considered to seriously impair posture or function or cause pain, consider a trial of oral drug treatment—for example, with trihexyphenidyl, levodopa, or baclofen.

Botulinum toxin type A

- Consider this treatment for focal spasticity of the upper limb that is impeding fine motor function; compromising care and hygiene; causing pain; impeding tolerance of other treatments, such as orthoses; or causing cosmetic concerns to the person.

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Previous articles in this series

- Risk identification and interventions to prevent type 2 diabetes in adults at high risk: summary of NICE guidance (*BMJ* 2012;345:e4624)
- Management of an acute painful sickle cell episode in hospital: summary of NICE guidance (*BMJ* 2012;344:e4063)
- Management of venous thromboembolic diseases and the role of thrombophilia testing (*BMJ* 2012;344:e3979)
- Recognition, referral, diagnosis, and management of adults with autism (*BMJ* 2012;344:e4082)
- Management of acute upper gastrointestinal bleeding (*BMJ* 2012;344:e3412)

- Consider this treatment where focal spasticity of the lower limb is impeding gross motor function; compromising care and hygiene; causing pain; disturbing sleep; impeding tolerance of other treatments, such as orthoses and equipment to support posture; or causing cosmetic concerns to the person.
- Consider this treatment after an acquired non-progressive brain injury if rapid onset spasticity is impairing posture or function.
- Consider a trial of this treatment for focal dystonia that is seriously impairing posture or function or causing pain.
- Before starting this treatment, advise people and their parents or carers of its rare but serious complications (swallowing and breathing difficulties) and how to recognise signs suggesting these complications. Advise that these complications may occur at any time during the first week after the treatment and that if these complications occur they should return to hospital immediately.

Intrathecal baclofen

- Consider treatment with continuous pump administered intrathecal baclofen if, despite the use of non-invasive treatments, spasticity or dystonia is causing pain or muscle spasms, or difficulties with posture, function, self care, or care by parents or carers.
- Be aware that those who benefit from this treatment typically have moderate or severe motor function problems (level III, IV, or V; box 2) or bilateral spasticity affecting upper and lower limbs.
- Support those receiving this treatment and their parents or carers by offering regular follow-up with the network team and a consistent point of contact with the specialist neurosurgical centre.

Orthopaedic surgery

- Consider orthopaedic surgery as an important adjunct to other interventions. Timely surgery can prevent deterioration and improve function.
- If clinical or radiological findings indicate possible spinal deformity or hip displacement, an orthopaedic surgeon in the network team should do an assessment.
- Consider an assessment by an orthopaedic surgeon in the network team if limb function is limited by unfavourable posture or pain as a result of muscle shortening, contractures, or bony deformities; any upper limb contractures cause difficulty with skin hygiene; or the cosmetic appearance of the upper limb causes major concern for the person.

Selective dorsal rhizotomy

- Consider selective dorsal rhizotomy to improve walking ability in children and young people who are at level II or III in the gross motor function classification system (box 2). Patient selection and treatment should be carried out by a multidisciplinary team with specialist training and expertise in the care of spasticity, and with access to the full range of treatment options. Discuss the irreversibility of the treatment, the known complications and the uncertainties over long term outcomes with children and young people, and their parents and/or carers (and see also NICE's interventional procedure guidance[4]). Teams offering selective dorsal rhizotomy should participate in a coordinated, national, agreed programme to collect information on short and long term outcomes on all patients assessed for selective dorsal rhizotomy, regardless of whether selective dorsal rhizotomy is performed. These recorded outcomes should include measures of muscle tone, gross motor function, neurological impairment, spinal deformity, quality of life, and need for additional operations, with nationally agreed consistent definitions.

Overcoming barriers

Delivering services for the individual needs of children and young people with spasticity may be difficult if they cannot express their views and preferences clearly. The aim of the recommendation to carefully assess the impact of spasticity in those with cognitive impairments is to help formulate appropriate management programmes. Delivering integrated services that encompass the entire range of recommended interventions may be difficult to achieve in some localities. The definition of the network team is deliberately flexible to facilitate commissioning of services unrestricted by institutional or regional boundaries.

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A PATIENT'S JOURNEY

Gitelman syndrome

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This is one of a series of occasional articles by patients about their experiences that offer lessons to doctors. The BMJ welcomes contributions to the series. Please contact Peter Lapsley (plapsley@bmj.com) for guidance.

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Previous articles in this series

- Kidney dialysis—the need for humanity (*BMJ* 2012;345:e4492)
- After repair of tracheo-oesophageal atresia (*BMJ* 2012;344:e3517)
- Lyme neuroborreliosis (*BMJ* 2012;344:e3250)
- Gitelman syndrome (*BMJ* 2012;344:e3590)
- Ulcerative colitis (*BMJ* 2012;344:e2947)

Peter Park describes the not always easy interaction between the informed patient with a rare disorder and the medical profession, which he calls "a critical quadripartite relationship"

General practitioner: "No, you cannot possibly take that quantity of magnesium, and besides, magnesium glycerophosphate is not on the formulary on my NHS computer."
Me: "But Doctor, as a Gitelman syndrome sufferer, that is, in fact, the dosage and formulation prescribed by the consultant nephrologist, and, in actual fact, Doctor, you will need to hand write a prescription for this drug to be specially ordered and manufactured."
GP: "Gitel what? …"

The above exchange is one variant on how I, as a fully diagnosed Gitelman syndrome patient already under the care of a nephrologist, began my relationship with each of the five general practitioners in the four surgeries I have been assigned to as I have moved around the UK. Despite early frustration, with a little patience and education by myself and the occasional "enlightened guidance" from the consultant nephrologist, each of these GPs save one (who was inexplicably reluctant to contact the nephrologist for advice) came to be a full participant on the proactive team that keeps me on track today, adjusting drugs over time.

A journey that began in the 1950s

When I was 10 years old, lack of energy and what I now know was tetany were the initial warning signs that, along with low potassium levels, began my medical journey. It quickly led to Dr C Everett Koop, a pioneering children's surgeon, and Dr Wallace McCrory, a paediatric nephrologist, at the Children's Hospital of Philadelphia in my native Pennsylvania. In the absence of today's technology, the tentative diagnosis was a probable tumour on the kidneys or adrenal glands, and an exploratory laparotomy was performed, with negative results. I went on a course of (then) poorly coated and foul tasting potassium chloride tablets, and Dr Koop went on a medico-political path to become Surgeon General of the United States (1982–89).

Managed for several years solely with large doses of potassium chloride, I often felt lethargic, but my condition began to come into sharper focus shortly after 1962, when Dr Frederic Bartter described the syndrome that bears his name. The addition to my drugs list of Aldactone (spironolactone) to regulate potassium wastage was an immediate change, followed later by the addition of magnesium to the mix by successive endocrinologists. I grew up, married, and my wife and I had a child (who, now aged 29, exhibits none of the signs of Gitelman syndrome). I ran, biked, and sailed in my 20s and 30s, albeit never with the stamina of my peers, and I generally felt OK.

I always thought I had Bartter syndrome, and it was not until I nearly died in 2005, after a severe stomach bug that caused kidney failure and, paradoxically, a dangerously high potassium level, that I was referred to a specialist clinic at Addenbrooke's Hospital, Cambridge. There, the true Gitelman syndrome variant was diagnosed through genetics, and a comprehensive and well monitored treatment regimen began. Since then, I have twice had knee replacement surgery without incident (though I suspect I had to have a lot more blood tests than most patients), and I feel well most of the time, except when I miss a dose of a key drug such as potassium chloride for eight or more hours.

GP meets informed patient with a rare condition

When someone such as myself first presents to a doctor for a related, or unrelated, ailment, either at a surgery or at a hospital, and claims to be a Gitelman or Bartter syndrome patient, several possible reactions can be expected. The most likely one is a vague recollection by the doctor of the syndrome name, perhaps from a single lecture during medical training years ago, but no further immediate knowledge.

Another reaction (this one a bit tedious for the patient) is the doctor whose face tells you that, as a "walking medical curiosity," you have just brightened up an otherwise boring day spent treating ingrowing toenails and hard to diagnose itches. You just know this doctor will be late for the next patient as he or she fairly leaps for the computer to access Wikipedia the second you leave the treatment room.

A third reaction is the (thankfully quite rare these days) physician who believes that an "informed patient" is an oxymoron and is thus inclined to dismiss your description of the condition and its treatment unless and until it is confirmed by a consultant medical professional in the subject. This reaction is especially likely for juvenile and young adult patients.

The good news is that almost certainly each of these reactions by a GP can be guided with a little effort toward the fully supportive role that is essential for optimal care of a rare and sometimes unpredictable condition. It is important to note that, at core, I think that is what our GPs want as well.

Equally important is for us, as Gitelman/Bartter patients, to understand that, although the consultant nephrologist may be the pinnacle of knowledge on the disease and in the best position to guide GPs and ourselves, he or she simply cannot be called on by patient or GP for every small event in our treatment. As informed patients, we need to be able to take responsibility, shared

A CLINICIAN'S PERSPECTIVE

Me: "Thank you for completing the urine collections and blood tests after our first meeting, when your GP asked me to look into your low potassium. The results show that your potassium and magnesium levels are both still low, and there's too much of both in your urine. So I think we can put a name to the symptoms of tiredness, weakness, pins and needles, palpitations, cramps, nausea, and chest pain that have made your life a misery for a long time. I think you have Gitelman syndrome."

Patient: "I've never heard of that."

Me: "It's quite rare, but there are probably at least a thousand people with it in the UK. Tell me, when you were a child did you prefer sweets or crisps as treats?"

Patient: "Oh, always crisps. I used to eat jars of pickled cucumbers as well."

Me: "That's a very familiar response. Your body was telling you it needed salt. OK, let's start talking about how you are going to manage both your diet and the potassium and magnesium tablets you will need to take for the rest of your life."

This is a highly truncated summary of some essential features of Gitelman syndrome. As Mr Park describes, Gitelman syndrome and a similar disorder, type 3 Bartter syndrome, are rare, autosomal recessive renal tubular disorders that usually emerge after childhood and affect the kidneys' ability to conserve potassium or magnesium, or both. The usual scenario is of someone with rather non-specific symptoms, perhaps "off sick" from work a lot or reluctant to do sports at school, who is found to have a low blood potassium level and lowish (sometimes disablingly low) blood pressure. Sometimes, however, life threatening arrhythmia may be the presenting feature.

In most cases both potassium and magnesium levels are low. Treatment, with potassium and magnesium supplements, is at best unpalatable and at worst intolerable, mainly because the quantities required may be very large. There are additional options—including amiloride, spironolactone, angiotensin receptor blockers or ACE inhibitors, and β blockers—but use of these may be constrained by hypotension. Every patient's needs are different, so protocols cannot easily be prepared.

Among my patients, there is a wide variety of knowledge. Those like Mr Park, who was diagnosed in childhood, are, of course, the easiest. He has a pretty comprehensive understanding of his own pathophysiology and is able to self manage to the same extent that a well informed person with diabetes does. Newer or less able patients may rely much more on their specialists or GPs to interpret their potassium, magnesium, and blood pressure levels and advise them when they need to change treatment. We try to make sure that our patients' GPs are "in the know," but of course this must be balanced against their other commitments. This is particularly relevant for unlicensed treatments, of which magnesium supplementation in this context is a good example: there is almost nothing at all in the *British National Formulary*.

One thing unites my cohort of close to 50 patients with Gitelman or Bartter syndrome (apart from a general feeling of diagnostic isolation and their long term symptoms): most have been doubted by someone in a healthcare role at one time or another. A GP friend commented that she thinks that patients with this type of disorder (or their carers) should always be the ones in the driving seat—carrying with them paperwork on hospital headed notepaper that can provide authoritative confirmation that the treatment being proposed by the patient is valid, and also inform other healthcare staff. This seems like a good goal to work towards: above all, patient and family empowerment and good communication are key.

Fiona E Karet Frankl

RESOURCES FOR PATIENTS AND CLINICIANS

The Bartter Site (http://barttersite.org/)—
Provides information and support for Bartter and Gitelman syndromes

RareRenal.org.
Hypokalaemic alkalosis (www.rarerenal.org/diseases/hypokalaemic-alkalosis/)
—Provides information for patients and professionals, and a forthcoming opportunity to enrol in a national patient registry

Cambridge University Hospitals, Renal Genetics and Tubular Disorders Clinic.
Information about Gitelman syndrome (www.cuh.org.uk/resources/pdf/patient_information_leaflets/PIN1446_gitelmans_information.pdf)

British Kidney Patient Association (BKPA) (www.britishkidney-pa.co.uk)—UK registered charity working to improve quality of life for patients with kidney disease. It provides information, advice, and small grants to help patients and families with kidney disease and financial support to kidney units throughout the UK

with our GP and coordinated with the pharmacist, for the day to day management of the syndrome, and of syndrome related aspects of other ailments.

While the relationship with the consultant nephrologist (or endocrinologist) is based on far more shared knowledge and therefore much easier, it too needs to be managed for good outcomes. The patient presenting for perhaps a semi-annual appointment with the specialist can maximise the precious minutes of this encounter by such simple things as working with the GP to ensure recent blood work is available before the appointment, and writing down questions before the day, including reporting on experience, good or bad, with drugs and other healthcare professionals.

My pharmacist: a VIP

An often overlooked part of managing Gitelman or Bartter syndrome is the role of the pharmacist. This medical professional, who in the UK has completed not only a four year Master of Pharmacy degree but at least one extra year before registration, can be the linchpin in care, and a most welcome and informed advocate with the prescription clerk in the GP's surgery, and often, via the clerk, with the GP. I have also learnt that, though in a lesser role, the surgery prescription clerk is another person the patient will want to have "on side"—well worth a quick word when you are at the surgery.

In my case, a committed young pharmacist, after researching the drug, spent hours convincing the surgery that, by ordering magnesium glycerophosphate in quantities starting with 5000 capsules, amazing savings in NHS costs would follow for this specially formulated drug from the economies of a larger production run.

Any patients who fail to get to know their pharmacist and discuss their prescriptions with him or her, and any GP who fails to listen to this professional on drug formulations and interactions, are missing good information. Like GPs, pharmacists may initially question prescribed doses and medications (a fellow patient described this as the "policeman" role), but all will benefit from the time invested in education and discussion, as this medical professional becomes a member of your personal care team.

My life today

Although I live with a condition that most people have never heard of, and have had to become an "expert patient," I am pretty lucky: although the treatments I must take don't taste good and I almost need an extra bag when I travel just for them, they and my care team have meant that I have had enough energy to hold down a job and now look forward to retirement.

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