MOTOR DEVELOPMENT TOOLKIT

iHV Insights

Child Development:
Launching iHV's Motor Development Toolkit

21 November 2024

#iHVInsights

www.ihv.org.uk

Welcome

Make the most of the opportunity

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Ask - type your questions to the panel as you think of them during the presentations – use the Q&A function:

- **Name of presenter** you would like to answer your question
- Brief **question**
- **Your name** (if you wish to do so)

Feedback
Participate in the poll or contact us at events@ihv.org.uk

Hashtag X (formally Twitter) - #iHVInsights

Continue your CPD:

- Access recording of live session after the event on our website (no recording on other devices is allowed)
- Attendance at this event and engagement with the iHV reflection template will support you with demonstrating CPD activities that align to the 2022 NMC Standards of Proficiency for SCPHN Health Visiting.

The Institute of Health Visiting is a charity and centre of excellence for health visiting. Its core purpose is:
To improve outcomes for children and families and reduce health inequalities through strengthened health visiting services

Agenda

Welcome

- Vicky Gilroy - Director of Innovation and Research, iHV

Guest Speakers

- Dr Anne-Marie Childs - Consultant Paediatric Neurologist, Leeds Teaching Hospital
- Pilar Cloud - Chief Executive, Action Cerebral Palsy
- Tori Blake - Parent with lived experience
- Victoria Jackson - Lead Programme Manager - Innovation and Research, iHV

Q&A with Speakers

Close and Evaluation

Why do we need a motor development toolkit?

Insights from practitioners

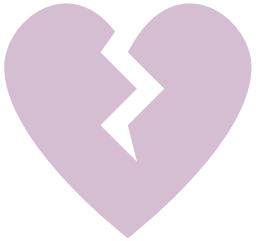
50%	Reported lack of confidence in identifying red flags
32%	Use a motor development assessment tool
60%	Have received no training on motor development
75%	Feel confident in having conversations with families

Impact of motor development delay

Physical activity

Social engagement

Parent and child mental health



Relationships

Education achievement

Impact on school readiness of 4-5 year olds

Gross motor skills

46% cannot sit still

4% cannot walk up and down stairs without help in 2023 and 2024

90% of parents say their child was ready for school **compared** to teachers reporting only **50%** being ready

Fine motor skills in 2023 & 2024

39% struggle to hold a pencil

35% cannot draw a person with a body

- 25% of girls and 48% of boys

26% cannot copy a triangle, circle and square

- 19% girls and 37% of boys

40% cannot dress and undress without assistance

- 35% of girls and 46% of boys

Next speaker

Dr Anne-Marie Childs

Consultant Paediatric Neurologist, Leeds Teaching Hospital

Type your questions as you think of them during the presentations

Use the Zoom Q&A function

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zolgensma® (onasemnogene abeparvovec)

Motor development and the importance of early intervention in Spinal Muscular Atrophy

Dr Anne-Marie Childs

Consultant Paediatric Neurologist

Leeds Teaching Hospitals NHS Trust

ZOLGENSMA is indicated for the treatment of:[1]

- Patients with 5q SMA with a bi-allelic mutation in the *SMN1* gene and a clinical diagnosis of SMA Type 1, or
- Patients with 5q SMA with a bi-allelic mutation in the *SMN1* gene and up to 3 copies of the *SMN2* gene

Prescribing information for Great Britain and Northern Ireland is available at the end of this presentation

Adverse Event Reporting: Adverse events should be reported. Reporting forms and information can be found at www.mhra.gov.uk/yellowcard. Adverse events should also be reported to Novartis online through the pharmacovigilance intake (PVI) tool at www.novartis.com/report or alternatively email medinfo.uk@novartis.com or call 01276 698370.

SMA, spinal muscular atrophy; SMN, survival motor neuron. **Reference: 1.** Zolgensma Summary of Product Characteristics.

This material is intended for UK healthcare professionals only. This symposium is organised and funded by Novartis Pharmaceuticals.

UK | NOV 2024 | FA-11311513

Disclosures

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Dr Anne-Marie Childs

- Received professional fees from Novartis, Biogen, Roche, PTC Therapeutics, Santhera, Sarepta
- Received complimentary registration, travel bursary and accommodation from Novartis to the World Muscle Society 2024 Conference, Prague, Czech Republic
- Paediatric clinical expert for NHSE panel advising on disease modifying treatments under Managed Access Agreement
- Trustee of SMA UK

Objectives

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- Overview of normal developmental milestones
- Motor skills in the first few years
  - Tone, posture and movement
- What does ‘abnormal’ look like?
- What should trigger a referral?
- Spinal muscular atrophy
  - Importance of early diagnosis

Opportunities to ‘pick up’ abnormalities

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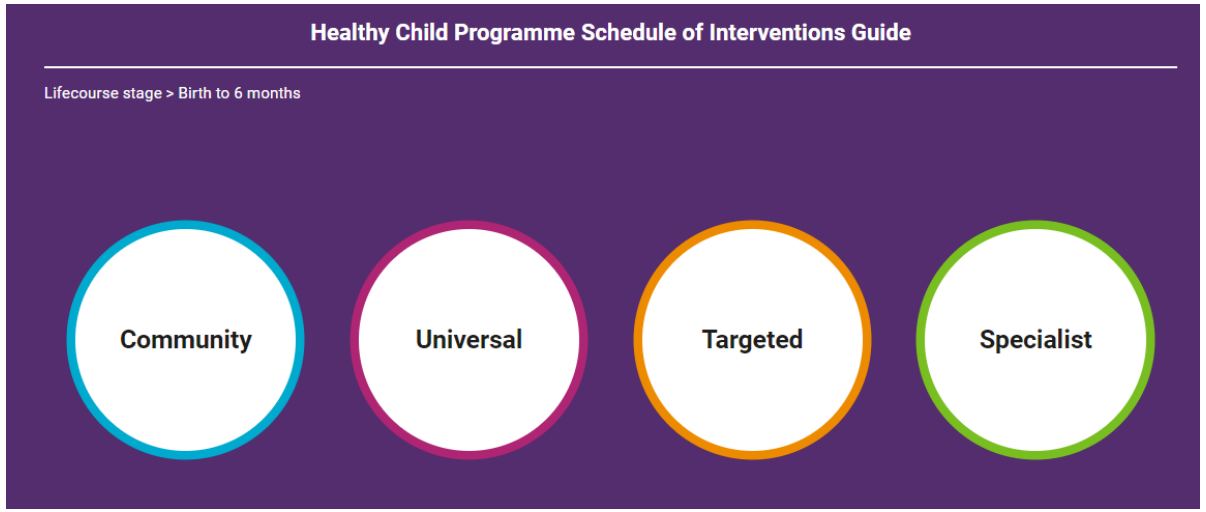
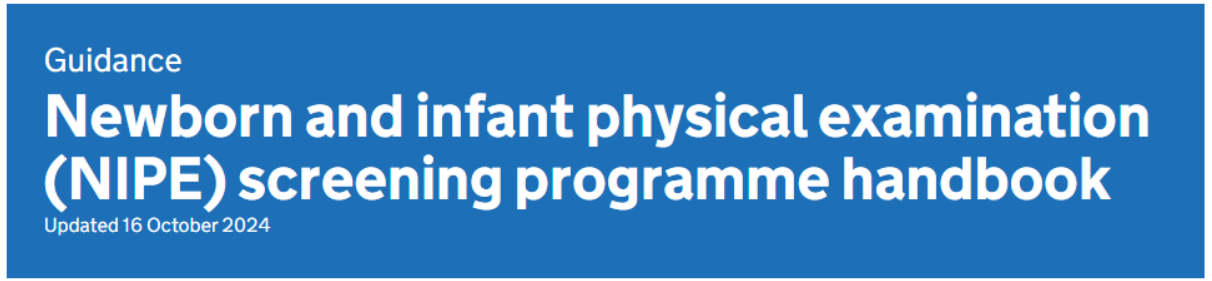
- Scheduled assessments
- Opportunistic visits

- NIPE screening (birth and 6-8 weeks)
  - Focus on tone and posture
  - Observation of movement

- 3-months to 5-years
  - Motor skills and power
  - Tone and posture





NIPE, newborn and infant physical examination.

References: 1. Office for Health Improvement and Disparities. Healthy Child Programme Schedule of Interventions Guide. Birth to 6 months. Available at: https://www.e-lfh.org.uk/pathways-healthy-child/birth-to-6-months/index.html. Date accessed. November 2024. **2.** NHS England. Guidance, Newborn and infant physical examination (NIPE) screening programme handbook. October 2024. Available at: https://www.gov.uk/government/publications/newborn-and-infant-physical-examination-programme-handbook/newborn-and-infant-physical-examination-screening-programme-handbook. Date accessed: November 2024.

Information on this slide provided by speaker.

Typical age of development of infant motor milestones[1]

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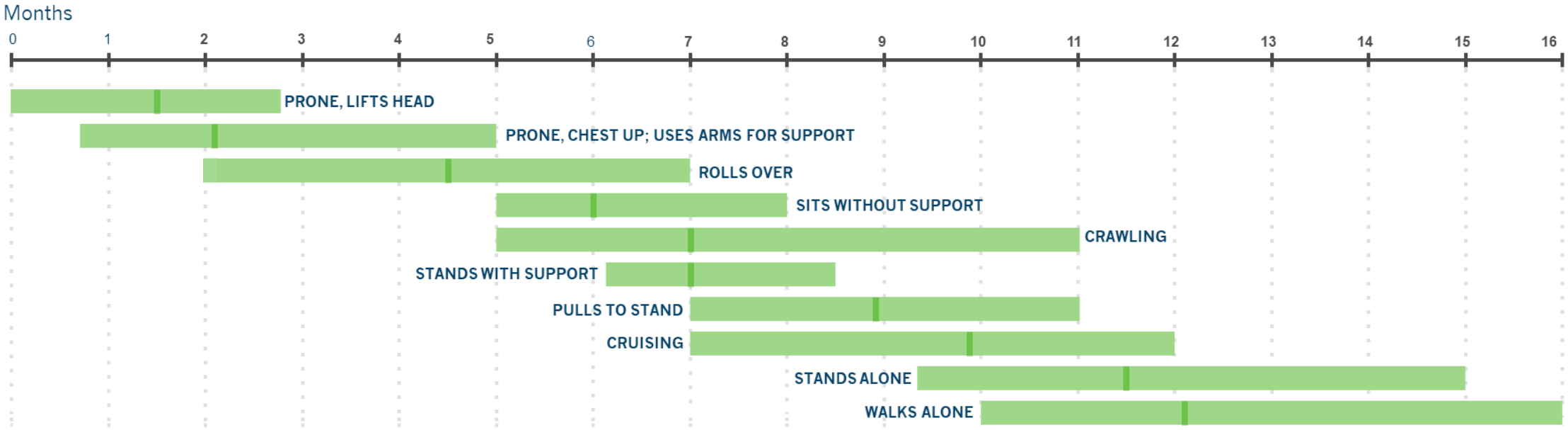

Vertical lines represent average ages of attainment; bars represent the normative range

Adapted from Adolph, *et al.* 2010.

SMA, spinal muscular atrophy.

Reference: 1. Adolph KE, *et al. Motor skills*. In: Bornstein, MH., editor. Handbook of cultural development science. Vol. 1. Domains of development across cultures. New York, NY: Taylor and Francis; 2010. p. 61-88.

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Motor assessment at different ages

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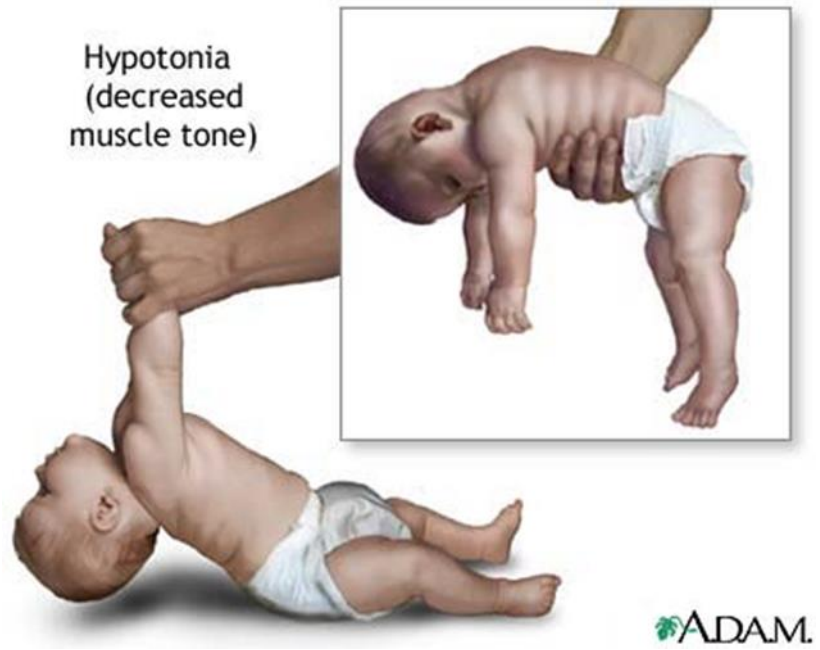
Movement

- Passive and active
- Facial movements
- Breathing pattern and chest shape



Hypotonia = floppy baby[1]

- "Decreased resistance to passive joint movement"[2]
- Subjective
- Reduction in spontaneous movements does NOT = weakness[2]
- Often associated joint hypermobility/laxity[3]

References: 1. Adam. Hypotonia. December 2023. Available at: https://ssl.adam.com/content.aspx?productid=117&pid=1&gid=003298&site=makatimed.adam.com&login=MAKA1603. Date accessed: November 2024. **2.** Sparks, SE. *Clin Perinatol.* 2015;42(2):363-71. **3.** De Santos-Moreno MG, *et al. Pediatr Neurol*. 2023;138:107-117.
Information on this slide provided by speaker.

Infantile hypotonia – easy tests

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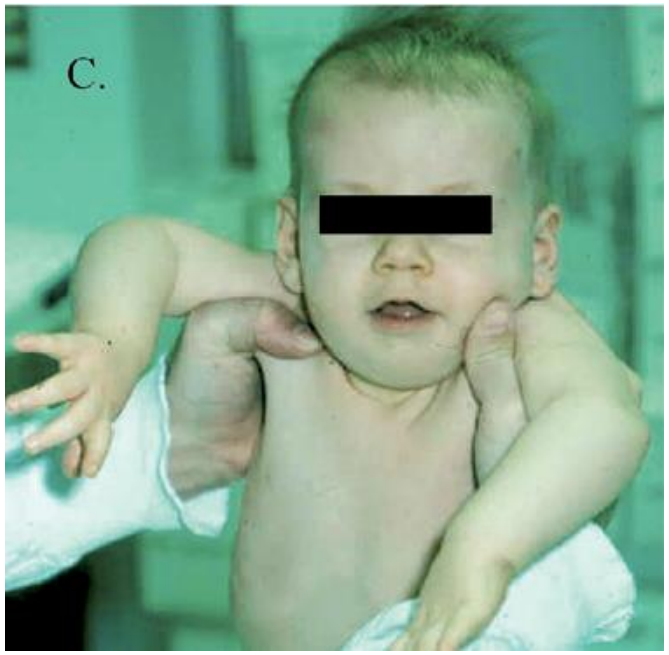
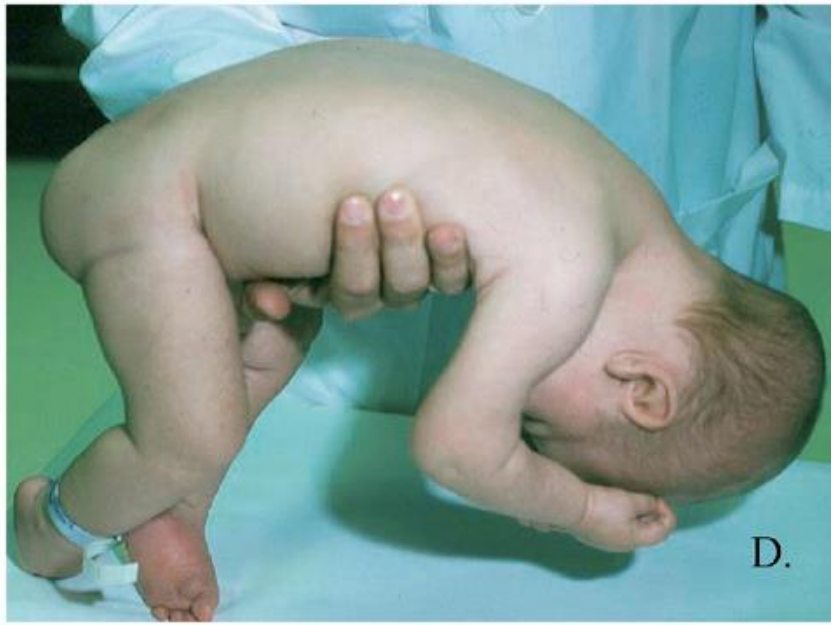
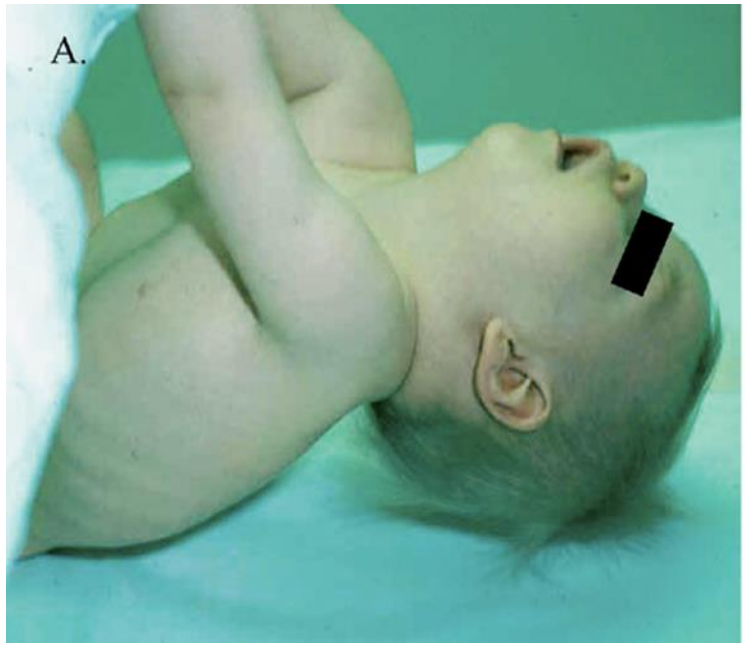


Shoulder/axillary suspension
Hypotonic infant requires significant support otherwise would ‘slip through’



Ventral suspension
Hypotonic baby will droop loosely over the palm with limbs dangling



Pull to sit
Significant head lag in the hypotonic infant
Infant may try to counter this manoeuvre by flexing arms

References: 1. Sparks E. Neonatal Hypotonia [Review]. Clinics in Perinatology. 42(2): 363-71. ix, 2015. **2.** Bodensteiner JB. The Evaluation of the Hypotonic Infant. Seminars in Pediatric Neurology. 15(1): 10-20, 2008.

Abnormal motor development

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- Easier to detect when children are older
  - More is expected so discrepancies are obvious
  - But… time is critical for some disorders
- Global delay vs motor delay
  - Early infancy main skills are motor
  - Is baby smiling, fixing and following?
- Look at other motor skills
  - Feeding
  - Breathing
- Most floppy babies do not have an underlying NM disorder
  - 60-80% central 15-30% peripheral or neuromuscular condition[1]
  - In practice **weakness** = neuromuscular condition

NM, neuromuscular.

Reference: 1. Kaur J, Punia S. *Int J Physiother Res*. 2016;4(3):1554-63.

Information on this slide provided by speaker.

Signs to be closely monitored[1,2]

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1 Head lag

- Baby cannot support their own head when picked up
- When baby is lying on their tummy, baby is not able to lift their head up, or can only do so for a very short period of time

2 Hypotonia

- When held, baby feels limp and shows an inability to move arms, legs and neck
- Baby displays little or no brace in their arms resulting in the shoulders pulling forward and no bend in their hips

3 Limited arm & leg movements

- Baby is unable to lift arms or legs or reach for objects
- E.g. when baby is lying on its back, baby is not reaching when a toy or object is held above them

References: 1. Hadders-Algra M. *Neuroscience and Biobehavioral Reviews*. 2018(90):411-427. **2.** Cambridgeshire Community Services NHS Trust. Child Development milestones. Available at: https://www.cambscommunityservices.nhs.uk/advice/childhood-development/milestones. Date accessed: November 2024.

SMA is a rare and devastating neuromuscular disease[1]

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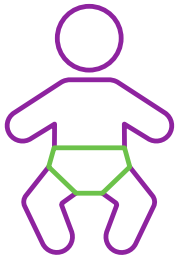
~1 in 10,000
live births affected with ~58% of cases being SMA Type 1[2,3]



Approximately **1 in 40 to 60 people** is an SMA carrier[4]

SMA is the 2nd most common autosomal recessive disorder after cystic fibrosis[2]

SMA is characterised by:[1]

- Degeneration of lower motor neurons in the spinal cord
- Progressive muscle wasting
- Loss of mobility/motor function

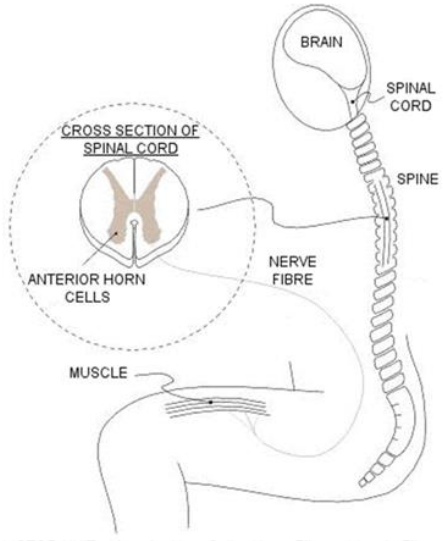


Image from The Jennifer Trust for Spinal Muscular Atrophy[5]

SMA is a progressive monogenic disease, usually occurring in childhood, caused by a missing or dysfunctional *SMN1* gene[6,7]

SMA, spinal muscular atrophy; SMN, survival motor neuron.

References: 1. Lin CW, *et al. Ped Neurol*. 2015;53:293–300. **2.** Armstrong EP, *et al. J Med Econ.* 2016;19:822–6. **3.** Lally C, *et al. Orphanet Journal of Rare Diseases*. 2017;12:175. **4.** SMA UK (2023). Summary information about SMA. Available at: https://smauk.org.uk/support-information/about-sma/sma-summary/. Date accessed: November 2024. **5.** The Jennifer Trust for Spinal Muscular Atrophy. Available at: http://www.daneverard.co.uk/dan/jtsma/sma/sma0.html. Date accessed: November 2024. **6.** Mendell JR, *et al. N Engl J Med*. 2017;377:1713–22. **7.** Finkel RS, *et al. N Engl J Med*. 2017;377:1723-32.

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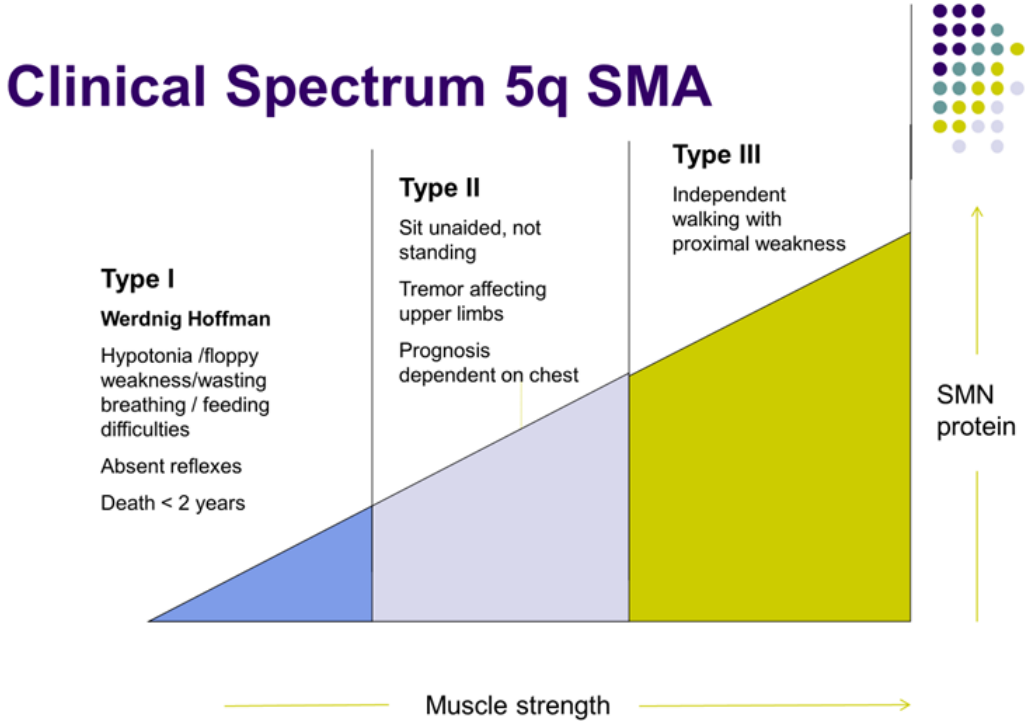

Image provided by speaker

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SMA, spinal muscular atrophy; SMN, survival motor neuron.

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Fast diagnosis is vital in SMA Type 1 as damage that occurs before treatment is irreversible[1]

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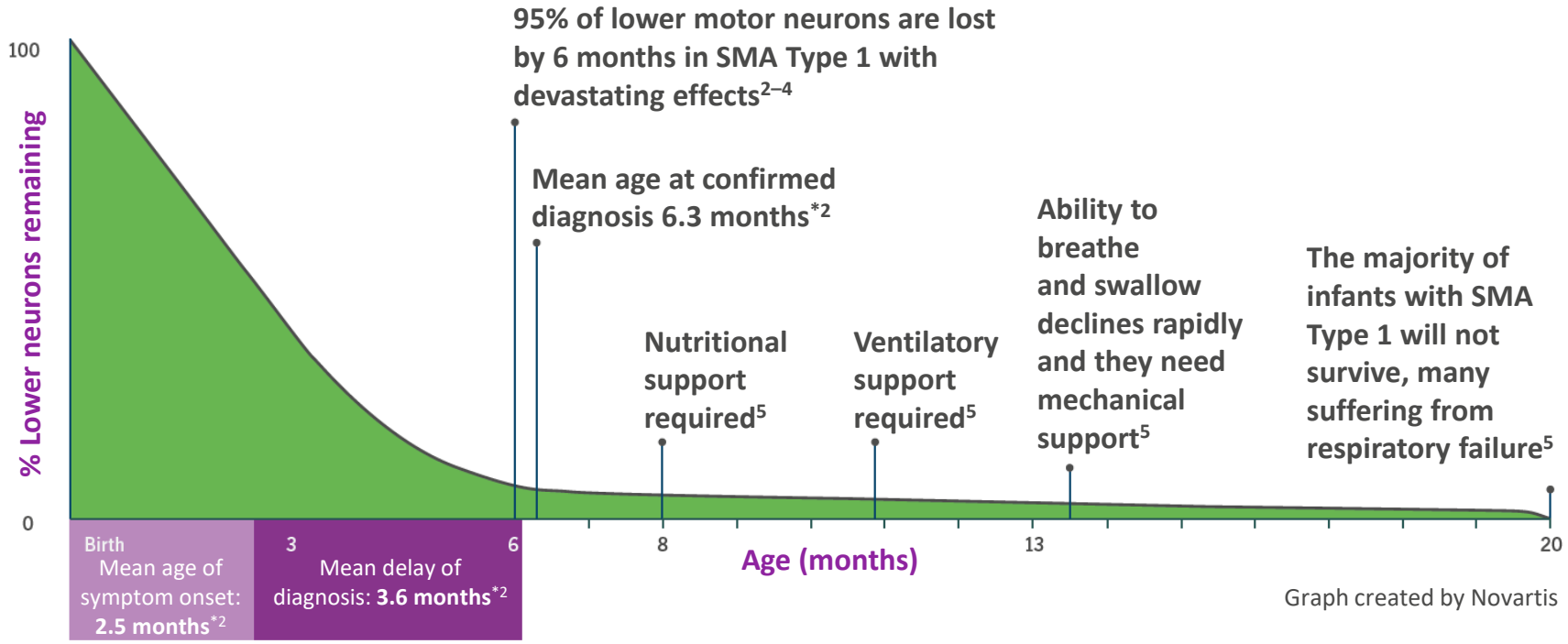

Graph created by Novartis

- 95% of lower motor neurons are lost within 6 months in SMA Type 1, with devastating effects[2–4]
- Delayed diagnosis enables continued neurodegeneration[4]

Early diagnosis and treatment is critical to modulate the rapid and progressive neurodegeneration seen in SMA Type 1[4]

SMA, spinal muscular atrophy.

*Based on the results of a systematic literature review of 21 studies published between 2000 and 2014. Mean age of onset, diagnosis and delay in diagnosis was extracted or calculated. All estimates were weighted by the number of patients and descriptive statistics reported. The weighted mean (standard deviation) ages of onset was 2.5 (0.6) months for SMA Type 1, and the weighted mean (standard deviation) age of confirmed SMA genetic diagnosis was 6.3 (2.2) months. The mean delay in diagnosis was 3.6 months.

References: 1. Anderton RS and Mastaglia FL. *Expert Rev Neurother.* 2015;15:895–908. **2.** Lin CW, *et al. Ped Neurol*. 2015;53:293–300. **3.** Govoni A, *et al. Mol Neurobiol.* 2018;55(8):6307–18. **4.** Glascock J, *et al. J Neuromusc Dis.* 2018;5:145–58. **5.** Finkel RS, *et al. Neurology*. 2014;83(9):810–7.

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Fast diagnosis is vital in SMA Type 1 as damage that occurs before treatment is irreversible[1]

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2024: 3 disease-modifying drugs for use in specific conditions for SMA available in the UK

- **Gene Therapy: Onasemnogene abeparvovec (ZOLGENSMA) in SMA 1**
  - **Symptomatic < 12 months (7-12m needs MDT approval)[1]**
  - **Presymptomatic: *SMN1* deletion with up to 3 copies of *SMN2*[2]**
- ***SMN2* modifying drugs: Managed Access Agreements**
  - **Nusinersen (Spinraza): Intermittent intrathecal delivery[3]**
  - **Risdiplam: daily oral medication[4]**

Longer term data from trials available

All have potential pros and cons for individual patients

Cannot rescue irreversibly damaged nerve cells

Please see individual product SmPCs prior to prescribing

MDT, multidisciplinary team; SMA, spinal muscular atrophy; SMN, survival motor neuron.

References: 1. NICE HST15 2023. Onasemnogene abeparvovec for treating spinal muscular atrophy. Available at: https://www.nice.org.uk/guidance/hst15. Date accessed: November 2024. **2.** NICE HST24 2023. Onasemnogene abeparvovec for treating presymptomatic spinal muscular atrophy. Available at: www.nice.org.uk/guidance/hst24. Date accessed: November 2024. **3.** NICE TA588 2019. Nusinersen for treating spinal muscular atrophy. Available at: www.nice.org.uk/guidance/ta588. Date accessed: November 2024. **4.** NICE TA755 2023. Risdiplam for treating spinal muscular atrophy. Available at: www.nice.org.uk/guidance/ta755. Date accessed: November 2024.

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Fast diagnosis is vital in SMA Type 1 as damage that occurs before treatment is irreversible[1]

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NICE recommendations for onasemnogene abeparvovec

Onasemnogene abeparvovec is recommended as an option for treating 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the *SMN1* gene and a clinical diagnosis of type 1 SMA in babies, only if:[1]

- they are 6 months or younger, or
- they are aged 7 to 12 months, and their treatment is agreed by the national multidisciplinary team

It is only recommended for these groups if:[1]

- permanent ventilation for more than 16 hours per day or a tracheostomy is not needed
- the company provides it according to the commercial arrangement.

For babies aged 7 to 12 months, the national multidisciplinary team should develop auditable criteria to enable onasemnogene abeparvovec to be allocated to babies in whom treatment will give them at least a 70% chance of being able to sit independently.[1]

Onasemnogene abeparvovec is recommended as an option for treating presymptomatic 5q spinal muscular atrophy (SMA) with a biallelic mutation in the *SMN1* gene and up to 3 copies of the *SMN2* gene in babies aged 12 months and under. It is only recommended if the company provides it according to the commercial arrangement.[2]

Please see individual product SmPCs prior to prescribing

SMA, spinal muscular atrophy; SMN, survival motor neuron.

References: 1. NICE HST15 2023. Onasemnogene abeparvovec for treating spinal muscular atrophy. Available at: https://www.nice.org.uk/guidance/hst15. Date accessed: November 2024. **2.** NICE HST24 2023. Onasemnogene abeparvovec for treating presymptomatic spinal muscular atrophy. Available at: www.nice.org.uk/guidance/hst24. Date accessed: November 2024.

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ZOLGENSMA shows durable efficacy through continued milestone achievement up to 7.5 years post-dosing*[1]

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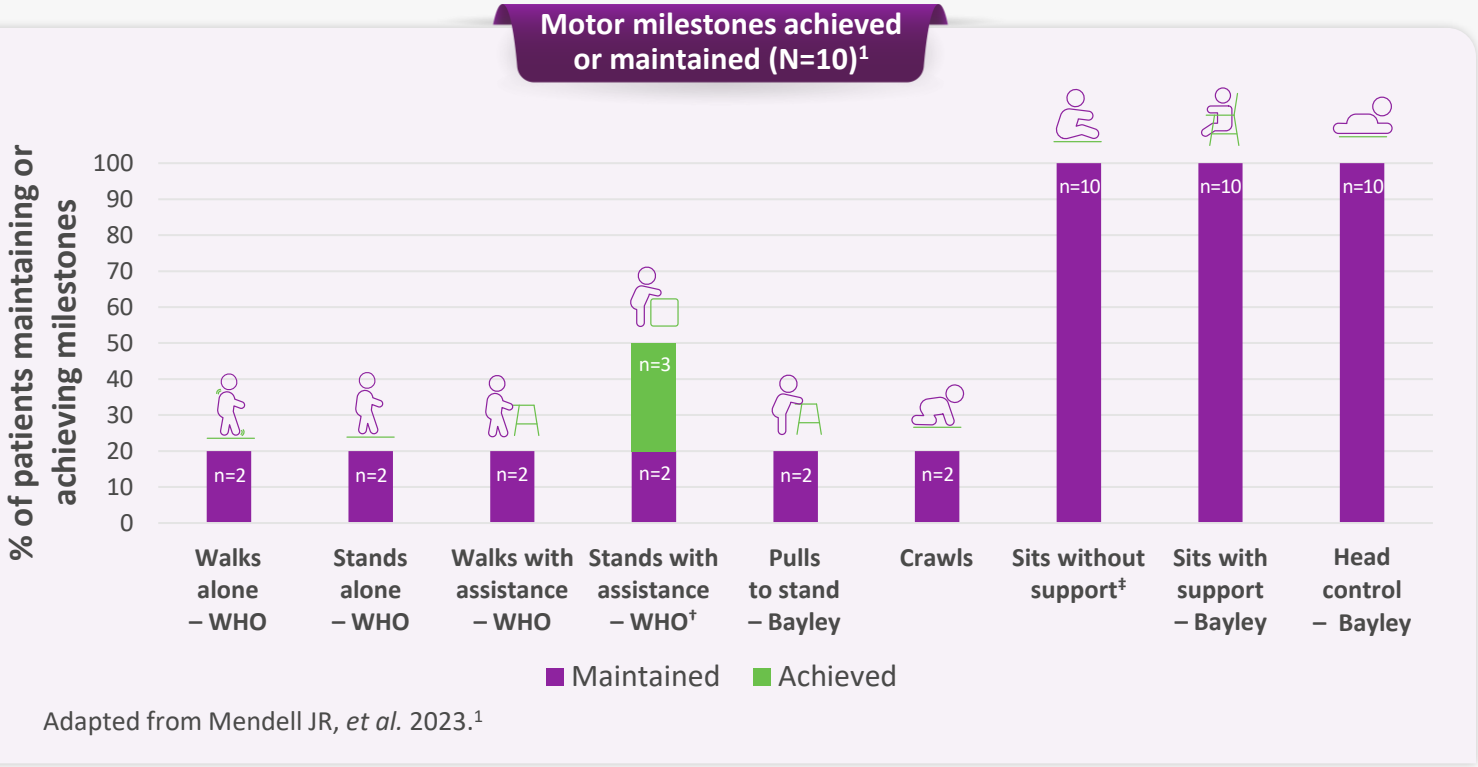

Adapted from Mendell JR, *et al.* 2023.[1]

100% (10/10) of patients who received the therapeutic dose either **maintained all previously attained milestones or gained new milestones§[1]**

- **60% (6/10)** of patients **received add-on therapy** (nusinersen and/or risdiplam); five patients are still receiving ongoing add-on therapy. **All six patients maintained achieved milestones** and **one patient achieved a new milestone following the addition of nusinersen[1]**
- **20%** (2/10) of patients who received the therapeutic dose have **newly achieved standing with assistance without add-on therapy[1]**

WHO, World Health Organization; WHO-MGRS, World Health Organization Multicentre Growth Reference Study.
*Reported as of May 23, 2022.[1] †Two patients achieved standing with assistance during the START study and three patients newly achieved the milestone during the LT-001 study.[1] ‡Prior to June 17, 2022, sitting without support was based on the Bayley Scales of Infant Development (sitting alone without support for at least 30 seconds). Starting on June 17, 2022, sitting without support was based on the WHO-MGRS definition (child sits up straight with head erect for ≥10 seconds without using arms or hands for balance or support). In the parent study, sits alone ≥5 seconds, sits alone ≥15 seconds, and sits alone ≥30 seconds are considered as sitting without support. The graph includes both video-confirmed and non-video-confirmed data.[1]

Reference: 1. Mendell JR, *et al.* Long-Term Follow-Up of Onasemnogene Abeparvovec Gene Therapy in Symptomatic Patients with Spinal Muscular Atrophy Type 1. Poster presented at: 2023 MDA Clinical and Scientific Conference. March 19–22, 2023.

Adverse events observed across 5 open-label ZOLGENSMA clinical studies were consistent with the adverse event profile[1]

zolgensma® (onasemnogene abeparvovec)

The safety profile of ZOLGENSMA was evaluated in 99 patients who received ZOLGENSMA at the recommended dose (1.1 x 10^{14} vg/kg) in 5 open-label clinical studies

The most frequently reported adverse reactions following administration were:

- Hepatic enzyme increased (24.2%)
- Hepatotoxicity (9.1%)
- Vomiting (8.1%)
- Thrombocytopenia (6.1%)
- Troponin increased (5.1%)
- Pyrexia (5.1%)

Please see the SmPC for more information regarding adverse events and special warnings and precautions for use

SmPC, Summary of Product Characteristics.

Reference: 1. Zolgensma Summary of Product Characteristics.

Case Study: Essa

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- 3rd child of 1st cousin parents
- 1st child born in 2014, died at 8-months-old
- 2nd child healthy girl now 3-years-old

- Born at term in 2019, planned LSCS
- Active and moving all 4 limbs at birth
- Presented at 6 days
- Treated at 21 days

- Confirmed *SMN1* deletion
- Diagnosis: SMA type 1

Individual patient responses will vary

LSCS, lower segment Caesarean section; SMA, spinal muscular atrophy; SMN, survival motor neuron.
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Progress at 3.5 years

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- Continues to gain skills
- Sitting securely, can pivot
- Stands with support at trunk
- Fed via PEG
- NIV at night, stopped and restarted with infection
- Cognitive concerns

- Brother born in June 2023 - cord blood tested: *SMN1* deletion
- Started treatment day 5 of life
- Walking at 15 months
- Orally fed, no breathing difficulties

- Days make a difference:
  - Good HV awareness
  - Newborn screening

Individual patient responses will vary

HV, Health Visitor; NIV, non-invasive ventilation; PEG, percutaneous endoscopic gastrostomy; SMA, spinal muscular atrophy; SMN, survival motor neuron.
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Signs to be closely monitored[1,2]

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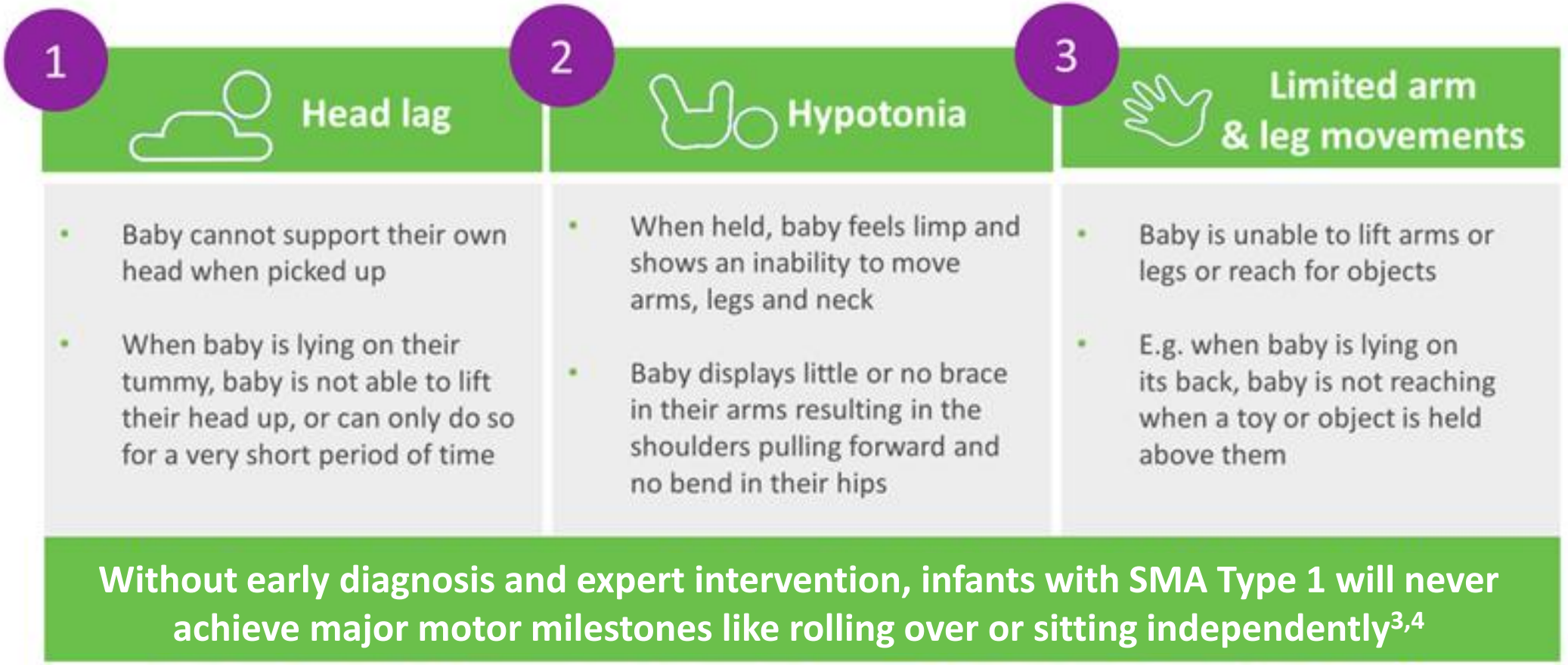


SMA, spinal muscular atrophy.

References: 1. Hadders-Algra M. *Neuroscience and Biobehavioral Reviews*. 2018(90):411-427. **2.** Cambridgeshire Community Services NHS Trust. Child Development milestones. Available at: https://www.cambscommunityservices.nhs.uk/advice/childhood-development/milestones. Date accessed: November 2024. **3.** De Sanctis R, *et al. Neuromusc Disord*. 2016;26(11):754–9. **4.** Mendell J, *et al. N Engl J Med*. 2017;377(18):1713–22.

Red flags for onward referral

- Motor delay >>> Other developmental milestones
- Hypotonia AND weakness
  - Doesn’t lift arms or legs off the bed? Can kick in the bath

- Feeding difficulties
  - Choking
  - Poor weight gain

- Breathing difficulties
  - Weak cry
  - Weak cough
  - Abnormal chest shape/pattern of breathing

- WEAKNESS + FEEDING and/or breathing difficulties = **URGENT referral**

Information on this slide provided by speaker.

Targeted messages on the early signs

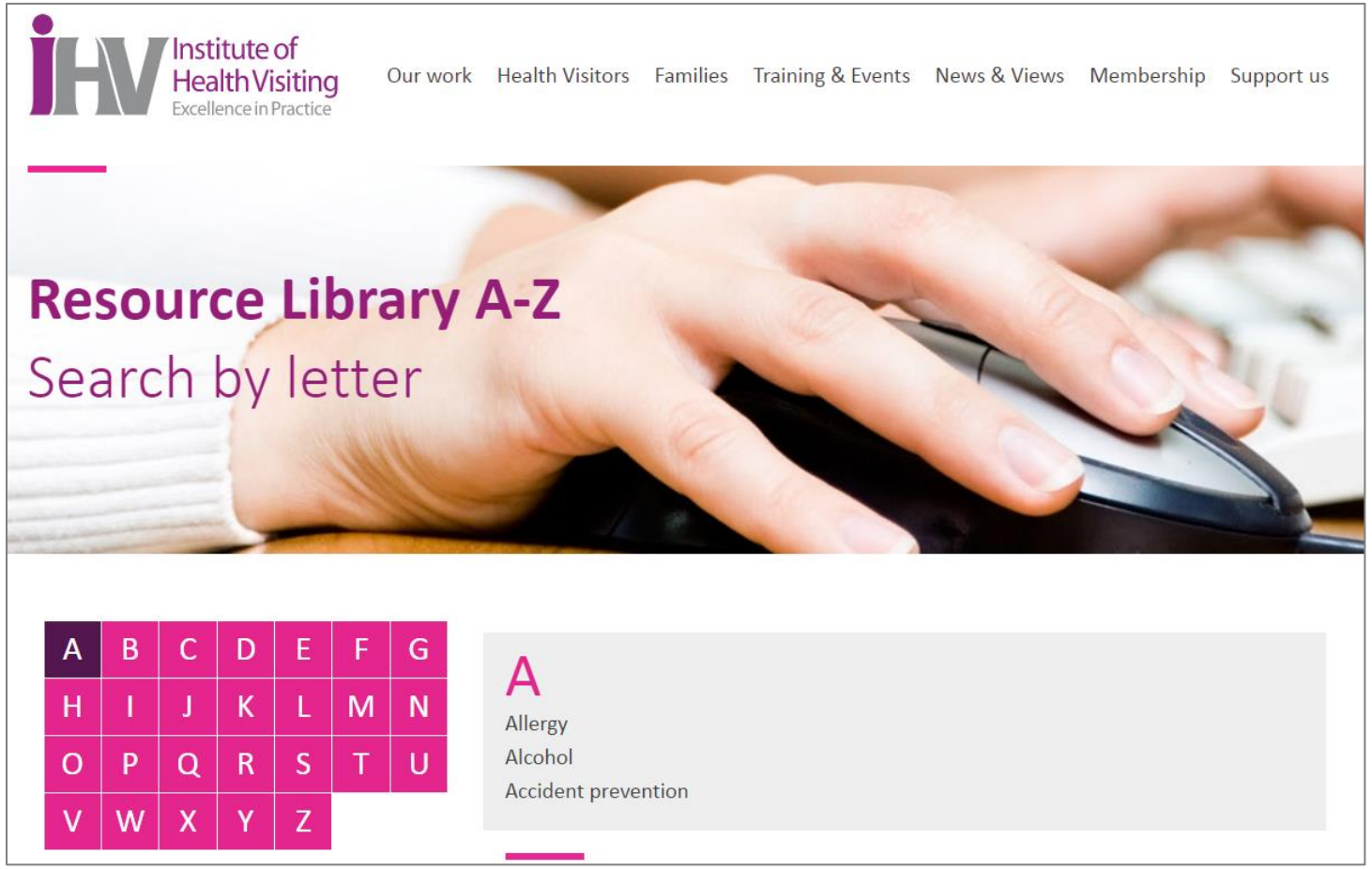
zolgensma® (onasemnogene abeparvovec)

Audience	**Paeds & GPs**	**Nurses & HVs**	**Parents**
Messaging	**Think SMA** • Head lag • Hypotonia • Inability to reach	**Think NMD** • Head lag • Hypotonia • Inability to reach	**Think movement** • Cannot raise their head on tummy • Floppy arms and legs • Is not reaching for things
Action	**Refer to Paediatrician**	**Refer to GP**	**Book a GP appt**

GP, general practitioner; HV, Health Visitor; NMD, neuromuscular disease; SMA, spinal muscular atrophy.
Information on this slide provided by speaker.

SMA Good Practice Points: Download today from the iHV Resource Library[1]

zolgensma® (onasemnogene abeparvovec)



- Visit https://ihv.org.uk/for-health-visitors/resources/good-practice-points/ to access all iHV Good Practice Points
- Free of charge for iHV members
- Parent Tips for Spinal Muscular Atrophy Type 1: Spotting the signs & symptoms also available on the iHV website[2]

Novartis has no control over this website

iHV; Institute of Health Visiting; SMA, spinal muscular atrophy.

References: 1. Institute of Health Visiting. The vital role of health visitors in supporting families with SMA type 1. September 2021. Available at: https://ihv.org.uk/for-health-visitors/resources-for-members/resource/good-practice-points/health-wellbeing-and-development-of-the-child/gpp-identifying-referring-and-supporting-infants-with-spinal-muscular-atrophy-type-1/. Date accessed: November 2024. **2.** Institute of Health Visiting. PT – Spinal Muscular Atrophy Type 1: Spotting the signs & symptoms. Available at: https://ihv.org.uk/for-health-visitors/resources-for-members/resource/pt-spinal-muscular-atrophy-type-1-spotting-the-signs-symptoms/. Date accessed: November 2024.

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SPINAL MUSCULAR ATROPHY (SMA) IS NOW A TREATABLE CONDITION

NORTH WEST Neuromuscular Network

Diagnostic Clues

- Hypotonic baby who is alert but does not kick legs
- Has difficulty raising arms when held upright (see overleaf for more resources)

Effective treatments are now available for SMA

Early treatment leads to better outcome

There's no time to "watch and wait" SMA is now a "time critical diagnosis"

Early diagnosis is now essential as progression of weakness may be very rapid.

Previously SMA1 was the most common genetic cause of infant death

If you suspect a child has SMA please contact your local Neuromuscular Team ASAP
Paediatricians - Arrange genetic testing R70 and contact lab to expedite

NM team contact details: RMCH 01617010679 nm.team@mft.nhs.uk, AHCH neuromuscularnurses@alderhey.co.uk 07966308957

Printing Supported by Novartis

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EDUCATIONAL RESOURCES

Institute of Health Visiting	Think3 of SMA	Royal College of Paediatrics and Child Health
ihv.org.uk	think3sma.co.uk	rcpch.ac.uk

Novartis has no control over the iHV and RCPCH websites. Think 3 for SMA is a campaign initiated and funded by Novartis

SMA, spinal muscular atrophy.
Information on this slide provided by speaker.

Additional resources

zolgensma® (onasemnogene abeparvovec)

Further reading:

www.nhs.uk/conditions/spinal-muscular-atrophy-sma

The below organisations can provide advice and support:

- Muscular Dystrophy UK: 0800 652 6352
- Spinal Muscular Atrophy UK: 01789 267520
- Treat SMA: 0300 800 0202

If a child receives an SMA diagnosis, parents can register for the UK SMA patient registry at: sma-registry.org.uk

SMA, spinal muscular atrophy.
Information on this slide provided by speaker.

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THANK YOU

zolgensma® (onasemnogene abeparvovec)

Prescribing Information – Great Britain

zolgensma® (onasemnogene abeparvovec)

ZOLGENSMA® ▼ 2 × 10^{13} VECTOR GENOMES/ML SOLUTION FOR INFUSION (ONASEMNOGENE ABEPARVOVEC)

Important note: Before prescribing, consult Summary of Product Characteristics (SmPC).

Presentation: Each mL contains 2 × 10^{13} vector genomes (vg) of onasemnogene abeparvovec. Vials contain an extractable volume of not less than either 5.5 mL or 8.3 mL.

Indication(s): Zolgensma is indicated for the treatment of patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or, patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.

Dosage and administration: Treatment should be initiated and administered in clinical centres and supervised by a physician experienced in the management of patients with SMA. Refer to full SmPC for full information on posology and administration. Prior to Zolgensma infusion, baseline laboratory testing, including but not limited to: AAV9 antibody testing, liver function, creatinine, complete blood count and troponin-I should be conducted. Due to the increased risk of serious systemic immune response, it is recommended that patients are clinically stable in their overall health status (e.g. hydration and nutritional status, absence of infection) prior to infusion. Treatment should be postponed in patients with acute or chronic active uncontrolled infections until the infection has resolved and the patient is clinically stable (see SmPC Section 4.2 and 4.4). *Zolgensma recommended dosage:* Patients will receive a dose of nominal 1.1 x 10^{14} vg/kg onasemnogene abeparvovec. The total volume is determined by patient body weight (see SmPC section 4.2, Table 1 for recommended dosing based on patient body weight). *Concomitant corticosteroid recommended dosage:* An immune response to the AAV9 capsid will occur following Zolgensma treatment therefore it is recommended to administer concomitant corticosteroid prior to and following Zolgensma infusion to dampen the immune response (see SmPC section 4.2, Table 2 for recommended dosing). Prior to initiation of the immunomodulatory regimen and prior to administration of onasemnogene abeparvovec, the patient must be checked for signs and symptoms of active infectious disease of any nature. *Renal impairment:* The safety and efficacy of Zolgensma have not been established in patients with renal impairment, and therefore treatment with Zolgensma should be carefully considered. A dose adjustment should not be considered. *Hepatic impairment:* Zolgensma therapy should be carefully considered. A dose adjustment should not be considered. *0SMN1/1SMN2 genotype:* No dose adjustment should be considered in patients with a bi-allelic mutation of the SMN1 gene and only one copy of SMN2. *Anti-AAV9 antibodies:* No dose adjustment should be considered in patients with baseline anti-AAV9 antibody titres above 1:50. *Paediatric population:* Zolgensma treatment should be carefully considered in premature neonates as the safety and efficacy of Zolgensma have not been established and no data are available. Administration of Zolgensma should be carefully considered as concomitant treatment with corticosteroids may adversely affect neurological development. There is limited experience in patients 2 years of age and older or with body weight above 13.5 kg. Dose adjustment should not be considered – refer to SmPC for dosing table. *Method of administration:* Zolgensma is administered as a single-dose intravenous infusion. It should be administered as a slow infusion over 60 minutes. Do not infuse as an intravenous push or bolus. Please refer to the SmPC for detailed guidance on preparation, handling, accidental exposure and disposal (including proper handling of bodily waste) of Zolgensma.

Contraindications: Hypersensitivity to active substance or any excipient listed in SmPC section 6.1.

Warnings/Precautions: *Traceability:* Name and the batch number of the administered product should be clearly recorded for each patient. *Pre-existing immunity against AAV9:* Patients should be tested for the presence of AAV9 antibodies prior to Zolgensma treatment. Retesting may be performed if AAV9 antibody titres are reported as above 1:50. *Advanced SMA:* While advanced symptomatic SMA patients will not achieve the same gross motor development as unaffected healthy peers, they may clinically benefit from gene replacement therapy, dependent on the advancement of disease at the time of treatment. The benefit of onasemnogene abeparvovec is seriously reduced in patients with profound muscle weakness and respiratory failure, patients on permanent ventilation, and patients not able to swallow. The benefit/risk profile of onasemnogene abeparvovec in patients with advanced SMA, kept alive through permanent ventilation and without the ability to thrive, is not established. *Immunogenicity:* An immune response to the AAV9 capsid will occur after infusion of Zolgensma, including antibody formation against the AAV9 capsid and T-cell mediated immune response. *Hepatotoxicity:* Acute serious liver injury and acute liver failure, including fatal cases have been reported with Zolgensma, typically within 2 months after infusion and despite receiving corticosteroids before and after infusion. Immune mediated hepatotoxicity may require adjustment of the immunomodulatory regimen including longer duration, increased dose, or prolongation of the corticosteroid taper. The risks and benefits of onasemnogene abeparvovec therapy should be carefully considered in patients with pre-existing hepatic impairment. Patients with pre-existing hepatic impairment or acute hepatic viral infection may be at higher risk of acute serious liver injury. Data from a small study in children weighing ≥8.5 kg to ≤21 kg (aged approximately 1.5 to 9 years), indicate a higher frequency of AST or ALT elevations (in 23 out of 24 patients) compared with frequencies of AST/ALT elevations observed in other studies in patients weighing <8.5 kg (in 31 out of 99 patients) (see SmPC section 4.8). Zolgensma treatment often results in aminotransferase elevations. Liver function (ALT, AST, total bilirubin) should be assessed prior to Zolgensma treatment and monitored at regular intervals for at least 3 months after infusion (weekly in the first month and during the entire corticosteroid taper period, followed by every two weeks for another month), and at other times as clinically indicated. A systemic corticosteroid should be administered to all patients before and after Zolgensma infusion. Patients with worsening liver function test results and/or signs or symptoms of acute illness should be promptly clinically assessed and monitored closely. In case hepatic injury is suspected, prompt consultation with a paediatric gastroenterologist or hepatologist, adjustment of the recommended immunomodulatory regimen and further testing is recommended (e.g. albumin, prothrombin time, PTT, and INR). AST/ALT/bilirubin should be assessed weekly for the first month after infusion and during the entire corticosteroid taper period. If the patient is clinically stable with unremarkable findings at the end of the corticosteroid taper period, liver function should continue to be monitored every two weeks for another month. *Thrombocytopenia:* Transient decreases in platelet counts, including thrombocytopenia, have been reported. Platelet counts should be obtained prior to Zolgensma treatment, it should be closely monitored within the first three weeks following infusion and on a regular basis afterwards, at least weekly for the first month and every other week for the second and third months until platelet counts return to baseline. Data from a small study in children weighing ≥8.5 kg to ≤21 kg (aged approximately 1.5 to 9 years), indicate a higher frequency of thrombocytopenia (in 20 out of 24 patients) compared with frequencies of thrombocytopenia observed in other studies in patients weighing <8.5 kg (in 22 out of 99 patients) (see SmPC section 4.8). *Thrombotic microangiopathy:* Several cases of thrombotic microangiopathy (TMA) have been reported following Zolgensma treatment. Cases generally occurred within the first two weeks after onasemnogene abeparvovec infusion. Fatal outcomes have been reported. Acute kidney injury and concurrent immune system activation have also been reported. 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Disposable nappies can be sealed in double plastic bags and disposed of in household waste. *Blood, organ, tissue and cell donation:* Patients treated with Zolgensma should not donate blood, organs, tissues or cells for transplantation. *Sodium content:* Zolgensma contains 4.6 mg sodium per mL. Each 5.5 mL vial contains 25.3 mg sodium, and each 8.3 mL vial contains 38.2 mg sodium.

Interactions: Experience with use of concomitant hepatotoxic medicinal products or substances, or 5q SMA targeting agents is limited. The patient's vaccination schedule should be adjusted to accommodate concomitant corticosteroid administration prior to and following Zolgensma treatment. Live vaccines, such as MMR and varicella, should not be administered to patients on an immunosuppressive steroid dose. Please refer to the SmPC for further information on interactions.

Fertility, pregnancy and lactation: Human data on use during pregnancy or lactation are not available.

Undesirable effects: Very common (≥1/10): hepatic enzyme increase. Common (≥1/100 to <1/10): hepatotoxicity, vomiting, pyrexia, thrombocytopenia, increased troponin. Uncommon (≥1/1000 to <1/100): thrombotic microangiopathy, acute liver failure. *Other Adverse Effects:* Please consult the Summary of Product Characteristics for a detailed listing of all adverse events before prescribing.

Pack Size(s): The dose of onasemnogene abeparvovec and exact number of vials required for each patient is calculated according to the patient's weight (see section 4.2 and Table 5 SmPC)

Legal classification: POM

Marketing Authorisation (MA) number, quantities and price:

PLGB 00101/1223 - each carton will contain between 2 to 14 vials (see SmPC section 6.5, Table 5 for carton/kit configurations): £1,795,000 per single-dose treatment.

Date of last revision of prescribing information: May 2024

Full Prescribing Information available from: Novartis Pharmaceuticals UK Limited 2nd Floor, The WestWorks Building, White City Place, 195 Wood Lane, London, W12 7FQ Telephone: (01276) 692255.

Adverse Event Reporting:

Adverse events should be reported. Reporting forms and information can be found at www.mhra.gov.uk/yellowcard.

Adverse events should also be reported to Novartis via uk.patientsafety@novartis.com or online through the pharmacovigilance intake (PVI) tool at www.novartis.com/report

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Prescribing Information – Northern Ireland

zolgensma® (onasemnogene abeparvovec)

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Legal classification: POM

Marketing Authorisation Holder: Novartis Europharm Limited, Vista Building, Elm Park, Merrion Road, Dublin 4

Marketing Authorisation (MA) number, quantities and price:

EU/1/20/1443/001 - 037 - each carton will contain between 2 to 14 vials (see SmPC section 6.5, Table 5 for carton/kit configurations): £1,795,000 per single-dose treatment.

Date of last revision of prescribing information: April 2024

Full Prescribing Information available from: Novartis Europharm Limited, Vista Building, Elm Park, Merrion Road, Dublin 4, Ireland. Tel: 01-2601255. Detailed information on this product is also available on the website of the European Medicines Agency http://www.ema.europa.eu

Adverse Event Reporting:

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Adverse events should also be reported to Novartis via uk.patientsafety@novartis.com or online through the pharmacovigilance intake (PVI) tool at www.novartis.com/report

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Next speaker

Pilar Cloud

Chief Executive, Action Cerebral Palsy

Type your questions as you think of them during the presentations

Use the Zoom Q&A function

- **Name of presenter** you would like to answer your question
- Brief **question**
- **Your name** (if you wish to do so)

Understanding Cerebral Palsy: Risk Factors, Spotting the Signs, and Early Intervention

21 November 2024

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT
Registered Charity Number: 1165217

About Action Cerebral Palsy

A campaigning charity who works to improve public, professional and political understanding of cerebral palsy and the issues affecting children and young people with the condition and their families.

Our vision is that every child and young person in the UK with cerebral palsies is able to access from birth onwards the best possible intervention, care, education and support which meets their complex and changing needs

Introductions:

Pilar Cloud, CEO – pilar.cloud@actioncp.org

Amanda Richardson MBE, Founder and Director of Policy - Amanda.Richardson@actioncp.org

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT
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Cerebral palsy is an “umbrella” term that is used to describe a range of **movement disorders** which can have an impact on many aspects of normal childhood development **and is a lifelong condition**. It is sometimes referred to as “the cerebral palsies” or CP. It happens as a result of interference or damage to the development of a baby’s central nervous system (the brain and spinal cord) between conception and 3 years of age.

- Cerebral palsy (CP) is a **lifelong condition** and the most common physical disability in childhood.
- It affects approximately **1:400 children** with approximately 1,800 children diagnosed each year
- There are approximately **30,000 children in the UK** living with cerebral palsy
- **CP primarily affects muscle tone and depending on the type of CP, the ability to make smooth purposeful movements across all muscle groups in the body. It also affects the ability to register, process and regulate sensory information.**
- **1:3 with the condition will be unable to walk; 1:4 are unable to talk; 1:2 has intellectual impairment**
- Cerebral palsy is 3 times more common than Downs syndrome and similar in population to multiple sclerosis and Parkinson’s disease

For more information about Cerebral Palsy, please see:

- Action Cerebral Palsy | What is cerebral palsy? (actioncp.org)
- Cerebral palsy - NHS (www.nhs.uk)
- Gross Motor Function Classification System (GMFCS) - Cerebral Palsy Alliance
- https://www.youtube.com/watch?v=MlhgsM1Sbck

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Early Motor Development and Warning Signs

- Approximately 50% of the babies and very young children who are diagnosed with cerebral palsy will not be known or believed to be at risk from birth.
- Too often, these babies at risk go undetected because parents, early education professionals and first line health practitioners are unaware of the signs of cerebral palsy.
- Children at risk require assessment and intervention at the earliest possible opportunity in order to harness brain plasticity and reduce the risk of developmental deterioration.

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT

Risk Factors

Antenatal

- Interruption of the blood flow or oxygen to the baby's brain before, during or after birth
- Low birth weight
- Prematurity
- Maternal disease, infection or pre-eclampsia during pregnancy
- Hemorrhages (blood loss during pregnancy or in baby after birth)
- Toxins passed to the baby during pregnancy, for example tobacco, alcohol, prohibited drugs
- Heart/respiratory distress
- Blood group incompatibility
- Multiple births
- Maternal history of miscarriage or still birth
- Genetic predisposition

Post Natal

- Infection affecting the brain/CNS, e.g. meningitis
- Stroke
- Accidental or non-accidental brain injury in baby

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Child is not meeting typical motor developmental milestones.	Child feels floppy, stiff or a combination of both.	Child has jerky movements or difficulty controlling their own movement.
Child is not kicking legs or moving arms and legs when lying down, or hands finding feet	Child is not bringing hands together.	Child is using one side of their body a lot less than the other.
Child is distressed by or not reacting to movement, touch, sound or smell.	Child is not making eye contact or following movements with their head and eyes.	Child has difficulty sucking, feeding or swallowing.

Download Posters and Parent Information Leaflets

www.actioncp.org/signs

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT

Parent Information Leaflet

- Parent-friendly language
- Focusing on typical motor developmental milestones and warning signs at each age/stage from birth to 3 years
- Does not try to pre-judge or diagnose
- Encouraging parents to speak with their doctor or Health Visitor if they have any concerns

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT

Screening Tools

- Qualitative Assessment of General Movements (GMs or Prechtel) – at 3 months of age
- Hammersmith Infant Neurological Exam (HINE)
- Bayley Scale of Infant and Toddler Development
- Cranial Ultrasound
- MRI scan
- CT Scan
- EEG

ACTION CEREBRAL PALSY | IDENTIFY INTERVENE IMPACT

Early Intervention - Referral Routes

Cerebral palsy and associated neurodisabilities have been widely demonstrated to be highly responsive to early intervention, when plasticity (ability to change) in the child's developing brain is at its greatest.

Statutory Services

Non-Statutory Services – No need for a referral

Further Information and Resources

- Types and Presentations of Cerebral Palsy
- Health-related links
- General Information links
- Equipment and resources
- CPD and SEND Professional Resources

Please also see our website: www.actioncp.org

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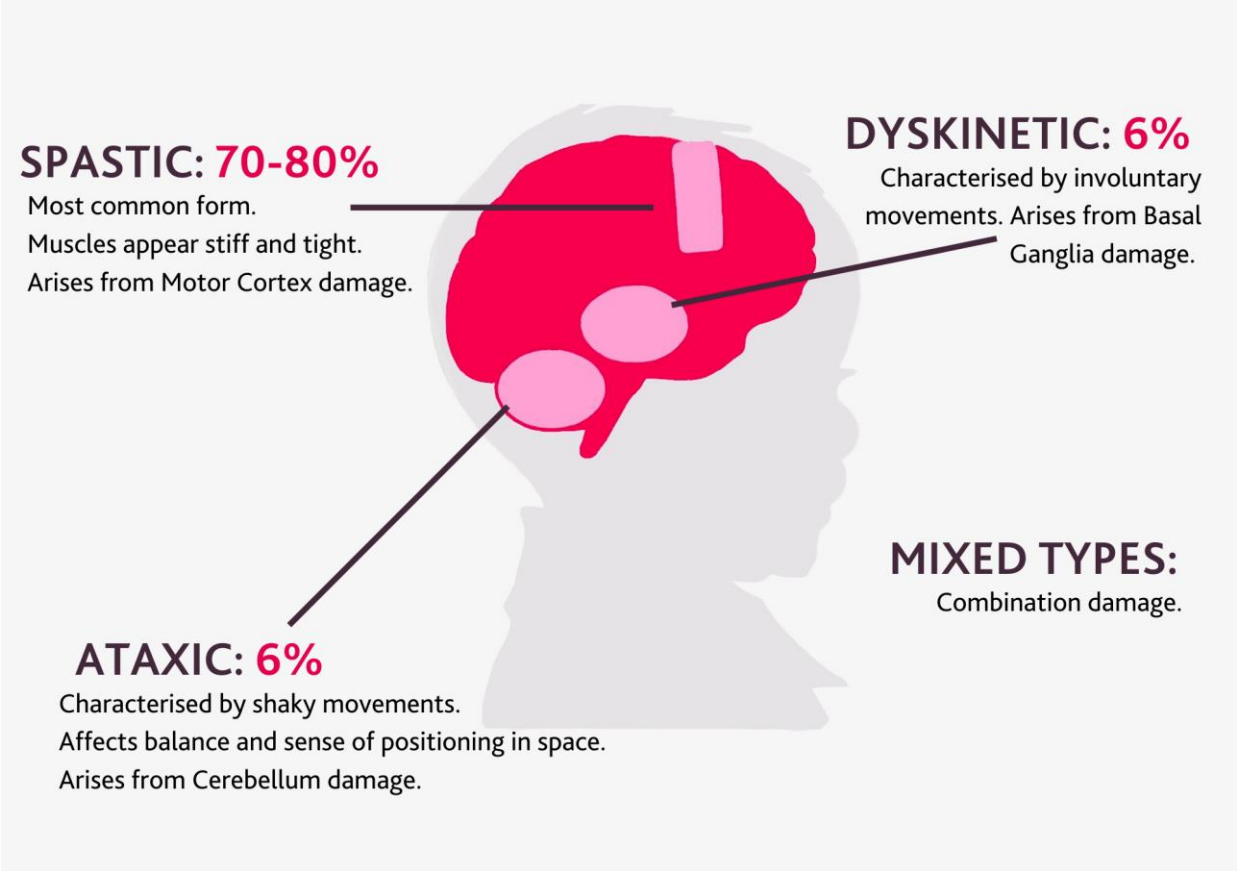
Registered Charity Number: 1165217

Types of Cerebral Palsy



The Gross Motor Function Classification System is often used to describe the level of complexity affecting a child with cerebral palsy. Gross Motor Function Classification System (GMFCS) | Cerebral Palsy Alliance

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Presentations of Cerebral Palsy

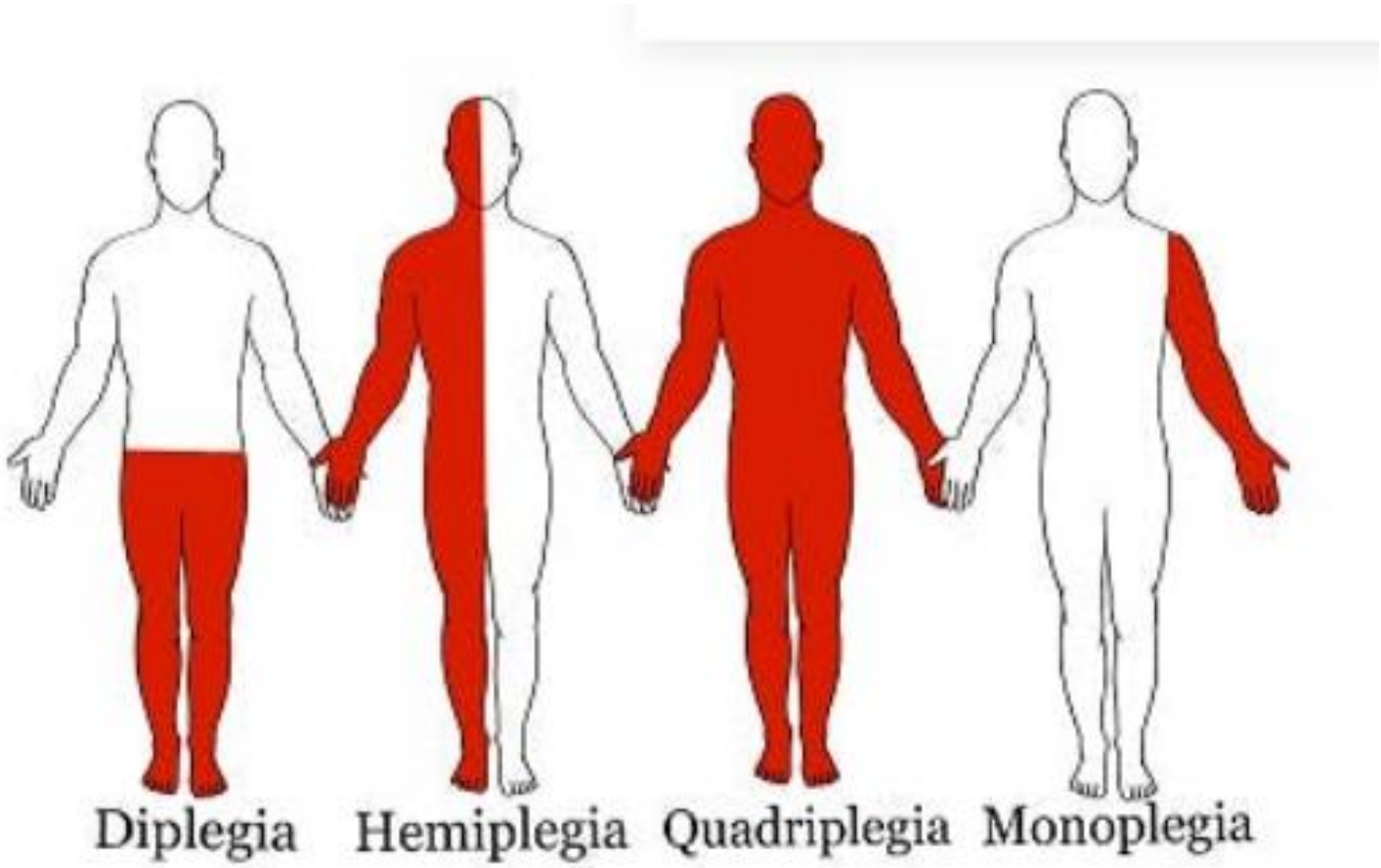

Diagram courtesy of Flint Rehab

Health

- Cerebral palsy - NHS (www.nhs.uk)
- Cerebral Palsy: The Basics – YouTube
- Cerebral palsy in under 25s: assessment and management | Guidance | NICE
- Gross Motor Function Classification System (GMFCS) | Cerebral Palsy Alliance
- How to care for children with complex needs - NHS (www.nhs.uk)
- children-young-people-complex-health-needs.pdf (mentalhealth.org.uk)

General Information

- Action Cerebral Palsy (actioncp.org)
- Conductive Education centres | PCA | Professional Conductors Association (cepeg.org.uk)
- Home - Ei SMART
- Local Offer from your Council
- We’re here to make life better for carers - Carers UK
- Contact - for families with disabled children | Contact
- Home | Disability charity Scope UK
- Home | CP Sport | Cerebral Palsy Sport
- Meeting and Greeting Someone with Cerebral Palsy (abilities.com)

Resources and Equipment

- Toys for Children with Cerebral Palsy That Improve Mobility (flintrehab.com)
- Sensory Direct | Weighted Blankets, Sensory Toys & Equipment
- Special Needs Equipment | Paediatric Equipment by Smirthwaite
- Sensory Education sensory toys,sensory toys uk,special needs toys,educational resources,sensory toys
- Inclusive Technology - All the Help You Need
- LDA - SEN, special educational needs supplies & resources | LDA Resources (ldalearning.com)
- Home - Sensory Guru Ltd
- Toys for those with Special Needs - Special Needs Toys (fatbraintoys.com)
- TTS | School Supplies for Primary, Secondary & Early Years (tts-group.co.uk)
- The UK's Leading Sensory Company | Snoezelen® Multi-Sensory Environments and Sensory Equipment | Rompa

CPD and Professional Resources

For Healthcare Professionals

- Training in Early Detection for Early Intervention course at Newcastle University - Dr. Anna Basu:
  - TEDEI | Study With Us | Newcastle University (ncl.ac.uk)'How to Manage: Recognising Neuromuscular Disorders' - Dr. Henriette Van Ruiten
- PTC Therapeutics - Improving Neuromuscular Disorders Diagnosis Through e-Learning (ptcbio.com)
- Functional Neuroanatomy | Cambridge Advance Online

For Social Care Professionals

- Disability Matters has a range of e learning courses on best practice for people working with individuals with disabilities
  - https://www.e-lfh.org.uk/programmes/disability-matters/
  - https://www.e-lfh.org.uk/wp-content/uploads/2017/07/Easy-Read-Disability-Matters-Overview-only-21.01.2016-AB-edit.pdf

For Education Professionals

- pdnet Training – pdnet – Network for supporting learners with physical disability
- For SEN professionals and private organisations | (IPSEA) Independent Provider of Special Education Advice

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www.actioncp.org

info@actioncp.org

actioncerebralpalsy

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Next speaker

Tori Blake

Parent with lived experience

Type your questions as you think of them during the presentations

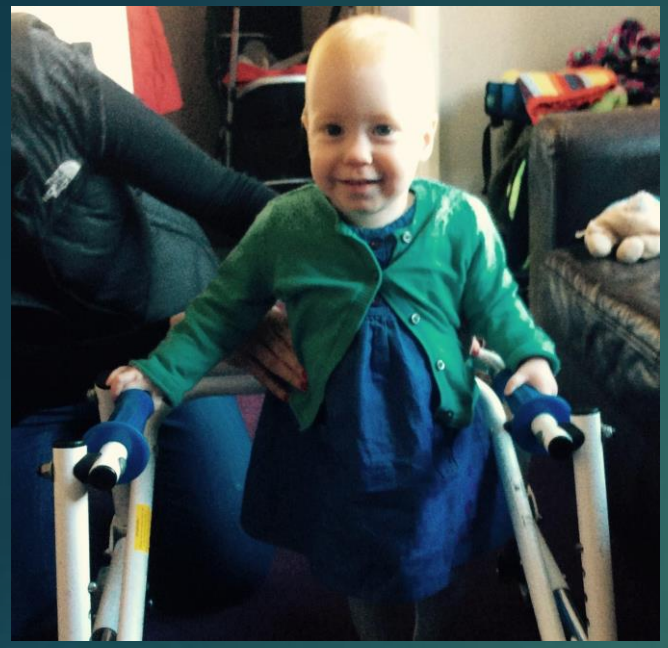
Use the Zoom Q&A function

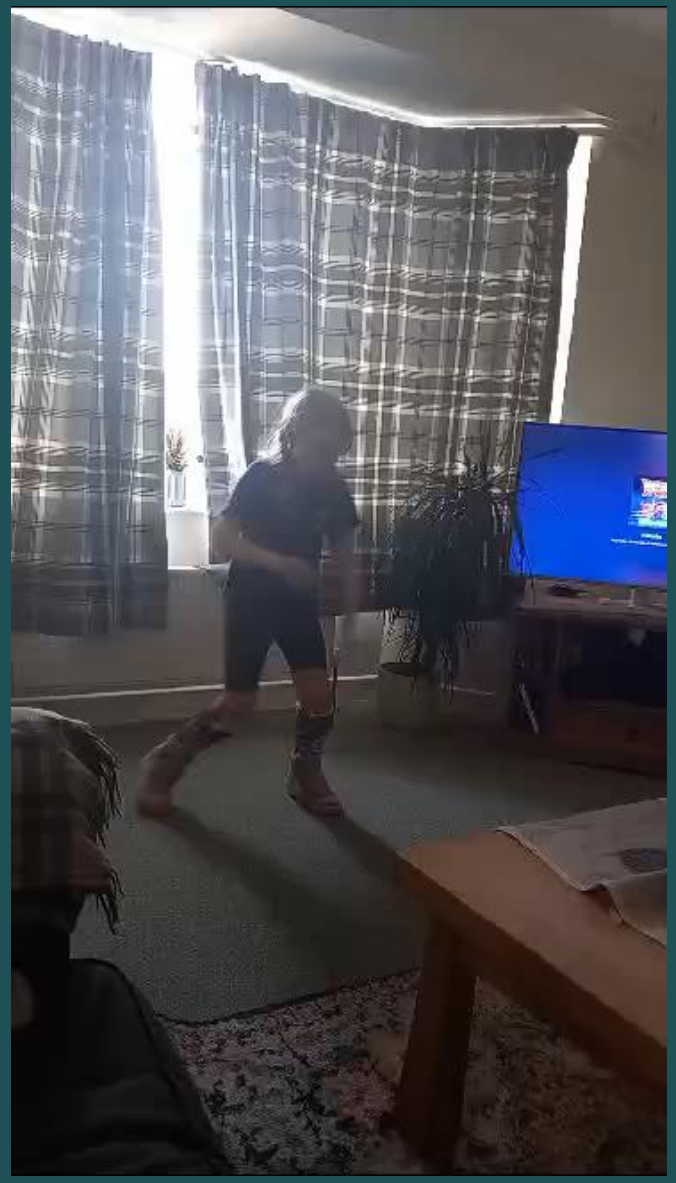
- **Name of presenter** you would like to answer your question
- Brief **question**
- **Your name** (if you wish to do so)

Welcome to Elsie's story


Bumbo


DVD
£10
Malt Easter
Mini Bunnies
THE GOOD DINOSAUR
farmyard
heads & tails
ORCHARD TOYS





Next speaker

Victoria Jackson

Senior Programme Manager - Innovation and Research, iHV

Type your questions as you think of them during the presentations

Use the Zoom Q&A function

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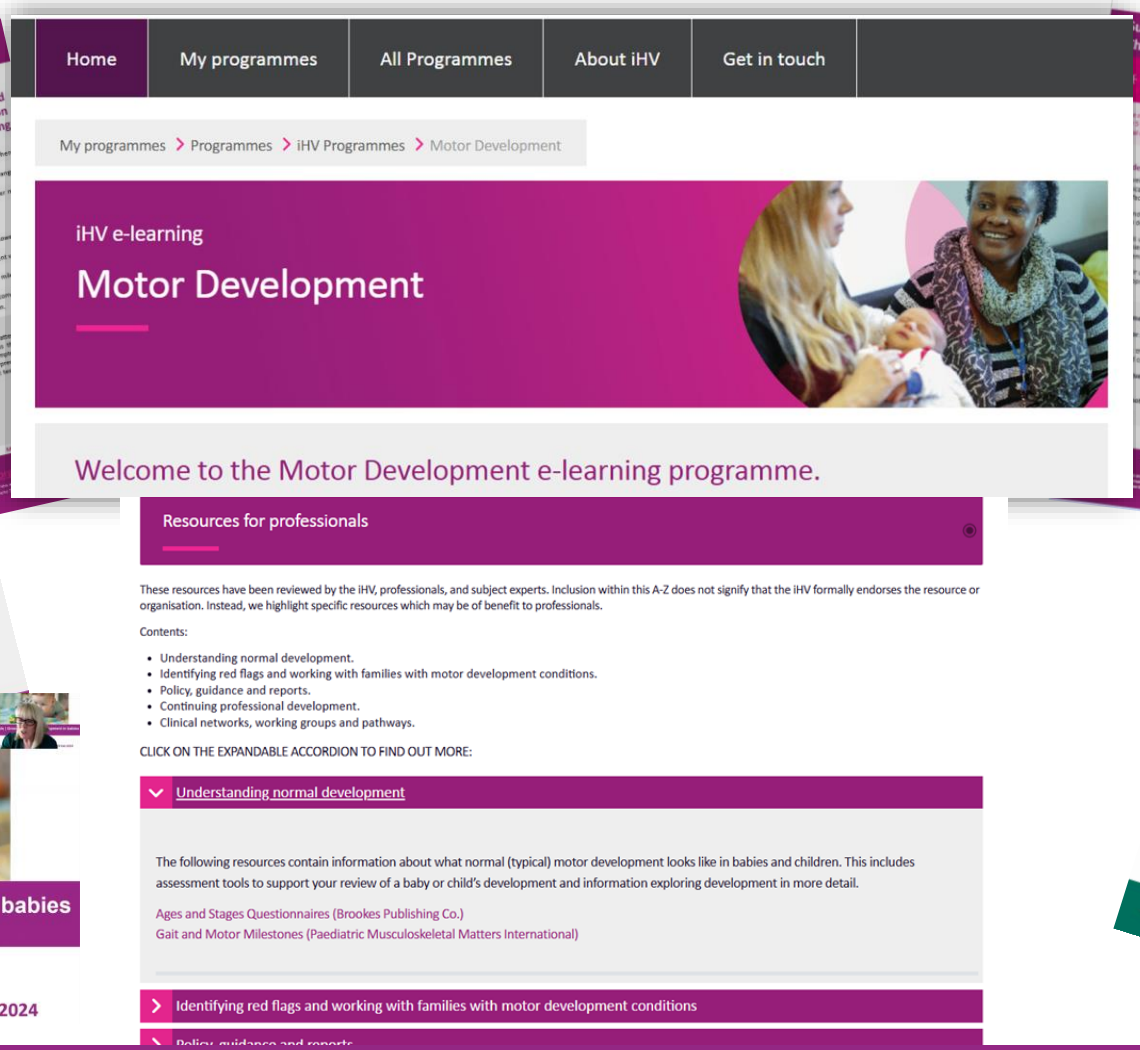
- Brief **question**
- **Your name** (if you wish to do so)

iHV Motor Development Resources

MOTOR DEVELOPMENT TOOLKIT

iHV Insights



#iHVInsights

Child Development: Launching iHV's Motor Development Toolkit

Q&A

We will aim to answer as many questions as possible during the live session.

Use the Zoom Q&A function

- **Name of presenter** you would like to answer your question
- Brief **question**
- **Your name** (if you wish to do so)

Over to you!

Help us to tailor our events to meet your needs!

- Submit your anonymised responses to the poll
- What more can we do to support you in your practice?
- Email events@ihv.org.uk with any suggestions

Thank you so much for joining us

Continue your CPD!

After this event you will be able to access the following resources on the iHV website:

- A recording of this live session (no recording on other devices is allowed)
- This slide set
- Links and background reading
- A link to download a Record of Attendance & reflection template

Log in at www.ihv.org.uk and head to the Insights page

Future iHV Member Benefit Event Dates

TOPIC	DATE
iHV Insights | Public health role in reducing A&E attendances	16 January 2025
iHV webinar | Tools to support conversations with families – applying the Family Partnership Model	30 January 2025
SCPHN Student network event	13 February 2025

All future dates can be found at:
bit.ly/4enHn2H

Join us again!

IHV Insights

Public health role in reducing A&E attendances

16 January 2025 at 3:30pm

#iHVInsights

www.ihv.org.uk

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Wednesday 4 December 2024 | London

iHV Leadership Conference

Hybrid Conference